Santhera Pharmaceuticals (USA) Inc.
25 Corporate Drive, Suite 250
Burlington, MA 01803 USA
Phone: +781-365-0700 | Fax: +781-365-0230
www.santhera.com

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VISION-DMD 48-week study readout: Update from Santhera

Dear Duchenne community,

We are writing to provide an update on the VISION-DMD Phase 2b study conducted by Santhera and our partner ReveraGen BioPharma investigating vamorolone for the treatment of Duchenne muscular dystrophy. Vamorolone is an investigational drug in clinical development that has not been filed in any market so far.

We are pleased to report in this letter to the Duchenne community, and in our **press release,** that the 48-week top line data readout for vamorolone shows that the statistically robust efficacy we demonstrated at 24-weeks is maintained across multiple efficacy endpoints at 48 weeks for vamorolone 6mg/kg/day. This dose was superior to vamorolone 2m/kg/day for time to stand (TTSTAND) velocity and 6-minute walk test (6-MWT) but was not statistically different for time to run walk 10 meters (TTRW) and the North Star Ambulatory Assessment (NSAA).

The 48-week data showed that switching from prednisone to vamolorone after a 4-week taper did not result in a loss of efficacy. Results also showed reversal of growth stunting and fewer adverse events usually associated with prednisone use, including fewer reports of behavioral changes. Body Mass Index (BMI) was stable for subjects who were treated with either dose of vamorolone throughout the study in assessments at week 24 and week 48. The BMI of subjects who were initially randomized to prednisone until week 24 stabilized their BMI when switched to either dose, 6 or 2 mgs/kg/day, of vamorolone until the end of the study. Overall, treatment was well tolerated with two subjects discontinuing treatment during the second half of the study. There were three serious adverse events reported in the study but they were not thought to be related to vamorolone by investigators. Further details of the study topline outcomes can be found in the press release. As these are top line data, additional analyses are currently being conducted which Santhera will present at upcoming congresses.

Corticosteroids are a cornerstone of treatment in DMD and have been shown to be efficacious but their long-term use is limited due to their side effects. These data presented today support our ambition that vamorolone may have the potential to be an alternative to prednisone.

On November 17, Santhera and ReveraGen announced the successful completion of a first pre-NDA (New Drug Application) meeting with the U.S. Food and Drug Administration (FDA) for vamorolone for the treatment of DMD. The FDA considered both the proposed clinical efficacy and safety data of the 24-week outcomes sufficient for an NDA filing. Acceptance of the NDA will be subject to FDA`s review of the complete filing. Based on previously granted Fast Track Designation for vamorolone, the FDA also deemed the plan to pursue a rolling NDA review acceptable and that is planned to commence in Q1-2022. For more information about the NDA, see our press release. In the EU, the submission of the full Marketing Authorization Application is planned by the end of Q2-2022.

In closing, we would like to take this moment to express our deep appreciation to the families who participated in the VISION-DMD study. Your commitment to the study, even in the midst of a global pandemic, was remarkable and is acknowledged. As a company, Santhera is unyielding in our pursuit of treatments to slow the progression of Duchenne muscular dystrophy. On behalf of Santhera, we will continue to keep you posted on our progress.

If you have any questions or if we can be of any further support, please do not hesitate to contact Mindy Cameron, our Global Patient Advocacy ambassador, at Mindy.Cameron@santhera.com

With warm regards,

Dario Eklund
Chief Executive Officer

Mindy Cameron
Head of Global Patient Advocacy