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Santhera's Transformation Should Turn Investors' Heads

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Publication date: 28 January 2022

THE SWISS BIOTECH HAD A TOUGH time after the failure of idebenone for Duchenne muscular dystrophy, but CEO Dario Eklund tells Scrip that with a new soon-to-be submitted compound, vamorolone, it has an effective and much safer alternative to steroids for the treatment of the progressive muscle degeneration genetic disorder.

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The scale and speed of the successful turnaround at Santhera Pharmaceuticals has gone largely unnoticed by the investment community but a restructuring and a very promising Duchenne muscular dystrophy (DMD) project suggest that the Swiss biotech's comeback is nearly complete.

The change in fortune has been led by Dario Eklund who joined Santhera as CEO in December 2019 and he has had a baptism of fire. The trouble started not long after, when the firm was forced to halt a Phase III study for its now-defunct DMD drug, idebenone, after it failed an interim analysis. Plans had been well advanced for a potential launch throughout Europe and tough decisions had to be made on staffing and financing. (Also see "Santhera Pulls EU Filing For Failed DMD Candidate" - Pink Sheet, 11 Nov, 2020.)

"By the end of summer 2020, we were close to being clinically dead," Eklund told Scrip in a recent interview. "Idebenone had failed in Phase III and we had no pipeline," he noted, as this was before it got hold of the rights

from US biotech Reveragen BioPharma, Inc. to another DMD project in vamorolone, a novel steroid-like drug.

Getting hold of those rights would have cost CHF30m ($33m) and Santhera had 125 full time employees on board ready to launch idebenone for DMD – it is already approved as Raxone for the treatment of Leber's hereditary optic neuropathy (LHON) and partnered with Chiesi. "We also had a CHF60m convertible loan that was due in February 2022," Eklund said. "Almost an impossible situation to get out of but we did."

Santhera restructured the debt, raised cash, reduced headcount to 40 and acquired the vamorolone rights globally, in all indications and geographies. That move looks to have paid off following the successful results from the pivotal Phase IIb VISION-DMD study where 6mg/kg/day doses of vamorolone proved to be statistically superior versus placebo, meeting the primary endpoint of time to stand. The study also showed that vamorolone had comparable efficacy to prednisone, commonly prescribed in DMD but importantly had significantly fewer side effects typically associated with steroid treatment such as stunted growth and behavioral problems in pre-specified analyses. (Also see "Santhera Raises CHF45m Ahead Of Duchenne Drug Filing" - Scrip, 23 Sep, 2021.)

Shabir Hasham, head of medical affairs at Santhera, told Scrip that while steroids work, they come with "phenomenally life changing side effects," such as obesity, stunting growth, delayed puberty, fractures, skin problems, cataracts, diabetes and behavioral issues, "and that's on top of the disease." Every child is put on steroids as soon as they are diagnosed but often "they just can't handle the side effects, so there's a huge unmet medical need for something that has a more benign side effect profile while giving you the inflammatory efficacy that steroids do."

He noted that when DMD patients start treatment on prednisone, they stop growing but "we've seen very clearly with vamorolone that it does not stunt growth, so kids grow at a normal rate compared to their peers." Hasham said that in addition to the "phenomenal height advantage," studies of bone biomarkers are revealing differences between vamorolone and standard-of-care steroids, "so we hope to show there'll be a fracture advantage."

Vamorolone will be filed with the US Food and Drug Administration before the end of the first quarter and Eklund said: "If everything goes according to plan and with a little bit of tailwind, we should get approved by the end of this year, though it may slip into early next year. We don't really have a lot of time, so I've got a year, year and a half to build a robust organization there."

Having said that, he pointed out that Santhera had had a presence in the US for a number of years even before the vamorolone program and had a good relationship with key opinion leaders in the DMD field. "The company is not unknown in the DMD community and among the patient advocacy groups, and we have a pretty good insight into the market already but it's about getting more feet on the ground so we can have an impactful launch when we go."

Going It Alone

Eklund stressed that Santhera could make a success of the launch on its own. "The DMD market is very concentrated market, there's only about 150 centers of excellence that treat these patients and all of them are diagnosed. So you don't need to run a huge campaign and it's relatively easy for a small company like ours to tap into the market without having to give away a big chunk of our margin to a partner."

A strong senior management team has been assembled by Eklund who spent over a decade with Organogenesis in the US himself before taking on role of chief commercial officer of Vifor Pharma Group in Switzerland. This includes Stephanie Brown, who was hired as North America president in December from Ipsen SA where she headed up its rare diseases franchise across the Atlantic.

While the US is the major focus, with Eklund predicting that two-thirds of future vamorolone revenues will come from there, earlier this month Santhera inked a license agreement with Sperogenix for the drug in rare diseases in the Greater China region. It will receive a double-digit upfront cash payment plus short-term US regulatory milestones amounting to $20m combined, in a deal valued at up to $124m.

Sperogenix plans to file vamorolone for DMD in China upon FDA approval which could lead to market entry as early as 2024. DMD is on the list of rare diseases with a high medical need recognized by the Chinese government and its prevalence of DMD could be as high as 70,000 patients.

Santhera estimates the peak sales potential for vamorolone in DMD alone to be over $500m in the US and the largest five European countries combined. Eklund told Scrip that for Santhera, "it's a very different world we live in right now than a year and a half ago, but I don't really feel the story is well understood yet by the market."

Not many analysts cover Santhera at the moment and the COVID-19 pandemic "has prevented us from physically meeting with a lot of people we would like to meet with and it's not always the same to do it over a Zoom call," he added. "Hopefully, that will change now and people will see we have consistently delivered on what we had committed to do over the last two years. "