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To the European members of the United Parent Project Muscular Dystrophy (UPPMD)

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Liestal, 3 October 2017

Subject: Update on the Application for Marketing Authorization for Raxone® (idebenone 150 mg) in Duchenne Muscular Dystrophy (DMD)

Dear Member of UPPMD,

I am writing today to provide you with an update on our European marketing authorisation application for Raxone® for patients with Duchenne muscular dystrophy (DMD). On the 15th of September, the EMA's Committee for Human Medicinal Products (CHMP) issued a negative opinion and expressed uncertainties whether the phase III DELOS study provides sufficient evidence of efficacy to allow for approval of Raxone in this indication at this time. It should be noted that this opinion was based on a majority vote by the CHMP; there were also dissenting votes in favour of granting a marketing authorization.

While my colleagues and I are disappointed by this opinion, I realize that this is even more difficult for patients and their families. I would like to assure you that we remain fully committed to providing this much needed treatment to patients. As a first step we have filed a request for re-examination. With this, we are appealing the negative opinion issued by the CHMP. In our arguments for the CHMP we will focus on the existing high unmet medical need as well as the fact that the DELOS study met its primary endpoint and showed statistically significant, and in our view, clinically relevant efficacy in this patient population, as has been extensively published. We are convinced that DMD patients not receiving corticosteroids and in respiratory function decline deserve to have this treatment option available.

As you are aware, Santhera has been committed to developing Raxone for the treatment of respiratory function decline in patients with DMD for more than 10 years now. During this time, we have conducted the Phase III DELOS study, the first and only successful randomized, placebo-controlled clinical study in non-ambulatory patients. We have been collaborating with internationally recognized experts to better understand the natural history of respiratory function decline, and we have engaged with many patient groups around the globe in order to understand patients' needs and concerns. The objective has

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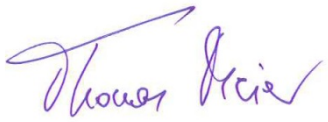
always been to provide a medicine that can deliver benefit to patients who have insufficient treatment options.

In order to help patients and their families address some of the questions that might come up in the current situation, I am attaching a Question & Answer document, which should provide additional background and answers related to this recent event. Please share this information as you see appropriate.

I look forward to meeting you at some point in the near future. In the meantime, please don't hesitate to contact members of our team for further clarifications or to share your comments.

Sincerely,



Thomas Meier, PhD
CEO

Answers to Frequently Asked Questions

Q1. What was the regulatory process for approval of Raxone in DMD?

Santhera submitted the marketing authorization application for Raxone in DMD as an extension to its pre-existing marketing authorization for Leber's Hereditary Optic Neuropathy (LHON). The new indication in patients with DMD would be added to the existing label for patients with LHON.

Q2. Why did EMA's Committee for Human Medicinal Products (CHMP) issue a negative opinion?

The CHMP expressed doubts whether the phase III DELOS trial provides sufficient evidence of efficacy to allow a Type II variation of Santhera's existing marketing authorization for Raxone.

Q3. Were there any safety concerns?

Raxone is currently approved in Europe for another indication (LHON) and its safety profile is well-known and remains unchanged.

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Q4. What is the appeal process with the CHMP?

It's a regulatory process whereby the CHMP re-examines the application dossier. This re-examination process will allow us to re-position our arguments and to further interact with regulators, with the goal to making this medicine available to eligible patients at the earliest time possible.

Q5. What is the time frame for this re-examination procedure?

We expect a final outcome in the first quarter 2018.

Q6. How does this decision affect the ongoing SIDEROS study?

As the safety profile of Raxone remains unchanged, all clinical trials should continue as planned. The CHMP has issued a similar statement in their communication.
The objective of the SIDEROS study is to establish the efficacy of Raxone on respiratory function in DMD patients using glucocorticoids.

Q7. How does this decision affect local Early Access Programs?

All ongoing early access programs, particularly the EAMS in the UK, proceed as planned.
There is no change in the regulatory position by the MHRA on the status of the EAMS.

Q8. How will Santhera prepare for the Appeal procedure?

We will work with clinical experts and representatives of the patient organizations to prepare our arguments for this Appeal procedure.

Q9. What happens if the Appeal isn't successful?

Should the Appeal also result in a negative opinion, then the decision of the CHMP is final. This closes the procedure. In this case a new file with additional data can be submitted for review by the CHMP under a new marketing authorization application. Such data could come from the SIDEROS study or possibly other sources (e.g. retrospective data collections).

Q10. Can I get access to Raxone in my country until the granting of a marketing authorization has been granted?

Today, Raxone is approved only for the treatment of LHON. Patients with DMD may have access for not approved medicines via compassionate use, clinical studies or other specific early access programs according to local regulations.