



The ONLY FDA-approved treatment that has long-term clinical experience in adults with indolent systemic mastocytosis<sup>1,2</sup>

~3.5 YEARS OF EFFICACY AND SAFETY DATA<sup>1,2</sup>



Patient portrayal

FDA=Food and Drug Administration.

**INDICATION**

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

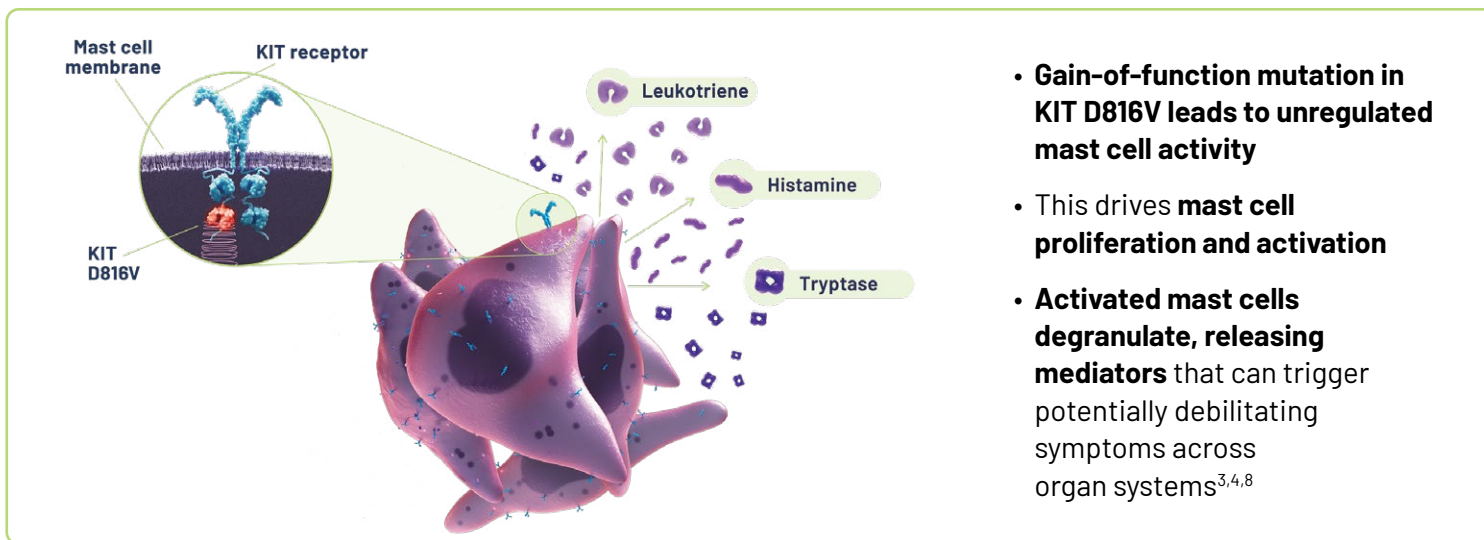
Limitations of Use: AYVAKIT is not recommended for the treatment of patients with ISM with platelet counts of <math> < 50 \times 10^9/L </math>.

Please see Important Safety Information on page 17 and [click here](#) to see the full Prescribing Information for AYVAKIT.

- BURDEN OF ISM
- ABOUT AYVAKIT
- PIONEER STUDY DESIGN
- EFFICACY DATA
- TSS AND MC-QoL THROUGH ~3.5 YEARS
- SAFETY THROUGH ~4 YEARS
- DOSING & ADMINISTRATION
- EXPERIENCE
- ACCESS & SUPPORT
- IMPORTANT SAFETY INFORMATION

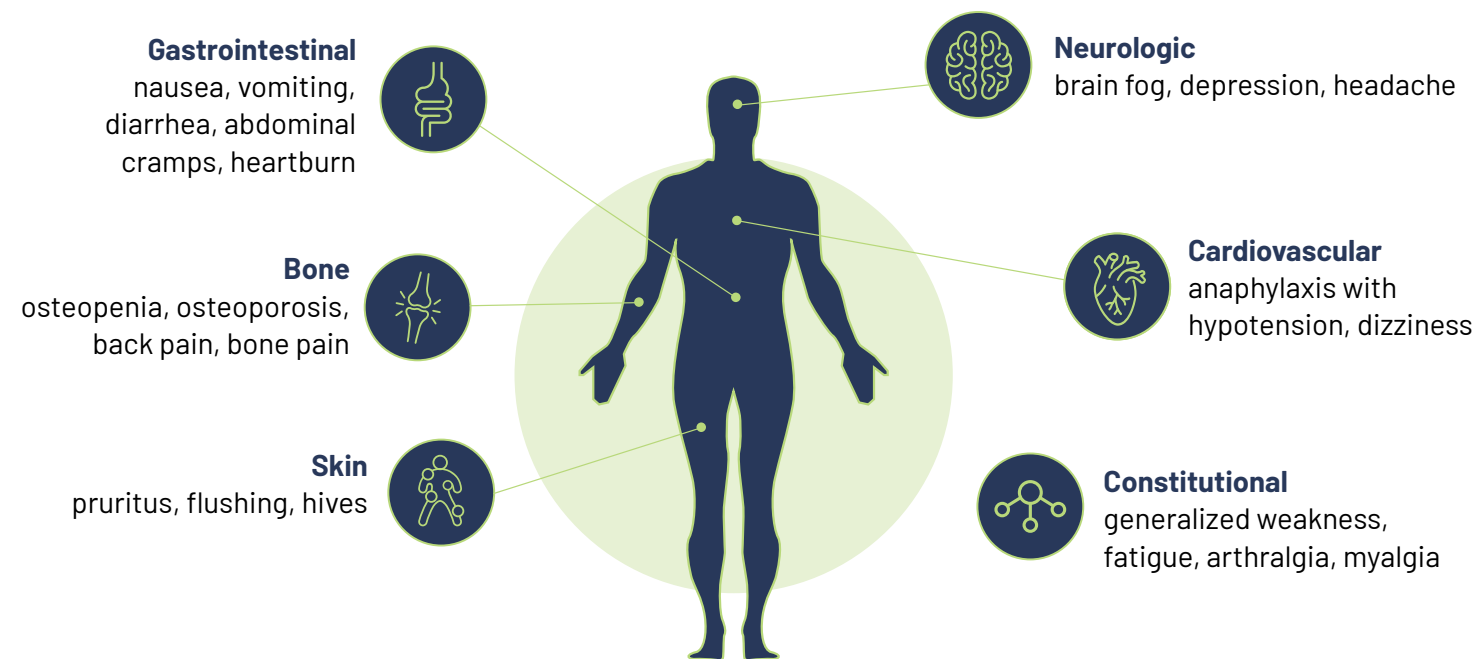
## ISM is a rare, chronic, systemic disease driven by the overproduction of mutated and hyperactive mast cells<sup>3,4</sup>

