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November 19, 2019

SIDEROS clinical trial and BreatheDMD program update

Dear Duchenne community,

In February 2018, Santhera created the BreatheDMD program, an Expanded Access Program (EAP) in the United States aimed to offer investigational idebenone free-of-charge to boys and men with Duchenne muscular dystrophy (DMD) who do not meet the criteria to enroll in the ongoing SIDEROS clinical trial. The EAP program was established in response to calls from the DMD community to gain wider access to investigational drugs ahead of sometimes long regulatory processes and to address a high unmet need in the DMD population. At the time, and still now, Santhera is the only company offering a compassionate use program in the Duchenne community. In general, the program was anticipated to provide an additional treatment option to older patients experiencing respiratory decline who were not on steroids or did not meet the enrollment criteria for SIDEROS.

Expanded Access Programs like BreatheDMD are permitted by the U.S. Food and Drug Administration (FDA). Such programs allow eligible patients with serious or life-threatening diseases or conditions, where there is a lack of satisfactory therapeutic alternatives, to gain access to a medicine under investigation before it is approved by regulatory authorities.

Under these regulations, offering an EAP cannot interfere with or compromise the overall development of the drug for the broader patient community, and those who would otherwise be eligible for an ongoing clinical trial cannot be included in EAPs.

While it was our best of intentions to meet an unmet need, upon further examination it appears that offering the BreatheDMD EAP at this time is interfering with trial recruitment. Because of this, we have decided to suspend enrolling new participants in the BreatheDMD EAP until the SIDEROS clinical trial is fully enrolled. All current patients in the EAP will continue receiving investigational idebenone, but no new patients will be enrolled in BreatheDMD and no new sites will be opened at this time.

We ask the community's help, now more than ever, to achieve the goal of full recruitment of the SIDEROS clinical trial. As of today, the trial is ~90% enrolled.

The SIDEROS clinical trial investigating the safety and efficacy of idebenone in boys using steroids is the only 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 receiving 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 or an investigational therapy.

To be connected with a trial site, please contact sideros@santhera.com.

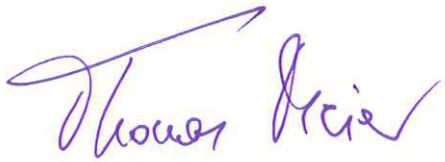
We know as a community that the ultimate goal is to get meaningful therapies approved for the broadest group of patients possible. Empowered communities of patients and families can support the development of new treatments and to help answer important scientific questions about the safety and efficacy of those treatments. SIDEROS is our second phase III trial of idebenone for boys with DMD, with the first phase III trial (DELOS) having met its primary endpoint, the change from baseline in peak expiratory flow percent predicted (PEF%p).

While the European regulators currently are reviewing our submission for conditional approval of idebenone in steroid non-users based on currently available data, the FDA has requested the results of SIDEROS before reviewing our submission for approval in the U.S. The FDA prefers to review an application of idebenone for all boys with DMD regardless of steroid use. Therefore, the full enrollment of the SIDEROS trial is essential to continued investigation or access of this therapy in the U.S.

We thank the community for their continued support, and we cannot emphasize enough our deep appreciation to the 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.

If you are interested in being a part of the SIDEROS trial or have questions about the trial, please contact us at sideros@santhera.com. To learn more about the trial, talk with your health care provider and visit www.siderosdmd.com and www.clinicaltrials.gov NCT 02814019.

With warm regards,



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