EXPANDED ACCESS TO INVESTIGATIONAL THERAPIES

Expanded access, sometimes called “compassionate use” or “named patient program,” refers to the use of an investigational therapy outside a clinical trial when the main purpose is to diagnose, prevent, or treat a serious condition in a patient. This is different from a clinical trial, where more complete safety and efficacy data are collected.

When considering expanded access, it’s important to take into account the guidelines of the US Food and Drug Administration (FDA) and other regulatory agencies. Specific considerations include the following:

- The illness must be serious or life-threatening with no other satisfactory treatments (such as approved products or enrolling clinical trials)
- Enough evidence that the potential benefit to the patient would likely outweigh the potential risks, based on available safety and efficacy information
- Ability to provide a product in a fair and equal manner, so that enough of the product can be produced for ongoing programs
- Whether granting expanded access would affect the scientific validity of broader development programs or interfere with or delay current clinical trials or regulatory filings designed to make the therapy available to many more patients

At this time, AveXis does not offer an expanded access program. Our goal is to provide patients with access to the investigational gene therapies we’re developing as safely and quickly as possible, and currently participation in one of our clinical trials is the most appropriate way to do so. If you have additional questions, please speak with your healthcare provider or contact medinfo@avexis.com. We expect to acknowledge receipt of requests sent to this email within 5 business days.

In line with the 21st Century Cures Act, AveXis may revise this policy at any time.