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): **June 6, 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 8.01 Other Events.

On June 6, 2020, Blueprint Medicines Corporation issued a press release announcing updated data from the dose-finding portion (part 1) of its ongoing Phase 2 PIONEER clinical trial in patients with indolent systemic mastocytosis. These data were presented during the European Academy of Allergy and Clinical Immunology Digital Congress 2020. A copy of the press release is filed herewith as Exhibit 99.1 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	Press release issued by Blueprint Medicines Corporation on June 6, 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: June 8, 2020

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

Exhibit 99.1

Blueprint Medicines Presents Updated Part 1 Data from PIONEER Trial of Avapritinib Showing Robust Reductions in Cutaneous Disease Symptoms in Patients with Indolent Systemic Mastocytosis

-- Updated PIONEER trial data in patients with indolent SM showed response rate of 60% for avapritinib versus 0% for placebo at 24 weeks, with response defined as ≥30% reduction in total symptom score --

-- Plan to present updated data from EXPLORER trial of avapritinib in patients with advanced SM at the European Hematology Association Virtual Congress on June 12 --

-- Initiated Phase 1 trial of next-generation KIT inhibitor BLU-263 in healthy volunteers --

CAMBRIDGE, Mass., June 6, 2020 – Blueprint Medicines Corporation (NASDAQ: BPMC), a precision therapy company focused on genomically defined cancers, rare diseases and cancer immunotherapy, today announced updated clinical data from part 1 of the PIONEER trial showing robust and consistent clinical activity for avapritinib across multiple qualitative and quantitative measures of cutaneous disease in patients with indolent systemic mastocytosis (SM). Additional data showed avapritinib treatment resulted in deepening improvements in overall disease symptoms, as measured by the Indolent SM Symptom Assessment Form (ISM-SAF) total symptom score (TSS), and was well-tolerated through 24 weeks of follow-up. These data were presented today during the European Academy of Allergy and Clinical Immunology (EAACI) Digital Congress 2020.

SM is a rare disease driven by the KIT D816V mutation in nearly all patients and characterized by uncontrolled mast cell proliferation and activation. The disorder can lead to debilitating systemic, gastrointestinal and neurocognitive symptoms, including life-threatening anaphylaxis. Additional skin manifestations such as itching, flushing and pigmented skin lesions are common and can significantly impact quality of life. Avapritinib is a potent and highly selective inhibitor of D816V mutant KIT.

“Patients with indolent systemic mastocytosis often suffer from extensive skin lesions and severe mediator symptoms that can be life-threatening,” said Karin Hartmann, M.D., Professor of Medicine and Head of the Division of Allergy at the University of Basel and an investigator on the PIONEER trial. “The updated PIONEER trial data showed that patients treated with avapritinib had clinically meaningful reductions in mediator symptoms, patient-reported quality of life and mast cell burden across multiple measures. With further evidence that these reductions deepen over time, avapritinib has the potential to provide important clinical benefits to patients with this debilitating disease.”

“These promising data reinforce our commitment to rapidly advance development of avapritinib to meet persistent medical needs in indolent systemic mastocytosis, which are poorly addressed by a patchwork of available symptomatic therapies,” said Andy Boral, M.D., Ph.D., Chief Medical Officer at Blueprint Medicines. “Avapritinib was specifically designed to potently target the underlying disease driver of systemic mastocytosis and has the potential to fundamentally change the treatment of this disease.”

The presentation is available on-demand via the EAACI Digital Congress 2020 website at www.eaaci.org/eaaci-congresses/eaaci-2020.

Highlights from the EAACI Presentation of PIONEER Trial Data

Previously reported data from part 1 of the PIONEER trial showed that treatment with avapritinib was well-tolerated and resulted in robust and clinically meaningful improvements on measures of mast cell burden, disease symptoms and patient-reported quality of life through 16 weeks. Based on these data, avapritinib 25 mg once daily (QD) was selected as the recommended part 2 dose. Updated data on disease symptoms through 24 weeks and new skin assessment results were reported in the EAACI presentation.

