Clinical trial results:
A Phase 1/2, open-label, safety, tolerability and efficacy study of FLT201 in adult patients with Gaucher disease Type 1 (Galileo-1)

Summary

EudraCT number	2020-005032-30
Trial protocol	ES
Global end of trial date	04 December 2024

Results information

Result version number	v1 (current)
This version publication date	13 February 2026
First version publication date	13 February 2026

Trial information

Trial identification

Sponsor protocol code	FLT201-01

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT05324943
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Spur Therapeutics (formerly known as Freeline Therapeutics Ltd)
Sponsor organisation address	Sycamore House Gunnels Wood Road, Stevenage, Hertfordshire, United Kingdom, SG1 2BP
Public contact	Spur Clinical Trials Contact , Spur Therapeutics (formerly known as Freeline Therapeutics Ltd), +44 (0)1438 906870, clinicaltrials@spurtherapeutics.com
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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	04 December 2024
Is this the analysis of the primary completion data?	Yes
Primary completion date	04 December 2024
Global end of trial reached?	Yes
Global end of trial date	04 December 2024
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To assess the safety and tolerability of a single intravenous administration of FLT201 in adults with Gaucher disease Type 1

Protection of trial subjects:

The study was conducted in accordance with the Declaration of Helsinki and with all applicable laws and regulations of the locales and countries where the study was conducted, and in compliance with Good Clinical Practice Guidelines.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	31 October 2022
Long term follow-up planned	Yes
Long term follow-up rationale	Safety, Efficacy
Long term follow-up duration	5 Years
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Spain: 1
Country: Number of subjects enrolled	Brazil: 2
Country: Number of subjects enrolled	United States: 2
Country: Number of subjects enrolled	United Kingdom: 1
Worldwide total number of subjects	6
EEA total number of subjects	1

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)	0
Adolescents (12-17 years)	0
Adults (18-64 years)	6
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

This was a multicenter study with 15 study sites in USA, Brazil, Paraguay, Spain, Germany, UK, and Israel. Ten participants met all the inclusion criteria for the trial and were enrolled, with 6 participants ultimately being dosed with FLT201 at 4 trial sites in Brazil, Spain, UK, and USA

Pre-assignment

Screening details:

Participants were to undergo screening assessments for up to 16 weeks prior to Day 1 (gene therapy infusion). Treatment-eligible participants reported to the infusion trial site on the day prior to receiving the gene therapy infusion (Day -1).

Period 1

Period 1 title	Treatment Period (overall period)
Is this the baseline period?	Yes
Allocation method	Not applicable
Blinding used	Not blinded

Arms

Arm title	FLT201

Arm description:

FLT201 (4.5 × 10^11 vg/kg) was administered in the controlled environment of a trial site, which had been assessed for its ability to store, handle, and administer gene therapy products per local regulations, as well
as their ability to comply with procedures in the FLT201 Pharmacy Manual. The administration of FLT201 was performed by suitably qualified and trained trial staff.

Arm type	Experimental
Investigational medicinal product name	FLT201 Solution for Infusion
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Concentrate and solvent for solution for infusion
Routes of administration	Infusion

Dosage and administration details:

FLT201 is a replication-incompetent ss recombinant AAV vector. The vector is composed of a ss DNA genome packaged in an AAV-derived protein capsid.
The FLT201 drug product was supplied as a sterile solution for infusion in 10 mL Crystal Zenith® vials, each vial containing 5 mL extractable volume. The vials were sealed with rubber stoppers and aluminum seals with plastic flip tops. The product was formulated as an approximately isotonic, aqueous solution at neutral pH. The formulation also contained 0.25% w/v recombinant human albumin.
FLT201 was administered as a single dose, slow IV infusion (over up to 2 hours) into a peripheral vein, and the participant was monitored closely for at least 8 hours following the infusion.

