AYVAKIT®
avapritinib | 25mg tablets

The ONLY FDA-approved therapy to treat **adults with indolent systemic mastocytosis (ISM)**[1]

~3 YEARS OF EFFICACY AND SAFETY DATA[2]

Patient portrayal

FDA=Food and Drug Administration.

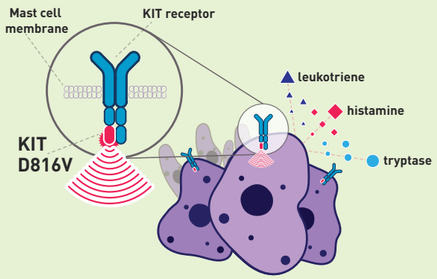
INDICATION

AYVAKIT® (avapritinib) is indicated for the treatment of adult patients with indolent systemic mastocytosis (ISM).

Limitations of Use: AYVAKIT is not recommended for the treatment of patients with ISM with platelet counts of <50 x 10^9/L.

Please see Important Safety Information on page 17 and click here to see the full Prescribing Information for AYVAKIT.

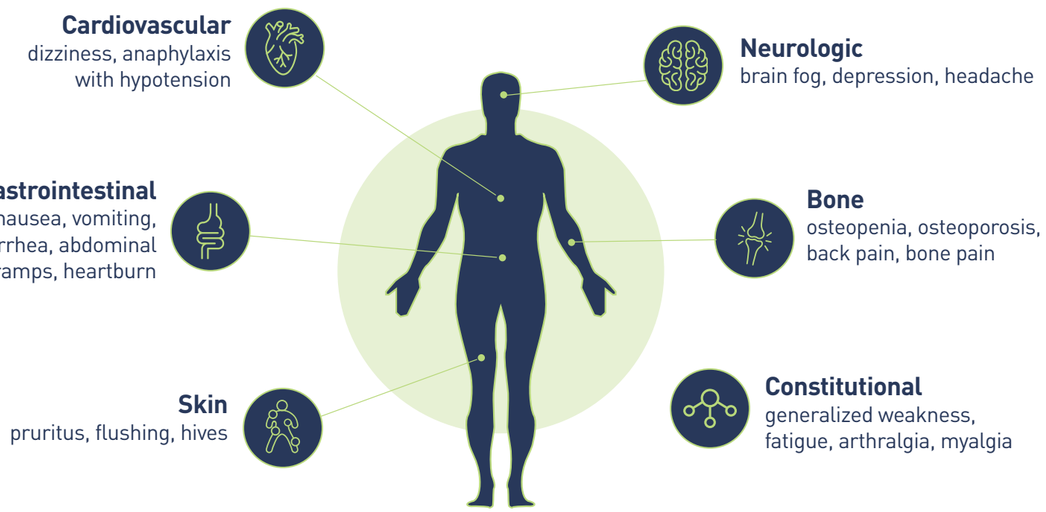
ISM: AN OVERPRODUCTION OF MUTATED AND HYPERACTIVE MAST CELLS CREATES DISRUPTION THROUGHOUT THE BODY[3,4]



~95% of ISM cases involve the KIT D816V mutation[5-7]

- This mutation causes the KIT receptor to be constantly "switched on," disrupting normal cell growth regulation, leading to the overproduction of mutated, hyperactive mast cells[3,4,8,9]
- These mutated, hyperactive mast cells then proliferate, accumulate, and release mediators that can trigger potentially debilitating symptoms across organ systems[3,4,10]

SYMPTOMS CAN FLARE UNEXPECTEDLY AND ARE TRIGGERED BY ASPECTS OF EVERYDAY LIFE[3,4,10]



These symptoms are not comprehensive but represent the clinical spectrum of ISM. Symptoms may vary in individual patients.

BMI=body mass index; HCP=healthcare provider; ISM-SAF=Indolent Systemic Mastocytosis-Symptom Assessment Form; KIT=KIT proto-oncogene, receptor tyrosine kinase; SM=systemic mastocytosis; TSS=total symptom score; WHO=World Health Organization.

PATIENTS OFTEN ADAPT THEIR DAILY LIVES TO COPE WITH THE SYMPTOM BURDEN OF SM[11]*

- **Increased polypharmacy[2]**
- **Considerable family and caregiver burden[12]**
- **Multiple HCP visits per year[2]**

In a Blueprint Medicines–sponsored survey (n=32)[2†]

- **72%** reported **avoiding leaving home**
- **56%** reported **reducing working hours**
- **28%** reported **going on medical disability**

ISM MAY LEAD TO SERIOUS HEALTH RISKS BEYOND LIFESTYLE DISRUPTIONS[11,13]

Symptom frequency and severity often worsen over time[14]: 55% of ISM patients experience **more frequent symptoms** and 47.5% report **increased severity** since diagnosis (N=40)‡

Increased risk of bone complications[2]: **Osteoporosis was observed in 66.9% of patients** vs 34.3% of a similar patient population without ISM§

