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): **April 29, 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 April 29, 2021, Blueprint Medicines Corporation announced its financial results for the financial results for the quarter ended March 31, 2021 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 April 29, 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: April 29, 2021

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

Blueprint Medicines Reports First Quarter 2021 Financial Results

-- 7 research- and clinical-stage programs highlighted at AACR, including recently nominated development candidate with best-in-class potential targeting CDK2 --

-- GAVRETO® (pralsetinib) becomes the first selective RET inhibitor approved in China; AYVAKIT™/AYVAKYT® (avapritinib) becomes the first precision therapy for patients with PDGFRA exon 18 mutant GIST in China --

-- Preparing to launch avapritinib in advanced SM in Q2 2021 --

-- On track to initiate Phase 1 trial of BLU-945 in EGFR-driven NSCLC in Q2 2021 and Phase 2/3 HARBOR trial of BLU-263 in non-advanced SM in mid-2021 --

CAMBRIDGE, Mass., April 29, 2021 -- Blueprint Medicines Corporation (NASDAQ: BPMC) today reported financial results and provided a business update for the first quarter ended March 31, 2021.

“In recent months, we made significant progress toward achieving our key 2021 portfolio goals, illustrating our clinical leadership in systemic mastocytosis and the rapid advancement of our next wave of therapeutic candidates with first- or best-in-class potential,” said Jeff Albers, Chief Executive Officer of Blueprint Medicines. “With a potential U.S. approval for AYVAKIT in advanced systemic mastocytosis in the second quarter, we are looking forward to welcoming a new era of precision medicines care for people living with this debilitating disease. In parallel, we are initiating multiple clinical trials across our pipeline, beginning with the Phase 1 trial of BLU-945 and Phase 2/3 HARBOR trial of BLU-263, as we continue to translate our research productivity and precision therapy expertise to address patient need across cancers and hematologic malignancies. Taken together, these accomplishments position Blueprint Medicines to help an increasing number of patients globally as we advance our robust portfolio.”

First Quarter 2021 Highlights and Recent Progress

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

- Reported data at the virtual American Association for Cancer Research (AACR) Annual Meeting, including registrational PATHFINDER trial data in advanced systemic mastocytosis (SM), which demonstrated an overall confirmed response rate of 75 percent, as well as PIONEER Part 1 data highlighting the impact of AYVAKIT on skin manifestations in non-advanced SM. Read the full data here.
- Received European Medicines Agency (EMA) validation of the Type II variation marketing authorization application (MAA) for AYVAKYT for the treatment of advanced SM. Validation of the MAA confirms that the submission is sufficiently complete to begin the formal review process. Read the press release here.

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

- Recorded $7.1 million in net product revenue during the first quarter of 2021 for AYVAKIT/AYVAKYT, which was approved by the U.S. Food and Drug Administration (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 as a monotherapy for the treatment of adult patients with unresectable or metastatic GIST harboring the PDGFRA D842V mutation.
- Received National Medical Products Administration (NMPA) approval in China, via our collaboration with CStone Pharmaceuticals, for the treatment of adults with unresectable or metastatic PDGFRA exon 18 mutant GIST, the first approved precision therapy for this patient population in China.

GAVRETO® (pralsetinib): RET-altered cancers

- Recorded $1.8 million in net product revenue during the first quarter of 2021 for GAVRETO, which was approved by the FDA in September 2020 for the treatment of adult patients with metastatic rearranged during transfection (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.
- Received approval in China, via our collaboration with CStone Pharmaceuticals, for the treatment of adults with locally advanced or metastatic RET fusion-positive NSCLC after platinum-based chemotherapy, the first approved selective RET inhibitor in China.

BLU-263: SM

- Reported new data at AACR from a Phase 1 trial in healthy volunteers, showing that BLU-263 was generally well-tolerated across a range of single- and multiple-ascending doses, with a half-life supporting once-daily dosing. Based on these data, Blueprint Medicines plans to evaluate BLU-263 at doses ranging from 25 to 100 mg once daily in Part 1 of the Phase 2/3 HARBOR trial in non-advanced SM. Read the full data here.

BLU-945 and BLU-701: treatment-resistant EGFR-driven NSCLC

- Reported new preclinical data at AACR showing the potential for the company's potent and selective double-mutant EGFR inhibitor, BLU-701, and potent and selective triple-mutant EGFR inhibitor, BLU-945, to be used alone or in combination, together or with other agents, to overcome or prevent on-target resistance across multiple lines of treatment. Read the full data here.
- Received clearance from the FDA for an investigational new drug (IND) application for BLU-945 for the treatment of patients with EGFR-driven NSCLC.

BLU-222: Cyclin E-aberrant cancers

- Nominated BLU-222, a potentially best-in-class selective and potent CDK2 inhibitor development candidate, for the treatment of cyclin E-aberrant cancers.
- Reported new preclinical data at AACR for a set of CDK2 inhibitors, showing that selective CDK2 inhibition arrested the cell cycle and blocked tumor proliferation in cyclin E (CCNE)-amplified cell lines and demonstrated robust and sustained anti-tumor activity *in vivo* in models of CCNE-amplified ovarian, breast and gastric cancer, with improved tolerability compared to a pan-CDK inhibitor and chemotherapy. Read the full data here.

