NEW MIPLYFFA™ arimoclomol capsules

Caregiver Fact Sheet

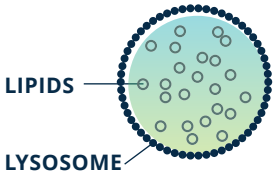
For caregivers and those living with NPC

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

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.

About Niemann-Pick disease type C

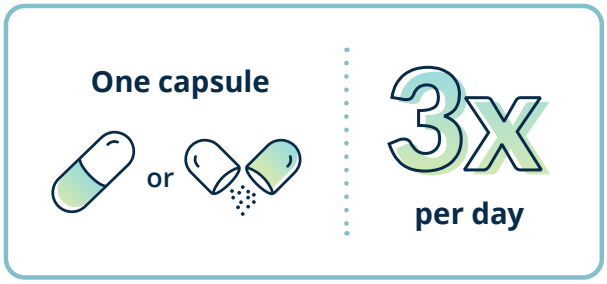
- NPC is an ultra-rare genetic progressive disease that affects an estimated 1 in 100,000 people
- NPC is caused by mutations in either the *NPC1* or *NPC2* gene resulting in a deficiency of enzymes that instruct proteins to transport lipids in the body. A mutation in either gene can result in a buildup of lipids in cells of the body causing the lysosomes to not work properly
- NPC can be diagnosed at any age, and can be confirmed through a genetic test



Disease-causing mutations in NPC result in accumulation of lipids (fats) in the lysosome

MIPLYFFA is an oral medication that can be taken at home.

- MIPLYFFA capsules are taken 3x per day, either by swallowing the whole capsule, 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 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.

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.

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

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

How MIPLYFFA was studied

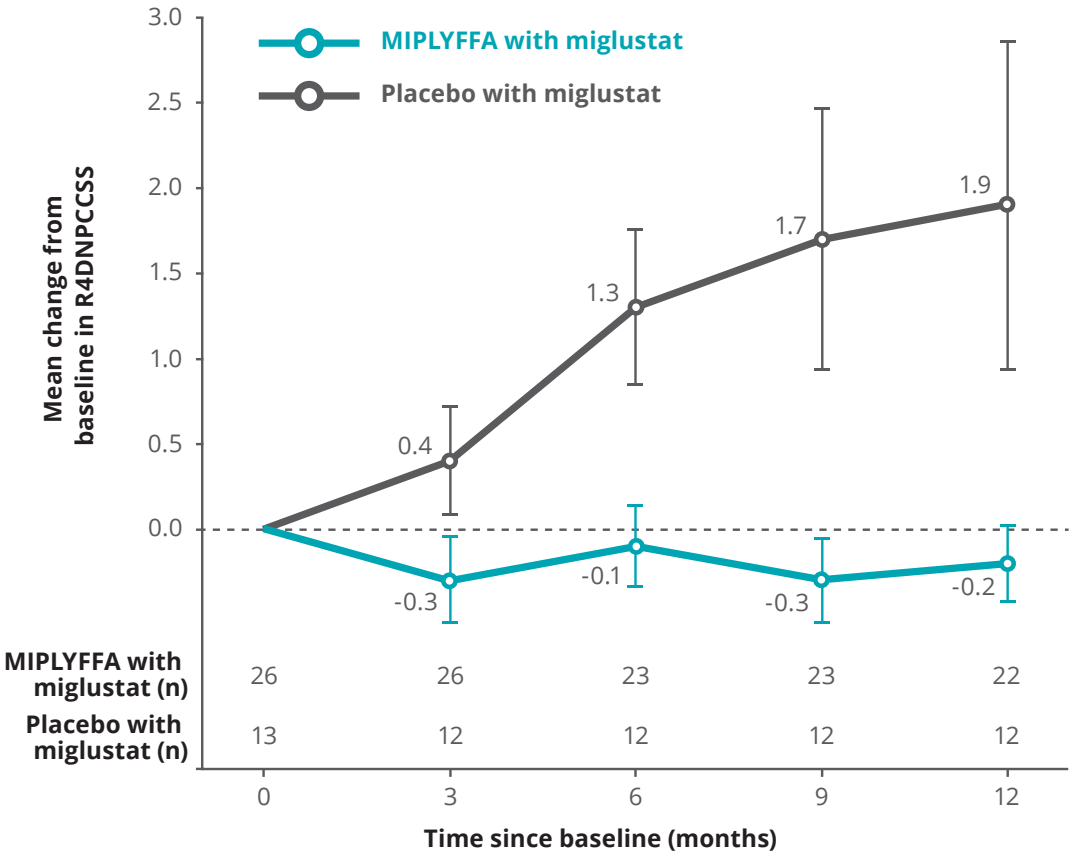
Safety and effectiveness of MIPLYFFA were studied in a 12-month multi-center, randomized, double-blind, placebo-controlled trial in patients with NPC aged 2 to 19 years.

50 people, aged 2 to 19 years, with a confirmed NPC diagnosis	
34 trial participants were given **MIPLYFFA** + background treatment*	**16** trial participants were given **placebo** + background treatment*

*Throughout the trial, patients stayed on their ongoing prescribed background treatment, including miglustat and symptom management. Randomization was stratified 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.

Results from the MIPLYFFA study

NPC SYMPTOM REDUCTION AT 12 MONTHS
as measured by the R4DNPCCSS score:
ambulation, speech, swallow, and fine motor skills



The estimated mean change from baseline at month 12 was -2.2 pts. vs miglustat alone

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.

Side Effects

The most common adverse reactions in Trial 1 (≥15%) in MIPLYFFA-treated patients who also received miglustat were upper respiratory tract infection, diarrhea, and decreased weight.

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.

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To learn more, visit **MIPLYFFA.com**

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

*Eligibility requirements apply

AmplifyAssist™
Your ally in what's ahead.

1-888-668-4198
Monday – Friday 8 am to 6 pm CT

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 when approved, the medication will be mailed to your home.

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

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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.**

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ZEVRA THERAPEUTICS

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