

作成日：2025 年 8 月 7 日

ゾルゲンスマ点滴静注 特定使用成績調査 (COAV101A11401, 脊髄性筋萎縮症) の中間集計結果

最新情報に基づき、薬剤を適正かつ安全にご使用いただくために、ゾルゲンスマ点滴静注 特定使用成績調査 (COAV101A11401, 脊髄性筋萎縮症) の中間集計結果を、ノバルティスファーマ株式会社 医療関係者向け情報サイトに掲載致しました。

＜留意点＞

- 2020 年 5 月～2025 年 5 月の間で収集された情報です
- 中間の結果であるため、今後、結果は更新されます
- 安全性情報を掲載しています

添付文書 2025 年 5 月改訂（第 7 版）※ 抜粋

【効能又は効果】

脊髄性筋萎縮症

ただし、抗 AAV9 抗体が陰性の患者に限る

【用法及び用量】

通常、体重 2.6kg 以上の患者（2 歳未満）には、 1.1×10^{14} ベクターゲノム（vg）/kg を 60 分かけて静脈内に単回投与する。本品の再投与はしないこと。本品の投与液量は下記表に従い体重に基づき算出する。

患者の体重（kg）	投与液量（mL） ^{注）}
2.6 – 3.0	16.5
3.1 – 3.5	19.3
3.6 – 4.0	22
4.1 – 4.5	24.8
4.6 – 5.0	27.5
5.1 – 5.5	30.3
5.6 – 6.0	33
6.1 – 6.5	35.8
6.6 – 7.0	38.5
7.1 – 7.5	41.3
7.6 – 8.0	44
8.1 – 8.5	46.8
8.6 – 9.0	49.5
9.1 – 9.5	52.3
9.6 – 10.0	55
10.1 – 10.5	57.8
10.6 – 11.0	60.5
11.1 – 11.5	63.3
11.6 – 12.0	66
12.1 – 12.5	68.8
12.6 – 13.0	71.5
13.1 – 13.5	74.3

注）投与液量は体重幅の上限値に基づき算出した。

2 歳未満で 13.6kg 以上の患者には、体重に基づき投与液量を算出すること。

※本剤の使用に際しては、最新の添付文書をご参照ください。

当該調査単位期間終了日：2025 年 5 月 23 日

結果の概要

製造販売後調査の標題	ゾルゲンスマ点滴静注 特定使用成績調査（脊髄性筋萎縮症，COAV101A11401） ¹⁾
調査の課題及び目的	脊髄性筋萎縮症患者を対象としたゾルゲンスマ点滴静注の長期安全性及び有効性を検討する。
調査デザイン	前向き，多施設共同，単群，全例調査方式，非介入の観察研究
調査項目	患者の臨床的特性，本品の投与状況，プレドニゾロン及び他 SMA 治療薬の投与状況，有害事象 ²⁾ ，有効性（発達マイルストーン，CHOP-INTEND，HINE-2，HFMSE 等）
結果	当該調査開始日（2020 年 5 月 18 日）から登録期限（2023 年 6 月 30 日）までに 80 例が登録され，データカットオフ日（2025 年 5 月 23 日）までに 80 例の調査票データが収集された。このうち適応外症例はなく，80 例を安全性解析対象症例とした（Table 14.1.0.jpn）。 本品投与後のフォローアップ期間の中央値（範囲）は 43.71 カ月（12.6 カ月～59.9 カ月）であった（Table 14.1.6.age3.jpn）。調査を継続している症例の割合は 91.3%（73 例），調査を中止した症例の割合は 8.8%（7 例）であった。中止理由は Lost to Follow-up（4 例），他の臨床試験への参加（2 例），死亡（1 例）であった。SMA 治療歴は，本品が初回治療となる症例（OAV101 mono）は 30.0%（24 例），ヌシネルセン又はリスジブラムの短期投与後³⁾に本品に切り替えた症例（Bridge to OAV101）の割合は 35.0%（28 例），ヌシネルセン又はリスジブラムを長期投与後⁴⁾に本品に切り替えた症例（Switch to OAV101）は 18.8%（15 例），本品投与後に初めてヌシネルセン又はリスジブラムを投与し継続している症例（Add-on）は 2.5%（2 例），本品投与後に初めてヌシネルセン又はリスジブラムを投与し，その後投与を中止した症例（Transient Add-on）は 1.3%（1 例），ヌシネルセン又はリスジブラムを投与後に本品を投与し，再びヌシネルセン又はリスジブラムを投与し継続している症例（Combo w/OAV101）が 12.5%（10 例）であった（Table 14.1.1.age3.jpn）。なお，死亡例はリスジブラムを長期投与後⁴⁾に本品に切り替えたため，本品投与後にリスジブラムを死亡前日まで投与していたが，Combo w/OAV101 ではなく，Switch to OAV101 として集計した（Listing 16.1.jpn, Listing 16.3.jpn, Listing 16.4.jpn）。 Demography & Medical history SMA と遺伝子診断された時点の年齢（月齢）の中央値（範囲）は 6.0 カ月（0 カ月～24 カ月）であった。生存運動ニューロン 2（survival motor neuron 2, SMN2）遺伝子コピー数は，2 コピーが 50.0%，3 コピーが 50.0%であった（Table 14.1.3.age3.jpn）。男児の割合は 52.5%，女児の割合は 47.5%であった（Table 14.1.2.age3.jpn）。 OAV101 Treatment 本品投与時の年齢（月齢）⁵⁾の中央値（範囲）は 10.0 カ月（0 カ月～24 カ月）であった（Table 14.1.6.age3.jpn）。データカットオフ時点の年齢（月齢）の中央値（範囲）は 57.62 カ月（22.8 カ月～83.3 カ月）であった（Table 14.1.2.age3.jpn）。 本品投与後にヌシネルセン又はリスジブラムを追加投与した症例は 14 例であった（ヌシネルセン 1 例，リスジブラム 13 例）。ヌシネルセンを追加投与した理由は，薬効欠如（1 例）であり，リスジブラムを追加投与した理由は，薬効欠如（4 例），有害事象（2 例），保護者・介護者・患者による意思決定（2 例），利用可能な代替治療（1 例），その他（1 例）であった（Table 14.1.7.txn.jpn, Table 14.1.9.txr.jpn, Listing 16.1.jpn）。なお，いずれの症例も本品投与後に発達マイルストーンの消失は確認されていない（Listing 16.4.jpn）。 グルココルチコステロイドを投与した症例は 80 例であり，全例がプレドニゾロンを投与していた。プレドニゾロンの投与期間の中央値（範囲）は 2.69 カ月（1.0 カ月～7.5 カ月）であった（Table 14.1.10.cpy.jpn）。

	Safety 80 例全例に有害事象が発現し、最も多く報告された有害事象は発熱が 81.3% (65 例)、次いでアスパラギン酸アミノトランスフェラーゼ (AST) 増加が 66.3% (53 例)、及びアラニンアミノトランスフェラーゼ (ALT) 増加が 65.0% (52 例) であった (Table 14.3.1.1.oav.age3.jpn)。 初発の有害事象の発現時期は、本品投与後 2 週間以内が多く、80 例中 77 例で認められた (Table 14.3.1.1.2.oav.aeptin.jpn)。また、CTCAE Grade3 以上の有害事象の発現割合は、81.3% (65 例) であった (Table 14.3.1.0.oav.age3.jpn)。 副作用の発現割合は 98.8% (79 例) であり、最も多く報告された副作用は発熱が 80.0% (64 例)、次いで AST 増加が 66.3% (53 例)、及び ALT 増加が 62.5% (50 例) であった (Table 14.3.1.4.oav.age3.jpn)。 重篤な有害事象の発現割合 68.8% (55 例) であり、最も多く報告された重篤な有害事象は、肺炎が 16.3% (13 例)、次いで誤嚥性肺炎が 12.5% (10 例)、及び AST 増加が 11.3% (9 例) であった (Table 14.3.1.3.oav.age3.jpn)。 重篤な副作用の発現割合は 26.3% (21 例) であった。最も多く報告された重篤な副作用は、AST 増加が 11.3% (9 例)、次いで ALT 増加が 10.0% (8 例)、及び血栓性微小血管症 (TMA) が 5.0% (4 例) であった (Table 14.3.1.5.oav.age3.jpn)。 死亡した症例は 1 例で主な死因は呼吸不全であった (Listing 16.3.jpn)。本症例は、本品投与後 482 日に心肺停止を発現し、同日死亡した。なお、本品との因果関係は否定された。(Listing 16.1.jpn)。 特に注目すべき有害事象の発現割合は、肝障害が 90.0% (72 例)、一過性の血小板減少症が 62.5% (50 例)、TMA が 5.0% (4 例)、心臓関連有害事象が 32.5% (26 例)、神経障害の新規発現が 2.5% (2 例)、自己免疫疾患の新規発現が 1.3% (1 例)、血液疾患の新規発現が 16.3% (13 例) であった。なお、特に注目すべき有害事象のうち、神経節障害に関連する感覚異常、悪性腫瘍の新規発現は認められなかった (Table 14.3.1.6.1.age3.jpn)。 特に注目すべき副作用の発現割合は、肝障害が 88.8% (71 例)、一過性の血小板減少症が 62.5% (50 例)、TMA が 5.0% (4 例)、心臓関連有害事象が 32.5% (26 例)、神経障害の新規発現が 2.5% (2 例)、自己免疫疾患の新規発現が 1.3% (1 例)、血液疾患の新規発現が 16.3% (13 例) であった。なお、特に注目すべき有害事象のうち、神経節障害に関連する感覚異常、悪性腫瘍の新規発現は認められなかった (Table 14.3.1.6.2.age3.jpn)。 特に注目すべき副作用のうち、重篤な副作用として報告されたのは肝障害が 18.8% (15 例)、一過性の血小板減少症が 6.3% (5 例)、TMA が 5.0% (4 例)、心臓関連有害事象が 5.0% (4 例) (TMA 4 例と同一症例)、血液疾患の新規発現が 7.5% (6 例) (TMA 2 例、一過性の血小板減少症 2 例、TMA 及び一過性の血小板減少症 2 例) であった。1 例 (TMA、心臓関連有害事象、血液疾患の新規発現) は回復後、後遺症ありと報告されているが、それ以外の症例はすべて回復した (Table 14.3.1.6.4.age3.jpn, Listing 16.1.2.jpn)。 Effectiveness 有効性の結果は再審査結果通知書受領後に本報告書に記載する。
結論	本調査は実施中であり、本品の長期的な安全性及び有効性を結論づけることは困難であるものの、報告された副作用の特性は、本品投与後に発現が予測される事象が主であり、安全性検討事項を含め新たな安全性の懸念はなかった。今後も本品の安全性情報を収集し、新たな懸念事項が認められた場合には適切な措置を講じることとする。 有効性の結果は再審査結果通知書受領後に本報告書に記載する。
備考	添付資料 1：解析結果 添付資料 2：安全性検討事項の各リスクの定義

CHOP INTEND : Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders

HFMSE : Hammersmith Functional Motor Scale Expanded

HINE-2 : Hammersmith Infant Neurological Examination – Section 2

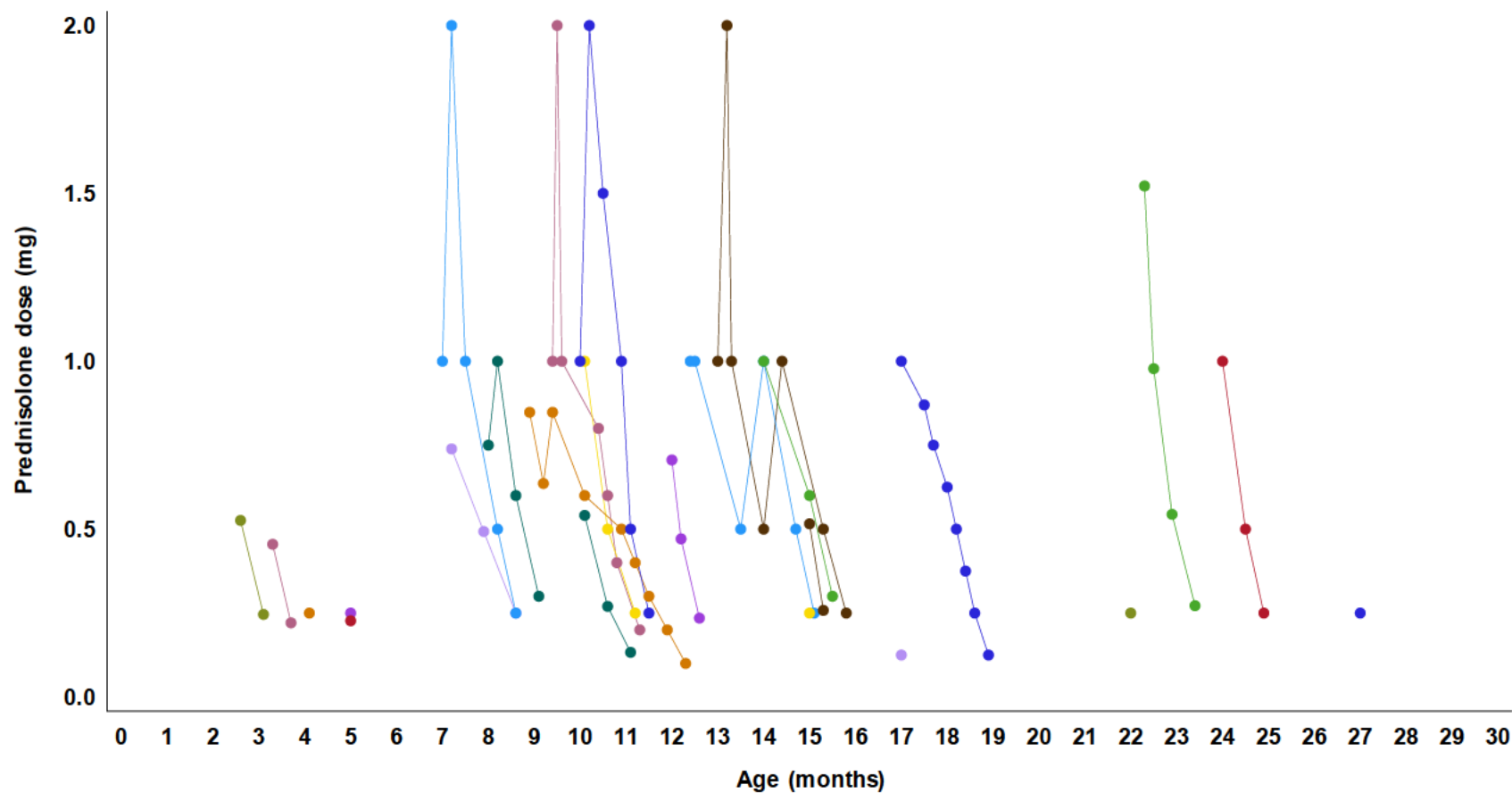
- 1) 本調査はSMAと診断された患者を対象とした長期観察の疾患レジストリであるRESTORE (ClinicalTrials.gov Identifier: NCT04174157) の一部として日本で実施している。
- 2) 本品投与開始後12ヵ月後まではすべての有害事象を収集し、残りの調査期間中は、重篤な有害事象、特に注目すべき有害事象 [肝障害、神経節障害に関連する感覚異常、心臓関連有害事象、一過性の血小板減少症、血栓性微小血管症 (TMA)、遺伝子治療に関連する遅発性の有害事象 (悪性腫瘍の新規発現、自己免疫疾患の新規発現、神経障害の新規発現、血液疾患の新規発現)]、非重篤な副作用のみ収集
- 3) 短期間；ヌシネルセン：負荷投与まで、リスジプラム：3ヵ月投与まで
- 4) 長期間；ヌシネルセン：維持期の投与、リスジプラム：3ヵ月を超えた投与
- 5) 集計には本品投与時月齢が 24 ヲ月を超えた結果が含まれているが、実際には 24 ヲ月齡未満で投与されていることを確認している。これは本品投与時月齡を算出するための情報が不完全であるため、データが補完されていることによるものである。

Figure 3.jpg	Spaghetti Chart of Prednisolone Dose (mg) Over Age Japan OAV101 Treated Patients
Listing 16.1.2.jpg	Listing of Related Serious Adverse Events Japan OAV101 Treated Patients
Listing 16.1.jpg	Listing of Adverse Events of Special Interest Japan OAV101 Treated Patients
Listing 16.2.jpg	Listing SMA Japan OAV101 Treated Patients that were Screened for SMA as a Newborn
Listing 16.3.1.jpg	Listing of Tracheostomy History Japan OAV101 Treated Patients
Listing 16.3.jpg	Listing of Patient Deaths Japan OAV101 Treated Patients
Listing 16.4.1.jpg	Listing of Laboratory Values at Baseline Japan OAV101 Treated Patients
Listing 16.4.2.jpg	Listing of Clinically Significant Laboratory Values Japan OAV101 Treated Patients
Listing 16.4.jpg	Listing of Lack of Drug Effect Japan OAV101 Treated Patients that Switched Treatment due to Lack of Drug Effect
Listing 16.10.jpg	Listing of Lost to Follow-Up Patients Japan OAV101 Treated Patients
Listing 16.11.jpg	Listing of Patients Excluded from the Japan Safety Analysis Set All Japan Enrolled Patients
Listing 16.13.jpg	Listing of Japan OAV101 Treated Patients Japan OAV101 Treated Patients
Table 14.1.0.jpg	Analysis Datasets All Japan Enrolled Patients
Table 14.1.1.age3.jpg	Patient Enrollment and Disposition by Age at OAV101 Infusion Japan Analysis Set
Table 14.1.2.age3.jpg	Patient Demographics by Age at OAV101 Infusion Japan OAV101 Treated Patients
Table 14.1.3.age3.jpg	SMA Medical History by Age at OAV101 Infusion Japan OAV101 Treated Patients
Table 14.1.6.age3.jpg	OAV101 Treatment by Age at OAV101 Infusion Japan OAV101 Treated Patients
Table 14.1.6.g5tx.jpg	OAV101 Treatment by Therapy at OAV101 Infusion Japan OAV101 Treated Patients
Table 14.1.7.cpy.jpg	Nusinersen Treatment by Number of Copies of the SMN2 Gene Japan OAV101 Treated Patients
Table 14.1.7.txn.jpg	Nusinersen Treatment by Therapy Japan OAV101 Treated Patients
Table 14.1.8.age3.jpg	AAV9 Antibody Testing Results by Age at OAV101 Infusion Japan OAV101 Treated Patients
Table 14.1.9.cpy.jpg	Risdiplam Treatment by Number of Copies of the SMN2 Gene Japan OAV101 Treated Patients
Table 14.1.9.txr.jpg	Risdiplam Treatment by Therapy Japan OAV101 Treated Patients
Table 14.1.10.cpy.jpg	Glucocorticosteroid Treatment by Number of Copies of the SMN2 Gene Japan OAV101 Treated Patients

[illegible]

Table 14.3.1.2.2.oav.age3.jpn	OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.1.3.oav.age3.jpn	Serious OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.1.4.oav.age3.jpn	OAV101 Related Treatment Emergent Adverse Events by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.1.5.oav.age3.jpn	OAV101 Related Serious Treatment Emergent Adverse Events by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.1.6.1.age3.jpn	Events of Special Interest by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.1.6.2.age3.jpn	OAV101 Related Treatment Emergent Adverse Events of Special Interest by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.1.6.3.age3.jpn	Serious Events of Special Interest by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.1.6.4.age3.jpn	OAV101 Serious Related Events of Special Interest by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.2.age3.jpn	Clinically Significant Abnormal Laboratory Results any Time During Follow-up by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.3.jpn	Patients with Elevated AST or ALT After OAV101 Infusion Japan Safety Analysis Set

Figure 3.jpj
Spaghetti Chart of Prednisolone Dose (mg) Over Age
Japan OAV101 Treated Patients



Note. Figure includes only patients with a recorded age for the prednisolone dose measurement.

Listing 16.1.2.jpn
Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	6	No/ 70	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	6	No/ 70	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE					Proteinuria/ Proteinuria/ Renal and urinary disorders	7	Yes/ -	Grade 3/ Related to OAV101/ None/ Not recovered/not resolved	Yes/ 2, 3, 6

^a A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy

Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical
intervention to prevent one of the outcomes listed above.

AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 5.0), SOC = System Organ Class,
SAE = Serious Adverse Event.

Listing 16.1.2.jpn
Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Transient Thrombocytopenia	Hematuria/ Haematuria/ Renal and urinary disorders	7	No/ 35	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3, 6
RESTORE				Thrombotic Microangiopathy, Cardiac, New Incidence of Hematological Disorder	thrombotic microangiopathy/ Thrombotic microangiopathy/ Blood and lymphatic system disorders	7	No/ 42	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3, 6
RESTORE					vomit/ Vomiting/ Gastrointestinal disorders	3	No/ 5	Grade 2/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

^a A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy

Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical
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AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 5.0), SOC = System Organ Class,
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Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	fever/ Pyrexia/ General disorders and administration site conditions	4	No/ 5	Grade 2/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE					Increased ALT/ Alanine aminotransferase increased/ Investigations	4	No/ 359	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE					Increased AST/ Aspartate aminotransferase increased/ Investigations	4	No/ 359	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

^a A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy

Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

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RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	5	No/ 162	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	5	No/ 162	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	4	No/ 72	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

^a A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy

Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

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AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 5.0), SOC = System Organ Class, SAE = Serious Adverse Event.

Listing 16.1.2.jpn
Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				New Incidence of Hematological Disorder	haemolytic Anemia/ Haemolytic anaemia/ Blood and lymphatic system disorders	6	No/ 30	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Thrombotic Microangiopathy, Cardiac, New Incidence of Hematological Disorder	thrombotic microangiopathy/ Thrombotic microangiopathy/ Blood and lymphatic system disorders	6	No/ 31	Grade 4/ Related to OAV101/ None/ Recovered/resolved with sequelae	Yes/ 2, 3, 4, 6
RESTORE				Transient Thrombocytopenia	platelet count decreased/ Platelet count decreased/ Investigations	6	No/ 34	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3, 4, 6

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Listing 16.1.2.jpn
Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE					acute kidney injury/ Acute kidney injury/ Renal and urinary disorders	7	No/ 99	Grade 4/ Related to OAV101/ None/ Recovered/resolved with sequelae	Yes/ 2, 3, 4, 6
RESTORE				Cardiac	Cardiac failure congestive/ Cardiac failure congestive/ Cardiac disorders	15	No/ 21	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3, 6
RESTORE				Hepatotoxicity	AST elevated./ Aspartate aminotransferase increased/ Investigations	4	No/ 15	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

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RESTORE				Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	8	No/ 15	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE					Pyrexia/ Pyrexia/ General disorders and administration site conditions	4	No/ 5	Grade 2/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	AST increased/liver enzymes increased/ Aspartate aminotransferase increased/ Investigations	4	No/ 22	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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Listing 16.1.2.jpn
Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	ALT increased/liver enzymes increased/ Alanine aminotransferase increased/ Investigations	6	No/ 22	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE					Ferritin high/ Serum ferritin increased/ Investigations	6	No/ 22	Grade 2/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	AST elevated/ Aspartate aminotransferase increased/ Investigations	4	No/ 7	Grade 1/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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Japan OAV101 Treated Patients

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RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	5	No/ 13	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	5	No/ 17	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE					Neutrophil count decreased/ Neutrophil count decreased/ Investigations	4	No/ 19	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6

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Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	4	No/ 228	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6
RESTORE				Transient Thrombocytopenia	PLT decreased/ Platelet count decreased/ Investigations	8	No/ 17	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	7	No/ 120	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6

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Listing 16.1.2.jpn
Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

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RESTORE				Thrombotic Microangiopathy, Cardiac, New Incidence of Hematological Disorder	Thrombotic microangiopathy/ Thrombotic microangiopathy/ Blood and lymphatic system disorders	7	No/ 34	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3
RESTORE				Hepatotoxicity	elevated liver enzyme/ Hepatic enzyme increased/ Investigations	6	No/ 15	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	liver dysfunction/ Hepatic function abnormal/ Hepatobiliary disorders	6	No/ 20	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6

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Listing of Related Serious Adverse Events
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RESTORE				Transient thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	4	No/ 9	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	4	No/ 85	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	7	No/ 85	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6

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Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE					Renal impairment/ Renal impairment/ Renal and urinary disorders	11	No/ 16	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Thrombotic Microangiopathy, Cardiac, New Incidence of Hematological Disorder	Thrombotic microangiopathy/ Thrombotic microangiopathy/ Blood and lymphatic system disorders	11	No/ 16	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	Impaired liver function/ Hepatic function abnormal/ Hepatobiliary disorders	5	No/ 78	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	Multi organ failure/ Multiple organ dysfunction syndrome/ General disorders and administration site conditions	51	No/ 71	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2
RESTORE					Acute liver failure/ Acute hepatic failure/ Hepatobiliary disorders	52	No/ 73	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3

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Listing 16.1.jpj
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Transient Thrombocytopenia	plt decreased/ Platelet count decreased/ Investigations	6	No/ 13	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	6	No/ 70	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	6	No/ 70	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	189	No/ 203	Grade 2/ Unrelated/ None/ Recovered/resolved	No

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Listing 16.1.jpj
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	189	No/ 203	Grade 2/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	230	No/ 267	Grade 2/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	230	No/ 267	Grade 2/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Cardiac	Hypertension/ Hypertension/ Vascular disorders	7	No/ 28	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

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RESTORE				Transient Thrombocytopenia	Hematuria/ Haematuria/ Renal and urinary disorders	7	No/ 35	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3, 6
RESTORE				Thrombotic Microangiopathy, Cardiac, New Incidence of Hematological Disorder	thrombotic microangiopathy/ Thrombotic microangiopathy/ Blood and lymphatic system disorders	7	No/ 42	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3, 6
RESTORE				Transient Thrombocytopenia	Bleeding from the peritoneal tube insertion site/ Medical device site haemorrhage/ General disorders and administration site conditions	15	No/ 16	Grade 3/ Unrelated/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

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RESTORE				New Incidence of Neurological Disorder	Cerebral atrophy/ Cerebral atrophy/ Nervous system disorders	21	No/ 210	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Increased ALT/ Alanine aminotransferase increased/ Investigations	4	No/ 359	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	Increased AST/ Aspartate aminotransferase increased/ Investigations	4	No/ 359	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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RESTORE				Transient thrombocytopenia	decreased platelet count/ Platelet count decreased/ Investigations	7	No/ 10	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	5	No/ 162	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	5	No/ 162	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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RESTORE				Transient Thrombocytopenia	Platelet decreased/ Platelet count decreased/ Investigations	8	No/ 12	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	hepatic function abnormal/ Hepatic function abnormal/ Hepatobiliary disorders	4	No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	5	No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	4	No/ 72	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Cardiac	NT-proBNP increased/ N-terminal prohormone brain natriuretic peptide increased/ Investigations	5	No/ 20	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	5	No/ 105	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity	hepatic enzyme increased/ Hepatic enzyme increased/ Investigations	4	No/ 395	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	6	No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				New Incidence of Hematological Disorder	haemolytic Anemia/ Haemolytic anaemia/ Blood and lymphatic system disorders	6	No/ 30	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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Listing 16.1.jpj
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Thrombotic Microangiopathy, Cardiac, New Incidence of Hematological Disorder	thrombotic microangiopathy/ Thrombotic microangiopathy/ Blood and lymphatic system disorders	6	No/ 31	Grade 4/ Related to OAV101/ None/ Recovered/resolved with sequelae	Yes/ 2, 3, 4, 6
RESTORE				Transient Thrombocytopenia	platelet count decreased/ Platelet count decreased/ Investigations	6	No/ 34	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3, 4, 6
RESTORE				Cardiac	CPK increased/ Blood creatine phosphokinase increased/ Investigations	6	No/ 57	Grade 1/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	6	No/ 330	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

^a A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy

Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

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Japan OAV101 Treated Patients

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RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	6	No/ 330	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	hematuria/ Haematuria/ Renal and urinary disorders	7	No/ 169	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	hypertension/ Hypertension/ Vascular disorders	8	Yes/ -	Grade 2/ Related to OAV101/ None/ Not recovered/not resolved	No
RESTORE				Cardiac	cardiac troponin T increased/ Troponin T increased/ Investigations	14	No/ 57	Grade 1/ Unrelated/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

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RESTORE				Cardiac	cardiac troponinI increased/ Troponin I increased/ Investigations	14	No/ 57	Grade 1/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Cardiac	Cardiac failure congestive/ Cardiac failure congestive/ Cardiac disorders	15	No/ 21	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3, 6
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	7	No/ 14	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	7	No/ 14	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	CPK increased/ Blood creatine phosphokinase increased/ Investigations	7	No/ 14	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Platelets decreased/ Platelet count decreased/ Investigations	7	No/ 14	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	56	No/ 133	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	56	No/ 133	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	platelet decreased/ Platelet count decreased/ Investigations	6	No/ 11	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	6	No/ 15	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	6	No/ 15	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	2	No/ 127	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Plate decreased/ Platelet count decreased/ Investigations	5	No/ 16	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	5	No/ 85	Grade 4/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST elevated./ Aspartate aminotransferase increased/ Investigations	4	No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT elevated./ Alanine aminotransferase increased/ Investigations	6	No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

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RESTORE				Transient Thrombocytopenia, New Incidence of Hematological Disorder	thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	6	No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT elevated/ Alanine aminotransferase increased/ Investigations	22	No/ 114	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST elevated./ Aspartate aminotransferase increased/ Investigations	22	No/ 114	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity	AST elevated/ Aspartate aminotransferase increased/ Investigations	4	No/ 15	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	ALT elevated/ Alanine aminotransferase increased/ Investigations	8	No/ 15	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	8	No/ 15	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Cardiac	Elevated troponin I level/ Troponin I increased/ Investigations	8	No/ 57	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				New Incidence of Hematological Disorder	anemia/ Anaemia/ Blood and lymphatic system disorders	392	No/ 448	Grade 1/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/liver enzymes increased/ Aspartate aminotransferase increased/ Investigations	4	No/ 22	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	ALT increased/liver enzymes increased/ Alanine aminotransferase increased/ Investigations	6	No/ 22	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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RESTORE				Hepatotoxicity	ALT elevated/ Alanine aminotransferase increased/ Investigations	43	No/ 85	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST elevated/ Aspartate aminotransferase increased/ Investigations	43	No/ 85	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST elevated/ Aspartate aminotransferase increased/ Investigations	4	No/ 7	Grade 1/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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RESTORE				Cardiac	elevated Troponin I./ Troponin I increased/ Investigations	7	No/ 56	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT elevated/ Alanine aminotransferase increased/ Investigations	70	No/ 252	Grade 1/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST elevated/ Aspartate aminotransferase increased/ Investigations	70	No/ 252	Grade 1/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	7	No/ 57	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

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RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	15	No/ 71	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	CPK increased/ Blood creatine phosphokinase increased/ Investigations	5	No/ 8	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	5	No/ 13	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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RESTORE				Transient thrombocytopenia	PLT decreased/ Platelet count decreased/ Investigations	5	No/ 16	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	5	No/ 17	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Cardiac	troponin I increased/ Troponin I increased/ Investigations	13	No/ 19	Grade 1/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	elevation of hepatic deactivating enzymes/ Hepatic enzyme increased/ Investigations	138	No/ 341	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity	Elevated AST (liver disorder)/ Aspartate aminotransferase increased/ Investigations	5	No/ 436	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Elevated ALT (liver disorder)/ Alanine aminotransferase increased/ Investigations	7	No/ 226	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	9	No/ 12	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity	increased/ALT increased again/elevations in serum amino transferases concentrations/ Alanine aminotransferase increased/ Investigations	5	No/ 71	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	increased/AST increased again/elevations in serum amino transferases concentrations/ Aspartate aminotransferase increased/ Investigations	5	No/ 71	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	8	No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity, Cardiac	Hepatomegaly -2 transverse finger palpable, no splenomegaly/ Hepatomegaly/ Hepatobiliary disorders	4	No/ 4	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Hepatic function disorder (AST, ALT increased)/ Hepatic function abnormal/ Hepatobiliary disorders	6	No/ 15	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	6	No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity	Hepatic function abnormal/ Hepatic function abnormal/ Hepatobiliary disorders	29	No/ 657	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Bleeding from gastric mucosa/ Gastric haemorrhage/ Gastrointestinal disorders	36	No/ 64	Grade 1/ Unrelated/ None/ Recovered/resolved	No
RESTORE				New Incidence of Hematological Disorder	eosinophilia/ Eosinophilia/ Blood and lymphatic system disorders	1343	No/ 1454	Grade 3/ Unrelated/ None/ Recovered/resolved	Yes/ 3
RESTORE				Transient Thrombocytopenia	Plt decreased/ Platelet count decreased/ Investigations	4	No/ 11	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

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Listing 16.1.jpj
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	6	No/ 11	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	6	No/ 67	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	platelet decreased/ Platelet count decreased/ Investigations	6	No/ 14	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

^a A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy

Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical
intervention to prevent one of the outcomes listed above.

AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 5.0), SOC = System Organ Class,
SAE = Serious Adverse Event.

Listing 16.1.jpj
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/	5	No/	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
					Aspartate aminotransferase increased/		8		
					Investigations				
RESTORE				Hepatotoxicity	Alanine aminotransferase increased/	5	No/	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
					Alanine aminotransferase increased/		10		
					Investigations				
RESTORE				Transient Thrombocytopenia	Platelet count decreased/	5	No/	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
					Platelet count decreased/		19		
					Investigations				

^a A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy

Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity, 5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.

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Listing 16.1.jpj
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	hepatic enzyme increased/ Hepatic enzyme increased/ Investigations	3	No/ 21	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Platelet decreased/ Platelet count decreased/ Investigations	6	No/ 13	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	troponin I increased/ Troponin I increased/ Investigations	6	No/ 28	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	troponin I increased/ Troponin I increased/ Investigations	42	No/ 55	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Listing 16.1.jpj
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Cardiac	CK increased/ Blood creatine phosphokinase increased/ Investigations	8	No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	8	No/ 43	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	29	No/ 43	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	3	No/ 22	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	6	No/ 22	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	PLT decreased/ Platelet count decreased/ Investigations	8	No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	Troponin I increased/ Troponin I increased/ Investigations	8	No/ 71	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

^a A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy

Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

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Japan OAV101 Treated Patients

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RESTORE				Hepatotoxicity	elevated AST/ Aspartate aminotransferase increased/ Investigations	4	No/ 127	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	elavated ALT/ Alanine aminotransferase increased/ Investigations	43	No/ 127	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	4	No/ 108	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	4	No/ 108	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				New Incidence of Autoimmune Disorder	hypersensitivity/ Hypersensitivity/ Immune system disorders	14	No/ 45	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	Troponin I increased/ Troponin I increased/ Investigations	2	No/ 168	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

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RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	4	No/ 13	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	4	No/ 13	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	Nocturnal Dyspnea/ Nocturnal dyspnoea/ Respiratory, thoracic and mediastinal disorders	504	No/ 506	Grade 1/ Unrelated/ None/ Recovered/resolved	Yes/ 3

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Japan OAV101 Treated Patients

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RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	6	No/ 14	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	6	No/ 14	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	28	No/ 65	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	4	No/ 14	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	6	No/ 14	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	43	No/ 57	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	50	No/ 57	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	4	No/ 22	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	4	No/ 89	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	32	No/ 89	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

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RESTORE				Hepatotoxicity	Alanine aminotransferase increased/	5	No/	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
					Alanine aminotransferase increased/ Investigations		50		
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/	5	No/	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
					Aspartate aminotransferase increased/ Investigations		50		
RESTORE				Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	6	No/ 13	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	Hepatic function disorder/ Hepatic function abnormal/ Hepatobiliary disorders	407	No/ 435	Grade 2/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	4	No/ 19	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	5	No/ 12	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Cardiac	Cardiac troponin I increased/ Troponin I increased/ Investigations	6	No/ 33	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	61	No/ 784	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	166	No/ 196	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	Alanine aminotransferase increased/	6	No/	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
					Alanine aminotransferase increased/		122		
					Investigations				
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/	6	No/	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
					Aspartate aminotransferase increased/		122		
					Investigations				
RESTORE				Transient Thrombocytopenia	Platelets decreased/ Platelet count decreased/ Investigations	8	No/ 10	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

^a A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy

Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity, 5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.

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Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Cardiac	Creatine phosphokinase increased/ Blood creatine phosphokinase increased/ Investigations	100	No/ 162	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	5	No/ 14	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Plt decreased/ Platelet count decreased/ Investigations	5	No/ 14	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity, 5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.

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RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	5	No/ 21	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	5	No/ 20	Grade 4/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	5	No/ 20	Grade 4/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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Japan OAV101 Treated Patients

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RESTORE				Cardiac	elevated Troponin-I/ Troponin I increased/ Investigations	11	No/ 41	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	27	No/ 83	Grade 4/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	27	No/ 83	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

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RESTORE				Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	8	No/ 13	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increase/ Aspartate aminotransferase increased/ Investigations	8	No/ 89	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	8	No/ 229	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	CK-MB increased/ Blood creatine phosphokinase MB increased/ Investigations	27	No/ 166	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

^a A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy

Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical
intervention to prevent one of the outcomes listed above.

AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 5.0), SOC = System Organ Class,
SAE = Serious Adverse Event.

Listing 16.1.jp
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	AST increase/ Aspartate aminotransferase increased/ Investigations	7	No/ 132	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	GPT increase/ Alanine aminotransferase increased/ Investigations	7	No/ 132	Grade 3/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	GGT increased/ Gamma-glutamyltransferase increased/ Investigations	23	No/ 28	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Transient Thrombocytopenia	Platelets decreased/ Platelet count decreased/ Investigations	4	No/ 14	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	4	No/ 228	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	4	No/ 228	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	GGT increased/ Gamma-glutamyltransferase increased/ Investigations	20	No/ 53	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/	4	No/	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
					Aspartate aminotransferase increased/		14		
					Investigations				
RESTORE				Hepatotoxicity	Alanine aminotransferase increased/	4	No/	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
					Alanine aminotransferase increased/		21		
					Investigations				
RESTORE				Cardiac	Cardiac troponin I increased/	14	No/	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
					Troponin I increased/		40		
					Investigations				

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Listing 16.1.jpj
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	Alanine aminotransferase increased/	40	No/	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
					Alanine aminotransferase increased/		237		
					Investigations				
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/	40	No/	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
					Aspartate aminotransferase increased/		237		
					Investigations				
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/	4	No/	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
					Aspartate aminotransferase increased/		126		
					Investigations				

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Listing 16.1.jpj
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	5	No/ 63	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	4	No/ 12	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	4	No/ 22	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

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RESTORE				Transient Thrombocytopenia	PLT decreased/ Platelet count decreased/ Investigations	8	No/ 17	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6
RESTORE				Hepatotoxicity	AST elevation/ Aspartate aminotransferase increased/ Investigations	6	No/ 13	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	CK elevation/ Blood creatine phosphokinase increased/ Investigations	6	No/ 22	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Transient Thrombocytopenia	Platelets decreased/ Platelet count decreased/ Investigations	8	No/ 11	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	Tn I elevation/ Troponin I increased/ Investigations	8	No/ 83	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	48	No/ 83	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

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RESTORE				Hepatotoxicity	elavation of liver function enzyme(AST/ALT), and y-GTP/ Hepatic enzyme increased/ Investigations	5	No/ 12	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	increased troponin -I/ Troponin I increased/ Investigations	5	No/ 12	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				New Incidence of Neurological Disorder	Motor developmental delay/ Motor developmental delay/ Nervous system disorders	201	Yes/ -	Grade 2/ Related to OAV101/ None/ Not recovered/not resolved	No

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RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	23	No/ 112	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	23	No/ 189	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	4	No/ 291	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	7	No/ 14	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	7	No/ 291	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	5	No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	5	No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	plt decreased/ Platelet count decreased/ Investigations	5	No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	7	No/ 120	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6

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RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	7	No/ 120	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Plt decreased/ Platelet count decreased/ Investigations	7	No/ 120	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	CK increased/ Blood creatine phosphokinase increased/ Investigations	78	Yes/ -	Grade 2/ Related to OAV101/ None/ Not recovered/not resolved	No

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RESTORE				Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	8	No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	elevation hepatic enzyme/ Hepatic enzyme increased/ Investigations	8	No/ 22	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	Cardiac troponin I increased/ Troponin I increased/ Investigations	15	No/ 162	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Transient Thrombocytopenia	Platelets decreased/ Platelet count decreased/ Investigations	4	No/ 21	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	4	No/ 357	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Thrombotic Microangiopathy, Cardiac, New Incidence of Hematological Disorder	Thrombotic microangiopathy/ Thrombotic microangiopathy/ Blood and lymphatic system disorders	7	No/ 34	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3

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RESTORE				Cardiac	arrhythmia/ Arrhythmia/ Cardiac disorders	25	No/ 25	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	35	No/ 357	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	liver dysfunction/ Hepatic function abnormal/ Hepatobiliary disorders	50	No/ 70	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity	liver dysfunction/ Hepatic function abnormal/ Hepatobiliary disorders	324	No/ 330	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	platelets decreased/ Platelet count decreased/ Investigations	6	No/ 10	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	Creatine kinase increased/ Blood creatine phosphokinase increased/ Investigations	6	No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity	elevated liver enzyme/ Hepatic enzyme increased/ Investigations	6	No/ 15	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Cardiac	bradycardia/ Bradycardia/ Cardiac disorders	8	No/ 10	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	platelets decreased/ Platelet count decreased/ Investigations	6	No/ 10	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity	liver dysfunction/ Hepatic function abnormal/ Hepatobiliary disorders	6	No/ 20	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6
RESTORE				Hepatotoxicity	liver dysfunction/ Hepatic function abnormal/ Hepatobiliary disorders	65	No/ 79	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	4	No/ 29	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	4	No/ 29	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	5	No/ 16	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT mildly increased/ Alanine aminotransferase increased/ Investigations	56	No/ 99	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST mildly increased/ Aspartate aminotransferase increased/ Investigations	56	No/ 99	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

^a A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy

Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical
intervention to prevent one of the outcomes listed above.

AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 5.0), SOC = System Organ Class,
SAE = Serious Adverse Event.

