

What is Miplyffa?

Miplyffa is a prescription medicine used in combination with miglustat to treat neurological symptoms caused by Niemann-Pick Disease Type C (NPC) in patients 2 years and older. It helps slow down disease progression. The non-proprietary (generic) name is arimoclomol. Miplyffa is manufactured by Zevra Therapeutics.

How It Works

Miplyffa is thought to improve lysosomal functioning by influencing the function of certain genes known as CLEAR genes.

Why It Matters

In clinical studies, Miplyffa slowed neurological decline over 12 months when used with miglustat. Many patients also showed continued benefit at 5 years.

How to Take Miplyffa

Form: Oral capsules

Route: By mouth or through a feeding tube (e.g., G-tube). Capsule can be opened and sprinkled in water, apple juice, or soft foods.

Food: Can be taken with or without food

Dosing Schedule

Your dose of Miplyffa depends on your body weight. Take capsules three times a day, about every 8 hours, in addition to how your physician has instructed you to take miglustat:

Weight Range	Capsule Strength	Frequency
8–15 kg	47 mg	3× per day
15–30 kg	62 mg	3× per day
30–55 kg	93 mg	3× per day
55 kg (incl. adults)	124 mg	3× per day

What If You Miss a Dose?

- Take the next dose at the scheduled time
- Do not double-dose to make up for a missed one

Common Side Effects

- Upper respiratory infections ("colds") • Diarrhea • Decreased weight • Other possible: tremor, urticaria (hives), headache, seizures

Important Safety Information

- **Hypersensitivity reactions** (such as hives or swelling) - stop taking and call your doctor right away
- **Can cause fetal harm** - do not take if pregnant or planning to become pregnant
- **May increase creatinine** (a kidney-related blood test) without harming kidney function
- **Drug interactions:** MIPLYFFA may affect medications processed by kidney transporters. Your doctor may need to check or adjust other treatments

Who Should NOT Take It?

- Anyone with a known allergy to arimoclomol or capsule ingredients
- Pregnant women or those planning to become pregnant
- Always tell your doctor about all health conditions and medicines - especially kidney issues or pregnancy

Storage & Safety Tips

- Store at room temperature
- Keep out of reach of children
- Follow your doctor's schedule
- Keep all follow-up appointments for monitoring

Reporting Side Effects

In addition to reporting any side effects to your healthcare providers, you are encouraged to report side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088.

Questions?

Always talk to your doctor or pharmacist if you have questions about your medicine.

If you ever have questions about Miplyffa - how to take it, side effects, or anything else - please call your doctor or pharmacist.

For more information see miplyffa.com