In ~95% of patients with ISM, the KIT D816V mutation drives abnormal mast cell activity<sup>3-8</sup>



- Gain-of-function mutation in KIT D816V leads to unregulated mast cell activity
- This drives **mast cell proliferation and activation**
- Activated mast cells **degranulate, releasing mediators** that can trigger potentially debilitating symptoms across organ systems<sup>3,4,8</sup>

## Symptoms can flare unexpectedly, persist across multiple organ systems, and worsen over time<sup>3,4,8,9</sup>



These symptoms represent the clinical spectrum of ISM but are not comprehensive. Symptoms may vary in individual patients.<sup>2-4</sup>

ISM=indolent systemic mastocytosis; KIT=KIT proto-oncogene, receptor tyrosine kinase.

## In patients with ISM, symptoms may appear controlled, yet significant disease burden can persist<sup>2-4</sup>

Patients often adapt their daily lives to cope with the symptom burden of ISM,\* including<sup>10</sup>:

- Increased polypharmacy<sup>2</sup>
- Considerable family and caregiver burden<sup>11</sup>
- Multiple HCP visits per year<sup>2</sup>

In a Blueprint Medicines-sponsored survey (n=32)<sup>2†</sup>

- 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<sup>10,12</sup>

- Symptom frequency and severity often worsen over time<sup>9</sup>:** 55% of patients with ISM experience **more frequent symptoms** and 47.5% report **increased severity** since diagnosis<sup>†</sup>
- Increased risk of bone complications<sup>13</sup>:** **Osteopenia/osteoporosis was present in 68% of patients with ISM** vs 28% of controls<sup>§</sup>
- Higher risk of anaphylaxis<sup>13</sup>:** 63% of patients with ISM had anaphylaxis (and 89% had allergic reactions)<sup>§</sup>

**While ISM management often involves multiple symptom-directed therapies (SDTs), they may not be enough<sup>2-4</sup>**



Assess disease control in your patients with ISM: Scan or [click here](#) for the **Mastocytosis Control Test**, 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.<sup>10</sup>

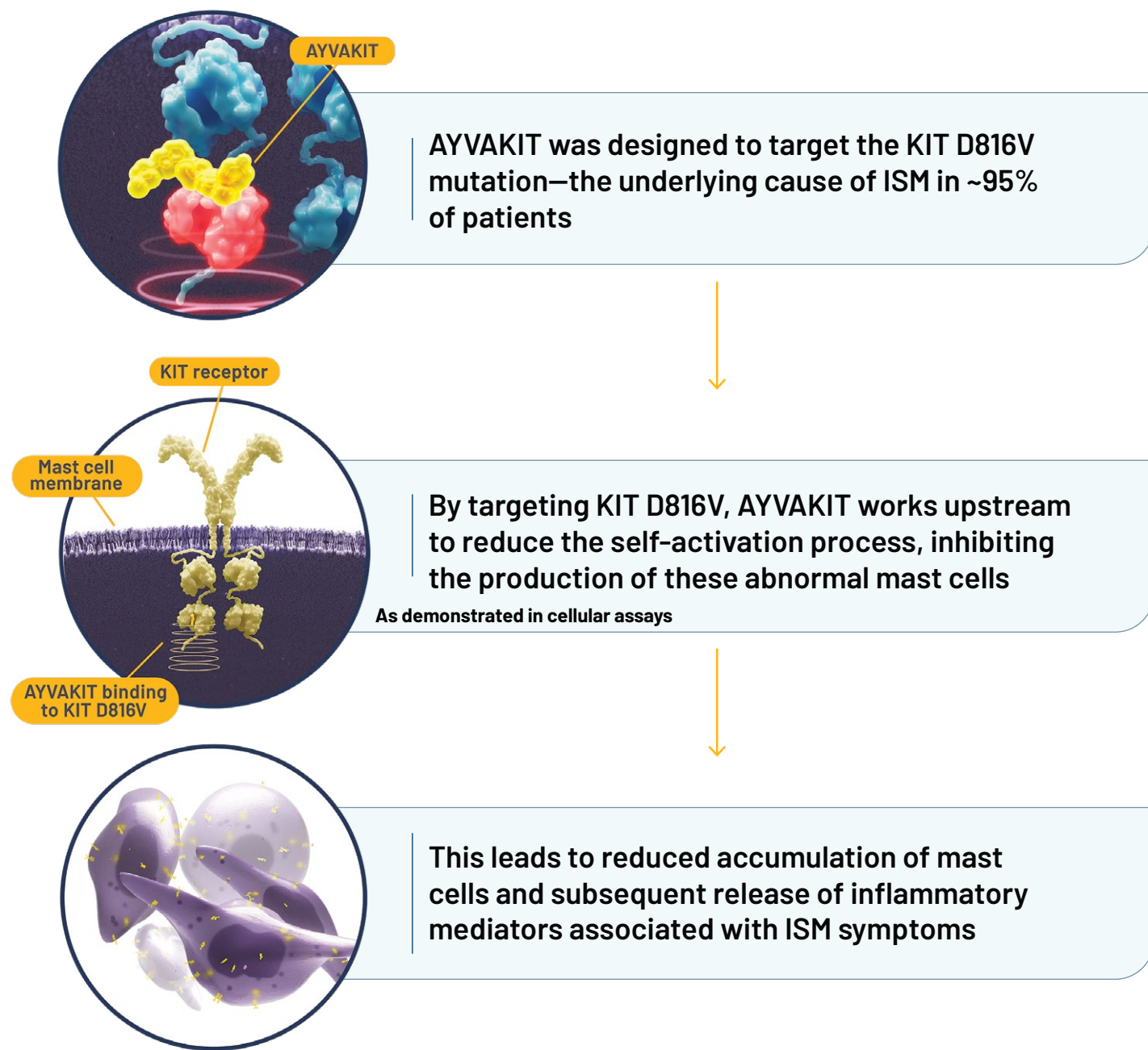
<sup>†</sup>In the Blueprint Medicines-sponsored TouchStone SM Patient Survey, US adults with a self-reported SM diagnosis (N=56) completed an online survey of 100 items. An analysis was conducted in patients with ISM (n=37), including 32 patients with moderate to severe ISM (defined as an ISM-SAF TSS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