Listing 16.1.jpj
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	ALT elevation/ Alanine aminotransferase increased/ Investigations	6	No/ 13	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST elevation/ Aspartate aminotransferase increased/ Investigations	6	No/ 13	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	4	No/ 9	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	4	No/ 638	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	5	No/ 295	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Haemoglobin decreased/ Haemoglobin decreased/ Investigations	6	No/ 12	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	4	No/ 85	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6
RESTORE				Cardiac	Blood pressure increased/ Blood pressure increased/ Investigations	7	No/ 15	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Platelets decreased/ Platelet count decreased/ Investigations	7	No/ 18	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	7	No/ 85	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6

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Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Thrombotic Microangiopathy, Cardiac, New Incidence of Hematological Disorder	Thrombotic microangiopathy/ Thrombotic microangiopathy/ Blood and lymphatic system disorders	11	No/ 16	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Transient Thrombocytopenia	Hb decreased/ Haemoglobin decreased/ Investigations	11	No/ 29	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increase/ Alanine aminotransferase increased/ Investigations	4	No/ 74	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

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RESTORE				Hepatotoxicity	AST increase/ Aspartate aminotransferase increased/ Investigations	4	No/ 123	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	PLT decrease/ Platelet count decreased/ Investigations	6	No/ 11	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	Troponin I increase/ Troponin I increased/ Investigations	15	No/ 158	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Transient Thrombocytopenia	platelet decrease/ Platelet count decreased/ Investigations	5	No/ 15	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	hepatic enzyme increased/ Hepatic enzyme increased/ Investigations	5	No/ 20	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	7	No/ 17	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	Hepatic enzyme increased/ Hepatic enzyme increased/ Investigations	7	No/ 42	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	liver dysfunction/ Hepatic function abnormal/ Hepatobiliary disorders	4	No/ 14	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	5	No/ 319	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

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Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	5	No/ 319	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	gamma-glutamyl transpeptidase increased/ Gamma-glutamyltransferase increased/ Investigations	15	No/ 30	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	6	No/ 139	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	6	No/ 139	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Plt decreased/ Platelet count decreased/ Investigations	9	No/ 13	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Hepatic function abnormal/ Hepatic function abnormal/ Hepatobiliary disorders	5	No/ 14	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	5	No/ 14	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Impaired liver function/ Hepatic function abnormal/ Hepatobiliary disorders	5	No/ 78	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	6	No/ 10	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				Hepatotoxicity	Hepatic function disorder/ Hepatic function abnormal/ Hepatobiliary disorders	29	No/ 76	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	liver dysfunction/ Hepatic function abnormal/ Hepatobiliary disorders	2	No/ 79	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	decreasing platelet count/ Platelet count decreased/ Investigations	5	No/ 6	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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Japan OAV101 Treated Patients

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RESTORE				Hepatotoxicity	liver dysfunction/ Hepatic function abnormal/ Hepatobiliary disorders	5	Yes/ -	Grade 3/ Related to OAV101/ None/ Not recovered/not resolved	No
RESTORE				Transient Thrombocytopenia	platelet decreased/ Platelet count decreased/ Investigations	5	No/ 12	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	platelet count decreased/ Platelet count decreased/ Investigations	8	No/ 13	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	8	No/ 57	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	8	No/ 57	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	platelet count decreased/ Platelet count decreased/ Investigations	4	No/ 16	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	4	No/ 29	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	4	No/ 29	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	Troponin T increased/ Troponin T increased/ Investigations	5	Yes/ -	Grade 1/ Related to OAV101/ None/ Not recovered/not resolved	No

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3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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Japan OAV101 Treated Patients

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RESTORE				Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	8	No/ 10	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	8	No/ 22	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	8	No/ 22	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	71	No/ 83	Grade 3/ Unrelated/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

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RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	71	No/ 83	Grade 2/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Hepatic function abnormal/ Hepatic function abnormal/ Hepatobiliary disorders	4	No/ 22	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Acute liver failure/ Acute hepatic failure/ Hepatobiliary disorders	52	No/ 73	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3
RESTORE				Cardiac	Cardio-respiratory arrest/ Cardio-respiratory arrest/ Cardiac disorders	269	No/ 351	Grade 4/ Unrelated/ None/ Recovered/resolved	Yes/ 2, 3

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Japan OAV101 Treated Patients

Source	Patient	Therapy ^a Start Date	Therapy ^a Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE				New Incidence of Neurological Disorder	seizure/ Seizure/ Nervous system disorders	275	Yes/ -	Grade 3/ Unrelated/ None/ Not recovered/not resolved	No
RESTORE				Cardiac	Cardio-respiratory arrest/ Cardio-respiratory arrest/ Cardiac disorders	482	No/ 482	Grade 5/ Unrelated/ None/ Fatal	Yes/ 1

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,

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Listing 16.2.jpj
Listing SMA

Japan OAV101 Treated Patients that were Screened for SMA as a Newborn

Patient	Therapy Group	SMN2 Copy Number	Displays SMA Symptoms at Diagnosis?	SMA Symptoms	Date of Genetically Confirmed SMA Diagnosis	Age at SMA Diagnosis (Months)	Displays SMA Symptoms at Enrollment?
	Bridge to OAV101	2 Copies	Yes	Hypotonia, Pneumonia or respiratory symptoms		1	Yes
	Bridge to OAV101	2 Copies	No			1	No
	Bridge to OAV101	3 Copies	No			3	No
	Combo w/OAV101	2 Copies	Yes	Hypotonia, Limb weakness		0	Yes
	Combo w/OAV101	3 Copies	Yes	Hypotonia, Limb weakness, Tongue fasciculations, Swallowing or feeding difficulties		2	Yes
	OAV101 mono	3 Copies	No			1	No
	OAV101 mono	2 Copies	No			1	Yes
	OAV101 mono	3 Copies	No			1	No
	OAV101 mono	3 Copies	No			0	No
	Switch to OAV101	3 Copies	No			0	No

Listing 16.3.1.jpn
Listing of Tracheostomy History
Japan OAV101 Treated Patients

Patient	Therapy Group	SMN2 Copy Number	Age At OAV101 Therapy (Months)	Time To Tracheostomy From First Therapy (Months)	Age At Tracheostomy (Months)	Age At Data Cut (Months)	Reason for Tracheostomy	Ongoing?
	Bridge to OAV101	2 Copies	7	1.1	2.9	66.3	Progression of disease without acute cause	Yes
	Bridge to OAV101	2 Copies	7	2.1	5.1	47.5	Pneumonia	Yes
	Bridge to OAV101	2 Copies	7	58.0	58.7	63.6	Other - aspiration pneumonia (onset date 29/Nov/2024)	Yes
	Combo w/OAV101	2 Copies	14	59.2	65.7	73.3	Progression of disease without acute cause	Yes
	Combo w/OAV101	3 Copies	3	17.3	19.8	32.7	Other - due to respiratory failure associated with RSV infection	Yes
	Combo w/OAV101	2 Copies	4	7.8	10.5	41.2	Pneumonia	Yes
	OAV101 mono	2 Copies	2	17.2	19.5	44.7	Upper respiratory illness	Yes
	Switch to OAV101	2 Copies	23	3.4	8.8	80.4	Progression of disease without acute cause	Yes

Listing 16.3.1.jp
Listing of Tracheostomy History
Japan OAV101 Treated Patients

Patient	Therapy Group	SMN2 Copy Number	Age At OAV101 Therapy (Months)	Time To Tracheostomy From First Therapy (Months)	Age At Tracheostomy (Months)	Age At Data Cut (Months)	Reason for Tracheostomy	Ongoing?
	Switch to OAV101	2 Copies	24	6.5	8.1	82.7	Upper respiratory illness	Yes
	Switch to OAV101	2 Copies	7	14.7	16.6	22.8	Progression of disease without acute cause	Yes
	Transient add-on	2 Copies	2	23.1	24.9	60.3	Pneumonia	Yes

Listing 16.3.jpn
Listing of Patient Deaths
Japan OAV101 Treated Patients

Patient	Date of Death	Primary cause of death
		Respiratory failure

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment /	366 ALB	4.9 (g/dL)	3.9	4.9
	Enrollment /	366 ALP	718 (U/L)	104	338
	Enrollment /	366 ALT	19 (U/L)	3	49
	Enrollment /	366 AST	41 (U/L)	9	37
	Enrollment /	366 BILD	0 (mg/dL)	0	0.6
	Enrollment /	366 BILI	0.4 (mg/dL)	0.1	1.1
	Enrollment /	366 HCT	38.9 (%)	33.4	44.9
	Enrollment /	366 HGB	13.3 (g/dL)	11.3	15.2
	Enrollment /	366 PLAT	294 (10E3/uL)	131	369
	Enrollment /	366 PROT	7.2 (g/dL)	6.6	8.2
	Enrollment /	366 RBC	4.84 (10E6/uL)	3.76	5
	Enrollment /	366 WBC	10.4 (10E3/uL)	3.5	9.1
	Enrollment /	366			
	Enrollment / 1	ALB	4.1 (g/dL)	3.9	4.9
	Enrollment / 1	ALP	431 (U/L)	38	113
	Enrollment / 1	ALT	27 (U/L)	3	49
	Enrollment / 1	AST	41 (U/L)	9	37
	Enrollment / 1	BILD	0 (mg/dL)	0	0.6
	Enrollment / 1	BILI	0.2 (mg/dL)	0.1	1.1
	Enrollment / 1	HCT	37 (%)	33.4	44.9
	Enrollment / 1	HGB	12 (g/dL)	11.3	15.2
	Enrollment / 1	PLAT	379 (10E3/uL)	131	369
	Enrollment / 1	PROT	6.7 (g/dL)	6.6	8.2

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[REDACTED]	Enrollment / [REDACTED] 1	RBC	4.63 (10E6/uL)	3.76	5
	Enrollment / [REDACTED] 1	WBC	10.4 (10E3/uL)	3.5	9.1
	Enrollment / [REDACTED] 515	ALP	614 (U/L)	106	322
	Enrollment / [REDACTED] 530	PROT	7.3 (g/dL)	6.6	8.1
	Enrollment / [REDACTED] 538	ALB	5.1 (g/dL)	4.1	5.1
	Enrollment / [REDACTED] 538	ALT	28 (U/L)	7	23
	Enrollment / [REDACTED] 538	AST	40 (U/L)	13	30
	Enrollment / [REDACTED] 538	BILI	0.58 (mg/dL)	0.4	1.5
	Enrollment / [REDACTED] 538	HCT	42.3 (%)	35.1	44.4
	Enrollment / [REDACTED] 538	HGB	13.9 (g/dL)	11.6	14.8
	Enrollment / [REDACTED] 538	PLAT	372 (10E3/uL)	158	348
	Enrollment / [REDACTED] 538	RBC	4.98 (10E6/uL)	3.86	4.92
	Enrollment / [REDACTED] 538	WBC	9.9 (10E3/uL)	3.3	8.6
	Enrollment / [REDACTED] 93	ALB	4.7 (g/dL)	4.1	5.1
	Enrollment / [REDACTED] 93	ALP	155 (U/L)	38	113
	Enrollment / [REDACTED] 93	ALT	17 (U/L)	10	42
	Enrollment / [REDACTED] 93	AST	36 (U/L)	13	30
	Enrollment / [REDACTED] 93	BILD	0.03 (mg/dL)	0	0.2
	Enrollment / [REDACTED] 93	BILI	0.34 (mg/dL)	0.4	1.5
	Enrollment / [REDACTED] 93	HCT	42.8 (%)	40.7	50.1
	Enrollment / [REDACTED] 93	HGB	13.1 (g/dL)	13.7	16.8

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits		
				Very Low	Very High	
[REDACTED]	Enrollment / [REDACTED]	/ 93	PLAT	365 (10E3/uL)	158	348
	Enrollment / [REDACTED]	/ 93	PROT	7.1 (g/dL)	6.6	8.1
	Enrollment / [REDACTED]	/ 93	RBC	5.21 (10E6/uL)	4.35	5.55
	Enrollment / [REDACTED]	/ 93	WBC	12 (10E3/uL)	3.3	8.6
	Enrollment / [REDACTED]	/ 97	ALT	56 (U/L)	10	42
	Enrollment / [REDACTED]	/ 97	AST	123 (U/L)	13	30
	Enrollment / [REDACTED]	100	PLAT	146 (10E3/uL)	158	348
	Enrollment / [REDACTED]	105	ALT	2871 (U/L)	10	42
	Enrollment / [REDACTED]	105	AST	1856 (U/L)	13	30
	Enrollment / [REDACTED]	176	ALB	4.4 (g/dL)	3.26	4.76
	Enrollment / [REDACTED]	176	ALP	687 (IU/L)	400	1550
	Enrollment / [REDACTED]	176	ALT	16 (IU/L)	12	50.5
	Enrollment / [REDACTED]	176	AST	35 (IU/L)	24.5	66.5
	Enrollment / [REDACTED]	176	BILI	0.1 (mg/dL)	0.12	0.59
	Enrollment / [REDACTED]	176	HCT	35.3 (%)	30.5	41.7
	Enrollment / [REDACTED]	176	HGB	11.8 (g/dL)	10.2	14.3
	Enrollment / [REDACTED]	176	PLAT	448 (10E3/uL)	200	740
	Enrollment / [REDACTED]	176	PROT	6.8 (g/dL)	5.45	7.25
	Enrollment / [REDACTED]	176	RBC	4.54 (10E6/uL)	3.86	5.3
	Enrollment / [REDACTED]	176	WBC	7.6 (10E3/uL)	4.4	19.2
Enrollment / [REDACTED]	152	ALB	4.1 (g/dL)	3.26	4.76	

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits		
				Very Low	Very High	
	Enrollment /	152	ALP	607 (U/L)	400	1550
	Enrollment /	152	ALT	25 (U/L)	12	50.5
	Enrollment /	152	AST	34 (U/L)	24.5	66.5
	Enrollment /	152	BILI	0.2 (mg/dL)	0.12	0.59
	Enrollment /	152	HCT	40.2 (%)	30.5	41.7
	Enrollment /	152	HGB	14 (g/dL)	10.2	14.3
	Enrollment /	152	PLAT	587 (10E3/uL)	200	740
	Enrollment /	152	PROT	6 (g/dL)	5.45	7.25
	Enrollment /	152	RBC	5.13 (10E6/uL)	3.86	5.3
	Enrollment /	152	WBC	16.5 (10E3/uL)	4.4	19.2
	Enrollment	14	ALB	4.2 (g/dL)	3.36	4.74
	Enrollment	14	ALP	204 (U/L)	-	-
	Enrollment	14	ALT	11 (U/L)	9.4	38.4
	Enrollment	14	AST	32 (U/L)	23	56.5
	Enrollment	14	BILI	0.4 (mg/dL)	0.16	0.67
	Enrollment	14	HCT	36.1 (%)	32	42.4
	Enrollment	14	HGB	12.1 (g/dL)	10.5	14.1
	Enrollment	14	PLAT	27.8 (10E4/uL)	16	65
	Enrollment	14	PROT	6.6 (g/dL)	5.7	7.5
	Enrollment	14	RBC	4.55 (10E6/uL)	3.93	5.38
	Enrollment	14	WBC	8.8 (10E3/uL)	4.3	19.6
	Enrollment	17	ALT	13 (U/L)	9.4	38.4

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jp
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment / / 17	AST	59 (U/L)	23	56.5
	Enrollment / / 19	AST	585 (U/L)	23	56.5
	Enrollment / / 19	PLAT	14.3 (10E4/uL)	16	65
	Enrollment / / 21	PLAT	11.5 (10E4/uL)	16	65
	Enrollment / / 49	ALT	617 (U/L)	9.4	38.4
	Enrollment / / 20	ALB	4.9 (g/dL)	3.27	4.76
	Enrollment / / 20	ALP	158 (U/L)	-	-
	Enrollment / / 20	ALT	8 (U/L)	11.5	48
	Enrollment / / 20	AST	32 (U/L)	24	65.5
	Enrollment / / 20	BILI	0.5 (mg/dL)	0.12	0.59
	Enrollment / / 20	HCT	31.1 (%)	30.8	41.7
	Enrollment / / 20	HGB	10.4 (g/dL)	10.3	14.3
	Enrollment / / 20	PLAT	57 (10E4/uL)	20	72
	Enrollment / / 20	PROT	6.8 (g/dL)	5.5	7.3
	Enrollment / / 20	RBC	4.02 (10E6/uL)	3.88	5.32
	Enrollment / / 20	WBC	5.4 (10E3/uL)	4.4	19.4
	Enrollment / / 487	ALB	3.7 (g/dL)	3.5	5
	Enrollment / / 487	ALT	10 (IU/L)	6	27
	Enrollment / / 487	AST	24 (IU/L)	13	30
	Enrollment / / 487	BILI	0.1 (mg/dL)	0.2	1
	Enrollment / / 487	HCT	32.3 (%)	36	47

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment	487 HGB	10.6 (g/dL)	11.3	14.9
	Enrollment	487 PLAT	396 (10E3/uL)	180	340
	Enrollment	487 PROT	6.6 (g/dL)	6.6	8.1
	Enrollment	487 RBC	4.14 (10E6/uL)	3.95	4.65
	Enrollment	487 WBC	5900 (cells/uL)	4300	8000
	Enrollment	355 ALB	4.1 (g/dL)	4.1	5.1
	Enrollment	355 ALP	378 (U/L)	106	322
	Enrollment	355 ALT	40 (U/L)	7	23
	Enrollment	355 AST	39 (U/L)	13	30
	Enrollment	355 BILI	0.39 (mg/dL)	0.4	1.5
	Enrollment	355 HCT	37.8 (%)	35.1	44.4
	Enrollment	355 HGB	12.2 (g/dL)	11.6	14.8
	Enrollment	355 PLAT	326 (10E3/uL)	158	348
	Enrollment	355 PROT	6.7 (g/dL)	6.6	8.1
	Enrollment	355 RBC	4.48 (10E6/uL)	3.86	4.92
	Enrollment	355 WBC	5.9 (10E3/uL)	3.3	8.6
	Enrollment	/ 1 ALB	5.3 (g/dL)	4.1	5.1
	Enrollment	/ 1 ALT	55 (U/L)	10	42
	Enrollment	/ 1 AST	63 (U/L)	13	30
	Enrollment	/ 1 HCT	43.1 (%)	40.7	50.1
	Enrollment	/ 1 HGB	14.1 (g/dL)	13.7	16.8

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment / / 1	PLAT	342 (10E3/uL)	158	348
	Enrollment / / 1	PROT	7.4 (g/dL)	6.6	8.1
	Enrollment / / 1	RBC	5.37 (10E6/uL)	4.35	5.55
	Enrollment / / 1	WBC	7.2 (10E3/uL)	3.3	8.6
	Enrollment / / 238	ALP	237 (U/L)	38	113
	Enrollment / / 238	ALT	23 (U/L)	7	23
	Enrollment / / 238	AST	35 (U/L)	13	30
	Enrollment / / 238	BILI	0.51 (mg/dL)	0.4	1.5
	Enrollment / / 238	HCT	38.3 (%)	35.1	44.4
	Enrollment / / 238	HGB	12.1 (g/dL)	11.6	14.8
	Enrollment / / 238	PLAT	441 (10E3/uL)	158	348
	Enrollment / / 238	PROT	6.4 (g/dL)	6.6	8.1
	Enrollment / / 238	RBC	4.7 (10E6/uL)	3.86	4.92
	Enrollment / / 238	WBC	11.1 (10E3/uL)	3.3	8.6
	Enrollment / / 239	AST	45 (U/L)	13	30
	Enrollment / / 242	ALT	78 (U/L)	7	23
	Enrollment / / 242	AST	152 (U/L)	13	30
	Enrollment / / 242	PLAT	58 (10E3/uL)	158	348
	Enrollment / / 244	PLAT	47 (10E3/uL)	158	348
	Enrollment / / 275	AST	514 (U/L)	13	30
	Enrollment / / 279	ALT	768 (U/L)	7	23

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment	-19 ALB	4.4 (g/dL)	4	5
	Enrollment	-19 ALT	14 (IU/L)	5	25
	Enrollment	-19 AST	35 (IU/L)	15	50
	Enrollment	-19 BILI	0.3 (mg/dL)	0.1	1.1
	Enrollment	-19 HCT	38.9 (%)	33.4	44.9
	Enrollment	-19 HGB	13.1 (g/dL)	11.3	15.2
	Enrollment	-19 PLAT	246 (10E3/uL)	130	369
	Enrollment	-19 PROT	6.7 (g/dL)	5.7	7.3
	Enrollment	-19 RBC	5.1 (10E6/uL)	3.76	5
	Enrollment	-19 WBC	11.3 (10E3/uL)	3.5	9.1
	Enrollment	105 ALB	4.2 (g/dL)	3.5	4.8
	Enrollment	105 ALT	25 (IU/L)	5	65
	Enrollment	105 AST	32 (IU/L)	15	80
	Enrollment	105 BILI	0.8 (mg/dL)	0.2	1.1
	Enrollment	105 HCT	34.6 (%)	33.4	44.9
	Enrollment	105 HGB	11.4 (g/dL)	11.3	15.2
	Enrollment	105 PLAT	548 (10E3/uL)	130	369
	Enrollment	105 PROT	6 (g/dL)	5	7
	Enrollment	105 RBC	4.36 (10E6/uL)	3.76	5
	Enrollment	105 WBC	6.7 (10E3/uL)	3.5	9.1
	Enrollment	-17 ALB	4.3 (g/dL)	3.4	4.7

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment	ALT	17 (IU/L)	9	38
	Enrollment	AST	38 (IU/L)	23	57
	Enrollment	BILI	0.3 (mg/dL)	0.2	0.7
	Enrollment	HCT	37.4 (%)	39.8	51.8
	Enrollment	HGB	12.6 (g/dL)	13.5	17.6
	Enrollment	PLAT	377 (10E3/uL)	131	362
	Enrollment	PROT	6.4 (g/dL)	5.7	7.5
	Enrollment	RBC	4.64 (10E6/uL)	4.27	5.7
	Enrollment	WBC	9.4 (10E3/uL)	3.9	9.8
	Enrollment	ALB	3.7 (g/dL)	3	4.1
	Enrollment	ALT	28 (U/L)	11	45
	Enrollment	AST	39 (U/L)	20	62
	Enrollment	BILI	2.3 (mg/dL)	0.4	3.2
	Enrollment	HCT	47.2 (%)	33.4	44.9
	Enrollment	HGB	16.4 (g/dL)	11.3	15.2
	Enrollment	PLAT	52.6 (10E4/uL)	13	36.9
	Enrollment	PROT	5.5 (g/dL)	4.7	6.4
	Enrollment	RBC	50.2 (10E6/uL)	37.6	50
	Enrollment	WBC	11 (10E3/uL)	3.5	9.1
	Enrollment	AST	65 (IU/L)	20	62
	Enrollment	ALT	69 (IU/L)	11	45
	Enrollment	AST	78 (IU/L)	20	62

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Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment / 77	ALT	80 (IU/L)	11	45
	Enrollment / 77	AST	94 (IU/L)	20	62
	Enrollment 0	ALB	3.7 (g/dL)	3.1	4.3
	Enrollment 0	ALT	32 (U/L)	12	50
	Enrollment 0	AST	39 (U/L)	21	64
	Enrollment 0	BILI	0.8 (mg/dL)	0.3	2.3
	Enrollment 0	HCT	34.9 (%)	39.8	51.8
	Enrollment 0	HGB	12.1 (g/dL)	13.5	17.6
	Enrollment 0	PLAT	34.1 (10E4/uL)	13	36.2
	Enrollment 0	PROT	5.5 (g/dL)	4.9	6.6
	Enrollment 0	RBC	3.9 (10E6/uL)	4.27	5.7
	Enrollment 0	WBC	9.8 (10E3/uL)	3.9	9.8
	Enrollment 7	AST	67 (IU/L)	21	64
	Enrollment / 15	ALT	80 (IU/L)	12	50
	Enrollment / 43	ALT	118 (IU/L)	12	50
	Enrollment / 43	AST	176 (IU/L)	21	64
	Enrollment 1	ALB	3.5 (g/dL)	3	4.1
	Enrollment 1	ALT	20 (U/L)	11	45
	Enrollment 1	AST	29 (U/L)	20	62
	Enrollment 1	BILI	0.6 (mg/dL)	0.4	3.2
	Enrollment 1	HCT	29.3 (%)	33.4	44.9

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment / / 1	HGB	9.9 (g/dL)	11.3	15.2
	Enrollment / / 1	PLAT	38.2 (10E4/uL)	13	36.9
	Enrollment / / 1	PROT	5.4 (g/dL)	4.7	6.4
	Enrollment / / 1	RBC	3.02 (10E6/uL)	3.76	5
	Enrollment / / 1	WBC	8.8 (10E3/uL)	3.5	9.1
	Enrollment / / 24	ALP	685 (IU/L)	104	338
	Enrollment / / 24	ALT	23 (IU/L)	0	40
	Enrollment / / 24	AST	23 (IU/L)	0	37
	Enrollment / / 24	BILI	0.3 (mg/dL)	0.2	1.2
	Enrollment / / 24	HCT	34.1 (%)	39.8	51.8
	Enrollment / / 24	HGB	11.4 (g/dL)	13.5	17.6
	Enrollment / / 24	PLAT	422 (10E3/uL)	131	362
	Enrollment / / 24	PROT	5.3 (g/dL)	5.5	8
	Enrollment / / 24	RBC	4.21 (10E6/uL)	4.27	5.7
	Enrollment / / 24	WBC	7.8 (10E3/uL)	3.9	9.8
	Enrollment / / -2	ALB	4.1 (g/dL)	4.2	4.9
	Enrollment / / -2	ALP	602 (IU/L)	104	338
	Enrollment / / -2	ALT	17 (IU/L)	0	40
	Enrollment / / -2	AST	27 (IU/L)	0	37
	Enrollment / / -2	BILI	0.2 (mg/dL)	0.2	1.2
	Enrollment / / -2	HCT	36.1 (%)	39.8	51.8

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment / / -2	HGB	11.7 (g/dL)	13.5	17.6
	Enrollment / / -2	PLAT	564 (10E3/uL)	131	362
	Enrollment / / -2	PROT	6 (g/dL)	5.5	8
	Enrollment / / -2	RBC	4.48 (10E6/uL)	4.27	5.7
	Enrollment / / -2	WBC	13.6 (10E3/uL)	3.9	9.8
	Enrollment / / 266	ALB	4.6 (g/dL)	4.1	5.1
	Enrollment / / 266	ALP	602 (U/L)	106	322
	Enrollment / / 266	ALT	19 (U/L)	7	23
	Enrollment / / 266	AST	44 (U/L)	13	30
	Enrollment / / 266	BILI	0.6 (mg/dL)	0.4	1.5
	Enrollment / / 266	HCT	34.9 (%)	35.1	44.4
	Enrollment / / 266	HGB	11.6 (g/dL)	11.6	14.8
	Enrollment / / 266	PLAT	257 (10E3/uL)	158	348
	Enrollment / / 266	RBC	4.14 (10E6/uL)	3.86	4.92
	Enrollment / / 266	WBC	6.49 (10E3/uL)	3.3	8.6
	Enrollment / / 673	ALB	4.4 (g/dL)	4.1	5.1
	Enrollment / / 673	ALP	640 (U/L)	106	322
	Enrollment / / 673	ALT	35 (U/L)	10	42
	Enrollment / / 673	AST	55 (U/L)	13	30
	Enrollment / / 673	BILI	0.2 (mg/dL)	0.4	1.5
	Enrollment / / 673	HCT	37.4 (%)	40.7	50.1

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment	673 HGB	12.7 (g/dL)	13.7	16.8
	Enrollment	673 PLAT	456 (10E3/uL)	158	348
	Enrollment	673 PROT	6.9 (g/dL)	6.6	8.1
	Enrollment	673 RBC	4.46 (10E6/uL)	4.35	5.55
	Enrollment	673 WBC	11.32 (10E3/uL)	3.3	8.6
	Enrollment	-22 ALB	4.5 (g/dL)	4.1	5.1
	Enrollment	-22 ALP	214 (U/L)	395	1339
	Enrollment	-22 ALT	16 (U/L)	10	38
	Enrollment	-22 AST	33 (U/L)	13	57
	Enrollment	-22 BILI	0.3 (mg/dL)	0.4	1.5
	Enrollment	-22 HCT	39 (%)	32	42.4
	Enrollment	-22 HGB	13.2 (g/dL)	10.5	14.1
	Enrollment	-22 PLAT	336 (10E3/uL)	158	348
	Enrollment	-22 PROT	6 (g/dL)	6.6	8.1
	Enrollment	-22 RBC	4.93 (10E6/uL)	4.35	5.55
	Enrollment	-22 WBC	6.87 (10E3/uL)	3.3	8.6
	Enrollmen	/ 4 ALB	4.4 (g/dL)	4.1	5.1
	Enrollmen	/ 4 ALT	13 (U/L)	10	42
	Enrollmen	/ 4 AST	20 (U/L)	13	30
	Enrollmen	/ 4 BILI	0.2 (mg/dL)	0.4	1.5
	Enrollmen	/ 4 HCT	34.4 (%)	40.7	50.1

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment / / 4	HGB	11.5 (g/dL)	13.7	16.8
	Enrollment / / 4	PLAT	218 (10E3/uL)	158	348
	Enrollment / / 4	PROT	6.3 (g/dL)	6.6	8.1
	Enrollment / / 4	RBC	4.21 (10E6/uL)	4.35	5.55
	Enrollment / / 4	WBC	11.16 (10E3/uL)	3.3	8.6
	Enrollment / / 225	ALB	4.2 (g/dL)	3.4	4.7
	Enrollment / / 225	ALT	14 (IU/L)	9	38
	Enrollment / / 225	AST	28 (IU/L)	24	57
	Enrollment / / 225	BILI	0.39 (mg/dL)	0.2	0.7
	Enrollment / / 225	HCT	35.1 (%)	33	42.4
	Enrollment / / 225	HGB	10.8 (g/dL)	10.7	14.1
	Enrollment / / 225	PLAT	280 (10E3/uL)	320	650
	Enrollment / / 225	PROT	6.3 (g/dL)	5.7	7.5
	Enrollment / / 225	RBC	4.04 (10E6/uL)	3.93	5.38
	Enrollment / / 225	WBC	6.6 (10E3/uL)	4.3	19.6
	Enrollment / / 12	ALB	3.6 (g/dL)	4.1	5.1
	Enrollment / / 12	ALT	36 (IU/L)	7	23
	Enrollment / / 12	AST	39 (IU/L)	13	30
	Enrollment / / 12	BILD	0.07 (mg/dL)	0	0.2
	Enrollment / / 12	BILI	0.22 (mg/dL)	0.4	1.5
	Enrollment / / 12	HCT	31.6 (%)	35.1	44.4

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment / / 12	HGB	10.4 (g/dL)	11.6	14.8
	Enrollment / / 12	PLAT	518 (10E3/uL)	158	348
	Enrollment / / 12	PROT	5.4 (g/dL)	6.6	8.1
	Enrollment / / 12	RBC	3.51 (10E6/uL)	3.86	4.92
	Enrollment / / 12	WBC	10.1 (10E3/uL)	3.3	8.6
	Enrollment / / 9	ALB	4.4 (g/dL)	4.1	5.1
	Enrollment / / 9	ALT	10 (IU/L)	10	42
	Enrollment / / 9	AST	22 (IU/L)	13	30
	Enrollment / / 9	BILD	0.31 (mg/dL)	0	0.2
	Enrollment / / 9	BILI	13.21 (mg/dL)	0.4	1.5
	Enrollment / / 9	HCT	42.4 (%)	40.7	50.1
	Enrollment / / 9	HGB	15 (g/dL)	13.7	16.8
	Enrollment / / 9	PLAT	725 (10E3/uL)	158	348
	Enrollment / / 9	PROT	6.4 (g/dL)	6.6	8.1
	Enrollment / / 9	RBC	4.52 (10E6/uL)	4.35	5.55
	Enrollment / / 9	WBC	7.1 (10E3/uL)	3.3	8.6
	Enrollment / / 499	ALB	4.4 (g/dL)	4.1	5.1
	Enrollment / / 499	ALP	336 (IU/L)	173	353
	Enrollment / / 499	ALT	21 (IU/L)	5	31
	Enrollment / / 499	AST	45 (IU/L)	22	50
	Enrollment / / 499	BILI	0.2 (mg/dL)	0.4	1.5

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment /	499 HCT	39.5 (%)	34	40
	Enrollment /	499 HGB	13.3 (g/dL)	10.5	13.5
	Enrollment /	499 PLAT	391 (10E3/uL)	150	400
	Enrollment /	499 PROT	6.5 (mg/dL)	6.5	8.1
	Enrollment /	499 RBC	437 (10E10/L)	15	35
	Enrollment /	499 WBC	11600 (cmm)	7000	15000
	Enrollment	/ -1 ALT	85 (U/L)	10	30
	Enrollment	/ -1 AST	73 (U/L)	13	30
	Enrollment	/ -1 BILD	0.1 (mg/dL)	0	0.2
	Enrollment	/ -1 BILI	0.5 (mg/dL)	0.4	1.2
	Enrollment	/ -1 HCT	35.9 (%)	40.7	50.7
	Enrollment	/ -1 HGB	12.2 (g/dL)	13.7	16.8
	Enrollment	/ -1 PLAT	677 (10E3/uL)	158	348
	Enrollment	/ -1 RBC	4.58 (10E6/uL)	4.35	5.55
	Enrollment	/ -1 WBC	7.3 (10E3/uL)	3.3	8.6
	Enrollment /	211 ALB	4.3 (g/dL)	4.1	5.1
	Enrollment /	211 ALP	163 (U/L)	38	113
	Enrollment /	211 ALT	12 (U/L)	10	30
	Enrollment /	211 AST	25 (U/L)	13	30
	Enrollment /	211 BILD	0 (mg/dL)	0	0.2
	Enrollment /	211 BILI	0.3 (mg/dL)	0.4	1.2

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment /	211 HCT	37.7 (%)	40.7	50.1
	Enrollment /	211 HGB	12.3 (g/dL)	13.7	16.8
	Enrollment /	211 PLAT	466 (10E3/uL)	158	348
	Enrollment /	211 PROT	6.7 (g/dL)	6.6	8.1
	Enrollment /	211 RBC	4.51 (10E6/uL)	4.35	5.55
	Enrollment /	211 WBC	10.5 (10E3/uL)	3.3	8.6
	Enrollment /	224 ALB	4.6 (g/dL)	4.1	5.1
	Enrollment /	224 ALP	167 (U/L)	38	113
	Enrollment /	224 ALT	132 (U/L)	10	30
	Enrollment /	224 AST	232 (U/L)	13	30
	Enrollment /	224 BILD	0.2 (mg/dL)	0	0.2
	Enrollment /	224 BILI	0.5 (mg/dL)	0.4	1.2
	Enrollment /	224 HCT	35.4 (%)	40.7	50.1
	Enrollment /	224 HGB	11.1 (g/dL)	13.7	16.8
	Enrollment /	224 PLAT	433 (10E3/uL)	158	348
	Enrollment /	224 PROT	6.9 (g/dL)	6.6	8.1
	Enrollment /	224 RBC	4.18 (10E6/uL)	4.35	5.55
	Enrollment /	224 WBC	13.9 (10E3/uL)	3.3	8.6
	Enrollment /	70 ALB	3.6 (g/dL)	4.1	5.1
	Enrollment /	70 ALP	286 (U/L)	38	113
	Enrollment /	70 ALT	24 (U/L)	10	42

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jp
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits		
				Very Low	Very High	
	Enrollment	/ 0	AST	33 (U/L)	13	30
	Enrollment	/ 0	HCT	34 (%)	40.7	50.1
	Enrollment	/ 0	HGB	11.8 (g/dL)	13.7	16.8
	Enrollment	/ 0	PLAT	699 (10E3/uL)	158	348
	Enrollment	/ 0	PROT	5.3 (g/dL)	6.6	8.1
	Enrollment	/ 0	RBC	3.77 (10E6/uL)	4.35	5.55
	Enrollment	/ 0	WBC	10.7 (10E3/uL)	3.3	8.6
	Enrollment /	133	ALB	4.4 (g/dL)	4.1	5.1
	Enrollment /	133	ALP	292 (U/L)	38	113
	Enrollment /	133	ALT	19 (U/L)	7	23
	Enrollment /	133	AST	23 (U/L)	13	30
	Enrollment /	133	BILD	0.1 (mg/dL)	0	0.2
	Enrollment /	133	BILI	0.2 (mg/dL)	0.4	1.5
	Enrollment /	133	HGB	11.3 (g/dL)	11.6	14.8
	Enrollment /	133	PLAT	414 (10E3/uL)	158	348
	Enrollment /	133	PROT	6.6 (g/dL)	6.6	8.1
	Enrollment /	133	RBC	4.34 (10E6/uL)	3.86	4.92
	Enrollment /	133	WBC	9.6 (10E3/uL)	3.3	8.6
	Enrollment /	176	AST	37 (U/L)	12	30
	Enrollment /	176	WBC	21.1 (10E3/uL)	3.3	8.6
Enrollment /	113	ALB	4.8 (g/dL)	4.1	5.1	

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment	113 ALP	305 (U/L)	38	113
	Enrollment	113 ALT	66 (U/L)	7	23
	Enrollment	113 AST	73 (U/L)	13	30
	Enrollment	113 BILD	0.1 (mg/dL)	0	0.2
	Enrollment	113 BILI	0.5 (mg/dL)	0.4	1.5
	Enrollment	113 HCT	42.9 (%)	35.1	44
	Enrollment	113 HGB	14.3 (g/dL)	11.6	14.8
	Enrollment	113 PLAT	269 (10E3/uL)	158	348
	Enrollment	113 PROT	6.5 (g/dL)	6.6	8.1
	Enrollment	113 RBC	5.11 (10E6/uL)	3.86	4.92
	Enrollment	113 WBC	7.4 (10E3/uL)	3.3	8.6
	Enrollment	119 ALT	149 (U/L)	7	23
	Enrollment	119 AST	243 (U/L)	13	30
	Enrollment	119 WBC	3.1 (10E3/uL)	3.3	8.6
	Enrollment	141 AST	97 (U/L)	13	30
	Enrollment	144 AST	123 (U/L)	13	30
	Enrollment	225 ALB	5.2 (g/dL)	4.1	5.1
	Enrollment	225 ALP	300 (U/L)	38	113
	Enrollment	225 ALT	27 (U/L)	7	23
	Enrollment	225 AST	37 (U/L)	13	30
	Enrollment	225 HCT	38.4 (%)	35.1	44.4
	Enrollment	225 HGB	12.5 (g/dL)	11.6	14.8

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment /	225 PLAT	613 (10E3/uL)	158	348
	Enrollment /	225 PROT	7 (g/dL)	6.6	8.1
	Enrollment /	225 RBC	4.6 (10E6/uL)	3.86	4.92
	Enrollment /	225 WBC	8.1 (10E3/uL)	3.3	8.6
	Enrollment /	229 ALT	39 (U/L)	7	23
	Enrollment /	229 AST	90 (U/L)	13	30
	Enrollment /	231 ALT	81 (U/L)	7	23
	Enrollment /	231 AST	134 (U/L)	13	30
	Enrollment /	268 ALT	32 (U/L)	7	23
	Enrollment /	268 AST	51 (U/L)	13	30
	Enrollment	64 ALB	4.1 (g/dL)	3.9	4.9
	Enrollment	64 ALP	466 (IU/L)	130	350
	Enrollment	64 ALT	11 (IU/L)	4	43
	Enrollment	64 AST	32 (IU/L)	8	38
	Enrollment	64 BILD	0.1 (mg/dL)	0	0.2
	Enrollment	64 BILI	0.9 (mg/dL)	0.2	1.2
	Enrollment	64 HCT	38.3 (%)	35	48
	Enrollment	64 HGB	12.6 (g/dL)	12	16
	Enrollment	64 PLAT	406 (10E3/uL)	130	406
	Enrollment	64 PROT	6.6 (g/dL)	6.7	8.8
	Enrollment	64 RBC	4.8 (10E6/uL)	3.8	4.8
	Enrollment	64 WBC	7.4 (10E3/uL)	5	8.5

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits		
				Very Low	Very High	
P001	Enrollment	/ 7	ALB	3.7 (g/dL)	3.9	4.9
	Enrollment	/ 7	ALP	258 (IU/L)	38	113
	Enrollment	/ 7	ALT	11 (IU/L)	4	43
	Enrollment	/ 7	AST	21 (IU/L)	8	38
	Enrollment	/ 7	BILD	0.2 (mg/dL)	0	0.2
	Enrollment	/ 7	BILI	2.6 (mg/dL)	0.2	1.2
	Enrollment	/ 7	HCT	30.8 (%)	35	48
	Enrollment	/ 7	HGB	10.7 (g/dL)	12	16
	Enrollment	/ 7	PLAT	271 (10E3/uL)	130	400
	Enrollment	/ 7	PROT	5.2 (g/dL)	6.7	8.3
	Enrollment	/ 7	RBC	3.25 (10E6/uL)	3.8	4.8
	Enrollment	/ 7	WBC	6.2 (10E3/uL)	5	8.5
P002	Enrollment /	50	ALB	4.6 (g/dL)	3.9	4.9
	Enrollment /	50	ALP	132 (U/L)	38	113
	Enrollment /	50	ALT	20 (U/L)	4	43
	Enrollment /	50	AST	40 (U/L)	8	38
	Enrollment /	50	BILD	0.1 (mg/dL)	0	0.2
	Enrollment /	50	BILI	0.6 (mg/dL)	0.2	1.2
	Enrollment /	50	HCT	36.1 (%)	35	48
	Enrollment /	50	HGB	11.9 (g/dL)	12	16
	Enrollment /	50	PLAT	287 (10E3/uL)	130	400

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment /	50 PROT	6.2 (g/dL)	6.7	8.3
	Enrollment /	50 RBC	4.34 (10E6/uL)	3.8	4.8
	Enrollment /	50 WBC	9.8 (10E3/uL)	5	8.3
	Enrollment /	56 ALT	119 (U/L)	4	43
	Enrollment /	56 ALT	266 (U/L)	4	43
	Enrollment /	56 AST	298 (U/L)	8	38
	Enrollment /	56 AST	700 (U/L)	8	38
	Enrollment /	59 ALT	259 (U/L)	4	43
	Enrollment /	59 AST	397 (U/L)	8	38
	Enrollment /	64 ALT	70 (U/L)	4	43
	Enrollment /	64 AST	61 (U/L)	8	38
	Enrollment /	77 AST	50 (U/L)	8	38
	Enrollment /	14 ALB	3.4 (g/dL)	3.9	4.9
	Enrollment /	14 ALP	222 (U/L)	38	113
	Enrollment /	14 ALT	18 (U/L)	4	44
	Enrollment /	14 AST	20 (U/L)	8	38
	Enrollment /	14 BILI	0.3 (mg/dL)	0.2	1.2
	Enrollment /	14 HCT	30.8 (%)	41.3	52.1
	Enrollment /	14 HGB	10.3 (g/dL)	13.3	16.6
	Enrollment /	14 PLAT	447 (10E3/uL)	172	359
	Enrollment /	14 RBC	3.55 (10E6/uL)	4.29	5.7
	Enrollment /	14 WBC	14.98 (10E3/uL)	3.58	8.15

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[REDACTED]	Enrollment / [REDACTED] 21	PLAT	96 (10E3/uL)	172	359
	Enrollment / [REDACTED] 1	ALB	4 (g/dL)	4.1	5.1
	Enrollment / [REDACTED] 1	ALP	389 (U/L)	38	113
	Enrollment / [REDACTED] 1	ALT	34 (U/L)	10	42
	Enrollment / [REDACTED] 1	AST	41 (U/L)	13	30
	Enrollment / [REDACTED] 1	BILD	0.2 (mg/dL)	0	0.2
	Enrollment / [REDACTED] 1	BILI	1.7 (mg/dL)	0.4	1.5
	Enrollment / [REDACTED] 1	HCT	31.6 (%)	40.7	50.1
	Enrollment / [REDACTED] 1	HGB	10.8 (g/dL)	13.7	16.8
	Enrollment / [REDACTED] 1	PLAT	388 (10E3/uL)	158	348
	Enrollment / [REDACTED] 1	PROT	5.6 (g/dL)	6.6	8.1
	Enrollment / [REDACTED] 1	RBC	3.47 (10E6/uL)	4.35	5.55
	Enrollment / [REDACTED] 1	WBC	5.9 (10E3/uL)	3.3	8.6
	Enrollment / [REDACTED] 1	ALB	4.7 (g/dL)	3.3	5.2
	Enrollment / [REDACTED] 1	ALP	475 (IU/L)	171	1330
	Enrollment / [REDACTED] 1	ALT	12 (IU/L)	3	27
	Enrollment / [REDACTED] 1	AST	28 (IU/L)	11	47
	Enrollment / [REDACTED] 1	BILI	0.52 (mg/dL)	0.14	0.95
	Enrollment / [REDACTED] 1	HCT	34.3 (%)	32	50
	Enrollment / [REDACTED] 1	HGB	11.8 (g/dL)	11.5	15.5
	Enrollment / [REDACTED] 1	PLAT	327 (10E3/uL)	130	400

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[REDACTED]	Enrollment / [REDACTED] / 1	PROT	6.8 (g/dL)	5.5	8
	Enrollment / [REDACTED] / 1	RBC	4.21 (10E6/uL)	3.8	5.3
	Enrollment / [REDACTED] / 1	WBC	7.4 (10E3/uL)	5	10
	Enrollment / [REDACTED] / 182	ALB	4.8 (g/dL)	4.1	5.1
	Enrollment / [REDACTED] / 182	ALP	246 (U/L)	38	113
	Enrollment / [REDACTED] / 182	ALT	31 (U/L)	10	30
	Enrollment / [REDACTED] / 182	AST	57 (U/L)	13	30
	Enrollment / [REDACTED] / 182	BILI	0.4 (mg/dL)	0.4	1.5
	Enrollment / [REDACTED] / 182	HCT	34.5 (%)	40.7	50.1
	Enrollment / [REDACTED] / 182	HGB	10.7 (g/dL)	13.7	16.8
	Enrollment / [REDACTED] / 182	PLAT	294 (10E3/uL)	158	348
	Enrollment / [REDACTED] / 182	PROT	6.6 (g/dL)	6.6	8.1
	Enrollment / [REDACTED] / 182	RBC	4 (10E6/uL)	4.35	5.55
	Enrollment / [REDACTED] / 182	WBC	10860 (cells/uL)	3300	8600
	Enrollment / [REDACTED] / -1	ALB	4.6 (g/dL)	4.1	5.1
	Enrollment / [REDACTED] / -1	ALP	175 (U/L)	38	113
	Enrollment / [REDACTED] / -1	ALT	18 (U/L)	7	23
	Enrollment / [REDACTED] / -1	AST	32 (U/L)	13	30
	Enrollment / [REDACTED] / -1	BILD	0.1 (mg/dL)	0	0.2
	Enrollment / [REDACTED] / -1	BILI	0.2 (mg/dL)	0.4	1.5
	Enrollment / [REDACTED] / -1	HCT	38.9 (%)	35.1	44.4

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

				Clinically Significant Limits		
Patient	Visit/ Date/ Study Day	Test	Result (units)	Very Low	Very High	
	Enrollment	-1	HGB	12.6 (g/dL)	11.6	14.8
	Enrollment	-1	PLAT	777 (10E3/uL)	158	348
	Enrollment	-1	PROT	6.7 (g/dL)	6.6	8.1
	Enrollment	-1	RBC	4.76 (10E6/uL)	3.86	4.92
	Enrollment	-1	WBC	12.74 (10E3/uL)	3.3	8.6
	Enrollment	/ 5	ALT	491 (U/L)	7	23
	Enrollment	/ 5	AST	781 (U/L)	13	30
	Enrollment	/ 6	ALT	1067 (U/L)	7	23
	Enrollment	/ 6	AST	1678 (U/L)	13	30
	Enrollment	27	ALT	115 (U/L)	7	23
	Enrollment	27	AST	124 (U/L)	13	30
	Enrollment	34	ALT	480 (U/L)	7	23
	Enrollment	34	AST	257 (U/L)	13	30
		Enrollment /	-24	ALB	3.8 (g/dL)	3.9
Enrollment /		-24	ALP	653 (IU/L)	494	1550
Enrollment /		-24	ALT	19 (IU/L)	9	53
Enrollment /		-24	AST	33 (IU/L)	23	70
Enrollment /		-24	BILD	0.1 (mg/dL)	0	0.2
Enrollment /		-24	BILI	0.4 (mg/dL)	0.1	0.6
Enrollment /		-24	HCT	34 (%)	32.8	42.7
Enrollment /		-24	HGB	11.7 (g/dL)	11.1	14.5
Enrollment /		-24	PLAT	428 (10E3/uL)	189	487

