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 the doctor BEFORE my child takes this drug?

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

This drug may interact with other drugs or health problems.

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

What are some things I need to know or do while my child takes this drug?

- Tell all of your child's health care providers that your child is taking this drug. This includes your child's doctors, nurses, pharmacists, and dentists.
- Have your child's blood work and other lab tests checked as you have been told by the doctor.
- This drug may affect certain lab tests. Tell all of your child's health care providers and lab workers that your child takes 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.

If your child is or may be sexually active:

- This drug may cause harm to an unborn baby. If your child may become pregnant, talk with the doctor about having your child use birth control while taking this drug. If your child gets pregnant, call the doctor right away.

If your child is breast-feeding a baby:

- Tell the doctor if your child is breast-feeding a baby. You will need to talk about any risks to the baby.

What are some side effects that I need to call my child's 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 child's doctor or get medical help right away if your child has 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 child's doctor or get medical help if any of these side effects or any other side effects bother your child 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 child's doctor. Call your child's doctor for medical advice about side effects.

You may report side effects to your national health agency.

How is this drug best given?

Give this drug as ordered by your child's doctor. Read all information given to you. Follow all instructions closely.

- Give this drug with or without food.
- Have your child swallow whole. Do not let your child chew or crush.
- If your child has 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 have your child 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 my child misses a dose?

- Skip the missed dose and go back to your child's normal time.
- Do not give 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 original container.
- 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 child's symptoms or health problems do not get better or if they become worse, call your child's doctor.
- Do not share your child's drug with others and do not give anyone else's drug to your child.
- Some drugs may have another patient information leaflet. If you have any questions about this drug, please talk with your child's 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.

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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.

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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 to search our virtual library.

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