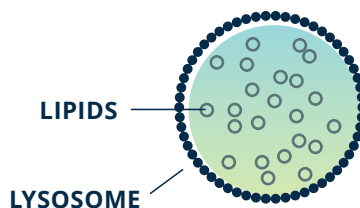


**MIPLYFFA is the first FDA-approved treatment
for Niemann-Pick disease type C (NPC)**

MIPLYFFA is a 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.

About Niemann-Pick disease type C

- NPC is an **ultra-rare genetic neurodegenerative disease** that affects an estimated 1 in 100,000 live births
- **Individuals who have NPC are born with it**, and diagnosis can be confirmed at any age through a genetic test



◀ Gene mutations lead to a buildup of lipids (fats) in cells of the body.

Recognizing symptom patterns in NPC

Identifying symptoms is important for a timely and accurate diagnosis of NPC. These symptoms can vary from person to person in how they appear and how severe they are, and can change as the person gets older.

NPC generally affects movement, posture, cognition, and memory.

Some combinations of symptoms that could indicate NPC might include:



A history of jaundice at birth and slow or slurred speech



Learning difficulties and a seizure



Unsteady walking and abnormal posture

Symptoms can manifest as developmental milestone delays, cognitive impairment, problems with coordination, seizures, and in older patients psychiatric illness.

SELECT 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.

Please see additional Important Safety Information on the following pages and full Prescribing Information, including Instructions for Use.

The R4DNPCSS is an established scale that helps measure the progression and severity of NPC

4 key areas have been identified as some of the most important by NPC expert clinicians, caregivers, and patients, and allow for an assessment of NPC symptom progression.



AMBULATION



FINE MOTOR
SKILLS



SWALLOW



SPEECH

Doctors will assess symptoms by focusing on areas impacted by NPC, within a validated scale or measuring system. **The scale includes scores for how serious symptoms are, ranging from normal to highly impacted, and can be used to measure change over time.**

The rescored 4-domain NPC Clinical Severity Scale (R4DNPCSS) also provides a way to evaluate the impact of treatment and see how you or your loved one may be responding to treatment.

Safety and effectiveness of MIPLYFFA in combination with miglustat were assessed over a period of 12 months

Safety and effectiveness of MIPLYFFA were studied in a 12-month, multicenter, randomized, double-blind, placebo-controlled trial in patients with NPC aged 2 to 19 years.

50 people, aged 2 to 19 years, with a confirmed NPC diagnosis

34 trial participants were given
MIPLYFFA + background treatment*

17 trial participants were given
placebo + background treatment*

*Throughout the trial, patients stayed on their ongoing prescribed background treatment, including miglustat and symptom management. Patients were randomized at enrollment by miglustat treatment, which is considered routine clinical care in some countries. In Trial 1, 76% of patients in the MIPLYFFA group and 81% of those in the placebo group received miglustat as part of their routine care.

SELECT IMPORTANT SAFETY INFORMATION

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

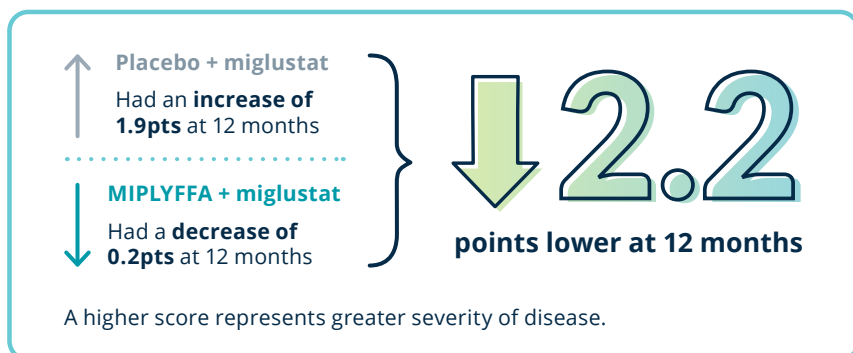
These are not all the possible side effects of MIPLYFFA. Call your HCP for medical advice about side effects. You are encouraged to report side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

Please see additional Important Safety Information on the following pages and full Prescribing Information, including Instructions for Use.

 **MIPLYFFA**[®]
arimoclomol capsules

Results from the MIPLYFFA study

In the clinical trial, **MIPLYFFA, in combination with miglustat, stopped disease progression at month 12** when compared to placebo treatment, as measured by the R4DNPCSS score. The R4DNPCSS measures ambulation, speech, swallow, and fine motor skills—the areas identified as some of the most important to measure by NPC expert clinicians, caregivers, and patients.



