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): **December 17, 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 December 17, 2020, Blueprint Medicines Corporation (the "Company") issued a press release announcing the submission of a supplemental new drug application ("sNDA") to the U.S. Food and Drug Administration ("FDA)" for AYVAKIT™ (avapritinib) for the treatment of adult patients with advanced systemic mastocytosis. The Company has requested priority review for the sNDA, which, if granted, could result in a six-month review process. The FDA has a 60-day filing review period to determine whether the NDA is complete and acceptable for filing. 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 December 17, 2020
104	Cover Page Interactive Data File (embedded within the Inline XBRL document and incorporated as Exhibit 101)

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: December 17, 2020

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

Blueprint Medicines Submits Supplemental New Drug Application to FDA for AYVAKIT™ (avapritinib) for the Treatment of Advanced Systemic Mastocytosis

CAMBRIDGE, Mass., December 17, 2020 -- Blueprint Medicines Corporation (NASDAQ: BPMC), a precision therapy company focused on genomically defined cancers, rare diseases and cancer immunotherapy, today announced the submission of a supplemental new drug application (sNDA) to the U.S. Food and Drug Administration (FDA) for AYVAKIT™ (avapritinib) for the treatment of adult patients with advanced systemic mastocytosis (SM). AYVAKIT is a potent and selective inhibitor of D816V mutant KIT, the primary driver of SM, and is being developed to treat advanced and non-advanced forms of the disease.

Blueprint Medicines requested priority review for this application, which, if granted, could result in a six-month review process. The FDA has a 60-day filing review period to determine whether the sNDA is complete and acceptable for filing. The FDA granted breakthrough therapy designation to AYVAKIT for the treatment of advanced SM, including the subtypes of aggressive SM, SM with an associated hematological neoplasm and mast cell leukemia.

“Today’s submission is an important step toward our goal of bringing AYVAKIT to patients with advanced systemic mastocytosis, a debilitating and life-threatening rare disease,” said Fouad Namouni, M.D., President, Research & Development. “Our application is based on an unprecedented clinical dataset in this disease, which showed that patients receiving AYVAKIT had high overall response and complete remission rates, with prolonged survival, and the treatment was generally well-tolerated. We look forward to working closely with the FDA during the review, as we seek to introduce the first precision therapy targeting the underlying cause of systemic mastocytosis.”

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 hematological 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, including anaphylaxis, maculopapular rash, pruritis, diarrhea, brain fog, fatigue and bone pain, often persist across all forms of SM despite treatment with a number of symptomatic therapies. Patients often live in fear of severe, unexpected symptoms, have limited ability to work or perform daily activities, or isolate themselves to protect against unpredictable triggers. Currently, there are no approved therapies for the treatment of SM 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 www.AYVAKIT.com. This medicine is approved in Europe under the brand name AYVAKYT® for the treatment of adults with unresectable or metastatic GIST harboring the PDGFRA D842V mutation.

AYVAKIT/AYVAKYT is not approved for the treatment of any other indication, including SM, in the U.S. by the FDA or in Europe by the European Commission, or for any indication in any other jurisdiction by any other health authority.

Blueprint Medicines is developing AYVAKIT globally for the treatment of advanced and indolent SM.

Blueprint Medicines has an exclusive collaboration and license agreement with CStone Pharmaceuticals for the development and commercialization of AYVAKIT in Mainland China, Hong Kong, Macau and Taiwan. Blueprint Medicines retains development and commercial rights for AYVAKIT 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 two approved precision therapies and are currently advancing multiple investigational medicines in clinical and pre-clinical development, along with a number of earlier-stage 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 AYVAKIT for advanced and non-advanced SM; plans and timelines for commercializing AYVAKIT for advanced SM, if approved; the potential benefits of Blueprint Medicines' current and future approved drugs or drug candidates in treating patients, including expectations regarding the potential of AYVAKIT for the treatment of 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 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 plans in continuing to establish and maintain a commercial infrastructure, and successfully launching, marketing and selling current or future approved products; Blueprint Medicines' ability to successfully expand the approved indications for AYVAKIT/AYVAKYT and GAVRETO™ (pralsetinib) or obtain marketing approval for AYVAKIT/AYVAKYT and GAVRETO in additional geographies in the future; the delay of any current or planned clinical trials or the development of Blueprint Medicines' current or future drug candidates; 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 pre-clinical 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 obtain, maintain and enforce patent and other intellectual property protection for AYVAKIT/AYVAKYT, GAVRETO or any drug candidates it is developing; Blueprint Medicines' ability to develop and commercialize companion diagnostic tests for AYVAKIT/AYVAKYT, GAVRETO or any of its current and future drug candidates; and the success of Blueprint Medicines' current and future collaborations, partnerships 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.

Trademarks

Blueprint Medicines, AYVAKIT, AYVAKYT, GAVRETO and associated logos are trademarks of Blueprint Medicines Corporation.

Media Contact

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

Investor Relations Contact

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