BLU-852: Cancer immunotherapy

- Nominated BLU-852, a potentially best-in-class selective and potent MAP4K1 inhibitor development candidate, developed under the company’s cancer immunotherapy collaboration with Roche.
- Reported new preclinical data at AACR for a set of MAP4K1 inhibitors, including BLU-852, which were shown to enhance intratumoral immune cell activation, overcome T cell suppression, and reduce tumor burden both as a monotherapy and in combination with checkpoint inhibition. Read the full data here.

Key Upcoming Milestones

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

- Obtain regulatory approval from the FDA and, if approved, launch AYVAKIT for the treatment of patients with advanced SM in the second quarter of 2021.
- Present clinical data from the ARROW trial of pralsetinib in patients with RET fusion–positive non-small cell lung cancer and in patients with solid tumors at the 2021 American Society of Clinical Oncology (ASCO) Annual Meeting in the second quarter of 2021.
- Initiate a global Phase 1 trial of BLU-945 in patients with treatment-resistant EGFR-driven NSCLC in the second quarter 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 2/3 HARBOR trial of BLU-263 in patients with non-advanced SM in mid-2021.
- Initiate a Phase 1 trial of BLU-701 in patients with treatment-resistant EGFR-driven NSCLC in the second half of 2021.
- Present preclinical data supporting combination of BLU-945 and BLU-701 in treatment-naïve EGFR-driven NSCLC in the second half of 2021.

First Quarter 2021 Financial Results

- **Revenues:** Revenues were $21.6 million for the first quarter of 2021, including $7.1 million of net product revenues from sales of AYVAKIT/AYVAKYT, $1.8 million of net product revenues from sales of GAVRETO and $12.6 million in collaboration revenues. Blueprint Medicines recorded revenues of $6.2 million in the first quarter of 2020, including $3.5 million of net product revenues from sales of AYVAKIT and $2.7 million in collaboration revenues.
- **Cost of Sales:** Cost of sales was $0.1 million for the first quarter of 2021, as compared to less than $0.1 million for the first quarter of 2020.
- **R&D Expenses:** Research and development expenses were $79.7 million for the first quarter of 2021, as compared to $84.1 million for the first quarter of 2020. This decrease was primarily due to reimbursement from the global development cost sharing arrangement under our collaboration with Roche for pralsetinib. Research and development expenses included $8.9 million in stock-based compensation expenses for the first quarter of 2021.
- **SG&A Expenses:** Selling, general and administrative expenses were $42.0 million for the first quarter of 2021, as compared to $35.7 million for the first quarter of 2020. This increase was primarily due to increased costs associated with building our commercial infrastructure for commercialization of AYVAKIT/AYVAKYT and GAVRETO, partially offset by reimbursement under our collaboration with Roche for pralsetinib. General and administrative expenses included $11.7 million in stock-based compensation expenses for the first quarter of 2021.
- **Net Loss:** Net loss was $99.7 million for the first quarter of 2021, or a net loss per share of $1.72, as compared to a net loss of $111.0 million for the first quarter of 2020, or a net loss per share of $2.11.
- **Cash Position:** As of March 31, 2021, cash, cash equivalents and investments were $1,430.1 million, as compared to $1,549.7 million as of December 31, 2020.

Conference Call Information

Blueprint Medicines will host a live conference call and webcast at 8:30 a.m. ET today to discuss first quarter 2021 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 9292306. 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 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' collaborations; 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, AYVAKIT, AYVAKYT, GAVRETO and associated logos are trademarks of Blueprint Medicines Corporation.

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

	March 31, 2021	December 30, 2020
Cash, cash equivalents and marketable securities	$ 1,430,129	$ 1,549,722
Working capital (1)	825,273	796,957
Total assets	1,625,387	1,718,393
Deferred revenue	40,973	41,158
Total liabilities	225,366	248,305
Total stockholders' equity	1,400,021	1,470,088

(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,	
	2021	2020
Revenues:		
Product revenue, net	$ 8,955	$ 3,458
Collaboration revenue	12,621	2,709
Total revenues	$ 21,576	$ 6,167
Cost and operating expenses:		
Cost of sales	102	24
Research and development	79,710	84,146
Selling, general and administrative	42,002	35,655
Total cost and operating expenses	$ 121,814	$ 119,825
Other income (expense):		
Interest income, net	738	2,904
Other income (expense), net	(214)	(201)
Total other income (expense)	$ 524	$ 2,703
Income (loss) before income taxes	$ (99,714)	$ (110,955)
Income tax expense	—	—
Net income (loss)	$ (99,714)	$ (110,955)
Net income (loss) per share applicable to common stockholders — basic and diluted	$ (1.72)	$ (2.11)
Weighted-average number of common shares used in net income (loss) per share applicable to common stockholders — basic and diluted	58,023	52,655

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