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): **February 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 February 6, 2020, Blueprint Medicines Corporation (the "Company") issued a press release announcing that the U.S. Food and Drug Administration ("FDA") has extended the Prescription Drug User Fee Act ("PDUFA") date for the Company's new drug application ("NDA") seeking accelerated approval of avapritinib for the treatment of adults with fourth-line gastrointestinal stromal tumor ("GIST"). The FDA extended the PDUFA action date from February 14, 2020 to May 14, 2020. As previously reported, the FDA has requested top-line data from the Company's ongoing Phase 3 VOYAGER clinical trial, which is comparing avapritinib to regorafenib in third-line or fourth-line GIST, to inform the pending action on the NDA for fourth-line GIST. The Company expects to provide the top-line data to the FDA early in the second quarter of 2020 to enable the FDA to take action by the PDUFA action date.

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 February 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: February 6, 2020

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

Blueprint Medicines Announces PDUFA Date Extension for New Drug Application of Avapritinib for the Treatment of Adults with Fourth-Line Gastrointestinal Stromal Tumor

-- PDUFA action date extended by three months to May 14, 2020 --

-- NCCN guidelines now include avapritinib as recommended treatment for PDGFRA exon 18 mutant GIST and fourth-line GIST --

CAMBRIDGE, Mass., February 6, 2020 – Blueprint Medicines Corporation (NASDAQ: BPMC), a precision therapy company focused on genomically defined cancers, rare diseases and cancer immunotherapy, today announced that the U.S. Food and Drug Administration (FDA) has extended the Prescription Drug User Fee Act (PDUFA) date for its New Drug Application (NDA) seeking accelerated approval of avapritinib for the treatment of adults with fourth-line gastrointestinal stromal tumor (GIST). The FDA extended the PDUFA action date by three months from February 14, 2020 to May 14, 2020.

As previously announced, the FDA has requested top-line data from Blueprint Medicines' ongoing Phase 3 VOYAGER clinical trial, which is comparing avapritinib to regorafenib in third- or fourth-line GIST, to inform the pending action on the NDA for fourth-line GIST. Blueprint Medicines expects to provide the top-line data to the FDA early in the second quarter of 2020 to enable the FDA to take action by the May 14, 2020 PDUFA date.

In addition, Blueprint Medicines today announced that the National Comprehensive Cancer Network has updated its Clinical Practice Guidelines in Oncology (NCCN Guidelines®) for Soft Tissue Sarcoma to include avapritinib as a recommended category 2A treatment for unresectable or metastatic GIST with a PDGFRA exon 18 mutation, including PDGFRA D842V mutations, as well as fourth-line GIST. This rating indicates that there is uniform NCCN consensus that the intervention is appropriate. The NCCN Guidelines are the recognized clinical standard for cancer care by U.S. healthcare providers and payers, and are maintained by a committee of expert physicians from leading U.S. cancer centers.

About AYVAKIT™ (avapritinib)

AYVAKIT (avapritinib) is a kinase inhibitor approved by the FDA for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations. AYVAKIT is a selective and potent inhibitor of KIT and PDGFRA mutant kinases. It is the only FDA-approved type 1 inhibitor for GIST that works by directly binding to the active kinase conformation from which mutant KIT and PDGFRA signal. AYVAKIT has demonstrated inhibition of a broad range of KIT and PDGFRA mutations associated with GIST, including potent clinical activity against activation loop mutations that are associated with resistance to currently approved therapies. For more information, visit AYVAKIT.com.

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

Blueprint Medicines is pursuing a broad clinical development program for avapritinib across multiple lines of GIST treatment, as well as for advanced, smoldering and indolent systemic mastocytosis (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. For more information about avapritinib clinical trials, visit www.clinicaltrials.gov or www.blueprintclinicaltrials.com.

Important Safety Information

Intracranial hemorrhage (e.g., subdural hematoma, intracranial hemorrhage, and cerebral hemorrhage) occurred in 1% of 267 patients (0.7% Grade 3 or 4) with GIST and overall in 3% of 335 patients (1.2% Grade 3 or 4) who received AYVAKIT. Overall, 0.9% of patients receiving AYVAKIT required permanent discontinuation for an intracranial hemorrhage. Withhold AYVAKIT and then resume at a reduced dose upon resolution, or permanently discontinue AYVAKIT based on severity.

In 335 patients receiving AYVAKIT, CNS adverse reactions occurred overall in 58% of patients including cognitive impairment (41%; 3.6% Grade 3 or 4), dizziness (20%; 0.6% Grade 3 or 4), sleep disorders (15%; 0.3% Grade 3 or 4), mood disorders (13%; 1.5% Grade 3 or 4), speech disorders (6%; none Grade 3 or 4), and hallucinations (2.1%; none Grade 3 or 4). Overall, 3.9% of patients required permanent discontinuation of AYVAKIT for a CNS adverse reaction. Depending on severity, withhold AYVAKIT and then resume at the same dose or at a reduced dose upon improvement, or permanently discontinue AYVAKIT.

AYVAKIT can cause fetal harm when administered to a pregnant woman. Advise females of reproductive potential and pregnant women of the potential risk to a fetus. Advise females and males of reproductive potential to use an effective method of contraception during

treatment with AYVAKIT and for 6 weeks after the final dose of AYVAKIT. Advise women not to breastfeed during treatment with AYVAKIT and for two weeks after the final dose. Advise females and males of reproductive potential that AYVAKIT may impair fertility.

In 204 patients with unresectable or metastatic GIST, the most common adverse reactions (≥ 20%) were edema, nausea, fatigue/asthenia, cognitive impairment, vomiting, decreased appetite, diarrhea, hair color changes, increased lacrimation, abdominal pain, constipation, rash and dizziness.

Avoid coadministration of AYVAKIT with strong and moderate CYP3A inhibitors. If coadministration with a moderate CYP3A inhibitor cannot be avoided, reduce dose of AYVAKIT. Avoid coadministration of AYVAKIT with strong and moderate CYP3A inducers.

Please click here to see the full **Prescribing Information** for AYVAKIT.

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 the potential benefits of avapritinib in treating patients with GIST; plans, timelines and expectations for top-line data from the VOYAGER trial; plans to prioritize completion of the VOYAGER trial; timelines and expectations regarding an FDA decision on the NDA for fourth-line GIST; 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 delay of any current or planned clinical trials or the development of Blueprint Medicines' drug candidates; 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, including its cancer immunotherapy collaboration with F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc., its collaboration with CStone Pharmaceuticals and its license to Clementia Pharmaceuticals. 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.

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