SPUR THERAPEUTICS

Spur Therapeutics Completes Enrollment in Phase 1/2 GALILEO-1 Trial of FLT201 in Gaucher Disease and Selects Dose for Planned Phase 3 Trial

Single infusion of FLT201 at low dose of 4.5e11 vg/kg demonstrates compelling safety and benefit

Expect to initiate Phase 3 registrational trial in 2025

LONDON, July 8, 2024 – Spur Therapeutics, formerly Freeline Therapeutics, today announced it has completed enrollment in the Phase 1/2 GALILEO-1 clinical trial of FLT201, its adeno-associated virus (AAV) gene therapy candidate for Gaucher disease. Based on compelling safety and efficacy data from the GALILEO-1 trial, Spur has selected a single infusion of FLT201 at a low dose of 4.5e11 vg/kg for further development in a Phase 3 trial expected to begin next year.

"We are very pleased with the progress of FLT201," said Michael Parini, Chief Executive Officer of Spur Therapeutics. "The data from the GALILEO-1 trial strengthen our conviction in FLT201's potential to set a new standard of care for Gaucher disease and give us confidence to move forward at the current dose, which has shown strong signals of efficacy combined with a favorable safety and tolerability profile. We are preparing to start the Phase 3 trial next year and are committed to bringing forward what we believe is a first- and best-in-class gene therapy to redefine the treatment landscape for Gaucher disease."

Six patients have been dosed in GALILEO-1, a first-in-human, international, multicenter dose-finding study in adults with Gaucher disease Type 1. All patients were treated with a single infusion of FLT201 at a dose of 4.5e11 vg/kg. Two of the six patients have completed the full nine months of follow-up. The others have been followed for between 16 and 38 weeks after dosing. All six patients are included in the safety analysis. Five of the six patients are included in the efficacy analysis; one patient with detectable pre-existing neutralizing antibodies (NAbs) to the AAVS3 capsid below the protocol cut-off has been excluded from the efficacy analysis.

Data as of the June 30, 2024 data cut-off demonstrate:

- Favorable safety and tolerability.
- Dramatic reductions in glucosylsphingosine (lyso-Gb1) – a Gaucher-specific biomarker that is one of the best predictors of disease severity and clinical response – in patients with persistently high levels despite years of treatment with currently approved therapies (4/4).
- Maintenance of lyso-Gb1 levels in a patient who entered the trial with well-controlled lyso-Gb1 (1/1).
- Maintenance of normal levels or improvement to normal levels of hemoglobin and platelets, which are well-accepted regulatory endpoints for Gaucher disease (5/5).
- Improvement in bone marrow burden (5/5), which shows FLT201 is reaching deeper tissues that currently approved therapies poorly address.
- Clinically relevant improvement in patient-reported pain and fatigue, leading to improved function, in the one patient who entered trial with debilitating chronic pain and fatigue.

- Patients experienced these benefits regardless of antibodies to glucocerebrosidase (GCase) (n=2), the enzyme that is deficient in people with Gaucher disease, consistent with clinical experience with enzyme replacement therapy and preclinical data for FLT201.
    - Improvements in lyso-Gb1 and bone marrow burden from baseline, as well as maintenance of hemoglobin and platelets in normal ranges, continue to be observed after antibodies were detected.
    - In the patient with longest exposure (greater than three months beyond detection of antibodies), the antibodies appear to have been transient and improvements in clinical parameters continue to be observed.

Spur expects to report additional data from the GALILEO-1 trial in the second half of 2024.

FLT201 has been granted Regenerative Medicine Advanced Therapy (RMAT) designation by the US Food and Drug Administration, Priority Medicines (PRIME) designation by the European Medicines Agency and the Innovative Licensing and Access Pathway (ILAP) by the U.K. Medicines and Healthcare products Regulatory Agency. Designed to expedite the drug development process for investigational therapies intended to treat, modify, reverse or cure a serious or life-threatening disease, the designations provide opportunities for enhanced interactions with regulators and expedited review processes. Spur is actively engaging with regulators as it prepares for the planned Phase 3 trial of FLT201.

About FLT201
FLT201 is an adeno-associated virus (AAV) gene therapy candidate that is currently being investigated in the Phase 1/2 GALILEO-1 clinical trial in adults with Gaucher disease. FLT201 leverages Spur's proprietary and potent AAVS3 capsid to deliver GCase85, a rationally engineered longer-acting version of the enzyme deficient in people with Gaucher disease, with the goal of stopping disease progression, reducing or eliminating symptoms, and allowing patients to come off current lifelong treatments. Preclinical and clinical data for FLT201 have shown robust and durable expression and a substantial reduction in the toxic buildup of substrate that results from the enzyme deficiency. For more information about the GALILEO-1 trial, please visit clinicaltrials.gov (NCT05324943).

About Gaucher Disease
Gaucher disease is caused by a mutation in the *GBA1* gene that results in abnormally low levels of glucocerebrosidase (GCase), an enzyme needed to metabolize a certain type of lipid. As a result, harmful substrates glucosylceramide (Gb-1) and glucosylsphingosine (lyso-Gb1) build up in cells, which then accumulate in tissues and organs throughout the body, causing inflammation and dysfunction. Despite treatment with currently approved therapies, many people with Gaucher disease continue to experience debilitating symptoms, including enlarged organs, fatigue, bone pain and reduced lung function. Gaucher disease affects approximately 18,000 people in the United States, United Kingdom, France, Germany, Spain, Italy and Israel.

About Spur Therapeutics
Spur Therapeutics is a clinical-stage biotechnology company focused on developing life-changing gene therapies for debilitating chronic conditions. By optimizing every component of its product candidates, Spur aims to unlock the true potential of gene therapy to realize outsized clinical results. Spur is advancing a breakthrough gene therapy candidate for Gaucher disease and a potential first-in-class gene therapy candidate for adrenomyeloneuropathy, as well as a research strategy to move

gene therapy into more prevalent diseases, including forms of Parkinson's, dementia, and cardiovascular disease. Expanding our impact, and advancing the practice of genetic medicine.

Toward life-changing therapies, and brighter futures. Toward More™

For more information, visit www.spurtherapeutics.com or connect with Spur on LinkedIn and X.

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