Clinical trial results:
A Phase 1/2 Open-label, Multiple-dose, Dose-escalating Clinical Trial of the Safety and Tolerability of GTX-102 in Pediatric Patients With Angelman Syndrome (AS)

Summary

EudraCT number	2021-001793-36
Trial protocol	DE ES FR
Global end of trial date	08 January 2025

Results information

Result version number	v1
This version publication date	23 January 2026
First version publication date	23 January 2026

Trial information

Trial identification

Sponsor protocol code	GTX-102-001

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT04259281
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Ultragenyx Pharmaceutical Inc.
Sponsor organisation address	60 Leveroni Court, Novato, United States, 94949
Public contact	Trial Recruitment, Ultragenyx Pharmaceutical Inc., +1 8887568657, trialrecruitment@ultragenyx.com
Scientific contact	Medical Information, Ultragenyx Pharmaceutical Inc., +1 8887568657, medinfo@ultragenyx.com

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	No
Does article 45 of REGULATION (EC) No 1901/2006 apply to this trial?	No
Does article 46 of REGULATION (EC) No 1901/2006 apply to this trial?	No

Notes:

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	08 January 2025
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	08 January 2025
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To evaluate the safety and tolerability of multiple-ascending doses of GTX-102 administered by intrathecal (IT) injection to patients with AS.

Protection of trial subjects:

All participants, parents and/or legal guardians of the participant will be provided with an Information Sheet and/or Consent Form describing this study and providing sufficient information to make an informed decision about the participation of their represented participant in this study. Where required, consent will be sought from the parents and/or legal guardian of the participant. The Informed Consent Forms (ICFs) and Patient Informed Consent Forms (PICFs) will include all elements required by ICH, Good Clinical Practice (GCP) and applicable regulatory requirements. The Information Sheet and/or Consent Form will be submitted with the protocol for review and approval by the relevant Ethical Review Board for the study in each country. The formal consent of a participant, using the Informed Consent Form approved by the relevant Ethical Review Board, must be obtained before that participant undergoes any study procedure. The consent form must be signed by the participant's parent/legal guardian, and the Investigator-designated research professional obtaining the consent. Where required by the relevant Ethical Review Board, a witness may sign the consent form.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	24 February 2020
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Spain: 5
Country: Number of subjects enrolled	France: 7
Country: Number of subjects enrolled	Germany: 4
Country: Number of subjects enrolled	Australia: 5
Country: Number of subjects enrolled	Canada: 20
Country: Number of subjects enrolled	United Kingdom: 7
Country: Number of subjects enrolled	Israel: 2
Country: Number of subjects enrolled	United States: 24
Worldwide total number of subjects	74
EEA total number of subjects	16

Notes:

Subjects enrolled per age group	
In utero	0
Preterm newborn - gestational age < 37 wk	0
Newborns (0-27 days)	0
Infants and toddlers (28 days-23 months)	0
Children (2-11 years)	60
Adolescents (12-17 years)	14
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

A total of 74 participants were enrolled sequentially into 13 cohorts. After a dosing pause, participants enrolled in Cohorts 1 & 2 restarted in a redosing cohort. The Cohort 3 participant continued to be followed in an expanded access trial.

Period 1

Period 1 title	Original Dosing Period
Is this the baseline period?	No
Allocation method	Non-randomised - controlled
Blinding used	Not blinded

Arms

Arm title	GTX-102 Cohorts 1-3

Arm description:

Participants received doses ranging from 3.3 to 20 mg followed by dose escalation up to a maximum of 36 mg.

Arm type	Experimental
Investigational medicinal product name	GTX-102
Investigational medicinal product code	GTX-102
Other name	apazunursen
Pharmaceutical forms	Solution for injection
Routes of administration	Intrathecal use

Dosage and administration details:

GTX-102 is given through an intrathecal injection via lumbar puncture.

Number of subjects in period 1	GTX-102 Cohorts 1-3
Started	5
Completed	0
Not completed	5
Adverse event, non-fatal	5

Period 2

Period 2 title	Amended Dosing Period
Is this the baseline period?	Yes[1]
Allocation method	Non-randomised - controlled
Blinding used	Not blinded

Arms

Are arms mutually exclusive?	No

Arm title	GTX-102 US Redosing Cohorts 1-2

Arm description:

Participants from Cohorts 1-3 were restarted at doses ranging from 5 to 10 mg followed by dose escalation up to a maximum dose of 14 mg Q3M.

Arm type	Experimental
Investigational medicinal product name	GTX-102
Investigational medicinal product code	GTX-102
Other name	apazunursen
Pharmaceutical forms	Solution for injection
Routes of administration	Intrathecal use

Dosage and administration details:

GTX-102 is given through an intrathecal injection via lumbar puncture.

Arm title	GTX-102 Cohorts 4-7

Arm description:

Participants received up to 4 loading doses ranging from 3.3 to 10 mg followed by dose escalation up to a maximum dose of 14 mg Q3M.

Arm type	Experimental
Investigational medicinal product name	GTX-102
Investigational medicinal product code	GTX-102
Other name	apazunursen
Pharmaceutical forms	Solution for injection
Routes of administration	Intrathecal use

Dosage and administration details:

GTX-102 is given through an intrathecal injection via lumbar puncture.

Arm title	GTX-102 Cohorts A&B

Arm description:

Participants received up to 4 loading doses of 7.5 mg followed by dose escalation up to a maximum dose of 14 mg Q3M.

Arm type	Experimental
Investigational medicinal product name	GTX-102
Investigational medicinal product code	GTX-102
Other name	apazunursen
Pharmaceutical forms	Solution for injection
Routes of administration	Intrathecal use

Dosage and administration details:

GTX-102 is given through an intrathecal injection via lumbar puncture.

