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): **May 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 2.02 Results of Operations and Financial Condition.

On May 6, 2020, Blueprint Medicines Corporation (the "Company") announced its financial results for the quarter ended March 31, 2020 and other business highlights. The full text of the press release issued in connection with the announcement is furnished as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated by reference herein.

The information in 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 9.01 Financial Statements and Exhibits.

(d) Exhibits.

The following exhibit relating to Item 2.02 of this Form 8-K shall be deemed to be furnished and not filed:

Exhibit No.	Description
99.1	Press release issued by Blueprint Medicines Corporation on May 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: May 6, 2020

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

Blueprint Medicines Reports First Quarter 2020 Financial Results

-- Submitted US and EU marketing applications for pralsetinib for RET fusion-positive NSCLC --

-- On track to submit NDA to FDA for pralsetinib for previously treated RET mutant MTC in Q2 2020 under FDA's Real-Time Oncology Review pilot program --

-- Multiple abstracts for pralsetinib in RET-altered cancers and avapritinib in systemic mastocytosis accepted for presentation at the ASCO Annual Meeting, EAACI 2020 Congress and EHA Annual Congress --

-- IND for BLU-263 for indolent systemic mastocytosis cleared by FDA --

-- AYVAKIT™ (avapritinib) launch in PDGFRA exon 18 mutant GIST underway with reported net product revenue of $3.5M in first partial quarter of launch --

CAMBRIDGE, Mass., May 6, 2020 – Blueprint Medicines Corporation (NASDAQ: BPMC), a precision therapy company focused on genomically defined cancers, rare diseases and cancer immunotherapy, today reported financial results and provided a business update for the first quarter ended March 31, 2020.

"Our progress in recent months is a testament to the agility of the Blueprint Medicines team and the strength of our fully-integrated business," said Jeff Albers, Chief Executive Officer of Blueprint Medicines. "Despite challenges related to the COVID-19 pandemic and top-line results from the VOYAGER trial, we broadly executed against our planned milestones, including the submission of marketing applications for pralsetinib for RET fusion-positive non-small cell lung cancer in the U.S. and Europe and the reporting of transformative datasets for avapritinib in systemic mastocytosis and pralsetinib in RET-altered cancers, paving the way for multiple planned launches through 2021."

First Quarter 2020 Highlights and Recent Updates

Avapritinib: systemic mastocytosis (SM)

- Announced results from Part 1 of the Phase 2 PIONEER trial in patients with indolent SM, which showed treatment with avapritinib at the recommended Part 2 dose of 25 mg once daily showed improvements in symptom scores, measures of mast cell burden and patient-reported quality of life. Avapritinib was well-tolerated, and no patients discontinued treatment due to an adverse event (AE). Read the press release here.

Avapritinib: gastrointestinal stromal tumors (GIST)

- Launched AYVAKIT in the U.S. for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutations, including PDGFRA D842V mutations, and achieved net product revenue of $3.5 million in the first partial quarter of the launch.
- Announced top-line data from the Phase 3 VOYAGER trial of avapritinib versus regorafenib in third- or fourth-line GIST. The VOYAGER trial did not meet its primary endpoint of an improvement in progression-free survival for avapritinib versus regorafenib. Based on the top-line results, Blueprint Medicines plans to discontinue further development of avapritinib in GIST indications other than PDGFRA exon 18 mutant GIST. Read the press release here.

Pralsetinib: RET-altered cancers

- Announced top-line data from the Phase 1/2 ARROW trial of pralsetinib in patients with RET fusion-positive non-small cell lung cancer (NSCLC). The top-line data showed overall response rates (ORRs) of 61 percent in patients previously treated with platinum-based chemotherapy and 73 percent in patients naïve to prior systemic therapy. In both populations, the median duration of response (DOR) was not reached. Pralsetinib was well-tolerated, and most AEs were grade 1 or 2. Read the press release here.
- Completed the submission of a new drug application (NDA) to the U.S. Food and Drug Administration (FDA) and a marketing authorization application to the European Medicines Agency for pralsetinib for the treatment of patients with RET fusion-positive NSCLC. In connection with the NDA submission, Blueprint Medicines plans to participate in the FDA's Project Orbis initiative, which provides a framework for concurrent submission and review of marketing applications for oncology drugs across participating global health authorities. Additional information about the FDA's Project Orbis initiative can be found here.
- Announced top-line data from the Phase 1/2 ARROW trial of pralsetinib in patients with RET mutant medullary thyroid cancer (MTC). The top-line data showed ORRs of 60 percent in patients previously treated with a multi-kinase inhibitor and 74 percent in patients naïve to prior systemic treatment. In both populations, the median DOR was not reached. Pralsetinib was well-tolerated, and most AEs were grade 1 or 2. Read the press release here.

