UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of Report (Date of Earliest Event Reported): **March 16, 2020**

Blueprint Medicines Corporation

(Exact name of registrant as specified in its charter)

Delaware	**001-37359**	**26-3632015**
(State or other jurisdiction of incorporation)	(Commission File Number)	(I.R.S. Employer Identification No.)

45 Sidney Street **Cambridge, Massachusetts** (Address of principal executive offices)	**02139** (Zip Code)

Registrant's telephone number, including area code: **(617) 374-7580**

(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Securities registered pursuant to Section 12(b) of the Exchange Act:

Title of each class	Trading symbol(s)	Name of each exchange on which registered
Common stock, par value $0.001 per share	BPMC	Nasdaq Global Select Market

Item 7.01 Regulation FD Disclosure.

On March 16, 2020, Blueprint Medicines Corporation (the "Company") is hosting an investor conference call and webcast to report updated data from the dose-finding portion (part 1) of its ongoing Phase 2 PIONEER clinical trial in patients with indolent systemic mastocytosis. A copy of the presentation from the investor conference call and webcast is furnished as Exhibit 99.1 to this Current Report on Form 8-K.

The information in Item 7.01 of this Current Report on Form 8-K, including Exhibit 99.1 attached hereto, is intended to be furnished and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 8.01 Other Events.

On March 16, 2020, the Company issued a press release reporting updated data from part 1 of the PIONEER clinical trial in patients with indolent systemic mastocytosis. A copy of the press release is filed herewith as Exhibit 99.2 to this Current Report on Form 8-K and incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits.

Exhibit No.	Description
99.1	Corporate slide presentation of Blueprint Medicines Corporation dated March 16, 2020
99.2	Press release issued by Blueprint Medicines Corporation on March 16, 2020
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

BLUEPRINT MEDICINES CORPORATION

Date: March 16, 2020

By: /s/ Jeffrey W. Albers
Jeffrey W. Albers
Chief Executive Officer

PIONEER clinical trial in indolent systemic mastocytosis

PART 1 DATA & PROGRAM UPDATE

MARCH 16, 2020

blueprint MEDICINES

Forward-looking statements

This presentation contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended. The words "aim," "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. In this presentation, forward-looking statements include, without limitation, statements regarding the plans, strategies, timelines and expectations of Blueprint Medicines Corporation (the "Company") for the preclinical and clinical development and commercialization of AYVAKIT™ (avapritinib), pralsetinib, fisogatinib, and BLU-263, including the timing, design, implementation, enrollment, plans and announcement of results regarding Blueprint Medicines' ongoing and planned clinical trials for avapritinib in SM; plans and timelines for submitting marketing applications for avapritinib and pralsetinib and, if approved, commercializing avapritinib for additional indications or pralsetinib; the potential benefits of any of Company's current or future approved drugs or drug candidates in treating patients; plans, timelines and expectations for initiating patient screening in Part 2 of the PIONEER trial and for completing enrollment in Part 2 of the PIONEER trial; and Blueprint Medicines' strategy, goals and anticipated milestones, business plans and focus.

The Company has based these forward-looking statements on management's current expectations, assumptions, estimates and projections. While the Company believes these expectations, assumptions, estimates and projections are reasonable, such forward-looking statements are only predictions and involve known and unknown risks, uncertainties and other important factors, many of which are beyond the Company's control and may cause actual results, performance or achievements to differ materially from those expressed or implied by any forward-looking statements. These risks and uncertainties include, without limitation, risks and uncertainties related to the delay of any current or planned clinical trials or the development of the Company's drug candidates, including avapritinib for additional indications, pralsetinib, fisogatinib and BLU-263, or the licensed drug candidate; the Company's advancement of multiple early-stage efforts; the Company's ability to successfully demonstrate the efficacy and safety of its drug candidates and gain approval of its drug candidates on a timely basis, if at all; the preclinical and clinical results for the Company's drug candidates, which may not support further development of such drug candidates; actions or decisions of regulatory agencies or authorities, which may affect the initiation, timing and progress of clinical trials or marketing applications; the Company's ability to obtain, maintain and enforce patent and other intellectual property protection for any drug candidates it is developing or AYVAKIT; the Company' ability and plans for maintaining a commercial infrastructure, and successfully launching, marketing and selling its current or future approved drugs; the Company's ability to successfully expand the approved indications for AYVAKIT or obtain marketing approval for AYVAKIT in additional geographies; the Company's ability to develop and commercialize companion diagnostic tests for any of Company's current or future approved drugs or drug candidate; and the success of the Company's current and future collaborations, partnerships and licenses.

These and other risks and uncertainties are described in greater detail under "Risk Factors" in the Company's filings with the Securities and Exchange Commission ("SEC"), including its most recent Annual Report on Form 10-K, as supplemented by its most recent Quarterly Report on Form 10-Q and any other filings it has made or may make with the SEC in the future. The Company cannot guarantee future results, outcomes, levels of activity, performance, developments, or achievements, and there can be no assurance that its expectations, intentions, anticipations, beliefs, or projections will result or be achieved or accomplished. The forward-looking statements in this presentation are made only as of the date hereof, and except as required by law, the Company undertakes no obligation to update any forward-looking statements contained in this presentation as a result of new information, future events or otherwise.

This presentation also contains estimates, projections and other statistical data made by independent parties and by the Company relating to market size and growth and other data about the Company's industry. These data involve a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions and estimates of the Company's future performance and the future performance of the markets in which the Company operates are necessarily subject to a high degree of uncertainty and risk.

Blueprint Medicines call participants

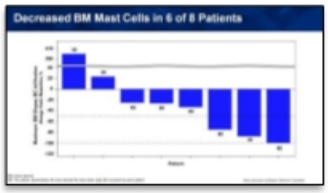
Introduction	**Kristin Hodous**, Sr. Manager, Investor Relations
Overview of avapritinib program in SM	**Jeff Albers**, Chief Executive Officer
Updated data from Part 1 of the PIONEER trial in indolent SM	**Brenton Mar, MD, PhD,** Senior Medical Director
Registration strategy in indolent SM	**Andy Boral, MD, PhD,** Chief Medical Officer
Opportunity to deliver therapeutic value to patients with SM	**Christy Rossi**, Chief Commercial Officer
Q&A	**All**

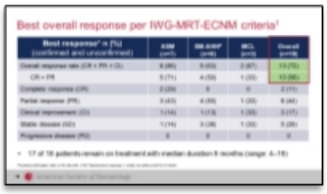
Target profile of a transformative therapy for systemic mastocytosis

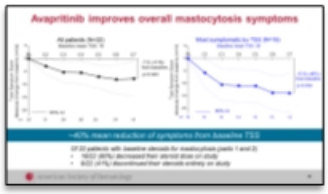
- Potent inhibitor of KIT D816V, the SM disease driver
- Reduces mast cell burden
- Improves disease symptoms to deliver meaningful clinical benefit
- Improves patient-reported quality of life
- Favorable safety profile supporting chronic treatment

Robust body of data for avapritinib establishes the foundation in SM

ASH 2016[1] | ASH 2017[2] | ASH 2018[3] | EHA 2019[4]



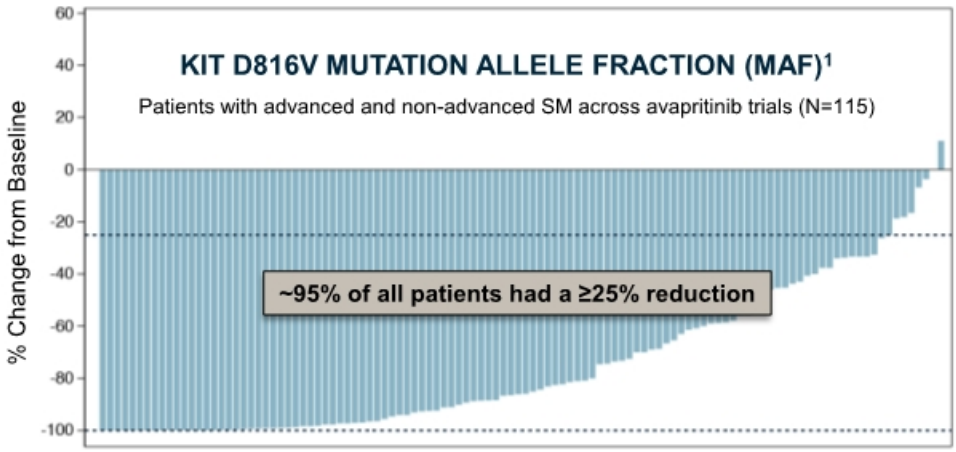




Selected for plenary scientific session

[1] Presented at American Society of Hematology (ASH) 2016 Annual Meeting. Data cutoff: November 11, 2016. [2] Presented at ASH 2017 Annual Meeting. Data cutoff: October 4, 2017. [3] Presented at ASH 2018 Annual Meeting. Data cutoff: September 30, 2018. [4] European Hematology Association 2019 Congress. Data cutoff: January 2, 2019.