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[REDACTED]	Enrollment / [REDACTED] -24	PROT	5.9 (g/dL)	5.3	7.7
	Enrollment / [REDACTED] -24	RBC	4.3 (10E6/uL)	4.08	5.29
	Enrollment / [REDACTED] -24	WBC	7.8 (10E3/uL)	5.6	14.5
	Enrollment / [REDACTED] -1	ALB	4.1 (g/dL)	3.9	5.2
	Enrollment / [REDACTED] -1	ALT	26 (IU/L)	7	53
	Enrollment / [REDACTED] -1	AST	51 (IU/L)	17	70
	Enrollment / [REDACTED] -1	BILD	0 (mg/dL)	0	0.2
	Enrollment / [REDACTED] -1	BILI	0.7 (mg/dL)	0.1	0.9
	Enrollment / [REDACTED] -1	HCT	42.3 (%)	32.8	42.7
	Enrollment / [REDACTED] -1	HGB	13.2 (g/dL)	11.1	14.5
	Enrollment / [REDACTED] -1	PLAT	260 (10E3/uL)	189	487
	Enrollment / [REDACTED] -1	PROT	6.3 (g/dL)	5.3	8
	Enrollment / [REDACTED] -1	RBC	5.36 (10E6/uL)	4.08	5.29
	Enrollment / [REDACTED] -1	WBC	10.4 (10E3/uL)	5.6	14.5
	Enrollment / [REDACTED] -9	ALB	4.4 (g/dL)	4.1	5.1
	Enrollment / [REDACTED] -9	ALT	15 (U/L)	7	23
	Enrollment / [REDACTED] -9	AST	47 (U/L)	13	30
	Enrollment / [REDACTED] -9	BILI	0.3 (mg/dL)	0.4	1.5
	Enrollment / [REDACTED] -9	HCT	41.8 (%)	35.1	44.4
	Enrollment / [REDACTED] -9	HGB	13.9 (g/dL)	11.6	14.8
	Enrollment / [REDACTED] -9	PLAT	470 (10E9/L)	150	348

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jp
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment / / -9	PROT	6.7 (g/dL)	6.6	8.1
	Enrollment / / -9	RBC	520 (10E10/L)	386	492
	Enrollment / / -9	WBC	12070 (10E6/L)	3300	8600
	Enrollment / / 8	PLAT	511 (10E9/L)	158	348
	Enrollment / / 9	ALT	43 (U/L)	7	23
	Enrollment / / 9	AST	50 (U/L)	13	30
	Enrollment / / 25	ALT	351 (U/L)	7	23
	Enrollment / / 25	AST	282 (U/L)	13	30
	Enrollment / / 29	ALT	110 (U/L)	7	23
	Enrollment / / 29	AST	153 (U/L)	13	30
	Enrollment / / 32	ALT	63 (U/L)	7	23
	Enrollment / / 58	ALT	51 (U/L)	7	23
	Enrollment / / 58	AST	67 (U/L)	13	30
	Enrollment / / 78	ALT	60 (U/L)	7	23
	Enrollment / / 78	AST	76 (U/L)	13	30
	Enrollment / / 99	ALT	60 (U/L)	7	23
	Enrollment / / 99	AST	75 (U/L)	13	30
	Enrollment / / 73	ALB	4.8 (g/dL)	4.1	5.1
	Enrollment / / 73	ALP	262 (U/L)	36	113
	Enrollment / / 73	ALT	24 (U/L)	10	42
	Enrollment / / 73	AST	30 (U/L)	13	30

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment / / 73	BILD	0.1 (mg/dL)	0	0.4
	Enrollment / / 73	BILI	0.4 (mg/dL)	0.4	1.5
	Enrollment / / 73	HCT	36.2 (%)	40.7	50.1
	Enrollment / / 73	HGB	12.3 (g/dL)	13.7	16.8
	Enrollment / / 73	PLAT	39.9 (10E4/uL)	15	34.8
	Enrollment / / 73	PROT	6.9 (g/dL)	6.6	8.1
	Enrollment / / 73	RBC	4.5 (10E6/uL)	4.35	5.55
	Enrollment / / 73	WBC	12.5 (10E3/uL)	3.3	8.6
	Enrollment / / 76	AST	51 (U/L)	13	30
	Enrollment / / 77	ALT	51 (U/L)	10	42
	Enrollment / / 77	AST	113 (U/L)	13	30
	Enrollment / / 79	ALT	82 (U/L)	10	42
	Enrollment / / 79	AST	83 (U/L)	13	30
	Enrollment / / 100	AST	53 (U/L)	13	30
	Enrollment / / 583	ALB	4 (g/dL)	4.1	5.1
	Enrollment / / 583	ALP	222 (U/L)	38	113
	Enrollment / / 583	ALT	27 (U/L)	10	42
	Enrollment / / 583	AST	34 (U/L)	13	30
	Enrollment / / 583	BILI	0.2 (mg/dL)	0.4	1.5
	Enrollment / / 583	HCT	38.6 (%)	40.7	50.1
	Enrollment / / 583	HGB	12.8 (g/dL)	13.7	16.8
	Enrollment / / 583	PLAT	334 (10E3/uL)	158	348

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Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[REDACTED]	Enrollment / [REDACTED] 583	PROT	8.6 (g/dL)	6.6	8.1
	Enrollment / [REDACTED] 583	RBC	4.65 (10E6/uL)	4.35	5.55
	Enrollment / [REDACTED] 583	WBC	8.4 (10E3/uL)	3.3	8.6
	Enrollment / [REDACTED] 7	ALB	3.7 (g/dL)	4.1	5.1
	Enrollment / [REDACTED] 7	ALP	264 (U/L)	38	113
	Enrollment / [REDACTED] 7	ALT	58 (IU/L)	7	23
	Enrollment / [REDACTED] 7	AST	66 (IU/L)	13	30
	Enrollment / [REDACTED] 7	BILI	0.87 (mg/dL)	0.4	1.5
	Enrollment / [REDACTED] 7	HCT	30.4 (%)	35.1	44.4
	Enrollment / [REDACTED] 7	HGB	10.5 (g/dL)	11.6	14.8
	Enrollment / [REDACTED] 7	PLAT	834 (10E3/uL)	158	348
	Enrollment / [REDACTED] 7	PROT	5.5 (g/dL)	6.6	8.1
	Enrollment / [REDACTED] 7	RBC	3.24 (10E6/uL)	3.86	4.92
	Enrollment / [REDACTED] 7	WBC	9.1 (10E3/uL)	3.3	8.6
	Enrollment / [REDACTED] 3	ALB	3.2 (g/dL)	3.8	5.2
	Enrollment / [REDACTED] 3	ALP	187 (U/L)	-	-
	Enrollment / [REDACTED] 3	ALT	17 (U/L)	13	56
	Enrollment / [REDACTED] 3	AST	37 (U/L)	24	68
	Enrollment / [REDACTED] 3	BILD	0.54 (mg/dL)	0	0.4
	Enrollment / [REDACTED] 3	BILI	8.4 (mg/dL)	0.11	0.66
	Enrollment / [REDACTED] 3	HCT	41 (%)	29.6	41.5

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment / / 3	HGB	13.9 (g/dL)	9.8	14.1
	Enrollment / / 3	PLAT	269 (10E3/uL)	230	780
	Enrollment / / 3	PROT	4.9 (g/dL)	5.3	7.1
	Enrollment / / 3	RBC	1.39 (10E6/uL)	3.71	5.19
	Enrollment / / 3	WBC	6.6 (10E3/uL)	4.5	19
	Enrollment / / 0	ALB	4.5 (g/dL)	3.8	5.2
	Enrollment / / 0	ALP	145 (U/L)	138	451
	Enrollment / / 0	ALT	27 (U/L)	9	38
	Enrollment / / 0	AST	60 (U/L)	24	57
	Enrollment / / 0	BILD	0.1 (mg/dL)	0	0.4
	Enrollment / / 0	BILI	0.24 (mg/dL)	0.16	0.67
	Enrollment / / 0	HCT	32.8 (%)	31.7	42.4
	Enrollment / / 0	HGB	11.1 (g/dL)	10.7	14.1
	Enrollment / / 0	PLAT	428 (10E3/uL)	168	650
	Enrollment / / 0	PROT	6.5 (g/dL)	5.7	7.5
	Enrollment / / 0	RBC	4.2 (10E6/uL)	3.93	5.38
	Enrollment / / 0	WBC	9.2 (10E3/uL)	4.3	19.1
	Enrollment / / 23	ALT	49 (U/L)	9	38
	Enrollment / / 23	AST	96 (U/L)	24	57
	Enrollment / / 26	ALT	433 (U/L)	9	38
	Enrollment / / 26	AST	668 (U/L)	24	57
	Enrollment / / 27	ALT	463 (U/L)	9	38

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment / / 27	AST	617 (U/L)	24	57
	Enrollment / / 34	ALT	342 (U/L)	9	38
	Enrollment / / 34	AST	253 (U/L)	24	57
	Enrollment / / 41	ALT	385 (U/L)	9	38
	Enrollment / / 41	AST	348 (U/L)	24	57
	Enrollment / / 43	ALT	489 (U/L)	9	38
	Enrollment / / 43	AST	415 (U/L)	24	57
	Enrollment / / 58	ALT	174 (U/L)	9	38
	Enrollment / / 58	AST	132 (U/L)	24	57
	Enrollment / / 84	ALT	48 (U/L)	9	38
	Enrollment / / 84	AST	76 (U/L)	24	57
	Enrollment / / 112	AST	67 (U/L)	24	57
	Enrollment / / 14	ALB	4.7 (g/dL)	4.1	5.1
	Enrollment / / 14	ALP	213 (U/L)	38	113
	Enrollment / / 14	ALT	17 (U/L)	7	23
	Enrollment / / 14	AST	33 (U/L)	13	30
	Enrollment / / 14	BILI	0.3 (mg/dL)	0.4	1.5
	Enrollment / / 14	HCT	40.6 (%)	35.1	44.4
	Enrollment / / 14	HGB	12.7 (g/dL)	11.6	14.8
	Enrollment / / 14	PLAT	444 (10E3/uL)	158	348
	Enrollment / / 14	PROT	6.9 (g/dL)	6.6	8.1
	Enrollment / / 14	RBC	4.64 (10E6/uL)	3.86	4.92

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[REDACTED]	Enrollment / [REDACTED]	14 WBC	5.8 (10E3/uL)	3.3	8.6
	Enrollment / [REDACTED]	18 AST	64 (U/L)	13	30
	Enrollment / [REDACTED]	21 ALT	25 (U/L)	7	23
	Enrollment / [REDACTED]	21 AST	101 (U/L)	13	30
	Enrollment / [REDACTED]	21 PLAT	25 (10E3/uL)	158	348
	Enrollment / [REDACTED]	23 PLAT	39 (10E3/uL)	158	348
	Enrollment / [REDACTED]	81 ALT	53 (U/L)	7	23
	Enrollment / [REDACTED]	-14 ALB	4.3 (g/dL)	3.5	4.4
	Enrollment / [REDACTED]	-14 ALP	732 (U/L)	433	1140
	Enrollment / [REDACTED]	-14 ALT	38 (U/L)	11	55
	Enrollment / [REDACTED]	-14 AST	37 (U/L)	21	70
	Enrollment / [REDACTED]	-14 BILD	0 (mg/dL)	0	0.4
	Enrollment / [REDACTED]	-14 BILI	0.5 (mg/dL)	0.2	1.2
	Enrollment / [REDACTED]	-14 HCT	36.3 (%)	37.4	48.6
	Enrollment / [REDACTED]	-14 HGB	12.4 (g/dL)	12.2	16.2
	Enrollment / [REDACTED]	-14 PLAT	594 (10E3/uL)	138	309
[REDACTED]	Enrollment / [REDACTED]	-14 PROT	5.9 (g/dL)	5	6.5
	Enrollment / [REDACTED]	-14 RBC	4.11 (10E6/uL)	3.5	4.7
	Enrollment / [REDACTED]	-14 WBC	8.4 (10E3/uL)	5	19.5
	Enrollment / [REDACTED]	/ 1 ALB	4.1 (g/dL)	3.5	4.4
	Enrollment / [REDACTED]	/ 1 ALP	769 (U/L)	433	1140
[REDACTED]	Enrollment / [REDACTED]	/ 1 ALT	38 (U/L)	11	55

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jp
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment / / 1	AST	37 (U/L)	21	70
	Enrollment / / 1	BILD	0 (mg/dL)	0	0.4
	Enrollment / / 1	BILI	0.4 (mg/dL)	0.2	1.2
	Enrollment / / 1	HCT	35.7 (%)	37.4	48.6
	Enrollment / / 1	HGB	11.9 (g/dL)	12.2	16.2
	Enrollment / / 1	PLAT	613 (10E3/uL)	138	309
	Enrollment / / 1	PROT	5.7 (g/dL)	5	6.5
	Enrollment / / 1	RBC	4.14 (10E6/uL)	3.5	4.7
	Enrollment / / 1	WBC	9.2 (10E3/uL)	5	19.5
	Enrollment / / 15	ALB	4.4 (g/dL)	3.6	4.6
	Enrollment / / 15	ALP	713 (U/L)	406	1100
	Enrollment / / 15	ALT	38 (U/L)	12	62
	Enrollment / / 15	AST	45 (U/L)	23	39
	Enrollment / / 15	BILD	0 (mg/dL)	0	0.4
	Enrollment / / 15	BILI	0.4 (mg/dL)	0.2	1.2
	Enrollment / / 15	HCT	37.7 (%)	37.4	48.6
	Enrollment / / 15	HGB	12.3 (g/dL)	10.3	12.3
	Enrollment / / 15	PLAT	511 (10E3/uL)	138	309
	Enrollment / / 15	PROT	5.8 (g/dL)	5.2	6.7
	Enrollment / / 15	RBC	4.51 (10E6/uL)	3.35	4.05
	Enrollment / / 15	WBC	9.3 (10E3/uL)	5	19.5
	Enrollment / / 29	ALB	4.4 (g/dL)	3.6	4.6
	Enrollment / / 29	ALP	535 (U/L)	406	1100

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment / / 29	ALT	36 (U/L)	23	39
	Enrollment / / 29	AST	40 (U/L)	24	40
	Enrollment / / 29	BILD	0 (mg/dL)	0	0.4
	Enrollment / / 29	BILI	0.3 (mg/dL)	0.2	1.2
	Enrollment / / 29	HCT	36.8 (%)	37.4	48.6
	Enrollment / / 29	HGB	11.9 (g/dL)	10.3	12.3
	Enrollment / / 29	PLAT	484 (10E3/uL)	138	309
	Enrollment / / 29	PROT	6 (g/dL)	5.2	6.7
	Enrollment / / 29	RBC	4.47 (10E6/uL)	3.5	4.7
	Enrollment / / 29	WBC	10.4 (10E3/uL)	5	19.5
	Enrollment / / 36	ALB	4.4 (g/dL)	3.8	4.7
	Enrollment / / 36	ALP	580 (U/L)	386	1070
	Enrollment / / 36	ALT	35 (U/L)	13	64
	Enrollment / / 36	AST	41 (U/L)	24	43
	Enrollment / / 36	BILD	0 (mg/dL)	0	0.4
	Enrollment / / 36	BILI	0.3 (mg/dL)	0.2	1.2
	Enrollment / / 36	HCT	37.8 (%)	37.4	48.6
	Enrollment / / 36	HGB	12.4 (g/dL)	10.3	12.3
	Enrollment / / 36	PLAT	482 (10E3/uL)	138	309
	Enrollment / / 36	PROT	6 (g/dL)	5.4	6.9
	Enrollment / / 36	RBC	4.73 (10E6/uL)	3.35	4.05
	Enrollment / / 36	WBC	8.7 (10E3/uL)	5	19.5
	Enrollment / / 41	ALB	5.1 (g/dL)	3.8	4.7

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Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment / / 41	ALP	636 (U/L)	386	1070
	Enrollment / / 41	ALT	56 (U/L)	13	64
	Enrollment / / 41	AST	100 (U/L)	24	43
	Enrollment / / 41	BILD	0 (mg/dL)	0	0.4
	Enrollment / / 41	BILI	0.2 (mg/dL)	0.2	1.2
	Enrollment / / 41	HCT	38 (%)	37.4	48.6
	Enrollment / / 41	HGB	12.4 (g/dL)	10.3	12.3
	Enrollment / / 41	PLAT	214 (10E3/uL)	138	309
	Enrollment / / 41	PROT	7.1 (g/dL)	5.4	6.9
	Enrollment / / 41	RBC	4.71 (10E6/uL)	3.35	4.05
	Enrollment / / 41	WBC	4.7 (10E3/uL)	5	19.5
	Enrollment / / 44	ALB	4.5 (g/dL)	3.8	4.7
	Enrollment / / 44	ALP	575 (U/L)	386	1070
	Enrollment / / 44	ALT	42 (U/L)	13	64
	Enrollment / / 44	AST	90 (U/L)	24	43
	Enrollment / / 44	BILD	0 (mg/dL)	0	0.4
	Enrollment / / 44	BILI	0.3 (mg/dL)	0.2	1.2
	Enrollment / / 44	HCT	40.2 (%)	37.4	48.6
	Enrollment / / 44	HGB	13.3 (g/dL)	10.3	12.3
	Enrollment / / 44	PLAT	143 (10E3/uL)	138	309
	Enrollment / / 44	PROT	6.3 (g/dL)	5.4	6.9
	Enrollment / / 44	RBC	4.96 (10E6/uL)	3.35	4.05
	Enrollment / / 44	WBC	10.9 (10E3/uL)	5	19.5

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jp
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment /	51 ALB	4.2 (g/dL)	3.8	4.7
	Enrollment /	51 ALP	478 (U/L)	386	1070
	Enrollment /	51 ALT	26 (U/L)	13	64
	Enrollment /	51 AST	46 (U/L)	24	43
	Enrollment /	51 BILD	0.1 (mg/dL)	0	0.4
	Enrollment /	51 BILI	0.2 (mg/dL)	0.2	1.2
	Enrollment /	51 HCT	38.3 (%)	37.4	48.6
	Enrollment /	51 HGB	13 (g/dL)	10.3	12.3
	Enrollment /	51 PLAT	349 (10E3/uL)	138	309
	Enrollment /	51 PROT	5.9 (g/dL)	5.4	6.9
	Enrollment /	51 RBC	4.83 (10E6/uL)	3.35	4.05
	Enrollment /	51 WBC	7.2 (10E3/uL)	5	19.5
	Enrollment /	58 ALB	4.6 (g/dL)	3.8	4.7
	Enrollment /	58 ALP	478 (U/L)	386	1070
	Enrollment /	58 ALT	28 (U/L)	13	64
	Enrollment /	58 AST	49 (U/L)	24	43
	Enrollment /	58 BILD	0.1 (mg/dL)	0	0.4
	Enrollment /	58 BILI	0.3 (mg/dL)	0.2	1.2
	Enrollment /	58 HCT	43.2 (%)	37.4	48.6
	Enrollment /	58 HGB	14.1 (g/dL)	10.3	12.3
	Enrollment /	58 PLAT	373 (10E3/uL)	138	309
	Enrollment /	58 PROT	6.6 (g/dL)	5.4	6.9
	Enrollment /	58 RBC	5.41 (10E6/uL)	3.35	4.05

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment / / 58	WBC	9.5 (10E3/uL)	5	19.5
	Enrollment / / 65	ALB	4.8 (g/dL)	3.8	4.7
	Enrollment / / 65	ALP	474 (U/L)	386	1070
	Enrollment / / 65	ALT	29 (U/L)	13	64
	Enrollment / / 65	AST	46 (U/L)	24	43
	Enrollment / / 65	BILD	0 (mg/dL)	0	0.4
	Enrollment / / 65	BILI	0.2 (mg/dL)	0.2	1.2
	Enrollment / / 65	HCT	42.2 (%)	37.4	48.6
	Enrollment / / 65	HGB	13.8 (g/dL)	10.3	12.3
	Enrollment / / 65	PLAT	373 (10E3/uL)	138	309
	Enrollment / / 65	PROT	6.6 (g/dL)	5.4	6.9
	Enrollment / / 65	RBC	5.3 (10E6/uL)	3.35	4.05
	Enrollment / / 65	WBC	5.5 (10E3/uL)	5	19.5
	Enrollment / / 79	ALB	4.7 (g/dL)	3.8	4.7
	Enrollment / / 79	ALP	501 (U/L)	386	1070
	Enrollment / / 79	ALT	37 (U/L)	13	64
	Enrollment / / 79	AST	57 (U/L)	24	43
	Enrollment / / 79	BILD	0 (mg/dL)	0	0.4
	Enrollment / / 79	BILI	0.3 (mg/dL)	0.2	1.2
	Enrollment / / 79	HCT	43.2 (%)	37.4	48.6
	Enrollment / / 79	HGB	14 (g/dL)	10.3	12.3
	Enrollment / / 79	PLAT	421 (10E3/uL)	138	309
	Enrollment / / 79	PROT	6.6 (g/dL)	5.4	6.9

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[REDACTED]	Enrollment / [REDACTED] / 79	RBC	5.4 (10E6/uL)	3.35	4.05
	Enrollment / [REDACTED] / 79	WBC	7.9 (10E3/uL)	5	19.5
	Enrollment / [REDACTED] / 92	ALB	4.6 (g/dL)	3.8	4.7
	Enrollment / [REDACTED] / 92	ALP	526 (U/L)	386	1070
	Enrollment / [REDACTED] / 92	ALT	35 (U/L)	13	64
	Enrollment / [REDACTED] / 92	AST	59 (U/L)	24	43
	Enrollment / [REDACTED] / 92	BILD	0 (mg/dL)	0	0.4
	Enrollment / [REDACTED] / 92	BILI	0.3 (mg/dL)	0.2	1.2
	Enrollment / [REDACTED] / 92	HCT	41.5 (%)	37.4	48.6
	Enrollment / [REDACTED] / 92	HGB	13.5 (g/dL)	10.3	12.3
	Enrollment / [REDACTED] / 92	PLAT	375 (10E3/uL)	138	309
	Enrollment / [REDACTED] / 92	PROT	6.3 (g/dL)	5.4	6.9
	Enrollment / [REDACTED] / 92	RBC	5.27 (10E6/uL)	3.35	4.05
	Enrollment / [REDACTED] / 92	WBC	6.1 (10E3/uL)	5	19.5
[REDACTED]	Enrollment / [REDACTED] / -1	ALP	878 (IU/L)	106	322
	Enrollment / [REDACTED] / -1	ALT	14 (IU/L)	7	23
	Enrollment / [REDACTED] / -1	AST	24 (IU/L)	13	30
	Enrollment / [REDACTED] / -1	BILD	0.03 (mg/dL)	0.1	0.3
	Enrollment / [REDACTED] / -1	BILI	0.31 (mg/dL)	0.4	1.5
	Enrollment / [REDACTED] / -1	HCT	36.9 (%)	35.1	44.4
	Enrollment / [REDACTED] / -1	HGB	12.4 (g/dL)	11.6	14.8
	Enrollment / [REDACTED] / -1	PLAT	423 (10E3/uL)	158	348

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[REDACTED]	Enrollment / [REDACTED] -1	PROT	7.1 (g/dL)	6.6	8.1
	Enrollment / [REDACTED] -1	RBC	4.59 (10E12/L)	3.86	4.92
	Enrollment / [REDACTED] -1	WBC	11.2 (10E9/L)	3.3	8.6
	Enrollment / [REDACTED] 267	ALB	4.9 (g/dL)	4.1	5.1
	Enrollment / [REDACTED] 267	ALP	629 (U/L)	-	-
	Enrollment / [REDACTED] 267	ALT	22 (U/L)	10	42
	Enrollment / [REDACTED] 267	AST	42 (U/L)	13	30
	Enrollment / [REDACTED] 267	BILD	0.1 (mg/dL)	0	0.2
	Enrollment / [REDACTED] 267	BILI	0.3 (mg/dL)	0.4	1.5
	Enrollment / [REDACTED] 267	HCT	39.9 (%)	40.7	50.1
	Enrollment / [REDACTED] 267	HGB	12.7 (g/dL)	13.7	16.8
	Enrollment / [REDACTED] 267	PLAT	325 (10E3/uL)	158	348
	Enrollment / [REDACTED] 267	PROT	7.1 (g/dL)	6.6	8.1
	Enrollment / [REDACTED] 267	RBC	5.54 (10E6/uL)	4.35	5.55
	Enrollment / [REDACTED] 267	WBC	12 (10E3/uL)	3.3	8.6
	Enrollment / [REDACTED] / 0	ALT	21 (U/L)	7	23
	Enrollment / [REDACTED] / 0	AST	32 (U/L)	13	30
	Enrollment / [REDACTED] / 0	PLAT	396 (10E3/uL)	158	348
[REDACTED]	Enrollment / [REDACTED] 307	ALB	4.8 (g/dL)	-	-
	Enrollment / [REDACTED] 307	ALP	178 (U/L)	-	-

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Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[REDACTED]	Enrollment / [REDACTED]	307 ALT	23 (U/L)	-	-
	Enrollment / [REDACTED]	307 AST	43 (U/L)	-	-
	Enrollment / [REDACTED]	307 BILI	0.7 (mg/dL)	-	-
	Enrollment / [REDACTED]	307 HCT	36.4 (%)	-	-
	Enrollment / [REDACTED]	307 HGB	12.1 (g/dL)	-	-
	Enrollment / [REDACTED]	307 PLAT	327 (10E3/uL)	-	-
	Enrollment / [REDACTED]	307 PROT	6.9 (g/dL)	-	-
	Enrollment / [REDACTED]	307 RBC	4.32 (10E6/uL)	-	-
	Enrollment / [REDACTED]	307 WBC	6.2 (10E3/uL)	-	-
	Enrollment / [REDACTED]	311 ALT	956 (U/L)	-	-
	Enrollment / [REDACTED]	311 AST	1370 (U/L)	-	-
	Enrollment / [REDACTED]	311 PLAT	152 (10E3/uL)	-	-
	Enrollment / [REDACTED]	482 ALT	137 (U/L)	10	42
	Enrollment / [REDACTED]	482 AST	100 (U/L)	13	30
	Enrollment / [REDACTED]	362 ALB	4 (g/dL)	4.1	5.1
	Enrollment / [REDACTED]	362 ALP	472 (U/L)	106	322
[REDACTED]	Enrollment / [REDACTED]	362 ALT	80 (U/L)	7	23
	Enrollment / [REDACTED]	362 AST	55 (U/L)	13	30
	Enrollment / [REDACTED]	362 BILI	0.5 (mg/dL)	0.4	1.5
	Enrollment / [REDACTED]	362 HCT	33.9 (%)	35.1	44.4
	Enrollment / [REDACTED]	362 HGB	10.9 (g/dL)	11.6	14.8
	Enrollment / [REDACTED]	362 PLAT	336 (10E3/uL)	158	348

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Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[REDACTED]	Enrollment / [REDACTED]	362 PROT	6.3 (g/dL)	6.6	8.1
	Enrollment / [REDACTED]	362 RBC	4.07 (10E6/uL)	386	492
	Enrollment / [REDACTED]	362 WBC	6.2 (10E3/uL)	3.3	8.6
	Enrollment / [REDACTED]	134 ALB	4.1 (g/dL)	4.1	5.1
	Enrollment / [REDACTED]	134 ALP	528 (U/L)	106	322
	Enrollment / [REDACTED]	134 ALT	25 (U/L)	7	23
	Enrollment / [REDACTED]	134 AST	37 (U/L)	13	30
	Enrollment / [REDACTED]	134 BILI	0.2 (mg/dL)	0.4	1.5
	Enrollment / [REDACTED]	134 HCT	35.5 (%)	35.1	44.4
	Enrollment / [REDACTED]	134 HGB	12 (g/dL)	11.6	14.8
	Enrollment / [REDACTED]	134 PLAT	301 (10E3/uL)	158	348
	Enrollment / [REDACTED]	134 PROT	6.2 (g/dL)	6.6	8.1
	Enrollment / [REDACTED]	134 RBC	4.2 (10E6/uL)	3.86	4.92
	Enrollment / [REDACTED]	134 WBC	8 (10E3/uL)	3.3	8.6
	Enrollment / [REDACTED]	16 ALB	4.4 (g/dL)	4.1	5.1
	Enrollment / [REDACTED]	16 ALT	27 (U/L)	7	23
	Enrollment / [REDACTED]	16 AST	30 (U/L)	13	30
	Enrollment / [REDACTED]	16 BILI	0.3 (mg/dL)	0.4	1.5
	Enrollment / [REDACTED]	16 HCT	35.9 (%)	35.1	44.4
	Enrollment / [REDACTED]	16 HGB	12 (g/dL)	11.6	14.8
	Enrollment / [REDACTED]	16 PLAT	488 (10E3/uL)	158	348

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits		
				Very Low	Very High	
	Enrollment	16	PROT	6.5 (g/dL)	6.6	8.1
	Enrollment	16	RBC	4.35 (10E6/uL)	3.86	4.92
	Enrollment	16	WBC	10.5 (10E3/uL)	3.3	8.6
	Enrollment /	212	ALT	12 (U/L)	7	23
	Enrollment /	212	AST	29 (U/L)	13	30
	Enrollment /	212	BILI	0.8 (mg/dL)	0.4	1.5
	Enrollment /	212	HGB	11.8 (g/dL)	10.9	14.2
	Enrollment /	212	PLAT	176 (10E3/uL)	180	620
	Enrollment /	212	RBC	4.39 (10E6/uL)	4.35	5.05
	Enrollment /	212	WBC	10000 (cells/uL)	4200	18800
	Enrollment	/ 0	ALB	3.8 (g/dL)	4.1	5.1
	Enrollment	/ 0	ALT	35 (U/L)	10	42
	Enrollment	/ 0	AST	35 (U/L)	13	30
	Enrollment	/ 0	HCT	34.9 (%)	-	-
	Enrollment	/ 0	HGB	11.7 (g/dL)	-	-
	Enrollment	/ 0	PLAT	538 (10E3/uL)	-	-
	Enrollment	/ 0	PROT	5.3 (g/dL)	6.6	8.1
	Enrollment	/ 0	RBC	4.17 (10E6/uL)	-	-
	Enrollment	/ 0	WBC	810 (10E3/uL)	-	-
	Enrollment	20	ALB	4.1 (g/dL)	3.9	4.9

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jp
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment /	20 ALT	20 (U/L)	8	42
	Enrollment /	20 AST	26 (U/L)	13	33
	Enrollment /	20 BILI	0.38 (mg/dL)	0.3	1.2
	Enrollment /	20 HCT	38.3 (%)	40	50
	Enrollment /	20 HGB	12 (g/dL)	13.5	17
	Enrollment /	20 PLAT	39.8 (10E4/uL)	15	35
	Enrollment /	20 PROT	5.8 (g/dL)	6.7	8.3
	Enrollment /	20 RBC	4.85 (10E6/uL)	4.3	5.7
	Enrollment /	20 WBC	8.48 (10E3/uL)	3.5	8.5
	Enrollment /	24 AST	66 (U/L)	13	30
	Enrollment /	24 PLAT	19.1 (10E4/uL)	15	35
	Enrollment /	25 ALT	46 (U/L)	8	42
	Enrollment /	26 HGB	10.9 (g/dL)	13.5	17
	Enrollment /	27 PLAT	3 (10E4/uL)	15	35
	Enrollment /	28 ALB	4.19 (g/dL)	3.9	4.9
	Enrollment /	28 ALP	166 (IU/L)	128	469
	Enrollment /	28 ALT	14 (IU/L)	4	44
	Enrollment /	28 AST	28 (IU/L)	8	38
	Enrollment /	28 BILI	0.3 (mg/dL)	0.2	1.2
	Enrollment /	28 HGB	12.1 (g/dL)	13.5	17.6
	Enrollment /	28 PLAT	36.2 (10E4/uL)	13	36.2
	Enrollment /	28 PROT	6.25 (g/dL)	6.7	8.3

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment /	28 RBC	4.41 (10E6/uL)	4.1	5.3
	Enrollment /	28 WBC	10160 (cells/uL)	3900	9800
	Enrollment /	32 AST	54 (IU/L)	8	38
	Enrollment /	35 ALT	72 (IU/L)	4	44
	Enrollment /	35 AST	126 (IU/L)	8	38
	Enrollment /	35 PLAT	8.2 (10E4/uL)	13	36.2
	Enrollment /	39 ALT	403 (IU/L)	4	44
	Enrollment /	39 AST	633 (IU/L)	8	38
	Enrollment /	39 HGB	9.5 (g/dL)	13.5	17.6
	Enrollment /	39 PLAT	3 (10E4/uL)	13	36.2
	Enrollment /	43 ALT	625 (IU/L)	4	44
	Enrollment /	43 AST	505 (IU/L)	8	38
	Enrollment /	43 HGB	8.9 (g/dL)	13.5	17.6
	Enrollment /	43 PLAT	11.4 (10E4/uL)	13	36.2
	Enrollment /	46 ALT	504 (IU/L)	4	44
	Enrollment /	46 AST	251 (IU/L)	3	38
	Enrollment /	49 ALT	258 (IU/L)	4	44
	Enrollment /	49 AST	95 (IU/L)	8	38
	Enrollment /	57 ALT	161 (IU/L)	4	44
	Enrollment /	57 AST	151 (IU/L)	8	38
	Enrollment /	71 ALT	1710 (IU/L)	4	44
	Enrollment /	71 AST	2160 (IU/L)	8	38
	Enrollment /	78 ALT	1206 (IU/L)	4	44

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Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment / / 78	AST	1112 (IU/L)	8	38
	Enrollment / / 87	ALT	379 (IU/L)	4	44
	Enrollment / / 87	AST	272 (IU/L)	8	38
	Enrollment / / 99	ALT	71 (IU/L)	4	44
	Enrollment / / 99	AST	60 (IU/L)	8	38
	Enrollment / / 113	ALT	23 (IU/L)	4	44
	Enrollment / / 113	AST	35 (IU/L)	8	38
	Enrollment / / -6	ALB	4.3 (g/dL)	4.1	5.1
	Enrollment / / -6	ALP	193 (IU/L)	38	113
	Enrollment / / -6	ALT	12 (IU/L)	7	23
	Enrollment / / -6	AST	34 (IU/L)	13	30
	Enrollment / / -6	BILD	0.1 (mg/dL)	0	0.3
	Enrollment / / -6	BILI	0.3 (mg/dL)	0.4	1.5
	Enrollment / / -6	HCT	36.7 (%)	35.1	44.4
	Enrollment / / -6	HGB	11.6 (g/dL)	11.6	14.8
	Enrollment / / -6	PLAT	425 (10E3/uL)	158	348
	Enrollment / / -6	PROT	6.6 (g/dL)	6.6	8.1
	Enrollment / / -6	RBC	4.51 (10E6/uL)	3.86	4.92
	Enrollment / / -6	WBC	8.3 (10E3/uL)	3.3	8.6
	Enrollment / / 294	ALB	4.6 (g/dL)	4.1	5.1
	Enrollment / / 294	ALT	50 (U/L)	7	23

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Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment	294 AST	42 (U/L)	13	30
	Enrollment	294 HCT	38.6 (%)	35.1	44.4
	Enrollment	294 HGB	13.8 (g/dL)	11.6	14.8
	Enrollment	294 PLAT	295 (10E3/uL)	300	450
	Enrollment	294 PROT	7.1 (g/dL)	6.6	8.1
	Enrollment	294 RBC	4.55 (10E6/uL)	3.86	4.92
	Enrollment	294 WBC	10.4 (10E3/uL)	3.3	8.6
	Enrollment	9 ALB	4.6 (g/dL)	4.1	5.1
	Enrollment	9 ALP	156 (U/L)	38	113
	Enrollment	9 ALT	16 (U/L)	7	23
	Enrollment	9 AST	33 (U/L)	13	30
	Enrollment	9 BILD	0 (mg/dL)	0	0.4
	Enrollment	9 BILI	0.4 (mg/dL)	0.2	1.2
	Enrollment	9 HCT	35.1 (%)	35.1	44.4
	Enrollment	9 HGB	11.3 (g/dL)	11.6	14.8
	Enrollment	9 PLAT	320 (10E3/uL)	300	450
	Enrollment	9 PROT	6.8 (g/dL)	6.6	8.1
	Enrollment	9 RBC	4.35 (10E6/uL)	3.86	4.92
	Enrollment	9 WBC	16.3 (10E3/uL)	3.3	8.6
	Enrollment	15 AST	93 (U/L)	13	30
	Enrollment	15 PLAT	83 (10E3/uL)	300	450
	Enrollment	18 PLAT	63 (10E3/uL)	300	450

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment / / 21	AST	718 (U/L)	13	30
	Enrollment / / 0	ALT	29 (U/L)	10	42
	Enrollment / / 0	AST	44 (U/L)	13	30
	Enrollment / / 0	BILI	1 (mg/dL)	0.4	1.5
	Enrollment / / 0	HCT	35.1 (%)	40.7	50.1
	Enrollment / / 0	HGB	11.5 (g/dL)	13.7	16.8
	Enrollment / / 0	PLAT	355 (10E3/uL)	158	348
	Enrollment / / 0	RBC	4.13 (10E6/uL)	4.35	5.55
	Enrollment / / 0	WBC	12.8 (10E3/uL)	3.3	8.6
	Enrollment / / 101	ALB	4.4 (g/dL)	4	5
	Enrollment / / 101	ALP	163 (IU/L)	38	113
	Enrollment / / 101	ALT	14 (IU/L)	8	42
	Enrollment / / 101	AST	29 (IU/L)	13	33
	Enrollment / / 101	BILI	0.4 (mg/dL)	0.3	1.2
	Enrollment / / 101	HCT	35.9 (%)	39.7	51
	Enrollment / / 101	HGB	11.6 (g/dL)	13.5	17
	Enrollment / / 101	PLAT	438 (10E3/uL)	130	350
	Enrollment / / 101	PROT	6.4 (g/dL)	6.7	8.3
	Enrollment / / 101	RBC	4.51 (10E6/uL)	4.3	5.5
	Enrollment / / 101	WBC	11.17 (10E3/uL)	3.3	8.8

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits		
				Very Low	Very High	
	Enrollment /	-20	ALB	4.6 (g/dL)	3.5	5.5
	Enrollment /	-20	ALP	427 (IU/L)	106	322
	Enrollment /	-20	ALT	22 (IU/L)	10	42
	Enrollment /	-20	AST	54 (IU/L)	13	30
	Enrollment /	-20	BILI	0.4 (mg/dL)	0.1	1.2
	Enrollment /	-20	HCT	31.9 (%)	40	50
	Enrollment /	-20	HGB	10.9 (g/dL)	13	17
	Enrollment /	-20	PLAT	310 (10E3/uL)	150	350
	Enrollment /	-20	PROT	6.5 (g/dL)	6.5	8.2
	Enrollment /	-20	RBC	4.22 (10E6/uL)	4	5.4
	Enrollment /	-20	WBC	11.8 (10E3/uL)	4.7	8.7
	Enrollment	20	ALB	4 (g/dL)	3.5	5.5
	Enrollment	20	ALT	28 (IU/L)	10	42
	Enrollment	20	AST	46 (IU/L)	13	30
	Enrollment	20	BILD	1.5 (mg/dL)	0	0.3
	Enrollment	20	BILI	7.7 (mg/dL)	0.1	1.2
	Enrollment	20	HCT	47.4 (%)	40	50
	Enrollment	20	HGB	16.7 (g/dL)	13	17
	Enrollment	20	PLAT	39.9 (10E4/uL)	15	34.8
	Enrollment	20	PROT	5.5 (g/dL)	6.5	8.2
	Enrollment	20	RBC	5 (10E6/uL)	4.4	5.6
	Enrollment	20	WBC	12.3 (10E3/uL)	3.3	8.6

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jp
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment /	25 ALT	63 (IU/L)	10	42
	Enrollment /	25 AST	248 (IU/L)	13	30
	Enrollment /	25 PLAT	10 (10E4/uL)	15	34.8
	Enrollment /	26 ALT	71 (IU/L)	10	42
	Enrollment /	26 PLAT	6.9 (10E4/uL)	15	34.8
	Enrollment /	-15 ALT	24 (U/L)	12	51
	Enrollment /	-15 AST	37 (U/L)	25	67
	Enrollment /	-15 BILI	0.48 (mg/dL)	0.12	0.59
	Enrollment /	-15 HCT	40.9 (%)	30	41
	Enrollment /	-15 HGB	12.8 (g/dL)	10	14
	Enrollment /	-15 PLAT	348 (10E3/uL)	200	740
	Enrollment /	22 ALB	3.7 (g/dL)	3.1	3.6
	Enrollment /	22 ALT	39 (U/L)	13	66
	Enrollment /	22 AST	33 (U/L)	22	66
	Enrollment /	22 BILD	0.08 (mg/dL)	-	-
	Enrollment /	22 BILI	0.16 (mg/dL)	0.13	0.8
	Enrollment /	22 HCT	22.5 (%)	28.5	41.1
	Enrollment /	22 HGB	7.4 (g/dL)	9.5	13.7
	Enrollment /	22 PLAT	75.2 (10E4/uL)	25	82
	Enrollment /	22 PROT	5.8 (g/dL)	5.1	5.8
	Enrollment /	22 RBC	2.64 (10E6/uL)	3.4	5

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment / [REDACTED] 22	WBC	7.83 (10E3/uL)	4.56	18.9
	Enrollment / [REDACTED] 51	ALT	54 (U/L)	13	56
	Enrollment / [REDACTED] 51	AST	73 (U/L)	23	67
	Enrollment / [REDACTED] 67	ALT	112 (U/L)	13	56
	Enrollment / [REDACTED] 67	AST	117 (U/L)	23	67
	Enrollment / [REDACTED] -15	ALB	4 (g/dL)	3.7	5.5
	Enrollment / [REDACTED] -15	ALP	635 (U/L)	106	322
	Enrollment / [REDACTED] -15	ALT	22 (U/L)	5	45
	Enrollment / [REDACTED] -15	AST	40 (U/L)	10	40
	Enrollment / [REDACTED] -15	BILD	0.1 (mg/dL)	0	0.4
	Enrollment / [REDACTED] -15	BILI	0.5 (mg/dL)	0.3	1.2
	Enrollment / [REDACTED] -15	HCT	37.8 (%)	34.3	45.2
	Enrollment / [REDACTED] -15	HGB	11.5 (g/dL)	11.2	15.2
	Enrollment / [REDACTED] -15	PLAT	430 (10E3/uL)	140	380
	Enrollment / [REDACTED] -15	PROT	6.1 (g/dL)	6.5	8.2
	Enrollment / [REDACTED] -15	WBC	7.38 (10E3/uL)	3.5	9.7
	Enrollment / [REDACTED] / 3	ALT	21 (U/L)	4	44
	Enrollment / [REDACTED] / 3	AST	42 (U/L)	8	38
	Enrollment / [REDACTED] / 3	PLAT	414 (10E3/uL)	140	340
	Enrollment / [REDACTED] 35	ALB	4.9 (g/dL)	4.1	5.11

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
	Enrollment	35 ALP	163 (IU/L)	38	113
	Enrollment	35 ALT	14 (IU/L)	7	23
	Enrollment	35 AST	42 (IU/L)	13	30
	Enrollment	35 BILD	0 (mg/dL)	0	0.3
	Enrollment	35 BILI	0.4 (mg/dL)	0.4	1.5
	Enrollment	35 HCT	37.5 (%)	35.1	44.4
	Enrollment	35 HGB	12.4 (g/dL)	11.6	14.8
	Enrollment	35 PLAT	352 (10E3/uL)	158	348
	Enrollment	35 PROT	6.7 (g/dL)	6.6	8.1
	Enrollment	35 RBC	4.71 (10E12/L)	3.86	4.92
	Enrollment	35 WBC	9.1 (10E9/L)	3.3	8.6
	Enrollment	42 ALB	3.8 (g/dL)	3.1	4.6
	Enrollment	42 ALP	208 (U/L)	168	567
	Enrollment	42 ALT	26 (U/L)	13	56
	Enrollment	42 AST	38 (U/L)	22	66
	Enrollment	42 BILI	0.2 (mg/dL)	0.1	0.8
	Enrollment	42 HCT	36.6 (%)	30	41.6
	Enrollment	42 HGB	12.4 (g/dL)	10	14.2
	Enrollment	42 PLAT	560 (10E3/uL)	220	760
	Enrollment	42 PROT	5.5 (g/dL)	5.1	6.8
	Enrollment	42 RBC	4.34 (10E6/uL)	3.8	5.23
	Enrollment	42 WBC	11.1 (10E3/uL)	4.4	19.1

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[REDACTED]	Enrollment / [REDACTED] / 48	ALT	385 (U/L)	9	38
	Enrollment / [REDACTED] / 48	AST	661 (U/L)	23	57
	Enrollment / [REDACTED] / 48	PLAT	129 (10E3/uL)	220	760
	Enrollment / [REDACTED] / -50	ALP	186 (U/L)	38	113
	Enrollment / [REDACTED] / -5	ALB	4.4 (g/dL)	4.1	5.1
	Enrollment / [REDACTED] / -5	ALT	14 (U/L)	7	23
	Enrollment / [REDACTED] / -5	AST	28 (U/L)	13	30
	Enrollment / [REDACTED] / -5	BILI	0.4 (mg/dL)	0.4	1.5
	Enrollment / [REDACTED] / -5	HCT	41.1 (%)	35.1	44.4
	Enrollment / [REDACTED] / -5	HGB	12.4 (g/dL)	11.6	14.8
	Enrollment / [REDACTED] / -5	PLAT	31.4 (10E4/uL)	15	34
	Enrollment / [REDACTED] / -5	PROT	7 (g/dL)	6.6	8.1
	Enrollment / [REDACTED] / -5	RBC	4.99 (10E6/L)	3.86	4.92
	Enrollment / [REDACTED] / -5	WBC	8.8 (10E3/uL)	3.3	8.6
	Enrollment / [REDACTED] / 8	ALT	36 (U/L)	7	23
	Enrollment / [REDACTED] / 8	AST	101 (U/L)	13	30
	Enrollment / [REDACTED] / 8	PLAT	6 (10E4/uL)	15	34
	Enrollment / [REDACTED] / 71	ALT	121 (U/L)	7	23
	Enrollment / [REDACTED] / 71	AST	130 (U/L)	13	30
	Enrollment / [REDACTED] / 151	ALB	4.7 (g/dL)	4.1	5.1
	Enrollment / [REDACTED] / 151	ALP	324 (U/L)	38	113

