

## **AveXis Supports Investigator-Initiated Trials (IIT), Research Collaborations and Consortia**

This research can provide valuable information regarding the safety, efficacy, pharmacology, and tolerability of AveXis' products and supplement the comprehensive data generated in registration studies.

AveXis provides grants exclusively in our therapeutic areas of expertise (i.e. rare and life-threatening neurological genetic diseases) and based on the scientific merit of the investigator's proposal. The research must be intended to contribute knowledge to the medical community with a reasonable and appropriate budget for the proposed work. In considering applications for support, AveXis will also assess the expertise of the proposed principal investigator and any sub-investigators, including their experience in the relevant therapeutic area, demonstrated ability to successfully conduct clinical trials, and available resources.

### **Research Grant Types**

AveXis reviews research proposals seeking financial or material support. Investigators from the United States and all other geographies may use this process.

Investigator-initiated trials are completely managed by a sponsor-investigator, who independently performs all the necessary work on the study, including creating the concept and design, developing the protocol, following necessary regulatory and legal requirements, collecting data, conducting the statistical analysis and publishing the study. AveXis is not responsible for writing, submitting or conducting the study. Sponsor-investigators are required to follow their local regulatory requirements for investigational studies.

Research Collaboration & Consortium activities are undertaken by AveXis with one or more external organizations to advance specific, shared objectives. The nature of AveXis' involvement in the collaboration must be documented and disclosed. AveXis may provide funds, resources, or expertise to the collaboration.

### **Areas of Focus Specific to Spinal Muscular Atrophy (SMA) Research Grants**

The following topic areas are currently in scope for SMA-specific research grants:

- Biomarkers
- Methods/ Processes to reduce time to diagnosis & speed to treatment
- Expansion of treatment access for Zolgensma® (onasemnogene abeparvovec-xioi)
- Demonstrating or Validating Care Needs for SMA populations post-Zolgensma® Treatment
- Value of Zolgensma®: Cost of Care, Quality of Life, and Caregiver Burden

### **Eligibility**

The following organizations are eligible to submit requests for research funding:

- Academic medical centers
- Hospitals
- Medical/professional associations
- Medical schools and other health science schools
- Patient support/advocacy groups
- Universities/colleges
- Tax-exempt organizations (for example, in the U.S., 501(c)(3) entities)

If you are interested in applying for support for research, please review the submission process and apply online. The application and proposal form must be completed in full and is required in order to help us evaluate your funding request.

**SUBMISSION FORM:** <https://www.cybergrants.com/avexis/eligibilityquiz>