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): **October 29, 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 October 29, 2020, Blueprint Medicines Corporation (the "Company") announced its financial results for the quarter ended September 30, 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 October 29, 2020
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: October 29, 2020

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

Blueprint Medicines Reports Third Quarter 2020 Financial Results

-- Obtained FDA approval and launched GAVRETO™ for RET fusion-positive non-small cell lung cancer --
-- Announced top-line EXPLORER and PATHFINDER data for AVYAKIT™ in advanced systemic mastocytosis; plan to submit supplemental NDA to FDA in Q4 2020 --
-- Received European Commission approval of AYVAKYT® for PDGFRA D842V mutant gastrointestinal stromal tumors --
-- Ended Q3 2020 with approximately $1.4 billion in cash driven by the upfront payments from the Roche collaboration signed during the quarter --

CAMBRIDGE, Mass., October 29, 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 third quarter ended September 30, 2020.

“Following our tremendous progress in the third quarter, Blueprint Medicines is in a very strong position, with two precision therapies now approved and significant near-term growth opportunities expected across our portfolio,” said Jeff Albers, Chief Executive Officer of Blueprint Medicines. “In recent months, we bolstered our commercial foundation with the U.S. approval and launch of GAVRETO, and we are now preparing to file our supplemental NDA for AYVAKIT in advanced systemic mastocytosis, marking an important step toward delivering a much-needed new therapy to patients across the systemic mastocytosis disease spectrum. In parallel, we continued to strengthen our pipeline with the presentation of compelling preclinical proof-of-concept data for BLU-945, a potentially best-in-class therapy for treatment-resistant EGFR-driven lung cancer, that support our plan to initiate clinical development of BLU-945 in the first half of next year.”

Third Quarter 2020 Highlights and Recent Updates

AYVAKIT (avapritinib): systemic mastocytosis (SM)

- Announced positive top-line results from the Phase 1 EXPLORER and Phase 2 PATHFINDER clinical trials of AYVAKIT in patients with advanced SM. Consistent with previously reported EXPLORER trial results, the data showed profound reductions in mast cell burden, high overall response and complete remission rates, and durable clinical benefit, including prolonged median overall survival. AYVAKIT was generally well-tolerated, with an improved safety profile at the 200 mg once daily dose. Read the press release here.

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

- Recorded $6.1 million in net product revenue during the third quarter of 2020 for AYVAKIT, 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.
- Received European Commission (EC) conditional marketing authorization for avapritinib under the brand name AYVAKYT as a monotherapy for the treatment of adult patients with unresectable or metastatic GIST harboring the PDGFRA D842V mutation and initiated the first commercial launch following EC approval in Germany. Read the press release here.

GAVRETO (pralsetinib): RET-altered cancers

- Received FDA approval of GAVRETO 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 launched GAVRETO together with Genentech, a member of the Roche Group, in the U.S. in September. Read the press release here. Please click here to see the full Prescribing Information for GAVRETO and visit www.GAVRETO.com for more information on GAVRETO.
- Reported updated data from the ongoing ARROW clinical trial of GAVRETO in advanced RET-mutant medullary thyroid cancer (MTC) at the European Society for Medical Oncology (ESMO) Virtual Congress

2020. The data showed durable responses and a well-tolerated safety profile for GAVRETO in patients with advanced RET-mutant MTC, with consistent clinical activity in patients across lines of therapy and regardless of RET mutation genotypes. Read the press release here.
- Announced acceptance of U.S. marketing application for GAVRETO for the treatment of patients with advanced or metastatic RET-mutant MTC and RET fusion-positive thyroid cancer. The FDA accepted the new drug application (NDA) for priority review under its Real-Time Oncology Review (RTOR) pilot program, and set an action date of February 28, 2021 under the Prescription Drug User Fee Act.

BLU-945: EGFR-mutated NSCLC
- Reported preclinical proof-of-concept data for BLU-945 at the ESMO Virtual Congress 2020. The data showed BLU-945 potently and selectively inhibited triple-mutant epidermal growth factor receptor (EGFR) harboring the most common on-target resistance mutations to standard treatments for EGFR-mutated NSCLC, resulting in robust anti-tumor activity in multiple lung cancer models. Read the press release here.
- Announced plans to develop BLU-945 as a monotherapy and in combination with other agents for the treatment of patients with treatment-resistant EGFR NSCLC.

Corporate:
- Strengthened the company's leadership team with the appointment of Fouad Namouni, M.D., as President, Research & Development. In this new role, Dr. Namouni leads a joint research and development organization, overseeing all phases of product development from discovery through global registration under a unified portfolio vision.