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/	Date/	Study Day	Test	Result (units)	Clinically Significant Limits	
						Very Low	Very High
	Enrollment		151	ALT	19 (U/L)	10	42
	Enrollment		151	AST	42 (U/L)	13	30
	Enrollment		151	BILI	0.2 (mg/dL)	0.4	1.5
	Enrollment		151	HCT	40.8 (%)	40.7	50.1
	Enrollment		151	HGB	13.8 (g/dL)	13.7	16.8
	Enrollment		151	PLAT	444 (10E3/uL)	158	348
	Enrollment		151	PROT	6.6 (g/dL)	6.6	8.1
	Enrollment		151	RBC	5 (10E6/uL)	4.35	5.55
	Enrollment		151	WBC	7 (10E3/uL)	3.3	8.6
	Enrollment		155	AST	79 (U/L)	13	30
	Enrollment		157	AST	100 (U/L)	13	30
	Enrollment		203	ALB	2.7 (g/dL)	4.1	5.1
	Enrollment		203	ALT	6128 (U/L)	10	42
	Enrollment		203	AST	24297 (U/L)	13	30
	Enrollment		203	HGB	11.8 (g/dL)	13.7	16.8
	Enrollment		203	PLAT	142 (10E3/uL)	158	348
	Enrollment		203	PROT	4.5 (g/dL)	6.6	8.1

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

						Test Normal Low	Test Normal High	Result >3 x ULN	
Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)				
RESTORE		Switch to OAV101		371	ALT	342 (U/L)	3	49	Yes
RESTORE		Switch to OAV101		394	ALT	90 (U/L)	3	49	
RESTORE		Switch to OAV101		400	ALT	185 (U/L)	3	49	Yes
RESTORE		Switch to OAV101		406	ALT	329 (U/L)	3	49	Yes
RESTORE		Switch to OAV101		414	ALT	227 (U/L)	3	49	Yes
RESTORE		Switch to OAV101		421	ALT	128 (U/L)	3	49	
RESTORE		Switch to OAV101		429	ALT	69 (U/L)	3	49	
RESTORE		Switch to OAV101		435	ALT	50 (U/L)	3	49	
RESTORE		Switch to OAV101		554	ALT	210 (U/L)	3	49	Yes
RESTORE		Switch to OAV101		568	ALT	71 (U/L)	3	49	
RESTORE		Switch to OAV101		595	ALT	182 (U/L)	3	49	Yes
RESTORE		Switch to OAV101		632	ALT	86 (U/L)	3	49	
RESTORE		Switch to OAV101		371	AST	529 (U/L)	9	37	Yes
RESTORE		Switch to OAV101		394	AST	97 (U/L)	9	37	
RESTORE		Switch to OAV101		400	AST	158 (U/L)	9	37	Yes
RESTORE		Switch to OAV101		406	AST	228 (U/L)	9	37	Yes
RESTORE		Switch to OAV101		414	AST	132 (U/L)	9	37	Yes
RESTORE		Switch to OAV101		421	AST	86 (U/L)	9	37	
RESTORE		Switch to OAV101		429	AST	66 (U/L)	9	37	
RESTORE		Switch to OAV101		435	AST	55 (U/L)	9	37	
RESTORE		Switch to OAV101		554	AST	171 (U/L)	9	37	Yes
RESTORE		Switch to OAV101		568	AST	71 (U/L)	9	37	
RESTORE		Switch to OAV101		595	AST	139 (U/L)	9	37	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		Switch to OAV101	632	AST	86 (U/L)	9	37	
RESTORE		OAV101 mono	/ 1	ALP	431 (U/L)	38	113	Yes
RESTORE		OAV101 mono	/ 7	AST	142 (U/L)	9	37	Yes
RESTORE		OAV101 mono	/ 9	AST	334 (U/L)	9	37	Yes
RESTORE		OAV101 mono	15	AST	71 (U/L)	9	37	
RESTORE		OAV101 mono	14	WBC	37.5 (10E3/uL)	3.5	9.1	Yes
RESTORE		OAV101 mono	28	WBC	21.3 (10E3/uL)	3.5	9.1	
RESTORE		Switch to OAV101	542	ALT	145 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	896	ALT	17 (U/L)	7	23	
RESTORE		Switch to OAV101	541	AST	164 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	542	AST	287 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	896	AST	35 (U/L)	13	30	
RESTORE		Switch to OAV101	105	ALT	2871 (U/L)	10	42	Yes
RESTORE		Switch to OAV101	149	ALT	64 (U/L)	10	42	
RESTORE		Switch to OAV101	163	ALT	235 (U/L)	10	42	Yes
RESTORE		Switch to OAV101	97	AST	123 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	105	AST	1856 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	149	AST	75 (U/L)	13	30	

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jp
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		Switch to OAV101	/ 163	AST	280 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	/ 158	ALT	787 (U/L)	12	50.5	Yes
RESTORE		Bridge to OAV101	/ 159	ALT	924 (U/L)	12	50.5	Yes
RESTORE		Bridge to OAV101	/ 158	AST	1527 (U/L)	24.5	66.5	Yes
RESTORE		Bridge to OAV101	/ 49	ALT	617 (U/L)	9.4	38.4	Yes
RESTORE		Bridge to OAV101	/ 19	AST	585 (U/L)	23	56.5	Yes
RESTORE		Switch to OAV101	/ 494	ALT	97 (U/L)	6	27	Yes
RESTORE		Switch to OAV101	/ 498	ALT	239 (U/L)	6	27	Yes
RESTORE		Switch to OAV101	/ 550	ALT	333 (U/L)	6	27	Yes
RESTORE		Switch to OAV101	/ 678	ALT	65 (U/L)	6	27	
RESTORE		Switch to OAV101	/ 706	ALT	66 (U/L)	6	27	
RESTORE		Switch to OAV101	/ 736	ALT	41 (U/L)	6	27	
RESTORE		Switch to OAV101	/ 739	ALT	35 (U/L)	6	27	
RESTORE		Switch to OAV101	/ 741	ALT	32 (U/L)	6	27	
RESTORE		Switch to OAV101	/ 776	ALT	33 (U/L)	6	27	
RESTORE		Switch to OAV101	/ 494	AST	270 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	/ 498	AST	423 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	/ 550	AST	181 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	/ 678	AST	62 (U/L)	13	30	

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jp
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		Switch to OAV101	706	AST	65 (U/L)	13	30	
RESTORE		Switch to OAV101	736	AST	36 (U/L)	13	30	
RESTORE		Switch to OAV101	739	AST	39 (U/L)	13	30	
RESTORE		Switch to OAV101	741	AST	36 (U/L)	13	30	
RESTORE		Switch to OAV101	776	AST	41 (U/L)	13	30	
RESTORE		Switch to OAV101	877	ALT	123 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	878	ALT	106 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	879	ALT	82 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	926	ALT	67 (U/L)	7	23	
RESTORE		Switch to OAV101	942	ALT	55 (U/L)	7	23	
RESTORE		Switch to OAV101	961	ALT	52 (U/L)	7	23	
RESTORE		Switch to OAV101	977	AST	218 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	978	AST	186 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	979	AST	139 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	926	AST	61 (U/L)	13	30	
RESTORE		Switch to OAV101	942	AST	53 (U/L)	13	30	
RESTORE		Switch to OAV101	961	AST	51 (U/L)	13	30	
RESTORE		OAV101 mono	976	ALT	473 (U/L)	10	42	Yes
RESTORE		OAV101 mono	977	ALT	314 (U/L)	10	42	Yes
RESTORE		OAV101 mono	978	ALT	232 (U/L)	10	42	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpj
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		OAV101 mono	11	ALT	121 (U/L)	10	42	
RESTORE		OAV101 mono	/ 6	AST	524 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 7	AST	286 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 8	AST	184 (U/L)	13	30	Yes
RESTORE		OAV101 mono	11	AST	71 (U/L)	13	30	
RESTORE		Switch to OAV101	242	ALT	78 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	279	ALT	768 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	242	AST	152 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	275	AST	514 (U/L)	13	30	Yes
RESTORE		OAV101 mono	22	ALT	175 (IU/L)	5	25	Yes
RESTORE		OAV101 mono	30	ALT	317 (IU/L)	5	25	Yes
RESTORE		OAV101 mono	44	ALT	459 (IU/L)	5	25	Yes
RESTORE		OAV101 mono	58	ALT	317 (IU/L)	5	25	Yes
RESTORE		OAV101 mono	72	ALT	118 (IU/L)	5	25	Yes
RESTORE		OAV101 mono	86	ALT	50 (IU/L)	5	25	
RESTORE		OAV101 mono	100	ALT	41 (IU/L)	5	25	
RESTORE		OAV101 mono	30	AST	214 (IU/L)	15	50	Yes
RESTORE		OAV101 mono	44	AST	294 (IU/L)	15	50	Yes
RESTORE		OAV101 mono	58	AST	175 (IU/L)	15	50	Yes
RESTORE		OAV101 mono	72	AST	86 (IU/L)	15	50	

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpj
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		OAV101 mono	/ 86	AST	55 (IU/L)	15	50	
RESTORE		OAV101 mono	100	AST	57 (IU/L)	15	50	
RESTORE		Bridge to OAV101	112	ALT	247 (IU/L)	5	65	Yes
RESTORE		Bridge to OAV101	113	ALT	180 (IU/L)	5	65	
RESTORE		Bridge to OAV101	112	AST	427 (IU/L)	15	80	Yes
RESTORE		Bridge to OAV101	113	AST	242 (IU/L)	15	80	Yes
RESTORE		OAV101 mono	/ 6	ALT	1759 (IU/L)	9	38	Yes
RESTORE		OAV101 mono	/ 7	ALT	1218 (IU/L)	9	38	Yes
RESTORE		OAV101 mono	/ 43	ALT	107 (IU/L)	9	38	
RESTORE		OAV101 mono	/ 57	ALT	256 (IU/L)	9	38	Yes
RESTORE		OAV101 mono	/ 6	AST	2959 (IU/L)	23	57	Yes
RESTORE		OAV101 mono	/ 7	AST	1336 (IU/L)	23	57	Yes
RESTORE		OAV101 mono	/ 43	AST	112 (IU/L)	23	57	
RESTORE		OAV101 mono	/ 57	AST	207 (IU/L)	23	57	Yes
RESTORE		Bridge to OAV101	/ 28	ALT	952 (IU/L)	0	40	Yes
RESTORE		Bridge to OAV101	/ 29	ALT	960 (IU/L)	0	40	Yes
RESTORE		Bridge to OAV101	189	ALT	55 (IU/L)	0	40	
RESTORE		Bridge to OAV101	/ 28	AST	1676 (IU/L)	0	37	Yes
RESTORE		Bridge to OAV101	161	AST	43 (IU/L)	0	37	

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		Bridge to OAV101	17	AST	52 (IU/L)	0	37	
RESTORE		OAV101 mono	7	ALT	123 (IU/L)	0	40	Yes
RESTORE		OAV101 mono	19	ALT	64 (IU/L)	0	40	
RESTORE		OAV101 mono	28	ALT	78 (IU/L)	0	40	
RESTORE		OAV101 mono	7	AST	190 (IU/L)	0	37	Yes
RESTORE		OAV101 mono	19	AST	75 (IU/L)	0	37	
RESTORE		Switch to OAV101	72	ALT	206 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	73	ALT	273 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	72	AST	455 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	73	AST	565 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	30	ALT	220 (U/L)	10	42	Yes
RESTORE		Switch to OAV101	79	AST	181 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	02	AST	61 (U/L)	13	30	
RESTORE		Switch to OAV101	30	AST	190 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	80	AST	65 (IU/L)	13	30	
RESTORE		OAV101 mono	6	ALT	156 (U/L)	10	38	Yes
RESTORE		OAV101 mono	6	AST	331 (U/L)	13	57	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

						Test Normal Low	Test Normal High	Result >3 x ULN
Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)			
RESTORE		Combo w/OAV101	230	ALT	229 (IU/L)	9	38	Yes
RESTORE		Combo w/OAV101	238	ALT	45 (IU/L)	9	38	
RESTORE		Combo w/OAV101	396	ALT	31 (IU/L)	9	38	
RESTORE		Combo w/OAV101	424	ALT	26 (IU/L)	9	38	
RESTORE		Combo w/OAV101	1505	ALT	81 (IU/L)	9	38	
RESTORE		Combo w/OAV101	230	AST	444 (IU/L)	24	57	Yes
RESTORE		Combo w/OAV101	238	AST	53 (IU/L)	24	57	
RESTORE		Combo w/OAV101	396	AST	44 (IU/L)	24	57	
RESTORE		Combo w/OAV101	424	AST	41 (IU/L)	24	57	
RESTORE		Combo w/OAV101	1505	AST	46 (IU/L)	24	57	
RESTORE			Combo w/OAV101	/ 19	ALT	414 (IU/L)	7	23
RESTORE	Combo w/OAV101		/ 21	ALT	284 (IU/L)	7	23	Yes
RESTORE	Combo w/OAV101		/ 26	ALT	65 (IU/L)	7	23	
RESTORE	Combo w/OAV101		624	ALT	70 (IU/L)	7	23	Yes
RESTORE	Combo w/OAV101		/ 19	AST	743 (IU/L)	13	30	Yes
RESTORE	Combo w/OAV101		/ 21	AST	279 (IU/L)	13	30	Yes
RESTORE	Combo w/OAV101		/ 26	AST	46 (IU/L)	13	30	
RESTORE	Combo w/OAV101		624	AST	50 (IU/L)	13	30	
RESTORE		Bridge to OAV101	/ 9	BILI	13.21 (mg/dL)	0.4	1.5	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN	
RESTORE		Switch to OAV101	546	ALT	117 (IU/L)	5	31	Yes	
RESTORE		Switch to OAV101	499	RBC	437 (10E6/uL)	15	35	Yes	
RESTORE		OAV101 mono	/ 6	ALT	513 (U/L)	10	30	Yes	
RESTORE		OAV101 mono	/ 3	AST	111 (U/L)	13	30	Yes	
RESTORE		OAV101 mono	/ 6	AST	1085 (U/L)	13	30	Yes	
RESTORE		Bridge to OAV101	254	ALT	274 (U/L)	10	30	Yes	
RESTORE		Bridge to OAV101	268	ALT	379 (U/L)	10	30	Yes	
RESTORE		Bridge to OAV101	282	ALT	165 (U/L)	10	30	Yes	
RESTORE		Bridge to OAV101	268	AST	354 (U/L)	13	30	Yes	
RESTORE		Bridge to OAV101	282	AST	142 (U/L)	13	30	Yes	
RESTORE		Switch to OAV101	224	ALT	132 (U/L)	10	30	Yes	
RESTORE		Switch to OAV101	229	ALT	132 (U/L)	10	30	Yes	
RESTORE		Switch to OAV101	256	ALT	886 (U/L)	10	30	Yes	
RESTORE		Switch to OAV101	224	AST	232 (U/L)	13	30	Yes	
RESTORE		Switch to OAV101	229	AST	248 (U/L)	13	30	Yes	
RESTORE		Switch to OAV101	256	AST	1401 (U/L)	13	30	Yes	

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpj
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		Transient add-on	/ 4	AST	111 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	119	ALT	149 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101	119	AST	243 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	141	AST	97 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	144	AST	123 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	231	ALT	81 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101	268	ALT	32 (U/L)	7	23	
RESTORE		Bridge to OAV101	275	ALT	53 (U/L)	7	23	
RESTORE		Bridge to OAV101	231	AST	134 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	268	AST	51 (U/L)	13	30	
RESTORE		Bridge to OAV101	114	ALT	243 (IU/L)	4	43	Yes
RESTORE		Bridge to OAV101	114	AST	239 (IU/L)	8	38	Yes
RESTORE		Bridge to OAV101	56	ALT	119 (U/L)	4	43	
RESTORE		Bridge to OAV101	56	ALT	266 (U/L)	4	43	Yes
RESTORE		Bridge to OAV101	59	ALT	259 (U/L)	4	43	Yes
RESTORE		Bridge to OAV101	64	ALT	70 (U/L)	4	43	
RESTORE		Bridge to OAV101	91	ALT	49 (U/L)	4	43	
RESTORE		Bridge to OAV101	56	AST	298 (U/L)	8	38	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpj
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		Bridge to OAV101	/ 56	AST	700 (U/L)	8	38	Yes
RESTORE		Bridge to OAV101	/ 59	AST	397 (U/L)	8	38	Yes
RESTORE		Bridge to OAV101	/ 64	AST	61 (U/L)	8	38	
RESTORE		Bridge to OAV101	/ 77	AST	50 (U/L)	8	38	
RESTORE		Bridge to OAV101	/ 91	AST	93 (U/L)	8	38	
RESTORE		Combo w/OAV101	422	ALT	205 (U/L)	4	44	Yes
RESTORE		OAV101 mono	/ 1	ALP	389 (U/L)	38	113	Yes
RESTORE		OAV101 mono	/ 5	AST	111 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 6	AST	124 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 8	AST	71 (U/L)	13	30	
RESTORE		OAV101 mono	/ 12	AST	51 (U/L)	13	30	
RESTORE		OAV101 mono	/ 61	AST	44 (U/L)	13	30	
RESTORE		OAV101 mono	/ 75	AST	63 (U/L)	13	30	
RESTORE		OAV101 mono	/ 89	AST	57 (U/L)	13	30	
RESTORE		OAV101 mono	103	AST	65 (U/L)	13	30	
RESTORE		OAV101 mono	138	AST	64 (U/L)	13	30	
RESTORE		OAV101 mono	166	AST	72 (U/L)	13	30	
RESTORE		OAV101 mono	196	AST	51 (U/L)	13	30	
RESTORE		OAV101 mono	252	AST	54 (U/L)	13	30	
RESTORE		OAV101 mono	326	AST	53 (U/L)	13	30	

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpj
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		OAV101 mono	390	AST	53 (U/L)	13	30	
RESTORE		OAV101 mono	446	AST	48 (U/L)	13	30	
RESTORE		OAV101 mono	509	AST	47 (U/L)	13	30	
RESTORE		OAV101 mono	580	AST	49 (U/L)	13	30	
RESTORE		OAV101 mono	642	AST	47 (U/L)	13	30	
RESTORE		OAV101 mono	691	AST	54 (U/L)	13	30	
RESTORE		Bridge to OAV101	187	ALT	130 (IU/L)	10	30	Yes
RESTORE		Bridge to OAV101	189	ALT	179 (IU/L)	10	30	Yes
RESTORE		Bridge to OAV101	187	AST	279 (IU/L)	13	30	Yes
RESTORE		Bridge to OAV101	189	AST	295 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	5	ALT	491 (U/L)	7	23	Yes
RESTORE		OAV101 mono	6	ALT	1067 (U/L)	7	23	Yes
RESTORE		OAV101 mono	27	ALT	115 (U/L)	7	23	Yes
RESTORE		OAV101 mono	34	ALT	480 (U/L)	7	23	Yes
RESTORE		OAV101 mono	5	AST	781 (U/L)	13	30	Yes
RESTORE		OAV101 mono	6	AST	1678 (U/L)	13	30	Yes
RESTORE		OAV101 mono	27	AST	124 (U/L)	13	30	Yes
RESTORE		OAV101 mono	34	AST	257 (U/L)	13	30	Yes
RESTORE		OAV101 mono	20	ALT	454 (IU/L)	9	53	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpj
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		OAV101 mono	7	ALT	314 (IU/L)	7	53	Yes
RESTORE		OAV101 mono	20	ALT	550 (IU/L)	7	53	Yes
RESTORE		OAV101 mono	32	ALT	68 (U/L)	7	53	
RESTORE		OAV101 mono	7	AST	326 (IU/L)	17	70	Yes
RESTORE		OAV101 mono	20	AST	298 (IU/L)	17	70	Yes
RESTORE		OAV101 mono	32	AST	70 (U/L)	17	70	
RESTORE		OAV101 mono	14	ALT	94 (U/L)	7	23	Yes
RESTORE		OAV101 mono	20	ALT	365 (U/L)	7	23	Yes
RESTORE		OAV101 mono	26	ALT	464 (U/L)	7	23	Yes
RESTORE		OAV101 mono	32	ALT	342 (U/L)	7	23	Yes
RESTORE		OAV101 mono	39	ALT	167 (U/L)	7	23	Yes
RESTORE		OAV101 mono	53	ALT	98 (U/L)	7	23	Yes
RESTORE		OAV101 mono	69	ALT	45 (U/L)	7	23	
RESTORE		OAV101 mono	83	ALT	29 (U/L)	7	23	
RESTORE		OAV101 mono	02	ALT	32 (U/L)	7	23	
RESTORE		OAV101 mono	23	ALT	49 (U/L)	7	23	
RESTORE		OAV101 mono	51	ALT	73 (U/L)	7	23	Yes
RESTORE		OAV101 mono	93	ALT	66 (U/L)	7	23	
RESTORE		OAV101 mono	4	AST	100 (U/L)	13	30	Yes
RESTORE		OAV101 mono	6	AST	157 (U/L)	13	30	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		OAV101 mono	/ 9	AST	91 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 14	AST	152 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 20	AST	382 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 26	AST	398 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 32	AST	239 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 39	AST	107 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 53	AST	105 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 69	AST	59 (U/L)	13	30	
RESTORE		OAV101 mono	/ 83	AST	60 (U/L)	13	30	
RESTORE		OAV101 mono	102	AST	63 (U/L)	13	30	
RESTORE		OAV101 mono	123	AST	95 (U/L)	13	30	Yes
RESTORE		OAV101 mono	151	AST	101 (U/L)	13	30	Yes
RESTORE		OAV101 mono	193	AST	75 (U/L)	13	30	
RESTORE		Bridge to OAV101	/ 25	ALT	351 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101	/ 29	ALT	110 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101	/ 32	ALT	63 (U/L)	7	23	
RESTORE		Bridge to OAV101	/ 58	ALT	51 (U/L)	7	23	
RESTORE		Bridge to OAV101	/ 78	ALT	60 (U/L)	7	23	
RESTORE		Bridge to OAV101	/ 99	ALT	60 (U/L)	7	23	
RESTORE		Bridge to OAV101	134	ALT	100 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101	162	ALT	73 (U/L)	7	23	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpj
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		Bridge to OAV101		195	ALT	56 (U/L)	7	23
RESTORE		Bridge to OAV101		/ 25	AST	282 (U/L)	13	30
RESTORE		Bridge to OAV101		/ 29	AST	153 (U/L)	13	30
RESTORE		Bridge to OAV101		/ 58	AST	67 (U/L)	13	30
RESTORE		Bridge to OAV101		/ 78	AST	76 (U/L)	13	30
RESTORE		Bridge to OAV101		/ 99	AST	75 (U/L)	13	30
RESTORE		Bridge to OAV101		134	AST	133 (U/L)	13	30
RESTORE		Bridge to OAV101		162	AST	90 (U/L)	13	30
RESTORE		Bridge to OAV101		195	AST	71 (U/L)	13	30
RESTORE		Combo w/OAV101		/ 77	AST	113 (U/L)	13	30
RESTORE		Combo w/OAV101		/ 79	AST	83 (U/L)	13	30
RESTORE		Combo w/OAV101		100	AST	53 (U/L)	13	30
RESTORE		Combo w/OAV101		107	AST	77 (U/L)	13	30
RESTORE		Combo w/OAV101		114	AST	71 (U/L)	13	30
RESTORE		Combo w/OAV101		121	AST	71 (U/L)	13	30
RESTORE		Combo w/OAV101		163	AST	51 (U/L)	13	30
RESTORE		Combo w/OAV101		184	AST	50 (U/L)	13	30
RESTORE		Switch to OAV101		591	ALT	157 (U/L)	10	42
RESTORE		Switch to OAV101		589	AST	133 (U/L)	13	30
RESTORE		Switch to OAV101		591	AST	309 (U/L)	13	30

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpj
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		Bridge to OAV101	/ 13	AST	136 (IU/L)	13	30	Yes
RESTORE		Bridge to OAV101	/ 15	AST	101 (IU/L)	13	30	Yes
RESTORE		Bridge to OAV101	/ 18	AST	81 (IU/L)	13	30	
RESTORE		Bridge to OAV101	/ 55	AST	119 (IU/L)	13	30	Yes
RESTORE		Bridge to OAV101	/ 76	AST	88 (IU/L)	13	30	
RESTORE		Combo w/OAV101	/ 3	BILI	8.4 (mg/dL)	0.11	0.66	Yes
RESTORE		OAV101 mono	/ 26	ALT	433 (U/L)	9	38	Yes
RESTORE		OAV101 mono	/ 27	ALT	463 (U/L)	9	38	Yes
RESTORE		OAV101 mono	/ 34	ALT	342 (U/L)	9	38	Yes
RESTORE		OAV101 mono	/ 41	ALT	385 (U/L)	9	38	Yes
RESTORE		OAV101 mono	/ 43	ALT	489 (U/L)	9	38	Yes
RESTORE		OAV101 mono	/ 58	ALT	174 (U/L)	9	38	Yes
RESTORE		OAV101 mono	/ 84	ALT	48 (U/L)	9	38	
RESTORE		OAV101 mono	/ 26	AST	668 (U/L)	24	57	Yes
RESTORE		OAV101 mono	/ 27	AST	617 (U/L)	24	57	Yes
RESTORE		OAV101 mono	/ 34	AST	253 (U/L)	24	57	Yes
RESTORE		OAV101 mono	/ 41	AST	348 (U/L)	24	57	Yes
RESTORE		OAV101 mono	/ 43	AST	415 (U/L)	24	57	Yes
RESTORE		OAV101 mono	/ 58	AST	132 (U/L)	24	57	

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jp
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		OAV101 mono	/ 84	AST	76 (U/L)	24	57	
RESTORE		OAV101 mono	/ 112	AST	67 (U/L)	24	57	
RESTORE		Bridge to OAV101	/ 21	AST	101 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 7	ALT	134 (IU/L)	7	23	Yes
RESTORE		OAV101 mono	/ 36	ALT	1016 (IU/L)	7	23	Yes
RESTORE		OAV101 mono	/ 120	ALT	19 (IU/L)	7	23	
RESTORE		OAV101 mono	/ 7	AST	176 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	/ 36	AST	584 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	/ 120	AST	28 (IU/L)	13	30	
RESTORE		Switch to OAV101	/ 275	AST	112 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	/ 282	AST	57 (U/L)	13	30	
RESTORE		Switch to OAV101	/ 289	AST	41 (U/L)	13	30	
RESTORE		Switch to OAV101	/ 362	ALT	80 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	/ 413	ALT	247 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	/ 415	ALT	287 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	/ 687	ALT	159 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	/ 413	AST	211 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	/ 415	AST	228 (U/L)	13	30	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpj
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

						Test Normal Low	Test Normal High	Result >3 x ULN	
Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)				
RESTORE		Switch to OAV101		87	AST	138 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101		40	ALT	458 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101		40	AST	632 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101		29	ALT	1057 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101		88	ALT	65 (IU/L)	7	23	
RESTORE		Bridge to OAV101		29	AST	1377 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101		88	AST	67 (IU/L)	13	30	
RESTORE		Bridge to OAV101		16	ALT	77 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101		17	ALT	103 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101		68	ALT	88 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101		82	ALT	94 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101		16	AST	184 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101		17	AST	209 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101		68	AST	82 (U/L)	13	30	
RESTORE		Bridge to OAV101		82	AST	109 (U/L)	13	30	Yes
RESTORE		OAV101 mono		6	AST	107 (U/L)	13	30	Yes
RESTORE		OAV101 mono		8	AST	57 (U/L)	13	30	

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpj
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		Bridge to OAV101	46	ALT	158 (U/L)	8	42	Yes
RESTORE		Bridge to OAV101	46	AST	140 (U/L)	13	33	Yes
RESTORE		Bridge to OAV101	224	AST	69 (U/L)	13	33	
RESTORE		Bridge to OAV101	410	AST	37 (U/L)	13	33	
RESTORE		Bridge to OAV101	39	ALT	403 (IU/L)	4	44	Yes
RESTORE		Bridge to OAV101	43	ALT	625 (IU/L)	4	44	Yes
RESTORE		Bridge to OAV101	46	ALT	504 (IU/L)	4	44	Yes
RESTORE		Bridge to OAV101	49	ALT	258 (IU/L)	4	44	Yes
RESTORE		Bridge to OAV101	57	ALT	161 (IU/L)	4	44	Yes
RESTORE		Bridge to OAV101	71	ALT	1710 (IU/L)	4	44	Yes
RESTORE		Bridge to OAV101	78	ALT	1206 (IU/L)	4	44	Yes
RESTORE		Bridge to OAV101	87	ALT	379 (IU/L)	4	44	Yes
RESTORE		Bridge to OAV101	99	ALT	71 (IU/L)	4	44	
RESTORE		Bridge to OAV101	113	ALT	23 (IU/L)	4	44	
RESTORE		Bridge to OAV101	35	AST	126 (IU/L)	8	38	Yes
RESTORE		Bridge to OAV101	39	AST	633 (IU/L)	8	38	Yes
RESTORE		Bridge to OAV101	43	AST	505 (IU/L)	8	38	Yes
RESTORE		Bridge to OAV101	46	AST	251 (IU/L)	8	38	Yes
RESTORE		Bridge to OAV101	49	AST	95 (IU/L)	8	38	
RESTORE		Bridge to OAV101	57	AST	151 (IU/L)	8	38	Yes
RESTORE		Bridge to OAV101	71	AST	2160 (IU/L)	8	38	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jp
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

						Test Normal Low	Test Normal High	Result >3 x ULN	
Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)				
RESTORE		Bridge to OAV101		78	AST	1112 (IU/L)	8	38	Yes
RESTORE		Bridge to OAV101		87	AST	272 (IU/L)	8	38	Yes
RESTORE		Bridge to OAV101		99	AST	60 (IU/L)	8	38	
RESTORE		Bridge to OAV101		113	AST	35 (IU/L)	8	38	
RESTORE		OAV101 mono		/ 6	ALT	140 (IU/L)	7	23	Yes
RESTORE		OAV101 mono		/ 6	AST	230 (IU/L)	13	30	Yes
RESTORE		Combo w/OAV101		348	AST	117 (IU/L)	13	30	Yes
RESTORE		Combo w/OAV101		363	AST	32 (IU/L)	13	30	
RESTORE		Bridge to OAV101		15	AST	93 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101		21	AST	718 (U/L)	13	30	Yes
RESTORE		Add-on		/ 7	AST	152 (IU/L)	13	30	Yes
RESTORE		Combo w/OAV101		113	ALT	232 (IU/L)	8	42	Yes
RESTORE		Combo w/OAV101		116	ALT	570 (IU/L)	8	42	Yes
RESTORE		Combo w/OAV101		118	ALT	455 (IU/L)	8	42	Yes
RESTORE		Combo w/OAV101		120	ALT	266 (IU/L)	8	42	Yes
RESTORE		Combo w/OAV101		124	ALT	99 (IU/L)	8	42	
RESTORE		Combo w/OAV101		145	ALT	117 (IU/L)	8	42	

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		Combo w/OAV101		159 ALT	130 (IU/L)	8	42	Yes
RESTORE		Combo w/OAV101		173 ALT	54 (IU/L)	8	42	
RESTORE		Combo w/OAV101		189 ALT	43 (IU/L)	8	42	
RESTORE		Combo w/OAV101		208 ALT	57 (IU/L)	8	42	
RESTORE		Combo w/OAV101		224 ALT	104 (IU/L)	8	42	
RESTORE		Combo w/OAV101		238 ALT	119 (IU/L)	8	42	
RESTORE		Combo w/OAV101		252 ALT	94 (IU/L)	8	42	
RESTORE		Combo w/OAV101		280 ALT	54 (IU/L)	8	42	
RESTORE		Combo w/OAV101		308 ALT	51 (IU/L)	8	42	
RESTORE		Combo w/OAV101		336 ALT	48 (IU/L)	8	42	
RESTORE		Combo w/OAV101		364 ALT	49 (IU/L)	8	42	
RESTORE		Combo w/OAV101		106 AST	185 (IU/L)	13	33	Yes
RESTORE		Combo w/OAV101		113 AST	316 (IU/L)	13	33	Yes
RESTORE		Combo w/OAV101		116 AST	545 (IU/L)	13	33	Yes
RESTORE		Combo w/OAV101		118 AST	238 (IU/L)	13	33	Yes
RESTORE		Combo w/OAV101		120 AST	96 (IU/L)	13	33	
RESTORE		Combo w/OAV101		124 AST	40 (IU/L)	13	33	
RESTORE		Combo w/OAV101		131 AST	38 (IU/L)	13	33	
RESTORE		Combo w/OAV101		145 AST	122 (IU/L)	13	33	Yes
RESTORE		Combo w/OAV101		159 AST	72 (IU/L)	13	33	
RESTORE		Combo w/OAV101		173 AST	46 (IU/L)	13	33	
RESTORE		Combo w/OAV101		189 AST	46 (IU/L)	13	33	
RESTORE		Combo w/OAV101		208 AST	66 (IU/L)	13	33	

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		Combo w/OAV101	224	AST	113 (IU/L)	13	33	Yes
RESTORE		Combo w/OAV101	238	AST	100 (IU/L)	13	33	Yes
RESTORE		Combo w/OAV101	252	AST	79 (IU/L)	13	33	
RESTORE		Combo w/OAV101	280	AST	49 (IU/L)	13	33	
RESTORE		Combo w/OAV101	308	AST	57 (IU/L)	13	33	
RESTORE		Combo w/OAV101	336	AST	51 (IU/L)	13	33	
RESTORE		Combo w/OAV101	364	AST	50 (IU/L)	13	33	
RESTORE		OAV101 mono	/ 6	ALT	521 (IU/L)	10	42	Yes
RESTORE		OAV101 mono	/ 9	ALT	261 (IU/L)	10	42	Yes
RESTORE		OAV101 mono	13	ALT	106 (IU/L)	10	42	
RESTORE		OAV101 mono	15	ALT	68 (IU/L)	10	42	
RESTORE		OAV101 mono	44	ALT	386 (IU/L)	10	42	Yes
RESTORE		OAV101 mono	51	ALT	727 (IU/L)	10	42	Yes
RESTORE		OAV101 mono	53	ALT	708 (IU/L)	10	42	Yes
RESTORE		OAV101 mono	57	ALT	600 (IU/L)	10	42	Yes
RESTORE		OAV101 mono	63	ALT	376 (IU/L)	10	42	Yes
RESTORE		OAV101 mono	79	ALT	134 (IU/L)	10	42	Yes
RESTORE		OAV101 mono	93	ALT	89 (IU/L)	10	42	
RESTORE		OAV101 mono	121	ALT	56 (IU/L)	10	42	
RESTORE		OAV101 mono	132	ALT	50 (IU/L)	10	42	
RESTORE		OAV101 mono	/ 6	AST	890 (IU/L)	13	30	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		OAV101 mono	/ 9	AST	351 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	/ 13	AST	86 (IU/L)	13	30	
RESTORE		OAV101 mono	/ 15	AST	60 (IU/L)	13	30	
RESTORE		OAV101 mono	/ 20	AST	46 (IU/L)	13	30	
RESTORE		OAV101 mono	/ 30	AST	52 (IU/L)	13	30	
RESTORE		OAV101 mono	/ 44	AST	389 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	/ 51	AST	681 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	/ 53	AST	558 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	/ 57	AST	478 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	/ 63	AST	290 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	/ 79	AST	126 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	/ 93	AST	108 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	121	AST	70 (IU/L)	13	30	
RESTORE		OAV101 mono	132	AST	74 (IU/L)	13	30	
RESTORE		Bridge to OAV101	/ 25	AST	248 (IU/L)	13	30	Yes
RESTORE		Bridge to OAV101	/ 20	BILD	1.5 (mg/dL)	0	0.3	Yes
RESTORE		Bridge to OAV101	/ 20	BILI	7.7 (mg/dL)	0.1	1.2	Yes
RESTORE		OAV101 mono	/ 48	ALT	1153 (IU/L)	12	51	Yes
RESTORE		OAV101 mono	/ 48	AST	969 (IU/L)	25	67	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jp
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE		Add-on	/ 22	AST	151 (IU/L)	10	40	Yes
RESTORE		Add-on	/ 79	AST	55 (IU/L)	10	40	
RESTORE		Bridge to OAV101	/ 28	ALT	224 (U/L)	4	44	Yes
RESTORE		Bridge to OAV101	/ 74	ALT	54 (U/L)	4	44	
RESTORE		Bridge to OAV101	/ 77	ALT	56 (U/L)	4	44	
RESTORE		Bridge to OAV101	/ 88	ALT	49 (U/L)	4	44	
RESTORE		Bridge to OAV101	/ 102	ALT	66 (U/L)	4	44	
RESTORE		Bridge to OAV101	/ 133	ALT	37 (U/L)	4	44	
RESTORE		Bridge to OAV101	/ 298	ALT	26 (U/L)	4	44	
RESTORE		Bridge to OAV101	/ 329	ALT	24 (U/L)	4	44	
RESTORE		Bridge to OAV101	/ 453	ALT	39 (U/L)	4	44	
RESTORE		Bridge to OAV101	/ 28	AST	465 (U/L)	8	38	Yes
RESTORE		Bridge to OAV101	/ 70	AST	51 (U/L)	8	38	
RESTORE		Bridge to OAV101	/ 74	AST	90 (U/L)	8	38	
RESTORE		Bridge to OAV101	/ 77	AST	99 (U/L)	8	38	
RESTORE		Bridge to OAV101	/ 84	AST	71 (U/L)	8	38	
RESTORE		Bridge to OAV101	/ 88	AST	87 (U/L)	8	38	
RESTORE		Bridge to OAV101	/ 102	AST	88 (U/L)	8	38	
RESTORE		Bridge to OAV101	/ 133	AST	64 (U/L)	8	38	
RESTORE		Bridge to OAV101	/ 298	AST	50 (U/L)	8	38	
RESTORE		Bridge to OAV101	/ 327	AST	45 (U/L)	8	38	

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

						Test Normal	Test Normal	Result	
Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Low	High	>3 x ULN	
RESTORE		Bridge to OAV101		329	AST	63 (U/L)	8	38	
RESTORE		Bridge to OAV101		332	AST	56 (U/L)	8	38	
RESTORE		Bridge to OAV101		453	AST	58 (U/L)	8	38	
RESTORE		Bridge to OAV101		44	ALT	240 (IU/L)	7	23	Yes
RESTORE		Bridge to OAV101		58	ALT	335 (IU/L)	7	23	Yes
RESTORE		Bridge to OAV101		44	AST	229 (IU/L)	13	30	Yes
RESTORE		Combo w/OAV101		48	ALT	385 (U/L)	13	56	Yes
RESTORE		Combo w/OAV101		48	AST	661 (U/L)	22	66	Yes
RESTORE		OAV101 mono		71	ALT	121 (U/L)	7	23	Yes
RESTORE		OAV101 mono		78	AST	101 (U/L)	13	30	Yes
RESTORE		OAV101 mono		71	AST	130 (U/L)	13	30	Yes
RESTORE		Switch to OAV101		203	ALT	6128 (U/L)	10	42	Yes
RESTORE		Switch to OAV101		157	AST	100 (U/L)	13	30	Yes
RESTORE		Switch to OAV101		203	AST	24297 (U/L)	13	30	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.jp
Listing of Lack of Drug Effect
Japan OAV101 Treated Patients that Switched Treatment due to Lack of Drug Effect

Patient	Therapy Group	Primary Reason for Switching/Adding New Therapy	Lack of Drug Effect Specify
	Add-on	Perceived lack of drug effect	Motor function
	Combo w/OAV101	Perceived lack of drug effect	Swallowing or feeding ability for age
	Combo w/OAV101	Perceived lack of drug effect	Swallowing or feeding ability for age
	Combo w/OAV101	Perceived lack of drug effect	Motor function
	Combo w/OAV101	Perceived lack of drug effect	Motor function
	Combo w/OAV101	Perceived lack of drug effect	Respiratory function
	Switch to OAV101	Perceived lack of drug effect	Swallowing or feeding ability for age
	Transient add-on	Perceived lack of drug effect	

Listing 16.10.jpn
Listing of Lost to Follow-Up Patients
Japan OAV101 Treated Patients

Source	Patient	Age at time of consent /assent (months)	Therapy Group	SMN2 Gene Copy Number	SMA Type	Symptom Status	Patient Disposition	Primary Reason for Registry Discontinuation	Days/Age(months) at Last Data Point Captured
RESTORE		12	OAV101 mono	3 Copies	I	Symptomatic	Discontinued	Lost to Follow-up	89 / 14.9
RESTORE		30	Switch to OAV101	2 Copies	I	Symptomatic	Discontinued	Lost to Follow-up	1558 / 52.2
RESTORE		17	Switch to OAV101	2 Copies	I	Symptomatic	Discontinued	Lost to Follow-up	913 / 31.0
RESTORE		21	Switch to OAV101	2 Copies	I	Symptomatic	Discontinued	Lost to Follow-up	1446 / 52.5

Listing 16.11.jpn
Listing of Patients Excluded from the Japan Safety Analysis Set
All Japan Enrolled Patients

Patient	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
No patients qualify for this listing.					

