September 20, 2024

Dear NPC Community,

We are excited to share with you a milestone we have all been eagerly awaiting – today, the U.S. Food and Drug Administration (FDA) has approved MIPLYFFA™ (arimoclomol) capsules, for oral use, for use in combination with miglustat for the treatment of neurological manifestations of Niemann-Pick disease type C (NPC) in adult and pediatric patients 2 years of age and older. You can read the full news release here Zevra Therapeutics' MIPLYFFA™ (arimoclomol) Receives U.S. FDA Approval as Treatment for Niemann-Pick Disease Type C.

We want to express our deepest gratitude and appreciation to the patients, families, researchers and clinicians in the US and Europe who have participated in the arimoclomol clinical studies and the expanded access program. We are sincerely grateful for the unwavering support of both Zevra and the global NPC community. Your dedication and partnership throughout our journey have been vital in achieving this groundbreaking approval to bring a much-needed treatment option to those affected by NPC.

We are incredibly proud to be a part of the ongoing efforts to positively impact the lives of those living with rare diseases such as NPC patients. As such, the US Expanded Access Program will remain open for the time being for currently enrolled patients. Families should begin working with their healthcare provider to gain access to commercially available MIPLYFFA.

In addition, Zevra is proud to introduce AmplifyAssist. Through this program, Zevra will offer assistance with prescription and insurance navigation, as well as clinical support while receiving care.

Families are encouraged to speak with their treatment team as their healthcare provider will need to complete the AmplifyAssist Enrollment Form to initiate the process to access treatment. You may find more information at MIPLYFFA.com

If you have any questions or need further information, please don't hesitate to reach out to us at patientadvocacy@zevra.com.

Kind regards,

The Zevra Team

WHAT is MIPLYFFA [mye-plye'-fah]?
MIPLYFFA is prescription medicine used in combination with a drug called miglustat to treat neurological symptoms of Niemann-Pick disease type C (NPC) in patients 2 years of age and older.

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IMPORTANT SAFETY INFORMATION

Before starting MIPLYFFA, tell your healthcare provider about all your medical conditions, including if you are pregnant or plan to become pregnant, breastfeeding or plan to breastfeed.

Tell your healthcare provider about all the medicines you take, including any prescription and over-the-counter medicines, vitamins, or herbal supplements. MIPLYFFA may affect how other medicines work.

What are the possible side effects of MIPLYFFA?

MIPLYFFA may cause serious side effects including:

- **Hypersensitivity reactions**. Call your healthcare provider immediately if you get any of the following symptoms:
  - urticaria (hives),
  - shortness of breath,
  - persistent cough, or
  - facial swelling
- **Harm to your unborn baby.** If you are of childbearing age, take precautions to prevent pregnancy. Tell your healthcare provider right away if you become pregnant or think you might be pregnant during treatment with MIPLYFFA.
- **Infertility.** MIPLYFFA may affect your ability to have children.

The most common side effects of MIPLYFFA in patients also taking miglustat include upper respiratory tract infection, diarrhea and decreased weight.

These are not all the possible side effects of MIPLYFFA. Call your HCP for medical advice about side effects. **You are encouraged to report side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.**

Drug Interactions: MIPLYFFA can cause side effects if used together with certain drugs called OCT2 substrates. Talk to your healthcare provider about any drugs that you may be taking for other conditions.

MIPLYFFA capsules for oral use are available in the following strengths in a 90-count bottle: 47 mg, 62 mg, 93 mg, and 124 mg.

For more information, please see the full *Prescribing* Information*, including Instructions for Use.*

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