<sup>‡</sup>Survey data were collected from adults with ISM (N=40) 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.<sup>2</sup>

<sup>§</sup>In a retrospective, propensity score-matched cohort study using Mayo Clinic electronic health record data (2005-2022), 203 adult patients with ISM were matched 1:10 with 2030 patients without ISM based on demographic and clinical characteristics.<sup>13</sup>

AdvSM=Advanced systemic mastocytosis; HCP=healthcare provider; ISM-SAF=Indolent Systemic Mastocytosis-Symptom Assessment Form; SM=systemic mastocytosis; TSS=total symptom score; WHO=World Health Organization.

## AYVAKIT targets the primary driver of ISM<sup>1,3-8,14</sup>



When treating ISM, consider adding AYVAKIT to target the KIT D816V mutation, the underlying driver of disease<sup>1,5-7</sup>



Watch the full MOA video [here](#) or scan the QR code

MOA=mechanism of action.

### SELECT SAFETY INFORMATION

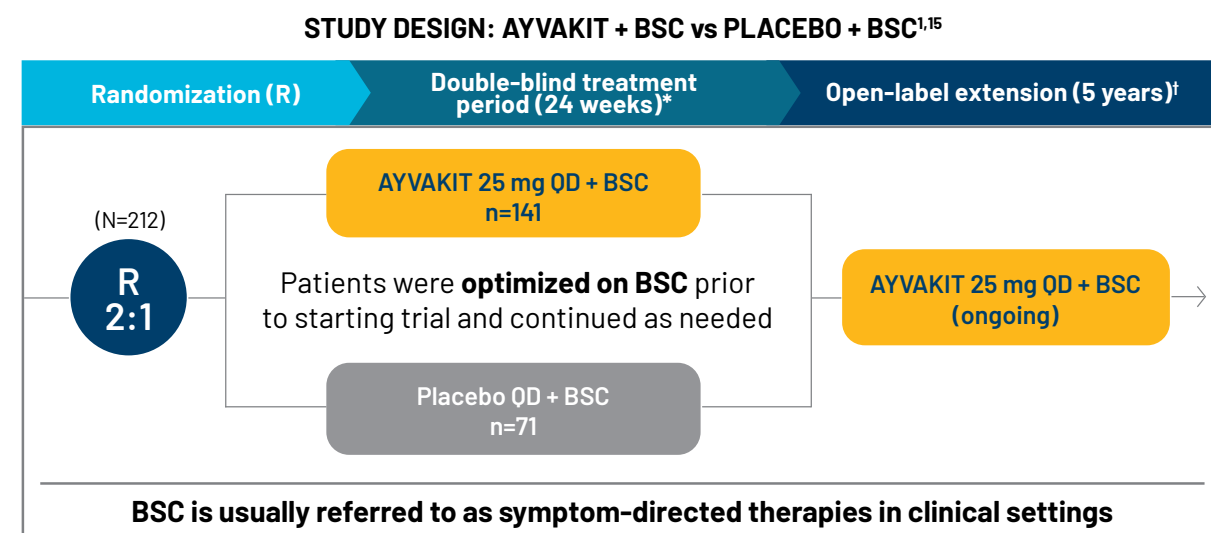
**Cognitive Effects**—Cognitive adverse reactions can occur in patients receiving AYVAKIT and occurred in 7.8% of patients with ISM who received AYVAKIT + best supportive care (BSC) versus 7.0% of patients who received placebo + BSC; <1% were Grade 3. Depending on the severity, withhold AYVAKIT and then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

**4** Please see Important Safety Information on page 17 and [click here](#) to see the full Prescribing Information for AYVAKIT.

