



---

PATIENT & CAREGIVER EDUCATION

# Arimoclomol

This information from UpToDate® Lexidrug™ explains what you need to know about this medication, including what it's used for, how to take it, its side effects, and when to call your healthcare provider.

## Brand Names: US

Miplyffa

## What is this drug used for?

- It is used with another drug (miglustat) to treat nervous system problems in people with Niemann-Pick disease type C (NPC).

## What do I need to tell my doctor BEFORE I take this drug?

- If you are allergic to this drug; any part of this drug; or any other drugs, foods, or substances. Tell your doctor about the allergy and what signs you had.

This drug may interact with other drugs or health problems.

Tell your doctor and pharmacist about all of your drugs (prescription or OTC, natural products, vitamins) and health problems. You must check to make sure that it is safe for you to take this drug with all of your drugs and health problems. Do not start, stop, or change the dose of any drug without checking with your doctor.

## What are some things I need to know or do while I take this drug?

- Tell all of your health care providers that you take this drug. This includes your doctors, nurses, pharmacists, and dentists.
- Have your blood work and other lab tests checked as you have been told by your doctor.
- This drug may affect certain lab tests. Tell all of your health care providers and lab workers that you take this drug.
- This drug may affect fertility. This may lead to not being able to get pregnant or father a child. If this happens, it is not known if fertility will go back to normal.
- This drug may cause harm to an unborn baby. If you may become pregnant, talk with your doctor about using birth control while you take this drug. If you get pregnant, call your doctor right away.
- Tell your doctor if you are breast-feeding. You will need to talk about any risks to your baby.

## What are some side effects that I need to call my doctor about right away?

**WARNING/CAUTION:** Even though it may be rare, some people may have very bad and sometimes deadly side effects when taking a drug. Tell your doctor or get medical help right away if you have any of the following signs or symptoms that may be related to a very bad side effect:

- Signs of an allergic reaction, like rash; hives; itching; red, swollen, blistered, or peeling skin with or without fever; wheezing; tightness in the chest or throat; trouble breathing, swallowing, or talking; unusual hoarseness; or swelling of the mouth, face, lips, tongue, or throat.
- Seizures.
- Cough that does not go away.

## What are some other side effects of this drug?

All drugs may cause side effects. However, many people have no side effects or only have minor side effects. Call your doctor or get medical help if any of these side effects or any other side effects bother you or do not go away:

- Signs of a common cold.
- Weight loss.
- Decreased appetite.
- Shakiness.
- Itching.
- Headache.

These are not all of the side effects that may occur. If you have questions about side effects, call your doctor. Call your doctor for medical advice about side effects.

You may report side effects to your national health agency.

You may report side effects to the FDA at 1-800-332-1088. You may also report side effects at <https://www.fda.gov/medwatch>.

## How is this drug best taken?

Use this drug as ordered by your doctor. Read all information given to you. Follow all instructions closely.

- Take with or without food.
- Swallow whole. Do not chew or crush.
- If you have trouble swallowing this drug, you can open the capsule and sprinkle the contents in 15 mL of water or apple juice. You can also sprinkle the contents on 15 mL of applesauce, pudding, or yogurt. Stir for 15 seconds and swallow the mixture right away. Do not store for future use.
- Those who have feeding tubes may use this drug. Use as you have been told. Flush the feeding tube after this drug is given.

## What do I do if I miss a dose?

- Skip the missed dose and go back to your normal time.
- Do not take 2 doses at the same time or extra doses.

## How do I store and/or throw out this drug?

- Store at room temperature protected from light. Store in a dry place. Do not store in a bathroom.
- Store in the original container. Do not take out the antimoisture cube or packet.
- Keep all drugs in a safe place. Keep all drugs out of the reach of children and pets.
- Throw away unused or expired drugs. Do not flush down a toilet or pour down a drain unless you are told to do so. Check with your pharmacist if you have questions about the best way to throw out drugs. There may be drug take-back programs in your area.

## General drug facts

- If your symptoms or health problems do not get better or if they become worse, call your doctor.
- Do not share your drugs with others and do not take anyone else's drugs.
- Some drugs may have another patient information leaflet. If you have any questions about this drug, please talk with your doctor, nurse, pharmacist, or other health care provider.
- Some drugs may have another patient information leaflet. Check with your pharmacist. If you have any questions about this drug, please talk with your doctor, nurse, pharmacist, or other health care provider.
- If you think there has been an overdose, call your poison control center or get medical care right away. Be ready to tell or show what was taken, how much, and when it happened.

# Consumer Information Use and Disclaimer

This generalized information is a limited summary of diagnosis, treatment, and/or medication information. It is not meant to be comprehensive and should be used as a tool to help the user understand and/or assess potential diagnostic and treatment options. It does NOT include all information about conditions, treatments, medications, side effects, or risks that may apply to a specific patient. It is not intended to be medical advice or a substitute for the medical advice, diagnosis, or treatment of a health care provider based on the health care provider's examination and assessment of a patient's specific and unique circumstances. Patients must speak with a health care provider for complete information about their health, medical questions, and treatment options, including any risks or benefits regarding use of medications. This information does not endorse any treatments or medications as safe, effective, or approved for treating a specific patient. UpToDate, Inc. and its affiliates disclaim any warranty or liability relating to this information or the use thereof. The use of this information is governed by the Terms of Use, available at <https://www.wolterskluwer.com/en/know/clinical-effectiveness-terms>.

## Last Reviewed Date

2024-09-26

## Copyright

© 2026 UpToDate, Inc. and its affiliates and/or licensors. All rights reserved.

If you have questions or concerns, contact your healthcare provider. A member of your care team will answer Monday through Friday from 9 a.m. to 5 p.m. Outside those hours, you can leave a message or talk with another MSK provider. There is always a doctor or nurse on call. If you're not sure how to reach your healthcare provider, call 212-639-2000.

For more resources, visit [www.mskcc.org/pe](http://www.mskcc.org/pe) to search our virtual library.

Arimoclomol - Last updated on September 29, 2024

All rights owned and reserved by Memorial Sloan Kettering Cancer Center

**Last Updated**

**September 29, 2024**

Learn about our [Health Information Policy](#).