Avapritinib is the only highly potent inhibitor of KIT D816V, the common disease driver across systemic mastocytosis

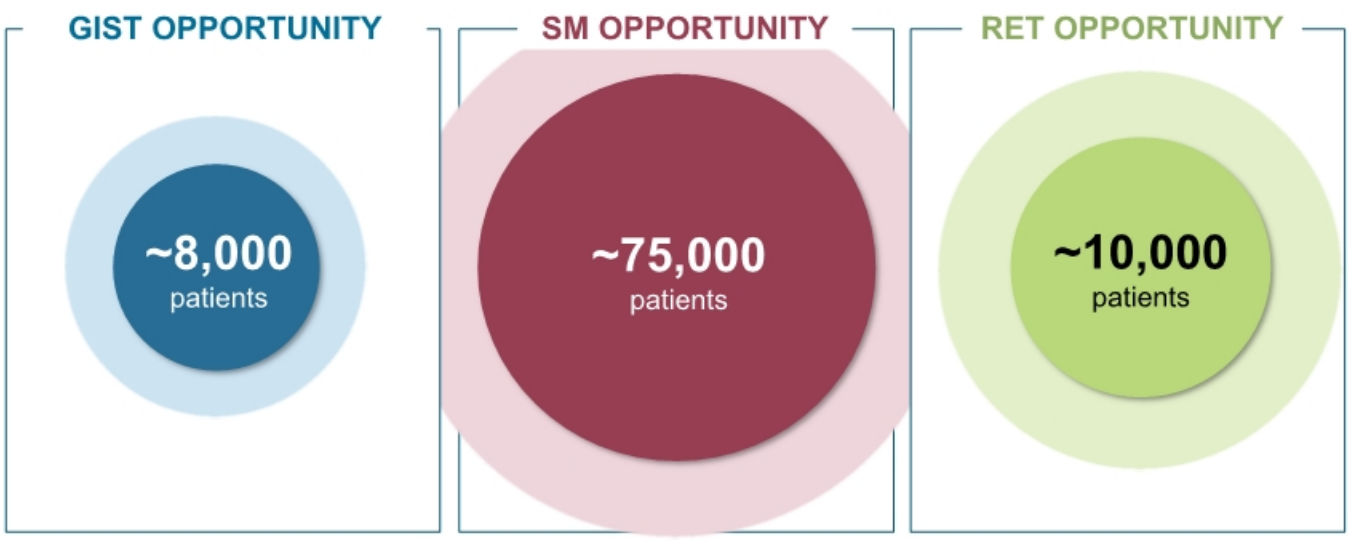


≥25% reduction in KIT D816V MAF is correlated with improved overall survival in advanced SM[2]

[1] Analysis of trial data from EXPLORER and PATHFINDER (data cutoff: August 30, 2019) and PIONEER (data cutoff: December 27, 2019).
[2] Jawhar, et al. Response and progression on midostaurin in advanced systemic mastocytosis: KIT D816V and other molecular markers. Blood, 2017.

Systemic mastocytosis represents a substantial portfolio value driver

PORTFOLIO OPPORTUNITIES ACROSS MAJOR MARKETS



Figures are illustrative. Approximate number of patients in major markets (US, EU5 and Japan) based on estimated prevalence for patients with advanced, indolent and smoldering SM and patients with ≥1L MTC with a RET mutation and estimated incidence for patients with ≥3L GIST and PDGFRA mutant GIST and patients with ≥1L NSCLC with a RET fusion.

Multiple significant clinical data disclosures planned in 2020

Q1 2020

- Top-line ARROW data for pralsetinib in RET+ NSCLC
- Updated PIONEER data for avapritinib in ISM

Q2 2020

- Top-line VOYAGER data for avapritinib in 3L GIST
- Top-line ARROW data for pralsetinib in RET+ MTC

Q3 2020

- Top-line EXPLORER and PATHFINDER data for avapritinib in advanced SM

– Additional medical conference presentations planned throughout 2020 –

ISM, indolent systemic mastocytosis; GIST, gastrointestinal stromal tumors. MTC, medullary thyroid cancer; NSCLC, non-small cell lung cancer.

PIONEER: A Randomized, Double-Blind, Placebo-Controlled, Phase 2 Study of Avapritinib in Patients with Indolent or Smoldering Systemic Mastocytosis with Symptoms Inadequately Controlled with Standard Therapy

Cem Akin, Hanneke Oude Elberink, Jason Gotlib, Vito Sabato, Karin Hartmann, Sigurd Broesby-Olsen, Mariana Castells, Michael W. Deininger, Mark L. Heaney, Tracy I. George, Deepti Radia, Massimo Triggiani, Paul van Daele, Daniel J. DeAngelo, Oleg Schmidt-Kittler, Hui-Min Lin, Andrew Morrison, Brenton Mar, Frank Siebenhaar, Marcus Maurer

American Academy of Allergy Asthma and Immunology Annual Meeting
March 16, 2020

PIONEER
Indolent & Smoldering SM

Disclosures

- AYVAKIT™ (avapritinib) is approved by the FDA for the treatment of adults with unresectable or metastatic gastrointestinal stromal tumor (GIST) harboring a platelet-derived growth factor receptor alpha (PDGFRA) exon 18 mutation, including PDGFRA D842V mutations, in the United States. Avapritinib has not been approved by the FDA or any other health authority for use in the United States for any other indication or in any other jurisdiction for any indication.

- All data in this presentation are based on a cut-off date of December 27, 2019 unless otherwise specified.

Systemic mastocytosis (SM) is a clonal mast cell (MC) neoplasm driven by *KIT* D816V

Hyperactivation and proliferation

Debilitating mediator symptoms in **skin**, **gastrointestinal** and **neurocognitive** areas

Significant symptom directed polypharmacy

SM Prevalence of ~1:10,000
~32,000 estimated in US

~5% Advanced SM
Organ damage and decreased survival

~95% Non-advanced SM
Indolent and Smoldering SM

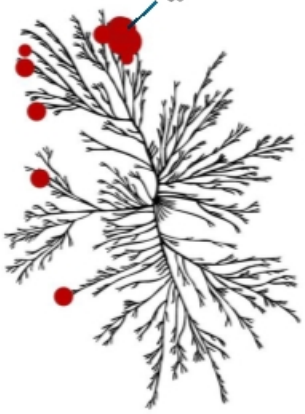
Suffer **long-term** with significant morbidity and **poor quality of life**

No effective approved therapies to reduce burden of disease

Avapritinib targets D816V with objective and symptomatic responses in SM

Highly potent on KIT D816V

Biochemical IC_{50} = 0.27 nM[1]



Highly selective kinome profile

Objective responses in AdvSM

Phase 1 EXPLORER trial

77% confirmed ORR[2] in Advanced SM at ≥200mg once daily

Responses deepen over time

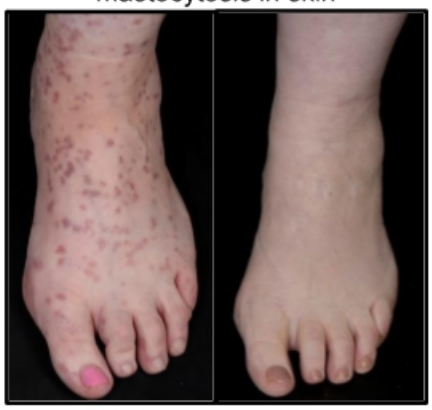
FDA Breakthrough Designation for AdvSM

Registration-enabling PATHFINDER trial in AdvSM is currently enrolling

Efficacy on AdvSM symptoms

Significant reduction in AdvSM-SAF total symptom score[3]

Potential for **resolution** of mastocytosis in skin[2]



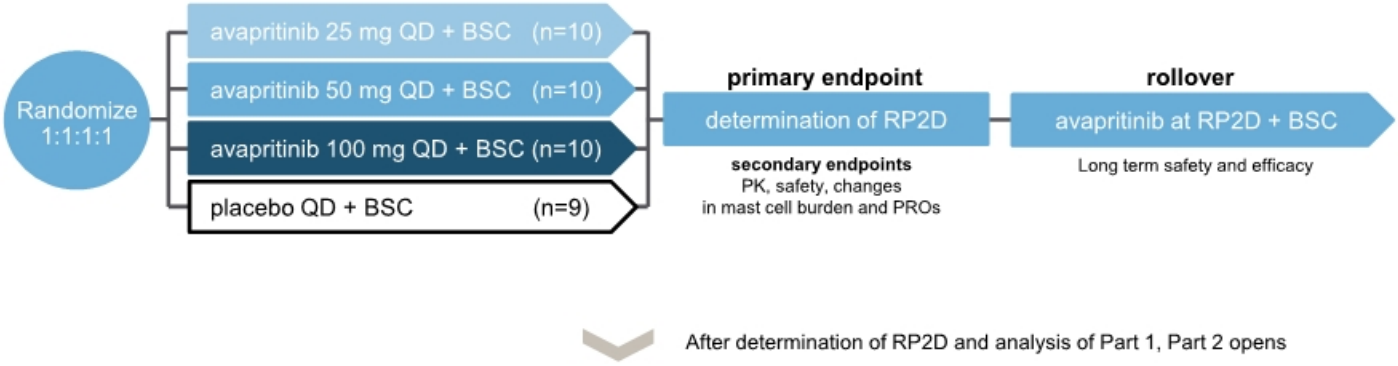
Baseline On study

Kinome illustration reproduced courtesy of Cell Signaling Technology, Inc. (www.cellsignal.com)(CSTI). The foregoing website is maintained by CSTI and Blueprint Medicines is not responsible for its content.
AdvSM, advanced systemic mastocytosis; IC50, half-maximal inhibitory concentration; ORR, overall response rate; QD, once daily.
1. Evans EK et al. *Sci Transl Med.* 2017;9:eaao1690. 2. Radia D et al. Presented at the 24th European Hematology Association Congress, Amsterdam, the Netherlands, July 1316, 2019. 3. Gotlib JR et al. *Blood.* 2016;132 (suppl 1):351.

Phase 2 PIONEER clinical trial in patients with non-advanced SM

Part 1: Dose Selection (fully enrolled)

Selection of well tolerated long term chronic dose with appropriate benefit-risk for indolent SM

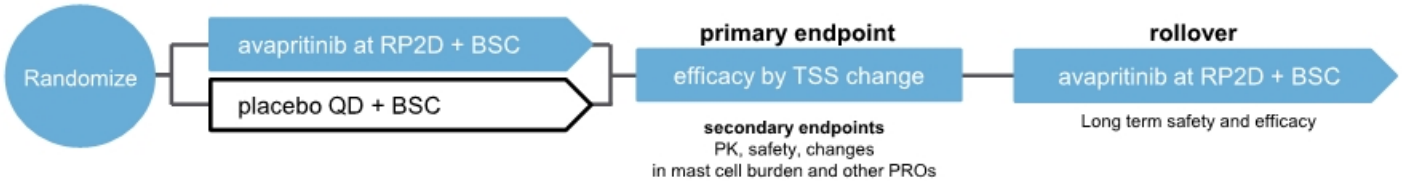


After determination of RP2D and analysis of Part 1, Part 2 opens

BSC, best supportive care; PK, pharmacokinetics; PRO, patient-reported outcome; RP2D, recommended part 2 dose.

Phase 2 PIONEER clinical trial in patients with non-advanced SM

Part 2: Pivotal Efficacy (pending)
Registration-enabling portion powered to demonstrate efficacy over placebo



Key Eligibility Criteria

- Age ≥18 years, ECOG performance status 0–2
- Indolent SM confirmed by central pathology review of bone marrow biopsy, according to WHO criteria
- Moderate-to-severe symptoms based on minimum mean TSS over the 14-day eligibility screening period despite ≥2 classes of best supportive care (BSC) medications

ECOG, Eastern Cooperative Oncology Group TSS, total symptom score; WHO, World Health Organization.

Baseline patient and disease characteristics

Patient Demographics	All doses (N=39)
Age (years), Median (range)	51 (21–75)
Sex, n (%), Female	30 (77)
ECOG PS, n (%), 0	12 (31)
1	19 (49)
2	8 (21)

Mast Cell Burden			
Central diagnosis of indolent ISM, n (%)			39 (100)
Tryptase (central), ng/mL, Mean (SD)			84 (101)
Median (range)			45 (6–416)
<11.4 ng/mL, n (%)			3 (8)
11.4 to 20 ng/mL, n (%)			6 (15)
>20 ng/mL, n (%)			30 (77)
Bone marrow core biopsy MC (central), %			
Mean (SD)			16 (16)
Median (range)			10 (1–60)
MC aggregates present, %			90
KIT D816V mutation	Local[a]	Central NGS[b]	Central ddPCR[c]
n (%) detected	31 (80)	11 (28)	37 (95)
Median MAF, % (range)	-	11 (1.9-31)	0.36 (0.0-30)

SM Therapy, n (%)	All doses (N=39)
Prior cytoreductive therapy	6 (16)
Midostaurin, imatinib, dasatinib, masitinib	5 (13)
Interferon alfa	1 (3)
Baseline Supportive Care Meds, median (range)	4 (2-9)
H1 blockers	37 (95)
H2 blockers	30 (77)
Leukotriene receptor antagonists	23 (59)
Proton pump inhibitors	18 (46)
Cromolyn sodium	12 (31)
Corticosteroids	6 (15)
Omalizumab	9 (23)

Patient Disposition	
Weeks on study, median (range)	18 (1–36)
Still on study, n (%)	37 (95)
Discontinued study, n (%)	2 (5)
Patient decision, n	1
Protocol non-compliance, n	1

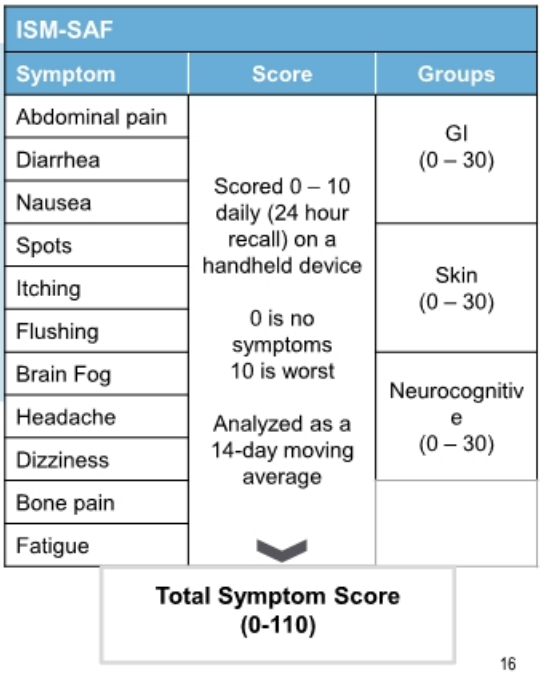
All data in this presentation are based on a cut-off of December 27, 2019 unless otherwise specified.

[a]Local quantitative and qualitative KIT testing of bone marrow and/or blood, various methods and sensitivities. [b]NGS=next generation sequencing targeted myeloid panel (central) in blood, algorithmic calling sensitivity to 1.9% MAF; [c]digital droplet PCR in blood (central), sensitivity to 0.02% MAF, detected: positive at screening or C1D1, Median MAF and range at C1D1 in those with any detection.
C1D1, cycle 1 day 1; ISM, indolent systematic mastocytosis; MAF, mutation allele fraction; MC, mast cells; PS, performance status; SD, standard deviation

ISM-SAF, a reliable construct valid patient reported outcomes tools for ISM

ISM-Symptom Assessment Form (SAF)

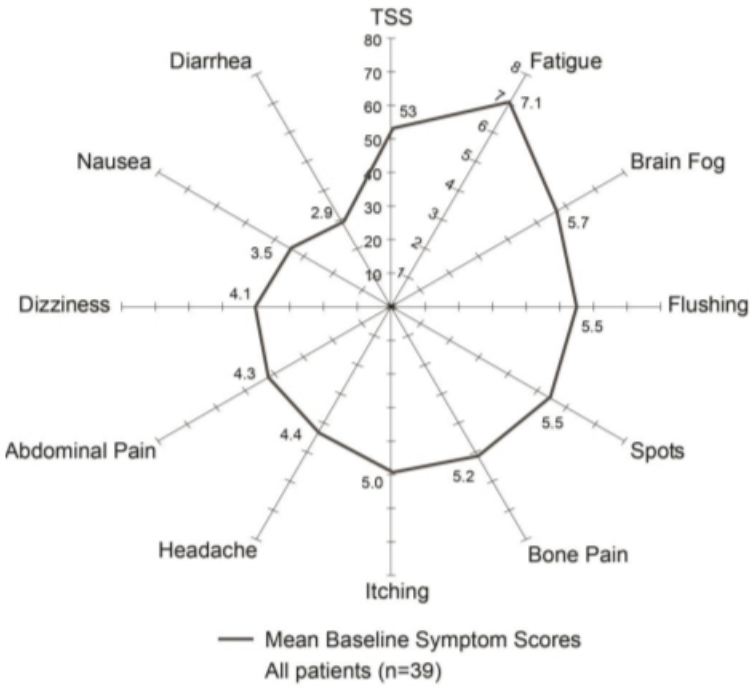
- Clinical benefit measure and primary endpoint for PIONEER trial
- Designed with input from disease experts, patients and regulatory authorities to support regulatory approval



GI, gastrointestinal; ISM, indolent SM.

1. Shields A et al. *Value Health*. 2019;22 (suppl 3):S867-868.

Significant baseline sign and symptom burden in patients enrolled on PIONEER

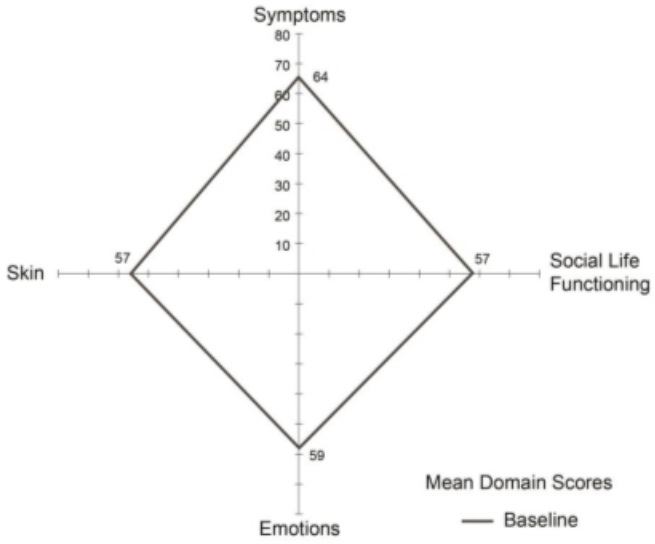


- Every patient with significant symptom burden at baseline
- Most severe symptoms in the 14 days prior to dosing were fatigue, brain fog, flushing and spots
- >99% daily adherence to ISM-SAF entry by patients
- Mean Total Symptom Score: 53

MC-QOL, a Quality of Life questionnaire for patients with ISM

Mastocytosis Quality of Life (MC-QoL) Questionnaire[1]

- A quality of life tool developed for mastocytosis
- 27 questions across 4 domains: Skin, Symptoms, Social Life/Functioning and Emotions
- 2 week recall, performed at every study visit
- Each domain with 3 to 9 symptoms, each domain score and Mc-QoL total score scaled to 0-100



Mean MC-QoL Total Score: 60

1. Siebenhaar F et al. *Allergy*. 2016;71:869-87.

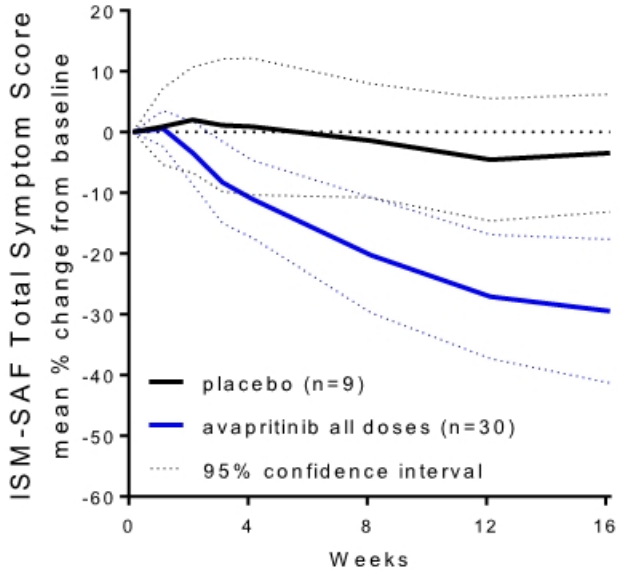
Well tolerated safety profile across all doses with no grade 3 AEs at 25 mg

AE in >15% of placebo or avapritinib arms			avapritinib					
Preferred term	**Placebo n=9**		**25 mg n=10**		**50 mg n=10**		**100 mg n=10**	
% of subjects with ≥1 AE	any grade	grade 3	any grade	grade 3	any grade	grade 3	any grade	grade 3
	89	**22**	**100**	**0**	**80**	**20**	**90**	**40**
Nausea	22	0	10	0	60	10	40	0
Dizziness	22	0	30	0	30	0	40	0
Headache	11	0	30	0	30	10	30	10
Diarrhea	11	0	0	0	40	10	30	10
Fatigue	11	0	40	0	10	0	10	0
Face edema	0	0	10	0	0	0	40	0
Peripheral edema	0	0	10	0	20	0	20	0
Periorbital edema	0	0	0	0	20	0	30	0
Bone Pain	22	0	0	0	0	0	0	0

- No grade 4 or 5 AEs on study
- No patients discontinued treatment due to AE or progression to AdvSM
- No neutropenia, anemia, thrombocytopenia or intracranial bleeding
- One grade 3 cognitive disorder in the 100 mg cohort resolved following dose modification; patient remains on treatment at 25 mg

AE, adverse event

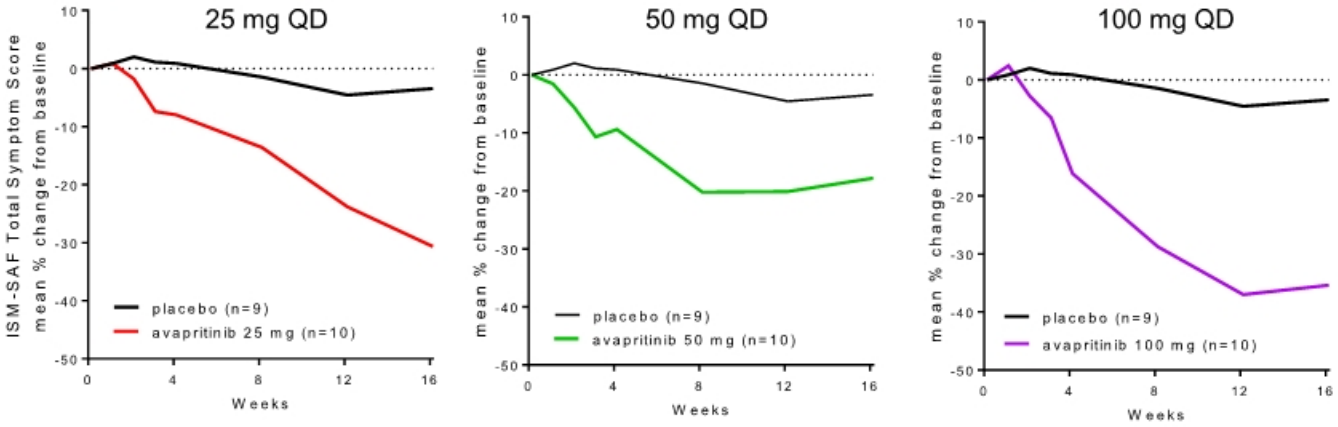
Avapritinib significantly improves overall SM symptoms compared to placebo



- ~30% mean symptom reduction at 16 weeks in avapritinib treated patients measured by ISM-SAF TSS
- ~3% mean symptom reduction in placebo
- Difference is statistically significant (p=0.001) at 16 weeks of therapy

n is number dosed in each cohort

Avapritinib 25 mg QD achieves similar reduction to 100 mg QD by week 16



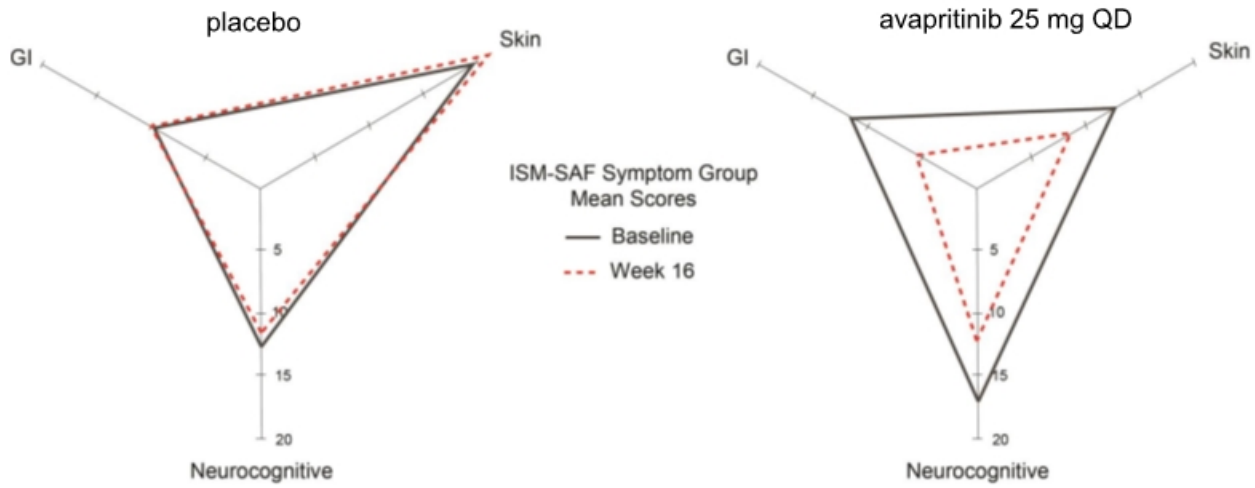
- 25 mg QD demonstrates similar % reduction in mean symptom burden to 100 mg dose at week 16

avapritinib 25 mg QD selected as the RP2D

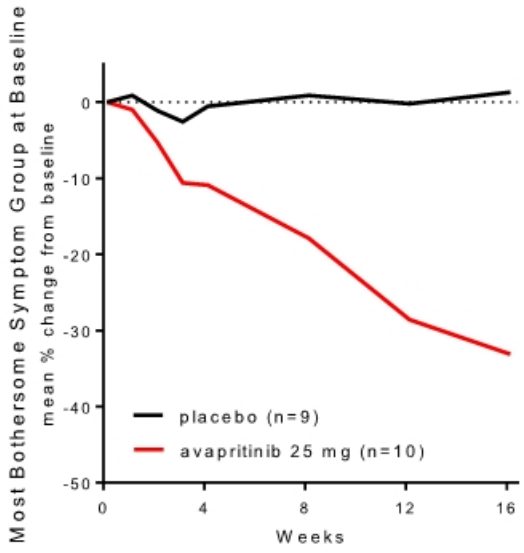
25 mg dose provided similar mean improvements as higher doses with better tolerability

n is number dosed in each cohort

Avapritinib 25 mg QD achieves symptom reduction in GI, Skin and Neurocognitive symptom groups compared to placebo



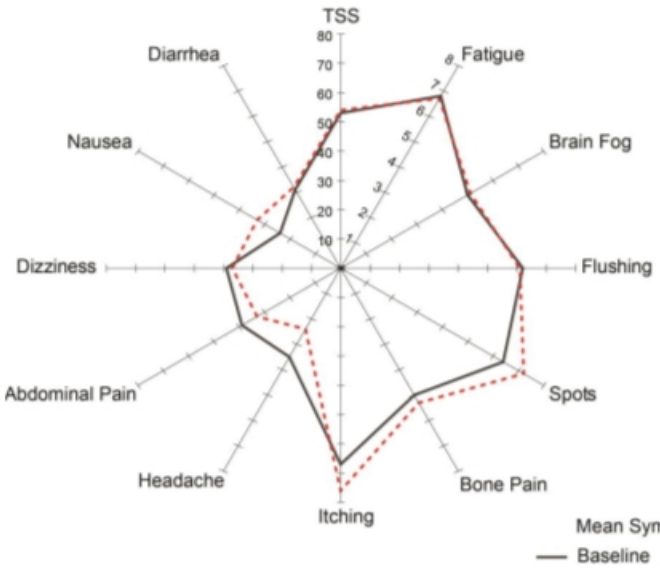
Avapritinib 25 mg QD improves most bothersome symptom group



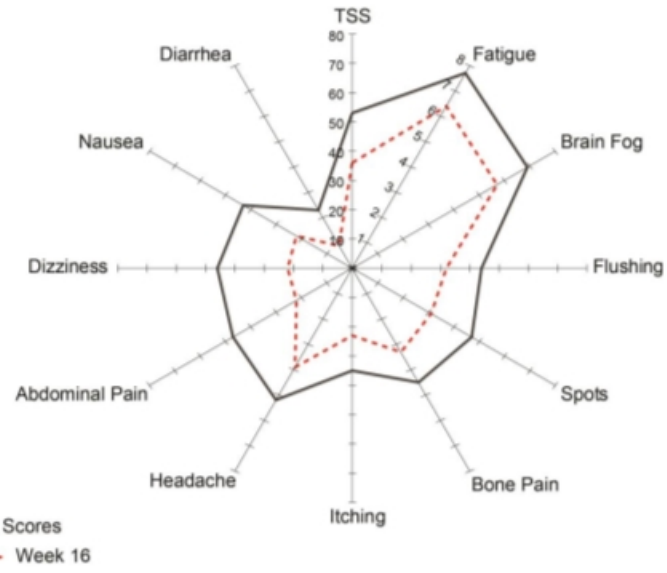
- Improvements in most bothersome symptom group at baseline for each patient
- The most bothersome symptom group for these patients were:
  - 47.4% Skin
  - 47.4% Neurocognitive
  - 5.2% GI

Avapritinib 25mg QD improves individual symptoms compared to placebo

placebo

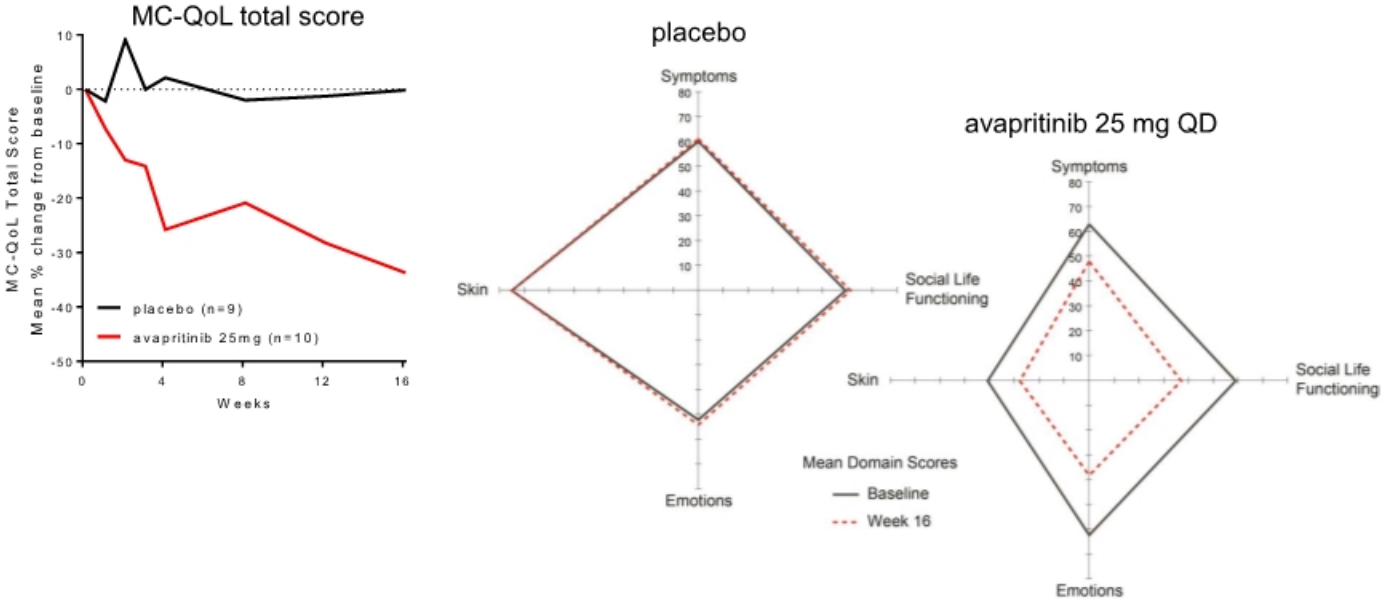


avapritinib 25 mg QD

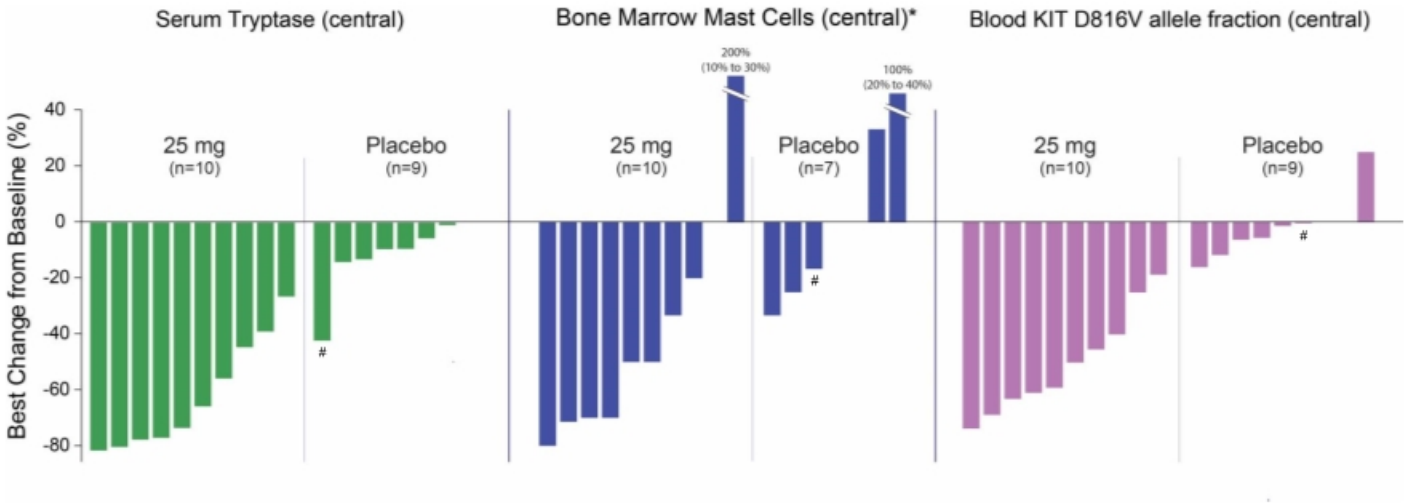


Mean Symptom Scores
Baseline
Week 16

Improvements in Quality of Life with avapritinib 25mg QD by MC-QoL



Objective reductions in mast cell burden at 25 mg vs placebo



*Bone marrow MC assessment in SM may have variability in sampling due to patchy nature of disease. No patient on study has progressed to advanced disease. #patient received high dose IV steroids.

Conclusions

- Avapritinib treatment results in a statistically significant reduction in total symptom score at 16 weeks
- Avapritinib has a favorable safety profile in patients with indolent SM, supporting further evaluation of a chronic dosing regimen
  - 95% of patients remain on study, with no discontinuations for AEs
  - No grade ≥3 AEs occurred in the 25 mg QD cohort
- Avapritinib 25 mg QD achieves clinically meaningful improvements at 16 weeks and is the recommended part 2 dose
  - Reductions in bone marrow MC burden, serum tryptase and blood KIT D816V allele fraction
  - Improvements in clinical outcomes, as measured by ISM-SAF total symptom score and all symptom domain scores, at week 16
  - Improvements in quality of life, as measured by MC-QoL overall score and all domain scores, at week 16
- Part 2 of the registration-enabling PIONEER study is anticipated to initiate patient screening in June 2020

Registration strategy in indolent SM

Andy Boral, MD, PhD, Chief Medical Officer

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Blueprint Medicines is pioneering the path to registration in indolent SM

Lowest effective dose to enable chronic treatment › **Validated, clinically important outcome measures** › **Efficient registration-enabling clinical trial design**

Safety results for avapritinib 25mg QD are similar to placebo at 16 weeks

AE in >15% of placebo or avapritinib arms	Placebo n=9		avapritinib 25 mg n=10	
Preferred term				
% of subjects with ≥1 AE	any grade	grade 3	any grade	grade 3
	89	**22**	**100**	**0**
Nausea	22	0	10	0
Dizziness	22	0	30	0
Headache	11	0	30	0
Diarrhea	11	0	0	0
Fatigue	11	0	40	0
Face edema	0	0	10	0
Peripheral edema	0	0	10	0
Periorbital edema	0	0	0	0
Bone Pain	22	0	0	0

AVAPRITINIB 25 MG QD

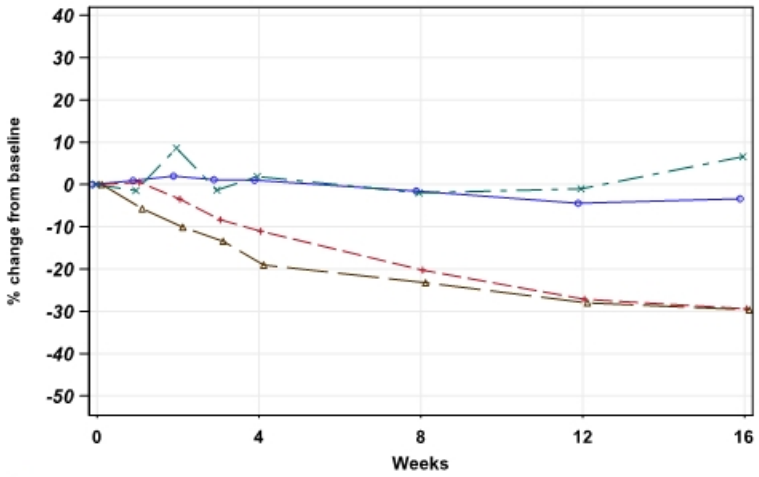
- **No patients had serious AEs**
  - 2 patients treated with placebo had serious AEs, 1 with psychogenic seizure and 1 with diffuse cutaneous mastocytosis
- **No patients had dose modifications**
- **No patients discontinued due to AEs**

Data presented in March 2020 at AAAAI annual meeting. Data cutoff: December 27, 2019.

The ISM-SAF and MC-QoL outcome measures are highly correlated

Mean change in ISM-SAF TSS and MC-QoL

(All avapritinib cohorts versus placebo)



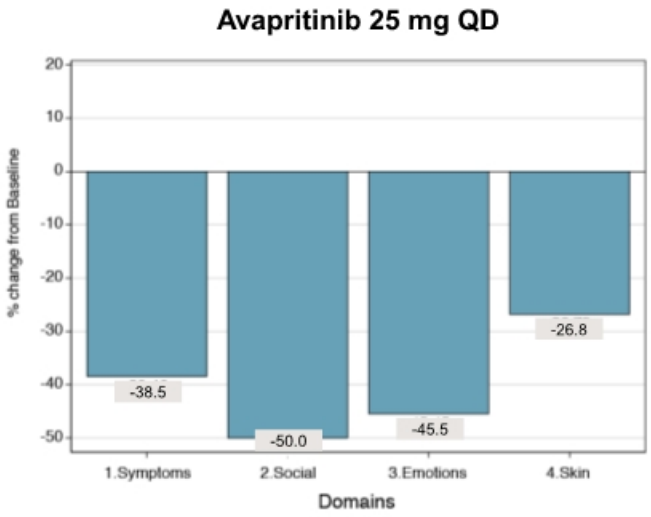
Correlation from baseline to Week 16

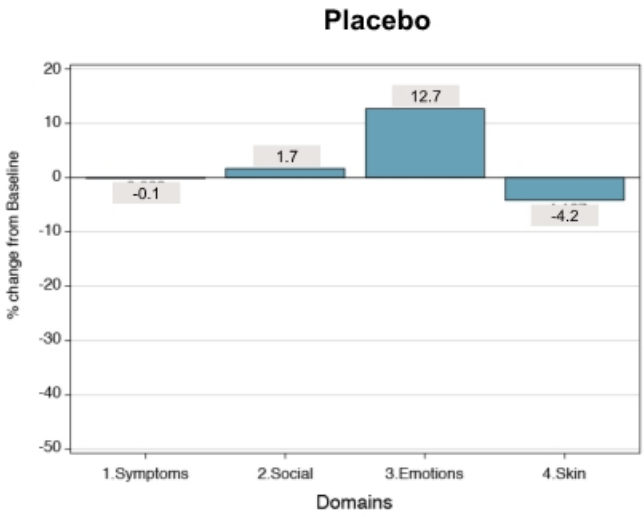
- Pearson correlation co-efficient: 0.65
- Spearman correlation co-efficient: 0.69

MC-QoL – placebo
ISM-SAF TSS – placebo
ISM-SAF TSS – all avapritinib
MC-QoL – all avapritinib

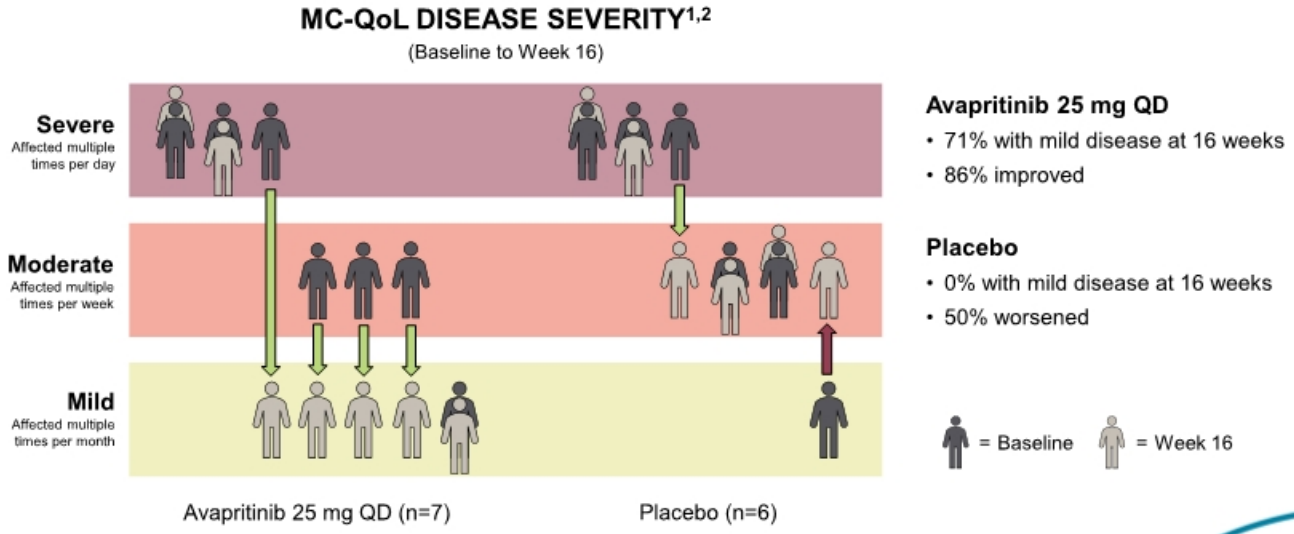
Data cutoff: December 27, 2019.

Avapritinib 25 mg QD improves all quality of life domains measured by the MC-QoL by 16 weeks



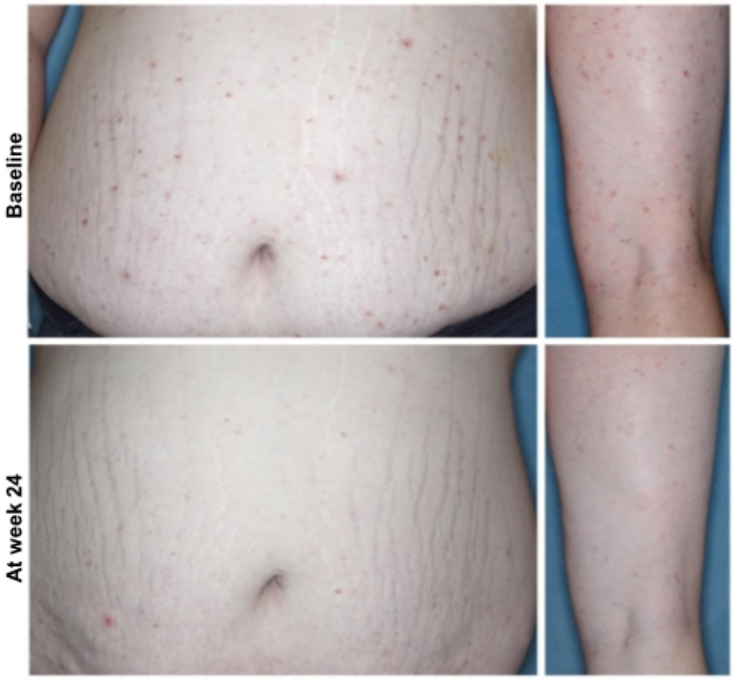


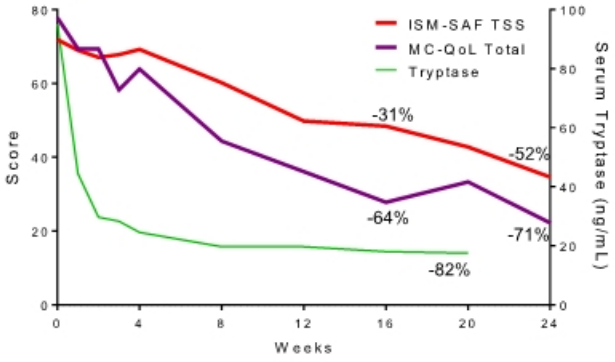
Avapritinib 25 mg QD demonstrates clinically meaningful changes in disease severity, as measured by the MC-QoL



[1] PIONEER trial analysis of patients with MC-QoL responses at baseline and 16 weeks. Data cutoff: December 27, 2019. [2] Siebenhaar, et al. Development and Validation of the Mastocytosis Quality of Life Questionnaire: MC-QoL. Allergy, 2016.

36 year old female with ISM previously diagnosed with cutaneous mastocytosis





At Baseline

- Cutaneous lesions with flaring and itching
- Diarrhea, abdominal pain, bloating
- History of anaphylaxis with facial swelling and throat closing
- Loratidine qAM, cetirizine qPM, ranitidine BID, cromalyn QID

On avapritinib 25 mg QD at week 24

- Eliminated MC aggregates, tryptase -82%, KIT D816V -40%
- Now minimal GI issues, skin improvement
- Only one related AE of dry eye (grade 1)

Next steps for PIONEER trial of avapritinib in indolent SM

PIONEER REGISTRATION-ENABLING PART 2

Design: Randomized, double-blind, placebo-controlled treatment period, followed by open-label expansion

Key endpoints: ISM-SAF total symptom score (primary), measures of mast cell burden, quality of life, concomitant medications

Sample size: ≤200 patients

Duration: ~6 months

Timeline: Plan to initiate patient screening in June 2020 and complete enrollment by the end of 2020

Opportunity to deliver therapeutic value to patients with SM

Christy Rossi, Chief Commercial Officer

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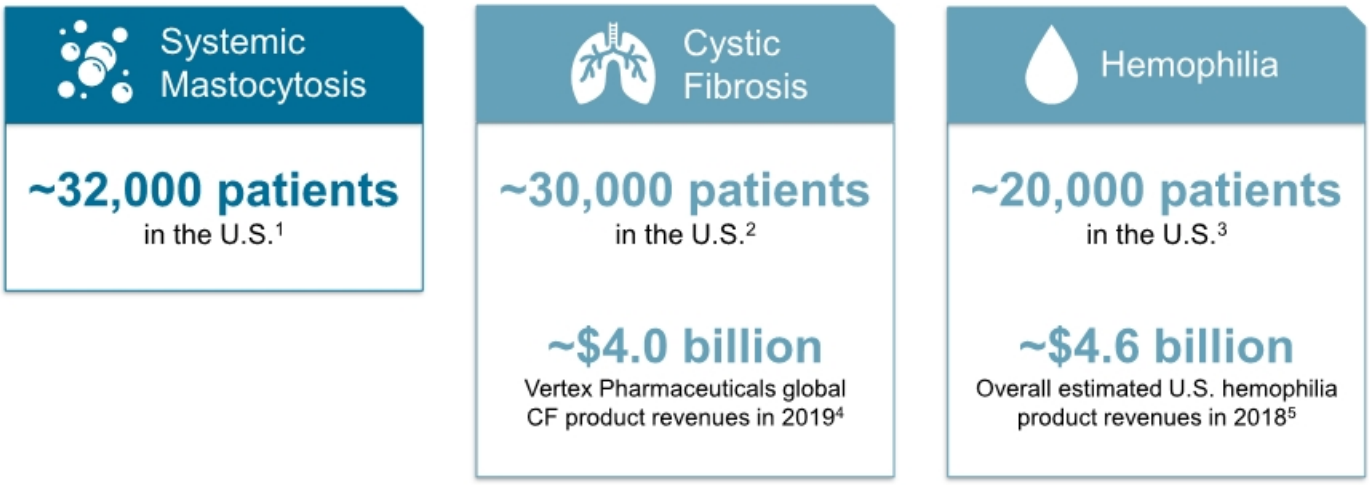
Systemic mastocytosis is a rare disease with high medical need and a population size comparable to cystic fibrosis and hemophilia



[1] Based on estimated prevalence for advanced, indolent and smoldering SM. [2] Cystic Fibrosis Foundation website. Available at: www.cff.org/What-is-CF/About-Cystic-Fibrosis. [3] National Hemophilia Foundation website. Available at: www.hemophilia.org/Bleeding-Disorders/Types-of-Bleeding-Disorders/Hemophilia-A. [4] Vertex Pharmaceuticals Inc. press release on January 30, 2020. [5] "A look at hemophilia drug prices and the market." Biospace. Available at: www.biospace.com/article/a-look-at-hemophilia-drug-prices-and-the-market. July 3, 2018. CF, cystic fibrosis. CF and hemophilia product revenues included for illustration purposes only.

Most patients with ISM experience debilitating symptoms that profoundly impact their quality of life

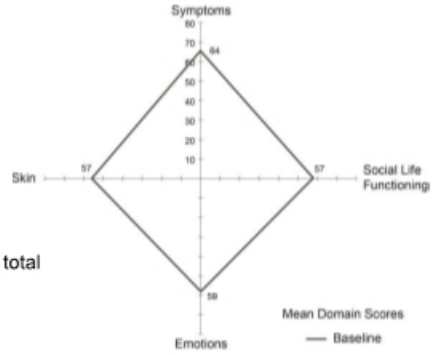
At baseline, patients in PIONEER had multiple unresolved morbidities related to their ISM:[1]

- **89%** had gastrointestinal disorders
- **67%** had nervous system disorders
- **67%** had skin or subcutaneous tissue disorders
- **55%** had musculoskeletal or connective tissue disorders

ISM symptoms have a profound impact on patient quality of life:[2]

MC-QoL Questionnaire (n=39)

Mean baseline total score: 60

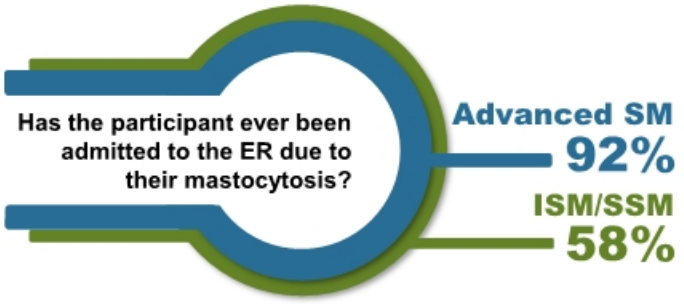


[1] Review of patient medical histories for PIONEER trial placebo cohort. [2] Data cutoff: December 27, 2019.

Management of ISM often requires extensive healthcare system utilization

- **1/3** of patients visited a doctor more than 10 times in the past year[1]
- **75%** of patients have taken ≥4 classes of drugs[1]
- **77%** of patients have been prescribed and **60%** carry self-injectable epinephrine at all times[2]

Symptoms result in need for urgent and/or inpatient hospital care[1]



[1] Mast Cell Connect registry data on file. Enrollment initiated December 1, 2015. Data cutoff: August 20, 2019.
[2] Jennings S et al. J Allergy Clin Immunol Pract. 2014;2(1):70-76.

Finding patients is about looking for them

German experience: SM patients are found near centers with capacity to diagnose

We are now actively engaging to enable patient identification globally

Hallmark SM signs and symptoms: skin lesions, anaphylaxis, GI distress

↓

Serum tryptase

↓

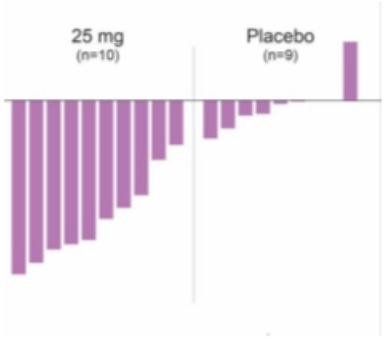
KIT D816V mutational testing with high-sensitivity assay

German Registry on Disorders of Eosinophils and Mast Cells; Schwaab et al., DGHO 2019; Graphic courtesy of Andreas Reiter.

Avapritinib is the only highly potent inhibitor of KIT D816V, the genetic driver of SM, and has an unparalleled clinical profile

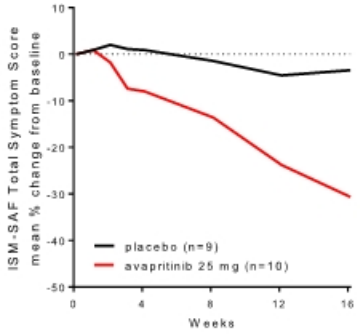
Reduces mast cell burden

KIT D816V mutant allele fraction



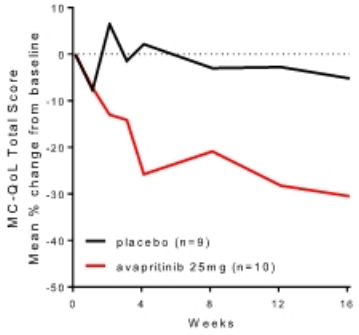
Improves disease symptoms

ISM-SAF total symptom score



Improves quality of life

MC-QoL total score



Favorable safety profile for once-daily oral avapritinib 25 mg supports evaluation of chronic treatment

Data reported at AAAAI Annual Meeting in March 2020. Data cutoff: December 27, 2019.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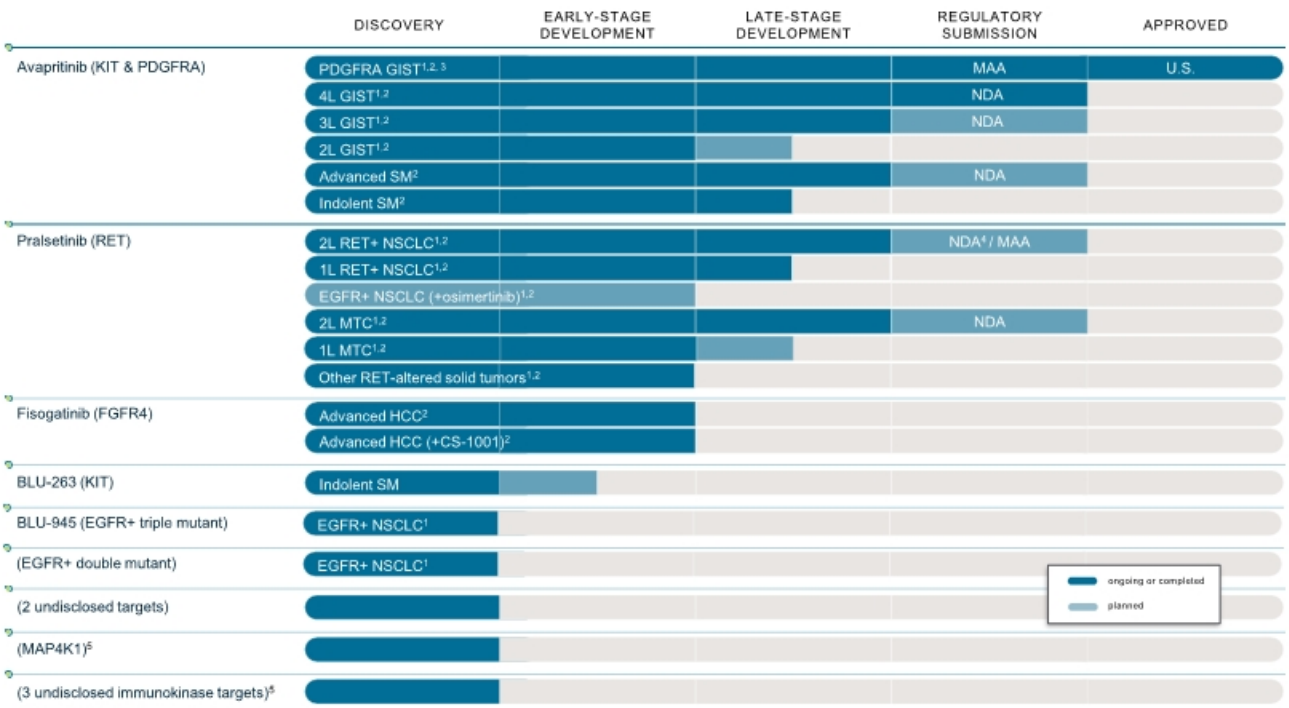
Questions & answers



1. Unresectable or metastatic disease. 2. CStone Pharmaceuticals has exclusive rights to develop and commercialize avapritinib, pralsetinib and fisogatinib in Mainland China, Hong Kong, Macau and Taiwan. Blueprint Medicines retains all rights in the rest of the world. 3. Approved in the U.S. for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations. The proposed MAA indication is unresectable or metastatic GIST harboring a PDGFRA D842V mutation. 4. Expect to complete rolling NDA in March 2020. 5. In collaboration with Roche. Blueprint Medicines has U.S. commercial rights for up to two programs. Roche has worldwide commercialization rights for up to two programs and ex-U.S. commercialization rights for up to two programs. 1L, first-line; 2L, second-line; 3L, third-line; 4L, fourth-line; GIST, gastrointestinal stromal tumors; HCC, hepatocellular carcinoma; MAA, marketing authorization application; MTC, medullary thyroid cancer; NDA, new drug application; NSCLC, non-small cell lung cancer.

Exhibit 99.2

Blueprint Medicines Announces Part 1 Results from PIONEER Trial Showing Broad Activity of Avapritinib Across Measures of Mast Cell Burden, Clinical Outcomes and Quality of Life in Indolent Systemic Mastocytosis

-- Avapritinib showed a statistically significant improvement in patient-reported outcomes, the primary measure of clinical benefit in PIONEER --

-- Avapritinib was well-tolerated across all three doses; no patient discontinuations due to AEs --

-- 25 mg QD selected as recommended Part 2 dose, based on consistent and clinically important improvements across multiple measures of efficacy and a well-tolerated safety profile --

-- Blueprint Medicines to host investor conference call and webcast today at 7:30 a.m. ET --

CAMBRIDGE, Mass., March 16, 2020 – Blueprint Medicines Corporation (NASDAQ: BPMC), a precision therapy company focused on genomically defined cancers, rare diseases and cancer immunotherapy, today announced updated results from the Phase 2 PIONEER trial of avapritinib in patients with indolent systemic mastocytosis (SM) showing significant clinical improvements versus placebo. In Part 1 of the PIONEER trial, patients treated with avapritinib showed a statistically significant mean decline of approximately 30 percent in total symptom score (TSS) at 16 weeks, as measured by the Indolent SM Symptom Assessment Form (ISM-SAF), and reductions in symptom scores have deepened over time. In addition, patients treated with avapritinib achieved consistent improvements across objective measures of mast cell burden and patient-reported quality of life. Avapritinib was well-tolerated with no patients discontinuing treatment due to adverse events (AEs). Based on the full Part 1 data, 25 mg once daily (QD) has been selected as the recommended Part 2 dose (RP2D). Results from this data presentation will be available on an American Academy of Allergy, Asthma & Immunology (AAAAI) virtual forum, which was established following the cancellation of the 2020 AAAAI Annual Meeting: https://education.aaaai.org/annual-meeting-abstracts/.

SM is a rare disease driven by the KIT D816V mutation and characterized by uncontrolled mast cell proliferation and activation. The disorder can lead to debilitating symptoms and life-threatening complications. Avapritinib is a potent and highly selective inhibitor of D816V mutant KIT.

“Indolent SM causes devastating symptoms that wreak havoc on patients’ lives, often involves a high polypharmacy burden, and may result in frequent urgent care visits and hospitalizations,” said Cem Akin, M.D., Ph.D., Professor of Medicine at the University of Michigan and an investigator on the PIONEER trial. “These data show the promise of avapritinib to offer sustained, clinically meaningful improvements across multiple measures of disease, including quality of life and reductions in mast cell burden, and a well-tolerated safety profile. Avapritinib has the potential to advance treatment beyond symptomatic therapies and fundamentally change the way we manage this debilitating disease.”

“Avapritinib was specifically designed to inhibit D816V mutant KIT, with the goal of delivering transformative benefit to patients,” said Andy Boral, M.D., Ph.D., Chief Medical Officer at Blueprint Medicines. “In indolent SM, these placebo-controlled data are the first to show consistent clinical activity across multiple measures of disease, from mast cell burden to clinical outcomes and quality of life. The results continue to build our confidence that by potently targeting the underlying cause of the disorder, we are enabling profound benefits across the spectrum of SM.”

Blueprint Medicines plans to initiate patient screening for the registration-enabling Part 2 of the PIONEER trial in June 2020. Part 2 is designed to evaluate the efficacy of avapritinib at the RP2D versus placebo. Blueprint Medicines anticipates completing enrollment in Part 2 of the PIONEER trial by the end of 2020.

Highlights from the Part 1 PIONEER Trial Data in Indolent SM

Part 1 of the PIONEER trial was designed to determine the RP2D by evaluating three doses of avapritinib (25 mg, 50 mg and 100 mg QD) versus placebo. Key eligibility criteria include adults with indolent SM confirmed by central pathology review and moderate-to-severe symptom burden despite best supportive care medicines. Overall, 39 patients were enrolled in Part 1 across four concurrent cohorts, consisting of 10 patients each in the avapritinib dose cohorts and nine patients in the placebo cohort.

Patient-reported outcomes (PRO) data were collected using the ISM-SAF, which was designed with input from disease experts, patients and regulatory authorities to support registration. All results are as of a data cutoff date of December 27, 2019.

Baseline Patient Characteristics

Patients had high symptom burden at baseline, with a mean ISM-SAF TSS of 53 on a scale of 0 to 110. Eight patients (21 percent) had an Eastern Cooperative Oncology Group Performance Status of 2, reflecting the inability to carry out any work activities. Patients received a median of four best supportive care medicines at baseline (range: 2-9). Median serum tryptase was 45 micrograms per liter (the upper limit of normal is 11.4 micrograms per liter). A high sensitivity polymerase chain reaction assay on peripheral blood detected the KIT D816V mutation in 37 patients (95 percent).

Clinical Activity

Avapritinib showed broad activity across measures of mast cell burden, the PRO clinical benefit measure and quality of life. The consistency of results observed across multiple measures of disease burden support the further evaluation of avapritinib in indolent SM. At 16 weeks, patients had a statistically significant reduction in ISM-SAF TSS, with a mean improvement of approximately 30 percent across all avapritinib dose cohorts compared to approximately 3 percent in the placebo cohort (p=0.001). As of the data cutoff date, 37 patients (95 percent) have remained on study with a median follow-up of 18 weeks.

Results from the 25 mg QD dose cohort show important clinical activity, including meaningful declines in serum tryptase, bone marrow mast cells and KIT D816V allele burden. Treatment with avapritinib led to consistent reductions in the ISM-SAF TSS, gastrointestinal domain, skin domain and each individual symptom. Symptom improvements in patients treated at 25 mg QD continued to deepen over time.

Mean Percent Changes in ISM-SAF at 16 Weeks

	Avapritinib, 25 mg QD	**Placebo**
TSS	-31%	-3%
Skin domain	-37%	+3%
Gastrointestinal domain	-25%	+6%
Neurological symptoms	-26%	-8%

Data from the Mastocytosis Quality of Life (MC-QoL) questionnaire, a PRO tool developed for mast cell disorders, show improvements in quality of life for patients receiving avapritinib and support the results observed with the ISM-SAF. Patients in the 25 mg QD dose cohort had a mean reduction of 34 percent in the total MC-QoL score and improvements in all four domains assessed (symptoms, social life functioning, emotions and skin). A 7 percent increase from baseline was observed in the placebo cohort.

Safety

The safety profile of avapritinib supports chronic dosing in indolent SM. All doses of avapritinib were well-tolerated and no patients discontinued treatment due to AEs. No patients treated with avapritinib in the 25 mg QD dose cohort had serious AEs, Grade 3 or higher AEs, or dose modifications. In the placebo cohort, two patients (22 percent) had Grade 3 AEs, one with seizure and one with diffuse cutaneous mastocytosis; these events also met criteria for serious AEs.

Conference Call Information

PIONEER trial data were previously accepted as a late-breaking oral abstract at the 2020 AAAAI Annual Meeting. Due to the cancellation of the AAAAI Annual Meeting, Blueprint Medicines is reporting the data during an investor conference call and webcast today at 7:30 a.m. ET.

To access the live call, please dial (855) 728-4793 (domestic) or (503) 343-6666 (international), and refer to conference ID 1590639. A webcast of the conference call will be available in the Investors & Media section of Blueprint Medicines'

website at http://ir.blueprintmedicines.com. The archived webcast will be available on Blueprint Medicines' website approximately two hours after the conference call and will be available for 30 days following the call.

About SM

SM is a rare disease driven by the KIT D816V mutation. Uncontrolled proliferation and activation of mast cells result in chronic, severe and often unpredictable symptoms for patients across the spectrum of SM. The vast majority of those affected have non-advanced (indolent or smoldering) SM, with debilitating symptoms that lead to a profound, negative impact on quality of life. A minority of patients have advanced SM, which encompasses a group of high-risk SM subtypes including aggressive SM, SM with an associated hematologic neoplasm and mast cell leukemia. In addition to mast cell activation symptoms, advanced SM is associated with organ damage due to mast cell infiltration and poor overall survival.

Debilitating symptoms associated with SM, including anaphylaxis, maculopapular rash, pruritis, brain fog, fatigue and bone pain, often persist despite treatment with a number of symptomatic therapies. Patients often live in fear of attacks, have limited ability to work or perform daily activities, or isolate themselves to protect against unpredictable triggers. Currently, there are no approved therapies that selectively inhibit D816V mutant KIT.

About AYVAKIT™ (avapritinib)

AYVAKIT™ (avapritinib) is a kinase inhibitor approved by the FDA for the treatment of adults with unresectable or metastatic gastrointestinal stromal tumor (GIST) harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations. For more information, visit AYVAKIT.com.

Avapritinib is not approved for the treatment of any other indication, including SM, in the U.S. or any other jurisdiction by the FDA or any other health authority.

About the Clinical Development Program for Avapritinib

Avapritinib is an oral precision therapy that selectively and potently inhibits KIT and PDGFRA mutant kinases. Blueprint Medicines is pursuing a broad clinical development program for avapritinib for advanced, smoldering and indolent SM, as well as across multiple lines of GIST treatment.

Avapritinib is uniquely designed to selectively bind and inhibit D816V mutant KIT, the common driver of disease in approximately 95 percent of all SM patients. Preclinical studies have shown avapritinib potently inhibited KIT D816V at sub-nanomolar potencies with minimal off-target activity. In addition, avapritinib has demonstrated broad inhibition of KIT and PDGFRA mutations associated with GIST, including potent activity against activation loop mutations that are associated with resistance to currently approved therapies.

The FDA has granted Breakthrough Therapy Designation to avapritinib for two indications: one for the treatment of advanced SM, including the subtypes of aggressive SM, SM with an associated hematologic neoplasm and mast cell leukemia, and one for the treatment of unresectable or metastatic GIST harboring the PDGFRA D842V mutation.

Patients and clinicians interested in ongoing or planned clinical trials can contact the Blueprint Medicines study director at studydirector@blueprintmedicines.com or 1-617-714-6707. Additional details are available at www.blueprintclinicaltrials.com or www.clinicaltrials.gov.

About the Phase 2 PIONEER Trial

PIONEER is a randomized, double-blind, placebo-controlled, registration-enabling trial evaluating avapritinib in patients with indolent and smoldering SM. The trial includes three parts: dose-finding Part 1, registration-enabling Part 2 and long-term treatment Part 3. All patients who complete Parts 1 or 2 will have an opportunity to continue to receive treatment with avapritinib in Part 3. Key trial endpoints include the change in patient-reported disease symptoms as measured by the ISM-SAF TSS, quantitative measures of mast cell burden and safety. Part 1 has completed patient enrollment. Blueprint Medicines plans to initiate patient screening for Part 2 in June 2020 at sites in the United States, Canada and European Union. Additional details are available at www.blueprintclinicaltrials.com/SM/ or www.clinicaltrials.gov.

About Blueprint Medicines

Blueprint Medicines is a precision therapy company striving to improve human health. With a focus on genomically defined cancers, rare diseases and cancer immunotherapy, we are developing transformational medicines rooted in our leading expertise in protein kinases, which are proven drivers of disease. Our uniquely targeted, scalable approach empowers the rapid design and development of new treatments and increases the likelihood of clinical success. We have one FDA-approved precision therapy and are currently advancing multiple investigational medicines in clinical development, along with a number of research programs. For more information, visit www.BlueprintMedicines.com and follow us on Twitter (@BlueprintMeds) and LinkedIn.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements regarding plans and timelines for the development of its drug candidates, including the timing, design, implementation, enrollment, plans and announcement of results regarding Blueprint Medicines' ongoing and planned clinical trials for avapritinib in SM; plans, timelines and expectations for initiating patient screening in Part 2 of the PIONEER trial and for completing enrollment in Part 2 of the PIONEER trial; expectations regarding the potential benefits of avapritinib in treating patients with SM; and Blueprint Medicines' strategy, goals and anticipated milestones, business plans and focus. The words "aim," "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, risks and uncertainties related to Blueprint Medicines' ability and plans in continuing to establish a commercial infrastructure and successfully launching, marketing and selling any current or future approved products; Blueprint Medicines' ability to successfully expand the approved indications for AYVAKIT or obtain marketing approval for AYVAKIT in additional geographies in the future; the delay of any current or planned clinical trials or the development of Blueprint Medicines' drug candidates, including any drug candidates licensed to third parties; Blueprint Medicines' advancement of multiple early-stage efforts; Blueprint Medicines' ability to successfully demonstrate the safety and efficacy of its drug candidates and gain approval of its drug candidates on a timely basis, if at all; the preclinical and clinical results for Blueprint Medicines' drug candidates, which may not support further development of such drug candidates; actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials; Blueprint Medicines' ability to develop and commercialize companion diagnostic tests for its current and future drug candidates; and the success of Blueprint Medicines' current and future collaborations or licensing arrangements. These and other risks and uncertainties are described in greater detail in the section entitled "Risk Factors" in Blueprint Medicines' filings with the Securities and Exchange Commission (SEC), including Blueprint Medicines' most recent Annual Report on Form 10-K, as supplemented by its most recent Quarterly Report on Form 10-Q and any other filings that Blueprint Medicines has made or may make with the SEC in the future. Any forward-looking statements contained in this press release represent Blueprint Medicines' views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. Except as required by law, Blueprint Medicines explicitly disclaims any obligation to update any forward-looking statements.

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