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 13, 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 February 13, 2020, Blueprint Medicines Corporation (the "Company") announced its financial results for the quarter and year ended December 31, 2019 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 February 13, 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 13, 2020

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

Exhibit 99.1

Blueprint Medicines Reports Fourth Quarter and Full Year 2019 Financial Results

– Obtained FDA approval and launched AYVAKIT™ (avapritinib), the first approved precision therapy for GIST and only highly active treatment for PDGFRA exon 18 mutant GIST –

– Announced top-line ARROW trial data for pralsetinib in RET fusion-positive NSCLC and initiated rolling NDA –

– Plan to present initial PIONEER trial data for avapritinib in indolent SM at AAAAI Annual Meeting –

– Completed follow-on offering and raised approximately $308.2 million in estimated net proceeds, extending cash runway into 2H 2022 –

CAMBRIDGE, Mass., February 13, 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 fourth quarter and full year ended December 31, 2019.

“In 2020, we will complete our evolution into a fully-integrated global biopharmaceutical company and fortify our leadership in the field of precision medicine with further expansion of our research pipeline,” said Jeff Albers, Chief Executive Officer of Blueprint Medicines. “Over the course of the year, we expect a sustained cadence of catalytic milestones across our portfolio, which began with the commercial launch of AYVAKIT in January and will continue with the presentation of updated data for avapritinib in indolent systemic mastocytosis and the completion of our rolling NDA submission for pralsetinib for RET fusion-positive non-small cell lung cancer later this quarter. Ultimately, we believe these and other anticipated achievements throughout the year will transform the profile of our company and create substantial value for patients and healthcare providers.”

Fourth Quarter 2019 Highlights and Recent Progress

Avapritinib: gastrointestinal stromal tumors (GIST)

- Received U.S. Food and Drug Administration (FDA) approval of AYVAKIT for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations. Read the press release here and visit www.AYVAKIT.com for full Prescribing Information.
- Announced that the 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 GIST, by three months from February 14, 2020 to May 14, 2020. Read the press release here.

Avapritinib: systemic mastocytosis (SM)

- Reported initial data from Part 1 of the PIONEER trial of avapritinib in patients with indolent SM at the American Society of Hematology Annual Meeting in December 2019 showing rapid and robust reductions in serum tryptase, a measure of mast cell burden, at all dose levels tested. Avapritinib was well-tolerated, and most reported adverse events were Grade 1 or 2. No patients discontinued due to an adverse event. Read the press release here.
- Announced updated data from Part 1 of the PIONEER trial will be reported in a late-breaking oral presentation at the American Academy of Allergy, Asthma & Immunology (AAAAI) annual meeting on March 14, 2020.

Pralsetinib: RET-altered cancers

- Announced centrally reviewed top-line data from the Phase 1/2 ARROW trial of pralsetinib in patients with RET fusion-positive non-small cell lung cancer (NSCLC) treated with pralsetinib at 400 mg QD, which is the proposed indicated dose. In patients previously treated with platinum-based chemotherapy, the overall response rate (ORR) was 61 percent (95% CI: 50-72%; two responses pending confirmation). In treatment-naïve patients, the ORR was 73 percent (95% CI: 52-88%; all responses confirmed), with 12 percent of patients achieving a complete response. The median duration of response, regardless of prior treatment, was not reached. Pralsetinib was well-tolerated, and most AEs were Grade 1 or 2. Across all patients enrolled in the ARROW trial and treated with pralsetinib at 400 mg QD, only four percent of patients discontinued treatment due to treatment-related adverse events. Read the press release here.
- Announced the initiation of a rolling new drug application (NDA) submission to the FDA for pralsetinib for the treatment of RET fusion-positive NSCLC.
- Activated the first clinical trial site for the Phase 3 AcceleRET Lung trial in patients with first-line RET fusion-positive NSCLC.

Fisogatinib: hepatocellular carcinoma (HCC)

- Dosed the first patient in a Phase 1b/2 trial evaluating fisogatinib in combination with CS1001 for the treatment of locally advanced or metastatic HCC, under Blueprint Medicine's collaboration with CStone Pharmaceuticals. Read the press release here.

Research portfolio

- Nominated a potential first-in-class development candidate for the treatment of resistant EGFR-positive triple mutant NSCLC.

Corporate

- Closed an underwritten public offering of 4,710,144 shares of common stock at a public offering price of $69.00 per share. Blueprint Medicines received estimated net proceeds of approximately $308.2 million, after deducting underwriting discounts and commissions and estimated offering expenses.

Key Upcoming Milestones

The company expects to achieve the following milestones in the first half of 2020.

- Present updated data from Part 1 of the PIONEER trial of avapritinib in indolent SM at AAAAI Annual Meeting in the first quarter of 2020.
- Complete the submission of a rolling NDA to the FDA for pralsetinib for RET fusion-positive NSCLC in the first quarter of 2020.
- Report top-line data from the Phase 3 VOYAGER trial of avapritinib in third-line GIST early in the second quarter of 2020.
- Gain FDA approval and, if approved, launch avapritinib in fourth-line GIST in the U.S. in the second quarter of 2020.
- Report top-line data from the Phase 1/2 ARROW trial of pralsetinib in patients with previously treated RET mutant medullary thyroid cancer (MTC).