## PIONEER measured symptom relief and change in mast cell burden<sup>1,15</sup>

**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, with both arms receiving concomitant BSC<sup>1,15</sup>**

**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<sup>15</sup>



### Symptom measurement

**Primary endpoint:** Absolute mean change in ISM Symptom Assessment Form Total Symptom Score (ISM-SAF TSS) compared with placebo + BSC from baseline to Week 24<sup>1</sup>

• **ISM-SAF:** A validated PRO assessment unique to PIONEER<sup>2,15</sup>

See page 6 for ISM-SAF overview

**Exploratory endpoints:** Mean change in ISM-SAF individual symptom scores; mean change in most severe symptom score at Week 24<sup>15</sup>

### Mast cell burden measurement

Select key secondary endpoints compared with placebo + BSC at Week 24<sup>1,15</sup>

Proportion of patients achieving:

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

\*Data cutoff was June 23, 2022.<sup>15</sup>

<sup>†</sup>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.<sup>15</sup>

<sup>‡</sup>In peripheral blood.<sup>1</sup>

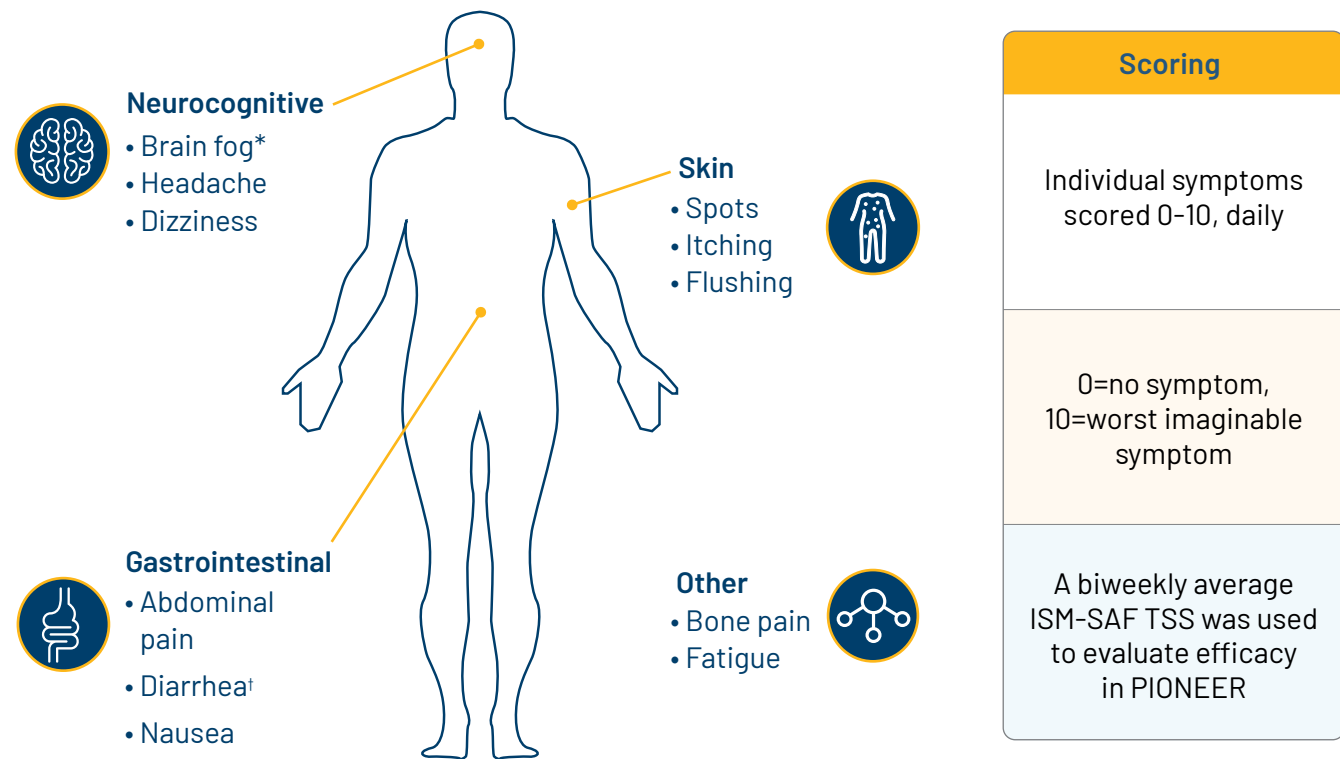
BSC=best supportive care; PRO=patient-reported outcome; QD=every day; VAF=variant allele fraction.

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avapritinib | 25mg tablets

## ISM-SAF: A validated symptom assessment tool designed for the PIONEER trial to measure symptom burden in ISM<sup>1,15,16</sup>

ISM-SAF is a 12-item patient questionnaire assessing the severity of 11 symptoms (scores ranging from 0-10) to calculate a TSS (0-110) with higher scores indicating greater symptom severity.<sup>1,15,16</sup>



**Developed by Blueprint Medicines with input from experts, patients, and regulatory authorities, to help capture the burden of ISM symptoms<sup>15,16</sup>**

\*Memory impairment and/or difficulty focusing/processing.<sup>1,16</sup>

<sup>†</sup>Diarrhea frequency is assessed as an additional item in the ISM-SAF but is not included in the TSS.<sup>16</sup>

### 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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**Please see Important Safety Information on page 17 and [click here](#) to see the full Prescribing Information for AYVAKIT.**

## PIONEER reflects the heterogeneous population of patients living with ISM<sup>15</sup>

**Patients were optimized on a range of BSC prior to trial enrollment<sup>15</sup>**

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

### Select baseline demographics and patient characteristics<sup>1,15</sup>

Patient demographic	AYVAKIT + BSC (n=141)	Placebo + BSC (n=71)
<b>Median age (range)</b>	50 years (18-77)	54 years (26-79)
<b>Sex (% female)</b>	71%	76%
<b>Mean ISM-SAF TSS (SD)</b>	50.2 (19.1)	52.4 (19.8)
<b>Polypharmacy burden</b> Median no. of BSCs (range)	3 (0-11)	4 (1-8)
<b>Varied tryptase levels</b> Median serum tryptase, ng/mL (range)	38.4 (3.6-256.0)	43.7 (5.7-501.6)
<b>Mast cell burden</b> Mast cell aggregates present	75%	80%

▶ **~80% of patients in PIONEER (n=167/212) reported skin involvement at baseline<sup>17</sup>**

▶ **93% of patients in the AYVAKIT + BSC arm (n=131/141) were KIT positive, with 7% KIT undetectable<sup>1,15</sup>**

SD=standard deviation.