Higher risk of anaphylaxis[15¶]: ~1 in 2 patients face **increased risk** of anaphylaxis

Mastocytosis Control Test: Scan the QR code to evaluate your ISM patient's disease control with a validated 5-question tool

*A descriptive qualitative study design investigating the lived experiences of 16 adult patients with SM (9 with ISM and 7 with AdvSM). Data were collected through semi-structured interviews and analyzed via inductive content analysis to identify key themes related to symptom burden, healthcare interactions, and psychosocial adaptation.[11]

†In the Blueprint Medicines–sponsored TouchStone SM Patient Survey, US adults with a self-reported SM diagnosis (N=56) completed an online survey of 100 items. An analysis was conducted in patients with ISM (n=37), including 32 patients with moderate to severe ISM (defined as an ISM-SAF TSS ≥28) and results were analyzed using descriptive statistics. These analyses were made from the TouchStone SM Patient Survey but have not been published.[2]

‡Survey data were collected from 40 adults with ISM meeting the WHO diagnostic criteria, including the validated ISM-SAF and the 12-item Short-Form Health Survey. ISM burden was analyzed by comparing moderate to severe TSS scores with mild TSS scores using Kruskal–Wallis and Fisher's exact tests.[2]

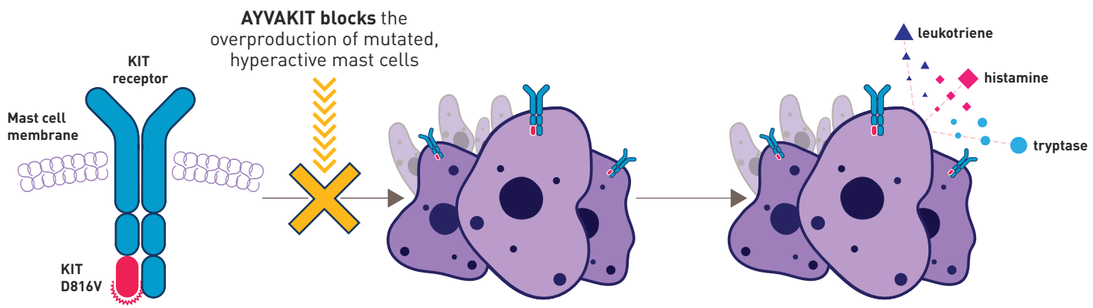
§In a retrospective study of Mayo Clinic data (full system, 2005-2023), 203 patients with ISM were identified and matched 1:10 with 2030 patients without ISM on demographic and clinical characteristics (age, sex, race, BMI, smoking status, Quan-Charlson Comorbidity Index [CCI] score, and year of index [defined as first ISM diagnosis for ISM patients]), generating comparable cohorts with ISM diagnosis being the differentiating factor between groups.[2]

¶As described by an expert-panel review of adult-onset mastocytosis (predominantly indolent population).[15]

Target the source, not just the symptoms[1,3]

Symptom-directed therapies alone may not be enough[1,3]

Symptom-directed therapies (SDT) may help address ISM symptoms but do not treat the underlying cause of the symptoms: the KIT D816V mutation. AYVAKIT inhibits the autophosphorylation of KIT in cellular assays, which ultimately blocks overproduction of abnormal and hyperactive mast cells thought to cause the symptoms of ISM.[1,3]



Mutated KIT continually sends signals causing abnormal mast cell growth and survival.

Abnormal, hyperactive mast cells accumulate throughout the body.

Mast cells degranulate, releasing **inflammatory mediators** such as **leukotriene, histamine, and tryptase**.

AYVAKIT TARGETS THE KIT D816V MUTATION—THE PRIMARY DRIVER OF ISM[1,3,4,10,16]

Consider targeting the cause of the symptoms when treating ISM[1,4,10]

SELECT SAFETY INFORMATION

Cognitive Effects—Cognitive adverse reactions can occur in patients receiving AYVAKIT and occurred in 7.8% of patients with ISM who received AYVAKIT + best supportive care (BSC) versus 7.0% of patients who received placebo + BSC; <1% were Grade 3. Depending on the severity, withhold AYVAKIT and then resume at the same dose, or permanently discontinue AYVAKIT.

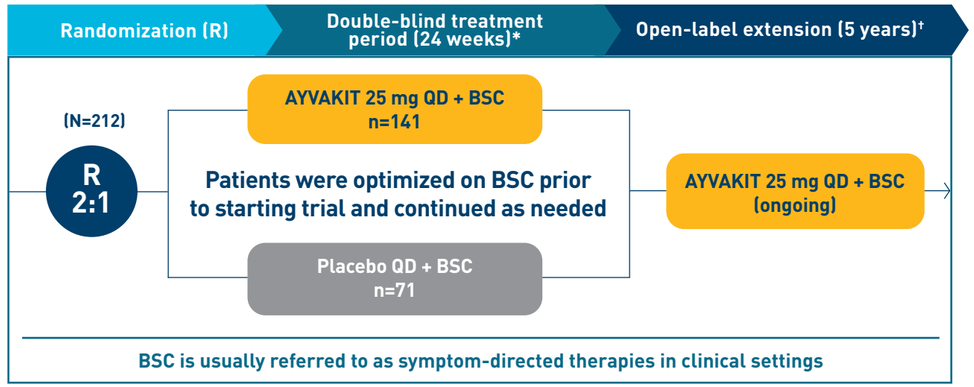
Please see Important Safety Information on page 17 and click here to see the full Prescribing Information for AYVAKIT.

