Spur Therapeutics (Formerly Freeline) Announces New Name and Brand

With a mission to redefine what gene therapy can do, Spur is optimizing every component of its product candidates to develop a new generation of gene therapies

Spur is advancing two potentially life-changing gene therapy candidates in clinical trials and a bold research strategy to move gene therapy beyond rare diseases into more prevalent conditions

Lead program FLT201 for Gaucher disease poised to enter Phase 3 development in 2025

Acquired SwanBio, adding Phase 1/2 program for adrenomyeloneuropathy to its clinical-stage pipeline and strengthening CNS expertise

Founding investor Syncona commits additional $50 million to support development of pipeline

LONDON, June 17, 2024 – Spur Therapeutics, formerly Freeline Therapeutics, today announced a new name and brand to reflect its focus on developing a new generation of gene therapies and advancing the practice of genetic medicine. Building on compelling data for its lead program FLT201, a highly differentiated gene therapy candidate for Gaucher disease, Spur is pursuing an ambitious research strategy to unlock the promise of gene therapy for more prevalent chronic conditions, starting with Parkinson's disease and certain forms of cardiovascular disease.

"At Spur Therapeutics, our mission is to redefine what gene therapy can do," said Michael Parini, Spur's Chief Executive Officer. "Our new name and new brand reflect our determination to alter the course of a disease with a single dose of genetic medicine and change the course of people's lives. By optimizing every component of our product candidates to get just the right expression, packaged and delivered to the body in just the right way, we are working to develop a new generation of therapies that spur gene therapy forward to transform the lives of even more patients."

Spur today also announced its acquisition of SwanBio Therapeutics, which adds a potential first-in-class gene therapy program for adrenomyeloneuropathy (AMN), a devastating neurodegenerative disease, to its clinical-stage pipeline, as well as strengthened capabilities in central nervous system (CNS) disorders that can be leveraged across both its AMN and Parkinson's disease programs. The AMN program, SBT101, is currently in a Phase 1/2 clinical trial, and Spur plans to report an initial safety update from the higher-dose cohort in this trial in the first half of next year. There are no approved treatments for AMN, and SBT101 is the only gene therapy candidate in development for the disease.

Syncona Ltd., the founding shareholder in both Freeline and SwanBio and a leading life science investor focused on creating, building and scaling global leaders in life science, has committed an additional $50 million (£40 million) to support development of the expanded pipeline. Syncona Executive Partner and former SwanBio Executive Chair John Tsai has joined Spur's Board of Directors.

"We see great promise across Spur's broadened pipeline, with a highly differentiated lead clinical program backed by compelling data and a second potentially first-in-class clinical asset," said Chris

Hollowood, CEO of Syncona Investment Management Limited and Chairman of the Board of Directors of Spur. “Building on its work, Spur has an exciting opportunity to become a leading gene therapy company developing one-time treatments for debilitating chronic diseases, potentially setting new standards of care and changing lives.

Spur expects to initiate a Phase 3 trial for FLT201 in 2025 in Gaucher disease. There is no cure for Gaucher disease, and even with current treatments, many patients continue to experience debilitating symptoms. Recently reported data from its Phase 1/2 GALILEO-1 trial highlight FLT201’s potential to set a new standard of care for Gaucher disease. The data show that a single dose of FLT201 resulted in dramatic reductions in the toxic buildup of glucosylsphinogsine (lyso-Gb1), one of the best predictors of clinical response and disease severity in Gaucher disease, in patients who have had persistently high levels despite years of treatment on currently approved therapies. Early signs of clinical improvement in fatigue and bone marrow burden were also observed. FLT201 has demonstrated a favorable safety and tolerability profile.

Building on its work in Gaucher disease, Spur’s research program in Parkinson’s disease is focused on a subset of patients with mutations in the *GBA1* gene, the same gene implicated in Gaucher disease. The program leverages the same transgene as FLT201. Spur is further optimizing the transgene for expression in the brain and identifying the best capsid and route of administration to deliver its proprietary *GBA1-85* transgene to key areas of the brain affected by Parkinson’s disease. Spur expects to select a development candidate later this year to progress into preclinical studies designed to support the program’s advancement into clinical trials. No disease-modifying therapies currently exist for Parkinson’s disease, and this program could be a first step toward a gene therapy for hundreds of thousands of people with GBA1 Parkinson’s worldwide.

Additionally, Spur has a research program, leveraging a suite of promising cardioprotective proteins to develop gene therapy candidates for cardiovascular diseases, starting with a severe subset of chronic heart failure.

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.

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