Number of subjects in period 1	FLT201
Started	6
Completed	6

Baseline characteristics

Reporting groups

Reporting group title	Treatment Period (overall period)

Reporting group description: -

Reporting group values	Treatment Period (overall period)	Total	
Number of subjects	6	6	
Age categorical Units: Subjects			
In utero	0	0	
Preterm newborn infants (gestational age < 37 wks)	0	0	
Newborns (0-27 days)	0	0	
Infants and toddlers (28 days-23 months)	0	0	
Children (2-11 years)	0	0	
Adolescents (12-17 years)	0	0	
Adults (18-64 years)	6	6	
From 65-84 years	0	0	
85 years and over	0	0	
Age continuous Units: years			
arithmetic mean	32.7		
standard deviation	± 13.14	-	
Gender categorical Units: Subjects			
Female	2	2	
Male	4	4	

End points

End points reporting groups

Reporting group title	FLT201

Reporting group description:

FLT201 (4.5 × 10^11 vg/kg) was administered in the controlled environment of a trial site, which had been assessed for its ability to store, handle, and administer gene therapy products per local regulations, as well
as their ability to comply with procedures in the FLT201 Pharmacy Manual. The administration of FLT201 was performed by suitably qualified and trained trial staff.

Primary: Incidence of treatment-emergent adverse events over time

End point title	Incidence of treatment-emergent adverse events over time[1]

End point description:

Incidence of treatment-emergent adverse events (TEAEs; including dose limiting toxicities) from Day 1 to the last follow-up visit.

Two Important Medical Events were reported in line with SAE procedures per protocol. No AEs met the criteria for SAEs.

End point type	Primary

End point timeframe:

From Day 1 to the last follow-up visit.

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 summaries of safety measures are based on observed data. No imputation of missing data were implemented.

End point values	FLT201			
Subject group type	Reporting group			
Number of subjects analysed	6			
Units: Number of participants affected				
Any AE	6			
Any TEAE	6			
Any IMP-related TEAE	6			
Any serious TEAE	2			
Any non-serious TEAE	6			
Any IMP-related serious TEAE	2			
Any TEAE leading to treatment discontinuation	0			
Any IMP-related TEAE leading to treatment disconti	0			
Any TEAE leading to death	0			
Any IMP-related TEAE leading to death	0			

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in Lyso-Gb1

End point title	Change from Baseline in Lyso-Gb1

End point description:

Change from baseline to week 38 of lyso-Gb1 in plasma.

Only 4 participants with a value at both baseline and the end of study are included. An addition 2 participants had Baseline and Month 9 values. Mean change (SD) for them was -19.860 (20.8314)

End point type	Secondary

End point timeframe:

From baseline to week 38 (end of study)

End point values	FLT201			
Subject group type	Reporting group			
Number of subjects analysed	4[2]			
Units: ng/ml				
arithmetic mean (standard deviation)	-15.478 (± 32.1514)			

Notes:

[2] - Only participants with a value at both baseline and the end of study are included.

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in Spleen Volume Measured by MRI

End point title	Change from Baseline in Spleen Volume Measured by MRI

End point description:

Change from baseline to week 38 in Spleen Volume Measured by MRI.

Only 4 participants with a value at both baseline and the end of study are included. An addition 2 participants had Baseline and Month 9 values. Mean change (SD) for them was -44.545 (111.8855).

End point type	Secondary

End point timeframe:

From baseline to week 38

End point values	FLT201			
Subject group type	Reporting group			
Number of subjects analysed	4[3]			
Units: mL				
arithmetic mean (standard deviation)	-49.453 (± 75.3262)			

Notes:

[3] - Only participants with a value at both baseline and the end of study are included.

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in Liver Volume Measured by MRI

End point title	Change from Baseline in Liver Volume Measured by MRI

End point description:

Change from baseline to week 38 in Liver Volume Measured by MRI.

Only 3 participants with a value at both baseline and the end of study are included. An addition 2 participants had Baseline and Month 9 values. Mean change (SD) for them was 41.435 (87.1651).