BLU-263: SM

- Received clearance from the FDA for an investigational new drug (IND) application for BLU-263 for the treatment of patients with indolent SM.

Key Upcoming Milestones

The company expects to achieve the following near-term milestones:

- Submit an NDA to the FDA for pralsetinib for the treatment of patients with MTC previously treated with an approved multi-kinase inhibitor in the second quarter of 2020 under the FDA's Oncology Center of Excellence Real-Time Oncology Review pilot program (RTOR program). The FDA's RTOR program aims to explore a more efficient review process to ensure that safe and effective treatments are available to patients as early as possible, while maintaining and improving review quality by the FDA. Additional information about the FDA's RTOR program can be found here.
- Present updated data from the ARROW trial of pralsetinib in RET-altered cancers at the American Society of Clinical Oncology (ASCO) Annual Meeting.
- Present additional data from Part 1 of the PIONEER trial of avapritinib in indolent SM at the European Academy of Allergy and Clinical Immunology (EAACI) 2020 Congress.
- Present updated data from the EXPLORER trial of avapritinib in advanced SM at the European Hematology Association (EHA) 25th Annual Congress.
- Initiate patient screening for the registration-enabling Part 2 of the PIONEER trial of avapritinib in indolent SM in June 2020.
- Initiate a Phase 1 trial of BLU-263 in healthy volunteers in June 2020.
- Report top-line data from the EXPLORER and PATHFINDER trials of avapritinib in advanced SM in the third quarter of 2020.

Response to the COVID-19 Pandemic and Potential Business Impacts

Due to the evolving and uncertain global impacts of the COVID-19 pandemic, Blueprint Medicines cannot precisely determine or quantify the impact this pandemic will have on its business, operations (including clinical trials) and financial performance for the remainder of our fiscal year ending December 31, 2020 and beyond.

- For ongoing and planned clinical trials, while the company anticipates and has experienced some temporary delays or disruptions due to the COVID-19 pandemic, Blueprint Medicines is working with any impacted clinical trial sites to ensure study continuity.
- Blueprint Medicines currently has sufficient supply to meet anticipated global commercial and clinical development needs for avapritinib, pralsetinib, fisogatinib and BLU-263 through 2021. However, the COVID-19 pandemic could adversely impact Blueprint Medicines' suppliers and result in delays or disruptions in the company's current or future supply chain.
- Blueprint Medicines has shifted commercial and medical affairs field activities across its portfolio toward virtual formats where possible in order to allow the company to continue to serve the needs of healthcare providers, patients and other stakeholders during this critical time. As the pandemic evolves, the company anticipates utilizing a mix of in-person and virtual engagement formats, as is locally and regionally appropriate.

Blueprint Medicines will continue to assess potential impacts of the COVID-19 pandemic and seek to advance the company's pipeline of targeted therapies as quickly as possible, while making the health and safety of the company's employees and their families, healthcare providers, patients and communities a top priority.

