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): **October 25, 2019**

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 October 25, 2019, Blueprint Medicines Corporation (the "Company") received written feedback from the U.S. Food and Drug Administration ("FDA") following its mid-cycle review meeting for the Company's new drug application ("NDA") for avapritinib for the treatment of adults with PDGFRA Exon 18 mutant gastrointestinal stromal tumors ("GIST"), regardless of prior therapy, and fourth-line GIST. On October 28, 2019, the Company issued a press release announcing updates regarding the FDA's review of the NDA and certain program updates related to the Company's current and planned clinical programs for avapritinib for the treatment of third-line and second-line GIST. A copy of the press release is filed herewith as Exhibit 99.1 and is 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 October 28, 2019
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: October 28, 2019

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

Blueprint Medicines Announces FDA Intent to Split Avapritinib New Drug Application into Separate Submissions for PDGFRA Exon 18 Mutant GIST and Fourth-Line GIST

-- Given proximity of NDA action date to anticipated top-line data readout for Phase 3 VOYAGER trial, FDA requested VOYAGER top-line data to inform its review of fourth-line GIST indication --

-- Plan to report top-line VOYAGER trial data in Q2 2020 --

CAMBRIDGE, Mass., October 28, 2019 /PRNewswire/ -- Blueprint Medicines Corporation (NASDAQ: BPMC), a precision therapy company focused on genomically defined cancers, rare diseases and cancer immunotherapy, today announced an update regarding its New Drug Application (NDA) for avapritinib for the treatment of adults with PDGFRA Exon 18 mutant GIST, regardless of prior therapy, and fourth-line GIST.

On October 25, 2019, Blueprint Medicines received written feedback from the U.S. Food and Drug Administration (FDA) following its NDA mid-cycle review meeting. The FDA informed Blueprint Medicines that it intends to administratively split the proposed indications for avapritinib into two separate NDAs, one for PDGFRA Exon 18 mutant GIST, regardless of prior therapy, and one for fourth-line GIST. Given the acceleration of the ongoing Phase 3 VOYAGER clinical trial of avapritinib in patients with third- and fourth-line GIST and the anticipated availability of top-line data in the second quarter of 2020, the FDA requested top-line data from the VOYAGER trial. The FDA indicated these data would be informative in its review of the proposed fourth-line indication and potential clinical benefit in this population, including its evaluation of response rate and safety for the fourth-line indication. An extension of the review period for the fourth-line GIST NDA will likely be required to enable Blueprint Medicines to provide the top-line VOYAGER data to the FDA.

“Throughout the development of avapritinib, we have had a productive and collaborative dialogue with the FDA about the potential of avapritinib to address important medical needs in subsets of patients with advanced GIST,” said Jeff Albers, Chief Executive Officer of Blueprint Medicines. “We plan to continue to work closely with the FDA during its review of the separate NDAs for PDGFRA Exon 18 mutant GIST and fourth-line GIST, and we plan to submit the requested VOYAGER trial data as expeditiously as possible.”

Blueprint Medicines has completed patient screening in the VOYAGER trial of avapritinib in patients with third- and fourth-line GIST and expects to complete patient enrollment by the end of November 2019 and report top-line data in the second quarter of 2020. Blueprint Medicines plans to prioritize completion of the VOYAGER trial and delay initiation of its COMPASS-2L trial in second-line GIST. Subject to an initial approval of avapritinib, Blueprint Medicines plans to submit a supplemental NDA to the FDA for avapritinib for third-line GIST in the second half of 2020.

“We are focusing our strategy and resourcing for the avapritinib clinical development program on three core activities: completing the VOYAGER trial and supporting the FDA’s review of NDAs for PDGFRA Exon 18 mutant GIST and fourth-line GIST, submitting an NDA for advanced systemic mastocytosis in the first quarter of 2020, and submitting an NDA for third-line GIST in the second half of 2020,” said Andy Boral, MD, PhD, Chief Medical Officer of Blueprint Medicines. “We remain committed to exploring the potential of avapritinib in patients with second-line GIST and working with the GIST community to advance precision medicine approaches that can transform patient care, both of which may be further

enabled by a robust dataset from the VOYAGER trial including nearly 500 patients with third- and fourth-line GIST."

About Avapritinib

Avapritinib is an investigational, oral precision therapy that selectively and potently inhibits KIT and PDGFRA mutant kinases. It is a type 1 inhibitor designed to target the active kinase conformation; all oncogenic kinases signal via this conformation. 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.

