November 22, 2024

Dear NPC Community,

We are excited to share with you another important milestone we have all been eagerly awaiting – MIPLYFFA™ (MY-PLY-FAH) (arimoclomol) **is NOW AVAILABLE for dispensing in the United States**. Please read the full news press release here **Zevra Therapeutics' Announces U.S. Commercial Availability of MIPLYFFA™ (arimoclomol) for Treatment of Niemann-Pick Disease Type C.**

On September 20, 2024, U.S. Food and Drug Administration (FDA) approved MIPLYFFA™ (arimoclomol) capsules, an orally delivered treatment, 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. We also introduced Zevra's comprehensive support program, AmplifyAssist™ for caregivers and those living with NPC.

**How Do I Learn More About MIPLYFFA?**
Please visit MIPLYFFA.com and download the MIPLYFFA Caregiver Fact Sheet that provides important information for caregivers and those living with NPC.

**Where Can My Doctor Learn More About MIPLYFFA?**
Please have your doctor and medical team visit MIPLYFFA.com where they can download the MIPLYFFA Fact Sheet – A Guide for healthcare providers, the MIPLYFFA Prescription Enrollment form, and learn more about the support offered by AmplifyAssist.
*Your doctor may also request to meet with a Zevra Representative at MIPLYFFA.com to learn more.*

**How Do I Get Started on MIPLYFFA?**
Please first talk with your doctor to discuss the risks and benefits of MIPLYFFA and see if it is appropriate for you or your loved one. Second, if appropriate, your doctor should complete and submit the MIPLYFFA Prescription Enrollment form to AmplifyAssist by faxing it to 1-888-668-2143.

Once received, AmplifyAssist will assist you/your healthcare provider's office with verifying your insurance coverage and submitting any requests for coverage that may be necessary. Once approved, AmplifyAssist will work with you to address any affordability barriers and schedule your delivery of MIPLYFFA to your home or desired location.

*Please note that a signature is required upon delivery – so it is important to speak with AmplifyAssist to ensure it is being delivered to you directly and that you will be home to sign and receive it.*

**What Are My Next Steps If My Doctor Has Already Submitted a MIPLYFFA Prescription?**
If your insurance company has approved MIPLYFFA, AmplifyAssist will be scheduling your initial delivery of MIPLYFFA as quickly as possible.

For those that are still waiting for insurance approval, AmplifyAssist will continue to work with you and your doctor on resolving any insurance coverage issues. During this time, you may be eligible for MIPLYFFA through the Quick Start program and AmplifyAssist will call to schedule delivery.
If you have questions about the status of your prescription or next steps, please contact AmplifyAssist directly to discuss at 1-(888) 668-4198.

*Please note that a signature is required upon delivery – so it is important to speak with AmplifyAssist to ensure it is being delivered to you directly and that you will be home to sign and receive it.*

**How Many People Have Been Prescribed MIPLYFFA since Approval?**
As of October 31, Zevra has received 90 MIPLYFFA Prescription Enrollment Forms, of which 30% are approved for reimbursement which means they are ready for shipment once AmplifyAssist is able to schedule the delivery with the patient and/or caregiver.

**How do I Reach AmplifyAssist?**
The AmplifyAssist team can be reached toll-free at (888) 668-4198 from 8 a.m. CT to 6 p.m. CT, Monday through Friday. If you are unable to speak with someone live, please leave a message with AmplifyAssist and a team member will call you back within 24 hours, if not sooner.

We remain very grateful to the entire NPC community who are at the center of all that we do here at Zevra, and the medical community who care for them.

Please review the **Important Safety Information** below and discuss it with your loved one's doctor if you have any questions or concerns.

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.

**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.