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

Looking ahead to 2019

Dear Duchenne community,

The beginning of a new year often is a time of reflection of the year gone by and anticipation of the year ahead. 2019 holds immense promise for the Duchenne muscular dystrophy field, and we at Santhera look forward to playing a prominent role in that progress. In 2018, we were hard at work securing the path forward for therapies for those with Duchenne with the fewest treatment options – boys and men who are experiencing declines in their respiratory function, a majority of whom are non-ambulatory. In 2019, we broaden our scope in DMD. Now with two investigational therapies in our pipeline, idebenone and vamorolone, we aim to provide new treatments to virtually all people with Duchenne regardless of ambulation status.

In 2018, Santhera, supported by clinical experts, was able to conduct additional research to support the validation of respiratory endpoints for DMD and examine the long-term effects of idebenone use, predominantly in non-ambulatory boys. We look forward to sharing that data with the community upon its publication by our external scientific partners. In the U.S., Santhera launched a formal expanded access program called *Breathe DMD*, which gives boys and men ages 8 and older an opportunity to obtain investigational idebenone, given certain criteria be met. Santhera continued to enroll its phase III placebo controlled trial (SIDEROS) with idebenone in boys and men using glucocorticoids at approximately 60 trial sites, 20 of which are distributed across the U.S. The first participants enrolled in the SIDEROS trial are now completing 78 weeks of randomized treatment and are invited to enroll in the open-label extension phase in which all participants receive idebenone.

In late 2018, we announced that Santhera has obtained the exclusive option to license *vamorolone*, the steroid-replacement drug in a phase IIb pivotal trial by ReveraGen. Their Vision-DMD trial is currently recruiting boys ages 4-6 years who have never taken steroids. To find out how to be a part of the trial visit https://vision-dmd.info/2b-trial-information/ and www.clinicaltrials.gov.

In 2019, with your help, we anticipate to complete enrollment of the SIDEROS clinical trial, which you can find out more information about at www.siderosdmd.com or www.clinicaltrials.gov. We will continue to expand the number of physicians participating in the Breathe DMD Expanded Access Program, which you can read more about at www.breathedmd.com. Most importantly, we continue to talk with the U.S. FDA to find the best path towards approval of idebenone to treat respiratory dysfunction in boys and men with DMD. Additionally, we look forward to working with the ReveraGen team to support their efforts to expedite the development of vamorolone.

Santhera has been a proud partner of the DMD community for over 10 years, and we enter into 2019 with a fighting spirit to bring treatments to you and your children as soon as possible. We remain thankful for the support of all of the advocacy groups for their continued collaboration and guidance.

On behalf of the Santhera team, we wish you a 2019 filled with happiness for your families and progress in the fight against Duchenne.

Kind regards,

Thomas Meier
CEO, Santhera Pharmaceuticals

Jodi Wolff
Head Patient Advocacy – US