Listing 16.13.jpn
Listing of Japan OAV101 Treated Patients
Japan OAV101 Treated Patients

Patient	HTA	Race	Age at Diagnosis (months)	SMA Type	Symptomatic at Diagnosis	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
	Yes	Japanese	2	1	Yes	Add-on				
	Yes	Japanese	5	1	Yes	Add-on				
	Yes	Japanese	3	1	Yes	Bridge to OAV101				
	Yes	Japanese	2	1	Yes	Bridge to OAV101				
	Yes	Japanese	14	1	Yes	Bridge to OAV101				
	Yes	Japanese	8	1	Yes	Bridge to OAV101				
	No	Japanese	0	5		Bridge to OAV101				
	Yes	Japanese	3	1	Yes	Bridge to OAV101				
	No	Taiwanese	9	1	Yes	Bridge to OAV101				

Listing 16.13.jpn
Listing of Japan OAV101 Treated Patients
Japan OAV101 Treated Patients

Patient	HTA	Race	Age at Diagnosis (months)	SMA Type	Symptomatic at Diagnosis	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
	No	Japanese	0	5		Bridge to OAV101				
	No	Japanese	14	2	Yes	Bridge to OAV101				
	Yes	Japanese	3	1	Yes	Bridge to OAV101				
	Yes	Japanese	1	1	Yes	Bridge to OAV101				
	No	Japanese	1	5		Bridge to OAV101				
	No	Japanese	22	2	Yes	Bridge to OAV101				
	No	Japanese	3	5		Bridge to OAV101				
	Yes	Japanese	7	1	Yes	Bridge to OAV101				
	Yes	Japanese	1	1	Yes	Bridge to OAV101				

Listing 16.13.jpn
Listing of Japan OAV101 Treated Patients
Japan OAV101 Treated Patients

Patient	HTA	Race	Age at Diagnosis (months)	SMA Type	Symptomatic at Diagnosis	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
	Yes	Japanese	2	1	Yes	Bridge to OAV101				
	Yes	Japanese	1	1	Yes	Bridge to OAV101				
	No	Japanese	6	2	Yes	Bridge to OAV101				
	Yes	Japanese	2	1	Yes	Bridge to OAV101				
	Yes	Japanese	6	1	Yes	Bridge to OAV101				
	No	Japanese	13	2	Yes	Bridge to OAV101				
	Yes	Japanese	6	1	Yes	Bridge to OAV101				
	Yes	Japanese	13	1	Yes	Bridge to OAV101				

Listing 16.13.jpn
Listing of Japan OAV101 Treated Patients
Japan OAV101 Treated Patients

Patient	HTA	Race	Age at Diagnosis (months)	SMA Type	Symptomatic at Diagnosis	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
	No	Japanese	21	2	Yes	Bridge to OAV101				
	No	Japanese	0	5		Bridge to OAV101				
	No	Japanese	9	2	Yes	Bridge to OAV101				
	No	Japanese	18	2	Yes	Bridge to OAV101				
	Yes	Japanese	5	1	Yes	Combo w/OAV101				
	Yes	Japanese	2	1	Yes	Combo w/OAV101				
	Yes	Japanese	1	1	Yes	Combo w/OAV101				
	Yes	Japanese	8	1	Yes	Combo w/OAV101				
	Yes	Japanese	0	1	Yes	Combo w/OAV101				

Listing 16.13.jpn
Listing of Japan OAV101 Treated Patients
Japan OAV101 Treated Patients

Patient	HTA	Race	Age at Diagnosis (months)	SMA Type	Symptomatic at Diagnosis	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
	Yes	Japanese	2	1	Yes	Combo w/OAV101				
	Yes	Japanese	4	1	Yes	Combo w/OAV101				
	Yes	Japanese	7	1	Yes	Combo w/OAV101				
	Yes	Japanese	2	1	Yes	Combo w/OAV101				
	Yes	Japanese	3	1	Yes	Combo w/OAV101				
	No	Japanese	14	2	Yes	OAV101 mono				
	No	Japanese	15	2	Yes	OAV101 mono				
	Yes	Japanese	16	1	Yes	OAV101 mono				
	Yes	Japanese	12	1	Yes	OAV101 mono				
	No	Vietnamese	1	5		OAV101 mono				

Listing 16.13.jpn
Listing of Japan OAV101 Treated Patients
Japan OAV101 Treated Patients

Patient	HTA	Race	Age at Diagnosis (months)	SMA Type	Symptomatic at Diagnosis	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
	No	Japanese	1	5		OAV101 mono				
	No	Japanese	1	5		OAV101 mono				
	Yes	Japanese	7	1	Yes	OAV101 mono				
	No	Indonesian	12	1	Yes	OAV101 mono				
	Yes	Japanese	14	1	Yes	OAV101 mono				
	No	Japanese	0	5		OAV101 mono				
	No	Japanese	17	2	Yes	OAV101 mono				
	No	Japanese	9	2	Yes	OAV101 mono				
	Yes	Japanese	7	1	Yes	OAV101 mono				
	Yes	Japanese	12	1	Yes	OAV101 mono				
	No	Japanese	16	2	Yes	OAV101 mono				
	Yes	Japanese	10	1	Yes	OAV101 mono				

Listing 16.13.jpn
Listing of Japan OAV101 Treated Patients
Japan OAV101 Treated Patients

Patient	HTA	Race	Age at Diagnosis (months)	SMA Type	Symptomatic at Diagnosis	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
	Yes	Japanese	13	1	Yes	OAV101 mono				
	No	Japanese	1	5	Yes	OAV101 mono				
	Yes	Japanese	2	1	Yes	OAV101 mono				
	No	Japanese	23	2	Yes	OAV101 mono				
	No	Japanese	10	2	Yes	OAV101 mono				
	Yes	Japanese	13	1	Yes	OAV101 mono				
	No	Japanese	24	2	Yes	OAV101 mono				
	Yes	Japanese	5	1	Yes	Switch to OAV101				
	Yes	Japanese	5	1	Yes	Switch to OAV101				
	No	Chinese	8	1	Yes	Switch to OAV101				

Listing 16.13.jpn
Listing of Japan OAV101 Treated Patients
Japan OAV101 Treated Patients

Patient	HTA	Race	Age at Diagnosis (months)	SMA Type	Symptomatic at Diagnosis	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
	Yes	Japanese	7	1	Yes	Switch to OAV101				
	Yes	Japanese	11	1	Yes	Switch to OAV101				
	No	Japanese	0	5		Switch to OAV101				
	Yes	Japanese	10	1	Yes	Switch to OAV101				
	Yes	Japanese	1	1	Yes	Switch to OAV101				
	Yes	Japanese	6	1	Yes	Switch to OAV101				
	Yes	Japanese	9	1	Yes	Switch to OAV101				
	Yes	Japanese	1	1	Yes	Switch to OAV101				
	Yes		1	1	Yes	Switch to OAV101				

Listing 16.13.jpn
Listing of Japan OAV101 Treated Patients
Japan OAV101 Treated Patients

Patient	HTA	Race	Age at Diagnosis (months)	SMA Type	Symptomatic at Diagnosis	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
	No	Chinese		1	Yes	Switch to OAV101				
	Yes	Japanese	4	1	Yes	Switch to OAV101				
	Yes	Japanese	2	1	Yes	Switch to OAV101				
	Yes	Japanese	1	1	Yes	Transient add-on				

Table 14.1.0.jp
Analysis Datasets
All Japan Enrolled Patients

	Total (N=80)
Patients Enrolled at Japan Centers	80 (100)
OAV101 Treated Patients Excluded Patients	0
OAV101 Treated Patients	80 (100)
Safety Analysis Set Excluded Patients	0
Safety Analysis Set	80 (100)

Table 14.1.1.age3.jp
Patient Enrollment and Disposition by Age at OAV101 Infusion
Japan Analysis Set

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
All enrolled patients (Effectiveness Analysis Set)	14 (100)	9 (100)	22 (100)	31 (100)	4 (100)	80 (100)
Disposition						
Completed MAP	0	0	0	0	0	0
Completed Registry	0	0	0	0	0	0
Still enrolled	14 (100)	9 (100)	20 (90.9)	26 (83.9)	4 (100)	73 (91.3)
Early Termination	0	0	2 (9.1)	5 (16.1)	0	7 (8.8)
Death	0	0	1 (4.5)	0	0	1 (1.3)
Discontinued due to Adverse Event	0	0	0	0	0	0
Lost to Follow-up	0	0	1 (4.5)	3 (9.7)	0	4 (5.0)
Withdrawal of consent	0	0	0	0	0	0
Physician decision	0	0	0	0	0	0
Site is terminated by sponsor	0	0	0	0	0	0
Registry is terminated by sponsor	0	0	0	0	0	0
Patient entered another clinical trial	0	0	0	2 (6.5)	0	2 (2.5)

Percentages are based on all patients with known therapy course.

^a Patient may have more than one response.

Data as of 23MAY2025: ADSL

Prg: TENRDISP.SAS

Table 14.1.1.age3.jpn
Patient Enrollment and Disposition by Age at OAV101 Infusion
Japan Analysis Set

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
If death, Age at death (months) by Number of Copies of the SMN2 Gene mean (SD) [min, max]						
2 Copies	0	0	22.76 (-) [22.8, 22.8]	0	0	22.76 (-) [22.8, 22.8]
Treatment Courses						
OAV101 mono	6 (42.9)	0	5 (22.7)	12 (38.7)	1 (25.0)	24 (30.0)
Add-on	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Transient add-on	1 (7.1)	0	0	0	0	1 (1.3)
Combo w/OAV101	2 (14.3)	4 (44.4)	2 (9.1)	2 (6.5)	0	10 (12.5)
Bridge to OAV101	4 (28.6)	4 (44.4)	12 (54.5)	7 (22.6)	1 (25.0)	28 (35.0)
Switch to OAV101	0	0	3 (13.6)	10 (32.3)	2 (50.0)	15 (18.8)
Nusi/risd combo	0	0	0	0	0	0
Risd mono	0	0	0	0	0	0
Nusi Mono	0	0	0	0	0	0
BSC	0	0	0	0	0	0
Treatment not yet established	0	0	0	0	0	0

Percentages are based on all patients with known therapy course.

^a Patient may have more than one response.

Data as of 23MAY2025: ADSL

Prg: TENRDISP.SAS

Table 14.1.1.age3.jpn
Patient Enrollment and Disposition by Age at OAV101 Infusion
Japan Analysis Set

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Ever exposed to OAV101	14 (100)	9 (100)	22 (100)	31 (100)	4 (100)	80 (100)
Ever exposed to NUSI	6 (42.9)	6 (66.7)	15 (68.2)	17 (54.8)	3 (75.0)	47 (58.8)
Ever exposed to RISD	4 (28.6)	5 (55.6)	3 (13.6)	4 (12.9)	0	16 (20.0)

Percentages are based on all patients with known therapy course.

^a Patient may have more than one response.

Data as of 23MAY2025: ADSL

Prg: TENRDISP.SAS

Table 14.1.1.age3.jpj
Patient Enrollment and Disposition by Age at OAV101 Infusion
Japan Analysis Set

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Did the patient receive SMA Treatment on or before the Informed Consent Date?						
Yes	14 (100)	9 (100)	22 (100)	28 (90.3)	4 (100)	77 (96.3)
No	0	0	0	3 (9.7)	0	3 (3.8)
Reason for no SMA treatment on or before the Informed Consent Date? ^a						
Waiting for insurance approval (US only)	0	0	0	0	0	0
Waiting for a new treatment option to be approved	0	0	0	0	0	0
Physician choice	0	0	0	1 (3.2)	0	1 (1.3)
Parent/Patient choice	0	0	0	1 (3.2)	0	1 (1.3)
Other	0	0	0	1 (3.2)	0	1 (1.3)

Percentages are based on all patients with known therapy course.

^a Patient may have more than one response.

Table 14.1.1.age3.jp
Patient Enrollment and Disposition by Age at OAV101 Infusion
Japan Analysis Set

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Patients with follow-up visit	14 (100)	9 (100)	22 (100)	31 (100)	4 (100)	80 (100)
Patients have been enrolled less than 1 year	0	0	0	0	0	0
Patients have been enrolled less than 1 year with Follow-up visit	0	0	0	0	0	0
Patients have been enrolled between 1-2 years	2 (14.3)	0	2 (9.1)	0	0	4 (5.0)
Patients have been enrolled between 1-2 years with Follow-up visit	2 (100)	0	2 (100)	0	0	4 (100)
Patients have been enrolled more than 2 years	12 (85.7)	9 (100)	20 (90.9)	31 (100)	4 (100)	76 (95.0)
Patients have been enrolled more than 2 years with Follow-up visit	12 (100)	9 (100)	20 (100)	31 (100)	4 (100)	76 (100)

Percentages are based on all patients with known therapy course.

^a Patient may have more than one response.

Data as of 23MAY2025: ADSL

Prg: TENRDISP.SAS

Table 14.1.2.age3.jp
Patient Demographics by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Age at time of data cut (months)						
n	14	9	22	31	4	80
Mean	41.41	49.16	49.68	66.59	80.79	56.28
Std Dev	11.036	11.885	13.973	9.684	3.074	15.856
SE	2.950	3.962	2.979	1.739	1.537	1.773
Median	40.87	45.23	45.96	65.47	81.63	57.62
(Min, Max)	(29.3, 60.3)	(32.7, 63.8)	(22.8, 68.5)	(51.9, 81.9)	(76.5, 83.3)	(22.8, 83.3)
95% CI of the mean	(35.63, 47.20)	(41.40, 56.93)	(43.84, 55.52)	(63.18, 70.00)	(77.78, 83.80)	(52.81, 59.76)
0-6 months	0	0	0	0	0	0
>6-24 months	0	0	1 (4.5)	0	0	1 (1.3)
>24 months	14 (100)	9 (100)	21 (95.5)	31 (100)	4 (100)	79 (98.8)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^aResponses of "Not Reported" may include cases where information was either never requested from the patient or requested but not received, or if the patient marked 'prefer not to answer', etc.

^bn restricted to number with insurance and is used as the denominator for calculating percentages.

Table 14.1.2.age3.jpj
Patient Demographics by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Age at consent/assent into the registry (months)						
n	14	9	22	31	4	80
Mean	6.6	8.9	13.2	23.0	32.0	16.3
Std Dev	3.84	4.01	4.74	5.43	6.22	8.85
SE	1.03	1.34	1.01	0.97	3.11	0.99
Median	6.0	8.0	12.5	23.0	30.0	15.0
(Min, Max)	(2, 16)	(4, 17)	(7, 23)	(12, 37)	(27, 41)	(2, 41)
95% CI of the mean	(4.6, 8.7)	(6.3, 11.5)	(11.2, 15.2)	(21.1, 24.9)	(25.9, 38.1)	(14.4, 18.2)
0-6 months	7 (50.0)	3 (33.3)	0	0	0	10 (12.5)
>6-24 months	7 (50.0)	6 (66.7)	22 (100)	19 (61.3)	0	54 (67.5)
>24 months	0	0	0	12 (38.7)	4 (100)	16 (20.0)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^aResponses of "Not Reported" may include cases where information was either never requested from the patient or requested but not received, or if the patient marked 'prefer not to answer', etc.

^bn restricted to number with insurance and is used as the denominator for calculating percentages.

Table 14.1.2.age3.jpn
Patient Demographics by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Gestational age at birth (weeks) n (%)						
n	14	9	22	30	4	79
>35 weeks	14 (100)	9 (100)	22 (100)	28 (93.3)	4 (100)	77 (97.5)
33-35 weeks	0	0	0	1 (3.3)	0	1 (1.3)
30-32 weeks	0	0	0	0	0	0
28-29 weeks	0	0	0	0	0	0
<= 27 weeks	0	0	0	1 (3.3)	0	1 (1.3)
Gender n (%)						
n	14	9	22	31	4	80
Female	4 (28.6)	4 (44.4)	8 (36.4)	19 (61.3)	3 (75.0)	38 (47.5)
Male	10 (71.4)	5 (55.6)	14 (63.6)	12 (38.7)	1 (25.0)	42 (52.5)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^aResponses of "Not Reported" may include cases where information was either never requested from the patient or requested but not received, or if the patient marked 'prefer not to answer', etc.

^bn restricted to number with insurance and is used as the denominator for calculating percentages.

Table 14.1.2.age3.jpn
Patient Demographics by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Race n (%)						
n	14	9	22	31	4	80
American Indian or Alaska Native	0	0	0	0	0	0
Asian	14 (100)	9 (100)	20 (90.9)	31 (100)	4 (100)	78 (97.5)
Black or African American	0	0	0	0	0	0
Native Hawaiian or other Pacific Islander	0	0	0	0	0	0
White	0	0	0	0	0	0
Multiple	0	0	1 (4.5)	0	0	1 (1.3)
Not Reported ^a	0	0	1 (4.5)	0	0	1 (1.3)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^aResponses of "Not Reported" may include cases where information was either never requested from the patient or requested but not received, or if the patient marked 'prefer not to answer', etc.

^bn restricted to number with insurance and is used as the denominator for calculating percentages.

Table 14.1.2.age3.jpn
Patient Demographics by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Ethnicity n (%)						
n	14	9	22	31	4	80
Hispanic or Latino	0	0	0	0	0	0
Not Hispanic or Latino	14 (100)	9 (100)	21 (95.5)	31 (100)	4 (100)	79 (98.8)
Not Reported ^a	0	0	1 (4.5)	0	0	1 (1.3)
Primary caregiver at home n (%)						
n	1	1	9	8	2	21
Mother	0	1 (100)	7 (77.8)	6 (75.0)	1 (50.0)	15 (71.4)
Father	0	0	0	0	0	0
Mother and Father	1 (100)	0	2 (22.2)	2 (25.0)	1 (50.0)	6 (28.6)
Grandparent(s)	0	0	0	0	0	0
Other	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^aResponses of "Not Reported" may include cases where information was either never requested from the patient or requested but not received, or if the patient marked 'prefer not to answer', etc.

^bn restricted to number with insurance and is used as the denominator for calculating percentages.

Table 14.1.2.age3.jpn
Patient Demographics by Age at OAV101 Infusion
Japan OAV101 Treated Patients

< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^aResponses of "Not Reported" may include cases where information was either never requested from the patient or requested but not received, or if the patient marked 'prefer not to answer', etc.

^bn restricted to number with insurance and is used as the denominator for calculating percentages.

Table 14.1.2.age3.jpn
Patient Demographics by Age at OAV101 Infusion
Japan OAV101 Treated Patients

< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^aResponses of "Not Reported" may include cases where information was either never requested from the patient or requested but not received, or if the patient marked 'prefer not to answer', etc.

^bn restricted to number with insurance and is used as the denominator for calculating percentages.

Table 14.1.2.age3.jp
Patient Demographics by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Age at start of Treatment n (%)						
n	14	9	22	31	4	80
< 6 months	14 (100)	9 (100)	10 (45.5)	5 (16.1)	1 (25.0)	39 (48.8)
=> 6 and < 12 months	0	0	12 (54.5)	6 (19.4)	1 (25.0)	19 (23.8)
=> 12 and < 24 months	0	0	0	20 (64.5)	1 (25.0)	21 (26.3)
=> 24 months	0	0	0	0	1 (25.0)	1 (1.3)
Age at start of Treatment (months)						
n	14	9	22	31	4	80
Mean	1.36	2.44	5.45	12.42	14.75	7.56
Std Dev	0.842	1.424	3.419	5.708	10.243	6.535
SE	0.225	0.475	0.729	1.025	5.121	0.731
Median	2.00	3.00	6.00	14.00	16.50	6.00
(Min, Max)	(0.0, 2.0)	(0.0, 5.0)	(1.0, 11.0)	(1.0, 22.0)	(2.0, 24.0)	(0.0, 24.0)
95% CI of the mean	(0.92, 1.80)	(1.51, 3.37)	(4.03, 6.88)	(10.41, 14.43)	(4.71, 24.79)	(6.13, 8.99)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^aResponses of "Not Reported" may include cases where information was either never requested from the patient or requested but not received, or if the patient marked 'prefer not to answer', etc.

^bn restricted to number with insurance and is used as the denominator for calculating percentages.

Table 14.1.2.age3.jp
Patient Demographics by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Weight at start of OAV101 Treatment n (%)						
n	13	8	22	28	3	74
< 8.5 kg	13 (100)	8 (100)	17 (77.3)	15 (53.6)	1 (33.3)	54 (73.0)
>= 8.5-13.5 kg	0	0	5 (22.7)	13 (46.4)	2 (66.7)	20 (27.0)
>= 13.5-21 kg	0	0	0	0	0	0
>= 21 kg	0	0	0	0	0	0
Weight at start of OAV101 Treatment (kg)						
n	13	8	22	28	3	74
Mean	4.38	5.11	7.77	8.46	8.73	7.19
Std Dev	1.094	1.169	1.010	1.473	1.250	2.043
SE	0.303	0.413	0.215	0.278	0.722	0.237
Median	4.10	5.30	7.55	8.30	9.30	7.40
(Min, Max)	(2.6, 6.4)	(3.1, 6.6)	(5.6, 10.1)	(5.5, 11.2)	(7.3, 9.6)	(2.6, 11.2)
95% CI of the mean	(3.78, 4.97)	(4.30, 5.92)	(7.35, 8.19)	(7.92, 9.01)	(7.32, 10.15)	(6.72, 7.65)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^aResponses of "Not Reported" may include cases where information was either never requested from the patient or requested but not received, or if the patient marked 'prefer not to answer', etc.

^bn restricted to number with insurance and is used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Was the patient screened for SMA as a new-born n (%)						
n	14	9	22	31	4	80
Yes	5 (35.7)	3 (33.3)	2 (9.1)	0	0	10 (12.5)
No	9 (64.3)	6 (66.7)	20 (90.9)	31 (100)	4 (100)	70 (87.5)
Did the patient display symptoms at the time of diagnosis n (%)						
n	14	9	22	31	4	80
Yes	8 (57.1)	7 (77.8)	20 (90.9)	31 (100)	4 (100)	70 (87.5)
No	6 (42.9)	2 (22.2)	2 (9.1)	0	0	10 (12.5)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a patient may have more than one response.

^b includes only patients with symptoms.

^c includes only patients with an immediate familial history of SMA.

^d Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jp
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
SMA symptoms at diagnosis. Did the patient display symptoms at the time of diagnosis ^a n (%)						
n ^b	8	7	20	31	4	70
Hypotonia	8 (100)	7 (100)	19 (95.0)	31 (100)	4 (100)	69 (98.6)
Limb weakness	8 (100)	5 (71.4)	20 (100)	27 (87.1)	4 (100)	64 (91.4)
Pneumonia or respiratory symptoms	4 (50.0)	4 (57.1)	5 (25.0)	6 (19.4)	1 (25.0)	20 (28.6)
Tongue fasciculations	3 (37.5)	5 (71.4)	15 (75.0)	15 (48.4)	0	38 (54.3)
Developmental delay	2 (25.0)	2 (28.6)	11 (55.0)	22 (71.0)	2 (50.0)	39 (55.7)
Constipation	1 (12.5)	1 (14.3)	2 (10.0)	5 (16.1)	0	9 (12.9)
Swallowing or feeding difficulties	4 (50.0)	3 (42.9)	6 (30.0)	7 (22.6)	1 (25.0)	21 (30.0)
Other	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a patient may have more than one response.

^b includes only patients with symptoms.

^c includes only patients with an immediate familial history of SMA.

^d Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Age at first symptoms onset (months) ^b						
n	9	7	20	31	4	71
Mean	0.2	0.7	3.7	5.9	7.8	4.2
Std Dev	0.44	0.76	2.83	4.23	7.59	4.21
SE	0.15	0.29	0.63	0.76	3.79	0.50
Median	0.0	1.0	4.0	5.0	6.5	3.0
(Min, Max)	(0, 1)	(0, 2)	(0, 9)	(0, 15)	(0, 18)	(0, 18)
95% CI of the mean	(-0.1, 0.5)	(0.2, 1.3)	(2.5, 4.9)	(4.4, 7.4)	(0.3, 15.2)	(3.2, 5.1)
< 6 months	9 (100)	7 (100)	13 (65.0)	16 (51.6)	2 (50.0)	47 (66.2)
=> 6 and < 12 months	0	0	7 (35.0)	12 (38.7)	1 (25.0)	20 (28.2)
=> 12 and < 24 months	0	0	0	3 (9.7)	1 (25.0)	4 (5.6)
=> 24 months	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a patient may have more than one response.

^b includes only patients with symptoms.

^c includes only patients with an immediate familial history of SMA.

^d Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Genetically confirmed SMA diagnosis prior to birth n (%)						
n	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a patient may have more than one response.

^b includes only patients with symptoms.

^c includes only patients with an immediate familial history of SMA.

^d Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jp
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Age at genetically confirmed SMA diagnosis (post-partum months)						
n	14	9	22	30	4	79
Mean	0.9	2.3	5.4	11.4	14.3	7.0
Std Dev	0.73	1.41	3.37	5.44	10.37	6.20
SE	0.20	0.47	0.72	0.99	5.19	0.70
Median	1.0	2.0	6.5	12.5	16.5	6.0
(Min, Max)	(0, 2)	(0, 5)	(0, 10)	(1, 24)	(1, 23)	(0, 24)
95% CI of the mean	(0.5, 1.3)	(1.4, 3.3)	(4.0, 6.8)	(9.4, 13.3)	(4.1, 24.4)	(5.6, 8.3)
< 6 months	14 (100)	9 (100)	9 (40.9)	6 (20.0)	1 (25.0)	39 (49.4)
=> 6 and < 12 months	0	0	13 (59.1)	6 (20.0)	1 (25.0)	20 (25.3)
=> 12 and < 24 months	0	0	0	17 (56.7)	2 (50.0)	19 (24.1)
=> 24 months	0	0	0	1 (3.3)	0	1 (1.3)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a patient may have more than one response.

^b includes only patients with symptoms.

^c includes only patients with an immediate familial history of SMA.

^d Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Has the Number of Copies of the SMN2 Gene been determined n (%)						
n	14	9	22	31	4	80
Yes	14 (100)	9 (100)	22 (100)	31 (100)	4 (100)	80 (100)
No	0	0	0	0	0	0
Number of Copies of the SMN2 Gene n (%)						
n	14	9	22	31	4	80
1 Copy	0	0	0	0	0	0
2 Copies	10 (71.4)	6 (66.7)	11 (50.0)	12 (38.7)	1 (25.0)	40 (50.0)
3 Copies	4 (28.6)	3 (33.3)	11 (50.0)	19 (61.3)	3 (75.0)	40 (50.0)
4 Copies	0	0	0	0	0	0
>4 Copies	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a patient may have more than one response.

^b includes only patients with symptoms.

^c includes only patients with an immediate familial history of SMA.

^d Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Genetic testing for SMA n (%)						
n	14	9	22	31	4	80
SMN1 homozygous deletion of exon 7 (or 7&8)	14 (100)	8 (88.9)	22 (100)	29 (93.5)	4 (100)	77 (96.3)
SMN1 heterozygous deletion of exon 7/8 and subtle mutation (intragenic deletion or duplication or point mutation)	0	1 (11.1)	0	2 (6.5)	0	3 (3.8)
Other SMN1 result	0	0	0	0	0	0
Patient has the c.859G>C SMN2 variant n (%)						
n	14	9	22	31	4	80
Yes	0	0	0	0	0	0
No	0	3 (33.3)	5 (22.7)	4 (12.9)	0	12 (15.0)
Not Tested	14 (100)	6 (66.7)	17 (77.3)	27 (87.1)	4 (100)	68 (85.0)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a patient may have more than one response.

^b includes only patients with symptoms.

^c includes only patients with an immediate familial history of SMA.

^d Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
SMA Type n (%)						
n ^b	7	7	20	31	4	69
0	0	0	0	0	0	0
I	7 (100)	7 (100)	17 (85.0)	21 (67.7)	2 (50.0)	54 (78.3)
II	0	0	3 (15.0)	10 (32.3)	2 (50.0)	15 (21.7)
III	0	0	0	0	0	0
IV	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a patient may have more than one response.

^b includes only patients with symptoms.

^c includes only patients with an immediate familial history of SMA.

^d Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
SMA function status by age at first dose n (%)						
n	13	9	22	31	4	79
Non-sitter	13 (100)	9 (100)	22 (100)	22 (71.0)	2 (50.0)	68 (86.1)
< 6 months ^d	13 (100)	9 (100)	10 (45.5)	5 (22.7)	1 (50.0)	38 (55.9)
=> 6 and < 12 months ^d	0	0	12 (54.5)	6 (27.3)	1 (50.0)	19 (27.9)
=> 12 and < 24 months ^d	0	0	0	11 (50.0)	0	11 (16.2)
=> 24 months ^d	0	0	0	0	0	0
Sitter	0	0	0	9 (29.0)	1 (25.0)	10 (12.7)
< 6 months ^d	0	0	0	0	0	0
=> 6 and < 12 months ^d	0	0	0	0	0	0
=> 12 and < 24 months ^d	0	0	0	9 (100)	1 (100)	10 (100)
=> 24 months ^d	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a patient may have more than one response.

^b includes only patients with symptoms.

^c includes only patients with an immediate familial history of SMA.

^d Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Standing	0	0	0	0	1 (25.0)	1 (1.3)
< 6 months ^d	0	0	0	0	0	0
=> 6 and < 12 months ^d	0	0	0	0	0	0
=> 12 and < 24 months ^d	0	0	0	0	0	0
=> 24 months ^d	0	0	0	0	1 (100)	1 (100)
Walker	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a patient may have more than one response.

^b includes only patients with symptoms.

^c includes only patients with an immediate familial history of SMA.

^d Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Does the patient have an immediate familial history of SMA? n (%)						
n	13	9	22	31	4	79
Yes	5 (38.5)	1 (11.1)	3 (13.6)	2 (6.5)	1 (25.0)	12 (15.2)
No	8 (61.5)	8 (88.9)	19 (86.4)	29 (93.5)	3 (75.0)	67 (84.8)
Relative(s) with history of SMA ^a n (%)						
n ^c	5	1	3	2	1	12
Biological Mother is a known carrier	2 (40.0)	0	0	0	0	2 (16.7)
Biological Father is a known carrier	2 (40.0)	0	0	0	0	2 (16.7)
Biological Sibling(s)	4 (80.0)	1 (100)	2 (66.7)	0	0	7 (58.3)
Other	1 (20.0)	0	1 (33.3)	2 (100)	1 (100)	5 (41.7)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a patient may have more than one response.

^b includes only patients with symptoms.

^c includes only patients with an immediate familial history of SMA.

^d Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Weight at diagnosis of SMA (kg)						
n	13	8	22	26	4	73
Mean	3.96	4.04	6.39	7.53	7.55	6.17
Std Dev	0.974	1.003	2.055	1.583	2.511	2.190
SE	0.270	0.355	0.438	0.310	1.255	0.256
Median	3.60	3.95	7.15	7.85	8.30	6.00
(Min, Max)	(2.7, 6.0)	(2.7, 5.4)	(3.1, 9.7)	(4.6, 10.0)	(4.0, 9.6)	(2.7, 10.0)
95% CI of the mean	(3.43, 4.49)	(3.34, 4.73)	(5.53, 7.25)	(6.92, 8.14)	(5.09, 10.01)	(5.67, 6.67)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a patient may have more than one response.

^b includes only patients with symptoms.

^c includes only patients with an immediate familial history of SMA.

^d Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Length/Height at diagnosis of SMA (cm)						
n	13	8	22	26	4	73
Mean	53.22	54.61	63.63	69.26	71.00	63.19
Std Dev	4.039	5.450	7.221	6.859	10.116	9.181
SE	1.120	1.927	1.539	1.345	5.058	1.075
Median	53.30	55.60	66.15	69.90	74.35	62.50
(Min, Max)	(47.3, 58.5)	(47.0, 61.2)	(51.4, 76.4)	(56.7, 80.0)	(56.8, 78.5)	(47.0, 80.0)
95% CI of the mean	(51.02, 55.41)	(50.84, 58.39)	(60.61, 66.64)	(66.62, 71.89)	(61.09, 80.91)	(61.09, 65.30)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a patient may have more than one response.

^b includes only patients with symptoms.

^c includes only patients with an immediate familial history of SMA.

^d Number in functional status used as the denominator for calculating percentages.

Table 14.1.6.age3.jpn
OAV101 Treatment by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Time of therapy relative to SMA diagnosis (months)						
n	14	9	22	31	4	80
Mean	0.57	1.59	3.33	6.57	9.82	4.23
Std Dev	0.159	1.275	2.787	6.447	10.061	5.384
SE	0.042	0.425	0.594	1.158	5.031	0.602
Median	0.54	1.25	2.20	1.87	7.67	1.38
(Min, Max)	(0.4, 0.9)	(0.4, 3.8)	(0.3, 9.0)	(0.6, 19.3)	(1.1, 22.8)	(0.3, 22.8)
95% CI of the mean	(0.49, 0.65)	(0.76, 2.42)	(2.16, 4.49)	(4.30, 8.84)	(-0.04, 19.68)	(3.05, 5.41)
Time from diagnosis to treatment n (%)						
n	14	9	22	31	4	80
0-6 months	14 (100)	9 (100)	18 (81.8)	17 (54.8)	2 (50.0)	60 (75.0)
> 6-24 months	0	0	4 (18.2)	14 (45.2)	2 (50.0)	20 (25.0)
> 24 months	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.6.age3.jp
OAV101 Treatment by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Age at OAV101 infusion (months)						
n	14	9	22	31	4	80
Mean	1.6	3.8	8.4	17.6	24.0	11.0
Std Dev	0.65	0.83	1.40	3.51	0.00	7.38
SE	0.17	0.28	0.30	0.63	0.00	0.83
Median	2.0	4.0	8.0	17.0	24.0	10.0
(Min, Max)	(0, 2)	(3, 5)	(6, 11)	(12, 23)	(24, 24)	(0, 24)
95% CI of the mean	(1.2, 1.9)	(3.2, 4.3)	(7.8, 8.9)	(16.3, 18.8)	(24.0, 24.0)	(9.4, 12.6)
Age at OAV101 infusion (categorical)						
n (%)						
< 6 months	14 (100)	9 (100)	0	0	0	23 (28.8)
≥ 6 and < 12 months	0	0	22 (100)	0	0	22 (27.5)
≥ 12 and < 24 months	0	0	0	31 (100)	0	31 (38.8)
≥ 24 months	0	0	0	0	4 (100)	4 (5.0)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.6.age3.jpn
OAV101 Treatment by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Primary reason for switching to OAV101? n (%)						
n	6	8	17	19	3	53
Perceived lack of drug effect	1 (16.7)	0	1 (5.9)	0	0	2 (3.8)
Motor function	1 (16.7)	0	0	0	0	1 (1.9)
Respiratory function	0	0	0	0	0	0
Swallowing or feeding ability for age	0	0	1 (5.9)	0	0	1 (1.9)
Electrophysiological/another biomarker response	0	0	0	0	0	0
No additional response chosen	0	0	0	0	0	0
Adverse Events	0	0	0	0	0	0
Parent/Caregiver/Patient decision	4 (66.7)	6 (75.0)	13 (76.5)	14 (73.7)	2 (66.7)	39 (73.6)
Alternative Treatment Available and Reimbursed	1 (16.7)	2 (25.0)	3 (17.6)	5 (26.3)	1 (33.3)	12 (22.6)
Other	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.6.age3.jpn
OAV101 Treatment by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Duration since OAV101 infusion (months)						
n	14	9	22	31	4	80
Mean	39.93	45.28	39.67	46.09	57.08	43.71
Std Dev	10.820	11.696	14.079	10.938	3.306	12.259
SE	2.892	3.899	3.002	1.964	1.653	1.371
Median	38.90	42.61	34.81	47.31	57.97	43.06
(Min, Max)	(28.3, 58.5)	(29.5, 59.6)	(15.8, 59.4)	(12.6, 59.5)	(52.5, 59.9)	(12.6, 59.9)
95% CI of the mean	(34.26, 45.60)	(37.64, 52.92)	(33.79, 45.55)	(42.24, 49.94)	(53.84, 60.32)	(41.02, 46.39)
OAV101 Exposure (person-year)	46.6	34.0	72.7	119.1	19.0	291.4

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.6.g5tx.jp
OAV101 Treatment by Therapy at OAV101 Infusion
Japan OAV101 Treated Patients

	OAV101 mono (N=24)	Add-on (N=2)	Transient add-on (N=1)	Combo w/OAV101 (N=10)	Bridge to OAV101 (N=28)	Switch to OAV101 (N=15)	Total OAV101 (N=80)
Time of therapy relative to SMA diagnosis (months)							
n	24	2	1	10	28	15	80
Mean	0.93	0.56	0.49	3.12	3.11	13.08	4.23
Std Dev	0.389	0.046	-	3.695	3.123	5.173	5.384
SE	0.079	0.033	-	1.169	0.590	1.336	0.602
Median	0.85	0.56	0.49	1.58	1.63	12.68	1.38
(Min, Max)	(0.3, 1.9)	(0.5, 0.6)	(0.5, 0.5)	(0.4, 11.4)	(0.4, 12.7)	(5.0, 22.8)	(0.3, 22.8)
95% CI of the mean	(0.77, 1.08)	(0.49, 0.62)	(-, -)	(0.83, 5.41)	(1.96, 4.27)	(10.46, 15.70)	(3.05, 5.41)
Time from diagnosis to treatment n (%)							
n	24	2	1	10	28	15	80
0-6 months	24 (100)	2 (100)	1 (100)	8 (80.0)	23 (82.1)	2 (13.3)	60 (75.0)
> 6-24 months	0	0	0	2 (20.0)	5 (17.9)	13 (86.7)	20 (25.0)
> 24 months	0	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.6.g5tx.jp
OAV101 Treatment by Therapy at OAV101 Infusion
Japan OAV101 Treated Patients

	OAV101 mono (N=24)	Add-on (N=2)	Transient add-on (N=1)	Combo w/OAV101 (N=10)	Bridge to OAV101 (N=28)	Switch to OAV101 (N=15)	Total OAV101 (N=80)
Age at OAV101 infusion (months)							
n	24	2	1	10	28	15	80
Mean	11.1	3.5	2.0	6.6	9.6	18.1	11.0
Std Dev	6.80	2.12	-	5.17	7.04	5.78	7.38
SE	1.39	1.50	-	1.63	1.33	1.49	0.83
Median	12.5	3.5	2.0	4.0	7.0	19.0	10.0
(Min, Max)	(1, 24)	(2, 5)	(2, 2)	(1, 15)	(0, 24)	(7, 24)	(0, 24)
95% CI of the mean	(8.4, 13.8)	(0.6, 6.4)	(-, -)	(3.4, 9.8)	(7.0, 12.2)	(15.1, 21.0)	(9.4, 12.6)
Age at OAV101 infusion (categorical) n (%)							
< 6 months	6 (25.0)	2 (100)	1 (100)	6 (60.0)	8 (28.6)	0	23 (28.8)
≥ 6 and < 12 months	5 (20.8)	0	0	2 (20.0)	12 (42.9)	3 (20.0)	22 (27.5)
≥ 12 and < 24 months	12 (50.0)	0	0	2 (20.0)	7 (25.0)	10 (66.7)	31 (38.8)
≥ 24 months	1 (4.2)	0	0	0	1 (3.6)	2 (13.3)	4 (5.0)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.6.g5tx.jp
OAV101 Treatment by Therapy at OAV101 Infusion
Japan OAV101 Treated Patients

	OAV101 mono (N=24)	Add-on (N=2)	Transient add-on (N=1)	Combo w/OAV101 (N=10)	Bridge to OAV101 (N=28)	Switch to OAV101 (N=15)	Total OAV101 (N=80)
Primary reason for switching to OAV101? n (%)							
n	0	0	0	10	28	15	53
Perceived lack of drug effect				1 (10.0)	0	1 (6.7)	2 (3.8)
Motor function				1 (10.0)	0	0	1 (1.9)
Respiratory function				0	0	0	0
Swallowing or feeding ability for age				0	0	1 (6.7)	1 (1.9)
Electrophysiological/another biomarker response				0	0	0	0
No additional response chosen				0	0	0	0
Adverse Events				0	0	0	0
Parent/Caregiver/Patient decision				6 (60.0)	22 (78.6)	11 (73.3)	39 (73.6)
Alternative Treatment Available and Reimbursed				3 (30.0)	6 (21.4)	3 (20.0)	12 (22.6)
Other				0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.6.g5tx.jp
OAV101 Treatment by Therapy at OAV101 Infusion
Japan OAV101 Treated Patients

	OAV101 mono (N=24)	Add-on (N=2)	Transient add-on (N=1)	Combo w/OAV101 (N=10)	Bridge to OAV101 (N=28)	Switch to OAV101 (N=15)	Total OAV101 (N=80)
Duration since OAV101 infusion (months)							
n	24	2	1	10	28	15	80
Mean	43.82	51.70	58.51	43.95	42.18	44.15	43.71
Std Dev	12.119	0.163	-	11.445	11.713	15.108	12.259
SE	2.474	0.115	-	3.619	2.214	3.901	1.371
Median	47.70	51.70	58.51	44.44	38.90	43.50	43.06
(Min, Max)	(12.6, 59.5)	(51.6, 51.8)	(58.5, 58.5)	(28.8, 59.4)	(25.6, 59.6)	(15.8, 59.9)	(12.6, 59.9)
95% CI of the mean	(38.97, 48.67)	(51.47, 51.92)	(-, -)	(36.86, 51.05)	(37.85, 46.52)	(36.51, 51.80)	(41.02, 46.39)
OAV101 Exposure (person-year)	87.6	8.6	4.9	36.6	98.4	55.2	291.4

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.7.cpy.jp
Nusinersen Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=28)	3 Copies (N=19)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=47)
Time of therapy relative to SMA diagnosis (months)						
n	0	28	19	0	0	47
Mean		0.72	1.64			1.09
Std Dev		2.604	3.794			3.133
SE		0.492	0.870			0.457
Median		0.26	0.33			0.26
(Min, Max)		(-2.4, 13.6)	(-0.3, 12.5)			(-2.4, 13.6)
95% CI of the mean		(-0.24, 1.69)	(-0.07, 3.34)			(0.20, 1.99)
Time from diagnosis to treatment						
0-6 months n (%)	0	26 (92.9)	16 (84.2)	0	0	42 (89.4)
>6-24 months n (%)	0	1 (3.6)	2 (10.5)	0	0	3 (6.4)
>24 months n (%)	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percent.

Table 14.1.7.cpy.jpn
Nusinersen Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=28)	3 Copies (N=19)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=47)
Age at first dose (months)						
n	0	28	19	0	0	47
Mean		3.9	9.8			6.3
Std Dev		3.67	6.93			5.95
SE		0.69	1.59			0.87
Median		3.0	9.0			5.0
(Min, Max)		(0, 15)	(0, 22)			(0, 22)
95% CI of the mean		(2.5, 5.2)	(6.7, 12.9)			(4.6, 8.0)
Age at first dose (categorical)	0	28	19	0	0	47
< 6 months n (%)	0	21 (75.0)	5 (26.3)	0	0	26 (55.3)
=> 6 and < 12 months n (%)	0	5 (17.9)	8 (42.1)	0	0	13 (27.7)
=> 12 and < 24 months n (%)	0	2 (7.1)	6 (31.6)	0	0	8 (17.0)
=> 24 months n (%)	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percent.

Table 14.1.7.cpy.jp
Nusinersen Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=28)	3 Copies (N=19)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=47)
Duration of therapy (months)						
n	0	28	19	0	0	47
Mean		6.71	6.31			6.55
Std Dev		9.362	11.469			10.146
SE		1.769	2.631			1.480
Median		2.14	2.07			2.14
(Min, Max)		(0.4, 45.0)	(0.4, 41.4)			(0.4, 45.0)
95% CI of the mean		(3.24, 10.18)	(1.16, 11.47)			(3.65, 9.45)
Has the patient received OAV101, Risdiplam, or other SMA treatment and then added/switched to Nusinersen? n (%)						
n	0	28	18	0	0	46
Yes	0	1 (3.6)	3 (16.7)	0	0	4 (8.7)
No	0	27 (96.4)	15 (83.3)	0	0	42 (91.3)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percent.

Table 14.1.7.cpy.jp
Nusinersen Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=28)	3 Copies (N=19)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=47)
Primary reason for adding/switching to Nusinersen and reason for discontinuing SMA Treatment (if applicable) ^a ? n (%)						
n	0	1	3	0	0	4
Perceived lack of drug effect	0	1 (100)	0	0	0	1 (25.0)
Motor function	0	1 (100)	0	0	0	1 (25.0)
Respiratory function	0	0	0	0	0	0
Swallowing or feeding ability for age	0	0	0	0	0	0
Electrophysiological/another biomarker response	0	0	0	0	0	0
No additional response chosen	0	0	0	0	0	0
Adverse event	0	0	0	0	0	0
Parent/Caregiver/Patient decision	0	0	2 (66.7)	0	0	2 (50.0)
Alternative Treatment Available and Reimbursed	0	0	1 (33.3)	0	0	1 (25.0)
Irregular compliance	0	0	0	0	0	0
Other	0	0	0	0	0	0
Nusinersen Exposure (person-year)	-	15.7	10.0	-	-	25.6

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percent.

Table 14.1.7.txn.jp
Nusinersen Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=0)	Transient add-on (N=1)	Combo w/OAV101 (N=7)	Bridge to OAV101 (N=25)	Switch to OAV101 (N=14)	Nusi/risd combo (N=0)	Nusi Mono (N=0)	Total (N=47)
Time of therapy relative to SMA diagnosis (months)								
n	0	1	7	25	14	0	0	47
Mean		13.63	0.30	0.74	1.23			1.09
Std Dev		-	0.231	2.405	3.364			3.133
SE		-	0.087	0.481	0.899			0.457
Median		13.63	0.33	0.16	0.33			0.26
(Min, Max)		(13.6, 13.6)	(0.1, 0.7)	(-0.3, 12.2)	(-2.4, 12.5)			(-2.4, 13.6)
95% CI of the mean		(-, -)	(0.12, 0.47)	(-0.21, 1.68)	(-0.53, 2.99)			(0.20, 1.99)
Time from diagnosis to treatment								
0-6 months n (%)	0	0	7 (100)	23 (92.0)	12 (85.7)	0	0	42 (89.4)
>6-24 months n (%)	0	1 (100)	0	1 (4.0)	1 (7.1)	0	0	3 (6.4)
>24 months n (%)	0	0	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percent.

Table 14.1.7.txn.jp
Nusinersen Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=0)	Transient add-on (N=1)	Combo w/OAV101 (N=7)	Bridge to OAV101 (N=25)	Switch to OAV101 (N=14)	Nusi/risd combo (N=0)	Nusi Mono (N=0)	Total (N=47)
Age at first dose (months)								
n	0	1	7	25	14	0	0	47
Mean		15.0	3.6	6.7	6.2			6.3
Std Dev		-	2.64	6.84	5.06			5.95
SE		-	1.00	1.37	1.35			0.87
Median		15.0	3.0	3.0	5.5			5.0
(Min, Max)		(15, 15)	(0, 7)	(0, 22)	(1, 20)			(0, 22)
95% CI of the mean		(-, -)	(1.6, 5.5)	(4.0, 9.4)	(3.6, 8.9)			(4.6, 8.0)
Age at first dose (categorical)	0	1	7	25	14	0	0	47
< 6 months n (%)	0	0	5 (71.4)	14 (56.0)	7 (50.0)	0	0	26 (55.3)
=> 6 and < 12 months n (%)	0	0	2 (28.6)	5 (20.0)	6 (42.9)	0	0	13 (27.7)
=> 12 and < 24 months n (%)	0	1 (100)	0	6 (24.0)	1 (7.1)	0	0	8 (17.0)
=> 24 months n (%)	0	0	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percent.

Table 14.1.7.txn.jp
Nusinersen Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=0)	Transient add-on (N=1)	Combo w/OAV101 (N=7)	Bridge to OAV101 (N=25)	Switch to OAV101 (N=14)	Nusi/risd combo (N=0)	Nusi Mono (N=0)	Total (N=47)
Duration of therapy (months)								
n	0	1	7	25	14	0	0	47
Mean		44.98	2.88	4.21	9.81			6.55
Std Dev		-	3.664	10.195	4.840			10.146
SE		-	1.385	2.039	1.293			1.480
Median		44.98	0.92	0.92	8.03			2.14
(Min, Max)		(45.0, 45.0)	(0.4, 9.9)	(0.4, 41.4)	(5.3, 19.2)			(0.4, 45.0)
95% CI of the mean		(-, -)	(0.16, 5.59)	(0.22, 8.21)	(7.27, 12.34)			(3.65, 9.45)
Has the patient received OAV101, Risdiplam, or other SMA treatment and then added/switched to Nusinersen? n (%)								
n	0	1	7	25	13	0	0	46
Yes	0	1 (100)	0	3 (12.0)	0	0	0	4 (8.7)
No	0	0	7 (100)	22 (88.0)	13 (100)	0	0	42 (91.3)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percent.

Table 14.1.7.txn.jp
Nusinersen Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=0)	Transient add-on (N=1)	Combo w/OAV101 (N=7)	Bridge to OAV101 (N=25)	Switch to OAV101 (N=14)	Nusi/risd combo (N=0)	Nusi Mono (N=0)	Total (N=47)
Primary reason for adding/switching to Nusinersen and reason for discontinuing SMA Treatment (if applicable) ^a ? n (%)								
n	0	1	0	3	0	0	0	4
Perceived lack of drug effect	0	1 (100)	0	0	0	0	0	1 (25.0)
Motor function	0	1 (100)	0	0	0	0	0	1 (25.0)
Respiratory function	0	0	0	0	0	0	0	0
Swallowing or feeding ability for age	0	0	0	0	0	0	0	0
Electrophysiological/another biomarker response	0	0	0	0	0	0	0	0
No additional response chosen	0	0	0	0	0	0	0	0
Adverse event	0	0	0	0	0	0	0	0
Parent/Caregiver/Patient decision	0	0	0	2 (66.7)	0	0	0	2 (50.0)
Alternative Treatment Available and Reimbursed	0	0	0	1 (33.3)	0	0	0	1 (25.0)
Irregular compliance	0	0	0	0	0	0	0	0
Other	0	0	0	0	0	0	0	0
Nusinersen Exposure (person-year)	-	3.7	1.7	8.8	11.4	-	-	25.6

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percent.

