Moving to definitive data

> Our goal is to bring life-changing gene therapies to people with chronic debilitating disease. We are excited by the data we have presented in our Gaucher programme, which we believe has the potential to challenge the standard of care for the disease."

Michael Parini

SPUR THERAPEUTICS CEO

COMBINING TWO LEADING GENE THERAPY COMPANIES

During the year, Syncona was able to take advantage of market conditions impacting the biotech sector and wholly acquire Freeline, an adeno-associated virus (AAV) gene therapy company previously listed on NASDAQ. Post-period end, Freeline completed the acquisition of Syncona portfolio company SwanBio, creating a new Syncona portfolio company Spur. This creates a consolidated AAV gene therapy pipeline, with the company focused on driving forward its two potentially first-in-class gene therapy assets in Gaucher disease and adrenomyeloneuropathy (AMN) towards late-stage development, supported by an increased capability in central nervous system (CNS) disorders, which supports its pre-clinical Parkinson's research programme.

CLINICAL DATA SUPPORTING COMMERCIAL OPPORTUNITY

Spur announced positive safety, tolerability and enzyme activity data during the year from its Phase I/II clinical trial of FLT201 in Gaucher disease, a debilitating genetic disorder in which a deficiency of the GCase enzyme leads to a buildup of fatty substances in the organs, causing symptoms including enlarged spleen and liver, low blood counts, bone pain and reduced lung function. This was followed by additional data released post-period end, which further supported the efficacy and safety profile of the therapy whilst also underlining the therapy's potential in improving quality of life for Gaucher patients.

DEMONSTRATING A CLEAR PATH TO A COMMERCIAL PRODUCT

The company expects to announce additional data from the Phase I/II Gaucher disease programme, a key value inflection point, in H2 CY2024, and an initial safety readout from the higher dose cohort of the Phase I/II trial in AMN, a devastating neurodegenerative disease for which there are currently no approved treatments, in H1 CY2025. Both programmes represent first-in-class opportunities to bring gene therapy to life-long debilitating diseases.

2024 PORTFOLIO HIGHLIGHT

Two strategic transactions in the year

The challenging market conditions impacting the biotech sector presented a differentiated opportunity to take Freeline private. Following this transaction, Freeline completed an acquisition of Syncona portfolio company SwanBio to form Spur, creating a consolidated AAV gene therapy pipeline that includes first-in-class gene therapies in Gaucher disease and AMN. The transaction consolidates costs, drives efficiencies, provides a broadened clinical pipeline, and brings strategic synergies including clinical capabilities and manufacturing know-how.

FREELINE SwanBio THERAPEUTICS

Delivering potentially first-in-class gene therapies

SPUR

£135.6m
Syncona valuation

$2bn
Annual Gaucher market size