PIONEER was designed to measure symptom relief and change in mast cell burden[1,17]

PIONEER: Phase 2, multipart, randomized, placebo-controlled, double-blind trial (N=212) evaluating the efficacy and safety of AYVAKIT 25 mg vs placebo at 24 weeks, both arms receiving concomitant BSC[1,17]

Key eligibility criteria: ≥18 years of age; centrally confirmed ISM diagnosis per WHO criteria; uncontrolled moderate to severe ISM symptoms (defined as ISM-SAF TSS ≥28) despite ≥2 BSC[17]

STUDY DESIGN: AYVAKIT + BSC VS PLACEBO + BSC[1,17]



SYMPTOM MEASUREMENT

Primary endpoint: Absolute mean change in ISM-SAF TSS compared with placebo + BSC from baseline to Week 24[1]

Exploratory endpoints: Mean change in ISM-SAF individual symptom scores; mean change in most severe symptom score at Week 24[2,17]

MAST CELL BURDEN MEASUREMENT

Select key secondary endpoints compared with placebo + BSC at Week 24[1,17]:

Proportion of patients achieving:

- ≥50% reduction in serum tryptase levels
- ≥50% reduction in KIT D816V VAF or undetectable‡
- ≥50% reduction in bone marrow mast cells or no aggregates

ISM-SAF TSS: A validated PRO assessment unique to PIONEER[17]

- The ISM-SAF assessed 11 ISM signs and symptoms (abdominal pain, nausea, diarrhea, spots, itching, flushing, bone pain, fatigue, dizziness, headache, and brain fog)§

*Data cutoff was June 23, 2022.[17]
†Patients had the option to enter part 3 of PIONEER, an open-label extension evaluating the long-term efficacy and safety of AYVAKIT 25 mg + BSC for up to 5 years. All eligible patients either continued AYVAKIT 25 mg + BSC daily or switched from placebo + BSC to AYVAKIT 25 mg + BSC.[17]
‡In peripheral blood.[1]
§Symptom severity scores (scored 0 [no symptoms] to 10 [worst imaginable symptoms] daily) are combined to calculate the TSS from 0-110, with higher scores representing greater symptom severity. A biweekly average in ISM-SAF TSS was used to evaluate efficacy endpoints.[1,18]

BSC=best supportive care; PRO=patient-reported outcome; QD=every day; VAF=variant allele fraction.

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PIONEER reflects the heterogeneous population of patients living with ISM[17]

Select baseline demographics and patient characteristics (AYVAKIT + BSC, n=141; placebo + BSC, n=71)[17]

Patients were optimized on a range of BSC prior to trial enrollment[17]

- Anti-immunoglobulin E antibody (omalizumab)
- Glucocorticoids
- Cromolyn sodium
- H1 antihistamines
- H2 antihistamines
- Leukotriene inhibitors
- Proton pump inhibitors

	AYVAKIT + BSC (n=141)	Placebo + BSC (n=71)
Age Range Median Age	50 years (18-77)	54 years (26-79)
Sex Female	71%	76%
Varied Symptom Severity Mean ISM-SAF TSS (SD)	50.2 (19.1)	52.4 (19.8)
Polypharmacy Burden Median no. of BSC treatments (range)	3 (0-11)	4 (1-8)
Varied Tryptase Levels Median serum tryptase, ng/mL (range)	38.4 (3.6-256.0)	43.7 (5.7-501.6)
Mast Cell Burden Mast cell aggregates present	75%	80%

93% of patients in the AYVAKIT + BSC arm were KIT positive, with 7% KIT undetectable[1,17]

SELECT SAFETY INFORMATION

Photosensitivity—AYVAKIT may cause photosensitivity reactions. In all patients treated with AYVAKIT in clinical trials (n=1049), photosensitivity reactions occurred in 2.5% of patients. Advise patients to limit direct ultraviolet exposure during treatment with AYVAKIT and for one week after discontinuation of treatment.

