Community Update

17th April 2019,

In response to your request for information, please find an update on the progress being made to register SPINRAZA (nusinersen) in Russia and the plans in place to open an Expanded Access Program (EAP).

Registration

Janssen, our partner in Russia submitted the Spinraza dossier for registration on the 8th November 2018. Regulators are reviewing the totality of the data available and will determine approval and the final nusinersen label. On the 24th January 2019, the Ministry of Health of the Russian Federation issued their decision to grant nusinersen orphan drug status and the possibility to consider the drug for the treatment of SMA. Once approved by the Russian Regulatory Authorities, the next important steps in the process are pricing and reimbursement.

Expanded Access Program (EAP)

In 2016, based on the high unmet need in SMA, Biogen sponsored an EAP for eligible individuals with infantile-onset SMA (most likely to develop Type 1). It was initiated at existing nusinersen clinical trial sites and then expanded to multiple countries where EAPs are permitted according to local laws and regulations, where it can be operationalized and where there is a path to long-term availability and reimbursement of SPINRAZA® (nusinersen). The EAP is now closed in nearly all countries and no new patients are being enrolled. To date, the EAP has provided treatment access to more than 750 patients across 29 countries.

In response to the progress being made and to the requests from clinicians and the SMA Family Foundation, Biogen is pleased to announce its decision to now open an EAP in Russia. The EAP is intended to treat the most vulnerable Russian citizens suffering from SMA (infantile-onset) ahead of obtaining Marketing Authorization Approval (MAA) from Russian Regulatory Authorities.

The program is expected to open in 23rd April 2019 and will close when the MAA for nusinersen is granted. During this time, Biogen will supply the medicine free of charge. This program is limited to 40 patients who meet the inclusion criteria as outlined by Biogen. The decision to treat these patients will be at the discretion of the physician, the patients and their families and the specific center's institutional policies. It is important to emphasise that Biogen and Janssen, by law, will not be proactively involved in the patient selection process for the EAP.

Given the complexities and operational and compliance aspects of opening an EAP in Russia, Biogen are limited in our ability to share any further details with the community at this stage. We greatly appreciate the critical role you and your organization play in representating the voice of those living with SMA and we will continue to work closely with you when possible to ensure the perspectives of patients and families are reflected in the ongoing decisions we make.

We look forward to the commencement of the EAP and remain committed to obtaining regulatory approval as soon as possible so that the lives of many people living with SMA in Russia are improved.

For further information, please do continue to contact us.

Best regards,

Sabrina Paillé, MD on behalf of the Biogen SMA IPM Team