End point type	Secondary

End point timeframe:

From baseline to week 38.

End point values	FLT201			
Subject group type	Reporting group			
Number of subjects analysed	3[4]			
Units: mL				
arithmetic mean (standard deviation)	-20.657 (± 47.5816)			

Notes:

[4] - Only participants with a value at both baseline and the end of study are included.

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in Hemoglobin

End point title	Change from Baseline in Hemoglobin

End point description:

Change from baseline to week 38 in hemoglobin.

Only 4 participants with a value at both baseline and the end of study are included. An addition 2 participants had Baseline and Month 9 values. Mean change (SD) for them was 0.30 (1.697).

End point type	Secondary

End point timeframe:

From baseline to week 38.

End point values	FLT201			
Subject group type	Reporting group			
Number of subjects analysed	4[5]			
Units: g/dL				
arithmetic mean (standard deviation)	0.03 (± 1.384)			

Notes:

[5] - Only participants with a value at both baseline and the end of study are included.

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in Platelet Count

End point title	Change from Baseline in Platelet Count

End point description:

Change from baseline to week 38 in Platelet Count.

Only 4 participants with a value at both baseline and the end of study are included. An addition 2 participants had Baseline and Month 9 values. Mean change (SD) for them was -4.0 (28.28).

End point type	Secondary

End point timeframe:

From baseline to week 38.

End point values	FLT201			
Subject group type	Reporting group			
Number of subjects analysed	4[6]			
Units: 10^9/L				
arithmetic mean (standard deviation)	38.8 (± 75.61)			

Notes:

[6] - Only participants with a value at both baseline and the end of study are included.

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

The time-period for reporting AEs was from the time the initial ICF was signed until end of study.

Adverse event reporting additional description:

Reporting included events occurring during the Screening Phase of the trial, regardless of whether FLT201 was subsequently administered.
Two Important Medical Events were reported in line with SAE procedures per protocol. No AEs met the criteria for SAEs.

Assessment type	Systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	27.1

Reporting groups

Reporting group title	Safety Population

Reporting group description:

The Safety Set included all participants who received any dose of FLT201. All data summaries used the Safety Set, unless otherwise specified.

Serious adverse events	Safety Population		
Total subjects affected by serious adverse events			
subjects affected / exposed	2 / 6 (33.33%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		
Investigations			
Neutralising antibodies			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Neutralising antibodies positive			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		

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

Non-serious adverse events	Safety Population		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	6 / 6 (100.00%)		
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Skin papilloma			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Vascular disorders			
Blood pressure fluctuation			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Hypertension			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Hypotension			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Peripheral coldness			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
General disorders and administration site conditions			
Adverse drug reaction			
subjects affected / exposed	2 / 6 (33.33%)		
occurrences (all)	2		
Discomfort			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Fatigue			
subjects affected / exposed	3 / 6 (50.00%)		
occurrences (all)	3		
Malaise			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Peripheral swelling			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		

Temperature intolerance subjects affected / exposed occurrences (all)	1 / 6 (16.67%) 1		
Respiratory, thoracic and mediastinal disorders Cough subjects affected / exposed occurrences (all) Nasal congestion subjects affected / exposed occurrences (all) Oropharyngeal pain subjects affected / exposed occurrences (all) Rhinitis allergic subjects affected / exposed occurrences (all)	 1 / 6 (16.67%) 1 1 / 6 (16.67%) 1 1 / 6 (16.67%) 1 1 / 6 (16.67%) 1		
Psychiatric disorders Anxiety subjects affected / exposed occurrences (all) Mood altered subjects affected / exposed occurrences (all) Panic attack subjects affected / exposed occurrences (all)	 1 / 6 (16.67%) 1 1 / 6 (16.67%) 1 1 / 6 (16.67%) 1		
Investigations Activated partial thromboplastin subjects affected / exposed occurrences (all) Alanine aminotransferase increased subjects affected / exposed occurrences (all) Aspartate aminotransferase increased subjects affected / exposed occurrences (all)	 2 / 6 (33.33%) 2 6 / 6 (100.00%) 6 2 / 6 (33.33%) 2		