Arm title	GTX-102 Cohorts C&D

Arm description:

Participants received up to 4 loading doses ranging from 5 to 7.5 mg followed by dose escalation up to a maximum dose of 10 mg Q3M.

Arm type	Experimental
Investigational medicinal product name	GTX-102
Investigational medicinal product code	GTX-102
Other name	apazunursen
Pharmaceutical forms	Solution for injection
Routes of administration	Intrathecal use

Dosage and administration details:

GTX-102 is given through an intrathecal injection via lumbar puncture.

Arm title	GTX-102 US 2 mg/Cohort E

Arm description:

Participants received either no treatment or GTX-102. Participants treated with GTX-102 received 4 monthly loading doses of 2 mg, followed by 2 mg Q3M. Participants who received no treatment during

the initial period then received 4 monthly doses of 2 mg, followed by 2 mg Q3M. All participants then transitioned to Cohort E regardless of prior GTX-102 treatment duration and received up to 4 loading doses of 5 to 7.5 mg, followed by 7.5 mg every 2 months (Q2M).

Arm type	Experimental
Investigational medicinal product name	GTX-102
Investigational medicinal product code	GTX-102
Other name	apazunursen
Pharmaceutical forms	Solution for injection
Routes of administration	Intrathecal use

Dosage and administration details:

GTX-102 is given through an intrathecal injection via lumbar puncture.

Notes:

[1] - Period 1 is not the baseline period. It is expected that period 1 will be the baseline period.

Justification: Period 2 is defined as the baseline.

Number of subjects in period 2	GTX-102 US Redosing Cohorts 1-2	GTX-102 Cohorts 4-7	GTX-102 Cohorts A&B
Started	4	15	34
Completed	4	13	31
Not completed	0	2	3
Consent withdrawn by subject	-	1	1
Adverse event, non-fatal	-	1	1
Parental Decision	-	-	1

Number of subjects in period 2	GTX-102 Cohorts C&D	GTX-102 US 2 mg/Cohort E
Started	12	8
Completed	12	7
Not completed	0	1
Consent withdrawn by subject	-	1
Adverse event, non-fatal	-	-
Parental Decision	-	-

Baseline characteristics

Reporting groups[1]	
Reporting group title	GTX-102 US Redosing Cohorts 1-2
Reporting group description:	
Participants from Cohorts 1-3 were restarted at doses ranging from 5 to 10 mg followed by dose escalation up to a maximum dose of 14 mg Q3M.	
Reporting group title	GTX-102 Cohorts 4-7
Reporting group description:	
Participants received up to 4 loading doses ranging from 3.3 to 10 mg followed by dose escalation up to a maximum dose of 14 mg Q3M.	
Reporting group title	GTX-102 Cohorts A&B
Reporting group description:	
Participants received up to 4 loading doses of 7.5 mg followed by dose escalation up to a maximum dose of 14 mg Q3M.	
Reporting group title	GTX-102 Cohorts C&D
Reporting group description:	
Participants received up to 4 loading doses ranging from 5 to 7.5 mg followed by dose escalation up to a maximum dose of 10 mg Q3M.	
Reporting group title	GTX-102 US 2 mg/Cohort E
Reporting group description:	
Participants received either no treatment or GTX-102. Participants treated with GTX-102 received 4 monthly loading doses of 2 mg, followed by 2 mg Q3M. Participants who received no treatment during the initial period then received 4 monthly doses of 2 mg, followed by 2 mg Q3M. All participants then transitioned to Cohort E regardless of prior GTX-102 treatment duration and received up to 4 loading doses of 5 to 7.5 mg, followed by 7.5 mg every 2 months (Q2M).	

Notes:

[1] - The number of subjects reported to be in the baseline period is not equal to the worldwide number of subjects enrolled in the trial. It is expected that these numbers will be the same.
Justification: A total of 74 subjects were enrolled worldwide across 2 periods.

Reporting group values	GTX-102 US Redosing Cohorts 1-2	GTX-102 Cohorts 4-7	GTX-102 Cohorts A&B
Number of subjects	4	15	34
Age categorical Units: Subjects			
Age continuous Units: years			
arithmetic mean	13.98	8.4	7.8
standard deviation	± 4.4	± 3.9	± 3.8
Gender categorical Units: Subjects			
Female	2	8	21
Male	2	7	13
Race Units: Subjects			
Not Allowed per Local Regulation	0	0	7
American Indian or Alaska Native	0	0	0
Asian	0	2	1
Black or African American	0	0	1
Native Hawaiian or Other Pacific Islander	0	0	1
White	4	7	23

Other	0	6	1

Reporting group values	GTX-102 Cohorts C&D	GTX-102 US 2 mg/Cohort E	Total
Number of subjects	12	8	73
Age categorical Units: Subjects			
Age continuous Units: years			
arithmetic mean	8.8	5.9	
standard deviation	± 4.3	± 1.2	-
Gender categorical Units: Subjects			
Female	9	5	45
Male	3	3	28
Race Units: Subjects			
Not Allowed per Local Regulation	0	0	7
American Indian or Alaska Native	0	0	0
Asian	1	1	5
Black or African American	0	0	1
Native Hawaiian or Other Pacific Islander	0	0	1
White	11	7	48
Other	0	0	7

Subject analysis sets

Subject analysis set title	GTX-102 Cohorts 1-3
Subject analysis set type	Per protocol

Subject analysis set description:

Participants received doses of 3.3 mg to 20 mg of GTX-102 which were escalated up to a maximum maintenance dose of 36 mg.

