

Patient guide to AYVAKIT for advanced systemic mastocytosis

INDICATION

AYVAKIT[®] (avapritinib) is a prescription medicine used to treat adults with advanced systemic mastocytosis (AdvSM), including aggressive systemic mastocytosis (ASM), systemic mastocytosis with an associated hematological neoplasm (SM-AHN), and mast cell leukemia (MCL). AYVAKIT is not recommended for the treatment of AdvSM in people with low platelet counts (less than $50 \times 10^9/L$). It is not known if AYVAKIT is safe and effective in children.

IMPORTANT SAFETY INFORMATION

AYVAKIT may cause serious side effects, including:

Bleeding in your brain. Serious bleeding in the brain may happen during treatment with AYVAKIT and may lead to death. Stop taking AYVAKIT and tell your healthcare provider right away if you develop any symptoms such as severe headache, nausea, vomiting, vision changes, drowsiness, dizziness, confusion, or severe weakness on one or more side of your body. Your healthcare provider will check your platelet counts before and during treatment with AYVAKIT.

Please see full [Important Safety Information](#) on pages 10–11, and [click here](#) to see full [Prescribing Information](#) and [Patient Information](#) for AYVAKIT.

Get to know AYVAKIT

Welcome to AYVAKIT—this is your guide for information on treatment with AYVAKIT for Advanced SM. AYVAKIT is a therapy for Advanced SM, a disease that has had limited treatment options.

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IMPORTANT SAFETY INFORMATION

AYVAKIT may cause serious side effects, including:

Cognitive effects. Cognitive side effects can happen during treatment with AYVAKIT and can be severe. Tell your healthcare provider if you develop any new or worsening cognitive symptoms including forgetfulness, confusion, getting lost, trouble thinking, drowsiness, trouble staying awake (somnolence), word finding problems, seeing objects or hearing things that are not there (hallucinations), or a change in mood or behavior.

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What is Advanced SM?

- Advanced SM is a rare disease that affects the production of mast cells (a type of white blood cell), which are part of your body's immune system
- Your body makes mast cells as part of an inflammatory response to keep you healthy
- For patients with Advanced SM, mast cells are overproduced and can build up in different parts of your body, such as the skin, bone marrow, and digestive tract
- The overproduction of mast cells can cause you to experience the symptoms of an allergic reaction, as well as other symptoms of Advanced SM

COMMON SYMPTOMS INCLUDE:



Skin lesions
(may look like a rash)



Unexplained severe
allergic reactions
(difficulty breathing)



Nausea, vomiting,
and diarrhea

There are more than 20 symptoms that patients living with Advanced SM may experience. Additional symptoms can include:

- Weight loss
- Fatigue

Each patient is different and some may not experience all the symptoms of Advanced SM.

What is AYVAKIT?

AYVAKIT is a prescription medicine used to treat adults with advanced systemic mastocytosis (AdvSM), including aggressive systemic mastocytosis (ASM), systemic mastocytosis with an associated hematological neoplasm (SM-AHN), and mast cell leukemia (MCL).



AYVAKIT **targets a genetic mutation behind Advanced SM** to help slow the growth of mast cells.



AYVAKIT is taken by mouth **once daily on an empty stomach.**

Take AYVAKIT exactly as prescribed, and talk to your doctor if you have any questions about treatment.

Please see additional dosing information on [page 8](#).

IMPORTANT SAFETY INFORMATION

AYVAKIT may cause serious side effects, including:

Skin sensitivity to sunlight (photosensitivity). Your skin may be sensitive to the sun or other forms of light (photosensitivity) during treatment with AYVAKIT. Avoid or limit exposure to direct sunlight, sunlamps, and other sources of ultraviolet radiation during treatment and for 1 week after stopping treatment with AYVAKIT. Use sunscreen or wear clothes that cover your skin if you need to be out in the sun.

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How does AYVAKIT work?

About 95% of patients with Advanced SM have the KIT D816V mutation.