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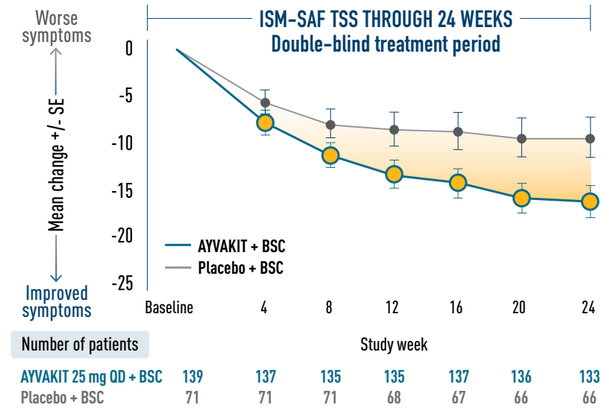
AYVAKIT significantly reduced symptom and mast cell burden vs placebo at 24 weeks[1]

PRIMARY ENDPOINT

AYVAKIT met the primary endpoint at 24 weeks, demonstrating significantly greater reduction in symptom burden when added to BSC vs placebo + BSC

Week 24	Absolute mean change in the ISM-SAF TSS (95% CI)
AYVAKIT + BSC (n=141)	-15.33 (-18.36, -12.31)
Placebo + BSC (n=71)	-9.64 (-13.61, -5.68)
2-sided *P* value	0.012



ITT analysis (table, left): Markov chain Monte Carlo simulation was used to impute the missing values at baseline or Week 24.

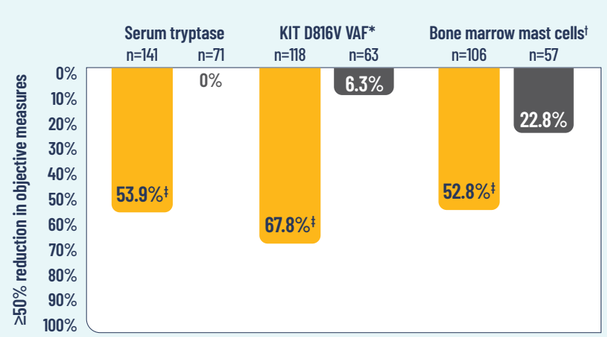
ITT analysis (linear graph, right): Patients with use of high-dose steroids (3 patients treated with AYVAKIT and 1 in the placebo group) within 7 days before Week 24, or greater than 14 consecutive days at any point from baseline, were included in this analysis that were not included in the primary analysis. Missing visit data were excluded from calculations for that visit.

LIMITATIONS (linear graph, right): Mean change in TSS at all time points except Week 24 were prespecified, nonranked endpoints and were not adjusted for multiplicity. Therefore, treatment differences at these time points cannot be regarded as statistically significant and results should be interpreted with caution.

KEY SECONDARY ENDPOINTS

AYVAKIT + BSC demonstrated significant reductions in measures of mast cell burden vs placebo + BSC at Week 24

PROPORTION OF PATIENTS ACHIEVING ≥50% REDUCTION IN OBJECTIVE MEASURES OF MAST CELL BURDEN AT 24 WEEKS[1]



ITT analysis: For patients with high-dose steroid use within 7 days before Week 24, or greater than 14 consecutive days at any point from baseline to Week 24, the Week 24 score was set to missing.

*Percent of patients with ≥50% reduction in peripheral blood KIT D816V VAF or undetectable.
†Percent of patients with ≥50% reduction in bone marrow mast cells or no aggregates.
‡2-sided *P*<0.0001.

ITT=intention to treat.

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ISM symptom changes were observed through ~3 years[2]

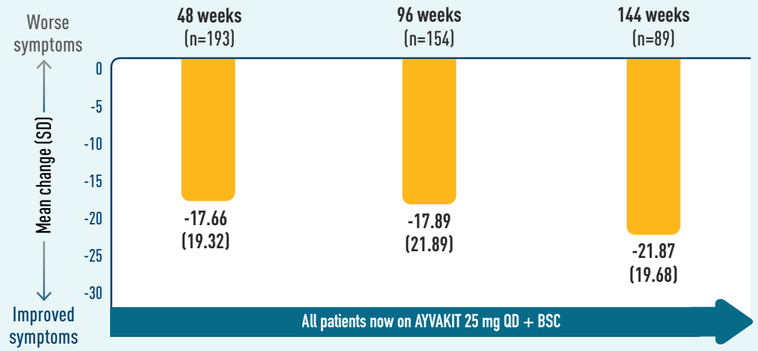
EXPLORATORY ENDPOINT

Decreases in TSS were observed through ~3 years in patients treated with AYVAKIT[2]*

- At 6 months, patients in the placebo + BSC arm could choose to receive AYVAKIT + BSC in an open-label extension trial for up to 5 years[17]
- 93% of patients in the placebo + BSC arm continued to the open-label extension and began receiving AYVAKIT[17]

POOLED ANALYSIS: MEAN CHANGE (SD) IN ISM-SAF TSS FROM THE FIRST AYVAKIT DOSE AT WEEKS 48, 96, AND 144[2]*

Pooled analysis includes patients initially randomized to AYVAKIT 25 mg QD + BSC and patients randomized to placebo + BSC who crossed over to AYVAKIT 25 mg QD + BSC at 6 months in the open-label extension. Data are displayed by time on treatment (cumulative exposure).



DATA LIMITATIONS: Mean change in ISM-SAF TSS at Weeks 24, 48, 96, and 144 from first AYVAKIT dose were nonranked endpoints and observational only, as such, results cannot be considered statistically significant. No statistical or clinical conclusions can be drawn and results should be interpreted with caution.