Reporting group values	GTX-102 Cohorts 1-3		
Number of subjects	5		
Age categorical Units: Subjects			
Age continuous Units: years			
arithmetic mean	9.4		
standard deviation	± 4.4		
Gender categorical Units: Subjects			
Female	2		
Male	3		

Race Units: Subjects			
Not Allowed per Local Regulation	0		
American Indian or Alaska Native	0		
Asian	0		
Black or African American	0		
Native Hawaiian or Other Pacific Islander	0		
White	5		
Other	0		

End points

End points reporting groups

Reporting group title	GTX-102 Cohorts 1-3
Reporting group description:	
Participants received doses ranging from 3.3 to 20 mg followed by dose escalation up to a maximum of 36 mg.	
Reporting group title	GTX-102 US Redosing Cohorts 1-2
Reporting group description:	
Participants from Cohorts 1-3 were restarted at doses ranging from 5 to 10 mg followed by dose escalation up to a maximum dose of 14 mg Q3M.	
Reporting group title	GTX-102 Cohorts 4-7
Reporting group description:	
Participants received up to 4 loading doses ranging from 3.3 to 10 mg followed by dose escalation up to a maximum dose of 14 mg Q3M.	
Reporting group title	GTX-102 Cohorts A&B
Reporting group description:	
Participants received up to 4 loading doses of 7.5 mg followed by dose escalation up to a maximum dose of 14 mg Q3M.	
Reporting group title	GTX-102 Cohorts C&D
Reporting group description:	
Participants received up to 4 loading doses ranging from 5 to 7.5 mg followed by dose escalation up to a maximum dose of 10 mg Q3M.	
Reporting group title	GTX-102 US 2 mg/Cohort E
Reporting group description:	
Participants received either no treatment or GTX-102. Participants treated with GTX-102 received 4 monthly loading doses of 2 mg, followed by 2 mg Q3M. Participants who received no treatment during the initial period then received 4 monthly doses of 2 mg, followed by 2 mg Q3M. All participants then transitioned to Cohort E regardless of prior GTX-102 treatment duration and received up to 4 loading doses of 5 to 7.5 mg, followed by 7.5 mg every 2 months (Q2M).	
Subject analysis set title	GTX-102 Cohorts 1-3
Subject analysis set type	Per protocol
Subject analysis set description:	
Participants received doses of 3.3 mg to 20 mg of GTX-102 which were escalated up to a maximum maintenance dose of 36 mg.	

Primary: Number of Participants with Treatment-emergent Adverse Events (TEAEs), Serious TEAEs (SAEs), Adverse Events of Special Interest (AESIs), TEAEs Leading to Discontinuation and TEAEs by Severity

End point title	Number of Participants with Treatment-emergent Adverse Events (TEAEs), Serious TEAEs (SAEs), Adverse Events of Special Interest (AESIs), TEAEs Leading to Discontinuation and TEAEs by Severity[1]
End point description:	
An AE is defined as any untoward medical occurrence associated with the use of a drug in humans, whether or not considered drug related. A TEAE is defined as any AE not present prior to the initiation of the drug treatment or any AE already present that worsens in either intensity or frequency following exposure to the drug treatment. An SAE is an AE that meets any of the following criteria in the view of either the Investigator or Ultragenyx: death; life-threatening; inpatient hospitalization or prolongation of existing hospitalization; disability/Incapacity; congenital anomaly/birth defect not present at screening; other important medical events. Severity of events were graded as mild (grade1), moderate (grade 2), severe (grade 3), life-threatening (grade 4), or death (grade 5). An AESI is an event of scientific or medical interest specific to the drug treatment requiring further investigation and close monitoring to further characterize them.	
End point type	Primary
End point timeframe:	
From the first dose of study drug through the last dose of study drug plus 42 (±14) days, for an overall	

mean study duration of 604.1 days.

Notes:

[1] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Descriptive statistics are presented per protocol.

End point values	GTX-102 Cohorts 1-3	GTX-102 US Redosing Cohorts 1-2	GTX-102 Cohorts 4-7	GTX-102 Cohorts A&B
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	5	4	15	34
Units: number of participants				
Treatment-Emergent Adverse Event (TEAE)	5	2	15	34
Related TEAE	5	0	4	17
Serious TEAE	5	0	6	10
Serious Related TEAE	5	0	1	2
Grade 3 or 4 Related TEAE	1	0	1	2
TEAE with Maximum Severity Grade 5	0	0	0	0
TEAE with Maximum Severity Grade 4	0	0	0	1
TEAE with Maximum Severity Grade 3	1	0	3	3
TEAE with Maximum Severity Grade 2	4	1	9	24
TEAE with Maximum Severity Grade 1	0	1	3	6
Adverse Event of Special Interest	5	0	1	2
TEAE Leading to Dose Reduced	0	0	0	4
TEAE Leading to Dose Interruption	1	0	3	2
TEAE Leading to Dose Withdrawn	0	0	1	2
TEAE Leading to Study Discontinuation	0	0	1	1
TEAE Leading to Death	0	0	0	0

End point values	GTX-102 Cohorts C&D	GTX-102 US 2 mg/Cohort E		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	12	8		
Units: number of participants				
Treatment-Emergent Adverse Event (TEAE)	12	8		
Related TEAE	4	1		
Serious TEAE	4	5		
Serious Related TEAE	0	0		
Grade 3 or 4 Related TEAE	0	0		
TEAE with Maximum Severity Grade 5	0	0		
TEAE with Maximum Severity Grade 4	0	0		
TEAE with Maximum Severity Grade 3	1	1		
TEAE with Maximum Severity Grade 2	8	6		
TEAE with Maximum Severity Grade 1	3	1		
Adverse Event of Special Interest	0	0		
TEAE Leading to Dose Reduced	0	0		
TEAE Leading to Dose Interruption	2	1		
TEAE Leading to Dose Withdrawn	0	0		
TEAE Leading to Study Discontinuation	0	0		

TEAE Leading to Death	0	0		

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

From signing of informed consent (SAEs) or first dose of study drug (non-serious AEs) through the last dose of study drug plus 42 (±14) days, for an overall mean study duration of 604.1 days.

