FDA NEWS RELEASE

FDA Approves New Drug to Treat Niemann-Pick Disease, Type C

For Immediate Release:

September 24, 2024

Today, the U.S. Food and Drug Administration approved Aqneursa (levacetylleucine) for the treatment of neurological symptoms associated with Niemann-Pick disease type C (NPC) in adults and pediatric patients weighing at least 15 kilograms.

“This is the second treatment the FDA has approved for NPC within the span of a week. Today’s action further underscores the agency’s commitment to support development of new treatments for rare diseases,” said Janet Maynard, M.D., M.H.S., director of the Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine, in the FDA’s Center for Drug Evaluation and Research. “This approval again demonstrates the FDA’s commitment to work with the scientific community to overcome the unique challenges that may arise with rare disease drug development.”

NPC is a rare genetic disease that results in progressive neurological symptoms and organ dysfunction. It is caused by changes in either the *NPC1* or *NPC2* gene, affecting the necessary transport of cholesterol and other lipids within a cell. As a result, these cells do not function as they should, ultimately causing organ damage. On average, individuals affected by this devastating disease only live for about 13 years.

The safety and efficacy of Aqneursa for the treatment of NPC were evaluated in a randomized, double-blind, placebo-controlled, two-period, 24-week crossover study. The duration was 12 weeks for each treatment period. The study enrolled 60 patients. To be eligible for the study patients had to be 4 years of age or older with a confirmed diagnosis of NPC and at least mild disease-related neurological symptoms. Participants could receive miglustat, an enzyme inhibitor, as background treatment in the study.

The primary efficacy outcome was a modified version of the Scale for the Assessment and Rating of Ataxia (SARA), referred to as the functional SARA (fSARA). The fSARA consists of the gait, sitting, stance and speech disturbance domains of the original SARA with modifications to

the scoring responses. On average, participants treated with Aqneursa for 12 weeks showed a better outcome in the fSARA score compared to when they were treated with placebo.

The prescribing information contains a warning that Aqneursa may cause embryo-fetal harm if used during pregnancy. Females should inform their healthcare provider of a known or suspected pregnancy before taking Aqneursa.

The most common side effects are abdominal pain, difficulty swallowing, upper respiratory tract infections and vomiting.

Aqneursa should be taken orally up to three times per day, with or without food. The recommended dose varies depending on the individual's body weight, as outlined in the prescribing information.

The FDA granted Aqneursa Priority Review (https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/priority-review), Fast Track (https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/fast-track), Orphan Drug (https://www.fda.gov/industry/medical-products-rare-diseases-and-conditions/designating-orphan-product-drugs-and-biological-products) and Rare Pediatric Disease (https://www.fda.gov/industry/medical-products-rare-diseases-and-conditions/rare-pediatric-disease-designation-and-priority-review-voucher-programs) designations for this application.

The FDA granted approval of Aqneursa to IntraBio Inc.

Related Information

- Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine (ORPURM) (https://www.fda.gov/about-fda/cder-offices-and-divisions/office-rare-diseases-pediatrics-urologic-and-reproductive-medicine-orpurm)

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