Key Upcoming Milestones

The company expects to achieve the following near-term milestones:
- Submit a supplemental new drug application to the FDA for avapritinib for the treatment of patients with advanced SM in the fourth quarter of 2020.
- Obtain U.S. approval of GAVRETO for RET-altered thyroid cancers in the first quarter of 2021.
- Initiate a global Phase 1 dose-escalation trial for BLU-945 in EGFR-mutated NSCLC in the first half of 2021.

Third Quarter 2020 Financial Results

- **Revenues:** Revenues were $745.1 million for the third quarter of 2020, including $6.1 million of net product revenues from sales of AYVAKIT, $0.2 million of net product revenues from sales of GAVRETO and $738.8 million in collaboration revenues under the collaboration agreements with Roche and CStone. Blueprint Medicines recorded $9.1 million in collaboration revenues for the third quarter of 2019.
- **Cost of Sales:** Cost of sales was $0.1 million for the third quarter of 2020. Blueprint Medicines did not incur cost of sales in the third quarter of 2019, as no product sales were generated during that period.
- **R&D Expenses:** Research and development expenses were $74.2 million for the third quarter of 2020, as compared to $81.5 million for the third quarter of 2019. This decrease was primarily due to reimbursement from the global development cost sharing arrangement under the collaboration agreement with Roche for pralsetinib. Research and development expenses included $8.6 million in stock-based compensation expenses for the third quarter of 2020.
- **SG&A Expenses:** Selling, general and administrative expenses were $37.4 million for the third quarter of 2020, as compared to $25.6 million for the third quarter of 2019. This increase was primarily due to an increase in costs and personnel expenses associated with building Blueprint Medicines' commercial infrastructure for commercialization of AYKAKIT and GAVRETO, partially offset by reimbursement for Roche's share of the loss generated from the commercialization of GAVRETO in the U.S. under the collaboration for pralsetinib. Selling, general and administrative expenses included $11.0 million in stock-based compensation expenses for the third quarter of 2020.

- **Net Income (Loss):** Net income was $634.0 million for the third quarter of 2020, or a diluted net income per share of $11.16, as compared to a net loss of $94.3 million for the third quarter of 2019, or a diluted net loss per share of $1.93.
- **Cash Position:** As of September 30, 2020, cash, cash equivalents and investments were $1,355.9 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 and $308.4 million in net proceeds received from Blueprint Medicines' January 2020 follow-on underwritten public offering, 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 third 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 2995408. 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 Conference

Blueprint Medicines will participate in the Jefferies Virtual London Healthcare Conference on November 17, 2020 at 2:55 p.m. ET. A live webcast of the presentation will be available by visiting the Investors & Media section of Blueprint Medicines' website at http://ir.blueprintmedicines.com. A replay of the webcast will be archived on Blueprint Medicines' website for 30 days following the presentation.

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 two approved precision therapies and are currently advancing multiple investigational medicines in clinical and pre-clinical development, along with a number of earlier-stage 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 plans, strategies, timelines and expectations of Blueprint Medicines for the preclinical and clinical development and commercialization of AYVAKIT/AYVAKYT, GAVRETO and other current or future drug candidates; plans, timing, design, initiation, enrollment, expectations and announcement of results for the Blueprint Medicines' ongoing and planned clinical trials; 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 establishing 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)

	September 30, 2020	December 31, 2019
Cash, cash equivalents and investments	$ 1,355,900	$ 547,960
Working capital (1)	731,547	410,304
Total assets	1,558,178	707,694
Deferred revenue	45,408	46,073
Total liabilities	238,178	243,335
Total stockholders' equity	1,320,000	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 September 30,	
	2020	2019
Revenues:		
Product revenue, net	$ 6,308	$ —
Collaboration revenue	738,810	9,139
Total revenues	745,118	9,139
Cost and operating expenses:		
Cost of sales	146	—
Research and development	74,230	81,453
Selling, general and administrative	37,375	25,647
Total cost and operating expenses	111,751	107,100
Other income (expense):		
Interest income, net	1,173	3,758
Other income (expense), net	(192)	(72)
Total other income	981	3,686
Income (loss) before income taxes	634,348	(94,275)
Income tax expense	370	—
Net income (loss)	$ 633,978	$ (94,275)
Net income (loss) per share — basic	$ 11.49	$ (1.93)
Net income (loss) per share – diluted	$ 11.16	$ (1.93)
Weighted-average number of common shares used in net income (loss) per share — basic	55,169	48,921
Weighted-average number of common shares used in net income (loss) per share — diluted	56,786	48,921

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