Assessment type	Systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	27.1

Reporting groups

Reporting group title	GTX-102 Cohorts 1 - 3

Reporting group description:

Participants received doses ranging from 3.3 to 20 mg followed by dose escalation up to a maximum of 36 mg.

Reporting group title	GTX-102 US Redosing Cohorts 1 - 2

Reporting group description:

Participants from Cohorts 1-3 were restarted at doses ranging from 5 to 10 mg followed by dose escalation up to a maximum dose of 14 mg Q3M.

Reporting group title	GTX-102 US 2mg/Cohort E

Reporting group description:

Participants received either no treatment or GTX-102. Participants treated with GTX-102 received 4 monthly loading doses of 2 mg, followed by 2 mg Q3M. Participants who received no treatment during the initial period then received 4 monthly doses of 2 mg, followed by 2 mg Q3M. All participants then transitioned to Cohort E regardless of prior GTX-102 treatment duration and received up to 4 loading doses of 5 to 7.5 mg, followed by 7.5 mg every 2 months (Q2M).

Reporting group title	GTX-102 Cohorts A & B

Reporting group description:

Participants received up to 4 loading doses of 7.5 mg followed by dose escalation up to a maximum dose of 14 mg Q3M.

Reporting group title	GTX-102 Cohorts C & D

Reporting group description:

Participants received up to 4 loading doses ranging from 5 to 7.5 mg followed by dose escalation up to a maximum dose of 10 mg Q3M.

Reporting group title	GTX-102 Cohorts 4 - 7

Reporting group description:

Participants received up to 4 loading doses ranging from 3.3 to 10 mg followed by dose escalation up to a maximum dose of 14 mg Q3M.

Serious adverse events	GTX-102 Cohorts 1 - 3	GTX-102 US Redosing Cohorts 1 - 2	GTX-102 US 2mg/Cohort E
Total subjects affected by serious adverse events			
subjects affected / exposed	5 / 5 (100.00%)	0 / 4 (0.00%)	5 / 8 (62.50%)
number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events	0	0	0
Injury, poisoning and procedural complications			
Humerus Fracture			

subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Post Lumbar Puncture Syndrome			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Skin Laceration			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Wound Decomposition			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nervous system disorders			
Epilepsy			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Febrile Convulsion			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Radiculopathy			
subjects affected / exposed	5 / 5 (100.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences causally related to treatment / all	5 / 5	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Seizure			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Seizure Cluster			

subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Status Epilepticus			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastrointestinal disorders			
Vomiting			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pancreatitis Acute			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pancreatitis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Intestinal Obstruction			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Faecaloma			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Constipation			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Respiratory, thoracic and mediastinal disorders			

Acute Respiratory Failure			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Choking			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Musculoskeletal and connective tissue disorders			
Muscular Weakness			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infections and infestations			
Streptococcal Infection			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Medical Device Site Infection			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Herpes Zoster Meningitis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastroenteritis Viral			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Metabolism and nutrition disorders			
Hypophagia			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Serious adverse events	GTX-102 Cohorts A & B	GTX-102 Cohorts C & D	GTX-102 Cohorts 4 - 7
Total subjects affected by serious adverse events			
subjects affected / exposed	10 / 34 (29.41%)	4 / 12 (33.33%)	6 / 15 (40.00%)
number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events	0	0	0
Injury, poisoning and procedural complications			
Humerus Fracture			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Post Lumbar Puncture Syndrome			
subjects affected / exposed	3 / 34 (8.82%)	0 / 12 (0.00%)	2 / 15 (13.33%)
occurrences causally related to treatment / all	0 / 3	0 / 0	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Skin Laceration			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Wound Decomposition			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nervous system disorders			
Epilepsy			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Febrile Convulsion			

subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Radiculopathy			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences causally related to treatment / all	1 / 1	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Seizure			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Seizure Cluster			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Status Epilepticus			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastrointestinal disorders			
Vomiting			
subjects affected / exposed	0 / 34 (0.00%)	1 / 12 (8.33%)	1 / 15 (6.67%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pancreatitis Acute			
subjects affected / exposed	0 / 34 (0.00%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pancreatitis			
subjects affected / exposed	0 / 34 (0.00%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Intestinal Obstruction			

subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Faecaloma			
subjects affected / exposed	0 / 34 (0.00%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Constipation			
subjects affected / exposed	0 / 34 (0.00%)	1 / 12 (8.33%)	1 / 15 (6.67%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Respiratory, thoracic and mediastinal disorders			
Acute Respiratory Failure			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Choking			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Musculoskeletal and connective tissue disorders			
Muscular Weakness			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infections and infestations			
Streptococcal Infection			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia			

subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	2 / 15 (13.33%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Medical Device Site Infection			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Herpes Zoster Meningitis			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastroenteritis Viral			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Metabolism and nutrition disorders			
Hypophagia			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Frequency threshold for reporting non-serious adverse events: 5 %

Non-serious adverse events	GTX-102 Cohorts 1 - 3	GTX-102 US Redosing Cohorts 1 - 2	GTX-102 US 2mg/Cohort E
Total subjects affected by non-serious adverse events			
subjects affected / exposed	5 / 5 (100.00%)	2 / 4 (50.00%)	8 / 8 (100.00%)
Vascular disorders			
Hypotension			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
General disorders and administration site conditions			
Asthenia			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0