**Please see Important Safety Information on page 17 and [click here](#) to see the full Prescribing Information for AYVAKIT.**



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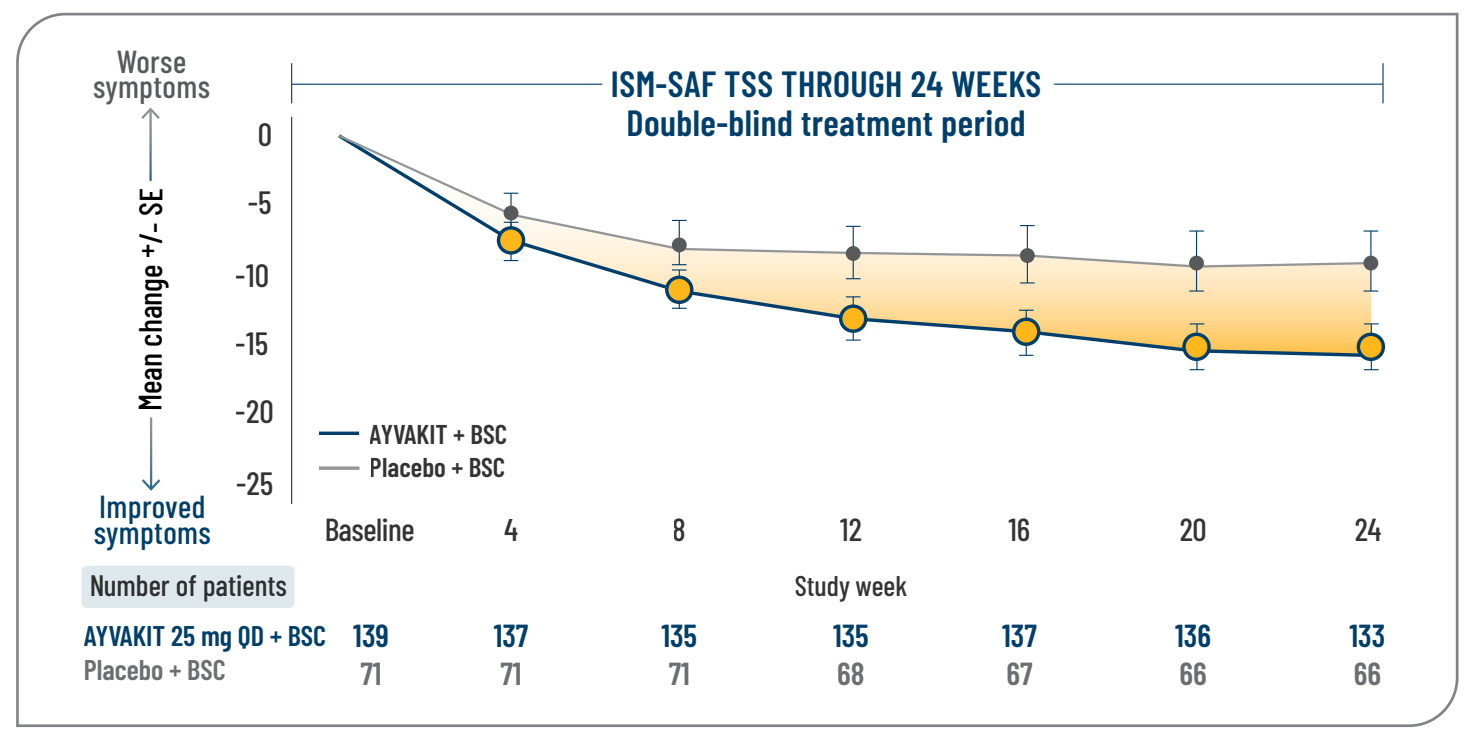
# AYVAKIT significantly reduced symptom burden vs placebo at 24 weeks<sup>1,15</sup>

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

This analysis was done in the ITT population. Markov chain Monte Carlo simulation was used to impute the missing values at baseline or Week 24.



This analysis was done in the ITT population. Patients with use of high-dose steroids (3 patients treated with AYVAKIT and 1 with placebo) 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:** 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.

CI=confidence interval; ITT=intention to treat; SE=standard error.