OLE LIMITATIONS: In an open-label extension, there is the potential for enrichment of the long-term data in the remaining patient populations since patients who are unable to tolerate or do not respond to the drug often drop out.

*Data cutoff: September 20, 2024.[2]

SD=standard deviation.

SELECT SAFETY INFORMATION

Embryo-Fetal Toxicity—AYVAKIT can cause fetal harm when administered to a pregnant woman. Advise pregnant women of the potential risk to a fetus. Advise females and males of reproductive potential to use an effective contraception during treatment with AYVAKIT and for 6 weeks after the final dose. Advise women not to breastfeed during treatment with AYVAKIT and for 2 weeks following the final dose.

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Mastocytosis quality of life mean score changes were observed through ~3 years[2]

EXPLORATORY ENDPOINT

The **MC-QoL Questionnaire**, a disease-specific QoL tool for use in patients with ISM, was used to assess QoL at each study visit[17]:

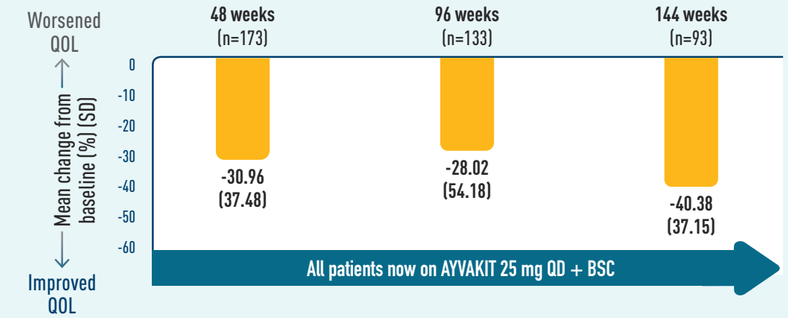
- Domains evaluated: **Symptoms**, **emotions**, **social life functioning**, **skin**
- Scores range 0–100; **higher scores = greater impairment (worse QoL)**

At 24 weeks, the mean percent changes in MC-QoL for patients receiving AYVAKIT + BSC and placebo + BSC were -34% (SD, -40% to -29%) and -18% (SD, -25% to -11%), respectively[11]

Through ~3 years, decreases in MC-QoL scores were observed in patients treated with AYVAKIT + BSC

POOLED ANALYSIS: MEAN PERCENT CHANGE (SD) IN MC-QOL SCORE FROM THE FIRST AYVAKIT DOSE AT WEEKS 48, 96, AND 144[2]*

Exploratory pooled analysis includes patients initially randomized to AYVAKIT 25 mg QD + BSC and patients randomized to placebo + BSC who crossed over to AYVAKIT 25 mg QD + BSC at 6 months in the open-label extension. Data are displayed by time on treatment (cumulative exposure).



DATA LIMITATIONS: Mean percent change in MC-QoL at Weeks 24, 48, 96, and 144 from first AYVAKIT dose were nonranked endpoints and observational only, as such, results cannot be considered statistically significant. No statistical or clinical conclusions can be drawn and results should be interpreted with caution.

OLE LIMITATIONS: In an open-label extension, there is the potential for enrichment of the long-term data in the remaining patient populations since patients who are unable to tolerate or do not respond to the drug often drop out.

*Data cutoff: September 20, 2024.[2]

MC-QoL=mastocytosis quality of life; QoL=quality of life; SD=standard deviation.

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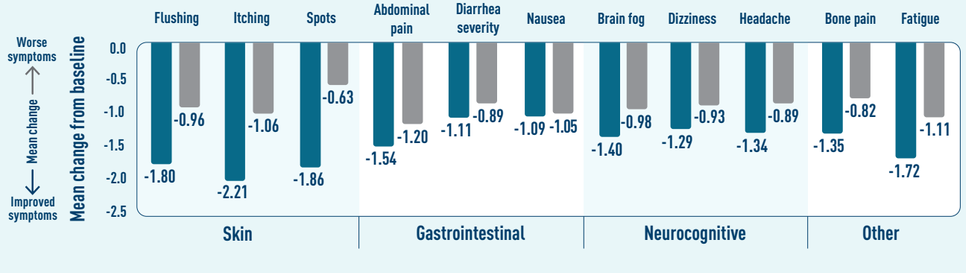
Individual symptom scores at 24 weeks[2]

EXPLORATORY ENDPOINT

Decreases in symptom severity were observed across all domains, including skin, gastrointestinal, and neurocognitive

MEAN CHANGE FROM BASELINE AT 24 WEEKS BY ISM-SAF INDIVIDUAL SYMPTOM SCORE



ITT analysis: In this analysis, if a patient was missing more than 7 days of the score between baseline score and Week 2 score, it was considered missing for the patient. If a patient was missing more than 7 days of the score from the 14-day period for calculating the Week 24 score, then the Week 24 score was considered as missing.