Decreased Activity			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Decreased Gait Velocity			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Fatigue			
subjects affected / exposed	2 / 5 (40.00%)	1 / 4 (25.00%)	1 / 8 (12.50%)
occurrences (all)	3	1	1
Gait Disturbance			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	2 / 8 (25.00%)
occurrences (all)	0	0	2
Influenza Like Illness			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Pain			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Pyrexia			
subjects affected / exposed	1 / 5 (20.00%)	1 / 4 (25.00%)	3 / 8 (37.50%)
occurrences (all)	1	1	3
Malaise			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Immune system disorders			
Hypersensitivity			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Reproductive system and breast disorders			
Bartholin's Cyst			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Dysmenorrhoea			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Respiratory, thoracic and mediastinal disorders			

Adenoidal Hypertrophy			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Dyspnoea			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Epistaxis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Nasal Congestion			
subjects affected / exposed	0 / 5 (0.00%)	1 / 4 (25.00%)	0 / 8 (0.00%)
occurrences (all)	0	1	0
Rhinorrhoea			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Tonsillar Hypertrophy			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Cough			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Psychiatric disorders			
Agitation			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Abnormal Behaviour			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Aggression			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Insomnia			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	1
Irritability			

subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Investigations			
Blood Alkaline Phosphatase Increased			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Blood Pressure Diastolic Decreased			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Body Temperature Increased			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Csf Immunoglobulin Increased			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Csf Pressure Increased			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	1
Csf Protein Increased			
subjects affected / exposed	3 / 5 (60.00%)	0 / 4 (0.00%)	2 / 8 (25.00%)
occurrences (all)	3	0	2
Monocyte Count Increased			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Csf White Blood Cell Count Increased			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Culture Urine Positive			
subjects affected / exposed	1 / 5 (20.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	1	0	0
Glucose Urine Present			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
International Normalised Ratio Increased			

subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Csf Red Blood Cell Count Positive			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Neutrophil Count Decreased			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Vitamin D Decreased			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Weight Decreased			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Weight Increased			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
White Blood Cell Count Increased			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Injury, poisoning and procedural complications			
Head Injury			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Accidental Exposure To Product			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	1
Arthropod Bite			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	2 / 8 (25.00%)
occurrences (all)	0	0	2
Contusion			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Eye Contusion			

subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Fall			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	1
Foreign Body In Respiratory Tract			
subjects affected / exposed	1 / 5 (20.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	1	0	0
Skin Laceration			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Soft Tissue Injury			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Thermal Burn			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Tooth Fracture			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Wrist Fracture			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Joint Injury			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Ligament Sprain			
subjects affected / exposed	1 / 5 (20.00%)	1 / 4 (25.00%)	0 / 8 (0.00%)
occurrences (all)	1	1	0
Limb Injury			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	1
Lip Injury			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Post Lumbar Puncture Syndrome			

subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Procedural Pain			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Cardiac disorders			
Tachycardia			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Nervous system disorders			
Ataxia			
subjects affected / exposed	5 / 5 (100.00%)	0 / 4 (0.00%)	3 / 8 (37.50%)
occurrences (all)	11	0	4
Atonic Seizures			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Balance Disorder			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Migraine			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Drooling			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Epilepsy			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Headache			
subjects affected / exposed	2 / 5 (40.00%)	1 / 4 (25.00%)	1 / 8 (12.50%)
occurrences (all)	3	1	1
Hyperreflexia			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	2
Hypotonia			

subjects affected / exposed	1 / 5 (20.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	1	0	0
Lethargy			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Disturbance In Attention			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Myoclonus			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Petit Mal Epilepsy			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Post-Traumatic Epilepsy			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Radicular Pain			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Radiculopathy			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Seizure			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	2 / 8 (25.00%)
occurrences (all)	0	0	2
Sensory Disturbance			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Somnolence			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Syncope			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Seizure Like Phenomena			

subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	1
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Lymphocytosis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Ear and labyrinth disorders			
Ear Discomfort			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Ear Haemorrhage			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Middle Ear Effusion			
subjects affected / exposed	0 / 5 (0.00%)	1 / 4 (25.00%)	0 / 8 (0.00%)
occurrences (all)	0	1	0
Ear Pain			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Eye disorders			
Eyelid Myoclonus			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Ocular Hyperaemia			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Gastrointestinal disorders			
Angular Cheilitis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Abdominal Pain			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Aphthous Ulcer			

subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Constipation			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Dental Discomfort			
subjects affected / exposed	0 / 5 (0.00%)	1 / 4 (25.00%)	0 / 8 (0.00%)
occurrences (all)	0	3	0
Diarrhoea			
subjects affected / exposed	1 / 5 (20.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	1	0	1
Food Poisoning			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Gastritis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Vomiting			
subjects affected / exposed	1 / 5 (20.00%)	0 / 4 (0.00%)	4 / 8 (50.00%)
occurrences (all)	1	0	8
Lower Gastrointestinal Haemorrhage			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Nausea			
subjects affected / exposed	1 / 5 (20.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	1	0	0
Retching			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Salivary Hypersecretion			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Stomatitis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Toothache			

subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Gastrooesophageal Reflux Disease			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Hepatobiliary disorders			
Hepatic Steatosis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Skin and subcutaneous tissue disorders			
Dermatitis Atopic			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	1
Dermatitis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Macule			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Perioral Dermatitis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	1
Rash			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	1
Skin Irritation			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Urticaria			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Skin Hyperpigmentation			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Renal and urinary disorders			