The KIT D816V mutation leads to the **uncontrolled growth of abnormal mast cells**.

AYVAKIT primarily **targets the KIT D816V mutation** to help slow the growth of mast cells.

HEALTHY INDIVIDUAL

Normal levels of mast cells

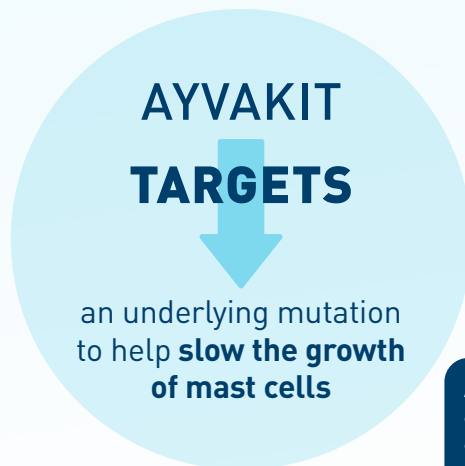
The body makes mast cells as part of an inflammatory response to keep us healthy.

ADVANCED SM

Abnormal levels of mast cells

In patients with Advanced SM, abnormal mast cells are overproduced and can build up in parts of the body.

The overproduction of mast cells can cause you to experience the symptoms of an allergic reaction, as well as other symptoms of Advanced SM.



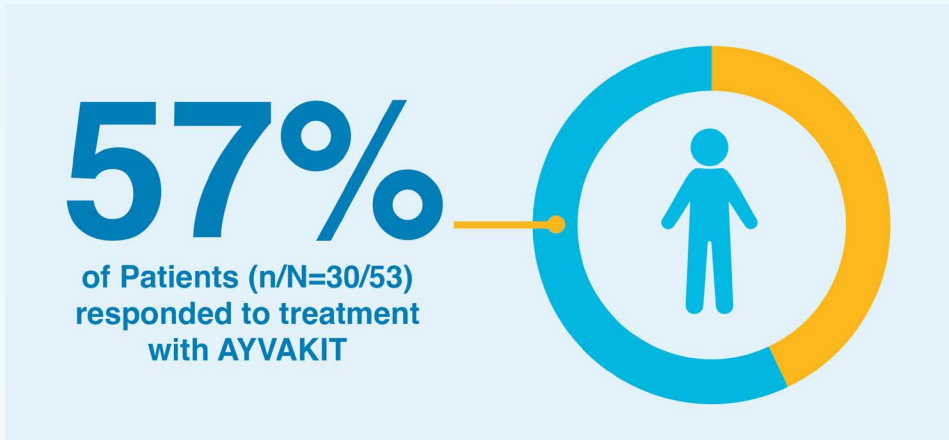
A mutation is an abnormal change to a gene, which may lead to diseases.

You do not have to have the KIT D816V mutation to take AYVAKIT.

How treatment with AYVAKIT may help

AYVAKIT was studied in 2 clinical trials to determine if it was safe and effective for patients with Advanced SM. In the studies, 53 patients had their response to treatment with AYVAKIT evaluated.

All 3 subtypes of Advanced SM were represented (ASM, SM-AHN, and MCL) and doses were taken as prescribed.



- This means that of the 53 patients with Advanced SM treated with AYVAKIT, 30 showed improvement in their disease
- The time to response for symptom improvement was a median of 2.1 months

Individual results may vary. It is important to note that having a response to treatment does not mean a person's Advanced SM has been cured.

IMPORTANT SAFETY INFORMATION

Before taking AYVAKIT, tell your healthcare provider about all of your medical conditions, including if you:

- have a history of bulging or weakening of a blood vessel wall (aneurysm) or bleeding in your brain
- have a history of stroke within the last year
- have low platelet counts
- have or have had liver problems

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Know the possible side effects of AYVAKIT

Always consult with your doctor regarding questions about treatment with AYVAKIT.

