A guide for patients and caregivers

AYVAKIT™
avapritinib | tablets

YourBlueprint™ is a patient support program designed to assist you
and your caregivers. Contact us to learn more.

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YourBlueprint™ is a patient support program designed to assist you
and your caregivers. Call 1-888-BLUPRNT (1-888-258-7768) to
connect with a Case Manager today.

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

Support starts here

AYVAKIT.COM

- Blueprint Medicines is not affiliated with these organizations, does not endorse any particular service or group, and is not responsible for the content on their websites or any services or resources they may provide.

Support starts here

OTHER HELPFUL RESOURCES FOR PATIENTS AND CAREGIVERS:

- The Life Raft Group®
  - 215-340-9374
gistsupport.org

- American Cancer Society®
  - 800-227-2345
cancer.org

- American Gastrointestinal and Endoscopic Society®
  - 800-212-5137
cancer.gov

- National Institute of Neurological Disorders and Stroke®
  - 800-352-9424

- Center for Disease Control and Prevention®
  - 800-CDC-INFO

- National Cancer Institute®
  - 800-422-6237

- Patient Portrayal
  - 1-888-BLUPRNT (1-888-258-7768)

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For more information about mutational testing, please visit mutationmatters.com.

For more information about AYVAKIT, speak to your healthcare provider or visit AYVAKIT.COM.

IMPORTANT SAFETY INFORMATION

AYVAKIT may cause serious side effects, including:

- Central nervous system (CNS) effects
- Bleeding in your brain
- Constipation
- Diarrhea
- Nausea
- Tiredness

Please see additional Important Safety Information and full Prescribing Information and Patient Information on the next page.
GIST Gastrointestinal stromal tumor (GIST) is a type of cancer called a sarcoma (a tumor that affects connective tissue). GIST can appear anywhere in the gastrointestinal tract, including the stomach and small intestines.

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.
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) gene, also known as a mutation.

- 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.

IMPORTANT SAFETY INFORMATION

AYVAKIT may cause serious side effects, including:

- **Bleeding in your brain.** Stop taking AYVAKIT and tell your healthcare provider if you develop any symptoms such as severe headache, vision problems, severe sleepiness, or severe weakness on one side of your body.

Your healthcare provider may temporarily stop treatment or decrease your dose which may help your symptoms improve. If symptoms do not improve, your healthcare provider may permanently stop treatment with AYVAKIT.

Please see additional Important Safety Information and 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. 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.

For more information about mutational testing in GIST, please visit mutationmatters.com.


IMPORTANT SAFETY INFORMATION

AYVAKIT may cause serious side effects, including:

- **Central nervous system (CNS) effects.** CNS side effects are common with AYVAKIT and can be severe. Tell your healthcare provider if you develop any new or worsening CNS symptoms including forgetfulness, confusion, getting lost, trouble thinking, drowsiness, dizziness, trouble sleeping, word finding problems, seeing objects or hearing things that are not there (hallucinations), or change in mood or behavior.

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AYVAKIT™ (avapritinib) 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.

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.
• 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.
AYVAKIT™ (avapritinib) is proven to work against GIST with a PDGFRA exon 18 mutation

**Most People Responded**

Overall response rate (ORR) was defined as the percentage of people who had target tumors that either:

- shrunk in size by at least 30% [partial response (PR)], or
- completely disappeared [complete response (CR)].

A response does not mean the cancer has been cured.

AYVAKIT was studied in a clinical trial called the NAVIGATOR trial (NCT02508532). The trial included 43 people with PDGFRA exon 18 mutations, 38 of whom had the PDGFRA D842V mutation. For the 38 people with a PDGFRA D842V mutation, the ORR was 89%. 82% (31 of 38) had a PR, and 8% (3 of 38) had a CR.

**Important 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. Talk to your healthcare provider prior to starting a new medicine.

What should I avoid while taking AYVAKIT?

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

Please see additional Important Safety Information and full Prescribing Information and Patient Information for AYVAKIT.
AYVAKIT™ (avapritinib) is proven to work against GIST with a PDGFRA exon 18 mutation PDGFRA D842V.

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For the 38 people with a PDGFRA D842V mutation, the ORR was 89%. 82% (31 of 38) had a PR, and 8% (3 of 38) had a CR.
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 and 2 hours after a meal

The recommended starting dose of AYVAKIT is 300 mg orally once daily. AYVAKIT is available in doses of 100 mg, 200 mg, and 300 mg.

**Additional instructions:**
- Store AYVAKIT at room temperature between 68°F to 77°F (20°C to 25°C).
- Keep AYVAKIT and all medicines out of reach of children.

**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 if you develop any symptoms such as severe headache, vision problems, severe sleepiness, or severe weakness on one side of your body.
- Tell your healthcare provider if you develop any new or worsening central nervous system symptoms, such as forgetfulness, confusion, getting lost, trouble thinking, drowsiness, dizziness, trouble sleeping, word finding problems, seeing objects or hearing things that are not there (hallucinations), or change in mood or behavior. These symptoms may mean you need to have your dose modified.

*It is common to have your AYVAKIT dose modified by your healthcare provider.*

Your healthcare provider may change your dose, temporarily stop, or permanently stop treatment with AYVAKIT if you develop side effects.
Talking about treatment

Consider having 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.** Stop taking AYVAKIT and tell your healthcare provider if you develop any symptoms such as severe headache, vision problems, severe sleepiness, or severe weakness on one side of your body.

- **Central nervous system (CNS) effects.** CNS side effects are common with AYVAKIT and can be severe. Tell your healthcare provider if you develop any new or worsening CNS symptoms including:
  - forgetfulness
  - confusion
  - getting lost
  - trouble thinking
  - drowsiness
  - dizziness
  - trouble sleeping
  - word finding problems
  - seeing objects or hearing things that are not there (hallucinations), or
  - change in mood or behavior

Your healthcare provider may temporarily stop treatment or decrease your dose which may help your symptoms improve. If symptoms do not improve, your healthcare provider may permanently stop treatment with AYVAKIT.

The most common side effects of AYVAKIT include:

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

These are not all of the possible side effects of AYVAKIT. Call your doctor for medical advice about side effects.

You may report side effects to the FDA at **1-800-FDA-1088**.