LIMITATIONS: Individual components in a descriptive exploratory analysis were prespecified, nonranked endpoints, not adjusted for multiplicity, and not powered. Therefore, data should be interpreted with caution, conclusions cannot be drawn, and treatment differences cannot be regarded as statistically significant.

Reductions were observed in patients' most severe symptom, defined as the symptom with the highest score at baseline[17]

SELECT SAFETY INFORMATION

Adverse Reactions—The most common adverse reactions (≥10%) in patients with ISM were eye edema, dizziness, peripheral edema, and flushing.

Drug Interactions—Avoid coadministration of AYVAKIT with strong or moderate CYP3A inhibitors or inducers. If contraception requires estrogen, limit ethinyl estradiol to ≤20 mcg unless a higher dose is necessary.

To report suspected adverse reactions, contact Blueprint Medicines Corporation at 1-888-258-7768 or the FDA at 1-800-FDA-1088 or visit www.fda.gov/medwatch.

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Skin outcomes on AYVAKIT at 24 weeks[2,19]

EXPLORATORY ENDPOINT

~80% of patients in PIONEER (167/212) reported skin involvement at baseline

- A subset of patients with skin biopsies agreed to optional skin photographs (AYVAKIT [n=74], placebo [n=37]), which were taken at baseline and every 12 weeks while patients received either AYVAKIT 25 mg or placebo—both with BSC. Skin changes were assessed by a blinded skin assessment committee (SAC), with affected surface area calculated by a computer-generated algorithm

A comprehensive assessment of skin changes was performed

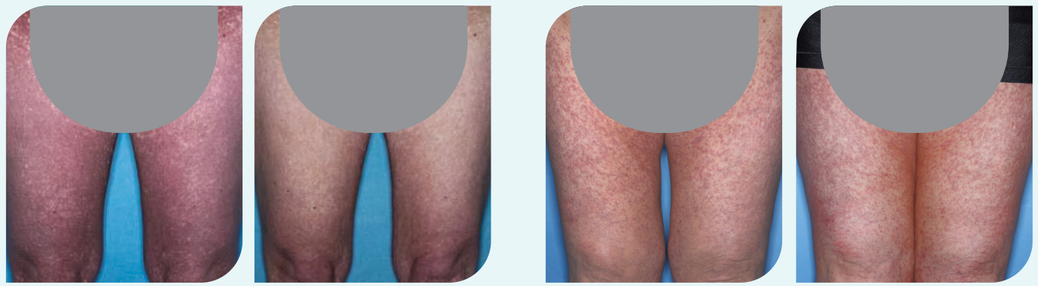
The blinded SAC evaluated

- Skin photographs to determine the most affected region at baseline
- Color change over time

The computer-generated algorithm measured

- Affected surface area
- Number of lesions
- Fractional area and percent fractional area

SKIN OUTCOMES AT 24 WEEKS (EXPLORATORY ENDPOINT)



These are example patient images and may not be representative of all patient responses to AYVAKIT.
Patient permission granted for use of photos.

LIMITATIONS: Images were optional in PIONEER and collected only in patients who consented to images and had baseline skin involvement. Evaluation of skin domain, including individual skin symptoms, were prespecified nonranked endpoints and not controlled for Type 1 error. As such, findings may be due to chance and results should be interpreted with caution. Individual results may vary.

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AYVAKIT was generally well tolerated in PIONEER through 24 weeks[1]

- Serious adverse reactions occurred in 1 patient (0.7%) who received AYVAKIT due to pelvic hematoma
- Permanent discontinuation of AYVAKIT due to an adverse reaction occurred in 1 patient (0.7%) due to dyspnea and dizziness
- Dosage interruptions of AYVAKIT due to an adverse reaction occurred in 5% of patients

ADVERSE REACTIONS OCCURRING IN PATIENTS WITH ISM AT 24 WEEKS

Adverse Reaction[a,b]	AYVAKIT (25 mg once daily) + BSC n=141, %	Placebo + BSC n=71, %
Eye edema[c]	13	7
Dizziness[d]	13	10
Peripheral edema[d]	12	6
Flushing[d]	11	4
Respiratory tract infection[e]	8	1
Face edema	7	1
Rash[d]	6	4
Liver transaminase increased[d]	6	3
Insomnia	6	3
Hematoma[f]	6	1
Blood alkaline phosphatase increased	6	1
Hemorrhage[g]	5	3

[a]Adverse reactions that occurred in ≥5% of AYVAKIT-treated patients and ≥2% more than placebo-treated patients.
[b]Per National Cancer Institute Common Terminology Criteria for Adverse Events (CTCAE) version 5.0.
[c]Eye edema includes periorbital edema, eye edema, swelling of eyelid, orbital edema, eye swelling, eyelid edema, and eyelid ptosis.
[d]Term includes several similar terms.
[e]Respiratory tract infection includes pneumonia, upper respiratory tract infection, bronchitis, and respiratory tract infection.
[f]Hematoma includes contusion, hematoma, and pelvic hematoma.
[g]Hemorrhage includes epistaxis, gingival bleeding, hematochezia, rectal hemorrhage, and retinal hemorrhage.

