Memorial Sloan Kettering Cancer Center

PATIENT & CAREGIVER EDUCATION

Mitapivat

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

Aqvesme; Pyrukynd; Pyrukynd Taper Pack

Warning

Aqvesme:

- Severe liver problems have happened with this drug. You will need to have liver tests before your first dose, every 4 weeks for 6 months, then as needed. Tell your doctor right away if you have any signs of liver problems like dark urine, tiredness, decreased appetite, upset stomach or stomach pain, light-colored stools, throwing up, or yellow skin or eyes.

What is this drug used for?

- It is used to treat hemolytic anemia in certain people.
- It is used to treat anemia in people with alpha- or beta-thalassemia.

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.

- If you have liver disease.
- If you take any drugs (prescription or OTC, natural products, vitamins) that must not be taken with this drug, like certain drugs that are used for HIV, infections, seizures, and others. There are many drugs that must not be taken with this drug.

This is not a list of all drugs or health problems that interact with this drug.

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?

All products:

- 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.
- Avoid grapefruit and grapefruit juice.
- Decreases in the reproductive hormones estrone and estradiol have happened in men taking this drug. If you have questions, talk with your doctor.
- Some hormone-based birth control may not work as well to prevent pregnancy. You may need to switch to a different hormone-based birth control or use a non-hormone form of birth control (like condoms) while taking this drug. You may also need to make these birth control changes for 28 days after your last dose. If you take hormone-based birth control, talk with your doctor.
- Tell your doctor if you are pregnant, plan on getting pregnant, or are breast-feeding. You will need to talk about the benefits and risks to you and the

baby.

Pyrukynd:

- Do not stop taking this drug all of a sudden. You may have a greater risk of red blood cell breakdown. If you need to stop this drug, slowly stop it as ordered by your doctor. Tell your doctor right away if you have yellow skin or eyes, dark urine, dizziness, confusion, feeling tired, or shortness of breath.
- High triglyceride levels have happened with this drug. Tell your doctor if you have ever had high triglyceride levels.

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:

All products:

- 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.
- Fast or abnormal heartbeat.

Pyrukynd:

- Signs of high blood pressure like very bad headache or dizziness, passing out, or change in eyesight.
- Breast pain.
- A burning, numbness, or tingling feeling that is not normal.
- People taking a higher dose of this drug for a different health problem had a raised chance of liver problems during the first 6 months of treatment. Tell

your doctor right away if you have any signs of liver problems like dark urine, tiredness, decreased appetite, upset stomach or stomach pain, light-colored stools, throwing up, or yellow skin or eyes.

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:

Pyrukynd:

- Back pain.
- Joint pain.
- Stomach pain or diarrhea.
- Flushing.
- Mouth or throat pain or irritation.
- Constipation.
- Dry mouth.
- Dry lips.

Aqvesme:

- Headache.
- Trouble sleeping.

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, break, crush, or dissolve.
- Keep taking this drug as you have been told by your doctor or other health care provider, even if you feel well.

What do I do if I miss a dose?

- Take a missed dose as soon as you think about it.
- If you miss a dose by more than 4 hours, 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 in a dry place. Do not store in a bathroom.
- Store the blister wallets in the original carton until you are ready to use.
- 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.

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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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