Glycosuria			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Musculoskeletal and connective tissue disorders			
Mobility Decreased			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Back Pain			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Jaw Disorder			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Limb Discomfort			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Muscular Weakness			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Neck Pain			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Pain In Extremity			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Infections and infestations			
Covid-19			
subjects affected / exposed	1 / 5 (20.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	1	0	1
Bronchitis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Croup Infectious			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	1
Ear Infection			

subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	2 / 8 (25.00%)
occurrences (all)	0	0	2
Enterobiasis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Eye Infection			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	1
Fungal Skin Infection			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Gastroenteritis			
subjects affected / exposed	1 / 5 (20.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	1	0	0
Lower Respiratory Tract Infection			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Hand-Foot-And-Mouth Disease			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	1
Hepatitis Infectious Mononucleosis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	1
Impetigo			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Influenza			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	1
Laryngitis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Lice Infestation			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Gastroenteritis Viral			

subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	1
Medical Device Site Infection			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Myringitis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	1
Nasopharyngitis			
subjects affected / exposed	0 / 5 (0.00%)	1 / 4 (25.00%)	1 / 8 (12.50%)
occurrences (all)	0	1	1
Oral Candidiasis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Otitis Externa			
subjects affected / exposed	1 / 5 (20.00%)	1 / 4 (25.00%)	0 / 8 (0.00%)
occurrences (all)	1	1	0
Otitis Media			
subjects affected / exposed	1 / 5 (20.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	1	0	0
Pneumonia			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Pantoea Agglomerans Infection			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Parvovirus B19 Infection			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Pertussis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Pharyngitis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Pharyngitis Streptococcal			

subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	2
Otitis Media Acute			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	1
Rhinitis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Rhinovirus Infection			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Sinusitis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Tonsillitis			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Upper Respiratory Tract Infection			
subjects affected / exposed	1 / 5 (20.00%)	1 / 4 (25.00%)	4 / 8 (50.00%)
occurrences (all)	1	1	5
Urinary Tract Infection			
subjects affected / exposed	2 / 5 (40.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	3	0	1
Viral Infection			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	1 / 8 (12.50%)
occurrences (all)	0	0	1
Metabolism and nutrition disorders			
Dehydration			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Hypoglycaemia			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0
Decreased Appetite			
subjects affected / exposed	0 / 5 (0.00%)	0 / 4 (0.00%)	0 / 8 (0.00%)
occurrences (all)	0	0	0

Non-serious adverse events	GTX-102 Cohorts A & B	GTX-102 Cohorts C & D	GTX-102 Cohorts 4 - 7
Total subjects affected by non-serious adverse events			
subjects affected / exposed	33 / 34 (97.06%)	12 / 12 (100.00%)	15 / 15 (100.00%)
Vascular disorders			
Hypotension			
subjects affected / exposed	3 / 34 (8.82%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	5	0	2
General disorders and administration site conditions			
Asthenia			
subjects affected / exposed	4 / 34 (11.76%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	5	2	0
Decreased Activity			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Decreased Gait Velocity			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Fatigue			
subjects affected / exposed	5 / 34 (14.71%)	1 / 12 (8.33%)	3 / 15 (20.00%)
occurrences (all)	6	1	6
Gait Disturbance			
subjects affected / exposed	1 / 34 (2.94%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	2	1	0
Influenza Like Illness			
subjects affected / exposed	3 / 34 (8.82%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	5	0	1
Pain			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Pyrexia			
subjects affected / exposed	7 / 34 (20.59%)	1 / 12 (8.33%)	4 / 15 (26.67%)
occurrences (all)	10	1	6
Malaise			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	2

Immune system disorders			
Hypersensitivity			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	3	0	0
Reproductive system and breast disorders			
Bartholin's Cyst			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Dysmenorrhoea			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	1	0	1
Respiratory, thoracic and mediastinal disorders			
Adenoidal Hypertrophy			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Dyspnoea			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	1	0	1
Epistaxis			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	2
Nasal Congestion			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Rhinorrhoea			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	2	0	0
Tonsillar Hypertrophy			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	1	0	1
Cough			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	2	0	1
Psychiatric disorders			
Agitation			

subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	2
Abnormal Behaviour			
subjects affected / exposed	1 / 34 (2.94%)	2 / 12 (16.67%)	2 / 15 (13.33%)
occurrences (all)	1	2	3
Aggression			
subjects affected / exposed	1 / 34 (2.94%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	1	1	0
Insomnia			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	2	0	0
Irritability			
subjects affected / exposed	1 / 34 (2.94%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	1	1	0
Investigations			
Blood Alkaline Phosphatase Increased			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Blood Pressure Diastolic Decreased			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Body Temperature Increased			
subjects affected / exposed	3 / 34 (8.82%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	5	0	0
Csf Immunoglobulin Increased			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	3	0	0
Csf Pressure Increased			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	0	0	0
Csf Protein Increased			
subjects affected / exposed	6 / 34 (17.65%)	2 / 12 (16.67%)	4 / 15 (26.67%)
occurrences (all)	7	2	5
Monocyte Count Increased			

subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Csf White Blood Cell Count Increased			
subjects affected / exposed	1 / 34 (2.94%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	1	1	0
Culture Urine Positive			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	0	0	0
Glucose Urine Present			
subjects affected / exposed	0 / 34 (0.00%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	0	1	0
International Normalised Ratio Increased			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Csf Red Blood Cell Count Positive			
subjects affected / exposed	0 / 34 (0.00%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	0	1	0
Neutrophil Count Decreased			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Vitamin D Decreased			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Weight Decreased			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Weight Increased			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	2	0	0
White Blood Cell Count Increased			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Injury, poisoning and procedural complications			