- 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.
- Submit a marketing authorization application to the European Medicines Agency for pralsetinib for RET fusion-positive NSCLC in the second quarter of 2020.
- Initiate a Phase 1 trial of BLU-263, a next-generation KIT inhibitor, in healthy volunteers in the first half of 2020.

Fourth Quarter and Year End 2019 Financial Results

- **Cash Position:** As of December 31, 2019, cash, cash equivalents and investments were $548.0 million, as compared to $494.0 million as of December 31, 2018. This increase was primarily related to $327.5 million in net proceeds received from the company's April 2019 follow-on underwritten public offering and the $25.0 million upfront cash payment under the license agreement Clementia, partially offset by an increase in cash used in operating activities. Cash, cash equivalents and investments as of December 31, 2019 do not include the estimated net proceeds of approximately $308.2 million from the company's follow-on underwritten public offering of common stock, which closed in January 2020.
- **Collaboration Revenues:** Collaboration revenues were $51.5 million for the fourth quarter of 2019 and $66.5 million for the year ended December 31, 2019, as compared to $1.0 million for the fourth quarter of 2018 and $44.5 million for the year ended December 31, 2018. Collaboration revenue for the year ended December 31, 2019 consisted primarily of the $25.0 million upfront payment and a $20.0 million cash milestone payment due in the third quarter of 2020 under the license agreement with Clementia, an aggregate of $12.0 million in development and regulatory milestones that were achieved in 2019 under the CStone collaboration agreement and $8.2 million under the Roche collaboration agreement. Collaboration revenue for the year ended December 31, 2018 consisted primarily of the $40.0 million upfront payment under the CStone collaboration agreement and $4.5 million under the Roche collaboration agreement.
- **R&D Expenses:** Research and development expenses were $88.6 million for the fourth quarter of 2019 and $331.5 million for the year ended December 31, 2019, as compared to $70.5 million for the fourth quarter of 2018 and $243.6 million for the year ended December 31, 2018. This increase was primarily due to increased clinical and manufacturing expenses driven by the company's lead programs and increased personnel expenses. Research and development expenses included $7.6 million in stock-based compensation expenses for the fourth quarter of 2019 and $28.6 million in stock-based compensation expenses for the year ended December 31, 2019.
- **G&A Expenses:** General and administrative expenses were $32.3 million for the fourth quarter of 2019 and $96.4 million for the year ended December 31, 2019, as compared to $13.6 million for the fourth quarter of 2018 and $47.9 million for the year ended December 31, 2018. This increase was primarily related to increased costs and personnel expenses associated with building the company's commercial infrastructure and to support the overall growth of the business. General and administrative expenses included $8.1 million in stock-based compensation expenses for the fourth quarter of 2019 and $26.1 million in stock-based compensation expenses for the year ended December 31, 2019.
- **Net Loss:** Net loss was $66.3 million for the fourth quarter of 2019 and $347.7 million for the year ended December 31, 2019, or a net loss per share of $1.35 and $7.27, respectively, as compared to a net loss of $80.3 million for the fourth quarter of 2018 and $236.6 million for the year ended December 31, 2018, or a net loss per share of $1.83 and $5.39, respectively.

Financial Guidance

Based on its current operating plans, Blueprint Medicines expects that its existing cash, cash equivalents and investments including the $308.2 million in estimated net proceeds from the January 2020 follow-on public offering, together with anticipated product revenues but excluding any additional 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 fourth quarter and full year 2019 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 26735762. A webcast of the conference call will be available in the Investors 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 FDA-approved precision therapy and are currently advancing multiple 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 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 for avapritinib, pralsetinib, fisogatinib and BLU-263; plans and timelines for nominating additional development candidates; plans and timelines for submitting marketing applications for avapritinib and pralsetinib and, if approved, commercializing avapritinib for additional indications or pralsetinib; the potential benefits of Blueprint Medicines' current and future approved drugs or drug candidates in treating patients; plans, timelines and expectations for top-line data from the VOYAGER trial; expectations regarding Blueprint Medicines' existing cash, cash equivalents and investments; and Blueprint Medicines' strategy, goals and anticipated milestones, business plans and focus; the potential benefits of Blueprint Medicines' current and future drug candidates in treating patients; 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 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)

	December 31, 2019	December 31, 2018
Cash, cash equivalents and investments	$ 547,960	$ 494,012
Working capital (1)	410,304	439,464
Total assets	707,694	540,124
Deferred revenue	46,073	46,167
Lease incentive obligation	-	14,617
Total stockholders' equity	464,359	419,009

(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 December 31,		Years Ended December 31,	
	2019	2018	2019	2018
Collaboration revenue	$ 51,533	$ 1,033	$ 66,512	$ 44,521
Operating expenses:				
Research and development	88,646	70,532	331,450	243,621
General and administrative	32,265	13,643	96,388	47,928
Total operating expenses	120,911	84,175	427,838	291,549
Other income (expense):				
Interest income (expense), net	2,990	2,871	13,732	10,566
Other income (expense), net	57	(51)	(100)	(180)
Total other income (expense)	3,047	2,820	13,632	10,386
Net loss	$ (66,331)	$ (80,322)	$ (347,694)	$ (236,642)
Net loss per share — basic and diluted	$ (1.35)	$ (1.83)	$ (7.27)	$ (5.39)
Weighted-average number of common shares used in net loss per share — basic and diluted	49,218	43,994	47,829	43,867

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