Santhera Pharmaceuticals (USA) Inc.
25 Corporate Drive, Suite 250
Burlington, MA 01803
Phone: (781)365-1794
www.santhera.com

January 29, 2021

U.S. Duchenne community update from Santhera

Dear Duchenne community,

As we head into the new year with all the promise that 2021 holds, Santhera continues to be a dedicated partner to the Duchenne community and remains committed to move the field forward through innovative therapy development. While 2020 brought great challenges to Santhera as a company, as it has for many in the Duchenne community, we ended the year a much leaner team than when we started. We continue to be hopeful about the year ahead and the pivotal phase 2b trial for vamorolone that will be completed in the second quarter. Vamorolone, a dissociative steroid, is being investigated as an alternative to traditional glucocorticoids to treat Duchenne muscular dystrophy.

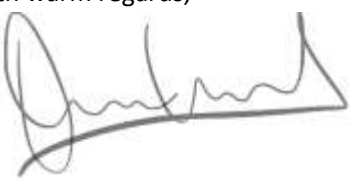
Glucocorticoids, or "steroids" as many people refer to them, have been the standard of care and first line of defense for years to slow the progression of muscle weakness in boys with Duchenne. The benefits of steroids are well-documented and study after study have shown they help prolong ambulation, maintain upper body strength, delay ventilator use, delay scoliosis, delay the onset of cardiomyopathy and in some cases eliminate the need for scoliosis surgery, and more[1234]. Unfortunately, the side effects of steroids can be life-altering and can include significant weight gain, bone fragility, growth stunting, delayed puberty, suppressed immunity, metabolic alterations, as well as mood and behavior changes[56]. For many families, this is a difficult price to pay for slowing the loss of function.

Dr. Eric Hoffman and his colleagues at ReveraGen aim to develop a drug that could potentially retain the anti-inflammatory benefits of steroids but decrease the side effects that typically accompany their long-term use. We await important results from the phase 2b clinical trial, which we expect to have data from in the second quarter. In September 2020, Santhera licensed vamorolone from ReveraGen and teams from both companies are working to develop vamorolone as an alternative to glucocorticoids in Duchenne.

We would like to close by expressing our appreciation to all the families participating in the VISION DMD and SIDEROS studies who have helped us answer important questions about these investigational drugs. We know that access to data is important to clinical trial participants and the DMD community. Santhera hopes to share individual data with the SIDEROS trial participants, and are working with advocacy groups, and clinical and regulatory experts to determine the feasibility of doing this after a trial has closed. If you are participating in one of the vamorolone clinical trials, please contact Suzanne Gaglianone at Suzanne.gaglianone@reveragen.com to opt-in to receive your study data. Suzanne will walk you through the process and timelines for receiving your individual study data.

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 in 2021.

With warm regards,



Dario Eklund
Chief Executive Officer

Jodi Wolff
Head of Global Patient Advocacy

[1]McDonald et al. (2018) Long-term effects of glucocorticoids on function, quality of life, and survival in patients with Duchenne muscular dystrophy: a prospective cohort study. *The Lancet, 391*(10119), 451–461. https://doi.org/10.1016/S0140-6736(17)32160-8

[2]Sawnani et al. (2019). Comparison of pulmonary function decline in steroid-treated and steroid-naïve patients with Duchenne muscular dystrophy. *The Journal of Pediatrics, 210,* 194-200. https://doi.org/10.1016/j.jpeds.2019.02.037

[3]Barber et al. (2013). Oral corticosteroids and onset of cardiomyopathy in Duchenne muscular dystrophy. *The Journal of Pediatrics, 163,* 1080-4. http://dx.doi.org/10.1016/j.jpeds.2013.05.060

[4]Angelini, C., & Peterle, E. (2012). Old and new therapeutic developments in steroid treatment in Duchenne muscular dystrophy. *Acta myologica : myopathies and cardiomyopathies: official journal of the Mediterranean Society of Myology*, *31*(1), 9–15.

[5]Parent Project Muscular Dystrophy. (2021). *Care Guidelines – Steroids.* https://www.parentprojectmd.org/care/care-guidelines/by-area/steroids/

[6]CureDuchenne. (2021). *Steroids in Duchenne: What you need to know.* https://www.cureduchenne.org/care/steroids/