



A guide for patients and caregivers



Words you will want to know



Whether you are reading this brochure for yourself or for someone you care about, understanding these words will help.

GIST Gastrointestinal stromal tumor (GIST) is a type of cancer that can appear anywhere in the gastrointestinal tract, including the stomach and small intestine.

MUTATIONS Abnormal changes in genes that can lead to cancer cell growth.

KIT and PDGFRA Two of the most common genes affected by mutations that can produce abnormal proteins involved in the growth of GIST. These names are used to refer to both the genes and proteins they produce.

EXON The location on the gene where mutations can occur.

PROTEINS Genes control how cells work by making proteins to do specific jobs in the cell. Mutated genes create abnormal proteins that can cause cells to multiply uncontrollably and become cancerous.

What is AYVAKIT® (avapritinib)?

AYVAKIT is a prescription medicine used to treat adults with a certain type of stomach, bowel, or esophagus cancer called gastrointestinal stromal tumor (GIST) that cannot be treated with surgery or that has spread to other parts of the body (metastatic), and that is caused by certain abnormal platelet-derived growth factor receptor alpha (PDGFRA) genes. Your healthcare provider will perform a test to make sure that you have this abnormal PDGFRA gene and that AYVAKIT is right for you.

It is not known if AYVAKIT is safe and effective in children.

AYVAKIT TREATMENT STARTS HERE

In this brochure you'll find information about:

- how AYVAKIT works
- how to take it, and
- what you or your loved one can expect while on treatment



SELECT 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.

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 change in mood or behavior.

Please see additional Select Safety Information throughout and click here for full [Prescribing Information](#) and [Patient Information](#) for AYVAKIT.

Understanding mutations in GIST

People diagnosed with GIST should be tested for mutations in KIT and PDGFRA to determine which type of therapy might be right for them.

The most common mutations in GIST are on the KIT gene. However, 10% of people with GIST have a mutation on a different gene called PDGFRA.¹ The most frequent type of PDGFRA mutations in GIST occur in exon 18, including the D842V mutation.

AYVAKIT is the first approved therapy for GIST caused by a PDGFRA exon 18 mutation including the PDGFRA D842V mutation.

Other available GIST therapies may not work for people whose cancer is caused by the PDGFRA D842V mutation.

Reference: 1. Nishida T, Blay J-Y, Hirota S, Kitagawa Y, Kang Y-K. The standard diagnosis, treatment, and follow-up of gastrointestinal stromal tumors based on guidelines. *Gastric Cancer*. 2016;19(1):3-14.

**BE SURE TO ASK
YOUR HEALTHCARE
PROVIDER IF
YOUR GIST HAS
BEEN TESTED
FOR MUTATIONS.**



SELECT 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.

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

- 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

Please see additional Select Safety Information throughout and click here for full [Prescribing Information](#) and [Patient Information](#) for AYVAKIT.

AYVAKIT is designed to work differently

HOW DOES AYVAKIT WORK?

AYVAKIT targets abnormal proteins involved in the growth of GIST that are caused by the KIT and PDGFRA mutation. **AYVAKIT is the only approved GIST treatment that binds to these abnormal proteins while they are in their “on” state.** This means it targets, binds to, and inhibits the specific proteins that lead to the growth and spread of GIST cancer cells.

Other approved GIST treatments bind to cancer-spreading proteins only when they are in their “off” state. However, for people with PDGFRA D842V GIST, their mutation results in the protein being locked in the “on” state. **This makes it difficult for other approved GIST treatments to bind.**



SELECT 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.
- 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

Please see additional Select Safety Information throughout and click here for full [Prescribing Information](#) and [Patient Information](#) for AYVAKIT.

AYVAKIT was proven to work against GIST with a PDGFRA exon 18 mutation

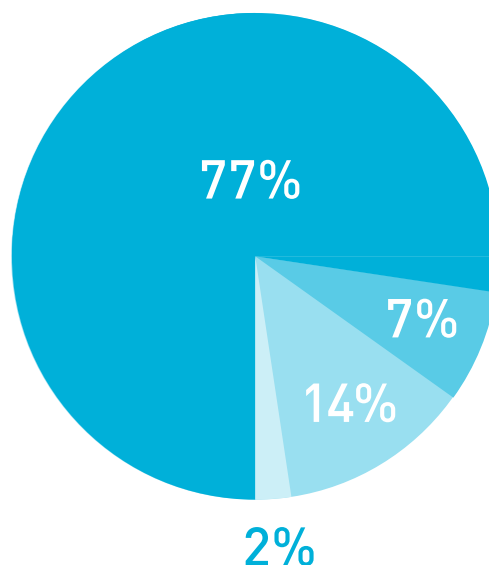
AYVAKIT was studied in a clinical trial called NAVIGATOR (NCT02508532). The trial included 43 people with PDGFRA exon 18 mutations, 38 of whom had the PDGFRA D842V mutation.

84% OF 43 PEOPLE RESPONDED:

- 33 people (77%) had their tumors shrink by at least 30% (partial response)
- 3 people (7%) had their tumors completely disappear (complete response)

IN ADDITION:

- 6 people (14%) had no change in their tumor size (stable disease)
- 1 person (2%) had their tumor grow (progressive disease)



For the 38 people with a PDGFRA D842V mutation, 89% responded. Thirty-one people (82%) had a partial response and 3 people (8%) had a complete response.