First Quarter 2020 Financial Results

- **Cash Position:** As of March 31, 2020, cash, cash equivalents and investments were $750.4 million, as compared to $548.0 million as of December 31, 2019. This increase was primarily related to $308.4 million in estimated net proceeds received from the company's January 2020 follow-on underwritten public offering, partially offset by cash used in operating activities.
- **Revenues:** Revenues were $6.2 million for the first quarter of 2020, including $3.5 million of net product revenues from sales of AYVAKIT and $2.7 million in collaboration revenues under the collaboration agreements with CStone and Roche. Blueprint Medicines recorded $0.7 million in collaboration revenues for the first quarter of 2019.
- **Cost of Sales:** Cost of sales was less than $0.1 million for the first quarter of 2020. Blueprint Medicines did not incur cost of sales in the first quarter of 2019 as no product sales were generated during that period.
- **R&D Expenses**: Research and development expenses were $84.1 million for the first quarter of 2020, as compared to $74.3 million for the first quarter of 2019. This increase was primarily due to increased clinical and manufacturing costs and personnel expenses driven by the progression of Blueprint Medicines' lead programs. Research and development expenses included $7.8 million in stock-based compensation expenses for the first quarter of 2020.
- **SG&A Expenses:** Selling, general and administrative expenses were $35.7 million for the first quarter of 2020, as compared to $16.6 million for the first quarter of 2019. This increase was primarily due to increased costs and personnel expenses associated with building Blueprint Medicines' commercial infrastructure for the commercialization of AYVAKIT and to support the

overall growth of the company's business. General and administrative expenses included $9.1 million in stock-based compensation expenses for the first quarter of 2020.

- **Net Loss:** Net loss was $111.0 million for the first quarter of 2020, or a net loss per share of $2.11, as compared to a net loss of $87.4 million for the first quarter of 2019, or a net loss per share of $1.98.

Financial Guidance

Based on its current operating plans, Blueprint Medicines expects that its existing cash, cash equivalents and investments, together with anticipated product revenues but excluding any potential option fees, milestone payments or other payments under its collaboration or license agreements, will be sufficient to enable it to fund its operating expenses and capital expenditure requirements into the second half of 2022.

Conference Call Information

Blueprint Medicines will host a live conference call and webcast at 8:30 a.m. ET today to discuss first quarter 2020 financial results and recent business activities. The conference call may be accessed by dialing (855) 728-4793 (domestic) or (503) 343-6666 (international), and referring to conference ID 7980947. A webcast of the call will be available under "Events and Presentations" in the Investors & Media section of the Blueprint Medicines website at http://ir.blueprintmedicines.com/. The archived webcast will be available on Blueprint Medicines' website approximately two hours after the conference call and will be available for 30 days following the call.

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 precision therapy approved by the U.S. Food and Drug Administration 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 AYVAKIT (avapritinib), pralsetinib, fisogatinib, and BLU-263, including the timing, designs, implementation, enrollment, plans and announcement of results regarding Blueprint Medicines' 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 impact of the COVID-19 pandemic on Blueprint Medicines' business, operations and financial performance; the potential benefits of the FDA's RTOR program or the FDA's Project Orbis initiative; the potential benefits of Blueprint Medicines' current and future approved drugs or drug candidates in treating patients; expectations regarding Blueprint Medicines' existing cash, cash equivalents and investments; 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 its approved product; Blueprint Medicines’ ability to successfully expand the approved indications for AYVAKIT or obtain marketing approval for AYVAKIT in additional geographies in the future; 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.

Blueprint Medicines Corporation
Selected Condensed Consolidated Balance Sheet Data
(in thousands)
(unaudited)

	March 31, 2020	December 31, 2019
Cash, cash equivalents and investments	$ 750,430	$ 547,960
Working capital (1)	606,395	410,304
Total assets	913,623	707,694
Deferred revenue	45,499	46,073
Total liabilities	230,692	243,335
Total stockholders' equity	682,931	464,359

(1) Blueprint Medicines defines working capital as current assets less current liabilities.

Blueprint Medicines Corporation
Condensed Consolidated Statements of Operations Data
(in thousands, except per share data)
(unaudited)

	Three Months Ended March 31,	
	2020	2019
Revenues:		
Product revenue, net	$ 3,458	$ -
Collaboration revenue	2,709	730
Total revenues	6,167	730
Cost and operating expenses:		
Cost of sales	24	-
Research and development	84,146	74,250
Selling, general and administrative	35,655	16,553
Total cost and operating expenses	119,825	90,803
Other income (expense):		
Interest income (expense), net	2,904	2,710
Other income (expense), net	(201)	(44)
Total other income (expense)	2,703	2,666
Net loss	$(110,955)	$(87,407)
Net loss per share — basic and diluted	$ (2.11)	$ (1.98)
Weighted-average number of common shares used in net loss per share — basic and diluted	52,655	44,097

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