Table 14.1.8.age3.jpn
AAV9 Antibody Testing Results by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Patients with ≥ 1 AAV9 tests meeting eligibility criteria ^a n(%)						
n	14	9	22	31	4	80
Patient with only 1 test reported	14 (100)	8 (88.9)	21 (95.5)	31 (100)	4 (100)	78 (97.5)
Patient with 2 or more tests reported ^b	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)
Time between tests (days) ^c						
n	0	1	1	0	0	2
Mean		21.00	14.00			17.50
Std Dev		-	-			4.950
SE		-	-			3.500
Median		21.00	14.00			17.50
(Min, Max)		(21.0, 21.0)	(14.0, 14.0)			(14.0, 21.0)
95% CI of the mean		(-, -)	(-, -)			(10.64, 24.36)

^a Eligibility criteria is defined as an AAV9 result of ≤ 1:50.

^b AAV9 retesting may be performed if an AAV9 result is ≥ 1:50.

^c Patients who reported 2 or more AAV9 test results.

Table 14.1.9.cpy.jpn
Risdiplam Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=13)	3 Copies (N=3)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=16)
Time of therapy relative to SMA diagnosis (months)						
n	0	13	3	0	0	16
Mean		17.71	9.48			16.16
Std Dev		19.659	15.945			18.817
SE		5.452	9.206			4.704
Median		7.92	0.53			7.20
(Min, Max)		(0.0, 59.5)	(0.0, 27.9)			(0.0, 59.5)
95% CI of the mean		(7.02, 28.39)	(-8.56, 27.53)			(6.94, 25.38)
Time of therapy relative to SMA diagnosis (categorical) n (%)						
0-6 months	0	5 (38.5)	2 (66.7)	0	0	7 (43.8)
>6-24 months	0	3 (23.1)	0	0	0	3 (18.8)
>24 months	0	5 (38.5)	1 (33.3)	0	0	6 (37.5)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.cpy.jp
Risdiplam Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=13)	3 Copies (N=3)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=16)
Age at first dose (months)						
n	0	13	3	0	0	16
Mean		21.8	16.7			20.9
Std Dev		19.55	16.17			18.58
SE		5.42	9.33			4.64
Median		19.0	14.0			16.5
(Min, Max)		(2, 65)	(2, 34)			(2, 65)
95% CI of the mean		(11.2, 32.5)	(-1.6, 35.0)			(11.8, 30.0)
Age at first dose (categorical) n (%)						
< 6 months	0	3 (23.1)	1 (33.3)	0	0	4 (25.0)
=> 6 and < 12 months	0	3 (23.1)	0	0	0	3 (18.8)
=> 12 and < 24 months	0	1 (7.7)	1 (33.3)	0	0	2 (12.5)
=> 24 months	0	6 (46.2)	1 (33.3)	0	0	7 (43.8)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.cpy.jpn
Risdiplam Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=13)	3 Copies (N=3)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=16)
Duration of therapy (months)						
n	0	13	3	0	0	16
Mean		18.28	12.48			17.20
Std Dev		13.348	11.105			12.823
SE		3.702	6.411			3.206
Median		17.08	13.47			15.54
(Min, Max)		(0.6, 45.2)	(0.9, 23.1)			(0.6, 45.2)
95% CI of the mean		(11.03, 25.54)	(-0.08, 25.05)			(10.91, 23.48)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.cpy.jp
Risdiplam Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=13)	3 Copies (N=3)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=16)
Has the patient received OAV101 Nusinersen or other SMA treatment and added Risdiplam? n (%)						
n	0	13	3	0	0	16
Yes	0	9 (69.2)	1 (33.3)	0	0	10 (62.5)
No	0	4 (30.8)	2 (66.7)	0	0	6 (37.5)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.cpy.jpn
Risdiplam Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=13)	3 Copies (N=3)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=16)
Primary reason for adding Risdiplam ^a ? n (%)						
n	0	9	1	0	0	10
Perceived lack of drug effect	0	3 (33.3)	1 (100)	0	0	4 (40.0)
Motor function	0	1 (11.1)	0	0	0	1 (10.0)
Respiratory function	0	0	1 (100)	0	0	1 (10.0)
Swallowing or feeding ability for age	0	2 (22.2)	0	0	0	2 (20.0)
Electrophysiological/another biomarker response	0	0	0	0	0	0
No additional response chosen	0	0	0	0	0	0
Adverse Events	0	2 (22.2)	0	0	0	2 (20.0)
Parent/Caregiver/Patient decision	0	2 (22.2)	0	0	0	2 (20.0)
Alternative Treatment Available and Reimbursed	0	1 (11.1)	0	0	0	1 (10.0)
Other	0	1 (11.1)	0	0	0	1 (10.0)
Risdiplam Exposure (person-year)	-	19.8	3.1	-	-	22.9

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.txr.jp
Risdiplam Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=2)	Transient add-on (N=0)	Combo w/OAV101 (N=10)	Bridge to OAV101 (N=3)	Switch to OAV101 (N=1)	Nusi/risd combo (N=0)	Risd mono (N=0)	Total (N=16)
Time of therapy relative to SMA diagnosis (months)								
n	2	0	10	3	1	0	0	16
Mean	32.90		19.13	0.47	0.13			16.16
Std Dev	9.827		19.917	0.187	-			18.817
SE	6.949		6.298	0.108	-			4.704
Median	32.90		15.59	0.53	0.13			7.20
(Min, Max)	(26.0, 39.9)		(0.0, 59.5)	(0.3, 0.6)	(0.1, 0.1)			(0.0, 59.5)
95% CI of the mean	(19.28, 46.52)		(6.78, 31.47)	(0.26, 0.68)	(-, -)			(6.94, 25.38)
Time of therapy relative to SMA diagnosis (categorical) n (%)								
0-6 months	0	0	3 (30.0)	3 (100)	1 (100)	0	0	7 (43.8)
>6-24 months	0	0	3 (30.0)	0	0	0	0	3 (18.8)
>24 months	2 (100)	0	4 (40.0)	0	0	0	0	6 (37.5)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.txr.jp
Risdiplam Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=2)	Transient add-on (N=0)	Combo w/OAV101 (N=10)	Bridge to OAV101 (N=3)	Switch to OAV101 (N=1)	Nusi/risd combo (N=0)	Risd mono (N=0)	Total (N=16)
Age at first dose (months)								
n	2	0	10	3	1	0	0	16
Mean	36.0		22.5	11.7	2.0			20.9
Std Dev	11.31		20.55	8.74	-			18.58
SE	8.00		6.50	5.04	-			4.64
Median	36.0		17.5	14.0	2.0			16.5
(Min, Max)	(28, 44)		(2, 65)	(2, 19)	(2, 2)			(2, 65)
95% CI of the mean	(20.3, 51.7)		(9.8, 35.2)	(1.8, 21.6)	(-, -)			(11.8, 30.0)
Age at first dose (categorical) n (%)								
< 6 months	0	0	2 (20.0)	1 (33.3)	1 (100)	0	0	4 (25.0)
=> 6 and < 12 months	0	0	3 (30.0)	0	0	0	0	3 (18.8)
=> 12 and < 24 months	0	0	0	2 (66.7)	0	0	0	2 (12.5)
=> 24 months	2 (100)	0	5 (50.0)	0	0	0	0	7 (43.8)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.txr.jp
Risdiplam Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=2)	Transient add-on (N=0)	Combo w/OAV101 (N=10)	Bridge to OAV101 (N=3)	Switch to OAV101 (N=1)	Nusi/risd combo (N=0)	Risd mono (N=0)	Total (N=16)
Duration of therapy (months)								
n	2	0	10	3	1	0	0	16
Mean	19.35		22.56	0.88	8.18			17.20
Std Dev	9.943		11.499	0.266	-			12.823
SE	7.031		3.636	0.153	-			3.206
Median	19.35		20.81	0.92	8.18			15.54
(Min, Max)	(12.3, 26.4)		(8.0, 45.2)	(0.6, 1.1)	(8.2, 8.2)			(0.6, 45.2)
95% CI of the mean	(5.57, 33.13)		(15.43, 29.69)	(0.58, 1.18)	(-, -)			(10.91, 23.48)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.txr.jp
Risdiplam Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=2)	Transient add-on (N=0)	Combo w/OAV101 (N=10)	Bridge to OAV101 (N=3)	Switch to OAV101 (N=1)	Nusi/risd combo (N=0)	Risd mono (N=0)	Total (N=16)
Has the patient received OAV101 Nusinersen or other SMA treatment and added Risdiplam? n (%)								
n	2	0	10	3	1	0	0	16
Yes	2 (100)	0	8 (80.0)	0	0	0	0	10 (62.5)
No	0	0	2 (20.0)	3 (100)	1 (100)	0	0	6 (37.5)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.txr.jp
Risdiplam Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=2)	Transient add-on (N=0)	Combo w/OAV101 (N=10)	Bridge to OAV101 (N=3)	Switch to OAV101 (N=1)	Nusi/risd combo (N=0)	Risd mono (N=0)	Total (N=16)
Primary reason for adding Risdiplam ^a ? n (%)								
n	2	0	8	0	0	0	0	10
Perceived lack of drug effect	1 (50.0)	0	3 (37.5)	0	0	0	0	4 (40.0)
Motor function	1 (50.0)	0	0	0	0	0	0	1 (10.0)
Respiratory function	0	0	1 (12.5)	0	0	0	0	1 (10.0)
Swallowing or feeding ability for age	0	0	2 (25.0)	0	0	0	0	2 (20.0)
Electrophysiological/ another biomarker response	0	0	0	0	0	0	0	0
No additional response chosen	0	0	0	0	0	0	0	0
Adverse Events	0	0	2 (25.0)	0	0	0	0	2 (20.0)
Parent/Caregiver/Patient decision	1 (50.0)	0	1 (12.5)	0	0	0	0	2 (20.0)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.txr.jp
Risdiplam Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=2)	Transient add-on (N=0)	Combo w/OAV101 (N=10)	Bridge to OAV101 (N=3)	Switch to OAV101 (N=1)	Nusi/risd combo (N=0)	Risd mono (N=0)	Total (N=16)
Alternative Treatment Available and Reimbursed	0	0	1 (12.5)	0	0	0	0	1 (10.0)
Other	0	0	1 (12.5)	0	0	0	0	1 (10.0)
Risdiplam Exposure (person-year)	3.2	-	18.8	0.2	0.7	-	-	22.9

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

^a Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.10.cpy.jpn
Glucocorticosteroid Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=40)	3 Copies (N=40)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=80)
Glucocorticosteroid medication name n (%)						
n	0	40	40	0	0	80
Prednisolone	0	40 (100)	40 (100)	0	0	80 (100)
Prednisone	0	0	0	0	0	0
Other	0	1 (2.5)	3 (7.5)	0	0	4 (5.0)
Prednisolone Dose (mg/kg)						
n	0	39	40	0	0	79
Mean		0.75	0.81			0.78
Std Dev		0.160	0.217			0.192
SE		0.026	0.034			0.022
Median		0.71	0.77			0.74
(Min, Max)		(0.6, 1.5)	(0.5, 1.5)			(0.5, 1.5)
95% CI of the mean		(0.70, 0.80)	(0.74, 0.87)			(0.74, 0.82)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
Patients may have more than one type of glucocorticosteroid.

Table 14.1.10.cpy.jp
Glucocorticosteroid Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=40)	3 Copies (N=40)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=80)
Duration of Prednisolone (months)						
n	0	39	40	0	0	79
Mean		2.60	3.26			2.93
Std Dev		0.780	1.393			1.173
SE		0.125	0.220			0.132
Median		2.37	3.12			2.69
(Min, Max)		(1.7, 4.6)	(1.0, 7.5)			(1.0, 7.5)
95% CI of the mean		(2.35, 2.84)	(2.83, 3.69)			(2.67, 3.19)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
Patients may have more than one type of glucocorticosteroid.

Table 14.3.1.0.oav.age3.jpn
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Treatment Emergent Adverse Events						
≥1 TEAE (any grade)	14 (100)	9 (100)	22 (100)	31 (100)	4 (100)	80 (100)
Grade ≥3 AE	7 (50.0)	8 (88.9)	19 (86.4)	27 (87.1)	4 (100)	65 (81.3)
Any Serious AE	9 (64.3)	8 (88.9)	15 (68.2)	21 (67.7)	2 (50.0)	55 (68.8)
Related AE	13 (92.9)	9 (100)	22 (100)	31 (100)	4 (100)	79 (98.8)
OAV101	13 (92.9)	9 (100)	22 (100)	31 (100)	4 (100)	79 (98.8)
NUSI	0	1 (11.1)	0	0	0	1 (1.3)
RISD	0	1 (11.1)	0	0	0	1 (1.3)
Serious Related AE	2 (14.3)	2 (22.2)	5 (22.7)	12 (38.7)	0	21 (26.3)
OAV101	2 (14.3)	2 (22.2)	5 (22.7)	12 (38.7)	0	21 (26.3)
NUSI	0	0	0	0	0	0
RISD	0	0	0	0	0	0

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Table 14.3.1.0.oav.age3.jpj
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
AEs of Special Interest:						
Any AEs of Special Interest	11 (78.6)	8 (88.9)	20 (90.9)	31 (100)	4 (100)	74 (92.5)
Hepatotoxicity	11 (78.6)	8 (88.9)	19 (86.4)	30 (96.8)	4 (100)	72 (90.0)
Transient Thrombocytopenia	3 (21.4)	6 (66.7)	12 (54.5)	25 (80.6)	4 (100)	50 (62.5)
Thrombotic microangiopathy	0	0	0	4 (12.9)	0	4 (5.0)
Cardiac Adverse Events	6 (42.9)	4 (44.4)	6 (27.3)	8 (25.8)	3 (75.0)	27 (33.8)
Sensory Abnormalities Suggestive of Ganglionopathy	0	0	0	0	0	0
New malignancies	0	0	0	0	0	0
New incidence of neurological disorders	1 (7.1)	0	1 (4.5)	1 (3.2)	0	3 (3.8)
New incidence of autoimmune disorders	0	0	0	1 (3.2)	0	1 (1.3)
New incidence of hematological disorders	0	1 (11.1)	2 (9.1)	9 (29.0)	1 (25.0)	13 (16.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Data as of 23MAY2025: ADAE

Prg: TEAE.SAS

Table 14.3.1.0.oav.age3.tmin.jp
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
≥1 TEAE (any grade), n (%)	14 (100)	9 (100)	22 (100)	31 (100)	4 (100)	80 (100)
≤2 weeks	13 (92.9)	8 (88.9)	22 (100)	30 (96.8)	4 (100)	77 (96.3)
>2 weeks and ≤4 weeks	2 (14.3)	3 (33.3)	8 (36.4)	9 (29.0)	1 (25.0)	23 (28.8)
>4 weeks and ≤3 months	6 (42.9)	3 (33.3)	9 (40.9)	11 (35.5)	3 (75.0)	32 (40.0)
>3 months and ≤6 months	2 (14.3)	4 (44.4)	5 (22.7)	10 (32.3)	0	21 (26.3)
>6 months and ≤12 months	5 (35.7)	3 (33.3)	9 (40.9)	11 (35.5)	0	28 (35.0)
>12 months and ≤24 months	6 (42.9)	5 (55.6)	7 (31.8)	8 (25.8)	0	26 (32.5)
>24 months	2 (14.3)	6 (66.7)	2 (10.0)	7 (23.3)	2 (50.0)	19 (24.7)

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jp
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
Grade ≥3 AE, n (%)	7 (50.0)	8 (88.9)	19 (86.4)	27 (87.1)	4 (100)	65 (81.3)
≤2 weeks	2 (14.3)	5 (55.6)	14 (63.6)	24 (77.4)	3 (75.0)	48 (60.0)
>2 weeks and ≤4 weeks	1 (7.1)	1 (11.1)	2 (9.1)	3 (9.7)	0	7 (8.8)
>4 weeks and ≤3 months	0	1 (11.1)	3 (13.6)	5 (16.1)	2 (50.0)	11 (13.8)
>3 months and ≤6 months	1 (7.1)	2 (22.2)	4 (18.2)	4 (12.9)	0	11 (13.8)
>6 months and ≤12 months	4 (28.6)	2 (22.2)	5 (22.7)	6 (19.4)	0	17 (21.3)
>12 months and ≤24 months	4 (28.6)	4 (44.4)	5 (22.7)	6 (19.4)	0	19 (23.8)
>24 months	2 (14.3)	6 (66.7)	2 (10.0)	7 (23.3)	2 (50.0)	19 (24.7)

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jp
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
Any Serious AE, n (%)	9 (64.3)	8 (88.9)	15 (68.2)	21 (67.7)	2 (50.0)	55 (68.8)
≤2 weeks	2 (14.3)	3 (33.3)	5 (22.7)	13 (41.9)	0	23 (28.8)
>2 weeks and ≤4 weeks	1 (7.1)	1 (11.1)	0	1 (3.2)	0	3 (3.8)
>4 weeks and ≤3 months	1 (7.1)	1 (11.1)	4 (18.2)	3 (9.7)	0	9 (11.3)
>3 months and ≤6 months	1 (7.1)	1 (11.1)	4 (18.2)	4 (12.9)	0	10 (12.5)
>6 months and ≤12 months	4 (28.6)	2 (22.2)	6 (27.3)	8 (25.8)	0	20 (25.0)
>12 months and ≤24 months	5 (35.7)	4 (44.4)	5 (22.7)	8 (25.8)	0	22 (27.5)
>24 months	2 (14.3)	6 (66.7)	2 (10.0)	7 (23.3)	2 (50.0)	19 (24.7)

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jp
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
Related AE, n (%)	13 (92.9)	9 (100)	22 (100)	31 (100)	4 (100)	79 (98.8)
≤2 weeks	13 (92.9)	8 (88.9)	22 (100)	30 (96.8)	4 (100)	77 (96.3)
>2 weeks and ≤4 weeks	1 (7.1)	1 (11.1)	6 (27.3)	8 (25.8)	1 (25.0)	17 (21.3)
>4 weeks and ≤3 months	3 (21.4)	2 (22.2)	3 (13.6)	7 (22.6)	3 (75.0)	18 (22.5)
>3 months and ≤6 months	1 (7.1)	1 (11.1)	0	2 (6.5)	0	4 (5.0)
>6 months and ≤12 months	1 (7.1)	0	0	1 (3.2)	0	2 (2.5)
>12 months and ≤24 months	0	0	0	0	0	0
>24 months	0	1 (11.1)	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jp
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
OAV101, n (%)	13 (92.9)	9 (100)	22 (100)	31 (100)	4 (100)	79 (98.8)
≤2 weeks	13 (92.9)	8 (88.9)	22 (100)	30 (96.8)	4 (100)	77 (96.3)
>2 weeks and ≤4 weeks	1 (7.1)	1 (11.1)	6 (27.3)	8 (25.8)	1 (25.0)	17 (21.3)
>4 weeks and ≤3 months	3 (21.4)	2 (22.2)	3 (13.6)	7 (22.6)	3 (75.0)	18 (22.5)
>3 months and ≤6 months	1 (7.1)	1 (11.1)	0	2 (6.5)	0	4 (5.0)
>6 months and ≤12 months	1 (7.1)	0	0	1 (3.2)	0	2 (2.5)
>12 months and ≤24 months	0	0	0	0	0	0
>24 months	0	1 (11.1)	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jp
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
NUSI, n (%)	5 (83.3)	6 (100)	15 (100)	17 (100)	3 (100)	46 (97.9)
≤2 weeks	5 (100)	6 (100)	15 (100)	16 (94.1)	3 (100)	45 (97.8)
>2 weeks and ≤4 weeks	0	1 (16.7)	3 (20.0)	4 (23.5)	0	8 (17.4)
>4 weeks and ≤3 months	1 (20.0)	1 (16.7)	2 (13.3)	5 (29.4)	3 (100)	12 (26.1)
>3 months and ≤6 months	0	1 (16.7)	0	1 (5.9)	0	2 (4.3)
>6 months and ≤12 months	1 (20.0)	0	0	1 (5.9)	0	2 (4.3)
>12 months and ≤24 months	0	0	0	0	0	0
>24 months	0	1 (16.7)	0	0	0	1 (2.2)

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jpn
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
RISD, n (%)	0	1 (20.0)	0	0	0	1 (6.3)
≤2 weeks	0	0	0	0	0	0
>2 weeks and ≤4 weeks	0	0	0	0	0	0
>4 weeks and ≤3 months	0	0	0	0	0	0
>3 months and ≤6 months	0	0	0	0	0	0
>6 months and ≤12 months	0	0	0	0	0	0
>12 months and ≤24 months	0	0	0	0	0	0
>24 months	0	1 (20.0)	0	0	0	1 (8.3)

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jp
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
Serious Related AE, n (%)	2 (14.3)	2 (22.2)	5 (22.7)	12 (38.7)	0	21 (26.3)
≤2 weeks	2 (14.3)	2 (22.2)	4 (18.2)	12 (38.7)	0	20 (25.0)
>2 weeks and ≤4 weeks	0	0	0	1 (3.2)	0	1 (1.3)
>4 weeks and ≤3 months	0	0	1 (4.5)	0	0	1 (1.3)
>3 months and ≤6 months	0	0	0	0	0	0
>6 months and ≤12 months	0	0	0	0	0	0
>12 months and ≤24 months	0	0	0	0	0	0
>24 months	0	0	0	0	0	0

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jp
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
OAV101, n (%)	2 (14.3)	2 (22.2)	5 (22.7)	12 (38.7)	0	21 (26.3)
≤2 weeks	2 (14.3)	2 (22.2)	4 (18.2)	12 (38.7)	0	20 (25.0)
>2 weeks and ≤4 weeks	0	0	0	1 (3.2)	0	1 (1.3)
>4 weeks and ≤3 months	0	0	1 (4.5)	0	0	1 (1.3)
>3 months and ≤6 months	0	0	0	0	0	0
>6 months and ≤12 months	0	0	0	0	0	0
>12 months and ≤24 months	0	0	0	0	0	0
>24 months	0	0	0	0	0	0

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jp
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
NUSI, n (%)	0	2 (33.3)	4 (26.7)	6 (35.3)	0	12 (25.5)
≤2 weeks	0	2 (33.3)	4 (26.7)	6 (35.3)	0	12 (26.1)
>2 weeks and ≤4 weeks	0	0	0	1 (5.9)	0	1 (2.2)
>4 weeks and ≤3 months	0	0	0	0	0	0
>3 months and ≤6 months	0	0	0	0	0	0
>6 months and ≤12 months	0	0	0	0	0	0
>12 months and ≤24 months	0	0	0	0	0	0
>24 months	0	0	0	0	0	0

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jpn
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
RISD, n (%)	0	0	0	0	0	0

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jp
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
Any AEs of Special Interest, n (%)	11 (78.6)	8 (88.9)	20 (90.9)	31 (100)	4 (100)	74 (92.5)
≤2 weeks	11 (78.6)	7 (77.8)	19 (86.4)	30 (96.8)	4 (100)	71 (88.8)
>2 weeks and ≤4 weeks	1 (7.1)	1 (11.1)	5 (22.7)	6 (19.4)	1 (25.0)	14 (17.5)
>4 weeks and ≤3 months	4 (28.6)	2 (22.2)	3 (13.6)	8 (25.8)	3 (75.0)	20 (25.0)
>3 months and ≤6 months	1 (7.1)	1 (11.1)	0	1 (3.2)	0	3 (3.8)
>6 months and ≤12 months	1 (7.1)	0	1 (4.5)	2 (6.5)	0	4 (5.0)
>12 months and ≤24 months	2 (14.3)	1 (11.1)	1 (4.5)	0	0	4 (5.0)
>24 months	0	0	0	0	1 (25.0)	1 (1.3)

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jp
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
Hepatotoxicity, n (%)	11 (78.6)	8 (88.9)	19 (86.4)	30 (96.8)	4 (100)	72 (90.0)
≤2 weeks	10 (71.4)	7 (77.8)	18 (81.8)	29 (93.5)	4 (100)	68 (85.0)
>2 weeks and ≤4 weeks	1 (7.1)	1 (11.1)	3 (13.6)	3 (9.7)	0	8 (10.0)
>4 weeks and ≤3 months	4 (28.6)	1 (11.1)	3 (13.6)	7 (22.6)	3 (75.0)	18 (22.5)
>3 months and ≤6 months	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
>6 months and ≤12 months	0	0	0	2 (6.5)	0	2 (2.5)
>12 months and ≤24 months	1 (7.1)	0	0	0	0	1 (1.3)
>24 months	0	0	0	0	0	0

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jp
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
Transient Thrombocytopenia, n (%)	3 (21.4)	6 (66.7)	12 (54.5)	25 (80.6)	4 (100)	50 (62.5)
≤2 weeks	3 (21.4)	6 (66.7)	12 (54.5)	25 (80.6)	4 (100)	50 (62.5)
>2 weeks and ≤4 weeks	0	0	0	1 (3.2)	0	1 (1.3)
>4 weeks and ≤3 months	0	0	0	0	1 (25.0)	1 (1.3)
>3 months and ≤6 months	0	0	0	0	0	0
>6 months and ≤12 months	0	0	0	0	0	0
>12 months and ≤24 months	0	0	0	0	0	0
>24 months	0	0	0	0	0	0

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jp
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
Thrombotic microangiopathy, n (%)	0	0	0	4 (12.9)	0	4 (5.0)
≤2 weeks	0	0	0	4 (12.9)	0	4 (5.0)
>2 weeks and ≤4 weeks	0	0	0	0	0	0
>4 weeks and ≤3 months	0	0	0	0	0	0
>3 months and ≤6 months	0	0	0	0	0	0
>6 months and ≤12 months	0	0	0	0	0	0
>12 months and ≤24 months	0	0	0	0	0	0
>24 months	0	0	0	0	0	0

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jp
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
Cardiac Adverse Events, n (%)	6 (42.9)	4 (44.4)	6 (27.3)	8 (25.8)	3 (75.0)	27 (33.8)
≤2 weeks	6 (42.9)	4 (44.4)	3 (13.6)	6 (19.4)	2 (50.0)	21 (26.3)
>2 weeks and ≤4 weeks	0	0	2 (9.1)	2 (6.5)	1 (25.0)	5 (6.3)
>4 weeks and ≤3 months	0	1 (11.1)	0	1 (3.2)	0	2 (2.5)
>3 months and ≤6 months	0	0	0	1 (3.2)	0	1 (1.3)
>6 months and ≤12 months	0	0	1 (4.5)	0	0	1 (1.3)
>12 months and ≤24 months	1 (7.1)	0	1 (4.5)	0	0	2 (2.5)
>24 months	0	0	0	0	0	0

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jpn
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
Sensory Abnormalities Suggestive of Ganglionopathy, n (%)	0	0	0	0	0	0

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jp
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
New malignancies, n (%)	0	0	0	0	0	0

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jp
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
New incidence of neurological disorders, n (%)	1 (7.1)	0	1 (4.5)	1 (3.2)	0	3 (3.8)
≤2 weeks	0	0	0	0	0	0
>2 weeks and ≤4 weeks	0	0	0	1 (3.2)	0	1 (1.3)
>4 weeks and ≤3 months	0	0	0	0	0	0
>3 months and ≤6 months	0	0	0	0	0	0
>6 months and ≤12 months	1 (7.1)	0	1 (4.5)	0	0	2 (2.5)
>12 months and ≤24 months	0	0	0	0	0	0
>24 months	0	0	0	0	0	0

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jp
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
New incidence of autoimmune disorders, n (%)	0	0	0	1 (3.2)	0	1 (1.3)
≤2 weeks	0	0	0	1 (3.2)	0	1 (1.3)
>2 weeks and ≤4 weeks	0	0	0	0	0	0
>4 weeks and ≤3 months	0	0	0	0	0	0
>3 months and ≤6 months	0	0	0	0	0	0
>6 months and ≤12 months	0	0	0	0	0	0
>12 months and ≤24 months	0	0	0	0	0	0
>24 months	0	0	0	0	0	0

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.0.oav.age3.tmin.jp
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion and Time from OAV101 Infusion
Japan OAV101 Treated Patients

	Age at OAV101 Infusion					Total (N=80)
	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	
New incidence of hematological disorders, n (%)	0	1 (11.1)	2 (9.1)	9 (29.0)	1 (25.0)	13 (16.3)
≤2 weeks	0	1 (11.1)	2 (9.1)	9 (29.0)	1 (25.0)	13 (16.3)
>2 weeks and ≤4 weeks	0	0	0	0	0	0
>4 weeks and ≤3 months	0	0	0	0	0	0
>3 months and ≤6 months	0	0	0	0	0	0
>6 months and ≤12 months	0	0	0	0	0	0
>12 months and ≤24 months	0	1 (11.1)	0	0	0	1 (1.3)
>24 months	0	0	0	0	1 (25.0)	1 (1.3)

Adverse Events were coded using MedDRA, version 28.0.

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Note: For each treatment-related adverse event category (e.g OAV101-related AE, NUSI-related AE, RISD-related AE), percentages are calculated using as the denominator only the number of patients within each age subgroup who received the respective treatment. This ensures that percentages reflect the risk among patients actually exposed to the treatment.

For by-time interval rows, percentages are based on the number of patients who were exposed to the treatment (e.g., Nusinersen, Risdiplam, OAV101) on or before the end of the interval and who remained under observation at the start of that interval. Patients who died or were censored (due to end of study or data cut-off) prior to entering an interval are excluded from that interval's denominator. For treatment-related sections (e.g., Nusinersen, Risdiplam), the "Any [Treatment]" row includes all patients who appear in one or more by-time interval rows.

Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=78)	> 24 months (N=70)	Total (N=80)
Any Adverse Event	77 (96.3)	19 (23.8)	22 (27.5)	19 (23.8)	26 (32.9)	22 (28.2)	19 (27.1)	80 (100)
Blood and lymphatic system disorders	13 (16.3)	0	0	0	0	1 (1.3)	1 (1.4)	13 (16.3)
Anaemia	0	0	0	0	0	1 (1.3)	0	1 (1.3)
Eosinophilia	0	0	0	0	0	0	1 (1.4)	1 (1.3)
Haemolytic anaemia	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Thrombocytopenia	9 (11.3)	0	0	0	0	0	0	9 (11.3)
Thrombotic microangiopathy	4 (5.0)	0	0	0	0	0	0	4 (5.0)
Cardiac disorders	1 (1.3)	2 (2.5)	0	0	1 (1.3)	0	0	4 (5.0)
Arrhythmia	0	1 (1.3)	0	0	0	0	0	1 (1.3)
Bradycardia	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Cardiac failure congestive	0	1 (1.3)	0	0	0	0	0	1 (1.3)
Cardio-respiratory arrest	0	0	0	0	1 (1.3)	0	0	1 (1.3)

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Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=78)	> 24 months (N=70)	Total (N=80)
Congenital, familial and genetic disorders	0	0	0	0	3 (3.8)	1 (1.3)	1 (1.4)	5 (6.3)
Cryptorchism	0	0	0	0	3 (3.8)	0	1 (1.4)	4 (5.0)
Developmental hip dysplasia	0	0	0	0	0	1 (1.3)	0	1 (1.3)
Endocrine disorders	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Cushingoid	0	0	1 (1.3)	0	0	0	0	1 (1.3)

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Table 14.3.1.1.2.oav.aeptin.jpj
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=78)	> 24 months (N=70)	Total (N=80)
Gastrointestinal disorders	33 (41.3)	2 (2.5)	2 (2.5)	1 (1.3)	1 (1.3)	3 (3.8)	5 (7.1)	40 (50.0)
Acetonaemic vomiting	0	0	0	0	0	0	1 (1.4)	1 (1.3)
Constipation	0	0	1 (1.3)	0	0	0	1 (1.4)	2 (2.5)
Diarrhoea	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Dysphagia	0	1 (1.3)	0	0	1 (1.3)	1 (1.3)	0	3 (3.8)
Gastric fistula	0	0	0	1 (1.3)	0	2 (2.6)	3 (4.3)	6 (7.5)
Gastric haemorrhage	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Gastrooesophageal reflux disease	0	0	0	0	0	0	1 (1.4)	1 (1.3)
Nausea	5 (6.3)	0	0	0	0	0	0	5 (6.3)
Vomiting	30 (37.5)	1 (1.3)	0	0	0	0	1 (1.4)	32 (40.0)

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OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=78)	> 24 months (N=70)	Total (N=80)
General disorders and administration site conditions	64 (80.0)	1 (1.3)	2 (2.5)	1 (1.3)	0	0	0	65 (81.3)
Asthenia	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Drug withdrawal syndrome	0	1 (1.3)	0	0	0	0	0	1 (1.3)
Generalised oedema	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Inflammation	0	0	0	1 (1.3)	0	0	0	1 (1.3)
Malaise	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Medical device site haemorrhage	0	1 (1.3)	0	0	0	0	0	1 (1.3)
Multiple organ dysfunction syndrome	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Pyrexia	64 (80.0)	0	1 (1.3)	0	0	0	0	65 (81.3)
Hepatobiliary disorders	9 (11.3)	0	3 (3.8)	0	0	1 (1.3)	0	12 (15.0)
Acute hepatic failure	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Cholelithiasis	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Hepatic function abnormal	9 (11.3)	0	2 (2.5)	0	0	1 (1.3)	0	12 (15.0)
Hepatomegaly	1 (1.3)	0	0	0	0	0	0	1 (1.3)

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Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC								
Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=78)	> 24 months (N=70)	Total (N=80)
Immune system disorders	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Hypersensitivity	1 (1.3)	0	0	0	0	0	0	1 (1.3)

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Data as of 23MAY2025: ADAE

Prg: TEA1ST.SAS

Table 14.3.1.1.2.oav.aeptin.jp
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=78)	> 24 months (N=70)	Total (N=80)
Infections and infestations	3 (3.8)	3 (3.8)	10 (12.5)	12 (15.0)	17 (21.5)	15 (19.2)	14 (20.0)	44 (55.0)
Bronchiolitis	0	0	0	0	0	1 (1.3)	1 (1.4)	2 (2.5)
Bronchitis	0	1 (1.3)	0	2 (2.5)	1 (1.3)	4 (5.1)	0	8 (10.0)
Bronchitis viral	0	0	1 (1.3)	0	0	0	2 (2.9)	3 (3.8)
COVID-19	0	0	1 (1.3)	0	3 (3.8)	1 (1.3)	3 (4.3)	8 (10.0)
Epstein-Barr virus infection	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Gastroenteritis	0	0	0	0	0	1 (1.3)	2 (2.9)	3 (3.8)
Gastroenteritis norovirus	0	0	0	0	0	1 (1.3)	0	1 (1.3)
Lower respiratory tract infection	0	0	0	0	0	0	1 (1.4)	1 (1.3)
Metapneumovirus bronchiolitis	0	0	0	0	1 (1.3)	0	0	1 (1.3)
Metapneumovirus infection	0	0	0	0	1 (1.3)	0	1 (1.4)	2 (2.5)
Metapneumovirus pneumonia	0	0	0	0	0	1 (1.3)	0	1 (1.3)
Nasopharyngitis	0	0	1 (1.3)	1 (1.3)	0	0	0	2 (2.5)
Norovirus infection	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Parainfluenzae virus infection	0	0	0	1 (1.3)	0	0	0	1 (1.3)
Pharyngitis	0	0	0	0	0	0	1 (1.4)	1 (1.3)
Pneumonia	0	0	0	0	2 (2.5)	5 (6.4)	6 (8.6)	13 (16.3)

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Table 14.3.1.1.2.oav.aeptin.jpj
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=78)	> 24 months (N=70)	Total (N=80)
Pneumonia aspiration	2 (2.5)	1 (1.3)	0	3 (3.8)	0	4 (5.1)	2 (2.9)	12 (15.0)
Pneumonia haemophilus	0	0	0	0	1 (1.3)	0	0	1 (1.3)
Pneumonia influenzal	0	0	0	0	0	0	1 (1.4)	1 (1.3)
Pneumonia parainfluenzae viral	0	0	0	0	1 (1.3)	0	0	1 (1.3)
Pneumonia respiratory syncytial viral	0	0	2 (2.5)	2 (2.5)	2 (2.5)	0	0	6 (7.5)
Pneumonia viral	0	0	0	0	0	0	2 (2.9)	2 (2.5)
Respiratory syncytial virus bronchiolitis	0	0	0	0	1 (1.3)	2 (2.6)	0	3 (3.8)
Respiratory syncytial virus infection	0	0	1 (1.3)	0	2 (2.5)	2 (2.6)	0	5 (6.3)
Respiratory tract infection	1 (1.3)	0	0	0	0	0	1 (1.4)	2 (2.5)
Rhinovirus infection	0	0	1 (1.3)	1 (1.3)	0	0	0	2 (2.5)
Serratia infection	0	1 (1.3)	0	0	0	0	0	1 (1.3)
Tonsillitis	0	0	0	1 (1.3)	0	0	0	1 (1.3)
Upper respiratory tract infection	0	0	2 (2.5)	3 (3.8)	5 (6.3)	1 (1.3)	2 (2.9)	13 (16.3)
Viral infection	0	0	0	0	0	1 (1.3)	1 (1.4)	2 (2.5)
Viral upper respiratory tract infection	0	0	0	0	2 (2.5)	0	0	2 (2.5)

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OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=78)	> 24 months (N=70)	Total (N=80)
Injury, poisoning and procedural complications	0	0	0	1 (1.3)	0	3 (3.8)	0	4 (5.0)
Femur fracture	0	0	0	1 (1.3)	0	1 (1.3)	0	2 (2.5)
Joint dislocation	0	0	0	0	0	2 (2.6)	0	2 (2.5)

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Japan OAV101 Treated Patients

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Investigations	72 (90.0)	10 (12.5)	6 (7.5)	2 (2.5)	2 (2.5)	0	0	72 (90.0)
Alanine aminotransferase increased	46 (57.5)	2 (2.5)	4 (5.0)	0	0	0	0	52 (65.0)
Amylase increased	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Aspartate aminotransferase increased	51 (63.8)	1 (1.3)	1 (1.3)	0	0	0	0	53 (66.3)
Blood creatine phosphokinase MB increased	0	1 (1.3)	0	0	0	0	0	1 (1.3)
Blood creatine phosphokinase increased	6 (7.5)	0	1 (1.3)	1 (1.3)	0	0	0	8 (10.0)
Blood creatinine increased	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Blood lactate dehydrogenase increased	21 (26.3)	0	0	0	0	0	0	21 (26.3)
Blood pressure increased	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Blood urea increased	1 (1.3)	0	0	0	0	0	0	1 (1.3)
C-reactive protein increased	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Gamma-glutamyltransferase increased	0	3 (3.8)	0	0	0	0	0	3 (3.8)
Haemoglobin decreased	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Haptoglobin decreased	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Hepatic enzyme increased	7 (8.8)	0	0	1 (1.3)	0	0	0	8 (10.0)

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Intraocular pressure increased	0	1 (1.3)	0	0	0	0	0	1 (1.3)
N-terminal prohormone brain natriuretic peptide increased	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Neutrophil count decreased	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Platelet count decreased	40 (50.0)	0	0	0	0	0	0	40 (50.0)
Serum ferritin increased	15 (18.8)	0	0	0	0	0	0	15 (18.8)
Troponin I increased	12 (15.0)	2 (2.5)	0	0	0	0	0	14 (17.5)
Troponin T increased	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Urine output decreased	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Weight decreased	3 (3.8)	1 (1.3)	0	0	2 (2.5)	0	0	6 (7.5)
Weight increased	1 (1.3)	0	0	0	0	0	0	1 (1.3)
White blood cell count decreased	7 (8.8)	0	0	0	0	0	0	7 (8.8)

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Japan OAV101 Treated Patients

SOC								
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Metabolism and nutrition disorders	18 (22.5)	0	1 (1.3)	1 (1.3)	1 (1.3)	1 (1.3)	0	21 (26.3)
Decreased appetite	14 (17.5)	0	0	0	0	0	0	14 (17.5)
Feeding disorder	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Hypercholesterolaemia	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Hypertriglyceridaemia	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Hypoglycaemia	0	0	0	0	0	1 (1.3)	0	1 (1.3)
Hypophagia	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Metabolic acidosis	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Underweight	0	0	0	0	1 (1.3)	0	0	1 (1.3)
Weight gain poor	2 (2.5)	0	0	1 (1.3)	0	0	0	3 (3.8)

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Japan OAV101 Treated Patients

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Musculoskeletal and connective tissue disorders	0	0	1 (1.3)	4 (5.0)	6 (7.6)	1 (1.3)	1 (1.4)	12 (15.0)
Joint contracture	0	0	0	1 (1.3)	0	0	0	1 (1.3)
Joint range of motion decreased	0	0	0	0	1 (1.3)	0	0	1 (1.3)
Kyphosis	0	0	0	0	1 (1.3)	0	0	1 (1.3)
Muscle atrophy	0	0	0	1 (1.3)	0	0	0	1 (1.3)
Scoliosis	0	0	1 (1.3)	2 (2.5)	5 (6.3)	1 (1.3)	1 (1.4)	10 (12.5)
Nervous system disorders	0	1 (1.3)	0	0	2 (2.5)	0	0	3 (3.8)
Cerebral atrophy	0	1 (1.3)	0	0	0	0	0	1 (1.3)
Motor developmental delay	0	0	0	0	1 (1.3)	0	0	1 (1.3)
Seizure	0	0	0	0	1 (1.3)	0	0	1 (1.3)

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Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=78)	> 24 months (N=70)	Total (N=80)
Renal and urinary disorders	3 (3.8)	0	1 (1.3)	1 (1.3)	0	0	0	4 (5.0)
Acute kidney injury	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Chronic kidney disease	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Glycosuria	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Haematuria	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Proteinuria	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Renal impairment	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Renal injury	0	0	0	1 (1.3)	0	0	0	1 (1.3)

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OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=78)	> 24 months (N=70)	Total (N=80)
Respiratory, thoracic and mediastinal disorders	0	2 (2.5)	3 (3.8)	2 (2.5)	2 (2.5)	4 (5.1)	5 (7.1)	13 (16.3)
Acute respiratory failure	0	0	0	0	1 (1.3)	3 (3.8)	0	4 (5.0)
Apnoea	0	1 (1.3)	0	0	0	0	0	1 (1.3)
Aspiration	0	0	0	0	0	0	1 (1.4)	1 (1.3)
Atelectasis	0	0	0	2 (2.5)	0	1 (1.3)	1 (1.4)	4 (5.0)
Hypoxia	0	0	0	0	0	1 (1.3)	0	1 (1.3)
Nocturnal dyspnoea	0	0	0	0	0	1 (1.3)	0	1 (1.3)
Pleural effusion	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Respiration abnormal	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Respiratory disorder	0	0	1 (1.3)	0	0	0	1 (1.4)	2 (2.5)
Respiratory failure	0	0	0	0	1 (1.3)	0	1 (1.4)	2 (2.5)
Sleep apnoea syndrome	0	1 (1.3)	0	0	0	0	0	1 (1.3)
Upper respiratory tract inflammation	0	0	1 (1.3)	0	0	0	1 (1.4)	2 (2.5)

Adverse Events were coded using MedDRA, version &medraver..

Percentages are based on the number of patients who had at least one Adverse Event Form completed and with exposure indicated in column header.

Patients are only counted once at each level of summarization.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=78)	> 24 months (N=70)	Total (N=80)
Skin and subcutaneous tissue disorders	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Urticaria	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Vascular disorders	2 (2.5)	0	0	0	1 (1.3)	0	3 (4.3)	6 (7.5)
Hypertension	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Kawasaki's disease	0	0	0	0	1 (1.3)	0	3 (4.3)	4 (5.0)
Not Coded	0	0	0	0	0	1 (1.3)	0	1 (1.3)
Gastrostomy	0	0	0	0	0	1 (1.3)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Percentages are based on the number of patients who had at least one Adverse Event Form completed and with exposure indicated in column header.