It's important to note that having a response does not mean the cancer has been cured.
Individual results may vary.

SELECT SAFETY INFORMATION

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 estrogen-containing hormonal birth control or medicines that prevent blood clots.

Please see additional Select Safety Information throughout and [click here for full Prescribing Information and Patient Information for AYVAKIT.](#)

Potential side effects

You should have an open conversation with your family or caregiver about the potential side effects of AYVAKIT. They may be able to help you recognize any changes and can help you share them with your healthcare provider.

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.
- **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 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.

The most common side effects of AYVAKIT in people with GIST include:

- fluid retention or swelling
- nausea
- tiredness or weakness
- trouble thinking
- vomiting
- decreased appetite
- diarrhea
- increased eye tearing
- stomach area (abdominal) pain
- constipation
- rash
- dizziness
- hair color changes, 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.

Please see additional Select Safety Information throughout and click here for full [Prescribing Information](#) and [Patient Information](#) for AYVAKIT.



How to take AYVAKIT® (avapritinib)

Take AYVAKIT exactly as your healthcare provider tells you to take it. Do not change your dose or stop taking AYVAKIT unless your healthcare provider tells you to.

PEOPLE SHOULD TAKE:



1 AYVAKIT
tablet



1 time
each day



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

The recommended dose of AYVAKIT is 300 mg orally once daily. Your healthcare provider may change your dose, temporarily stop, or permanently stop treatment with AYVAKIT if you develop side effects. AYVAKIT is available in doses of 100mg, 200mg, and 300mg for PDGFRA Exon 18 mutated GIST.

What if you miss a dose?

- If you miss a dose of AYVAKIT, take it as soon as you remember unless your next scheduled dose is due within 8 hours. Take the next dose at your regular time.
- If you vomit after taking a dose of AYVAKIT, do not take an extra dose. Take your next dose at your next scheduled time.

Monitoring how you feel is important.

- Stop taking AYVAKIT and tell your healthcare provider right away if you develop any symptoms such as severe headache, vomiting, drowsiness, dizziness, confusion, or severe weakness on one or more sides of your body.
- Tell your healthcare provider if you develop any new or worsening cognitive symptoms, such as 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 change in mood or behavior. Consider asking a caregiver, friend, or family member to let you know if they notice you having any unusual memory problems or other cognitive symptoms. It is important to let your doctor know about these symptoms immediately because it may mean you need to have your dose changed.

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

Additional instructions:

- Store AYVAKIT at room temperature between 68°F to 77°F (20°C to 25°C), and keep it out of reach of children.

People who care **for you** can help

Your doctor will want to know if you are experiencing any cognitive symptoms or other effects so they can change your dose.

Work with your doctor to come up with a list of questions that can help you monitor for any cognitive effects, such as how often in the past week you:

- Had trouble finding your way to a familiar place
- Lost your wallet or keys
- Had to search for words
- Seemed confused or lost
- Forgot why you walked into a room
- Wrote lists to keep from forgetting things
- Found it difficult to keep track of what you were doing



Share this message with your friend or family member:

You may be able to spot some signs of cognitive symptoms that your loved one may miss. Be sure to record symptoms you see and to share them with the doctor. The doctor may use that information to make a dose adjustment.

Enlist the help of a caregiver, friend, or family member to go through the questions with you periodically. You should both be looking for changes in your answers so that you can alert your doctor immediately if you think you are having cognitive effects.

TIP: CONSIDER RECORDING YOUR ANSWERS IN A NOTEBOOK OR ON YOUR PHONE.



SELECT SAFETY INFORMATION

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

AYVAKIT is available in 100-mg, 200-mg and 300-mg tablets.

Please see additional Select Safety Information throughout and click here for full [Prescribing Information](#) and [Patient Information](#) for AYVAKIT.





Support **starts here**



YourBlueprint® is a patient support program designed to assist you throughout your treatment with AYVAKIT by providing:

- 1-on-1 support
- Financial assistance options
- Helpful resources

Connect with a Case Manager today.

Call **1-888-BLUPRNT (1-888-258-7768)**

Monday-Friday from 8 AM-8 PM Eastern Time (ET)
or visit **YourBlueprint.com**.

OTHER HELPFUL RESOURCES FOR PATIENTS AND CAREGIVERS*:

American Cancer Society
cancer.org
800-227-2345

CancerCare
cancercare.org
800-813-4673

Cancer Support Community
cancersupportcommunity.org
888-793-9355

The Life Raft Group
liferaftgroup.org
973-837-9092

National Cancer Institute
cancer.gov
800-422-6237

*These independent advocacy groups are included as a reference for patients, and do not imply endorsement of the company or its products by the groups listed. Blueprint Medicines is not affiliated with and does not endorse any particular advocacy group. We make no guarantees about the accuracy of the information provided on advocacy groups' websites or the quality of support provided.

To learn more about AYVAKIT, speak to your healthcare provider or visit **AYVAKIT.com/GIST**.



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