FDA NEWS RELEASE

FDA Approves First Treatment for Niemann-Pick Disease, Type C

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Today, the U.S. Food and Drug Administration approved Miplyffa (arimoclomol) (https://www.accessdata.fda.gov/drugsatfda_docs/label/2024/214927s000lbl.pdf), an oral medication for the treatment of Niemann-Pick disease, type C (NPC). Miplyffa, in combination with the enzyme inhibitor miglustat, is approved to treat neurological symptoms associated with NPC in adults and children 2 years of age and older. Miplyffa is the first drug approved by the FDA to treat NPC.

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.

“NPC is a serious disease that leads to enormous adverse impacts on patients and families. Despite extensive research efforts, there have not been approved treatments to meet the significant needs of patients,” said Janet Maynard, M.D., M.H.S., director of the Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine (ORPURM), in the FDA’s Center for Drug Evaluation and Research. “The first-ever approval of a safe and effective drug option for NPC will undoubtedly support the essential medical needs of those suffering.”

Miplyffa was the first product application to be discussed (https://www.fda.gov/advisory-committees/advisory-committee-calendar/updated-public-participation-information-august-2-2024-meeting-genetic-metabolic-diseases-advisory) at the inaugural meeting of the Genetic Metabolic Diseases Advisory Committee (https://www.fda.gov/advisory-committees/human-drug-advisory-committees/genetic-metabolic-diseases-advisory-committee) (GeMDAC) in August. GeMDAC was established in December 2023 to advise the agency on products used for the diagnosis, prevention or treatment of genetic metabolic diseases.

The safety and effectiveness of Miplyffa were evaluated in a randomized, double-blind, placebo-controlled 12-month trial in patients two to 19 years of age who had a molecularly confirmed diagnosis of NPC. Fifty patients were randomized 2:1 to treatment with weight-adjusted Miplyffa (31 to 124 mg) or placebo orally three times per day. Among these 50 patients, 39 (78%) received miglustat as background treatment in the trial.

Miplyffa's efficacy was demonstrated by the rescored 4-domain NPC Clinical Severity Scale (R4DNPCCSS) score in the patients who used miglustat as their background treatment. The R4DNPCCSS is a measure of NPC disease progression that looks at four items that patients with NPC, their caregivers and physicians have identified as most relevant including ambulation, speech, swallow and fine motor skills. Higher scores signify a greater severity of the disease. Compared to placebo, Miplyffa resulted in a slower disease progression as measured by the R4DNPCCSS score.

The prescribing information for Miplyffa contains a warning for hypersensitivity reactions including hives and angioedema (swelling under the skin). Individuals experiencing these adverse reactions should stop using the drug. Females who are pregnant or plan to become pregnant should not use Miplyffa.

The most common side effects of Miplyffa include upper respiratory tract infection, diarrhea and decreased weight.

Miplyffa, along with miglustat, should be taken orally with or without food according to the recommended dose for the patient's body weight.

The FDA granted Miplyffa priority review (https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/priority-review), orphan drug (https://www.fda.gov/industry/medical-products-rare-diseases-and-conditions/designating-orphan-product-drugs-and-biological-products), rare pediatric disease (https://www.fda.gov/industry/medical-products-rare-diseases-and-conditions/rare-pediatric-disease-designation-and-priority-review-voucher-programs), fast track (https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/fast-track)and breakthrough therapy (https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/breakthrough-therapy) designations for this application.

The FDA granted approval of Miplyffa to Zevra Therapeutics.

Related Information

- August 2, 2024 Meeting of the Genetic Metabolic Diseases Advisory Committee (https://www.fda.gov/advisory-committees/advisory-committee-calendar/updated-public-participation-information-august-2-2024-meeting-genetic-metabolic-diseases-advisory)
- 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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Inquiries

Media:

April Grant (mailto:april.grant@fda.hhs.gov)

202-657-8179

Consumer:

888-INFO-FDA

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