## SELECT SAFETY INFORMATION

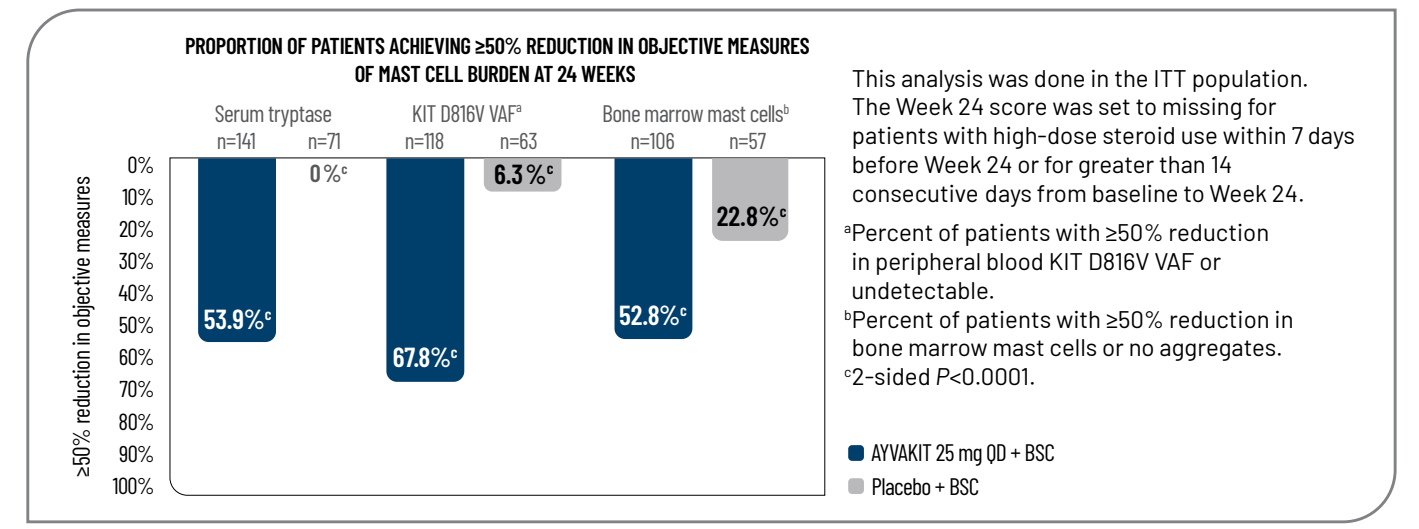
**Embryo-Fetal Toxicity**—AYVAKIT can cause fetal harm when administered to a pregnant woman. Advise pregnant women of the potential risk to a fetus. Advise females and males of reproductive potential to use an effective contraception during treatment with AYVAKIT and for 6 weeks after the final dose.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

**8** Please see Important Safety Information on page 17 and [click here to see the full Prescribing Information for AYVAKIT.](#)

# AYVAKIT significantly reduced mast cell burden, and reductions in individual symptom scores were observed vs placebo at 24 weeks<sup>1,2</sup>

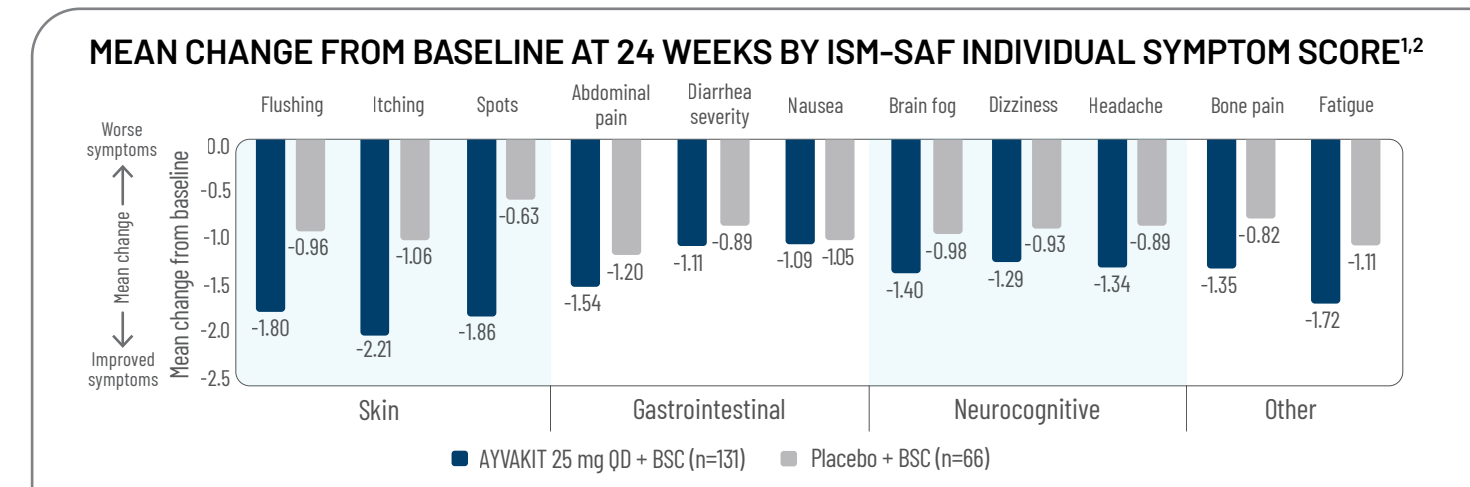
KEY SECONDARY ENDPOINTS

AYVAKIT + BSC went beyond symptom management and showed significant reductions in measures of mast cell burden vs placebo + BSC<sup>1</sup>



EXPLORATORY ENDPOINT

Decreases in symptom severity were observed across skin, gastrointestinal, and neurocognitive symptoms<sup>15</sup>



This analysis was done in the ITT population. Scores were considered missing if more than 7 days were missing between baseline and Week 2, or if more than 7 days were missing from the 14-day period used to calculate the Week 24 score (resulting in a missing Week 24 score).

**LIMITATIONS:** Individual components in a descriptive exploratory analysis were prespecified, nonranked endpoints that were 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 at 24 weeks<sup>15\*</sup>**

\*Most severe symptom defined as the symptom with the highest score at baseline.