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Long-term safety data through ~3 years[2*]

Safety profile was consistent with no new safety signals identified through 144 weeks

- Median follow-up was 35.3 months for all patients who received AYVAKIT (N=226)
- From baseline to 144 weeks, 99% of patients (n=224) taking AYVAKIT experienced any adverse event,[†] while 74% (n=168) experienced treatment-related adverse events (TRAEs)
  - **Adverse reactions** that occurred in ≥5% of AYVAKIT-treated patients through 144 weeks: peripheral edema, periorbital edema, headache, nausea, fatigue, diarrhea, alopecia
  - **Grade ≥3 adverse reactions** occurred in 46% (n=103) of patients, with Grade ≥3 TRAEs occurring in 6% (n=14)
  - **Serious adverse reactions** occurred in 20% (n=45)[†] of patients, with serious TRAEs occurring in 1% (n=3) of patients
  - **Permanent discontinuation** of AYVAKIT due to TRAEs occurred in 3% (n=7) of patients
  - **Serious TRAEs** included peripheral edema (1), gastric hemorrhage (1), and transient loss of vision (1). None of these events led to discontinuation

Most common TRAEs (≥5% of patients)	All patients with ISM who received AYVAKIT (N=226), n (%)
Peripheral edema	**29 (13)**
Periorbital edema	**22 (10)**
Headache	**21 (9)**
Nausea	**18 (8)**
Fatigue	**16 (7)**
Diarrhea	**14 (6)**
Alopecia	**13 (6)**

*Data cutoff was September 20, 2024. Long-term safety includes data from baseline through Week 144 of the PIONEER clinical trial, representing patients with varying lengths of treatment exposure and dosages. The long-term analysis set assessed safety of patients with ISM who received all administered doses of AYVAKIT, ranging from 25 mg to 50 mg QD + BSC in 226 patients through Week 96. Includes patients who received only 25 mg in Part 1. The FDA-approved dose is 25 mg once daily for adult patients with ISM.
[†] One death (Grade 5 serious AE) occurred during the study and was considered by the investigator to be unrelated to treatment; the patient had a medical history of anaphylaxis and atrial fibrillation, and the event was assessed as due to anaphylaxis in the context of atrial fibrillation.

No events of intracranial hemorrhage occurred in patients with ISM who received AYVAKIT

No new safety signals were identified with longer term exposure within the long-term follow-up of PIONEER Part 3

AE=adverse event.

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AYVAKIT has been studied in more than a thousand patients across 3 indications[2,20]

>1200
patients have received **AYVAKIT** across clinical trials globally for US-approved indications*

>3300
patients have been treated with **AYVAKIT** globally*

>2100
US healthcare providers have **prescribed AYVAKIT across US-approved indications***

*Data cutoff: June 2025.

See the story behind the numbers

Scan the code to watch the AYVAKIT Patient Story Series

"Deciding to take AYVAKIT is the best decision I could have made with my doctor." —Gloria, a real patient living with ISM

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One pill, once daily, start strong with AYVAKIT[1]

The recommended dosage for patients with ISM is 25 mg, orally, daily

AYVAKIT SHOULD BE TAKEN:

- **One 25-mg tablet orally**
- **On an empty stomach, at least 1 hour before or at least 2 hours after a meal**
- **Once a day**

A modified starting dosage of AYVAKIT is recommended for patients with severe hepatic impairment (Child-Pugh Class C): 25 mg orally every other day.[1]

Avoid concomitant use of AYVAKIT with strong or moderate CYP3A inhibitors or inducers. If contraception requires estrogen, limit ethinyl estradiol to ≤20 mcg unless a higher dose is necessary.[1]

Clear from the start: It is important to set the right expectations with patients who are beginning AYVAKIT treatment and check in along the way

TAKEN CONSISTENTLY

AYVAKIT is usually taken **1 time each day, not on an as-needed basis**[1]

WORKS DIFFERENTLY

AYVAKIT targets KIT D816V, blocking the proliferation of abnormal hyperactive mast cells—the source of ISM symptoms[1,5-7]

EFFICACY OVER TIME

AYVAKIT **may take time** to start working. In the clinical trial, patients saw a significant reduction in symptom burden **at 6 months** on treatment[1]

SELECT SAFETY INFORMATION

Cognitive Effects—Cognitive adverse reactions can occur in patients receiving AYVAKIT and occurred in 7.8% of patients with ISM who received AYVAKIT + best supportive care (BSC) versus 7.0% of patients who received placebo + BSC; <1% were Grade 3. Depending on the severity, withhold AYVAKIT and then resume at the same dose, or permanently discontinue AYVAKIT.

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Over 99% of commercial insurance plans and 99% of Medicare plans cover AYVAKIT*

Approximately 90% of patients with commercial insurance pay $0 per month*

With help from their insurance and with the YourBlueprint® Co-Pay Card† when they access AYVAKIT through our network of specialty pharmacies.

