January 21, 2020

New Year update from Santhera

Dear Duchenne community,

It is the beginning of a new year and at Santhera we continue to work hard to develop treatments for Duchenne muscular dystrophy and your boys. In January and February, our U.S.-based team will be busy attending Duchenne educational workshops for families in Worcester, Tampa, Houston, Mississippi and Portland. We hope to meet many of you at one of these upcoming events hosted by Duchenne patient advocacy organizations.

Our main focus in the U.S. in the first half of the year is to bring the SIDEROS clinical trial to full enrollment and we ask the community’s help, now more than ever, to achieve this goal. The SIDEROS clinical trial, which investigates the safety and efficacy of idebenone in boys using steroids, is the only U.S. trial currently recruiting boys with DMD regardless of ambulation status and genetic mutation. The trial also has no upper age limit and is the largest clinical trial ever to study respiratory function in DMD or non-ambulatory participants. Additionally, there are provisions in place to treat non-eligible siblings when a participant is enrolled. Trial participants who have completed the trial are able to receive idebenone as part of the post-trial open label extension period.

Who can be a part of the SIDEROS clinical trial? Trial participants must be aged 10 or older, be on a stable dose of steroids for six months, and have a forced vital capacity between 35% and 80% predicted. Participants must either have or be willing to get their annual influenza vaccination and a pneumococcal vaccination, and cannot currently be taking Exondys, Vyondys or an investigational therapy. Additional criteria apply. To be connected with a trial site, please contact sideros@santhera.com. We encourage any family interested in clinical trial participation who may meet the above criteria to talk with their physician to get screened for the trial as soon as possible.

We know as a community that the ultimate goal is to get meaningful therapies approved for the broadest group of patients possible. The full enrollment of the SIDEROS clinical trial is essential to seek approval from the FDA for idebenone as a treatment option for DMD. Researchers and physicians alike have argued that combination therapies may hold the key to the long-term treatment of Duchenne muscular dystrophy.¹ We need your help to answer important questions about idebenone through the SIDEROS trial and, in turn, to potentially add another tool to the DMD treatment toolbox.

We thank the community for their continued support, and we cannot emphasize enough our deep appreciation to the over 200 boys and young men who are participating in the SIDEROS trial. We feel a particularly strong obligation to those boys and families to push recruitment over the finish line as soon as possible. To those in the trial, please know that you are making a difference for your peers for possibly
generations to come by helping us learn not only about idebenone but also about respiratory dysfunction in DMD.

Also important to mention is that Santhera starts the new year with a new Chief Executive Officer, Dario Ecklund. Dario joined Santhera as CEO on December 1 and comes to us with decades of experience, including serving as Vifor Pharma’s Chief Commercial Officer, Vice President of Organogenesis Inc., a member of the Board of Directors for Fresenius Medical Care, and in other roles at Novartis and Sanofi. Dario believes in forging strong relationships with the patient community and to put patients first in our work at Santhera. Under Dario’s leadership, we vow to continue our pioneering work and to ensure that every person who may benefit has access to approved therapies.

On behalf of Santhera, we wish you a happy new year. We will continue to keep you posted on our progress.

With warm regards,

Dario Ecklund  
Chief Executive Officer

Jodi Wolff  
Head of Patient Advocacy – U.S