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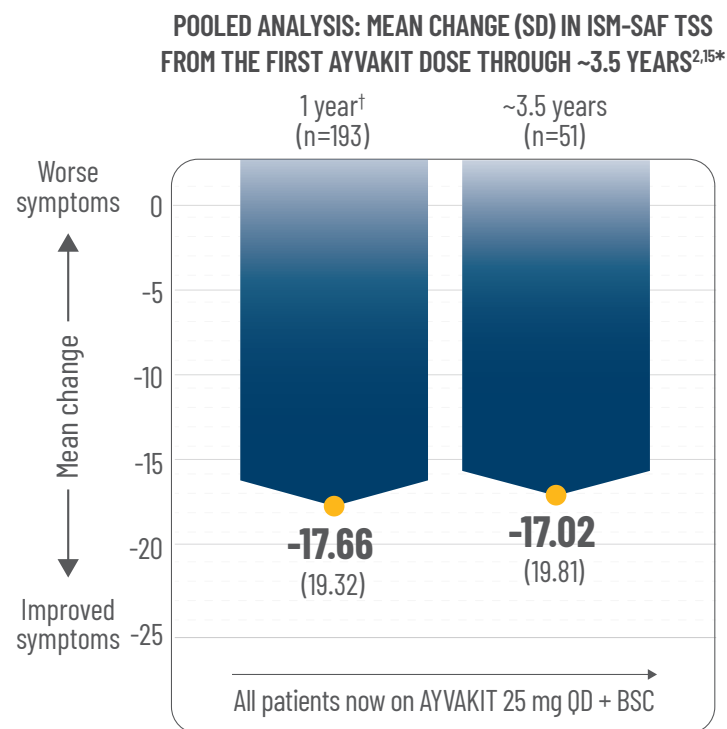


## ISM symptom severity changes observed through ~3.5 years<sup>2,15</sup>

EXPLORATORY ENDPOINT

### Decreases in ISM-SAF TSS were observed through ~3.5 years in patients treated with AYVAKIT<sup>2,15</sup>

- At 6 months, patients in the placebo + BSC arm could choose to receive AYVAKIT 25 mg QD + BSC in an open-label extension trial for up to 5 years<sup>15</sup>
- 93% of patients in the placebo + BSC arm continued to the open-label extension and began receiving AYVAKIT<sup>15</sup>



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 Year 1 and ~3.5 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 OLE, 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 17, 2025; median exposure, 41.0 months.<sup>2</sup>

†Data cutoff: June 23, 2022; prespecified endpoint: median exposure at 48 weeks.<sup>15</sup>  
OLE=open-label extension.

### 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](http://www.fda.gov/medwatch).

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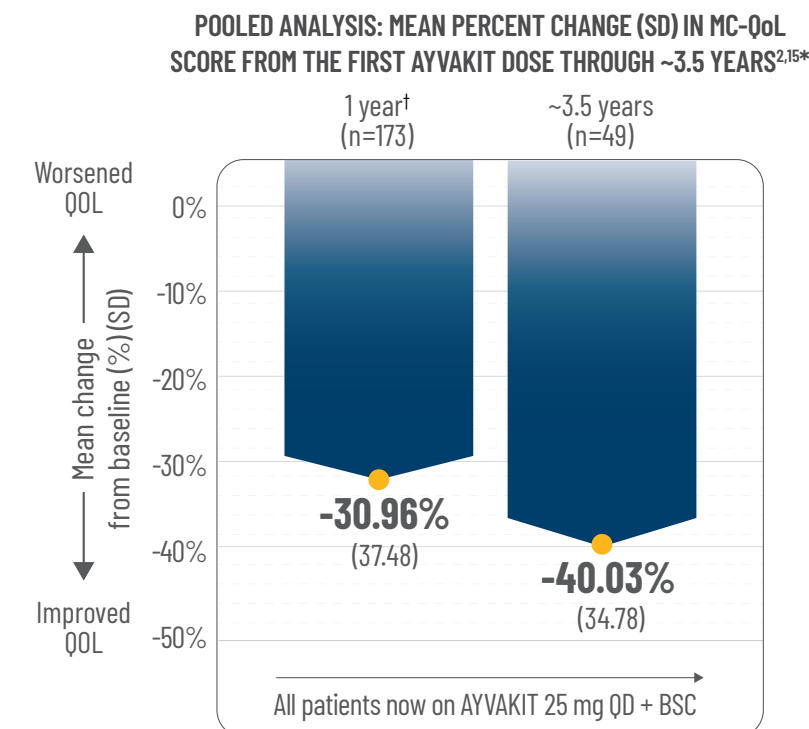
Please see Important Safety Information on page 17 and [click here](#) to see the full Prescribing Information for AYVAKIT.

## Mastocytosis QoL mean score changes observed through ~3.5 years<sup>2,15</sup>

EXPLORATORY ENDPOINT

At 24 weeks, the mean percent changes in MC-QoL scores for patients receiving AYVAKIT 25 mg QD + BSC and placebo + BSC were -34% (SD, -40% to -29%) and -18% (SD, -25% to -11%), respectively.<sup>1,15</sup>

### Mean percent decreases in MC-QoL scores were observed through ~3.5 years in patients treated with AYVAKIT + BSC<sup>2,15</sup>



The **MC-QoL Questionnaire**, a disease-specific tool for use in patients with ISM, was used to assess QoL at each study visit<sup>15</sup>:

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

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 Year 1 and ~3.5 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 OLE, 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.

MC-QoL=Mastocytosis Quality of Life Questionnaire; QoL=quality of life.

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## AYVAKIT was generally well tolerated in PIONEER through 24 weeks<sup>1</sup>

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

<sup>a</sup>Adverse reactions that occurred in ≥5% of AYVAKIT-treated patients and ≥2% more than placebo-treated patients.

<sup>b</sup>Per National Cancer Institute Common Terminology Criteria for Adverse Events (CTCAE) version 5.0.

<sup>c</sup>Eye edema includes periorbital edema, eye edema, swelling of eyelid, orbital edema, eye swelling, eyelid edema, and eyelid ptosis.

<sup>d</sup>Term includes several similar terms.

<sup>e</sup>Respiratory tract infection includes pneumonia, upper respiratory tract infection, bronchitis, and respiratory tract infection.

<sup>f</sup>Hematoma includes contusion, hematoma, and pelvic hematoma.

