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Spur Therapeutics announces new data at ASGCT

Syncona Ltd, ("Syncona" or the "Company") a leading life science investor focused on creating, building and scaling global leaders in life science, notes that its portfolio company Spur Therapeutics ("Spur") announced new data from its gene therapy programmes in Gaucher disease, GBA1 Parkinson's disease and adrenomyeloneuropathy (AMN). These data were presented at the American Society of Gene and Cell Therapy (ASGCT) 28th Annual Meeting in New Orleans, US.

Key highlights from the presentations include:

- Updated clinical data from the Phase I/II GALILEO-1 trial and a longer-term follow-up study of FLT201 in Gaucher disease demonstrated that the adeno-associated virus (AAV) gene therapy candidate continues to deliver durable enzyme expression and sustained clinical benefit
- Updated pre-clinical data for FLT201 demonstrated robust and sustained enzyme expression, maintained out past 3.5 years
- Pre-clinical data supported best-in-class potential for SPR301 for a form of Parkinson's disease characterised by mutations in *GBA1* (the same gene implicated in Gaucher disease); data demonstrated that the AAV gene therapy candidate enhanced enzyme expression in key brain regions
- A safety update from the Phase I/II PROPEL clinical trial of SBT101 demonstrated that the gene therapy candidate for AMN continues to be generally well-tolerated

Chris Hollowood, Chief Executive Officer of Syncona Investment Management Limited and Chair of Spur Therapeutics, said: "The data presented by Spur at ASGCT is very encouraging, for their lead programme in Gaucher disease but also for the broader pipeline. Targeting the same genetic pathway, SPR301 is demonstrating best-in-class potential in GBA1 Parkinson's disease, whilst FLT201 is demonstrating durable clinical benefit in Gaucher disease. We look forward to the company initiating their Phase III trial of FLT201."

Spur's announcement is copied below and can be accessed at the company's website at spurtherapeutics.com.

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About Syncona

Syncona's purpose is to invest to extend and enhance human life. We do this by creating, building and scaling companies to deliver transformational treatments to patients in areas of high unmet need

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We aim to build and maintain a diversified portfolio of 20-25 globally leading life science businesses, across development stage, modality and therapeutic area, for the benefit of all our stakeholders. We focus on developing treatments that deliver patient impact by working in close partnership with world-class academic founders and experienced management teams. Our balance sheet underpins our strategy, enabling us to take a long-term view as we look to improve the lives of patients with no or poor treatment options, build sustainable life science companies and deliver strong risk-adjusted returns to shareholders.

Syncona Limited seeks to achieve returns over the long term. Investors should seek to ensure they understand the risks and opportunities of an investment in Syncona Limited, including the information in our published documentation, before investing.

Spur Therapeutics Presents Positive New Clinical and Preclinical Data on Its Gene Therapy Programs at ASGCT 28th Annual Meeting

Oral presentation highlights data showing FLT201 results in durable enzyme expression and sustained clinical benefit in Gaucher disease

Preclinical data for SPR301 support best-in-class potential for GBA1 Parkinson's disease

SBT101 continues to be well tolerated in adrenomyeloneuropathy patients

LONDON, May 15, 2025 - Spur Therapeutics today announced positive new data from its gene therapy programs in Gaucher disease, GBA1 Parkinson's disease and adrenomyeloneuropathy (AMN). These data are being presented in oral and poster presentations at the American Society of Gene and Cell Therapy (ASGCT) 28th Annual Meeting.

"At Spur, we are advancing a new generation of gene therapies tailored to meet the unique needs of each disease we target," said Pamela Foulds, M.D., Chief Medical Officer at Spur Therapeutics. "Our lead program, FLT201, is designed to produce a more stable version of the enzyme deficient in Gaucher disease, with data showing strong safety and efficacy signals up to nearly two years after a single dose. As we prepare to initiate a Phase 3 trial for FLT201, these results strengthen our confidence in its potential to dramatically reduce both the disease and treatment burden for people with Gaucher. Alongside promising preclinical results from our Parkinson's program, which uses the same engineered enzyme further optimized for the brain, these findings highlight the power of our approach to provide gene therapies that set new standards of care for people living with serious diseases."

FLT201 for Gaucher disease: Durable enzyme expression and clinical benefit
Today's oral presentation includes updated data from the Phase 1/2 GALILEO-1 trial of FLT201, an adeno-associated virus (AAV) gene therapy candidate for Gaucher disease type 1, as well as the ongoing long-term follow-up study. Six patients were treated in the trial with a single infusion of FLT201 at the low dose of 4.5e11 vg/kg. The data are from four patients who were taken off prior enzyme replacement therapy (ERT) or substrate reduction therapy (SRT) after dosing. All were taken off ERT or SRT within 12 weeks and remain off prior therapies up to 21 months after dosing, as of the data cutoff. The presentation also includes data on glucocerebrosidase (GCase) expression in non-human primates (NHPs).

The clinical data as of March 28, 2025, and preclinical data showed:

- Robust reductions in glucosylsphingosine (lyso-Gb1) sustained up to 15 months after stopping prior therapy; stable lyso-Gb1 levels in the one patient who entered the trial with well-controlled levels 14 months after withdrawal of prior therapy. Lyso-Gb1 is one of the best predictors of treatment response in Gaucher disease.
- Maintenance of normal hemoglobin levels and stable or improved platelet counts up to 18 months after stopping prior therapy.
- Robust and sustained plasma GCase activity in NHPs, maintained out past 3.5 years.
- Anti-GCase antibodies were transient in humans and NHPs, with no impact on clinical benefit in the one patient who developed antibodies after successfully discontinuing prior therapy.[1]

SPR301 for GBA1 Parkinson's: Enhanced enzyme expression in key brain regions
Spur also presented preclinical data on SPR301, an AAV gene therapy candidate for a form of Parkinson's disease characterized by mutations in *GBA1*, the same gene implicated in Gaucher disease. SPR301 leverages GCase85, the same more stable, rationally engineered enzyme used in FLT201, further optimized for expression in the brain.

Key findings include:

- Superior GCase exposure in Parkinson's-affected regions of the brain, including the substantia nigra, in GCase-deficient mice compared to wildtype GCase gene therapy, while minimizing harmful microglia activation.
- Broader GCase distribution than wildtype gene therapy in GCase-deficient mice, with dose-dependent substrate reduction.
- Superior substrate reduction compared to wildtype gene therapy, providing a therapeutic window that potentially allows for greater efficacy at much lower doses with a favorable safety profile.
- Greater reduction of α-synuclein, a hallmark of Parkinson's disease, in neuronal cells *in vitro* compared to wildtype gene therapy.

SBT101 in AMN: Continues to be generally well-tolerated
Spur also presented a safety update from the Phase 1/2 PROPEL clinical trial of SBT101, a gene therapy candidate for AMN. Eight patients were treated in PROPEL across low- and high-dose cohorts and have been followed for four to 24 months. Both doses of SBT101 have been generally well tolerated, with most treatment-emergent adverse events being non-serious. One patient died of disease-related complications unrelated to SBT101, and another has progressed to cerebral ALD, a more severe form of the disease.

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, a potential first-in-class gene therapy candidate for adrenomyeloneuropathy and a preclinical gene therapy

candidate for Parkinson's disease, as well as a research strategy to move gene therapy into more prevalent diseases, including forms of dementia and cardiovascular disease. Expanding our impact, and advancing the practice of genetic medicine.

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

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[1] Transient anti-GCase antibodies were also observed in a second participant with low transduction efficiency who remains on SRT.

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