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# Sangamo Shoots Up As Regulatory Path Clears For Fabry Gene Therapy

by Kevin Grogan

The US group has had a tough year but the signs look more promising for isaralgagene civaparvovec as the FDA agrees on the design for a registrational trial for the gene therapy. Now Sangamo needs to secure a partner.

Shares in *Sangamo Therapeutics, Inc.* have jumped by 40% on promising data for isaralgagene civaparvovec for Fabry disease and positive talks with regulators on both sides of the Atlantic about getting the investigational gene therapy to market.

The genomic medicine company has held a type D meeting with the US Food and Drug Administration and the agency has agreed that data from "a single, adequate and well-controlled study may form the primary basis" of a biologics license application filing for isaralgagene civaparvovec, which is codenamed ST-920. Importantly, the proposed study would enrol up to 25 patients without the need for a control arm as the FDA has agreed there is no need for a head-to-head comparison with the more burdensome enzyme replacement therapies (ERTs) that are currently used to treat the rare genetic lysosomal storage disorder.

Sangamo said that this approach "enables a potentially more rapid, efficient and cost-effective pathway to BLA submission than originally anticipated." The California-headquartered group also noted that the European Medicines Agency has granted PRIME (priority medicines) scheme eligibility to ST-920, a program designed to optimize development plans and speed up approval processes for therapies that target an unmet medical need.

The company also expects data from the Phase I/II STAAR study to be used in future filings. Updated results from the trial showing sustained clinical benefit and a differentiated safety profile across 24 patients were shared at the WORLDSymposium in San Diego last week and Sangamo noted that all 13 patients withdrawn from ERT remained off it. Enrolment is complete with 33 patients signed up, 29 have been dosed so far with the one-time infusion and dosing of

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the other four is expected in the first half of 2024.

Chief development officer Nathalie Dubois Stringfellow said the company was "thankful for the FDA's support and alignment on a regulatory pathway that could potentially deliver a new treatment option for Fabry disease patients on an expedited, cost-effective timeline." She also expressed appreciation for support from the EMA, adding that "Fabry is a debilitating disease in need of new medicines and we are grateful that regulatory agencies across geographies recognize this and support our proposed development plans."

***Sangamo's Fabry Gene Therapy May Be A STAAR In The Making***

By Kevin Grogan

05 Nov 2021
The US biotech has presented interesting preliminary data from the first four patients treated in the Phase I/II STAAR study evaluating isaralgagene civaparvovec for Fabry.

*Read the full article here*

Sangamo stressed that it would not be making any investments in planning for a registrational trial "until a collaboration partnership is secured" but analysts at Jefferies issued a note on 13 February saying that the FDA alignment and the updated STAAR data should help with partnering talks. Sangamo management told the broker that it was sharing the study proposal and FDA correspondence on ST-920 with potential partners and "with regenerative medicine advanced therapy (RMAT) designation, they have an option for another multidisciplinary meeting with FDA, which they could exercise to attend with their partner."

Getting a partner would also bring in some much-needed cash for Sangamo which announced another round of job cuts in November which affected 162 staff, around 40% of the workforce and followed the loss of 120 roles earlier in 2023. The aim is to reduce operating costs from $240m-$260m last year to $115m-$135m in 2024; the company ended the third quarter of 2023 with cash equivalents of $132.1m which, with the restructuring measures, should be sufficient to fund operations into the third quarter of this year.

The cutbacks, which also led to the closure of the firm's headquarters in Brisbane, CA and the departure of various senior executives including chief operating officer Mark McClung and chief scientific officer Jason Fontenot, came after *Novartis AG* and *Biogen, Inc.* had walked away from respective deals that were focused on Sangamo's zinc finger gene-editing technology. The attraction of the latter has waned of late as more recent gene-editing technologies such as CRISPR and base editing have come to the fore. (Also see "*Double Blow For Sangamo's Zinc Finger Approach As Novartis And Biogen Deals Collapse*" - Scrip, 21 Mar, 2023.)

Sangamo is now focusing on epigenetic regulation therapies for neurological diseases and its

novel adeno-associated virus (AAV) capsid delivery technologies. Last summer, it inked a deal with *Prevail Therapeutics Inc.*, giving the *Eli Lilly and Company* subsidiary rights to AAV capsids developed using Sangamo's SIFTER platform in a deal that could be worth over $1bn. (Also see "*Deal Watch: Sangamo Inks Significant Option Deal With Prevail After Losing Tie-Ups With Novartis, Biogen*" - Scrip, 18 Jul, 2023.)

The company is on track to submit an investigational new drug application for its NaV1.7 inhibitor in pain and is looking for a partner for TX200, its autologous CAR-Treg cell therapy for patients receiving an HLA-A2 mismatched kidney from a living donor which is being evaluated in the Phase I/II STEADFAST study.