<sup>g</sup>Hemorrhage includes epistaxis, gingival bleeding, hematochezia, rectal hemorrhage, and retinal hemorrhage.

## Long-term safety data through ~4 years<sup>2\*</sup>

### Safety profile remained consistent, with no new safety signals identified through ~4 years

- Median follow-up was 46.5 months for all patients who initiated AYVAKIT 25 mg QD (N=226)
- Serious TRAEs occurred in 1% (n=3) of patients and included peripheral edema (1), gastric hemorrhage (1), and transient loss of vision (1). None of these events led to discontinuation

### All patients with ISM who initiated AYVAKIT 25 mg QD + BSC (N=226)

Safety overview		Most common TRAEs (≥5% of patients), n (%)	
<b>Any AEs, n (%)</b>	224 (99)	Peripheral edema	30 (13)
Any TRAEs, n (%)	168 (74)	Periorbital edema	23 (10)
<b>Grade ≥3 AEs, n (%)</b>	108 (48) <sup>a</sup>	Headache	22 (10)
Grade ≥3 TRAEs, n (%)	14 (6)	Nausea	18 (8)
<b>Serious AEs, n (%)</b>	53 (23)	Fatigue	17 (8)
Serious TRAEs, n (%)	3 (1)	Diarrhea	16 (7)
<b>TRAEs leading to discontinuation, n (%)</b>	6 (3)	Alopecia	13 (6)

<sup>a</sup>One death (Grade 5 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 reported in patients with ISM who received AYVAKIT<sup>2</sup>

\*Data cutoff was September 17, 2025. Long-term safety includes data from baseline through ~46 months 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 ~46 months. Includes patients who received only 25 mg in Part 1. The FDA-approved dose is 25 mg once daily for adult patients with ISM.<sup>1,2</sup>

AE=adverse event; TRAE=treatment-related adverse event.

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## One pill, once-daily dosing to help with ISM symptom reduction<sup>1,2</sup>



One 25-mg tablet orally<sup>1</sup>

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

Once a day

Image not to scale. Tablet appearance may vary. Hand shown for illustrative purposes only.

To help patients get the most out of AYVAKIT, it's important to set the right expectations and check in along the way.

**1x** Taken consistently, once daily, not on an as-needed basis<sup>1</sup>

Symptom improvement may take time. In the clinical trial, patients saw a significant reduction in symptom burden at 6 months<sup>1</sup>

For patients with severe hepatic impairment (Child-Pugh Class C), a modified starting dosage of 25 mg orally every other day is recommended. 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.<sup>1</sup>

### 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.

**14** Please see Important Safety Information on page 17 and [click here](#) to see the full Prescribing Information for AYVAKIT.

## AYVAKIT has been studied across 3 indications<sup>2,18</sup>



**Evidence**

>1000 patients have received AYVAKIT across clinical trials globally for US-approved indications<sup>2\*</sup>

**Experience**

>4300 patients treated with AYVAKIT globally in real-world practice<sup>2†</sup>

**Adoption**

>2600 US healthcare providers have prescribed AYVAKIT across US-approved indications<sup>2†</sup>

\*Data cutoff: June 2025.  
 †Data cutoff: March 2026.

### Hear from real patients about their experience

GLORIA

PATIENT > STORIES

Scan the code or click here to watch the AYVAKIT Patient Story Series

Gloria, a real patient living with ISM<sup>‡</sup>

<sup>‡</sup>Gloria was compensated by Blueprint Medicines for sharing her story.

Please see Important Safety Information on page 17 and [click here](#) to see the full Prescribing Information for AYVAKIT.



## AYVAKIT has broad national coverage and a personalized patient support program

### Broad national coverage

**98%** Over 98% of commercial insurance plans and 99% of Medicare plans cover AYVAKIT<sup>2\*</sup>

### Co-pay Assistance Program

**\$0** Approximately 90% of patients with commercial insurance paid \$0 per month with help from their insurance and with the YourBlueprint<sup>®</sup> Co-Pay Card<sup>†</sup> when they accessed AYVAKIT through our network of specialty pharmacies\*

### 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), SM subtype, and clinical notes.

**For the best experience, enroll your patients in YourBlueprint at the time of prescription to support access to our programs**

Click here or scan to see how we can help:



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

Email: [info@yourblueprint.com](mailto:info@yourblueprint.com) Fax: 1-866-370-3082

Visit: [www.YourBlueprint.com/HCP](http://www.YourBlueprint.com/HCP)

\*Data on coverage and co-pay assistance are as of February 2026. Cost-sharing data are for those patients with commercial insurance.

<sup>†</sup>Up to an annual maximum benefit of \$25,000. Terms and conditions apply.

## Important Safety Information

### INDICATION

AYVAKIT<sup>®</sup> (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<sup>9</sup>/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](http://www.fda.gov/medwatch).

**Please [click here](#) to see the full Prescribing Information for AYVAKIT.**



**AYVAKIT**<sup>®</sup>  
avapritinib | 25mg  
tablets

## Go beyond symptom management with AYVAKIT, the ONLY FDA-approved therapy for ISM<sup>1,3,4,8,14</sup>



### Target the source—the KIT D816V mutation<sup>1,5-7</sup>

AYVAKIT targets the underlying driver of disease in ISM, which causes abnormal, hyperactive mast cells.



### Reductions in symptom and mast cell burden<sup>1,2</sup>

AYVAKIT achieved all endpoints in the PIONEER study, including reductions in ISM symptoms and measures of mast cell burden at 24 weeks; patient follow-up is ongoing, with efficacy data currently available through ~3.5 years.



### Consistent, long-term safety profile<sup>1,2</sup>

AYVAKIT was generally well tolerated in PIONEER—with no new safety signals observed through ~4 years.

## 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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