Head Injury			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	2 / 15 (13.33%)
occurrences (all)	0	0	2
Accidental Exposure To Product			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	0	0	0
Arthropod Bite			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	0	0	0
Contusion			
subjects affected / exposed	2 / 34 (5.88%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	3	1	0
Eye Contusion			
subjects affected / exposed	0 / 34 (0.00%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	0	1	0
Fall			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	4 / 15 (26.67%)
occurrences (all)	0	0	6
Foreign Body In Respiratory Tract			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	0	0	0
Skin Laceration			
subjects affected / exposed	1 / 34 (2.94%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	1	1	0
Soft Tissue Injury			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	1	0	1
Thermal Burn			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Tooth Fracture			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Wrist Fracture			
subjects affected / exposed	0 / 34 (0.00%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	0	1	0

Joint Injury			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	1	0	2
Ligament Sprain			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	3 / 15 (20.00%)
occurrences (all)	1	0	3
Limb Injury			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Lip Injury			
subjects affected / exposed	1 / 34 (2.94%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	1	1	0
Post Lumbar Puncture Syndrome			
subjects affected / exposed	7 / 34 (20.59%)	2 / 12 (16.67%)	2 / 15 (13.33%)
occurrences (all)	7	2	3
Procedural Pain			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	2	0	1
Cardiac disorders			
Tachycardia			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	4	0	0
Nervous system disorders			
Ataxia			
subjects affected / exposed	4 / 34 (11.76%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	4	0	0
Atonic Seizures			
subjects affected / exposed	6 / 34 (17.65%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	7	0	0
Balance Disorder			
subjects affected / exposed	1 / 34 (2.94%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	1	1	0
Migraine			
subjects affected / exposed	0 / 34 (0.00%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	0	1	0
Drooling			

subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	1	0	1
Epilepsy			
subjects affected / exposed	4 / 34 (11.76%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	6	0	1
Headache			
subjects affected / exposed	8 / 34 (23.53%)	1 / 12 (8.33%)	3 / 15 (20.00%)
occurrences (all)	11	1	9
Hyperreflexia			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	0	0	0
Hypotonia			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	0	0	0
Lethargy			
subjects affected / exposed	0 / 34 (0.00%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	0	1	0
Disturbance In Attention			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	2	0	0
Myoclonus			
subjects affected / exposed	3 / 34 (8.82%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	7	0	0
Petit Mal Epilepsy			
subjects affected / exposed	3 / 34 (8.82%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	6	0	0
Post-Traumatic Epilepsy			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Radicular Pain			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Radiculopathy			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	2	0	0
Seizure			

subjects affected / exposed	12 / 34 (35.29%)	1 / 12 (8.33%)	2 / 15 (13.33%)
occurrences (all)	15	1	5
Sensory Disturbance			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Somnolence			
subjects affected / exposed	3 / 34 (8.82%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	3	1	0
Syncope			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Seizure Like Phenomena			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	0	0	0
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Lymphocytosis			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Ear and labyrinth disorders			
Ear Discomfort			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Ear Haemorrhage			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Middle Ear Effusion			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	0	0	0
Ear Pain			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	2
Eye disorders			

Eyelid Myoclonus			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	2	0	0
Ocular Hyperaemia			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Gastrointestinal disorders			
Angular Cheilitis			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Abdominal Pain			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Aphthous Ulcer			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	2	0	0
Constipation			
subjects affected / exposed	4 / 34 (11.76%)	1 / 12 (8.33%)	3 / 15 (20.00%)
occurrences (all)	4	1	8
Dental Discomfort			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	0	0	0
Diarrhoea			
subjects affected / exposed	4 / 34 (11.76%)	0 / 12 (0.00%)	2 / 15 (13.33%)
occurrences (all)	6	0	4
Food Poisoning			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Gastritis			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Vomiting			
subjects affected / exposed	15 / 34 (44.12%)	6 / 12 (50.00%)	7 / 15 (46.67%)
occurrences (all)	20	9	10
Lower Gastrointestinal Haemorrhage			

subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Nausea			
subjects affected / exposed	2 / 34 (5.88%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	4	1	0
Retching			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	1	0	1
Salivary Hypersecretion			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Stomatitis			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	2 / 15 (13.33%)
occurrences (all)	4	0	2
Toothache			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Gastrooesophageal Reflux Disease			
subjects affected / exposed	3 / 34 (8.82%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	3	1	0
Hepatobiliary disorders			
Hepatic Steatosis			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Skin and subcutaneous tissue disorders			
Dermatitis Atopic			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	0	0	0
Dermatitis			
subjects affected / exposed	0 / 34 (0.00%)	1 / 12 (8.33%)	1 / 15 (6.67%)
occurrences (all)	0	1	1
Macule			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Perioral Dermatitis			

subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	0	0	0
Rash			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	2	0	1
Skin Irritation			
subjects affected / exposed	0 / 34 (0.00%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	0	1	0
Urticaria			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	1	0	1
Skin Hyperpigmentation			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Renal and urinary disorders			
Glycosuria			
subjects affected / exposed	0 / 34 (0.00%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	0	1	0
Musculoskeletal and connective tissue disorders			
Mobility Decreased			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	3
Back Pain			
subjects affected / exposed	3 / 34 (8.82%)	1 / 12 (8.33%)	3 / 15 (20.00%)
occurrences (all)	3	1	5
Jaw Disorder			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Limb Discomfort			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Muscular Weakness			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	3	0	0
Neck Pain			

subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Pain In Extremity			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	2	0	0
Infections and infestations			
Covid-19			
subjects affected / exposed	4 / 34 (11.76%)	0 / 12 (0.00%)	5 / 15 (33.33%)
occurrences (all)	4	0	5
Bronchitis			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	2	0	0
Croup Infectious			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	1	0	0
Ear Infection			
subjects affected / exposed	3 / 34 (8.82%)	2 / 12 (16.67%)	1 / 15 (6.67%)
occurrences (all)	4	3	1
Enterobiasis			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	2	0	0
Eye Infection			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	1	0	0
Fungal Skin Infection			
subjects affected / exposed	0 / 34 (0.00%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	0	3	0
Gastroenteritis			
subjects affected / exposed	5 / 34 (14.71%)	2 / 12 (16.67%)	4 / 15 (26.67%)
occurrences (all)	7	3	4
Lower Respiratory Tract Infection			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	2 / 15 (13.33%)
occurrences (all)	0	0	3
Hand-Foot-And-Mouth Disease			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	3	0	0

Hepatitis Infectious Mononucleosis			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	0	0	0
Impetigo			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	1	0	2
Influenza			
subjects affected / exposed	5 / 34 (14.71%)	3 / 12 (25.00%)	0 / 15 (0.00%)
occurrences (all)	8	3	0
Laryngitis			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	3	0	0
Lice Infestation			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	2
Gastroenteritis Viral			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	2 / 15 (13.33%)
occurrences (all)	1	0	2
Medical Device Site Infection			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	2
Myringitis			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	0	0	0
Nasopharyngitis			
subjects affected / exposed	8 / 34 (23.53%)	5 / 12 (41.67%)	6 / 15 (40.00%)
occurrences (all)	12	6	14
Oral Candidiasis			
subjects affected / exposed	1 / 34 (2.94%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	1	0	1
Otitis Externa			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	0	0	0
Otitis Media			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	3	0	0

Pneumonia			
subjects affected / exposed	2 / 34 (5.88%)	1 / 12 (8.33%)	2 / 15 (13.33%)
occurrences (all)	2	1	2
Pantoea Agglomerans Infection			
subjects affected / exposed	0 / 34 (0.00%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	0	1	0
Parvovirus B19 Infection			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	2	0	0
Pertussis			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Pharyngitis			
subjects affected / exposed	3 / 34 (8.82%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	3	0	0
Pharyngitis Streptococcal			
subjects affected / exposed	1 / 34 (2.94%)	4 / 12 (33.33%)	0 / 15 (0.00%)
occurrences (all)	3	6	0
Otitis Media Acute			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	2	0	0
Rhinitis			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	2	0	0
Rhinovirus Infection			
subjects affected / exposed	0 / 34 (0.00%)	1 / 12 (8.33%)	0 / 15 (0.00%)
occurrences (all)	0	1	0
Sinusitis			
subjects affected / exposed	1 / 34 (2.94%)	2 / 12 (16.67%)	0 / 15 (0.00%)
occurrences (all)	1	2	0
Tonsillitis			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	0 / 15 (0.00%)
occurrences (all)	3	0	0
Upper Respiratory Tract Infection			
subjects affected / exposed	6 / 34 (17.65%)	6 / 12 (50.00%)	5 / 15 (33.33%)
occurrences (all)	10	8	15

Urinary Tract Infection			
subjects affected / exposed	2 / 34 (5.88%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	2	0	4
Viral Infection			
subjects affected / exposed	10 / 34 (29.41%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	12	0	1
Metabolism and nutrition disorders			
Dehydration			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Hypoglycaemia			
subjects affected / exposed	0 / 34 (0.00%)	0 / 12 (0.00%)	1 / 15 (6.67%)
occurrences (all)	0	0	1
Decreased Appetite			
subjects affected / exposed	0 / 34 (0.00%)	2 / 12 (16.67%)	2 / 15 (13.33%)
occurrences (all)	0	2	3

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
17 December 2019	*Changed number of patients treated by cohort *Medical monitor review of safety data prior to dose escalation within dose cohort *Change to DSMB requirements *Updated number of patients per cohort *Added new exclusion criteria *Open label extension will be conducted under a separate protocol, clarification that patients may remain on study without treatment until initiation of OLE *Added details on anesthesia use *Added details related to LP procedure *Eliminated obtaining weights every 2 weeks *Changed inflammatory marker to be analyzed.
01 May 2020	*LPs allowed without anesthesia *Added inclusion criteria *Given COVID-19 pandemic; allow EEG to be obtained outside in-patient hospital setting, allow safety visits via video conference/telemedicine and home visits from visiting nurses or health care provider *Added blood and urine collection for other biomarkers *Changed time of ECG for patient convenience *Changed time of neurological exam *Specified time limit on duration of AEs that require discontinuation from study *Updated management of thrombocytopenia and proteinuria.
29 July 2022	*Change of sponsor from GeneTx to Ultragenyx *Removed specificity of UK and Canada *Adjusted efficacy measures
13 March 2023	*Removed Dose-selection Cohorts 8-12 *Expansion Cohorts A and B updates to loading and maintenance period *Dose-selection Cohorts 4-7 maintenance dosing modified *Dexamethasone premedication required for loading doses *Radiculopathy defined as AESI *Contraception guidance added.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? Yes

Date	Interruption	Restart date
20 July 2020	The Sponsor suspended dosing and enrollment into the study according to the protocol-defined dose stopping criteria after the first patient experienced the related SAE of radiculopathy. On 03 November 2020, the FDA placed the study on a Full Clinical Hold. The Sponsor engaged with the Agency to review data and discuss changes to the study design.	24 September 2021

Notes:

Limitations and caveats

None reported