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SIDEROS trial interim analysis – update from Santhera

Dear Duchenne community,

Since 2016, Santhera has been conducting one of the largest clinical trials in Duchenne muscular dystrophy – the SIDEROS study. SIDEROS is a phase III double-blind, randomized, placebo-controlled study assessing the efficacy, safety and tolerability of idebenone in patients with Duchenne muscular dystrophy receiving glucocorticoids. We regret to inform the Duchenne community, that based on the results of the interim analysis and recommendation from the trial's Data and Safety Monitoring Board (DSMB), the SIDEROS trial has been deemed futile. Based on the results of the interim analysis, we have made the difficult decision to discontinue SIDEROS and the development of idebenone in Duchenne muscular dystrophy. This update serves to address the details of this morning's press release with the community.

What does futility mean and how was this determined?

Futility means that the probability for SIDEROS to reach its primary endpoint in the planned final analysis has become so small that continuation of the study cannot be justified any more. No difference was seen between the treatment and placebo groups in 197 trial participants who were included in the interim analysis. This was the basis for the DSMB recommendation.

What does this mean for the community?

Santhera has endorsed the DSMB recommendation and will discontinue the SIDEROS study. As a consequence, the development of idebenone in DMD will not be continued further, including SIDEROS-Extension and expanded access programs. Participating patients are asked to stop taking idebenone treatment immediately. Study sites will be in contact with study participants to schedule a final visit four weeks after stopping the investigational treatment. All study participants who have questions about the next steps should reach out to their clinical study site. Santhera will be in close communication with the study sites and investigators in the coming days.

It's important to note that the DSMB has been reviewing the safety data throughout the conduct of the study and has not detected any new safety concerns.

Why did we conduct an interim analysis?

An interim analysis in a trial is an analysis of data that is conducted before data collection has been completed. In May, Santhera determined that enough of the participants had completed the study, the power of the study was high, and the variability in the primary endpoint was lower than expected. By conducting the interim analysis and stopping the study for futility, this ensures that study participants are not exposed to a treatment that is unlikely to be of clinical benefit or exposed to placebo for longer than is necessary.

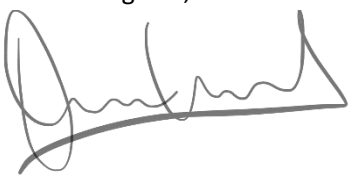
Why is this being announced in a press release?

Pharmaceutical and biotechnology companies that are traded publicly are governed by strict regulations that dictate how companies release information that may have any impact on the value of the company or stock prices. This includes the release of clinical trial results. Therefore, the interim analysis results must be released via a press release as soon as possible after the DSMB recommendation has been made. Federal guidelines do not allow us to notify the study participants first.

While this is not the news that we hoped to share with the Duchenne community, Santhera's team will fully analyze the study data to better understand the results of the interim analysis and will share additional insights with the Duchenne community via the patient advocacy groups. Santhera aims to continue data contributions to C-Path's Duchenne Regulatory Science Consortium to help with their efforts to design more efficient trials using disease modeling expertise.

In closing, we would like to take this moment to express our deep appreciation to the participants in the SIDEROS study and all of the families that support them. Your commitment to the study, even in the midst of a global pandemic, has been nothing short of remarkable. We acknowledge that the discontinuation of a trial and development program is disappointing to the Duchenne community and we share your sentiment on these results. As a company, Santhera will remain committed to developing therapies for Duchenne muscular dystrophy. The Duchenne community is a model of resilience and we will keep working hard for your families.

With warm regards,



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