*Data on coverage and co-pay assistance are as of December 2024. Cost-sharing data are for those patients with commercial insurance.
†Up to an annual maximum benefit of $25,000. Terms and conditions apply.

YourBlueprint® provides dedicated, personalized support to help your patients from Day 1

YourBlueprint is a patient support program designed with your patients in mind. YourBlueprint provides a variety of support to eligible patients throughout many aspects along the treatment journey:

- Co-pay Support
- Coverage Interruption
- QuickStart
- Patient Assistance Program
- Case Managers who can also help your patients through nonclinical aspects of therapy by providing 1:1 support calls and patient education resources

AYVAKIT will require a prior authorization with the enrollment form

Most denials for AYVAKIT are due to missing or incomplete information. When working on insurance coverage approval for AYVAKIT, YourBlueprint and our network of specialty pharmacies can help support your patient through the process of managing a prior authorization requirement.

AYVAKIT prior authorization requirements with the enrollment form:

- Diagnosis codes
- Lab results (platelet counts, per label)
- Clinical notes with SM subtype

For the best experience, enroll your patients in YourBlueprint to support their experience and access to our programs.

To see how we can help:

Call: **1-888-BLUEPRINT (1-888-258-7768)**
Monday-Friday, 8 AM-8 PM Eastern Time (ET)

Email: **info@yourblueprint.com**

Fax: **1-866-370-3082**

Visit: **www.YourBlueprint.com/HCP**

Please see Important Safety Information on page 17 and click here to see the full Prescribing Information for AYVAKIT.

Important Safety Information

INDICATION

AYVAKIT® (avapritinib) is indicated for the treatment of adult patients with indolent systemic mastocytosis (ISM).

Limitations of Use: AYVAKIT is not recommended for the treatment of patients with ISM with platelet counts of <50 x 10^9/L.

IMPORTANT SAFETY INFORMATION

Cognitive Effects—Cognitive adverse reactions can occur in patients receiving AYVAKIT and occurred in 7.8% of patients with ISM who received AYVAKIT + best supportive care (BSC) versus 7.0% of patients who received placebo + BSC; <1% were Grade 3. Depending on the severity, withhold AYVAKIT and then resume at the same dose, or permanently discontinue AYVAKIT.

Photosensitivity—AYVAKIT may cause photosensitivity reactions. In all patients treated with AYVAKIT in clinical trials (n=1049), photosensitivity reactions occurred in 2.5% of patients. Advise patients to limit direct ultraviolet exposure during treatment with AYVAKIT and for one week after discontinuation of treatment.

Embryo-Fetal Toxicity—AYVAKIT can cause fetal harm when administered to a pregnant woman. Advise pregnant women of the potential risk to a fetus. Advise females and males of reproductive potential to use an effective contraception during treatment with AYVAKIT and for 6 weeks after the final dose. Advise women not to breastfeed during treatment with AYVAKIT and for 2 weeks following the final dose.

Adverse Reactions—The most common adverse reactions (≥10%) in patients with ISM were eye edema, dizziness, peripheral edema, and flushing.

Drug Interactions—Avoid coadministration of AYVAKIT with strong or moderate CYP3A inhibitors or inducers. If contraception requires estrogen, limit ethinyl estradiol to ≤20 mcg unless a higher dose is necessary.

To report suspected adverse reactions, contact Blueprint Medicines Corporation at 1-888-258-7768 or the FDA at 1-800-FDA-1088 or visit www.fda.gov/medwatch.

Please click here to see the full Prescribing Information for AYVAKIT.

AYVAKIT® avapritinib | 25mg tablets

STUDIED FOR 10 YEARS[2,20]

AYVAKIT® avapritinib | 25mg tablets

Go beyond ISM symptom management with AYVAKIT, the only FDA-approved therapy for ISM[1]

ISM may have a significant impact on patients' lives

ISM causes an overproduction of abnormal, hyperactive mast cells, resulting in symptoms that disrupt patients' lives and may have serious health implications[2-4,14,15]

Target the source of ISM

AYVAKIT works by targeting the mutation that causes abnormal, hyperactive mast cells[1,5-7]

Efficacy data up to ~3 years

AYVAKIT achieved all endpoints in the PIONEER study, including reductions in ISM symptoms and measures of mast cell burden at 24 weeks; 5-year patient follow-up is ongoing, with data currently available through ~3 years[1,2,17]

Consistent, long-term safety profile

AYVAKIT was generally well-tolerated in PIONEER—with a consistent safety profile through 144 weeks[1,2]

For more information about AYVAKIT for ISM, visit AYVAKITHCP.com/ISM.

SELECT SAFETY INFORMATION

Photosensitivity—AYVAKIT may cause photosensitivity reactions. In all patients treated with AYVAKIT in clinical trials (n=1049), photosensitivity reactions occurred in 2.5% of patients. Advise patients to limit direct ultraviolet exposure during treatment with AYVAKIT and for one week after discontinuation of treatment.

Please see Important Safety Information on page 17 and click here to see the full Prescribing Information for AYVAKIT.

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