Updated clinical activity and safety data

As of a data cutoff date of March 31, 2020, updated data from part 1 of the PIONEER trial showed a deepening of symptom reductions in patients treated with avapritinib through 24 weeks of follow-up. The mean percent change from baseline in ISM-SAF TSS was -35 percent in patients treated with avapritinib 25 mg QD (n=10) compared to -4 percent in patients treated with placebo. In addition, the mean percent change from baseline in ISM-SAF skin domain score was -38 percent for avapritinib 25 mg QD versus +11 percent for placebo.

The updated data also showed a 60 percent response rate in patients treated with avapritinib 25 mg QD compared to a 0 percent response rate in patients treated with placebo at 24 weeks, with response defined as a 30 percent or greater reduction in ISM-SAF TSS. Based on these data and feedback from the U.S. Food and Drug Administration (FDA), Blueprint Medicines has selected response rate at 24 weeks as the primary endpoint for the registration-enabling part 2 of the PIONEER trial and plans to enroll approximately 200 patients. Blueprint Medicines continues to plan to initiate patient screening in part 2 of the PIONEER trial in June 2020.

As of a data cutoff date of March 31, 2020, avapritinib 25 mg QD was well-tolerated and safety results were consistent with previously reported data, with no Grade ≥3 adverse events or discontinuations due to adverse events.

Additional clinical activity data on measures of skin disease

High resolution skin photographs were taken at baseline and every 12 weeks during treatment for patients with significant cutaneous involvement who consented to photography. To assess changes in skin disease, photographs were assessed by a blinded independent review committee and a computational image analysis algorithm. Images and data as of a cutoff of March 31, 2020 were evaluated by the independent committee.

Based on skin photography at 24 weeks or the last available assessment, results showed that skin lesions lightened in 71 percent of patients treated with avapritinib (n=17; all doses) compared to 25 percent of patients treated with placebo (n=8), per blinded review by the independent committee. In addition, the median percent change from baseline in most affected surface area was -35 percent for avapritinib (n=18; all doses) compared to -8 percent for placebo (n=8), based on a computational image analysis algorithm.

Mast cell infiltration in skin lesions was also assessed by lesional skin biopsies obtained at baseline and 12 weeks. The median percent change from baseline in mast cell infiltration was -46 percent for avapritinib (n=18; all doses) compared to +51 percent for placebo (n=7).

Phase 1 Trial of BLU-263 in Healthy Volunteers

Blueprint Medicines today announced it has initiated dosing in a Phase 1 trial of BLU-263, an investigational next-generation KIT inhibitor, in healthy volunteers. Following completion of the Phase 1 trial and the analysis of data, the company plans to initiate clinical development of BLU-263 in patients with indolent SM, with the goal of complementing the ongoing avapritinib development program.

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. by the FDA or for any indication in any other jurisdiction by any other health authority.

Blueprint Medicines is developing avapritinib globally for the treatment of advanced, smoldering and indolent SM. The FDA granted Breakthrough Therapy Designation to avapritinib for the treatment of advanced SM, including the subtypes of aggressive SM, SM with an associated hematologic neoplasm and mast cell leukemia.

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

About the Phase 2 PIONEER Trial

PIONEER is a randomized, double-blind, placebo-controlled, registration-enabling trial evaluating avapritinib in patients with indolent 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, patient-reported quality of life, 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.

Patients and healthcare providers with questions about opportunities to participate in the PIONEER trial can contact the Blueprint Medicines study director at studydirector@blueprintmedicines.com or 1-617-714-6707. Additional details are also available at www.blueprintclinicaltrials.com 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 and BLU-

263; plans, timelines and expectations for initiating patient screening in part 2 of the PIONEER trial; expectations regarding the potential benefits of avapritinib and BLU-263 in treating patients with SM and mast cell disorders; 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 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 current or future approved products; the delay of any current or planned clinical trials or the development of Blueprint Medicines' drug candidates or licensed product 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.

Investor Relations Contact

Kristin Hodous
617-714-6674
ir@blueprintmedicines.com

Media Relations Contact

Andrew Law
617-844-8205
media@blueprintmedicines.com