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 17, 2021**

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 17, 2021, Blueprint Medicines Corporation (the "Company") announced its financial results for the quarter and year ended December 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 February 17, 2021
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: February 17, 2021

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

Blueprint Medicines Reports Fourth Quarter and Full Year 2020 Financial Results

-- Supplemental NDA accepted by FDA for AYVAKIT™ (avapritinib) for advanced systemic mastocytosis --
-- Type II variation MAA submitted to EMA for AYVAKYT® (avapritinib) for advanced systemic mastocytosis --
-- Announced a potential best-in-class research program targeting CDK2 for the treatment of multiple cancers with tumor-associated cell cycle defects --
-- Plan to provide updates across multiple clinical and research programs at AACR 2021 Annual Meeting --
-- Ended 2020 with approximately $1.5 billion in cash --

CAMBRIDGE, Mass., February 17, 2021 – Blueprint Medicines Corporation (NASDAQ:BPMC) today reported financial results and provided a business update for the fourth quarter and full year ended December 31, 2020.

“Last year was transformational for Blueprint Medicines, with four regulatory approvals in the United States and Europe, as we executed on our foundational mission of delivering innovative precision medicines to patients globally,” said Jeff Albers, Chief Executive Officer of Blueprint Medicines. “In 2021, as we accelerate ongoing efforts to drive the global adoption of AYVAKIT and GAVRETO, we look forward to a potential U.S. approval for AYVAKIT for the most advanced and deadly form of systemic mastocytosis for which no other precision medicines are currently available. We also continue to focus on our next wave of therapeutic candidates, including our two EGFR inhibitors, which we anticipate will enter the clinic this year with the goal of achieving rapid clinical proof-of-concept. In addition, we continue to expand our research pipeline with new assets that leverage our kinase inhibition expertise, and today, we are excited to announce our newest research program that selectively targets CDK2, a kinase involved in driving multiple cancers.”

Fourth Quarter 2020 Highlights and Recent Progress

AYVAKIT™/AYVAKYT® (avapritinib): systemic mastocytosis (SM)

- Today announced the U.S. Food and Drug Administration (FDA) has accepted our supplemental new drug application for AYVAKIT for advanced systemic mastocytosis (SM). The FDA granted priority review and set an action date of June 16, 2021 under the Prescription Drug User Fee Act.
- Today announced the submission of a Type II variation marketing authorization application to the European Medicines Agency for AYVAKYT for the treatment of adult patients with advanced SM.
- Received breakthrough therapy designation from the FDA for the treatment of moderate to severe indolent SM, which encompasses the majority of patients with SM. Avapritinib has also previously received breakthrough therapy designation for the treatment of patients with advanced SM.
- Presented new data at the virtual 62nd American Society of Hematology (ASH) Annual Meeting highlighting new proposed response criteria for advanced SM, the potential of highly sensitive droplet digital PCR-based KIT D816V testing to accelerate diagnosis across the spectrum of SM, and data from patients and healthcare providers characterizing the significant disease burden across the full spectrum of SM. Read the press release here.

AYVAKIT™/AYVAKYT® (avapritinib): gastrointestinal stromal tumor (GIST)

- Recorded $6.0 million in net product revenue during the fourth quarter of 2020 for AYVAKIT/AYVAKYT, which was approved by the FDA in January 2020 for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations, and by the European Commission in September 2020 for the treatment of adult patients with unresectable or metastatic GIST harboring the PDGFRA D842V mutation.

GAVRETO™ (pralsetinib): RET-altered cancers

- Recorded $0.7 million in net product revenue during the fourth quarter of 2020 for GAVRETO, which was approved by the FDA in September 2020 for the treatment of adult patients with metastatic RET fusion-positive non-small cell lung cancer (NSCLC) as detected by an FDA approved test and in December 2020 for the treatment of patients 12 years of age and older with advanced or metastatic RET-mutant

medullary thyroid cancer (MTC) and RET fusion-positive thyroid cancer. Blueprint Medicines is commercializing GAVRETO in the U.S. together with Genentech, Inc., a member of the Roche Group. Read the press release announcing the expansion of the FDA label for GAVRETO into RET-mutant MTC and RET fusion-positive thyroid cancer here.

BLU-263: systemic mastocytosis

- Reported positive top-line results from a Phase 1 trial in healthy volunteers, showing that BLU-263 was well-tolerated across a range of single- and multiple-ascending doses predicted to potently inhibit D816V mutant KIT, the underlying SM disease driver.