Patients are only counted once at each level of summarization.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Any Adverse Event	14 (100)	9 (100)	22 (100)	31 (100)	4 (100)	80 (100)
Blood and lymphatic system disorders	0	1 (11.1)	2 (9.1)	9 (29.0)	1 (25.0)	13 (16.3)
Thrombocytopenia	0	1 (11.1)	2 (9.1)	5 (16.1)	1 (25.0)	9 (11.3)
Thrombotic microangiopathy	0	0	0	4 (12.9)	0	4 (5.0)
Anaemia	0	1 (11.1)	0	0	0	1 (1.3)
Eosinophilia	0	0	0	0	1 (25.0)	1 (1.3)
Haemolytic anaemia	0	0	0	1 (3.2)	0	1 (1.3)
Cardiac disorders	0	0	2 (9.1)	2 (6.5)	0	4 (5.0)
Arrhythmia	0	0	0	1 (3.2)	0	1 (1.3)
Bradycardia	0	0	1 (4.5)	0	0	1 (1.3)
Cardiac failure congestive	0	0	0	1 (3.2)	0	1 (1.3)
Cardio-respiratory arrest	0	0	1 (4.5)	0	0	1 (1.3)
Congenital, familial and genetic disorders	2 (14.3)	0	3 (13.6)	0	0	5 (6.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jp
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Cryptorchism	2 (14.3)	0	2 (9.1)	0	0	4 (5.0)
Developmental hip dysplasia	0	0	1 (4.5)	0	0	1 (1.3)
Endocrine disorders	0	0	1 (4.5)	0	0	1 (1.3)
Cushingoid	0	0	1 (4.5)	0	0	1 (1.3)
Gastrointestinal disorders	4 (28.6)	6 (66.7)	14 (63.6)	13 (41.9)	3 (75.0)	40 (50.0)
Vomiting	4 (28.6)	5 (55.6)	10 (45.5)	11 (35.5)	2 (50.0)	32 (40.0)
Gastric fistula	1 (7.1)	2 (22.2)	3 (13.6)	0	0	6 (7.5)
Nausea	0	0	1 (4.5)	3 (9.7)	1 (25.0)	5 (6.3)
Dysphagia	1 (7.1)	1 (11.1)	1 (4.5)	0	0	3 (3.8)
Constipation	1 (7.1)	0	1 (4.5)	0	0	2 (2.5)
Diarrhoea	1 (7.1)	0	1 (4.5)	0	0	2 (2.5)
Acetonaemic vomiting	0	0	1 (4.5)	0	0	1 (1.3)
Gastric haemorrhage	0	0	0	0	1 (25.0)	1 (1.3)
Gastroesophageal reflux disease	1 (7.1)	0	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC	< 3 months	≥ 3 and < 6 months	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Preferred Term	(N=14)	(N=9)	months	months	(N=4)	(N=80)
			(N=22)	(N=31)		
General disorders and administration site conditions	9 (64.3)	7 (77.8)	20 (90.9)	25 (80.6)	4 (100)	65 (81.3)
Pyrexia	9 (64.3)	7 (77.8)	20 (90.9)	25 (80.6)	4 (100)	65 (81.3)
Asthenia	0	1 (11.1)	0	1 (3.2)	0	2 (2.5)
Malaise	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Drug withdrawal syndrome	0	0	0	1 (3.2)	0	1 (1.3)
Generalised oedema	0	0	0	1 (3.2)	0	1 (1.3)
Inflammation	0	0	0	1 (3.2)	0	1 (1.3)
Medical device site haemorrhage	0	0	0	1 (3.2)	0	1 (1.3)
Multiple organ dysfunction syndrome	0	0	1 (4.5)	0	0	1 (1.3)
Hepatobiliary disorders	3 (21.4)	2 (22.2)	4 (18.2)	2 (6.5)	1 (25.0)	12 (15.0)
Hepatic function abnormal	3 (21.4)	2 (22.2)	4 (18.2)	2 (6.5)	1 (25.0)	12 (15.0)
Acute hepatic failure	0	0	1 (4.5)	0	0	1 (1.3)
Cholelithiasis	0	0	0	1 (3.2)	0	1 (1.3)
Hepatomegaly	0	0	0	0	1 (25.0)	1 (1.3)
Immune system disorders	0	0	0	1 (3.2)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpj
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Hypersensitivity	0	0	0	1 (3.2)	0	1 (1.3)
Infections and infestations	5 (35.7)	8 (88.9)	11 (50.0)	19 (61.3)	1 (25.0)	44 (55.0)
Pneumonia	2 (14.3)	4 (44.4)	1 (4.5)	6 (19.4)	0	13 (16.3)
Upper respiratory tract infection	3 (21.4)	2 (22.2)	3 (13.6)	5 (16.1)	0	13 (16.3)
Pneumonia aspiration	2 (14.3)	2 (22.2)	2 (9.1)	6 (19.4)	0	12 (15.0)
Bronchitis	0	1 (11.1)	4 (18.2)	3 (9.7)	0	8 (10.0)
COVID-19	2 (14.3)	2 (22.2)	3 (13.6)	1 (3.2)	0	8 (10.0)
Pneumonia respiratory syncytial viral	1 (7.1)	1 (11.1)	1 (4.5)	3 (9.7)	0	6 (7.5)
Respiratory syncytial virus infection	1 (7.1)	1 (11.1)	0	3 (9.7)	0	5 (6.3)
Bronchitis viral	0	0	1 (4.5)	2 (6.5)	0	3 (3.8)
Gastroenteritis	1 (7.1)	0	0	2 (6.5)	0	3 (3.8)
Respiratory syncytial virus bronchiolitis	0	0	2 (9.1)	1 (3.2)	0	3 (3.8)
Bronchiolitis	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Metapneumovirus infection	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)
Nasopharyngitis	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC	< 3 months	≥ 3 and < 6 months	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Preferred Term	(N=14)	(N=9)	months	months	(N=4)	(N=80)
			(N=22)	(N=31)		
Pneumonia viral	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)
Respiratory tract infection	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Rhinovirus infection	0	0	2 (9.1)	0	0	2 (2.5)
Viral infection	0	1 (11.1)	0	0	1 (25.0)	2 (2.5)
Viral upper respiratory tract infection	0	1 (11.1)	0	1 (3.2)	0	2 (2.5)
Epstein-Barr virus infection	0	0	0	1 (3.2)	0	1 (1.3)
Gastroenteritis norovirus	0	0	1 (4.5)	0	0	1 (1.3)
Lower respiratory tract infection	0	0	0	1 (3.2)	0	1 (1.3)
Metapneumovirus bronchiolitis	0	0	0	1 (3.2)	0	1 (1.3)
Metapneumovirus pneumonia	0	1 (11.1)	0	0	0	1 (1.3)
Norovirus infection	0	0	1 (4.5)	0	0	1 (1.3)
Parainfluenzae virus infection	0	0	1 (4.5)	0	0	1 (1.3)
Pharyngitis	0	1 (11.1)	0	0	0	1 (1.3)
Pneumonia haemophilus	1 (7.1)	0	0	0	0	1 (1.3)
Pneumonia influenzal	0	0	0	1 (3.2)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpj
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC	< 3 months	≥ 3 and < 6 months	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Preferred Term	(N=14)	(N=9)	months	months	(N=4)	(N=80)
			(N=22)	(N=31)		
Pneumonia parainfluenzae viral	1 (7.1)	0	0	0	0	1 (1.3)
Serratia infection	0	0	0	1 (3.2)	0	1 (1.3)
Tonsillitis	0	0	1 (4.5)	0	0	1 (1.3)
Injury, poisoning and procedural complications	2 (14.3)	0	0	2 (6.5)	0	4 (5.0)
Femur fracture	1 (7.1)	0	0	1 (3.2)	0	2 (2.5)
Joint dislocation	1 (7.1)	0	0	1 (3.2)	0	2 (2.5)
Investigations	12 (85.7)	8 (88.9)	20 (90.9)	28 (90.3)	4 (100)	72 (90.0)
Aspartate aminotransferase increased	7 (50.0)	5 (55.6)	13 (59.1)	25 (80.6)	3 (75.0)	53 (66.3)
Alanine aminotransferase increased	6 (42.9)	5 (55.6)	13 (59.1)	25 (80.6)	3 (75.0)	52 (65.0)
Platelet count decreased	3 (21.4)	5 (55.6)	10 (45.5)	19 (61.3)	3 (75.0)	40 (50.0)
Blood lactate dehydrogenase increased	0	4 (44.4)	7 (31.8)	9 (29.0)	1 (25.0)	21 (26.3)
Serum ferritin increased	1 (7.1)	3 (33.3)	5 (22.7)	4 (12.9)	2 (50.0)	15 (18.8)
Troponin I increased	6 (42.9)	3 (33.3)	2 (9.1)	2 (6.5)	1 (25.0)	14 (17.5)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpj
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Blood creatine phosphokinase increased	1 (7.1)	1 (11.1)	1 (4.5)	4 (12.9)	1 (25.0)	8 (10.0)
Hepatic enzyme increased	1 (7.1)	2 (22.2)	2 (9.1)	3 (9.7)	0	8 (10.0)
White blood cell count decreased	2 (14.3)	2 (22.2)	3 (13.6)	0	0	7 (8.8)
Weight decreased	0	2 (22.2)	0	4 (12.9)	0	6 (7.5)
Gamma-glutamyltransferase increased	0	0	1 (4.5)	2 (6.5)	0	3 (3.8)
Blood creatinine increased	0	0	0	2 (6.5)	0	2 (2.5)
Haemoglobin decreased	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Neutrophil count decreased	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Troponin T increased	0	1 (11.1)	0	1 (3.2)	0	2 (2.5)
Amylase increased	0	0	1 (4.5)	0	0	1 (1.3)
Blood creatine phosphokinase MB increased	0	0	1 (4.5)	0	0	1 (1.3)
Blood pressure increased	0	0	0	1 (3.2)	0	1 (1.3)
Blood urea increased	0	0	0	1 (3.2)	0	1 (1.3)
C-reactive protein increased	0	0	0	1 (3.2)	0	1 (1.3)
Haptoglobin decreased	0	0	0	1 (3.2)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC	< 3 months	≥ 3 and < 6 months	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Preferred Term	(N=14)	(N=9)	months	months	(N=4)	(N=80)
			(N=22)	(N=31)		
Intraocular pressure increased	0	0	1 (4.5)	0	0	1 (1.3)
N-terminal prohormone brain natriuretic peptide increased	0	0	1 (4.5)	0	0	1 (1.3)
Urine output decreased	0	0	1 (4.5)	0	0	1 (1.3)
Weight increased	0	0	0	1 (3.2)	0	1 (1.3)
Metabolism and nutrition disorders	2 (14.3)	3 (33.3)	6 (27.3)	9 (29.0)	1 (25.0)	21 (26.3)
Decreased appetite	0	1 (11.1)	5 (22.7)	7 (22.6)	1 (25.0)	14 (17.5)
Weight gain poor	0	1 (11.1)	2 (9.1)	0	0	3 (3.8)
Feeding disorder	1 (7.1)	0	0	0	0	1 (1.3)
Hypercholesterolaemia	0	1 (11.1)	0	0	0	1 (1.3)
Hypertriglyceridaemia	0	1 (11.1)	0	0	0	1 (1.3)
Hypoglycaemia	0	0	0	1 (3.2)	0	1 (1.3)
Hypophagia	0	0	0	1 (3.2)	0	1 (1.3)
Metabolic acidosis	0	0	0	1 (3.2)	0	1 (1.3)
Underweight	1 (7.1)	0	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC	< 3 months	≥ 3 and < 6 months	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Preferred Term	(N=14)	(N=9)	months	months	(N=4)	(N=80)
			(N=22)	(N=31)		
Musculoskeletal and connective tissue disorders	0	3 (33.3)	5 (22.7)	4 (12.9)	0	12 (15.0)
Scoliosis	0	3 (33.3)	4 (18.2)	3 (9.7)	0	10 (12.5)
Joint contracture	0	0	0	1 (3.2)	0	1 (1.3)
Joint range of motion decreased	0	0	1 (4.5)	0	0	1 (1.3)
Kyphosis	0	1 (11.1)	0	0	0	1 (1.3)
Muscle atrophy	0	1 (11.1)	0	0	0	1 (1.3)
Nervous system disorders	1 (7.1)	0	1 (4.5)	1 (3.2)	0	3 (3.8)
Cerebral atrophy	0	0	0	1 (3.2)	0	1 (1.3)
Motor developmental delay	1 (7.1)	0	0	0	0	1 (1.3)
Seizure	0	0	1 (4.5)	0	0	1 (1.3)
Renal and urinary disorders	0	0	0	4 (12.9)	0	4 (5.0)
Haematuria	0	0	0	2 (6.5)	0	2 (2.5)
Proteinuria	0	0	0	2 (6.5)	0	2 (2.5)
Acute kidney injury	0	0	0	1 (3.2)	0	1 (1.3)
Chronic kidney disease	0	0	0	1 (3.2)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Glycosuria	0	0	0	1 (3.2)	0	1 (1.3)
Renal impairment	0	0	0	1 (3.2)	0	1 (1.3)
Renal injury	0	0	0	1 (3.2)	0	1 (1.3)
Respiratory, thoracic and mediastinal disorders	5 (35.7)	2 (22.2)	1 (4.5)	5 (16.1)	0	13 (16.3)
Acute respiratory failure	1 (7.1)	1 (11.1)	0	2 (6.5)	0	4 (5.0)
Atelectasis	0	1 (11.1)	0	3 (9.7)	0	4 (5.0)
Respiratory disorder	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Respiratory failure	2 (14.3)	0	0	0	0	2 (2.5)
Upper respiratory tract inflammation	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Apnoea	1 (7.1)	0	0	0	0	1 (1.3)
Aspiration	1 (7.1)	0	0	0	0	1 (1.3)
Hypoxia	1 (7.1)	0	0	0	0	1 (1.3)
Nocturnal dyspnoea	1 (7.1)	0	0	0	0	1 (1.3)
Pleural effusion	0	0	1 (4.5)	0	0	1 (1.3)
Respiration abnormal	0	1 (11.1)	0	0	0	1 (1.3)
Sleep apnoea syndrome	0	1 (11.1)	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Skin and subcutaneous tissue disorders	0	0	0	0	1 (25.0)	1 (1.3)
Urticaria	0	0	0	0	1 (25.0)	1 (1.3)
Vascular disorders	1 (7.1)	1 (11.1)	0	4 (12.9)	0	6 (7.5)
Kawasaki's disease	1 (7.1)	1 (11.1)	0	2 (6.5)	0	4 (5.0)
Hypertension	0	0	0	2 (6.5)	0	2 (2.5)
Not Coded	0	0	1 (4.5)	0	0	1 (1.3)
Gastrostomy	0	0	1 (4.5)	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Any Adverse Event	14 (100)	9 (100)	22 (100)	31 (100)	4 (100)	80 (100)
Blood and lymphatic system disorders	0	1 (11.1)	2 (9.1)	9 (29.0)	1 (25.0)	13 (16.3)
Grade 1	0	0	1 (4.5)	4 (12.9)	0	5 (6.3)
Grade 3	0	1 (11.1)	1 (4.5)	3 (9.7)	1 (25.0)	6 (7.5)
Grade 4	0	0	0	2 (6.5)	0	2 (2.5)
Thrombocytopenia	0	1 (11.1)	2 (9.1)	5 (16.1)	1 (25.0)	9 (11.3)
Grade 1	0	0	1 (4.5)	4 (12.9)	1 (25.0)	6 (7.5)
Grade 3	0	1 (11.1)	1 (4.5)	1 (3.2)	0	3 (3.8)
Thrombotic microangiopathy	0	0	0	4 (12.9)	0	4 (5.0)
Grade 3	0	0	0	2 (6.5)	0	2 (2.5)
Grade 4	0	0	0	2 (6.5)	0	2 (2.5)
Anaemia	0	1 (11.1)	0	0	0	1 (1.3)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Eosinophilia	0	0	0	0	1 (25.0)	1 (1.3)
Grade 3	0	0	0	0	1 (25.0)	1 (1.3)
Haemolytic anaemia	0	0	0	1 (3.2)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

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Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)
Cardiac disorders	0	0	2 (9.1)	2 (6.5)	0	4 (5.0)
Grade 1	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Grade 4	0	0	0	1 (3.2)	0	1 (1.3)
Grade 5	0	0	1 (4.5)	0	0	1 (1.3)
Arrhythmia	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Bradycardia	0	0	1 (4.5)	0	0	1 (1.3)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)
Cardiac failure congestive	0	0	0	1 (3.2)	0	1 (1.3)
Grade 4	0	0	0	1 (3.2)	0	1 (1.3)
Cardio-respiratory arrest	0	0	1 (4.5)	0	0	1 (1.3)
Grade 5	0	0	1 (4.5)	0	0	1 (1.3)
Congenital, familial and genetic disorders	2 (14.3)	0	3 (13.6)	0	0	5 (6.3)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Grade 2	0	0	1 (4.5)	0	0	1 (1.3)
Grade 3	2 (14.3)	0	1 (4.5)	0	0	3 (3.8)
Cryptorchism	2 (14.3)	0	2 (9.1)	0	0	4 (5.0)
Grade 2	0	0	1 (4.5)	0	0	1 (1.3)
Grade 3	2 (14.3)	0	1 (4.5)	0	0	3 (3.8)
Developmental hip dysplasia	0	0	1 (4.5)	0	0	1 (1.3)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)
Endocrine disorders	0	0	1 (4.5)	0	0	1 (1.3)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)
Cushingoid	0	0	1 (4.5)	0	0	1 (1.3)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)
Gastrointestinal disorders	4 (28.6)	6 (66.7)	14 (63.6)	13 (41.9)	3 (75.0)	40 (50.0)
Grade 1	2 (14.3)	3 (33.3)	6 (27.3)	8 (25.8)	2 (50.0)	21 (26.3)
Grade 2	0	1 (11.1)	3 (13.6)	5 (16.1)	1 (25.0)	10 (12.5)
Grade 3	1 (7.1)	2 (22.2)	5 (22.7)	0	0	8 (10.0)
Grade 4	1 (7.1)	0	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Vomiting	4 (28.6)	5 (55.6)	10 (45.5)	11 (35.5)	2 (50.0)	32 (40.0)
Grade 1	3 (21.4)	4 (44.4)	7 (31.8)	7 (22.6)	1 (25.0)	22 (27.5)
Grade 2	0	1 (11.1)	3 (13.6)	4 (12.9)	1 (25.0)	9 (11.3)
Grade 3	1 (7.1)	0	0	0	0	1 (1.3)
Gastric fistula	1 (7.1)	2 (22.2)	3 (13.6)	0	0	6 (7.5)
Grade 3	1 (7.1)	2 (22.2)	3 (13.6)	0	0	6 (7.5)
Nausea	0	0	1 (4.5)	3 (9.7)	1 (25.0)	5 (6.3)
Grade 1	0	0	1 (4.5)	2 (6.5)	0	3 (3.8)
Grade 2	0	0	0	1 (3.2)	1 (25.0)	2 (2.5)
Dysphagia	1 (7.1)	1 (11.1)	1 (4.5)	0	0	3 (3.8)
Grade 3	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)
Grade 4	1 (7.1)	0	0	0	0	1 (1.3)
Constipation	1 (7.1)	0	1 (4.5)	0	0	2 (2.5)
Grade 2	1 (7.1)	0	1 (4.5)	0	0	2 (2.5)
Diarrhoea	1 (7.1)	0	1 (4.5)	0	0	2 (2.5)
Grade 1	1 (7.1)	0	1 (4.5)	0	0	2 (2.5)
Acetonaemic vomiting	0	0	1 (4.5)	0	0	1 (1.3)
Grade 3	0	0	1 (4.5)	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

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Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Data as of 23MAY2025: ADAE

Prg: TEAEG.SAS

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Gastric haemorrhage	0	0	0	0	1 (25.0)	1 (1.3)
Grade 1	0	0	0	0	1 (25.0)	1 (1.3)
Gastrooesophageal reflux disease	1 (7.1)	0	0	0	0	1 (1.3)
Grade 3	1 (7.1)	0	0	0	0	1 (1.3)
General disorders and administration site conditions	9 (64.3)	7 (77.8)	20 (90.9)	25 (80.6)	4 (100)	65 (81.3)
Grade 1	9 (64.3)	5 (55.6)	13 (59.1)	16 (51.6)	4 (100)	47 (58.8)
Grade 2	0	2 (22.2)	5 (22.7)	7 (22.6)	0	14 (17.5)
Grade 3	0	0	1 (4.5)	2 (6.5)	0	3 (3.8)
Grade 4	0	0	1 (4.5)	0	0	1 (1.3)
Pyrexia	9 (64.3)	7 (77.8)	20 (90.9)	25 (80.6)	4 (100)	65 (81.3)
Grade 1	9 (64.3)	5 (55.6)	14 (63.6)	17 (54.8)	4 (100)	49 (61.3)
Grade 2	0	2 (22.2)	5 (22.7)	8 (25.8)	0	15 (18.8)
Grade 3	0	0	1 (4.5)	0	0	1 (1.3)
Asthenia	0	1 (11.1)	0	1 (3.2)	0	2 (2.5)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

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Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Malaise	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Drug withdrawal syndrome	0	0	0	1 (3.2)	0	1 (1.3)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Generalised oedema	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)
Inflammation	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Medical device site haemorrhage	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)
Multiple organ dysfunction syndrome	0	0	1 (4.5)	0	0	1 (1.3)
Grade 4	0	0	1 (4.5)	0	0	1 (1.3)
Hepatobiliary disorders	3 (21.4)	2 (22.2)	4 (18.2)	2 (6.5)	1 (25.0)	12 (15.0)
Grade 1	1 (7.1)	0	1 (4.5)	0	0	2 (2.5)
Grade 2	2 (14.3)	2 (22.2)	0	1 (3.2)	0	5 (6.3)
Grade 3	0	0	1 (4.5)	0	1 (25.0)	2 (2.5)

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Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Grade 4	0	0	2 (9.1)	1 (3.2)	0	3 (3.8)
Hepatic function abnormal	3 (21.4)	2 (22.2)	4 (18.2)	2 (6.5)	1 (25.0)	12 (15.0)
Grade 1	1 (7.1)	0	2 (9.1)	0	0	3 (3.8)
Grade 2	2 (14.3)	2 (22.2)	0	1 (3.2)	0	5 (6.3)
Grade 3	0	0	1 (4.5)	0	1 (25.0)	2 (2.5)
Grade 4	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Acute hepatic failure	0	0	1 (4.5)	0	0	1 (1.3)
Grade 4	0	0	1 (4.5)	0	0	1 (1.3)
Cholelithiasis	0	0	0	1 (3.2)	0	1 (1.3)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Hepatomegaly	0	0	0	0	1 (25.0)	1 (1.3)
Grade 1	0	0	0	0	1 (25.0)	1 (1.3)
Immune system disorders	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Hypersensitivity	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)

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Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Infections and infestations	5 (35.7)	8 (88.9)	11 (50.0)	19 (61.3)	1 (25.0)	44 (55.0)
Grade 1	0	0	2 (9.1)	1 (3.2)	0	3 (3.8)
Grade 2	0	1 (11.1)	2 (9.1)	5 (16.1)	0	8 (10.0)
Grade 3	4 (28.6)	3 (33.3)	5 (22.7)	10 (32.3)	1 (25.0)	23 (28.8)
Grade 4	1 (7.1)	4 (44.4)	2 (9.1)	3 (9.7)	0	10 (12.5)
Pneumonia	2 (14.3)	4 (44.4)	1 (4.5)	6 (19.4)	0	13 (16.3)
Grade 3	1 (7.1)	3 (33.3)	1 (4.5)	6 (19.4)	0	11 (13.8)
Grade 4	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Upper respiratory tract infection	3 (21.4)	2 (22.2)	3 (13.6)	5 (16.1)	0	13 (16.3)
Grade 1	0	0	1 (4.5)	2 (6.5)	0	3 (3.8)
Grade 2	1 (7.1)	1 (11.1)	0	2 (6.5)	0	4 (5.0)
Grade 3	2 (14.3)	1 (11.1)	2 (9.1)	1 (3.2)	0	6 (7.5)
Pneumonia aspiration	2 (14.3)	2 (22.2)	2 (9.1)	6 (19.4)	0	12 (15.0)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Grade 2	0	0	0	2 (6.5)	0	2 (2.5)
Grade 3	1 (7.1)	2 (22.2)	1 (4.5)	2 (6.5)	0	6 (7.5)
Grade 4	1 (7.1)	0	1 (4.5)	1 (3.2)	0	3 (3.8)
Bronchitis	0	1 (11.1)	4 (18.2)	3 (9.7)	0	8 (10.0)

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Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Grade 2	0	0	1 (4.5)	0	0	1 (1.3)
Grade 3	0	1 (11.1)	3 (13.6)	3 (9.7)	0	7 (8.8)
COVID-19	2 (14.3)	2 (22.2)	3 (13.6)	1 (3.2)	0	8 (10.0)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	2 (14.3)	2 (22.2)	2 (9.1)	0	0	6 (7.5)
Pneumonia respiratory syncytial viral	1 (7.1)	1 (11.1)	1 (4.5)	3 (9.7)	0	6 (7.5)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	1 (7.1)	0	0	2 (6.5)	0	3 (3.8)
Grade 4	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)
Respiratory syncytial virus infection	1 (7.1)	1 (11.1)	0	3 (9.7)	0	5 (6.3)
Grade 3	1 (7.1)	0	0	2 (6.5)	0	3 (3.8)
Grade 4	0	1 (11.1)	0	1 (3.2)	0	2 (2.5)
Bronchitis viral	0	0	1 (4.5)	2 (6.5)	0	3 (3.8)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Gastroenteritis	1 (7.1)	0	0	2 (6.5)	0	3 (3.8)
Grade 3	1 (7.1)	0	0	2 (6.5)	0	3 (3.8)

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Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Respiratory syncytial virus bronchiolitis	0	0	2 (9.1)	1 (3.2)	0	3 (3.8)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	0	0	2 (9.1)	0	0	2 (2.5)
Bronchiolitis	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Grade 3	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Metapneumovirus infection	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)
Grade 3	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)
Nasopharyngitis	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Grade 2	0	0	1 (4.5)	0	0	1 (1.3)
Pneumonia viral	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)
Grade 3	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)
Respiratory tract infection	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	0	0	1 (4.5)	0	0	1 (1.3)
Rhinovirus infection	0	0	2 (9.1)	0	0	2 (2.5)
Grade 3	0	0	2 (9.1)	0	0	2 (2.5)

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Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Viral infection	0	1 (11.1)	0	0	1 (25.0)	2 (2.5)
Grade 3	0	0	0	0	1 (25.0)	1 (1.3)
Grade 4	0	1 (11.1)	0	0	0	1 (1.3)
Viral upper respiratory tract infection	0	1 (11.1)	0	1 (3.2)	0	2 (2.5)
Grade 3	0	1 (11.1)	0	1 (3.2)	0	2 (2.5)
Epstein-Barr virus infection	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Gastroenteritis norovirus	0	0	1 (4.5)	0	0	1 (1.3)
Grade 3	0	0	1 (4.5)	0	0	1 (1.3)
Lower respiratory tract infection	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)
Metapneumovirus bronchiolitis	0	0	0	1 (3.2)	0	1 (1.3)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Metapneumovirus pneumonia	0	1 (11.1)	0	0	0	1 (1.3)
Grade 3	0	1 (11.1)	0	0	0	1 (1.3)
Norovirus infection	0	0	1 (4.5)	0	0	1 (1.3)
Grade 2	0	0	1 (4.5)	0	0	1 (1.3)
Parainfluenzae virus infection	0	0	1 (4.5)	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Data as of 23MAY2025: ADAE

Prg: TEAEG.SAS

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Grade 3	0	0	1 (4.5)	0	0	1 (1.3)
Pharyngitis	0	1 (11.1)	0	0	0	1 (1.3)
Grade 3	0	1 (11.1)	0	0	0	1 (1.3)
Pneumonia haemophilus	1 (7.1)	0	0	0	0	1 (1.3)
Grade 3	1 (7.1)	0	0	0	0	1 (1.3)
Pneumonia influenzal	0	0	0	1 (3.2)	0	1 (1.3)
Grade 4	0	0	0	1 (3.2)	0	1 (1.3)
Pneumonia parainfluenzae viral	1 (7.1)	0	0	0	0	1 (1.3)
Grade 3	1 (7.1)	0	0	0	0	1 (1.3)
Serratia infection	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)
Tonsillitis	0	0	1 (4.5)	0	0	1 (1.3)
Grade 3	0	0	1 (4.5)	0	0	1 (1.3)
Injury, poisoning and procedural complications	2 (14.3)	0	0	2 (6.5)	0	4 (5.0)
Grade 2	2 (14.3)	0	0	1 (3.2)	0	3 (3.8)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Femur fracture	1 (7.1)	0	0	1 (3.2)	0	2 (2.5)
Grade 2	1 (7.1)	0	0	1 (3.2)	0	2 (2.5)
Joint dislocation	1 (7.1)	0	0	1 (3.2)	0	2 (2.5)
Grade 2	1 (7.1)	0	0	0	0	1 (1.3)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)
Investigations	12 (85.7)	8 (88.9)	20 (90.9)	28 (90.3)	4 (100)	72 (90.0)
Grade 1	7 (50.0)	3 (33.3)	4 (18.2)	2 (6.5)	1 (25.0)	17 (21.3)
Grade 2	3 (21.4)	1 (11.1)	4 (18.2)	4 (12.9)	0	12 (15.0)
Grade 3	1 (7.1)	3 (33.3)	9 (40.9)	15 (48.4)	3 (75.0)	31 (38.8)
Grade 4	1 (7.1)	1 (11.1)	3 (13.6)	7 (22.6)	0	12 (15.0)
Aspartate aminotransferase increased	7 (50.0)	5 (55.6)	13 (59.1)	25 (80.6)	3 (75.0)	53 (66.3)
Grade 1	3 (21.4)	1 (11.1)	2 (9.1)	2 (6.5)	0	8 (10.0)
Grade 2	3 (21.4)	1 (11.1)	3 (13.6)	4 (12.9)	0	11 (13.8)
Grade 3	1 (7.1)	2 (22.2)	6 (27.3)	16 (51.6)	3 (75.0)	28 (35.0)
Grade 4	0	1 (11.1)	2 (9.1)	3 (9.7)	0	6 (7.5)
Alanine aminotransferase increased	6 (42.9)	5 (55.6)	13 (59.1)	25 (80.6)	3 (75.0)	52 (65.0)
Grade 1	5 (35.7)	2 (22.2)	2 (9.1)	2 (6.5)	0	11 (13.8)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Grade 2	0	1 (11.1)	2 (9.1)	5 (16.1)	1 (25.0)	9 (11.3)
Grade 3	1 (7.1)	1 (11.1)	7 (31.8)	13 (41.9)	2 (50.0)	24 (30.0)
Grade 4	0	1 (11.1)	2 (9.1)	5 (16.1)	0	8 (10.0)
Platelet count decreased	3 (21.4)	5 (55.6)	10 (45.5)	19 (61.3)	3 (75.0)	40 (50.0)
Grade 1	2 (14.3)	5 (55.6)	8 (36.4)	7 (22.6)	1 (25.0)	23 (28.8)
Grade 2	1 (7.1)	0	0	8 (25.8)	1 (25.0)	10 (12.5)
Grade 3	0	0	2 (9.1)	2 (6.5)	1 (25.0)	5 (6.3)
Grade 4	0	0	0	2 (6.5)	0	2 (2.5)
Blood lactate dehydrogenase increased	0	4 (44.4)	7 (31.8)	9 (29.0)	1 (25.0)	21 (26.3)
Grade 1	0	4 (44.4)	6 (27.3)	9 (29.0)	1 (25.0)	20 (25.0)
Grade 2	0	0	1 (4.5)	0	0	1 (1.3)
Serum ferritin increased	1 (7.1)	3 (33.3)	5 (22.7)	4 (12.9)	2 (50.0)	15 (18.8)
Grade 1	1 (7.1)	3 (33.3)	4 (18.2)	3 (9.7)	2 (50.0)	13 (16.3)
Grade 2	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Troponin I increased	6 (42.9)	3 (33.3)	2 (9.1)	2 (6.5)	1 (25.0)	14 (17.5)
Grade 1	6 (42.9)	3 (33.3)	2 (9.1)	2 (6.5)	1 (25.0)	14 (17.5)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Blood creatine phosphokinase increased	1 (7.1)	1 (11.1)	1 (4.5)	4 (12.9)	1 (25.0)	8 (10.0)
Grade 1	1 (7.1)	1 (11.1)	1 (4.5)	3 (9.7)	1 (25.0)	7 (8.8)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Hepatic enzyme increased	1 (7.1)	2 (22.2)	2 (9.1)	3 (9.7)	0	8 (10.0)
Grade 1	1 (7.1)	1 (11.1)	1 (4.5)	1 (3.2)	0	4 (5.0)
Grade 2	0	0	0	2 (6.5)	0	2 (2.5)
Grade 3	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)
White blood cell count decreased	2 (14.3)	2 (22.2)	3 (13.6)	0	0	7 (8.8)
Grade 1	1 (7.1)	1 (11.1)	2 (9.1)	0	0	4 (5.0)
Grade 2	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Grade 3	0	0	1 (4.5)	0	0	1 (1.3)
Weight decreased	0	2 (22.2)	0	4 (12.9)	0	6 (7.5)
Grade 1	0	1 (11.1)	0	3 (9.7)	0	4 (5.0)
Grade 2	0	1 (11.1)	0	0	0	1 (1.3)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)
Gamma-glutamyltransferase increased	0	0	1 (4.5)	2 (6.5)	0	3 (3.8)
Grade 1	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)

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Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)
Blood creatinine increased	0	0	0	2 (6.5)	0	2 (2.5)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Haemoglobin decreased	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Grade 1	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Neutrophil count decreased	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Grade 4	1 (7.1)	0	0	0	0	1 (1.3)
Troponin T increased	0	1 (11.1)	0	1 (3.2)	0	2 (2.5)
Grade 1	0	1 (11.1)	0	1 (3.2)	0	2 (2.5)
Amylase increased	0	0	1 (4.5)	0	0	1 (1.3)
Grade 2	0	0	1 (4.5)	0	0	1 (1.3)
Blood creatine phosphokinase MB increased	0	0	1 (4.5)	0	0	1 (1.3)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)
Blood pressure increased	0	0	0	1 (3.2)	0	1 (1.3)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)

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Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Blood urea increased	0	0	0	1 (3.2)	0	1 (1.3)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
C-reactive protein increased	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Haptoglobin decreased	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Intraocular pressure increased	0	0	1 (4.5)	0	0	1 (1.3)
Grade 2	0	0	1 (4.5)	0	0	1 (1.3)
N-terminal prohormone brain natriuretic peptide increased	0	0	1 (4.5)	0	0	1 (1.3)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)
Urine output decreased	0	0	1 (4.5)	0	0	1 (1.3)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)
Weight increased	0	0	0	1 (3.2)	0	1 (1.3)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Metabolism and nutrition disorders	2 (14.3)	3 (33.3)	6 (27.3)	9 (29.0)	1 (25.0)	21 (26.3)
Grade 1	2 (14.3)	2 (22.2)	4 (18.2)	4 (12.9)	1 (25.0)	13 (16.3)

Adverse Events were coded using MedDRA, version &medraver..

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Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Grade 2	0	0	1 (4.5)	2 (6.5)	0	3 (3.8)
Grade 3	0	1 (11.1)	1 (4.5)	3 (9.7)	0	5 (6.3)
Decreased appetite	0	1 (11.1)	5 (22.7)	7 (22.6)	1 (25.0)	14 (17.5)
Grade 1	0	1 (11.1)	4 (18.2)	4 (12.9)	1 (25.0)	10 (12.5)
Grade 2	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Grade 3	0	0	0	2 (6.5)	0	2 (2.5)
Weight gain poor	0	1 (11.1)	2 (9.1)	0	0	3 (3.8)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)
Grade 3	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)
Feeding disorder	1 (7.1)	0	0	0	0	1 (1.3)
Grade 1	1 (7.1)	0	0	0	0	1 (1.3)
Hypercholesterolaemia	0	1 (11.1)	0	0	0	1 (1.3)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Hypertriglyceridaemia	0	1 (11.1)	0	0	0	1 (1.3)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Hypoglycaemia	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)
Hypophagia	0	0	0	1 (3.2)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

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Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Data as of 23MAY2025: ADAE

Prg: TEAEG.SAS

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Metabolic acidosis	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Underweight	1 (7.1)	0	0	0	0	1 (1.3)
Grade 1	1 (7.1)	0	0	0	0	1 (1.3)
Musculoskeletal and connective tissue disorders	0	3 (33.3)	5 (22.7)	4 (12.9)	0	12 (15.0)
Grade 1	0	1 (11.1)	2 (9.1)	1 (3.2)	0	4 (5.0)
Grade 2	0	1 (11.1)	3 (13.6)	2 (6.5)	0	6 (7.5)
Grade 3	0	1 (11.1)	0	1 (3.2)	0	2 (2.5)
Scoliosis	0	3 (33.3)	4 (18.2)	3 (9.7)	0	10 (12.5)
Grade 1	0	1 (11.1)	2 (9.1)	0	0	3 (3.8)
Grade 2	0	1 (11.1)	2 (9.1)	2 (6.5)	0	5 (6.3)
Grade 3	0	1 (11.1)	0	1 (3.2)	0	2 (2.5)
Joint contracture	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Joint range of motion decreased	0	0	1 (4.5)	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

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Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Grade 2	0	0	1 (4.5)	0	0	1 (1.3)
Kyphosis	0	1 (11.1)	0	0	0	1 (1.3)
Grade 3	0	1 (11.1)	0	0	0	1 (1.3)
Muscle atrophy	0	1 (11.1)	0	0	0	1 (1.3)
Grade 3	0	1 (11.1)	0	0	0	1 (1.3)
Nervous system disorders	1 (7.1)	0	1 (4.5)	1 (3.2)	0	3 (3.8)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Grade 2	1 (7.1)	0	0	0	0	1 (1.3)
Grade 3	0	0	1 (4.5)	0	0	1 (1.3)
Cerebral atrophy	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Motor developmental delay	1 (7.1)	0	0	0	0	1 (1.3)
Grade 2	1 (7.1)	0	0	0	0	1 (1.3)
Seizure	0	0	1 (4.5)	0	0	1 (1.3)
Grade 3	0	0	1 (4.5)	0	0	1 (1.3)
Renal and urinary disorders	0	0	0	4 (12.9)	0	4 (5.0)

Adverse Events were coded using MedDRA, version &medraver..

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Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jp
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)
Grade 4	0	0	0	2 (6.5)	0	2 (2.5)
Haematuria	0	0	0	2 (6.5)	0	2 (2.5)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Grade 4	0	0	0	1 (3.2)	0	1 (1.3)
Proteinuria	0	0	0	2 (6.5)	0	2 (2.5)
Grade 3	0	0	0	2 (6.5)	0	2 (2.5)
Acute kidney injury	0	0	0	1 (3.2)	0	1 (1.3)
Grade 4	0	0	0	1 (3.2)	0	1 (1.3)
Chronic kidney disease	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Glycosuria	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Renal impairment	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)
Renal injury	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Respiratory, thoracic and mediastinal disorders	5 (35.7)	2 (22.2)	1 (4.5)	5 (16.1)	0	13 (16.3)
Grade 1	1 (7.1)	0	0	0	0	1 (1.3)
Grade 3	1 (7.1)	1 (11.1)	1 (4.5)	3 (9.7)	0	6 (7.5)
Grade 4	3 (21.4)	1 (11.1)	0	2 (6.5)	0	6 (7.5)
Acute respiratory failure	1 (7.1)	1 (11.1)	0	2 (6.5)	0	4 (5.0)
Grade 3	0	1 (11.1)	0	0	0	1 (1.3)
Grade 4	1 (7.1)	0	0	2 (6.5)	0	3 (3.8)
Atelectasis	0	1 (11.1)	0	3 (9.7)	0	4 (5.0)
Grade 3	0	1 (11.1)	0	3 (9.7)	0	4 (5.0)
Respiratory disorder	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Grade 3	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Respiratory failure	2 (14.3)	0	0	0	0	2 (2.5)
Grade 3	1 (7.1)	0	0	0	0	1 (1.3)
Grade 4	1 (7.1)	0	0	0	0	1 (1.3)
Upper respiratory tract inflammation	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Grade 1	1 (7.1)	0	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Grade 3	0	1 (11.1)	0	0	0	1 (1.3)
Apnoea	1 (7.1)	0	0	0	0	1 (1.3)
Grade 4	1 (7.1)	0	0	0	0	1 (1.3)
Aspiration	1 (7.1)	0	0	0	0	1 (1.3)
Grade 3	1 (7.1)	0	0	0	0	1 (1.3)
Hypoxia	1 (7.1)	0	0	0	0	1 (1.3)
Grade 2	1 (7.1)	0	0	0	0	1 (1.3)
Nocturnal dyspnoea	1 (7.1)	0	0	0	0	1 (1.3)
Grade 1	1 (7.1)	0	0	0	0	1 (1.3)
Pleural effusion	0	0	1 (4.5)	0	0	1 (1.3)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)
Respiration abnormal	0	1 (11.1)	0	0	0	1 (1.3)
Grade 4	0	1 (11.1)	0	0	0	1 (1.3)
Sleep apnoea syndrome	0	1 (11.1)	0	0	0	1 (1.3)
Grade 2	0	1 (11.1)	0	0	0	1 (1.3)
Skin and subcutaneous tissue disorders	0	0	0	0	1 (25.0)	1 (1.3)
Grade 1	0	0	0	0	1 (25.0)	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Data as of 23MAY2025: ADAE

Prg: TEAEG.SAS

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Urticaria	0	0	0	0	1 (25.0)	1 (1.3)
Grade 1	0	0	0	0	1 (25.0)	1 (1.3)
Vascular disorders	1 (7.1)	1 (11.1)	0	4 (12.9)	0	6 (7.5)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	1 (7.1)	1 (11.1)	0	3 (9.7)	0	5 (6.3)
Kawasaki's disease	1 (7.1)	1 (11.1)	0	2 (6.5)	0	4 (5.0)
Grade 3	1 (7.1)	1 (11.1)	0	2 (6.5)	0	4 (5.0)
Hypertension	0	0	0	2 (6.5)	0	2 (2.5)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)
Not Coded	0	0	1 (4.5)	0	0	1 (1.3)
Grade 3	0	0	1 (4.5)	0	0	1 (1.3)
Gastrostomy	0	0	1 (4.5)	0	0	1 (1.3)
Grade 3	0	0	1 (4.5)	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Any Adverse Event	13 (92.9)	9 (100)	22 (100)	31 (100)	4 (100)	79 (98.8)
Blood and lymphatic system disorders	0	1 (11.1)	2 (9.1)	9 (29.0)	1 (25.0)	13 (16.3)
Grade 1	0	0	1 (4.5)	4 (12.9)	1 (25.0)	6 (7.5)
Grade 3	0	1 (11.1)	1 (4.5)	3 (9.7)	0	5 (6.3)
Grade 4	0	0	0	2 (6.5)	0	2 (2.5)
Thrombocytopenia	0	1 (11.1)	2 (9.1)	5 (16.1)	1 (25.0)	9 (11.3)
Grade 1	0	0	1 (4.5)	4 (12.9)	1 (25.0)	6 (7.5)
Grade 3	0	1 (11.1)	1 (4.5)	1 (3.2)	0	3 (3.8)
Thrombotic microangiopathy	0	0	0	4 (12.9)	0	4 (5.0)
Grade 3	0	0	0	2 (6.5)	0	2 (2.5)
Grade 4	0	0	0	2 (6.5)	0	2 (2.5)
Haemolytic anaemia	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)
Cardiac disorders	0	0	1 (4.5)	2 (6.5)	0	3 (3.8)
Grade 1	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Data as of 23MAY2025: ADAE

Prg: TEAEG.SAS

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Grade 4	0	0	0	1 (3.2)	0	1 (1.3)
Arrhythmia	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Bradycardia	0	0	1 (4.5)	0	0	1 (1.3)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)
Cardiac failure congestive	0	0	0	1 (3.2)	0	1 (1.3)
Grade 4	0	0	0	1 (3.2)	0	1 (1.3)
Gastrointestinal disorders	3 (21.4)	3 (33.3)	10 (45.5)	12 (38.7)	2 (50.0)	30 (37.5)
Grade 1	3 (21.4)	2 (22.2)	7 (31.8)	8 (25.8)	1 (25.0)	21 (26.3)
Grade 2	0	1 (11.1)	3 (13.6)	4 (12.9)	1 (25.0)	9 (11.3)
Vomiting	3 (21.4)	3 (33.3)	10 (45.5)	10 (32.3)	2 (50.0)	28 (35.0)
Grade 1	3 (21.4)	2 (22.2)	7 (31.8)	7 (22.6)	1 (25.0)	20 (25.0)
Grade 2	0	1 (11.1)	3 (13.6)	3 (9.7)	1 (25.0)	8 (10.0)
Nausea	0	0	1 (4.5)	3 (9.7)	1 (25.0)	5 (6.3)
Grade 1	0	0	1 (4.5)	2 (6.5)	0	3 (3.8)
Grade 2	0	0	0	1 (3.2)	1 (25.0)	2 (2.5)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
General disorders and administration site conditions	9 (64.3)	6 (66.7)	20 (90.9)	25 (80.6)	4 (100)	64 (80.0)
Grade 1	9 (64.3)	4 (44.4)	13 (59.1)	16 (51.6)	4 (100)	46 (57.5)
Grade 2	0	2 (22.2)	5 (22.7)	8 (25.8)	0	15 (18.8)
Grade 3	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Grade 4	0	0	1 (4.5)	0	0	1 (1.3)
Pyrexia	9 (64.3)	6 (66.7)	20 (90.9)	25 (80.6)	4 (100)	64 (80.0)
Grade 1	9 (64.3)	4 (44.4)	14 (63.6)	17 (54.8)	4 (100)	48 (60.0)
Grade 2	0	2 (22.2)	5 (22.7)	8 (25.8)	0	15 (18.8)
Grade 3	0	0	1 (4.5)	0	0	1 (1.3)
Asthenia	0	1 (11.1)	0	1 (3.2)	0	2 (2.5)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Malaise	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Generalised oedema	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Multiple organ dysfunction syndrome	0	0	1 (4.5)	0	0	1 (1.3)
Grade 4	0	0	1 (4.5)	0	0	1 (1.3)
Hepatobiliary disorders	2 (14.3)	2 (22.2)	4 (18.2)	2 (6.5)	1 (25.0)	11 (13.8)
Grade 1	1 (7.1)	0	1 (4.5)	0	0	2 (2.5)
Grade 2	1 (7.1)	2 (22.2)	0	1 (3.2)	0	4 (5.0)
Grade 3	0	0	1 (4.5)	0	1 (25.0)	2 (2.5)
Grade 4	0	0	2 (9.1)	1 (3.2)	0	3 (3.8)
Hepatic function abnormal	2 (14.3)	2 (22.2)	4 (18.2)	2 (6.5)	1 (25.0)	11 (13.8)
Grade 1	1 (7.1)	0	2 (9.1)	0	0	3 (3.8)
Grade 2	1 (7.1)	2 (22.2)	0	1 (3.2)	0	4 (5.0)
Grade 3	0	0	1 (4.5)	0	1 (25.0)	2 (2.5)
Grade 4	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Acute hepatic failure	0	0	1 (4.5)	0	0	1 (1.3)
Grade 4	0	0	1 (4.5)	0	0	1 (1.3)
Hepatomegaly	0	0	0	0	1 (25.0)	1 (1.3)
Grade 1	0	0	0	0	1 (25.0)	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