AYVAKIT MAY CAUSE SERIOUS SIDE EFFECTS, INCLUDING:

Bleeding in your brain: Serious bleeding in the brain may happen during treatment with AYVAKIT and may lead to death. Stop taking AYVAKIT and tell your doctor immediately if you develop any symptoms such as severe headache, nausea, vomiting, vision changes, drowsiness, dizziness, confusion, or severe weakness on one or more side of your body.

Cognitive effects: Cognitive side effects can happen during treatment and can be severe. Tell your healthcare provider if you develop any new or worsening cognitive symptoms, including:

- Forgetfulness
- Confusion
- Getting lost
- Trouble thinking
- Drowsiness
- Trouble staying awake (somnolence)
- Word finding problems
- Seeing objects or hearing things that are not there (hallucinations)
- Change in mood or behavior

Skin sensitivity to sunlight (photosensitivity): Your skin may be sensitive to the sun or other forms of light (photosensitivity) during treatment with AYVAKIT. Avoid or limit exposure to direct sunlight, sunlamps, and other sources of ultraviolet radiation during treatment and for 1 week after stopping treatment with AYVAKIT. Use sunscreen or wear clothes that cover your skin if you need to be out in the sun.

Do not drive or operate hazardous machinery if you have confusion or trouble thinking during treatment with AYVAKIT.

COMMON SIDE EFFECTS TO TREATMENT WERE:

- Fluid retention or swelling
- Diarrhea
- Nausea
- Tiredness
- Changes in certain blood tests

AYVAKIT may cause fertility problems in females and males. Talk to your healthcare provider if this is a concern for you.

These are not all of the possible side effects of AYVAKIT. Inform your doctor about any side effects you experience.

AYVAKIT is taken once per day



One AYVAKIT tablet



One time
each day



On an empty stomach,
at least 1 hour before
or 2 hours after a meal

The recommended dose for AYVAKIT is 200 mg.

Take AYVAKIT exactly as prescribed by your doctor. Do not change your dose or stop taking AYVAKIT unless your doctor tells you to. Your doctor may change, temporarily stop, or permanently stop treatment with AYVAKIT if you develop side effects.

IF YOU MISS A DOSE OF AYVAKIT:

Take it as soon as you remember, unless your next scheduled dose is due within 8 hours. Then, take the next dose at your regular time.

IF YOU VOMIT AFTER TAKING AYVAKIT:

Do not take an extra dose. You should take your next dose at your next scheduled time.

IMPORTANT SAFETY INFORMATION

Before taking AYVAKIT, tell your healthcare provider about all of your medical conditions, including if you:

- are pregnant or plan to become pregnant. AYVAKIT can cause harm to your unborn baby

Females who are able to become pregnant: Your healthcare provider should do a pregnancy test before you start AYVAKIT. You should use effective birth control (contraception) during treatment with AYVAKIT and for 6 weeks after the final dose of AYVAKIT. Talk to your healthcare provider about birth control methods that may be right for you. Tell your healthcare provider right away if you become pregnant or think you may be pregnant during treatment with AYVAKIT.

Males with female partners who are able to become pregnant should use effective birth control (contraception) during treatment and for 6 weeks after the final dose of AYVAKIT.

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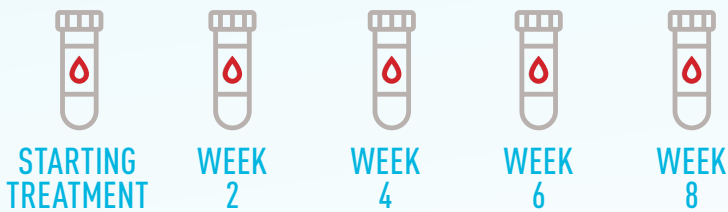
Treatment monitoring

BEFORE TAKING AYVAKIT, TELL YOUR DOCTOR:

- All of your medical conditions including if you have a history of bulging or weakening of a blood vessel wall (aneurysm), history of bleeding in your brain, history of stroke within the last year, low platelet counts, or have or had liver problems
- All the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements
- If you are pregnant or plan on becoming pregnant
- If you are breastfeeding or plan to breastfeed

MONITORING YOUR BLOOD COUNTS WITH YOUR DOCTOR

Monitoring blood counts by collecting regular samples is part of tracking your response to treatment and potential side effects. Your doctor will monitor your blood counts before you start treatment and throughout the treatment course according to the following schedule:



After 8 weeks, your doctor will determine how frequently to monitor your blood platelet counts.

