



Dear SMA Community,

Since its U.S. approval, AveXis has had increasing demand from families outside of the U.S. for access to AVXS-101. While we are pursuing registration in close to three dozen countries, we have heard the many requests from the SMA community for a global Managed Access Program (MAP)* to make this innovative gene therapy available to patients in immediate need.

We are pleased to take this critical step in offering a global MAP to provide AVXS-101 free of charge to eligible patients with SMA who are under the age of two and are a citizen or legal resident of a country where the therapy is not yet approved by local health authorities.

While we continue expanding our manufacturing capabilities, at this time AveXis has one facility licensed to produce AVXS-101 and our first obligation is to provide the therapy where it has been approved, is pending regulatory approval and to clinical trials. Given these constraints, we challenged ourselves and engaged in dialogue internally and externally to explore ways in which a global MAP could be achieved while meeting all of our obligations. AveXis designed a program anchored in principles of fairness, clinical need and global accessibility to best determine the equitable global distribution of a finite number of doses that doesn't favor one child or country over another. AveXis collaborated with an independent bioethics advisory committee to develop the program.

The global MAP will launch in January 2020 with 50 doses allocated for the first half of the year and up to 100 total doses planned for 2020. AveXis' intention is for this to be a long-term commitment, with additional doses added to the program on a rolling six-month basis based on patient need and the availability of supply.

At AveXis, we have worked diligently to create new pathways for access for one-time gene therapies, a new and developing area of medicine. Recognizing that the program will not be a solution for all families in all countries, we continue to work hard to design sustainable solutions to further expand global access to AVXS-101.

Sincerely,
The AveXis Team

**The Novartis "Managed Access" terminology covers all locally defined pre-approval access mechanisms and programs such as "Compassionate Use", "Expanded Access", "Named Patient Supply", "Special Access Schemes/Programs", "Autorisations temporaires d'utilisation (ATU)" and others.*

Disclaimer: The global Managed Access Program described here is not an advertisement for AVXS-101. It is intended solely as a preliminary safeguard of worldwide patient care until market approval is granted in all countries other than the United States. The provision, delivery and application to patients is subject to local country specific legal and regulatory framework.

Frequently Asked Questions

1. What are the eligibility requirements for the program?

Patients must be medically eligible to participate in the program: clinical criteria include any patient under the age of two with genetically confirmed SMA, regardless of type, symptom onset or prior treatment. Geographically, the patient must be a citizen or legal resident in a country where AVXS-101 is not approved by local health authorities. The treatment must be administered at a trained center and Health Authority approval must be obtained in the country where the patient will be treated and has been deemed eligible.

2. How are requests for the global MAP submitted?

Beginning January 2, 2020, a patient's treating physician may submit a request for managed access on their behalf by contacting AveXis' third-party partner, Durbin, at AveXisMAP@DurbinGlobal.com. Due to regulatory restrictions, a request must be submitted by a treating physician.

3. How will patients be selected to receive AVXS-101?

AveXis designed a program anchored in principles of fairness, clinical need and global accessibility to best determine the equitable global distribution of a finite number of doses that doesn't favor one child or country over another. AveXis collaborated with an independent bioethics advisory committee to develop the program.

The treating physician submits a request on behalf of their patient. Once medical eligibility is confirmed, Health Authority approval must be obtained in the country where the patient will be treated and has been deemed eligible. A third-party administers a blinded selection on a bi-weekly basis. If a patient is not selected to receive the therapy during that selection round, they automatically roll over to the pool for the next selection as long as they remain medically eligible.

Recognizing that the program will not be a solution for all families in all countries, AveXis encourages patients to work with their health care provider to determine the best treatment approach for this rapidly progressive disease.