1 July 2019

Dear SMA Family Foundation Russia,

Thank you for your letter and request for community information. We take the view of the patients very seriously and are very mindful of the immense frustration within the Russian community at the pace of drug development in spinal muscular atrophy (SMA).

We would also like to acknowledge the tremendous support and partnership with the SMA Family Foundation.

On 24th of May 2019, a major milestone has been reached in the fight against SMA! The US Food and Drug Administration (FDA) has approved ZOLGENSMA® (onasemnogene abeparvovec-xioi) for the treatment of children less than 2 years old with SMA. This monumental day represents years of collaboration and the perseverance of caregivers and patients, patient advocacy groups, researchers, healthcare professionals, treatment teams, AveXis employees, and the FDA. Because of this collaboration, families now have a new treatment option that targets the genetic root cause of SMA with a one-time-only gene therapy.

In other areas, including Europe, Middle East and Africa (EMEA), AVXS-101 is still an investigational drug. AveXis plans to make ZOLGENSMA® available to patients affected by SMA globally, and simultaneous priority registration filings started in 2018 in the US, Europe and Japan. Preparations are underway with the intention to file for registration in other countries, including Russia.

In the interim, AveXis has arranged to make the product available for international markets, subject to local laws and regulations, as a part of its paid Managed Access Program (MAP).

The intent of the MAP is to provide access to ZOLGENSMA® for eligible patients. The following criteria must be met by a patient to participate in the AveXis MAP:

•The patient to be treated has a serious or life-threatening disease or condition, and no satisfactory alternative therapy is available, or has exhausted approved treatment options to monitor or treat the disease or condition.
•The patient is ineligible for enrollment into or unable to access ongoing AveXis clinical trials.
•Sufficient information exists to believe the potential benefit of treatment outweighs the potential risk in the context of the disease or condition to be treated.
•AveXis has an adequate supply of product and providing the product will not interfere with ongoing clinical trial(s) or with the overall development program.
•The patient meets any other important medical criteria established by the medical experts working on the product development program.
•Pediatric patient less than 2 years of age with SMA that satisfied the following specified criteria:

◦Diagnosis of SMA based on gene mutation analysis with bi-allelic SMN1 mutations (deletion or point mutations).
◦Weight: ≥ 2.6 kg at dosing.
◦Anti Adeno Associated Virus Serotype 9 (AAV9) antibody titer < 1:50

For this program, funding will need to be secured to provide access to ZOLGENSMA®. A US commercial product will be used in this program. It is the responsibility of the patient, their family, or the healthcare institution to secure funding for treatment.

For inquiries about AVXS-101, please contact AveXis EMEA Medical Information at medinfo.emea@avexis.com or +351 30 880 0322.

We encourage the SMA community to monitor www.clinicaltrials.gov and patient organization websites such as https://www.sma-europe.eu/ for potential new clinical trials and treatment options. We also encourage families to talk to their physician about other available treatment options.

AveXis would like to thank the SMA Family Foundation in Russia for all the continued support and partnership.

Thanks again for your interest, and for reaching out to us.

Nadia Andelova

Medical Information EMEA