MONITORING HOW YOU ARE FEELING

Your doctor will also discuss with you monitoring and tracking side effects that may occur during treatment with AYVAKIT.

FOR MORE INFORMATION ABOUT AYVAKIT

please talk to your doctor and visit [AYVAKIT.com](https://www.ayvakit.com)

Important safety information

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Before taking AYVAKIT, tell your healthcare provider about all of your medical conditions, including if you:

- have a history of bulging or weakening of a blood vessel wall (aneurysm) or bleeding in your brain
- have a history of stroke within the last year
- have low platelet counts
- have or have had liver problems
- are pregnant or plan to become pregnant. AYVAKIT can cause harm to your unborn baby.

Females who are able to become pregnant: Your healthcare provider should do a pregnancy test before you start AYVAKIT. You should use effective birth control (contraception) during treatment with AYVAKIT and for 6 weeks after the final dose of AYVAKIT. Talk to your healthcare provider about birth control methods that may be right for you. Tell your healthcare provider right away if you become pregnant or think you may be pregnant during treatment with AYVAKIT.

Males with female partners who are able to become pregnant should use effective birth control (contraception) during treatment and for 6 weeks after the final dose of AYVAKIT.

- are breastfeeding or plan to breastfeed. It is not known if AYVAKIT passes into your breast milk. Do not breastfeed during treatment with AYVAKIT and for at least 2 weeks after the final dose of AYVAKIT. Talk to your healthcare provider about the best way to feed your baby during this time

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. AYVAKIT may affect the way other medicines work, and certain other medicines may affect how AYVAKIT works. **Especially tell your healthcare provider** if you take medicines that prevent blood clots.

Do not drive or operate hazardous machinery if you have confusion or trouble thinking during treatment with AYVAKIT.

The most common side effects of AYVAKIT in people with AdvSM include: fluid retention or swelling, diarrhea, nausea, tiredness or weakness, and changes in certain blood tests.

Your healthcare provider may change your dose, temporarily stop, or permanently stop treatment with AYVAKIT if you develop certain side effects. AYVAKIT may cause fertility problems in females and males. Talk to your healthcare provider if this is a concern for you.

These are not all of the possible side effects of AYVAKIT. Call your doctor 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**.

AYVAKIT is available in 25 mg, 50 mg, 100 mg and 200 mg tablets.

Click here to see full [Prescribing Information](#) and [Patient Information](#) for AYVAKIT.



YourBlueprint® is a patient support program designed to assist you with personalized support right from the start of your treatment with AYVAKIT by providing:

- **A dedicated support team**
- **Financial assistance options**
- **Helpful resources**

To see how we can help:

- Visit www.YourBlueprint.com
- ☎ Or call [1-888-BLUPRNT \(1-888-258-7768\)](tel:1-888-BLUPRNT)
Monday–Friday, 8 AM–8 PM Eastern Time (ET)

TO LEARN MORE ABOUT TREATMENT WITH AYVAKIT

sign up on AYVAKIT.com

Blueprint Medicines is the manufacturer of AYVAKIT. Blueprint Medicines is a precision therapy company focused on genomically defined cancers, rare diseases, and cancer immunotherapy, with headquarters in Cambridge, MA.



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ABOUT
ADVANCED SM

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AYVAKIT

TREATMENT
WITH AYVAKIT

TAKING
AYVAKIT

IMPORTANT SAFETY
INFORMATION