Cardiac murmur			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Electrocardiogram ST segment elevation			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Fibrin D dimer increased			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Glomerular filtration rate decreased			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Immature granulocyte count increased			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Mean cell volume increased			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Neutrophil count increased			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Prothrombin time prolonged			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Scan bone marrow abnormal			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Weight increased			
subjects affected / exposed	2 / 6 (33.33%)		
occurrences (all)	2		
Injury, poisoning and procedural complications			
Contusion			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Cardiac disorders			

Cardiomegaly			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Palpitations			
subjects affected / exposed	2 / 6 (33.33%)		
occurrences (all)	2		
Nervous system disorders			
Action tremor			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Burning sensation			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Headache			
subjects affected / exposed	3 / 6 (50.00%)		
occurrences (all)	3		
Migraine			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Tremor			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Blood and lymphatic system disorders			
Leukocytosis			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Leukopenia			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Lymphopenia			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Neutropenia			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Neutrophilia			

subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Splenomegaly			
subjects affected / exposed	2 / 6 (33.33%)		
occurrences (all)	2		
Thrombocytopenia			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Ear and labyrinth disorders			
Ear pain			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Eye disorders			
Visual field defect			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Vitreous floaters			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Gastrointestinal disorders			
Abdominal distension			
subjects affected / exposed	2 / 6 (33.33%)		
occurrences (all)	2		
Abdominal pain			
subjects affected / exposed	2 / 6 (33.33%)		
occurrences (all)	2		
Abdominal pain upper			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Constipation			
subjects affected / exposed	2 / 6 (33.33%)		
occurrences (all)	2		
Diarrhoea			
subjects affected / exposed	5 / 6 (83.33%)		
occurrences (all)	5		
Dyspepsia			

subjects affected / exposed	2 / 6 (33.33%)		
occurrences (all)	2		
Flatulence			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Frequent bowel movements			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Vomiting			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Skin and subcutaneous tissue disorders			
Dermatitis			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Hyperhidrosis			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Macule			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Pruritus			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Skin atrophy			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Renal and urinary disorders			
Micturition urgency			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Oliguria			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Pollakiuria			

subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Proteinuria			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Urinary incontinence			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Musculoskeletal and connective tissue disorders			
Arthralgia			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Muscle spasms			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Musculoskeletal chest pain			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Pain in extremity			
subjects affected / exposed	3 / 6 (50.00%)		
occurrences (all)	3		
Infections and infestations			
Epstein-Barr virus infection			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Folliculitis			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Gastroenteritis			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Gastrointestinal infection			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Hordeolum			

subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Nasopharyngitis			
subjects affected / exposed	2 / 6 (33.33%)		
occurrences (all)	2		
Pharyngitis			
subjects affected / exposed	2 / 6 (33.33%)		
occurrences (all)	2		
Sinusitis			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Urinary tract infection			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Dehydration			
subjects affected / exposed	2 / 6 (33.33%)		
occurrences (all)	2		
Hyperglycaemia			
subjects affected / exposed	3 / 6 (50.00%)		
occurrences (all)	3		
Vitamin D deficiency			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
08 February 2021	Global amendment. Protocol version 2.0.
25 October 2021	Global amendment. Protocol version 3.0.
21 January 2022	Country-specific amendment in USA. Protocol version 4.0.
22 March 2022	Country-specific amendment in Brazil. Protocol version 6.0.
16 November 2023	Country-specific amendment in Brazil. Protocol version 7.0.
16 November 2023	Country-specific amendment in UK, Spain, Germany, Israel, Paraguay. Protocol version 8.0.
26 April 2024	Country-specific amendment in USA. Protocol version 9.0.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

Limitations of the trial such as small numbers of subjects analysed or technical problems leading to unreliable data.

None reported.

Notes: