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): **April 1, 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**	**02139**
(Address of principal executive offices)	(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.

Blueprint Medicines Corporation (the "Company") from time to time presents and/or distributes to the investment community at various industry and other conferences slide presentations to provide updates and summaries of its business. The Company is posting to the "Investors & Media" portion of its website at http://ir.blueprintmedicines.com/ a copy of its current corporate slide presentation. A copy of the presentation 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 April 1, 2020, the Company issued a press release reporting the achievement of a number of key milestones, including the top-line data from the Phase 1/2 ARROW trial in patients with RET mutant medullary thyroid cancer, the completion of the rolling new drug application submission for pralsetinib for RET fusion-positive non-small cell lung cancer, and the submission of an investigational new drug application for BLU-263 for the treatment of 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 April 1, 2020
99.2	Press release issued by Blueprint Medicines Corporation on April 1, 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: April 1, 2020

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

PRECISION THAT MOVES™

Staying one step ahead of disease

APRIL 1, 2020

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; the plans, timing, design, initiation, enrollment, expectations and announcement of results for the Company's ongoing and planned clinical trials; 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 the Company's current or future approved drugs or drug candidates in treating patients; expectations regarding the Company's existing, cash, cash equivalents and investments; and the Company's 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 impact of the COVID-19 pandemic to the Company's business, operations, strategy, goals and anticipated milestones, including the Company's ongoing and planned research and discovery activities, ability to conduct ongoing and planned clinical trials, clinical supply of current or future drug candidates, commercial supply of current or future approved drugs, and launching, marketing and selling current or future approved drugs; 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 the Company's current or future approved drugs or drug candidates; 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.

Leadership in a time of challenge and uncertainty

OUR APPROACH TO NAVIGATING THE COVID-19 PANDEMIC

PATIENT CENTERED

Stay focused on the patients who need access to our innovation, perhaps now more than ever

VIGILANT

Constantly assess and customize approaches to potential business impacts

NIMBLE

Leverage global infrastructure including external collaborators and adapt to new ways of working

RESILIENT

Provide support and flexibility to our employees to enable resiliency

3 clinical datasets reported in 2020 to date, with additional disclosures planned

Q1 2020

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

Q2 2020

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

Q3 2020

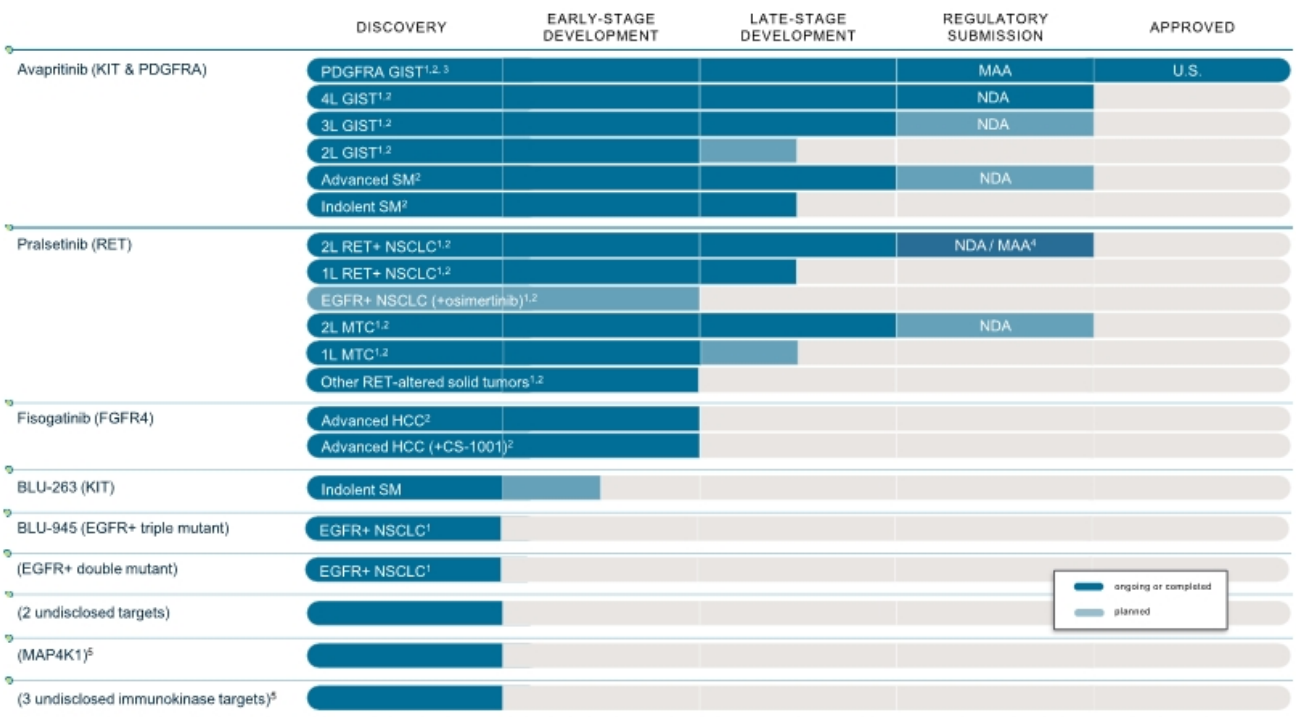
- Top-line EXPLORER and PATHFINDER data for avapritinib in advanced SM

On track to lock VOYAGER trial database in April 2020 and provide top-line data to FDA to enable action on avapritinib NDA for 4L GIST by May 14 PDUFA date

FDA, U.S. Food and Drug Administration; ISM, indolent systemic mastocytosis; GIST, gastrointestinal stromal tumors. MTC, medullary thyroid cancer; NDA, new drug application; NSCLC, non-small cell lung cancer; PDUFA, the Prescription Drug User Fee Act; SM, systemic mastocytosis; 3L, third-line; 4L, fourth-line.

Anticipate multiple commercial launches through 2021

1. Approved in the U.S. for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutant, including PDGFRA D842V mutations. 2. Proposed MAA indication is unresectable or metastatic GIST harboring a PDGFRA D842V mutation. 3. Planned NDA or MAA submissions. MAA, marketing authorization application; 2L, second-line. *All planned commercial launches are subject to regulatory review and approval of marketing applications currently under review or planned.



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. NDA submitted to FDA in March 2020; plan to submit MAA to EMA in Q2 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; HCC, hepatocellular carcinoma

Pralsetinib: an investigational precision therapy for RET-altered cancers

	LATE CLINICAL DEVELOPMENT	U.S. REGULATORY SUBMISSION STATUS
RET fusion-positive NSCLC		Submitted
Previously treated MTC		Q2 2020*
Other RET-altered tumors		

Pralsetinib

Potent and highly selective RET inhibitor

* Planned NDA submission. 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.

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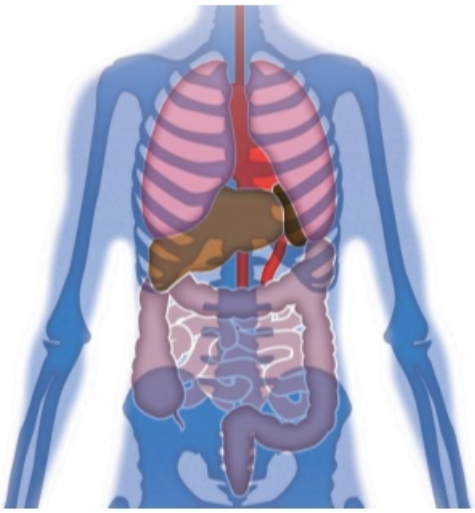
RET alterations: oncogenic drivers lacking a targeted therapeutic approach

Non-small cell lung cancer:
~1-2% RET fusions[1,2]

Advanced medullary thyroid cancer:
~90% RET mutations[3]

Papillary thyroid cancer:
~20% RET fusions[4]

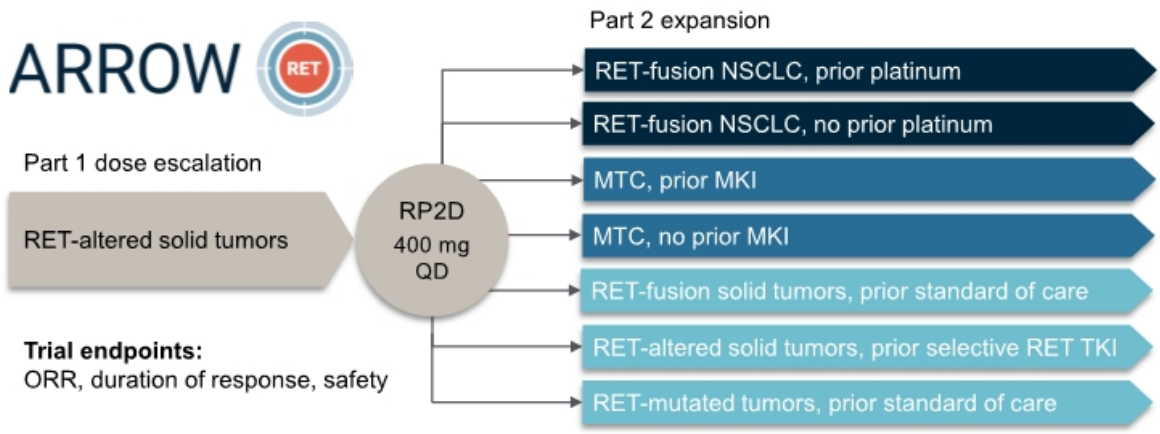


Multiple other tumor types <1% RET-altered, including:[5,6]

esophageal
pancreatic
breast
melanoma
colorectal
leukemia

1. Lipson, et al. Nat Med 2012. 2. Takeuchi, et al. Nat Med 2012. 3. Romei, et al. Oncotarget 2018. 4. Santoro, et al. J Clin Invest 1992. 5. Kato, et al. Clin Cancer Res 2017. 6. Ballerini, et al. Leukemia 2012.

Top-line ARROW trial data support registration plans for NSCLC and MTC



Top-line safety[1]
(n=438; 400 mg QD)

- Top-line safety results consistent with prior data
- Pralsetinib was well-tolerated and most AEs were Grade 1 or 2
- Across all patients, 4% discontinued due to treatment-related AEs

1. Phase 1/2 ARROW trial data in patients treated with pralsetinib 400 mg QD reported on April 1, 2020. Data cutoff: February 13, 2020. AE, adverse event; MKI, multi-kinase inhibitor; ORR, overall response rate; QD, once daily; RP2D, recommended phase 2 dose; TKI, tyrosine kinase inhibitor

NSCLC patients with RET fusions have no highly effective treatment options

- **Chemotherapy:** nonspecific, low response rates, significant toxicity
- **Checkpoint inhibition:** Preliminary evidence for lack of benefit in RET-altered NSCLC[1]
- **Multi-kinase inhibitors:** ↓ activity, ↑ off-target toxicity[2,3]
- Growing understanding of RET-driven resistance
- No selective RET inhibitors are approved

1. Mazieres, et al. JCO 2018. 2. Drilon, et al. Lancet 2017. 3. Yoh, et al. Lancet Respir Med 2017.

Top-line ARROW trial data: RET fusion-positive NSCLC

61%
ORR[1]

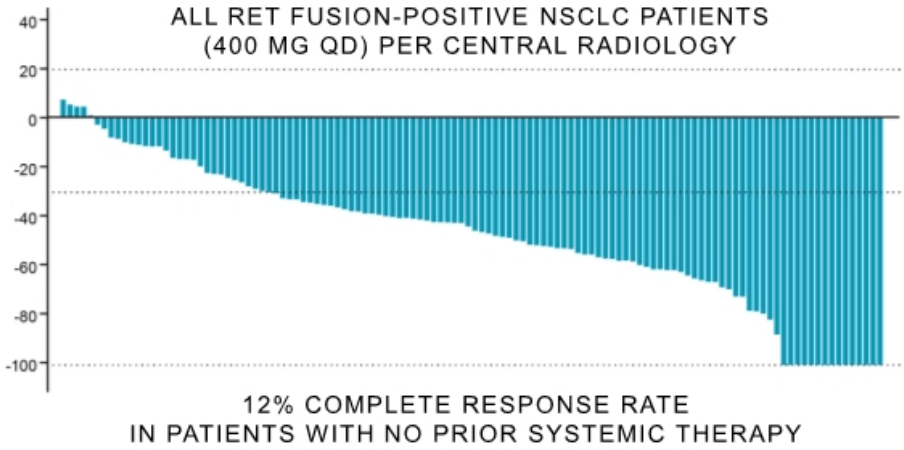
RET-fusion NSCLC with prior platinum chemotherapy

400 mg QD, N=80

73%
ORR[2]

RET-fusion NSCLC with no prior systemic therapy

400 mg QD, N=26



Median DOR not reached (95% CI: 11.3 months, NE) in patients treated with 400 mg QD

Phase 1/2 ARROW trial data in patients with RET fusion-positive NSCLC reported on January 8, 2020. Data cutoff: November 18, 2019.
1. Two responses pending confirmation. 2. All responses confirmed. DOR, duration of response; NE, not estimable.

RET-altered thyroid cancer patients may benefit from highly targeted therapy

- **Multi-kinase inhibitors** are approved for MTC, but have important limitations:[1]
  - 25-44% ORR
  - Off-target toxicity often requiring dose modification or discontinuation
  - Emergence of resistance
- No selective RET inhibitors are approved

1. Drillon, et al. Nature Reviews Clinical Oncology, 2017.

Top-line ARROW trial data: RET mutant medullary thyroid cancer

60% ORR[1]

RET-mutated MTC with prior cabozantinib and/or vandetinib treatment

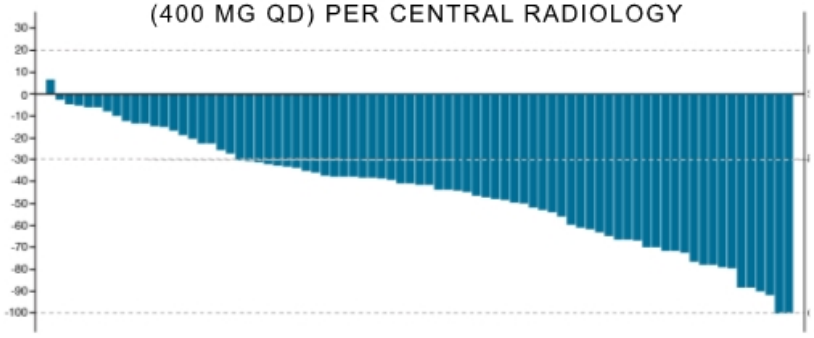
400 mg QD, N=53

74% ORR[2]

RET-mutated MTC with no prior systemic therapy

400 mg QD, N=19

ALL RET MUTANT MTC PATIENTS
(400 MG QD) PER CENTRAL RADIOLOGY

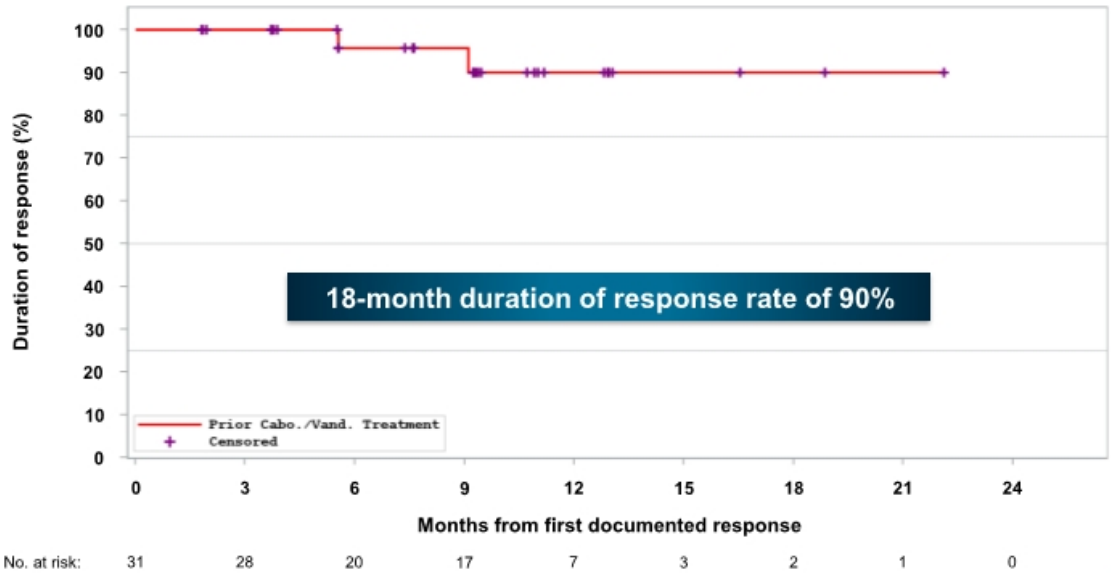


99% OF EVALUABLE PATIENTS HAD TUMOR REDUCTIONS

Median DOR not reached (95% CI: NE, NE) in patients treated with 400 mg QD

Phase 1/2 ARROW trial data in patients with RET mutated MTC reported on April 1, 2020. Data cutoff: February 13, 2020. 1. One response pending confirmation. 2. All responses confirmed.

Prolonged duration of response in patients with previously treated MTC



No. at risk:	31	28	20	17	7	3	2	1	0

Phase 1/2 ARROW trial data in patients with RET mutated MTC reported on April 1, 2020. Data cutoff: February 13, 2020.

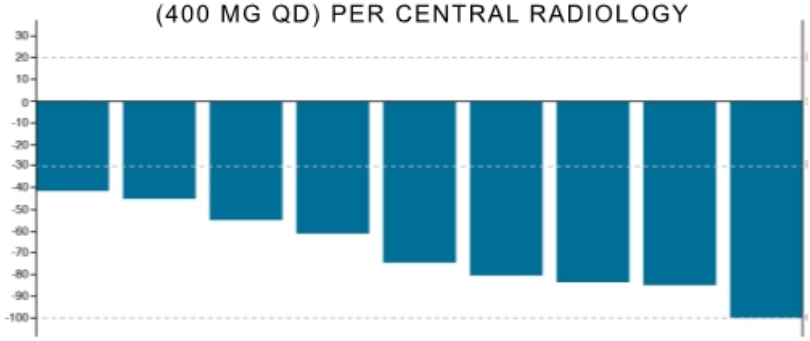
Top-line ARROW trial data: RET fusion-positive thyroid cancer

89%
ORR[1]

RET fusion-positive thyroid cancer with prior systemic therapy

400 mg QD, N=9

RET FUSION-POSITIVE THYROID CANCER PATIENTS (400 MG QD) PER CENTRAL RADIOLOGY



100% OF EVALUABLE PATIENTS HAD TUMOR REDUCTIONS

Median DOR not reached (95% CI: 8.2, NE) in patients treated with 400 mg QD

Phase 1/2 ARROW trial data in patients with RET fusion-positive thyroid cancer reported on April 1, 2020. Data cutoff: February 13, 2020. 1. All responses confirmed.

Pralsetinib is a potential best-in-class selective RET inhibitor and the cornerstone of our lung cancer portfolio

EQUIPOTENT INHIBITION
of RET fusions and mutations, including predicted gatekeeper resistance mutations

CLINICAL RESPONSES
in 2 of 4 patients previously treated with selpercatinib[3]

HIGH RESPONSE RATES AND DURABLE ACTIVITY
in RET+ NSCLC[1] and MTC[2] patients

FDA BREAKTHROUGH THERAPY DESIGNATIONS
for RET+ NSCLC and MTC[4]

STRONG ACTIVITY AGAINST BRAIN METASTASES
in patients with RET+ NSCLC[3]

WELL-TOLERATED WITH LOW DISCONTINUATION RATES in advanced cancer populations[1,2,3]

1. Top-line NSCLC data reported on January 8, 2020. Data cutoff: November 18, 2020. 2. Top-line MTC data reported on April 1, 2020. Data cutoff: February 13, 2020. 3. Data reported at ASCO 2019 Annual Meeting. Data cutoff: April 28, 2019. 4. FDA has granted breakthrough therapy designations to pralsetinib for the treatment of RET fusion-positive NSCLC that has progressed following platinum-based chemotherapy and RET-mutant MTC that requires systemic treatment and for which there are no acceptable alternative treatments.

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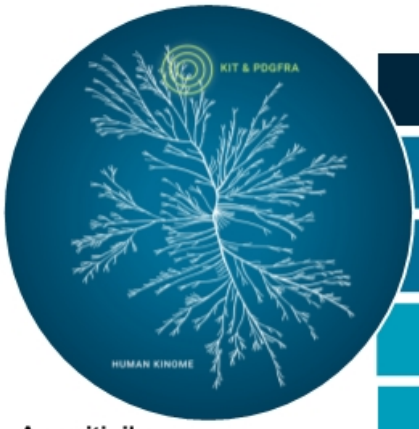
Avapritinib: a precision therapy with broad potential



Avapritinib
Potent and highly selective
KIT and PDGFRA inhibitor

LATE CLINICAL DEVELOPMENT	U.S. REGULATORY SUBMISSION STATUS
PDGFRA exon 18 mutant GIST	APPROVED[1]
4L GIST	SUBMITTED
3L GIST	2H 2020*
Advanced SM	2H 2020*
Indolent and smoldering SM	

1. 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. * Planned NDA submission. 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.

Not for promotional use.

AYVAKIT™ (avapritinib) is now approved in the United States

AYVAKIT™ avapritinib | tablets

INDICATION

AYVAKIT is indicated for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations

AVAILABLE DOSE STRENGTHS

100, 200 and 300 mg tablets

First precision therapy for GIST • Approved regardless of line of therapy
Only highly effective treatment for PDGFRA exon 18 mutant GIST

blueprint™ MEDICINES

Full prescribing information is available at www.AYVAKIT.com.

Not for promotional use.

Full approval of AYVAKIT based on Phase 1 NAVIGATOR trial

EFFICACY PARAMETER	PDGFRA EXON 18 (N=43)	PDGFRA D842V (N=38)
Overall response rate (95% CI)	84% (69%, 93%)	89% (75%, 97%)
Complete response, n (%)	3 (7%)	3 (8%)
Partial response, n (%)	33 (77%)	31 (82%)
Duration of response	n=36	N=34
Median in months (range)	Not reached (1.9+, 20.3+)	Not reached (1.9+, 20.3+)

Full prescribing information is available at www.AYVAKIT.com. CI, confidence interval.

Not for promotional use.

Safety highlights from AYVAKIT prescribing information

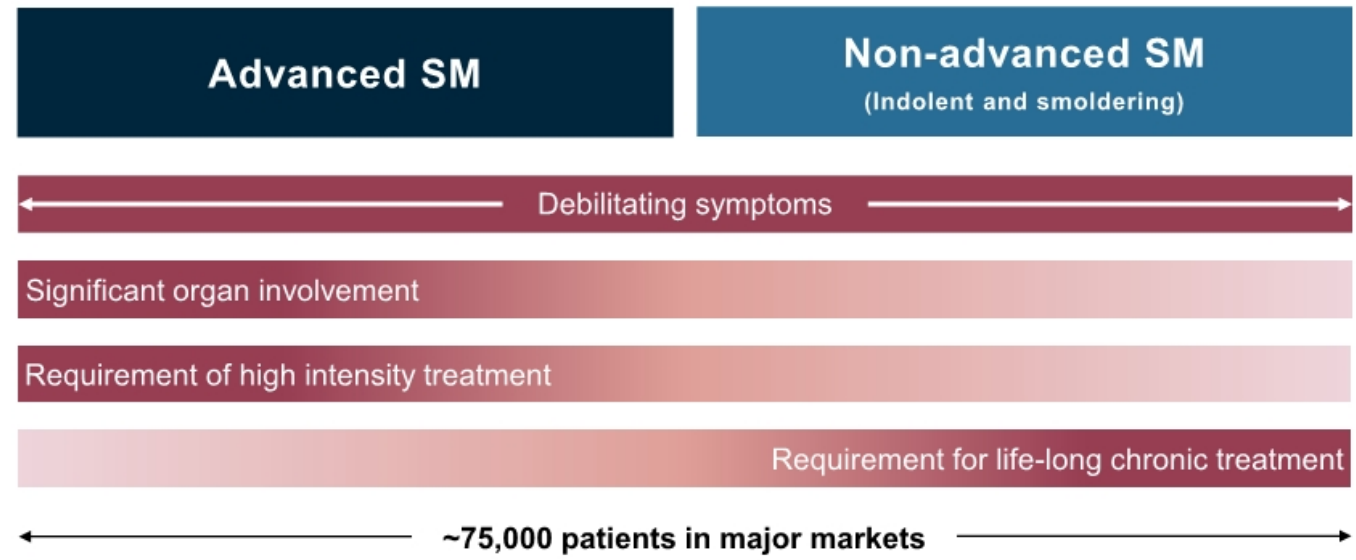
MOST COMMON ADVERSE REACTIONS (≥20%; ANY GRADE):[1]

- Edema, nausea, fatigue/asthenia, cognitive impairment, vomiting, decreased appetite, diarrhea, hair color changes, increased lacrimation, abdominal pain, constipation, rash, and dizziness

WARNINGS AND PRECAUTIONS:

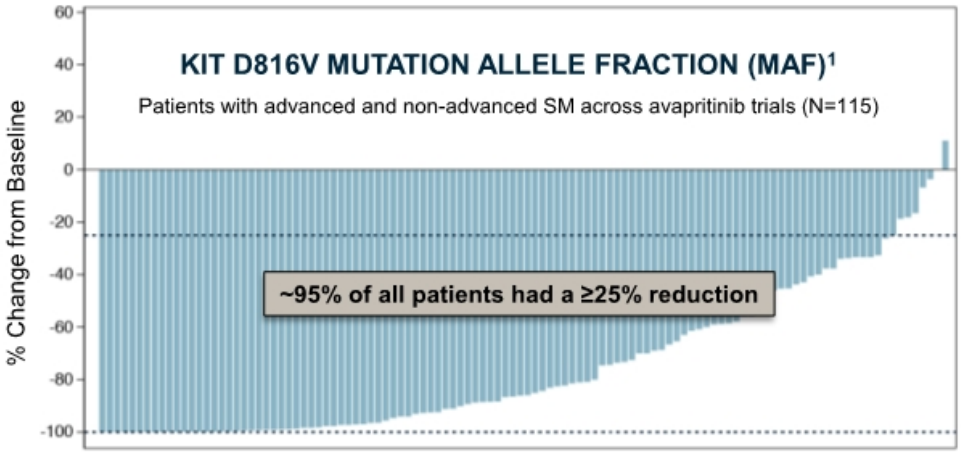
- Intracranial hemorrhage
  - Occurred in 1% of 267 patients with GIST who received AYVAKIT
- CNS adverse reactions
  - Occurred in 58% of 335 patients who received AYVAKIT
    - Cognitive impairment: 41% (3.6% Grade 3 or 4)
  - Overall, 3.9% of patients required treatment discontinuation due to a CNS adverse reaction
- Embryo-fetal toxicity

Systemic mastocytosis is one disease driven by KIT D816V



Patient numbers in major markets based on estimated prevalence for advanced, indolent and smoldering systemic mastocytosis in the US, EU5 and Japan.

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



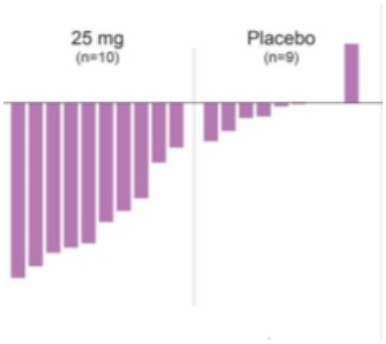
≥25% reduction in KIT D816V MAF is correlated with improved overall survival in advanced SM[2]

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.

PIONEER trial results: unparalleled clinical profile in patients with indolent SM

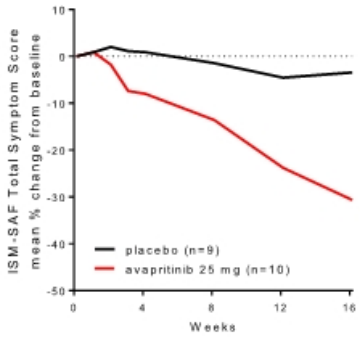
Reduces mast cell burden

KIT D816V mutant allele fraction



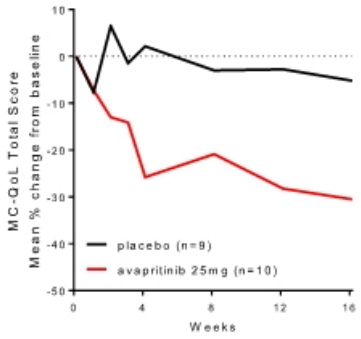
Improves disease symptoms

ISM-SAF total symptom score



Improves quality of life

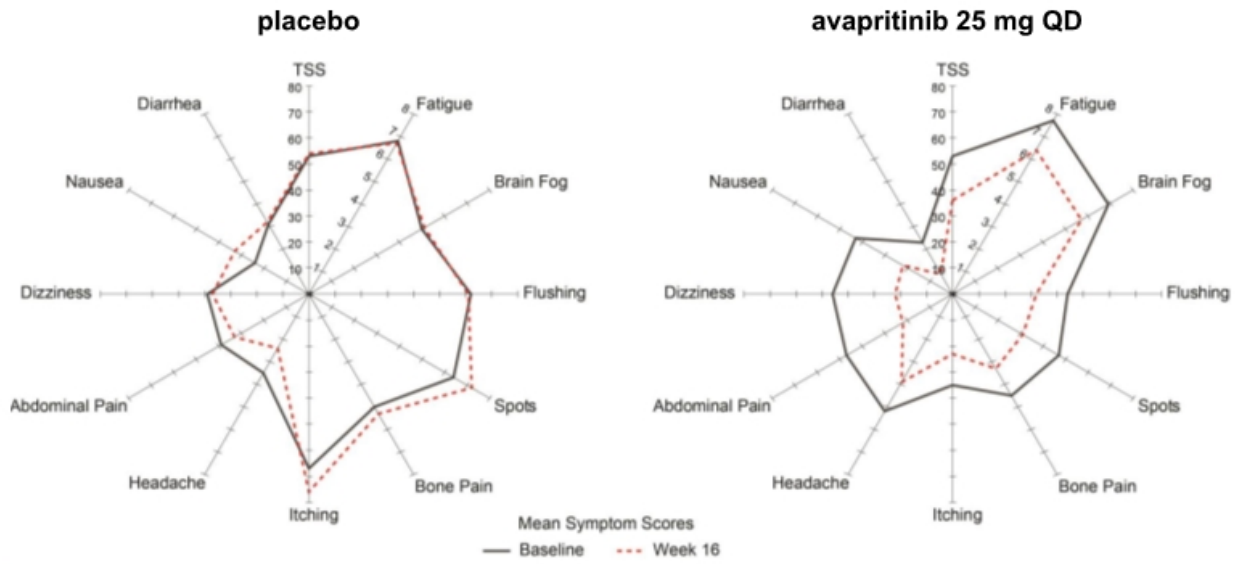
MC-QoL total score



Favorable safety profile supports the selection of avapritinib 25 mg QD as recommended Part 2 dose

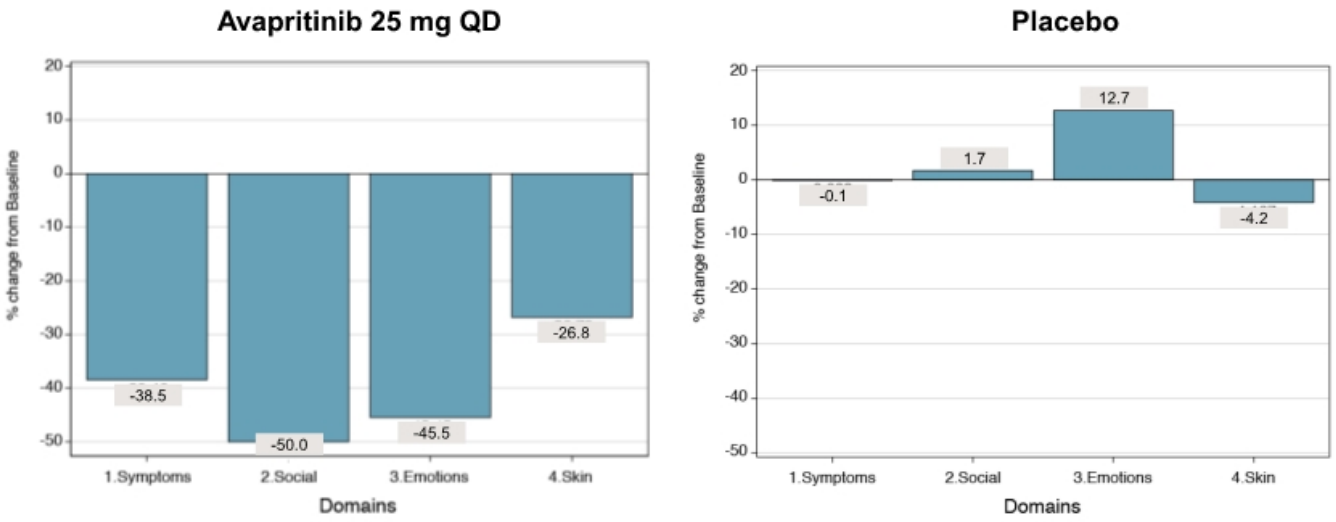
Data reported at AAAAI Annual Meeting in March 2020. Data cutoff: December 27, 2019.

Avapritinib improves all symptoms assessed by the ISM-SAF



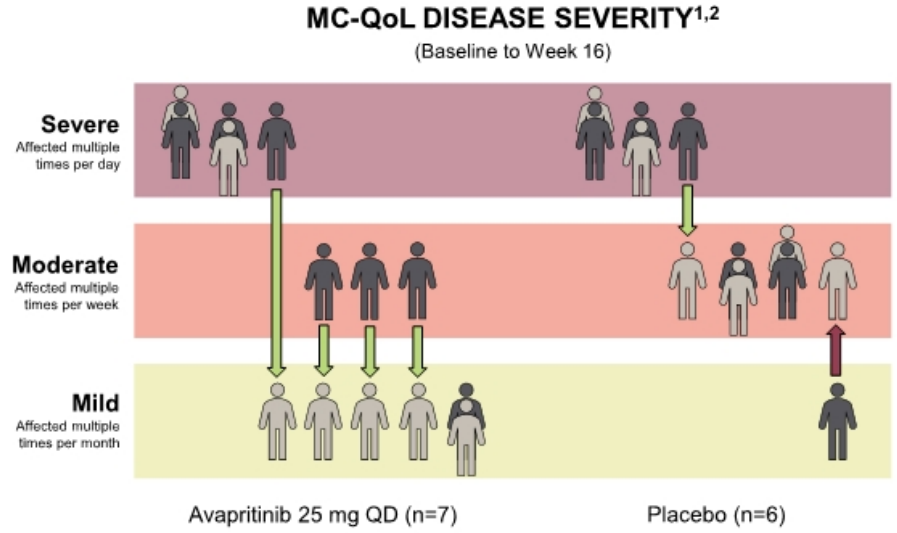
Data reported at AAAAI Annual Meeting in March 2020. Data cutoff: December 27, 2019. ISM-SAF, indolent systemic mastocytosis – symptom assessment form.

Avapritinib improves all quality of life domains measured by the MC-QoL



Data cutoff: December 27, 2019. MC-QoL, Mastocytosis Quality of Life Questionnaire.

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



Avapritinib 25 mg QD

- 71% with mild disease at 16 weeks
- 86% improved

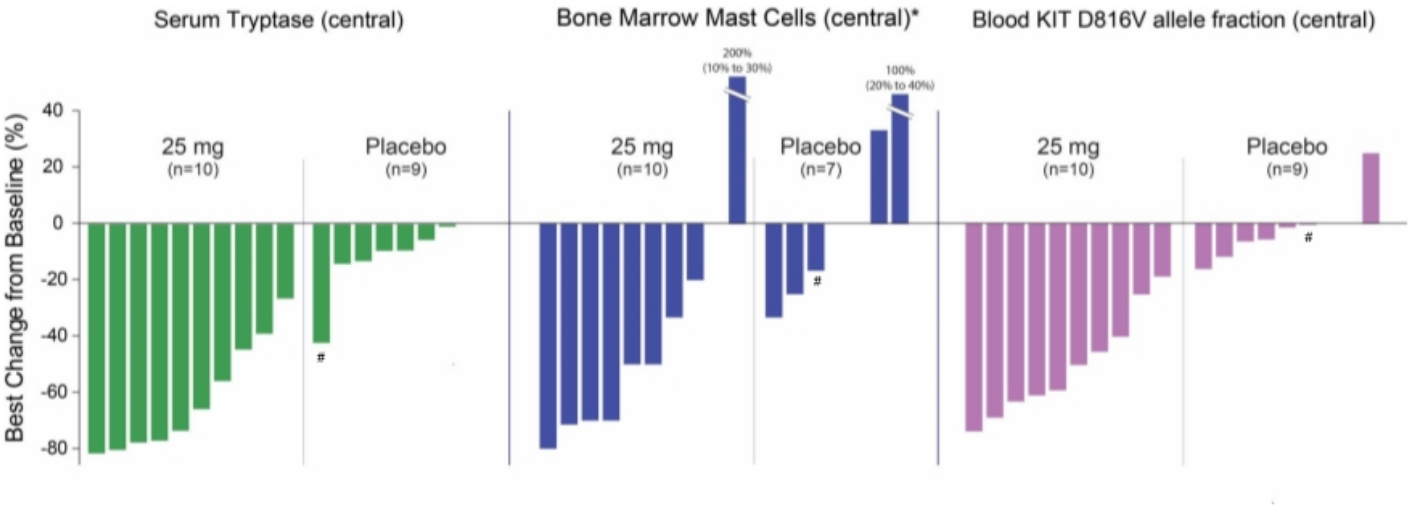
Placebo

- 0% with mild disease at 16 weeks
- 50% worsened

= Baseline = Week 16

[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.

Avapritinib improves objective measures of mast cell burden assessed



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

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.

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

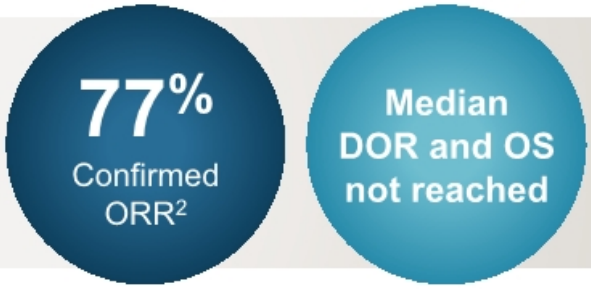
Duration: ~6 months

Timeline: Plan to initiate patient screening in June 2020

EXPLORER trial results: Remarkable response rate and prolonged duration of response in patients with advanced SM

- FDA breakthrough therapy designation[3]
- Robust activity across all disease subtypes
- Median follow up of 21 months with ongoing treatment up to ~3.5 years[1]

BEST RESPONSE PER IWG-MRT-ECNM CRITERIA
ALL DOSES (N=48)[1]



SAFETY
ALL DOSES (N=80)[1]

- Avapritinib was generally well-tolerated, and most AEs were grade 1 or 2[4]
- Most common treatment-emergent AEs were periorbital edema, anemia, diarrhea, fatigue, peripheral edema, nausea, thrombocytopenia, vomiting and cognitive effects
- Across all doses, 6 patients discontinued treatment due to treatment-related AEs

1. EXPLORER trial data reported on December 8, 2019. Data cutoff: August 30, 2019. 2. ORR defined as complete remission with full or partial recovery of peripheral blood counts, partial remission or clinical improvement. 3. Avapritinib granted Breakthrough Therapy Designation for the treatment of advanced SM, including the subtypes of aggressive SM, SM with an associated hematologic neoplasm and mast cell leukemia. 4. After the data cutoff date, one patient with SM and an associated hematologic neoplasm (SM-AHN) of myelodysplastic syndrome had a Grade 5 intracranial bleed. At the time of the bleeding event, the patient had severe thrombocytopenia and experienced a serious injury involving head trauma. DOR, duration of response; OS, overall survival.

Fourth quarter & full year 2019 financial results

Balance Sheet (unaudited)	FY '19	FY '18
Cash, Cash Equivalents and Investments	$548.0M	$494.0M

Statement of Operations (unaudited)	Q4 '19	Q4 '18	FY '19	FY '18
Collaboration Revenue	$51.5M	$1.0M	$66.5M	$44.5M
Research & Development Expenses	$88.6M	$70.5M	$331.5M	$243.6M
General & Administrative Expenses	$32.3M	$13.6M	$96.4M	$47.9M
Net Loss	$(66.3)M	$(80.3)M	$(347.7)M	$(236.6)M

Estimated net proceeds of $308.2M from January 2020 follow-on public offering

Based on current operating plans, expect existing cash balance will fund operations into 2H of 2022*

* Includes January 2020 follow-on public offering and anticipated product revenues. Excludes any potential option fees, milestone payments or other payments under collaboration or license agreements.

Exhibit 99.2

Blueprint Medicines Announces the Achievement of Key Portfolio Milestones

-- Top-line ARROW trial data for pralsetinib show 60% overall response rate and 18-month duration of response rate of 90% in previously treated RET-mutant medullary thyroid cancer; plan to submit NDA to FDA in Q2 2020 --

--74% overall response rate in treatment-naïve RET-mutant medullary thyroid cancer and 89% overall response rate in RET fusion-positive thyroid cancer --

-- NDA submitted to FDA for pralsetinib for RET fusion-positive non-small cell lung cancer --

-- IND application for BLU-263 in indolent systemic mastocytosis submitted to FDA --

CAMBRIDGE, Mass., April 1, 2020 – Blueprint Medicines Corporation (NASDAQ: BPMC), a precision therapy company focused on genomically defined cancers, rare diseases and cancer immunotherapy, today announced the achievement of key milestones reflecting portfolio-wide progress against the company's 2020 goals. These milestones include the compilation of top-line data for pralsetinib in patients with RET-mutant medullary thyroid cancer (MTC), supporting plans to submit a new drug application (NDA) to the U.S. Food and Drug Administration (FDA) in the second quarter of 2020.

"As our company and the communities we serve face the COVID-19 pandemic, I am exceptionally proud of our team's nimbleness and persistency in advancing multiple therapies across our portfolio for the patients who need them," said Andy Boral, M.D., Ph.D., Chief Medical Officer at Blueprint Medicines. "I am particularly encouraged that we have advanced pralsetinib toward registration across multiple tumor types and have made strong progress on the avapritinib program, with a compelling dataset in patients with systemic mastocytosis reported last month. The top-line data announced today demonstrate the potential of pralsetinib to be a best-in-class therapy for patients with RET-altered thyroid cancers, with deep and durable responses in both the first-line and relapsed settings."

Top-line Data from Phase 1/2 ARROW Trial in RET-Altered Thyroid Cancers

Top-line results announced today support Blueprint Medicines' plans to submit an NDA for pralsetinib in patients with RET-mutant MTC previously treated with an approved multi-kinase inhibitor (MKI) in the second quarter of 2020. The registration endpoints are overall response rate (ORR) and duration of response (DOR), based on independent central radiology and Response Evaluation Criteria in Solid Tumors version 1.1 (RECIST 1.1) criteria.

Top-line efficacy data were reported for patients treated with pralsetinib who were evaluable for response assessment per RECIST 1.1, as determined by blinded independent central review. All patients received the proposed indicated dose of 400 mg once daily (QD). All results were as of a data cutoff date of February 13, 2020.

In 53 patients with RET-mutant MTC previously treated with cabozantinib or vandetanib, the ORR was 60 percent (95% CI: 46-74%) with one response pending confirmation. Nearly all patients (98 percent) had tumor shrinkage. The median DOR was not reached (95% CI: not estimable, not estimable), and the 18-month DOR rate was 90 percent (95% CI: 77-100%).

In addition, the top-line data showed robust clinical activity in treatment-naïve patients, supporting the potential of pralsetinib across lines of therapy. In 19 patients with RET-mutant MTC who had not received prior systemic treatment, the confirmed ORR was 74 percent (95% CI: 49-91%), and all patients had tumor shrinkage. The median DOR was not reached (95% CI: 7 months, not estimable), with 12 of 14 responders remaining in response for up to 15 months as of the data cutoff date.

In nine patients with RET fusion-positive thyroid cancer, the confirmed ORR was 89 percent (95% CI: 52-100%), and all patients had tumor shrinkage. The median DOR was not reached (95% CI: 8 months, not estimable), with seven of eight responders remaining in response for up to 20 months as of the data cutoff date.

Top-line safety data were consistent with those previously reported. Pralsetinib was well-tolerated, and most treatment-related adverse events (AEs) were Grade 1 or 2. Across all patients enrolled in the ARROW trial treated at the proposed indicated dose of 400 mg QD (N=438), only 4 percent discontinued treatment with pralsetinib due to treatment-related AEs.

Blueprint Medicines plans to present the full data at a scientific meeting this year.

NDA Submission for Pralsetinib for RET Fusion-Positive NSCLC

Blueprint Medicines completed the rolling NDA submission for pralsetinib for RET fusion-positive non-small cell lung cancer (NSCLC). Blueprint Medicines requested priority review for the application, which, if granted, could result in a six-month review process.

Top-line Data from Phase 3 VOYAGER Trial in Third-Line GIST

Blueprint Medicines plans to lock the VOYAGER trial database in April and provide top-line data to the FDA for avapritinib in third-line gastrointestinal stromal tumor (GIST), to enable the FDA to take action on the proposed fourth-line GIST indication by the May 14, 2020 PDUFA date.

Submission of IND Application for BLU-263

Blueprint Medicines submitted an investigational new drug (IND) application to the FDA for BLU-263, a next-generation KIT inhibitor, for the treatment of patients with indolent systemic mastocytosis (SM). With its drug candidates avapritinib and BLU-263, Blueprint Medicines is pursuing a comprehensive strategy to address a broad population of patients with SM and other mast cell disorders.

About RET-Altered Solid Tumors

RET activating fusions and mutations are key disease drivers in many cancer types, including NSCLC and MTC. RET fusions are implicated in approximately 1 to 2 percent of patients with NSCLC and approximately 10 to 20 percent of patients with papillary thyroid cancer, while RET mutations are implicated in approximately 90 percent of patients with advanced MTC. In addition, oncogenic RET alterations are observed at low frequencies in colorectal, breast, pancreatic and other cancers, and RET fusions have been observed in patients with treatment-resistant EGFR-mutant NSCLC.

Currently, there are no approved therapies that selectively target RET-driven cancers, although there are several approved MKIs with RET activity being evaluated in clinical trials. To date, clinical activity attributable to RET inhibition has been uncertain for these approved MKIs, likely due to insufficient inhibition of RET and off-target toxicities. There is a need for precision therapies that provide durable clinical benefit by selectively targeting RET alterations and anticipated resistance mutations.

About Pralsetinib

Pralsetinib is an investigational, once-daily oral precision therapy specifically designed for highly potent and selective targeting of oncogenic RET alterations. Blueprint Medicines is developing pralsetinib for the treatment of patients with RET-altered NSCLC, MTC and other solid tumors. The FDA has granted Breakthrough Therapy Designation to pralsetinib for the treatment of RET fusion-positive NSCLC that has progressed following platinum-based chemotherapy, and RET mutation-positive MTC that requires systemic treatment and for which there are no acceptable alternative treatments.

Pralsetinib was designed by Blueprint Medicines' research team, leveraging the company's proprietary compound library. In preclinical studies, pralsetinib consistently demonstrated sub-nanomolar potency against the most common RET fusions, activating mutations and predicted resistance mutations. In addition, pralsetinib

demonstrated markedly improved selectivity for RET compared to pharmacologically relevant kinases, including approximately 90-fold improved potency for RET versus VEGFR2. By suppressing primary and secondary mutants, pralsetinib has the potential to overcome and prevent the emergence of clinical resistance. Blueprint Medicines believes this approach will enable durable clinical responses across a diverse range of RET alterations, with a favorable safety profile.

Blueprint Medicines has an exclusive collaboration and license agreement with CStone Pharmaceuticals for the development and commercialization of pralsetinib and certain other drug candidates in Mainland China, Hong Kong, Macau and Taiwan. Blueprint Medicines retains development and commercial rights for pralsetinib in the rest of the world.

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 pralsetinib and BLU-263, including the timing, design, implementation, initiation, enrollment, plans and announcement of results regarding Blueprint Medicines' ongoing and planned clinical trials for pralsetinib and BLU-263; plans and timelines for submitting marketing applications for pralsetinib; the potential benefits of Blueprint Medicines' current and future approved drugs or drug candidates in treating patients; 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 the impact of the COVID-19 pandemic to Blueprint Medicines' business, operations, strategy, goals and anticipated milestones, including Blueprint Medicines' ongoing and planned research and discovery activities, ability to conduct ongoing and planned clinical trials, clinical supply of current or future drug candidates, commercial supply of current or future approved products, and launching, marketing and selling current or future approved products; Blueprint Medicines' ability and plan in establishing a commercial infrastructure, and successfully launching, marketing and selling its approved product; the delay of any current or planned clinical trials or the development of Blueprint Medicines' drug candidates or licensed drug candidate; 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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