Blueprint Medicines is initially developing avapritinib for the treatment of advanced GIST, advanced systemic mastocytosis (SM), and indolent and smoldering SM. The FDA has granted Breakthrough Therapy Designation to avapritinib for two indications: one for the treatment of unresectable or metastatic GIST harboring the PDGFRA D842V mutation and one 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 GIST

GIST is a sarcoma, or tumor of bone or connective tissue, of the gastrointestinal (GI) tract. Tumors arise from cells in the wall of the GI tract and occur most often in the stomach or small intestine. Most patients are diagnosed between the ages of 50 to 80, and diagnosis is typically triggered by GI bleeding, incidental findings during surgery or imaging and, in rare cases, tumor rupture or GI obstruction.

Most GIST cases are caused by a spectrum of clinically relevant mutations that force the KIT or PDGFRA protein kinases into an increasingly active state. Because currently available therapies primarily bind to the inactive protein conformations, certain primary and secondary mutations typically lead to treatment resistance and disease progression.

In unresectable or metastatic GIST, clinical benefits from existing treatments can vary by mutation type. Mutational testing is critical to tailor therapy to the underlying disease driver and is recommended in expert guidelines. Currently, there are no approved therapies for patients with KIT-driven GIST whose disease progresses beyond imatinib, sunitinib and regorafenib. In patients with metastatic PDGFRA D842V-driven GIST, progression occurs in a median of approximately three to four months with available therapy.

About the Phase 3 VOYAGER Clinical Trial

The VOYAGER clinical trial is a global, open-label, randomized, Phase 3 trial designed to evaluate the safety and efficacy of avapritinib versus regorafenib in patients with third- or fourth-line advanced GIST. Eligible patients will have previously received imatinib and one or two additional tyrosine kinase inhibitors. The trial is designed to enroll approximately 460 patients randomized 1:1 to receive

avapritinib dosed at 300 mg once daily (QD) or regorafenib dosed at 160 mg QD for three weeks, followed by one week off, at multiple sites in the United States, European Union, Australia and Asia. Patients who are randomized to receive regorafenib and experience disease progression confirmed by central radiology review may be offered the opportunity to cross-over to the avapritinib treatment arm. The primary efficacy endpoint is progression free survival determined by central radiologic assessment per modified Response Evaluation Criteria in Solid Tumors (RECIST) version 1.1. Secondary endpoints include objective response rate, overall survival and quality of life outcome measures. Regorafenib, also known as Stivarga®, is an oral, multi-kinase inhibitor approved by the FDA for the treatment of patients with third-line GIST. Additional trial details are also available on www.clinicaltrials.gov (ClinicalTrials.gov Identifier: NCT03465722).

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 are currently advancing three investigational medicines in clinical development, along with multiple 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 the potential benefits of avapritinib in treating patients with GIST; plans, timelines and expectations for the FDA's review and administrative split of the NDA for avapritinib for the treatment of adult patients with PDGFRA Exon 18 mutant GIST, regardless of prior therapy, and fourth-line GIST; plans, timelines and expectations for top-line data from the VOYAGER trial; plans to prioritize completion of the VOYAGER trial; plans to delay initiation of the COMPASS-2L trial in second-line GIST; plans and timelines for submitting a supplemental NDA to the FDA for avapritinib for third-line GIST; plans, timelines and expectations for the commercialization of avapritinib for the treatment of GIST, if approved by the FDA; and Blueprint Medicines' strategy, goals and anticipated milestones, business plans and focus. The words "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 FDA's intent to administratively split the proposed indications for avapritinib into two separate NDAs, which may not mean that either indication is approved; a delay in the review of the proposed indications as a result of the administrative split of the current NDA; FDA concerns regarding whether the response rate in the fourth-line GIST population was reasonably likely to predict clinical benefit in that population; there can be no assurance that the FDA will not ask for additional clinical trials for avapritinib; there can be no assurance that the VOYAGER top-line data will be sufficient for the FDA's review of the proposed fourth-line indication or that there will not be a delay in the

availability of VOYAGER top-line data; the delay of any current or planned clinical trials or the development of Blueprint Medicines' drug candidates, including avapritinib; 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 or the regulatory pathway; Blueprint Medicines' ability to develop and commercialize companion diagnostic tests for its current and future drug candidates, including avapritinib; 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 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

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