



December 2025

Dear Alexander Disease Community,

We are writing to inform you that Ionis has opened an [Expanded Access Program \(EAP\) for people with Alexander disease to access zilganersen in the United States](#).¹

An Expanded Access Program is a pathway for a patient with a serious or immediately life-threatening disease or condition to gain access to an investigational medical product for treatment outside of clinical trials when no comparable or satisfactory alternative therapy options are available.²

The zilganersen EAP was opened in accordance with Ionis' EAP policy and general FDA EAP guidance.^{1,2} Zilganersen remains an investigational medical product and has not been approved for marketing as a treatment for any disease indication by any regulatory agency. Ionis announced it plans to submit a New Drug Application to the U.S. Food and Drug Administration (FDA) in the first few months of 2026.³

Sincerely,
The Ionis Zilganersen Team

Additional information you may find helpful:

How does the Expanded Access Program for Zilganersen in the U.S. work?

Only a patient's treating physician can submit a request to Ionis to access zilganersen through the EAP. Requests are reviewed to ensure the patient is eligible for the program. Ionis will then work with the treating physician to complete all necessary steps before zilganersen is distributed directly to the treating physician. Additional information about EAPs and how they work can be found at www.FDA.gov.³

Will Ionis open similar programs in other regions of the world?

Ionis is evaluating high-quality partners to make zilganersen available outside the United States. We will update the community when a partner has been selected.

Where can a person go for more information about Ionis' zilganersen EAP?

A patient's doctor should be their primary source of information regarding managing their disease. This includes discussing access to investigational medical products such as zilganersen through Ionis' EAP.

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For more information about Ionis, please visit www.ionis.com or contact us at padvocacy@ionis.com.

Abbreviations: EAP, expanded access program; FDA, U.S. Food and Drug Administration

References: 1. Ionis Expanded Access Policy. <https://ionis.com/patients-and-advocacy/expanded-access-policy>. Updated December 19, 2025. Accessed December 19, 2025., 2. Expanded Access. <https://www.fda.gov/news-events/public-health-focus/expanded-access>. Updated September 8, 2025. Accessed December 4, 2025., 3. Expanded Access | Information for Patients. <https://www.fda.gov/news-events/expanded-access/expanded-access-information-patients>. Updated November 7, 2022. Accessed December 4, 2025.

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ionis.com

2855 Gazelle Court
Carlsbad, CA 92010

(760) 931-9200