Research Portfolio:

- Today announced a highly selective and potent research program with best-in-class potential targeting CDK2. CDK2, a cyclin-dependent kinase involved in cell cycle biology, is activated by its regulatory partner Cyclin E, and can drive cancer cell proliferation when Cyclin E is aberrantly expressed. Dysregulated Cyclin E is associated with multiple malignancies and has been shown to be a mechanism of resistance to targeted therapies, including CDK4/6 inhibitors.
- Nominated BLU-701, a potential first-in-class, selective, brain-penetrant development candidate for treatment-resistant double-mutant EGFR-driven NSCLC.
- Nominated a potential best-in-class development candidate targeting MAP4K1, a kinase believed to play a role in T-cell regulation, under the company's cancer immunotherapy collaboration with Roche.

Corporate:

- Announced leadership transitions for the company's research and development organization, including the promotion of Becker Hewes, M.D. to Chief Medical Officer, effective January 11, 2021.

Key Upcoming Milestones

The company plans to achieve the following milestones by mid-2021:

- Provide portfolio updates across clinical and research programs at the AACR 2021 Annual Meeting.
- Obtain regulatory approval from the European Commission and launch GAVRETO in RET fusion-positive NSCLC in Europe in the first half of 2021, under the ongoing global collaboration with Roche.
- Initiate a Phase 1 trial of BLU-945, a triple-mutant EGFR inhibitor, in patients with treatment-resistant EGFR-driven NSCLC in the first half of 2021.
- Obtain FDA approval and launch AYVAKIT in advanced SM in the U.S. in the first half of 2021.
- Complete enrollment of the registration-enabling Part 2 of the PIONEER trial of AYVAKIT in non-advanced SM in mid-2021.
- Initiate the Phase 2 HARBOR trial of BLU-263, a next-generation KIT inhibitor, in patients with non-advanced SM in mid-2021.

Fourth Quarter and Year End 2020 Financial Results

- **Revenues:** Revenues were $34.1 million for the fourth quarter of 2020, including $6.0 million of net product revenues from sales of AYVAKIT, $0.7 million of net product revenues from sales of GAVRETO and $27.4 million in collaboration revenues. Revenues for the year ended December 31, 2020 were $793.7 million, including $21.2 million of net product revenues from sales of AYVAKIT, $0.9 million of net product revenues from sales of GAVRETO and $771.6 million in collaboration revenues. Blueprint Medicines recorded $51.5 million and $66.5 million in collaboration revenues in the fourth quarter and year ended December 31, 2019, respectively.
- **Cost of Sales:** Cost of sales was $0.1 million for the fourth quarter of 2020 and $0.4 million for the year ended December 31, 2020. Blueprint Medicines did not incur cost of sales in the fourth quarter or year ended December 31, 2019, as no product sales were generated during that period.

- **R&D Expenses:** Research and development expenses were $77.4 million for the fourth quarter of 2020 and $326.9 million for the year ended December 31, 2020, as compared to $88.6 million for the fourth quarter of 2019 and $331.5 million for the year ended December 31, 2019. This decrease was primarily due to reimbursement from the global development cost sharing arrangement under the collaboration with Roche for pralsetinib. Research and development expenses included $8.5 million in stock-based compensation expenses for the fourth quarter of 2020 and $33.6 million in stock-based compensation for the year ended December 31, 2020.
- **SG&A Expenses:** Selling, general and administrative expenses were $42.5 million for the fourth quarter of 2020 and $157.7 million for the year ended December 31, 2020, as compared to $32.3 million for the fourth quarter of 2019 and $96.4 million for the year ended December 31, 2019. This increase was primarily due to increased costs and personnel expenses associated with building Blueprint Medicines' commercial infrastructure for AYVAKIT/AYVAKYT and GAVRETO, partially offset by reimbursement under the collaboration with Roche for pralsetinib in connection with the commercialization of GAVRETO in the U.S.. Selling, general and administrative expenses included $11.0 million in stock-based compensation expenses for the fourth quarter of 2020 and $41.9 million in stock-based compensation for the year ended December 31, 2020.
- **Net Income (Loss):** Net loss was $85.7 million for the fourth quarter of 2020 and net income was $313.9 million for the year ended December 31, 2020, or a diluted net loss per share of $1.53 and diluted net income per share of $5.59, respectively, as compared to a net loss of $66.3 million for the fourth quarter of 2019 and a net loss of $347.7 million for the year ended December 31, 2019, or a diluted net loss per share of $1.35 and $7.27, respectively.
- **Cash Position:** As of December 31, 2020, cash, cash equivalents and marketable securities were $1,549.7 million, as compared to $548.0 million as of December 31, 2019. This increase was primarily related to upfront payments of $775.0 million received in the third quarter of 2020 under Blueprint Medicines' collaboration with Roche for pralsetinib, $308.4 million in net proceeds received from Blueprint Medicines' January 2020 follow-on underwritten public offering and $194.7 million in net proceeds received in the fourth quarter of 2020 from Blueprint Medicines' "at the market" stock offering program, partially offset by cash used in operating activities.

