Product and Application
Business Support
European Medicines Agency
Domenico Scarlattilaan 6
1083 HS Amsterdam
The Netherlands

Lörrach, 28 October 2020

Formal Notification of Withdrawal for the Puldysa EU Marketing Authorisation Application
Puldysa (idebenone) 150 mg film-coated tablets; Procedure No. EMEA/H/C/005123

On Monday 5 October 2020, the independent Data and Safety Monitoring Board (DSMB) for the ongoing pivotal study SIDEROS conducted an interim analysis for the primary efficacy endpoint on the available data, based on stopping rules pre-specified in the protocol amendment 8.0 dated 22 June 2020 and according to the Statistical Analysis Plan.

Upon review of the data, the DSMB recommended to stop SIDEROS due to futility, as the probability to achieve statistical significance in the final analysis for the primary endpoint was calculated to be very low (<5%), based on the interim results.

As a consequence, Santhera Pharmaceuticals made the difficult decision to discontinue the entire Duchenne Muscular Dystrophy (DMD) development program for idebenone, terminate SIDEROS and its extension SIDEROS-E and withdraw the ongoing EU Centralised application for Puldysa.

The Applicant understands that positive efficacy results from SIDEROS would have been instrumental not only to substantiate a positive benefit-risk ratio in the DMD patients using glucocorticoids, but also to provide confirmatory evidence of the efficacy previously shown with the smaller DELOS study in DMD patient not using glucocorticoids.

This letter follows the electronic communication made to the Agency on 6 October 2020, notifying the withdrawal.
We consider the procedure closed, unless the Agency requires additional submission of documentation.

Should you have any questions, or require any further information, please do not hesitate to contact me.

Yours sincerely,