MANAGED ACCESS PROGRAM

As a company, we are dedicated to developing innovative therapies for patients suffering from rare and life-threatening neurological genetic diseases. Before these therapies can be approved and commercially available to patients, they must undergo clinical trials to evaluate their safety and effectiveness.

While it is generally preferred that patients gain access to investigational therapies by participating in clinical trials, patients may not be eligible or able to take part in these studies. Patients with serious or life-threatening diseases or conditions sometimes seek therapies that are not yet approved or available in their country. AveXis' "Managed Access Program" addresses this need by making certain investigational or unapproved therapies available to eligible patients.

Clinical Trials

Before an investigational therapy can be placed on the market, it must undergo well-controlled clinical trials to evaluate its safety and effectiveness, as well understand whether its potential benefit to patients outweighs the possible risks. Clinical trial results and related product information are then submitted to the relevant health authorities for review. Clinical trials result in the generation of evidence that may lead to the approval of an investigational therapy, which can make it more widely available to patients.

Managed Access Program

There may be instances where a patient has a serious or life-threatening disease or condition, for which all currently available treatment options have been exhausted and enrollment into a clinical trial is not possible. AveXis' "Managed Access Program" addresses this need by making certain investigational or unapproved treatments available to eligible patients, provided it is allowed by the applicable local laws and regulations.

MANAGED ACCESS PROGRAM

The AveXis “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.

The following criteria* must be met by a patient to participate in an AveXis Managed Access Program:

- The patient to be treated has a serious or life-threatening disease or condition, and no satisfactory alternative therapy is available or the patient 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 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 the investigational 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

**All above criteria are subject to local laws and regulations*

MANAGED ACCESS PROGRAM

How to Submit a Request for Managed Access

Information for Healthcare Professionals

Patients who are not eligible or unable to access ongoing clinical trials may be able to access investigational or unapproved treatments through a Managed Access Program.

Listed below is AveXis' current Managed Access Program:

Zolgensma® (also known as AVXS-101) – Spinal muscular atrophy (SMA) Managed Access Program.

US: Zolgensma® is currently approved and commercially available in the US and, therefore, not eligible for this program.

Outside of the US: The Managed Access Program is available in countries where AVXS-101 is not approved by regulatory authorities in compliance with local laws and regulations. For this Managed Access Program, the physician will need to assess patient eligibility by determining whether the patient meets pre-defined medical and program criteria.

The program 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. Clinical criteria include any patient under the age of two with genetically confirmed SMA, regardless of type, symptom onset or prior treatment. The program is available to medically eligible patients with SMA under the age of two in countries where AVXS-101 is not yet approved by local health authorities. Implementation of the program will need to comply with the specific legal and regulatory framework that applies in every country.

MANAGED ACCESS PROGRAM

How to Submit a Request for Managed Access

Information for Healthcare Professionals

> 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. AVXS-101 will be provided free-of-charge.

The SMA treating physician can submit a request for this Managed Access Program by contacting our third party partner, Durbin (AveXisMAP@DurbinGlobal.com) beginning January 2, 2020.

How do I request Medical Information?

Healthcare professionals may submit requests for medical information directly to AveXis Medical Affairs at medinfo@avexis.com.

MANAGED ACCESS PROGRAM

How to Submit a Request for Managed Access

Information for Patients, Families, and Caregivers

AveXis is dedicated to developing innovative therapies for patients suffering from rare and life-threatening neurological genetic diseases. Our global goal is to allow access to therapies for patients with serious or life-threatening diseases who may not be eligible or able to participate in clinical trials – and their physicians may determine that they have no other appropriate treatment options.

Listed below is AveXis's current Managed Access Program:

Zolgensma® – Spinal muscular atrophy (SMA) Managed Access Program.

***US*:** Zolgensma® is currently approved and commercially available in the US and, therefore, not eligible for this program.

***Outside of the US*:** Zolgensma® is not approved outside of the U.S. AveXis' "Managed Access Program" allows patients access to the treatment in countries where the treatment is not approved or available, in accordance with eligibility criteria and local laws and regulations. For this Managed Access Program, the physician will need to assess patient eligibility by determining whether the patient meets pre-defined medical and program criteria.

The program 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. Clinical criteria include any patient under the age of two with genetically confirmed SMA, regardless of type, symptom onset or prior treatment. The program is available to medically eligible patients with SMA under the age of two in countries where AVXS-101 is not yet approved by local health authorities. Implementation of the program will need to comply with the specific legal and regulatory framework that applies in every country.

MANAGED ACCESS PROGRAM

How to Submit a Request for Managed Access

Information for Patients, Families, and Caregivers

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. AVXS-101 will be provided free-of-charge.

If you are a patient/parent/caregiver and would like to request participation in a Managed Access Program, a request must be submitted by the SMA treating physician on behalf of the patient.

How can I learn more?

Patients/parents/caregivers should contact their treating physician and ask him/her about this Managed Access Program.