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Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Data as of 23MAY2025: ADAE

Prg: TEAEG.SAS

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Immune system disorders	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Hypersensitivity	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Infections and infestations	0	0	1 (4.5)	0	0	1 (1.3)
Grade 2	0	0	1 (4.5)	0	0	1 (1.3)
Bronchitis	0	0	1 (4.5)	0	0	1 (1.3)
Grade 2	0	0	1 (4.5)	0	0	1 (1.3)
Investigations	12 (85.7)	8 (88.9)	20 (90.9)	28 (90.3)	4 (100)	72 (90.0)
Grade 1	7 (50.0)	3 (33.3)	4 (18.2)	2 (6.5)	1 (25.0)	17 (21.3)
Grade 2	3 (21.4)	1 (11.1)	4 (18.2)	5 (16.1)	0	13 (16.3)
Grade 3	1 (7.1)	3 (33.3)	9 (40.9)	14 (45.2)	3 (75.0)	30 (37.5)
Grade 4	1 (7.1)	1 (11.1)	3 (13.6)	7 (22.6)	0	12 (15.0)
Aspartate aminotransferase increased	7 (50.0)	5 (55.6)	13 (59.1)	25 (80.6)	3 (75.0)	53 (66.3)
Grade 1	3 (21.4)	1 (11.1)	2 (9.1)	2 (6.5)	0	8 (10.0)
Grade 2	3 (21.4)	1 (11.1)	3 (13.6)	4 (12.9)	0	11 (13.8)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Grade 3	1 (7.1)	2 (22.2)	6 (27.3)	16 (51.6)	3 (75.0)	28 (35.0)
Grade 4	0	1 (11.1)	2 (9.1)	3 (9.7)	0	6 (7.5)
Alanine aminotransferase increased	5 (35.7)	5 (55.6)	13 (59.1)	24 (77.4)	3 (75.0)	50 (62.5)
Grade 1	4 (28.6)	2 (22.2)	2 (9.1)	3 (9.7)	0	11 (13.8)
Grade 2	0	1 (11.1)	2 (9.1)	5 (16.1)	1 (25.0)	9 (11.3)
Grade 3	1 (7.1)	1 (11.1)	7 (31.8)	11 (35.5)	2 (50.0)	22 (27.5)
Grade 4	0	1 (11.1)	2 (9.1)	5 (16.1)	0	8 (10.0)
Platelet count decreased	3 (21.4)	5 (55.6)	10 (45.5)	19 (61.3)	3 (75.0)	40 (50.0)
Grade 1	2 (14.3)	5 (55.6)	8 (36.4)	7 (22.6)	1 (25.0)	23 (28.8)
Grade 2	1 (7.1)	0	0	8 (25.8)	1 (25.0)	10 (12.5)
Grade 3	0	0	2 (9.1)	2 (6.5)	1 (25.0)	5 (6.3)
Grade 4	0	0	0	2 (6.5)	0	2 (2.5)
Blood lactate dehydrogenase increased	0	4 (44.4)	7 (31.8)	9 (29.0)	1 (25.0)	21 (26.3)
Grade 1	0	4 (44.4)	6 (27.3)	9 (29.0)	1 (25.0)	20 (25.0)
Grade 2	0	0	1 (4.5)	0	0	1 (1.3)
Serum ferritin increased	1 (7.1)	3 (33.3)	5 (22.7)	4 (12.9)	2 (50.0)	15 (18.8)
Grade 1	1 (7.1)	3 (33.3)	4 (18.2)	3 (9.7)	2 (50.0)	13 (16.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Grade 2	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Troponin I increased	6 (42.9)	2 (22.2)	2 (9.1)	1 (3.2)	1 (25.0)	12 (15.0)
Grade 1	6 (42.9)	2 (22.2)	2 (9.1)	1 (3.2)	1 (25.0)	12 (15.0)
Hepatic enzyme increased	1 (7.1)	2 (22.2)	2 (9.1)	3 (9.7)	0	8 (10.0)
Grade 1	1 (7.1)	1 (11.1)	1 (4.5)	1 (3.2)	0	4 (5.0)
Grade 2	0	0	0	2 (6.5)	0	2 (2.5)
Grade 3	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)
Blood creatine phosphokinase increased	1 (7.1)	1 (11.1)	1 (4.5)	3 (9.7)	1 (25.0)	7 (8.8)
Grade 1	1 (7.1)	1 (11.1)	1 (4.5)	2 (6.5)	1 (25.0)	6 (7.5)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
White blood cell count decreased	2 (14.3)	2 (22.2)	3 (13.6)	0	0	7 (8.8)
Grade 1	1 (7.1)	1 (11.1)	2 (9.1)	0	0	4 (5.0)
Grade 2	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Grade 3	0	0	1 (4.5)	0	0	1 (1.3)
Gamma-glutamyltransferase increased	0	0	1 (4.5)	2 (6.5)	0	3 (3.8)
Grade 1	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Blood creatinine increased	0	0	0	2 (6.5)	0	2 (2.5)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Haemoglobin decreased	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Grade 1	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Neutrophil count decreased	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Grade 4	1 (7.1)	0	0	0	0	1 (1.3)
Weight decreased	0	1 (11.1)	0	1 (3.2)	0	2 (2.5)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Grade 2	0	1 (11.1)	0	0	0	1 (1.3)
Amylase increased	0	0	1 (4.5)	0	0	1 (1.3)
Grade 2	0	0	1 (4.5)	0	0	1 (1.3)
Blood creatine phosphokinase MB increased	0	0	1 (4.5)	0	0	1 (1.3)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)
Blood pressure increased	0	0	0	1 (3.2)	0	1 (1.3)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Blood urea increased	0	0	0	1 (3.2)	0	1 (1.3)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
C-reactive protein increased	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Haptoglobin decreased	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
N-terminal prohormone brain natriuretic peptide increased	0	0	1 (4.5)	0	0	1 (1.3)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)
Troponin T increased	0	1 (11.1)	0	0	0	1 (1.3)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Urine output decreased	0	0	1 (4.5)	0	0	1 (1.3)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)
Weight increased	0	0	0	1 (3.2)	0	1 (1.3)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Metabolism and nutrition disorders	0	2 (22.2)	4 (18.2)	8 (25.8)	1 (25.0)	15 (18.8)
Grade 1	0	2 (22.2)	3 (13.6)	4 (12.9)	1 (25.0)	10 (12.5)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.2.oav.age3.jp
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Grade 2	0	0	1 (4.5)	2 (6.5)	0	3 (3.8)
Grade 3	0	0	0	2 (6.5)	0	2 (2.5)
Decreased appetite	0	1 (11.1)	4 (18.2)	7 (22.6)	1 (25.0)	13 (16.3)
Grade 1	0	1 (11.1)	3 (13.6)	4 (12.9)	1 (25.0)	9 (11.3)
Grade 2	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Grade 3	0	0	0	2 (6.5)	0	2 (2.5)
Hypercholesterolaemia	0	1 (11.1)	0	0	0	1 (1.3)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Hypertriglyceridaemia	0	1 (11.1)	0	0	0	1 (1.3)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Hypophagia	0	0	0	1 (3.2)	0	1 (1.3)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Weight gain poor	0	0	1 (4.5)	0	0	1 (1.3)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)
Musculoskeletal and connective tissue disorders	0	1 (11.1)	0	0	0	1 (1.3)
Grade 2	0	1 (11.1)	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Scoliosis	0	1 (11.1)	0	0	0	1 (1.3)
Grade 2	0	1 (11.1)	0	0	0	1 (1.3)
Nervous system disorders	1 (7.1)	0	0	1 (3.2)	0	2 (2.5)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Grade 2	1 (7.1)	0	0	0	0	1 (1.3)
Cerebral atrophy	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Motor developmental delay	1 (7.1)	0	0	0	0	1 (1.3)
Grade 2	1 (7.1)	0	0	0	0	1 (1.3)
Renal and urinary disorders	0	0	0	4 (12.9)	0	4 (5.0)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)
Grade 4	0	0	0	2 (6.5)	0	2 (2.5)
Haematuria	0	0	0	2 (6.5)	0	2 (2.5)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Grade 4	0	0	0	1 (3.2)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Proteinuria	0	0	0	2 (6.5)	0	2 (2.5)
Grade 3	0	0	0	2 (6.5)	0	2 (2.5)
Acute kidney injury	0	0	0	1 (3.2)	0	1 (1.3)
Grade 4	0	0	0	1 (3.2)	0	1 (1.3)
Chronic kidney disease	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Glycosuria	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Renal impairment	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)
Renal injury	0	0	0	1 (3.2)	0	1 (1.3)
Grade 1	0	0	0	1 (3.2)	0	1 (1.3)
Respiratory, thoracic and mediastinal disorders	0	0	1 (4.5)	0	0	1 (1.3)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)
Pleural effusion	0	0	1 (4.5)	0	0	1 (1.3)
Grade 1	0	0	1 (4.5)	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC						
Preferred Term	< 3 months	≥ 3 and < 6	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Grade	(N=14)	months (N=9)	months (N=22)	months (N=31)	(N=4)	(N=80)
Skin and subcutaneous tissue disorders	0	0	0	0	1 (25.0)	1 (1.3)
Grade 1	0	0	0	0	1 (25.0)	1 (1.3)
Urticaria	0	0	0	0	1 (25.0)	1 (1.3)
Grade 1	0	0	0	0	1 (25.0)	1 (1.3)
Vascular disorders	0	0	0	2 (6.5)	0	2 (2.5)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)
Hypertension	0	0	0	2 (6.5)	0	2 (2.5)
Grade 2	0	0	0	1 (3.2)	0	1 (1.3)
Grade 3	0	0	0	1 (3.2)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Grade based on CTCAE version 5.0.

Data as of 23MAY2025: ADAE

Prg: TEAEG.SAS

Table 14.3.1.3.oav.age3.jpn
Serious OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Any Serious Adverse Event	9 (64.3)	8 (88.9)	15 (68.2)	21 (67.7)	2 (50.0)	55 (68.8)
Blood and lymphatic system disorders	0	1 (11.1)	1 (4.5)	4 (12.9)	1 (25.0)	7 (8.8)
Thrombotic microangiopathy	0	0	0	4 (12.9)	0	4 (5.0)
Thrombocytopenia	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)
Eosinophilia	0	0	0	0	1 (25.0)	1 (1.3)
Haemolytic anaemia	0	0	0	1 (3.2)	0	1 (1.3)
Cardiac disorders	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Cardiac failure congestive	0	0	0	1 (3.2)	0	1 (1.3)
Cardio-respiratory arrest	0	0	1 (4.5)	0	0	1 (1.3)
Congenital, familial and genetic disorders	2 (14.3)	0	2 (9.1)	0	0	4 (5.0)
Cryptorchism	2 (14.3)	0	2 (9.1)	0	0	4 (5.0)
Gastrointestinal disorders	2 (14.3)	2 (22.2)	4 (18.2)	1 (3.2)	0	9 (11.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.3.oav.age3.jpn
Serious OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Gastric fistula	1 (7.1)	2 (22.2)	3 (13.6)	0	0	6 (7.5)
Dysphagia	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Vomiting	1 (7.1)	0	0	1 (3.2)	0	2 (2.5)
Acetonaemic vomiting	0	0	1 (4.5)	0	0	1 (1.3)
Constipation	1 (7.1)	0	0	0	0	1 (1.3)
Gastrooesophageal reflux disease	1 (7.1)	0	0	0	0	1 (1.3)
General disorders and administration site conditions	0	1 (11.1)	1 (4.5)	2 (6.5)	0	4 (5.0)
Pyrexia	0	1 (11.1)	0	2 (6.5)	0	3 (3.8)
Inflammation	0	0	0	1 (3.2)	0	1 (1.3)
Multiple organ dysfunction syndrome	0	0	1 (4.5)	0	0	1 (1.3)
Hepatobiliary disorders	0	0	2 (9.1)	1 (3.2)	0	3 (3.8)
Hepatic function abnormal	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Acute hepatic failure	0	0	1 (4.5)	0	0	1 (1.3)
Infections and infestations	5 (35.7)	7 (77.8)	8 (36.4)	16 (51.6)	1 (25.0)	37 (46.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.3.oav.age3.jpn
Serious OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Pneumonia	2 (14.3)	4 (44.4)	1 (4.5)	6 (19.4)	0	13 (16.3)
Pneumonia aspiration	2 (14.3)	2 (22.2)	2 (9.1)	4 (12.9)	0	10 (12.5)
Upper respiratory tract infection	3 (21.4)	1 (11.1)	2 (9.1)	2 (6.5)	0	8 (10.0)
Bronchitis	0	1 (11.1)	3 (13.6)	3 (9.7)	0	7 (8.8)
COVID-19	2 (14.3)	2 (22.2)	2 (9.1)	0	0	6 (7.5)
Pneumonia respiratory syncytial viral	1 (7.1)	1 (11.1)	1 (4.5)	3 (9.7)	0	6 (7.5)
Respiratory syncytial virus infection	1 (7.1)	1 (11.1)	0	3 (9.7)	0	5 (6.3)
Bronchitis viral	0	0	1 (4.5)	2 (6.5)	0	3 (3.8)
Gastroenteritis	1 (7.1)	0	0	2 (6.5)	0	3 (3.8)
Respiratory syncytial virus bronchiolitis	0	0	2 (9.1)	1 (3.2)	0	3 (3.8)
Bronchiolitis	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Metapneumovirus infection	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)
Pneumonia viral	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)
Respiratory tract infection	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Rhinovirus infection	0	0	2 (9.1)	0	0	2 (2.5)
Viral infection	0	1 (11.1)	0	0	1 (25.0)	2 (2.5)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.3.oav.age3.jpj
Serious OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Viral upper respiratory tract infection	0	1 (11.1)	0	1 (3.2)	0	2 (2.5)
Gastroenteritis norovirus	0	0	1 (4.5)	0	0	1 (1.3)
Lower respiratory tract infection	0	0	0	1 (3.2)	0	1 (1.3)
Metapneumovirus bronchiolitis	0	0	0	1 (3.2)	0	1 (1.3)
Metapneumovirus pneumonia	0	1 (11.1)	0	0	0	1 (1.3)
Norovirus infection	0	0	1 (4.5)	0	0	1 (1.3)
Parainfluenzae virus infection	0	0	1 (4.5)	0	0	1 (1.3)
Pharyngitis	0	1 (11.1)	0	0	0	1 (1.3)
Pneumonia haemophilus	1 (7.1)	0	0	0	0	1 (1.3)
Pneumonia influenzal	0	0	0	1 (3.2)	0	1 (1.3)
Pneumonia parainfluenzae viral	1 (7.1)	0	0	0	0	1 (1.3)
Tonsillitis	0	0	1 (4.5)	0	0	1 (1.3)
Injury, poisoning and procedural complications	1 (7.1)	0	0	1 (3.2)	0	2 (2.5)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.3.oav.age3.jpn
Serious OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Femur fracture	1 (7.1)	0	0	0	0	1 (1.3)
Joint dislocation	0	0	0	1 (3.2)	0	1 (1.3)
Investigations	2 (14.3)	2 (22.2)	2 (9.1)	9 (29.0)	0	15 (18.8)
Aspartate aminotransferase increased	1 (7.1)	2 (22.2)	1 (4.5)	5 (16.1)	0	9 (11.3)
Alanine aminotransferase increased	0	1 (11.1)	0	7 (22.6)	0	8 (10.0)
Platelet count decreased	0	0	0	2 (6.5)	0	2 (2.5)
Hepatic enzyme increased	0	0	1 (4.5)	0	0	1 (1.3)
Neutrophil count decreased	1 (7.1)	0	0	0	0	1 (1.3)
Serum ferritin increased	0	0	0	1 (3.2)	0	1 (1.3)
Weight decreased	0	0	0	1 (3.2)	0	1 (1.3)
Metabolism and nutrition disorders	1 (7.1)	1 (11.1)	1 (4.5)	1 (3.2)	0	4 (5.0)
Weight gain poor	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)
Feeding disorder	1 (7.1)	0	0	0	0	1 (1.3)
Hypoglycaemia	0	0	0	1 (3.2)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.3.oav.age3.jpj
Serious OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Musculoskeletal and connective tissue disorders	0	1 (11.1)	0	0	0	1 (1.3)
Kyphosis	0	1 (11.1)	0	0	0	1 (1.3)
Scoliosis	0	1 (11.1)	0	0	0	1 (1.3)
Renal and urinary disorders	0	0	0	3 (9.7)	0	3 (3.8)
Acute kidney injury	0	0	0	1 (3.2)	0	1 (1.3)
Haematuria	0	0	0	1 (3.2)	0	1 (1.3)
Proteinuria	0	0	0	1 (3.2)	0	1 (1.3)
Renal impairment	0	0	0	1 (3.2)	0	1 (1.3)
Respiratory, thoracic and mediastinal disorders	4 (28.6)	2 (22.2)	1 (4.5)	5 (16.1)	0	12 (15.0)
Acute respiratory failure	1 (7.1)	1 (11.1)	0	2 (6.5)	0	4 (5.0)
Atelectasis	0	1 (11.1)	0	3 (9.7)	0	4 (5.0)
Respiratory disorder	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Respiratory failure	2 (14.3)	0	0	0	0	2 (2.5)
Apnoea	1 (7.1)	0	0	0	0	1 (1.3)
Aspiration	1 (7.1)	0	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.3.oav.age3.jpn
Serious OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Hypoxia	1 (7.1)	0	0	0	0	1 (1.3)
Nocturnal dyspnoea	1 (7.1)	0	0	0	0	1 (1.3)
Respiration abnormal	0	1 (11.1)	0	0	0	1 (1.3)
Upper respiratory tract inflammation	0	1 (11.1)	0	0	0	1 (1.3)
Vascular disorders	1 (7.1)	1 (11.1)	0	2 (6.5)	0	4 (5.0)
Kawasaki's disease	1 (7.1)	1 (11.1)	0	2 (6.5)	0	4 (5.0)
Not Coded	0	0	1 (4.5)	0	0	1 (1.3)
Gastrostomy	0	0	1 (4.5)	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Data as of 23MAY2025: ADAE

Prg: TEAEA.SAS

Table 14.3.1.4.oav.age3.jp
OAV101 Related Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Any OAV101 Related Adverse Event	13 (92.9)	9 (100)	22 (100)	31 (100)	4 (100)	79 (98.8)
Blood and lymphatic system disorders	0	1 (11.1)	2 (9.1)	9 (29.0)	1 (25.0)	13 (16.3)
Thrombocytopenia	0	1 (11.1)	2 (9.1)	5 (16.1)	1 (25.0)	9 (11.3)
Thrombotic microangiopathy	0	0	0	4 (12.9)	0	4 (5.0)
Haemolytic anaemia	0	0	0	1 (3.2)	0	1 (1.3)
Cardiac disorders	0	0	1 (4.5)	2 (6.5)	0	3 (3.8)
Arrhythmia	0	0	0	1 (3.2)	0	1 (1.3)
Bradycardia	0	0	1 (4.5)	0	0	1 (1.3)
Cardiac failure congestive	0	0	0	1 (3.2)	0	1 (1.3)
Gastrointestinal disorders	3 (21.4)	3 (33.3)	10 (45.5)	12 (38.7)	2 (50.0)	30 (37.5)
Vomiting	3 (21.4)	3 (33.3)	10 (45.5)	10 (32.3)	2 (50.0)	28 (35.0)
Nausea	0	0	1 (4.5)	3 (9.7)	1 (25.0)	5 (6.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.4.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC	< 3 months	≥ 3 and < 6 months	≥ 6 and < 12	≥ 12 and < 24	≥ 24 months	Total
Preferred Term	(N=14)	(N=9)	months	months	(N=4)	(N=80)
			(N=22)	(N=31)		
General disorders and administration site conditions	9 (64.3)	6 (66.7)	20 (90.9)	25 (80.6)	4 (100)	64 (80.0)
Pyrexia	9 (64.3)	6 (66.7)	20 (90.9)	25 (80.6)	4 (100)	64 (80.0)
Asthenia	0	1 (11.1)	0	1 (3.2)	0	2 (2.5)
Malaise	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Generalised oedema	0	0	0	1 (3.2)	0	1 (1.3)
Multiple organ dysfunction syndrome	0	0	1 (4.5)	0	0	1 (1.3)
Hepatobiliary disorders	2 (14.3)	2 (22.2)	4 (18.2)	2 (6.5)	1 (25.0)	11 (13.8)
Hepatic function abnormal	2 (14.3)	2 (22.2)	4 (18.2)	2 (6.5)	1 (25.0)	11 (13.8)
Acute hepatic failure	0	0	1 (4.5)	0	0	1 (1.3)
Hepatomegaly	0	0	0	0	1 (25.0)	1 (1.3)
Immune system disorders	0	0	0	1 (3.2)	0	1 (1.3)
Hypersensitivity	0	0	0	1 (3.2)	0	1 (1.3)
Infections and infestations	0	0	1 (4.5)	0	0	1 (1.3)
Bronchitis	0	0	1 (4.5)	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.4.oav.age3.jpj
OAV101 Related Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Investigations	12 (85.7)	8 (88.9)	20 (90.9)	28 (90.3)	4 (100)	72 (90.0)
Aspartate aminotransferase increased	7 (50.0)	5 (55.6)	13 (59.1)	25 (80.6)	3 (75.0)	53 (66.3)
Alanine aminotransferase increased	5 (35.7)	5 (55.6)	13 (59.1)	24 (77.4)	3 (75.0)	50 (62.5)
Platelet count decreased	3 (21.4)	5 (55.6)	10 (45.5)	19 (61.3)	3 (75.0)	40 (50.0)
Blood lactate dehydrogenase increased	0	4 (44.4)	7 (31.8)	9 (29.0)	1 (25.0)	21 (26.3)
Serum ferritin increased	1 (7.1)	3 (33.3)	5 (22.7)	4 (12.9)	2 (50.0)	15 (18.8)
Troponin I increased	6 (42.9)	2 (22.2)	2 (9.1)	1 (3.2)	1 (25.0)	12 (15.0)
Hepatic enzyme increased	1 (7.1)	2 (22.2)	2 (9.1)	3 (9.7)	0	8 (10.0)
Blood creatine phosphokinase increased	1 (7.1)	1 (11.1)	1 (4.5)	3 (9.7)	1 (25.0)	7 (8.8)
White blood cell count decreased	2 (14.3)	2 (22.2)	3 (13.6)	0	0	7 (8.8)
Gamma-glutamyltransferase increased	0	0	1 (4.5)	2 (6.5)	0	3 (3.8)
Blood creatinine increased	0	0	0	2 (6.5)	0	2 (2.5)
Haemoglobin decreased	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.4.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Neutrophil count decreased	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Weight decreased	0	1 (11.1)	0	1 (3.2)	0	2 (2.5)
Amylase increased	0	0	1 (4.5)	0	0	1 (1.3)
Blood creatine phosphokinase MB increased	0	0	1 (4.5)	0	0	1 (1.3)
Blood pressure increased	0	0	0	1 (3.2)	0	1 (1.3)
Blood urea increased	0	0	0	1 (3.2)	0	1 (1.3)
C-reactive protein increased	0	0	0	1 (3.2)	0	1 (1.3)
Haptoglobin decreased	0	0	0	1 (3.2)	0	1 (1.3)
N-terminal prohormone brain natriuretic peptide increased	0	0	1 (4.5)	0	0	1 (1.3)
Troponin T increased	0	1 (11.1)	0	0	0	1 (1.3)
Urine output decreased	0	0	1 (4.5)	0	0	1 (1.3)
Weight increased	0	0	0	1 (3.2)	0	1 (1.3)
Metabolism and nutrition disorders	0	2 (22.2)	4 (18.2)	8 (25.8)	1 (25.0)	15 (18.8)
Decreased appetite	0	1 (11.1)	4 (18.2)	7 (22.6)	1 (25.0)	13 (16.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.4.oav.age3.jp
OAV101 Related Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Hypercholesterolaemia	0	1 (11.1)	0	0	0	1 (1.3)
Hypertriglyceridaemia	0	1 (11.1)	0	0	0	1 (1.3)
Hypophagia	0	0	0	1 (3.2)	0	1 (1.3)
Weight gain poor	0	0	1 (4.5)	0	0	1 (1.3)
Musculoskeletal and connective tissue disorders	0	1 (11.1)	0	0	0	1 (1.3)
Scoliosis	0	1 (11.1)	0	0	0	1 (1.3)
Nervous system disorders	1 (7.1)	0	0	1 (3.2)	0	2 (2.5)
Cerebral atrophy	0	0	0	1 (3.2)	0	1 (1.3)
Motor developmental delay	1 (7.1)	0	0	0	0	1 (1.3)
Renal and urinary disorders	0	0	0	4 (12.9)	0	4 (5.0)
Haematuria	0	0	0	2 (6.5)	0	2 (2.5)
Proteinuria	0	0	0	2 (6.5)	0	2 (2.5)
Acute kidney injury	0	0	0	1 (3.2)	0	1 (1.3)
Chronic kidney disease	0	0	0	1 (3.2)	0	1 (1.3)
Glycosuria	0	0	0	1 (3.2)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.4.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Renal impairment	0	0	0	1 (3.2)	0	1 (1.3)
Renal injury	0	0	0	1 (3.2)	0	1 (1.3)
Respiratory, thoracic and mediastinal disorders	0	0	1 (4.5)	0	0	1 (1.3)
Pleural effusion	0	0	1 (4.5)	0	0	1 (1.3)
Skin and subcutaneous tissue disorders	0	0	0	0	1 (25.0)	1 (1.3)
Urticaria	0	0	0	0	1 (25.0)	1 (1.3)
Vascular disorders	0	0	0	2 (6.5)	0	2 (2.5)
Hypertension	0	0	0	2 (6.5)	0	2 (2.5)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Data as of 23MAY2025: ADAE

Prg: TEAEA.SAS

Table 14.3.1.5.oav.age3.jpn
OAV101 Related Serious Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Any Related Serious Adverse Event	2 (14.3)	2 (22.2)	5 (22.7)	12 (38.7)	0	21 (26.3)
Blood and lymphatic system disorders	0	1 (11.1)	1 (4.5)	4 (12.9)	0	6 (7.5)
Thrombotic microangiopathy	0	0	0	4 (12.9)	0	4 (5.0)
Thrombocytopenia	0	1 (11.1)	1 (4.5)	0	0	2 (2.5)
Haemolytic anaemia	0	0	0	1 (3.2)	0	1 (1.3)
Cardiac disorders	0	0	0	1 (3.2)	0	1 (1.3)
Cardiac failure congestive	0	0	0	1 (3.2)	0	1 (1.3)
Gastrointestinal disorders	0	0	0	1 (3.2)	0	1 (1.3)
Vomiting	0	0	0	1 (3.2)	0	1 (1.3)
General disorders and administration site conditions	0	0	1 (4.5)	2 (6.5)	0	3 (3.8)
Pyrexia	0	0	0	2 (6.5)	0	2 (2.5)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.5.oav.age3.jpn
OAV101 Related Serious Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Multiple organ dysfunction syndrome	0	0	1 (4.5)	0	0	1 (1.3)
Hepatobiliary disorders	0	0	2 (9.1)	1 (3.2)	0	3 (3.8)
Hepatic function abnormal	0	0	1 (4.5)	1 (3.2)	0	2 (2.5)
Acute hepatic failure	0	0	1 (4.5)	0	0	1 (1.3)
Investigations	2 (14.3)	2 (22.2)	2 (9.1)	9 (29.0)	0	15 (18.8)
Aspartate aminotransferase increased	1 (7.1)	2 (22.2)	1 (4.5)	5 (16.1)	0	9 (11.3)
Alanine aminotransferase increased	0	1 (11.1)	0	7 (22.6)	0	8 (10.0)
Platelet count decreased	0	0	0	2 (6.5)	0	2 (2.5)
Hepatic enzyme increased	0	0	1 (4.5)	0	0	1 (1.3)
Neutrophil count decreased	1 (7.1)	0	0	0	0	1 (1.3)
Serum ferritin increased	0	0	0	1 (3.2)	0	1 (1.3)
Renal and urinary disorders	0	0	0	3 (9.7)	0	3 (3.8)
Acute kidney injury	0	0	0	1 (3.2)	0	1 (1.3)
Haematuria	0	0	0	1 (3.2)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.5.oav.age3.jp
OAV101 Related Serious Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Proteinuria	0	0	0	1 (3.2)	0	1 (1.3)
Renal impairment	0	0	0	1 (3.2)	0	1 (1.3)

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Data as of 23MAY2025: ADAE

Prg: TEAEA.SAS

Table 14.3.1.6.1.age3.jpn
Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Hepatotoxicity ^a						
n (%)	11 (78.6)	8 (88.9)	19 (86.4)	30 (96.8)	4 (100)	72 (90.0)
n / person-year	0.24	0.24	0.25	0.24	0.21	0.24
Transient Thrombocytopenia						
n (%)	3 (21.4)	6 (66.7)	12 (54.5)	25 (80.6)	4 (100)	50 (62.5)
n / person-year	0.06	0.18	0.16	0.20	0.21	0.16

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.1.age3.jpn
Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Thrombotic microangiopathy						
n (%)	0	0	0	4 (12.9)	0	4 (5.0)
n / person-year	N/A	N/A	N/A	0.03	N/A	0.01
Cardiac Adverse Events						
n (%)	6 (42.9)	4 (44.4)	6 (27.3)	8 (25.8)	3 (75.0)	27 (33.8)
n / person-year	0.13	0.12	0.08	0.06	0.16	0.09

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Data as of 23MAY2025: ADAE, ADLB, ADSL

Prg: TEAESP2.SAS

Table 14.3.1.6.1.age3.jpn
Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Sensory Abnormalities Suggestive of Ganglionopathy						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A
New malignancies						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.1.age3.jpn
Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
New incidence of neurological disorders						
n (%)	1 (7.1)	0	1 (4.5)	1 (3.2)	0	3 (3.8)
n / person-year	0.02	N/A	0.01	0.01	N/A	0.01
New incidence of autoimmune disorders						
n (%)	0	0	0	1 (3.2)	0	1 (1.3)
n / person-year	N/A	N/A	N/A	0.01	N/A	<0.01

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.1.age3.jpn
Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
New incidence of hematological disorders						
n (%)	0	1 (11.1)	2 (9.1)	9 (29.0)	1 (25.0)	13 (16.3)
n / person-year	N/A	0.03	0.03	0.07	0.05	0.04

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.2.age3.jpn
OAV101 Related Treatment Emergent Adverse Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Hepatotoxicity ^a						
n (%)	10 (71.4)	8 (88.9)	19 (86.4)	30 (96.8)	4 (100)	71 (88.8)
n / person-year	0.21	0.24	0.25	0.24	0.21	0.23
Transient Thrombocytopenia						
n (%)	3 (21.4)	6 (66.7)	12 (54.5)	25 (80.6)	4 (100)	50 (62.5)
n / person-year	0.06	0.18	0.16	0.20	0.21	0.16

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.2.age3.jp
OAV101 Related Treatment Emergent Adverse Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Thrombotic microangiopathy						
n (%)	0	0	0	4 (12.9)	0	4 (5.0)
n / person-year	N/A	N/A	N/A	0.03	N/A	0.01
Cardiac Adverse Events						
n (%)	6 (42.9)	4 (44.4)	5 (22.7)	8 (25.8)	3 (75.0)	26 (32.5)
n / person-year	0.13	0.12	0.06	0.06	0.16	0.09

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.2.age3.jpn
OAV101 Related Treatment Emergent Adverse Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Sensory Abnormalities Suggestive of Ganglionopathy						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A
New malignancies						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.2.age3.jpn
OAV101 Related Treatment Emergent Adverse Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
New incidence of neurological disorders						
n (%)	1 (7.1)	0	0	1 (3.2)	0	2 (2.5)
n / person-year	0.02	N/A	N/A	0.01	N/A	0.01
New incidence of autoimmune disorders						
n (%)	0	0	0	1 (3.2)	0	1 (1.3)
n / person-year	N/A	N/A	N/A	0.01	N/A	<0.01

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Data as of 23MAY2025: ADAE, ADLB, ADSL

Prg: TEAESP2.SAS

Table 14.3.1.6.2.age3.jpj
OAV101 Related Treatment Emergent Adverse Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
New incidence of hematological disorders						
n (%)	0	1 (11.1)	2 (9.1)	9 (29.0)	1 (25.0)	13 (16.3)
n / person-year	N/A	0.03	0.03	0.07	0.05	0.04

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.3.age3.jpn
Serious Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Hepatotoxicity ^a						
n (%)	1 (7.1)	2 (22.2)	4 (18.2)	8 (25.8)	0	15 (18.8)
n / person-year	0.02	0.06	0.05	0.06	N/A	0.05
Transient Thrombocytopenia						
n (%)	0	1 (11.1)	1 (4.5)	3 (9.7)	0	5 (6.3)
n / person-year	N/A	0.03	0.01	0.02	N/A	0.02

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.3.age3.jpn
Serious Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Thrombotic microangiopathy n (%)	0	0	0	4 (12.9)	0	4 (5.0)
n / person-year	N/A	N/A	N/A	0.03	N/A	0.01
Cardiac Adverse Events n (%)	1 (7.1)	0	1 (4.5)	4 (12.9)	0	6 (7.5)
n / person-year	0.02	N/A	0.01	0.03	N/A	0.02

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.3.age3.jpn
Serious Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Sensory Abnormalities Suggestive of Ganglionopathy						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A
New malignancies						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.3.age3.jpn
Serious Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
New incidence of neurological disorders						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A
New incidence of autoimmune disorders						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Data as of 23MAY2025: ADAE, ADLB, ADSL

Prg: TEAESP2.SAS

Table 14.3.1.6.3.age3.jpn
Serious Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
New incidence of hematological disorders						
n (%)	0	1 (11.1)	1 (4.5)	4 (12.9)	1 (25.0)	7 (8.8)
n / person-year	N/A	0.03	0.01	0.03	0.05	0.02

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.4.age3.jpn
OAV101 Serious Related Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Hepatotoxicity ^a						
n (%)	1 (7.1)	2 (22.2)	4 (18.2)	8 (25.8)	0	15 (18.8)
n / person-year	0.02	0.06	0.05	0.06	N/A	0.05
Transient Thrombocytopenia						
n (%)	0	1 (11.1)	1 (4.5)	3 (9.7)	0	5 (6.3)
n / person-year	N/A	0.03	0.01	0.02	N/A	0.02

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.4.age3.jpn
OAV101 Serious Related Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Thrombotic microangiopathy						
n (%)	0	0	0	4 (12.9)	0	4 (5.0)
n / person-year	N/A	N/A	N/A	0.03	N/A	0.01
Cardiac Adverse Events						
n (%)	0	0	0	4 (12.9)	0	4 (5.0)
n / person-year	N/A	N/A	N/A	0.03	N/A	0.01

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.4.age3.jpj
OAV101 Serious Related Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Sensory Abnormalities Suggestive of Ganglionopathy						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A
New malignancies						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Data as of 23MAY2025: ADAE, ADLB, ADSL

Prg: TEAESP2.SAS

Table 14.3.1.6.4.age3.jpn
OAV101 Serious Related Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
New incidence of neurological disorders						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A
New incidence of autoimmune disorders						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Data as of 23MAY2025: ADAE, ADLB, ADSL

Prg: TEAESP2.SAS

Table 14.3.1.6.4.age3.jp
OAV101 Serious Related Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
New incidence of hematological disorders						
n (%)	0	1 (11.1)	1 (4.5)	4 (12.9)	0	6 (7.5)
n / person-year	N/A	0.03	0.01	0.03	N/A	0.02

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.

Adverse Events were coded using MedDRA, version &medraver..

Patients are only counted once at each level of summarization.

Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.

^a Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.2.age3.jp
Clinically Significant Abnormal Laboratory Results any Time During Follow-up by Age at OAV101 Infusion
Japan Safety Analysis Set

Laboratory Test	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Albumin						
n	1	5	14	16	3	39
Yes, n (%)	0	0	0	2 (12.5)	1 (33.3)	3 (7.7)
No, n (%)	1 (100)	5 (100)	14 (100)	14 (87.5)	2 (66.7)	36 (92.3)
Aspartate aminotransferase (AST)						
n	8	8	21	26	3	66
Yes, n (%)	6 (75.0)	5 (62.5)	14 (66.7)	21 (80.8)	3 (100)	49 (74.2)
No, n (%)	2 (25.0)	3 (37.5)	7 (33.3)	5 (19.2)	0	17 (25.8)
Alanine aminotransferase (ALT)						
n	7	8	21	25	3	64
Yes, n (%)	5 (71.4)	4 (50.0)	15 (71.4)	21 (84.0)	3 (100)	48 (75.0)
No, n (%)	2 (28.6)	4 (50.0)	6 (28.6)	4 (16.0)	0	16 (25.0)
Alkaline phosphatase						
n	0	4	13	12	3	32
Yes, n (%)		0	0	0	0	0
No, n (%)		4 (100)	13 (100)	12 (100)	3 (100)	32 (100)

Note: Percent is based on the non-missing responses for each laboratory parameter.

Note: Baseline assessments occurring within 15 days after OAV101 treatment are not included in this table.

Table 14.3.2.age3.jp
Clinically Significant Abnormal Laboratory Results any Time During Follow-up by Age at OAV101 Infusion
Japan Safety Analysis Set

Laboratory Test	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
Total Bilirubin						
n	1	6	14	15	3	39
Yes, n (%)	0	0	0	0	0	0
No, n (%)	1 (100)	6 (100)	14 (100)	15 (100)	3 (100)	39 (100)
Direct Bilirubin						
n	1	3	4	5	1	14
Yes, n (%)	0	0	0	0	0	0
No, n (%)	1 (100)	3 (100)	4 (100)	5 (100)	1 (100)	14 (100)
Total Protein						
n	1	6	15	15	3	40
Yes, n (%)	0	0	0	2 (13.3)	0	2 (5.0)
No, n (%)	1 (100)	6 (100)	15 (100)	13 (86.7)	3 (100)	38 (95.0)
Platelets						
n	2	7	17	21	3	50
Yes, n (%)	0	3 (42.9)	7 (41.2)	11 (52.4)	3 (100)	24 (48.0)
No, n (%)	2 (100)	4 (57.1)	10 (58.8)	10 (47.6)	0	26 (52.0)

Note: Percent is based on the non-missing responses for each laboratory parameter.

Note: Baseline assessments occurring within 15 days after OAV101 treatment are not included in this table.

Table 14.3.2.age3.jpn
Clinically Significant Abnormal Laboratory Results any Time During Follow-up by Age at OAV101 Infusion
Japan Safety Analysis Set

Laboratory Test	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=22)	≥ 12 and < 24 months (N=31)	≥ 24 months (N=4)	Total (N=80)
White Blood Count (WBC)						
n	2	8	16	17	3	46
Yes, n (%)	1 (50.0)	4 (50.0)	3 (18.8)	3 (17.6)	1 (33.3)	12 (26.1)
No, n (%)	1 (50.0)	4 (50.0)	13 (81.3)	14 (82.4)	2 (66.7)	34 (73.9)
Red Blood Count (RBC)						
n	1	6	15	17	3	42
Yes, n (%)	0	2 (33.3)	0	1 (5.9)	0	3 (7.1)
No, n (%)	1 (100)	4 (66.7)	15 (100)	16 (94.1)	3 (100)	39 (92.9)
Hemoglobin						
n	1	6	15	17	3	42
Yes, n (%)	0	2 (33.3)	0	3 (17.6)	0	5 (11.9)
No, n (%)	1 (100)	4 (66.7)	15 (100)	14 (82.4)	3 (100)	37 (88.1)
Hematocrit (HCT)						
n	1	6	14	15	3	39
Yes, n (%)	0	2 (33.3)	0	2 (13.3)	0	4 (10.3)
No, n (%)	1 (100)	4 (66.7)	14 (100)	13 (86.7)	3 (100)	35 (89.7)

Note: Percent is based on the non-missing responses for each laboratory parameter.

Note: Baseline assessments occurring within 15 days after OAV101 treatment are not included in this table.

Table 14.3.3.jpj
Patients with Elevated AST or ALT After OAV101 Infusion
Japan Safety Analysis Set

Elevated AST or ALT	OAV101 mono (N=24) n (%)	Add-on (N=2) n (%)	Transient add-on (N=1) n (%)	Combo w/OAV101 (N=10) n (%)	Bridge to OAV101 (N=28) n (%)	Switch to OAV101 (N=15) n (%)	Total OAV101 (N=80) n (%)
0-<3 months after OAV101 Infusion	22 (91.7)	2 (100)	1 (100)	9 (90.0)	24 (85.7)	15 (100)	73 (91.3)
3-<6 months after OAV101 Infusion	8 (33.3)	0	0	3 (30.0)	3 (10.7)	1 (6.7)	15 (18.8)
6 months-<1 year after OAV101 Infusion	2 (8.3)	0	0	2 (20.0)	3 (10.7)	6 (40.0)	13 (16.3)
1-<1.5 years after OAV101 Infusion	1 (4.2)	0	0	1 (10.0)	2 (7.1)	0	4 (5.0)
1.5-<2 years after OAV101 Infusion	1 (4.2)	0	0	1 (10.0)	0	0	2 (2.5)
2-<3 years after OAV101 Infusion	0	0	0	1 (10.0)	0	0	1 (1.3)
3-<4 years after OAV101 Infusion	0	0	0	1 (10.0)	0	0	1 (1.3)

A patient may be reported in multiple time frames.

Table 14.3.3.jp
Patients with Elevated AST or ALT After OAV101 Infusion
Japan Safety Analysis Set

	OAV101 mono (N=24) n (%)	Add-on (N=2) n (%)	Transient add-on (N=1) n (%)	Combo w/OAV101 (N=10) n (%)	Bridge to OAV101 (N=28) n (%)	Switch to OAV101 (N=15) n (%)	Total OAV101 (N=80) n (%)
Elevated AST or ALT							
At Any Time Post OAV101 Infusion	22 (91.7)	2 (100)	1 (100)	10 (100)	24 (85.7)	15 (100)	74 (92.5)

A patient may be reported in multiple time frames.

安全性検討事項の各リスクの定義

安全性検討事項	定義
Hepatotoxicity	肝障害 (Broad) (SMQ)
Transient thrombocytopenia	出血 (Broad) (SMQ)
	造血障害による血小板減少症 (Broad) (SMQ)
	血小板障害 N E C (HLT)
Thrombotic microangiopathy	血栓性微小血管症 (PT)
	溶血性尿毒症症候群 (PT)
	非定型溶血性尿毒症症候群 (PT)
Cardiac events	心筋症 (Broad) (SMQ)
	虚血性心疾患 (Broad) (SMQ)
	不整脈 (Broad) (SMQ)
	塞栓および血栓 (Broad) (SMQ)
	心筋梗塞 (Broad) (SMQ)
	心不全 (Broad) (SMQ)
	高血圧 (Broad) (SMQ)
Sensory Abnormalities Suggestive of Ganglionopathy	運動感覚消失 (PT)
	異痛症 (PT)
	無感覚 (PT)
	有痛性感覚消失 (PT)
	反射消失 (PT)
	灼熱足症候群 (PT)
	灼熱感 (PT)
	中枢痛症候群 (PT)
	複合性局所疼痛症候群 (PT)
	振動覚低下 (PT)
	不快感 (PT)
	異常感覚 (PT)
	蟻走感 (PT)
	知覚過敏 (PT)
	ヒベルパチー (PT)
	感覚鈍麻 (PT)
	反射減弱 (PT)
	刺激反応低下 (PT)
	肋間神経痛 (PT)
	関節位置覚低下 (PT)
	固有感覚の欠如 (PT)
	神経伝導検査異常 (PT)
	神経刺激検査異常 (PT)
	神経痛 (PT)
	神経炎 (PT)
	神経学的症状 (PT)
	神経筋痛 (PT)
	背部異常感覚 (PT)
	疼痛 (PT)
	痛覚閾値低下 (PT)
	逆説疼痛 (PT)
	錯感覚 (PT)
	末梢神経系機能検査異常 (PT)
	末梢性感覚運動ニューロパチー (PT)
	末梢性感覚ニューロパチー (PT)
	神経根痛 (PT)
	反射試験異常 (PT)
	反射異常 (PT)
	熱感－冷感逆転 (PT)
	感覚運動障害 (PT)
	感覚障害 (PT)
	感覚神経節炎 (PT)
	感覚統合機能障害 (PT)
	感覚レベル異常 (PT)
	感覚消失 (PT)
	皮膚灼熱感 (PT)
	刺激反応遅滞 (PT)
	共感覚 (PT)
	温度覚検査値低下 (PT)
	圧痛 (PT)
	温度覚消失 (PT)
	温度覚鈍麻 (PT)
	ティネル徴候 (PT)
	毛髮痛 (PT)
	刺激無反応 (PT)
	振動覚亢進 (PT)
New malignancies	良性、悪性および詳細不明の新生物（嚢胞およびポリープを含む） (SOC)
New incidence of neurological disorders	神経系障害 (SOC)
New incidence of autoimmune disorders	免疫系障害 (SOC)
New incidence of hematological disorders	血液およびリンパ系障害 (SOC)