MIPLYFFA, in combination with miglustat, stopped disease progression at 12 months

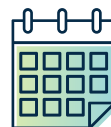
The mean baseline R4DNPCSS score was higher in the MIPLYFFA group (n=26; mean=8.9) than the placebo group (n=13; mean=7), with an overall mean R4DNPCSS score of 8.3.

There were insufficient data to determine the effectiveness of the use of MIPLYFFA without miglustat for the treatment of neurological manifestations in patients with NPC.

MIPLYFFA has been studied longer than any other FDA-approved therapy for NPC

Long-term use of MIPLYFFA has been studied for up to 5 years.

Patients in long-term studies continued to experience a slowed rate of disease progression.



◀ The rate of disease progression was low in the long-term extension of the original 12 month study.

Side effects

The most common side effects in Trial 1 (≥15%) in patients treated with MIPLYFFA, and who also received miglustat were upper respiratory tract infection, diarrhea, and decreased weight. Rate of side effects in the long-term extension was similar to what was seen in the original study. MIPLYFFA was well-tolerated over the long-term.



Talk to your doctor about more clinical results.

SELECT IMPORTANT SAFETY INFORMATION

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.

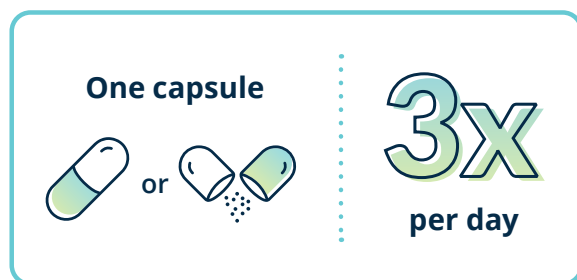
MIPLYFFA capsules for oral use are available in the following strengths in a 90-count bottle: 47 mg, 62 mg, 93 mg, and 124 mg.

Please see additional Important Safety Information on the following pages and full Prescribing Information, including Instructions for Use.



MIPLYFFA is an oral medication that can be taken at home

- MIPLYFFA capsules are taken **3x per day**, either by swallowing the whole capsule, or by adding the contents to a small amount of water, apple juice, or soft foods, or via a feeding tube
- MIPLYFFA can be taken **with or without food**
- Dosing for MIPLYFFA is **based on body weight** and should be determined by your doctor
- If not taking whole, be sure to take the medication immediately after preparation



For more details about dosing and administration, please talk to your doctor or refer to the [full Prescribing Information, including Instructions for Use](#).

Zevra is proud to introduce AmplifyAssist™, a support program for caregivers and those living with NPC and taking MIPLYFFA*



To learn more, visit zevraamplifyassist.com or call **800-650-CARE (2273)** Monday – Friday, 8 AM to 8 PM ET.

AmplifyAssist can help with:



Clinical education

Product and disease state information, including tips and tools for therapy management.

The AmplifyAssist team does not replace discussions with your healthcare provider(s).



Prescription fulfillment

Ongoing coordination of your prescription needs and deliveries.



Insurance coverage support

Help with understanding your insurance coverage, your options, and the steps to acquiring coverage for specialty medications.



Access support

Assistance with identifying and enrolling eligible patients in financial support programs/services to help manage the cost of treatment.

*Eligibility requirements apply.



To get started with MIPLYFFA, talk with your doctor.

Your MIPLYFFA prescription is ordered through a specialty pharmacy. To get started, your doctor will fill out an enrollment form at MIPLYFFA.com, order the prescription, and when approved, the medication will be mailed to your home.

SELECT IMPORTANT SAFETY INFORMATION

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

Please see additional Important Safety Information on the following pages and [full Prescribing Information, including Instructions for Use](#).



To learn more, visit MIPLYFFA.com

MIPLYFFA: Because every moment matters



MIPLYFFA was **well-studied and demonstrated effectiveness** across 4 of the most important domains*

*As identified by NPC expert clinicians, patients, and caregivers.



MIPLYFFA has **convenient dosing and flexible administration** options



AmplifyAssist™ may be able to help you **access and pay for treatment**



**Scan the QR code
for more information
about MIPLYFFA**

APPROVED USE AND IMPORTANT SAFETY INFORMATION

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),
 - persistent cough, or
 - shortness of breath,
 - 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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