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 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 4844138. 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.

Upcoming Investor Conferences

Blueprint Medicines will participate in three upcoming investor conferences:

- **10th Annual SVB Leerink Global Healthcare Conference** on Thursday, February 25, 2021 at 11:20 a.m. ET.
- **Cowen 41st Annual Health Care Conference** on Wednesday, March 3, 2021 at 3:20 p.m. ET.
- **Barclays Global Healthcare Conference** on Wednesday, March 10, 2021 at 9:10 a.m. ET.

A live webcast of each presentation will be available by visiting the Investors & Media section of Blueprint Medicines' website at http://ir.blueprintmedicines.com. A replay of the webcasts will be archived on Blueprint Medicines' website for 30 days following each presentation.

About Blueprint Medicines

Blueprint Medicines is a global precision therapy company that invents life-changing medicines for people with cancer and hematologic disorders. Applying an approach that is both precise and agile, we create therapies that selectively target genetic drivers, with the goal of staying one step ahead across stages of disease. Since 2011, we have leveraged our research platform, including expertise in molecular targeting and world-class drug design capabilities, to rapidly and reproducibly translate science into a broad pipeline of precision therapies. Today, we are delivering our approved medicines to patients in the United States and Europe, and we are globally advancing multiple programs for genomically defined cancers, systemic mastocytosis, and cancer immunotherapy. 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, strategies, timelines and expectations for Blueprint Medicines' current or future approved drugs and drug candidates, including timelines for marketing applications and approvals, the initiation of clinical trials or the results of ongoing and planned clinical trials; Blueprint Medicines' plans, strategies and timelines to nominate development candidates; plans and timelines for additional marketing applications for avapritinib and pralsetinib and, if approved, commercializing avapritinib and pralsetinib in additional geographies or for additional indications; the potential benefits of any of Blueprint Medicines' current or future approved drugs or drug candidates in treating patients; the potential benefits of Blueprint Medicines' collaboration with Roche and Genentech for pralsetinib; 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 expand 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 or obtain marketing approval for AYVAKIT/AYVAKYT 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 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 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 Corporation
Selected Condensed Consolidated Balance Sheet Data
(in thousands)
(unaudited)

	December 31, 2020	December 31, 2019
Cash, cash equivalents and marketable securities	$ 1,549,722	$ 547,960
Working capital (1)	796,957	410,304
Total assets	1,718,393	707,694
Deferred revenue	41,158	46,073
Total liabilities	248,305	243,335
Total stockholders' equity	1,470,088	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 December 31,		Years Ended December 31,	
	2020	2019	2020	2019
Revenues:				
Product revenue, net	$ 6,688	$ —	$ 22,134	$ —
Collaboration revenue	27,419	51,533	771,601	66,512
Total revenues	$ 34,107	$ 51,533	$ 793,735	$ 66,512
Cost and operating expenses:				
Cost of sales	128	—	425	—
Research and development	77,405	88,646	326,860	331,450
Selling, general and administrative	42,541	32,265	157,743	96,388
Total cost and operating expenses	$ 120,074	$ 120,911	$ 485,028	$ 427,838
Other income (expense):				
Interest income, net	936	2,990	6,599	13,732
Other income (expense), net	50	57	(366)	(100)
Total other income (expense)	$ 986	$ 3,047	$ 6,233	$ 13,632
Income (loss) before income taxes	$ (84,981)	$ (66,331)	$ 314,940	$ (347,694)
Income tax expense	688	—	1,058	—
Net income (loss)	$ (85,669)	$ (66,331)	$ 313,882	$ (347,694)
Net income (loss) per share applicable to common stockholders — basic	$ (1.53)	$ (1.35)	$ 5.76	$ (7.27)
Net income (loss) per share applicable to common stockholders —diluted	$ (1.53)	$ (1.35)	$ 5.59	$ (7.27)
Weighted-average number of common shares used in net income (loss) per share applicable to common stockholders — basic	56,072	49,218	54,534	47,829
Weighted-average number of common shares used in net income (loss) per share applicable to common stockholders —diluted	56,072	49,218	56,168	47,829

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