

March 31, 2020

Community update from Santhera

Dear Duchenne community,

Much has changed in the world since our last update to the Duchenne community in late January. Please know that during this unprecedented time, Santhera continues to work hard to develop treatments for Duchenne muscular dystrophy. In light of the existing COVID-19 pandemic, patient safety and well-being remains our highest priority in all ongoing studies.

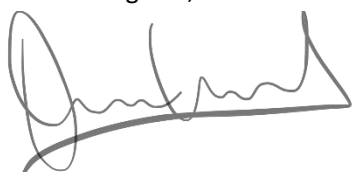
As you know from our previous updates, Santhera's main focus in the U.S. in the first half of the year has been to bring the SIDEROS clinical study to full enrollment. The SIDEROS clinical study, which investigates the safety and efficacy of idebenone in boys and young men using steroids, is the pivotal phase III study needed for FDA approval in the U.S. We are very close to full enrollment, and our efforts now are focused on supporting the current study participants and study site investigators.

Santhera is working diligently to ensure the safety of participants and the continued supply of study medication to participants in the SIDEROS and SIDEROS-Extension studies. Investigators and site staff have been provided instructions with immediate measures to guide them in case of visit cancellations. These procedures are in line with the most recent guidances provided by the U.S. Food and Drug Administration, the European Medicines Agency, and other national regulatory authorities. Provisions have been made for home or local assessments when necessary and requests for direct-to-patient shipping of medication have been received and allowed. As part of the SIDEROS clinical study protocol, participants perform home respiratory measurements using a hand held device on a weekly basis and should continue to do so throughout the COVID-19 pandemic.

We express our deep appreciation to the study participants for their dedication to SIDEROS and SIDEROS-Extension during what we recognize as a particularly stressful time for many families across the world. If you are a participant in the SIDEROS study and have questions about whether or not to attend your next study visit, please reach out directly to the team at your study site. As this is a global study, we acknowledge that conditions will vary based on country and city and that decision-making is best left to the family and their physician. Santhera will do whatever we can to support physicians and their families during this time to minimize the disruption to the study. Any additional questions to Santhera can be directed to sideros@santhera.com.

On behalf of Santhera, we will continue to keep you posted on our progress.

With warm regards,



Dario Eklund
Chief Executive Officer



Jodi Wolff
Head of Patient Advocacy – U.S