PAG Communication: Prosensa Outlines Next Steps for Drisapersen Program

Leiden, February 18th, 2014

Dear Patient Group Representative,

We hope this note finds you well. When we last communicated, we announced that Prosensa had regained all rights to drisapersen from GSK and retained the rights to the other DMD programs.

Upon the announcement of the transfer of rights, GSK and Prosensa entered into a 120 day transition period (beginning on January 13), in which a joint transition team will transfer to Prosensa relevant data, know-how, and drug product related to drisapersen. Prior to this announcement, we were involved in the ongoing analyses related to drisapersen, and we will shortly have complete access to the drisapersen database which comes with the transfer of rights to Prosensa. The drisapersen clinical program comprises a very large dataset in DMD, which will take time for us to completely transfer and evaluate, thus we do foresee the transition period as necessary in order to assess the feasibility and reach decisions on the possibility of bringing drisapersen to as many boys as possible in a timely and efficient manner. We have asked the investigators to contact boys and their families to assess their interest in redosing with a request to respond by March 10th. We will provide an update once we have this information.

We are actively assessing all available opportunities to potentially provide access to drisapersen to meet the needs of boys who have previously participated in drisapersen clinical trials. The extension study from the Phase II and Phase III studies, DMD114349, took place in more than 50 sites across 24 countries. Due to our size, Prosensa has limited operational capability and does not have the resources to take over management of the clinical trial sites immediately. Furthermore, as the protocol for the study was changed in September to hold dosing of drisapersen, the planned drug treatment period of two years cannot be achieved. We also want to consider the significant burden that continued monitoring in this study can place on patients and parents during this time. Therefore, we have decided that it is best to close the DMD114349 extension study and work on possible re-dosing plans for participants of this study separately. Others studies that are still open include DMD114501 (US only – 3 sites) and DMD114673 (Europe 2 sites). We will also consider re-dosing for boys involved in these studies.

The closure of study DMD114349 is only the first step in a complex path to providing renewed access to drisapersen for patients. During this time we will evaluate the options for potential re-initiation of drisapersen dosing. We ask for your continued support and patience as we work through this activity. Feedback from patients and investigators regarding the willingness and desire to go back on drisapersen. A survey has been sent to investigators with a request for response by March 10th.

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As we go through this process, several criteria are important for us to assess and implement, including:

- Feedback from patients and investigators regarding the willingness and desire to go back on drisapersen. A questionnaire has been sent to investigators with a request for response by March 10
- Prospect of an acceptable regulatory path forward for drisapersen
- Determination of the appropriate program to provide access to drug
- Sufficient clinical supplies to meet the needs of boys being re-dosed
- Regulatory approvals necessary to complete clinical trials
- Operational infrastructure in place within Prosensa to ensure the safety of participants in clinical trials

Given the number of sites and countries involved, potential re-dosing plans will likely take a staged approach. We will continue to work closely with patient groups, investigators, academia and regulators to ensure we do what is best for the patient community and ensure an optimal outcome for drisapersen and our other DMD programs. As we achieve necessary milestones in the ability to provide drug to boys, we will communicate these updates as soon as we are in a position to do so.

We thank you again for the crucial contributions that the patient community and patient groups have made to advance the Duchenne compounds and are extremely grateful for your support. With no long term disease modifying therapies currently available for patients with DMD, it is of critical importance to continue the development of treatment options for this devastating disease. Our primary focus remains with these boys and their families, and we will continue to work on ways to enable them a brighter future.

Kind regards,

Giles Campion, MD – Chief Medical Officer and SVP Research & Development

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