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 30, 2018**

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:

- o Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- o Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- o Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- o 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. ☐

Item 2.02 Results of Operations and Financial Condition.

On October 30, 2018, Blueprint Medicines Corporation announced its financial results for the quarter ended September 30, 2018. 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 Current Report on 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 30, 2018

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 30, 2018

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

Blueprint Medicines Reports Third Quarter 2018 Financial Results

– Avapritinib receives Breakthrough Therapy Designation for treatment of advanced SM –
– First patient screened in registration-enabling PATHFINDER trial in advanced SM –
– Received FDA feedback supporting expedited development of BLU-667 –
– Will present updated results from NAVIGATOR trial of avapritinib in advanced GIST at CTOS annual meeting; management to host conference call to review data at 7:30 a.m. ET on November 15, 2018 –

CAMBRIDGE, Mass., October 30, 2018 – Blueprint Medicines Corporation (NASDAQ:BPMC), a leader in discovering and developing targeted kinase medicines for patients with genomically defined diseases, today reported financial results and provided a business update for the third quarter ended September 30, 2018.

"Our third quarter and recent accomplishments represent tremendous progress across our portfolio, marked by the receipt of positive regulatory feedback from the FDA for avapritinib and BLU-667 and the presentation of new data across our clinical- and research-stage pipeline," said Jeff Albers, Chief Executive Officer of Blueprint Medicines. "As we prepare to enter a critical year, we are now accelerating our efforts with a focus on executing five registration-enabling studies across seven patient populations, building a global commercial enterprise to deliver medicines to patients and investing in the next generation of precision therapies."

Clinical Programs:

Avapritinib: Systemic Mastocytosis (SM)

- Blueprint Medicines announced that the U.S. Food and Drug Administration (FDA) granted Breakthrough Therapy Designation to avapritinib for the treatment of patients with advanced SM, including the subtypes of aggressive SM, SM with an associated hematologic neoplasm and mast cell leukemia.
- Blueprint Medicines announced that it has screened the first patient in PATHFINDER, its registration-enabling, open-label, single-arm Phase 2 clinical trial in patients with advanced SM. Blueprint Medicines expects to initiate PIONEER, its registration-enabling, randomized, placebo-controlled Phase 2 clinical trial in patients with indolent and smoldering SM, by the end of 2018.
- Blueprint Medicines announced that the European Medicines Agency has granted orphan drug designation to avapritinib for the treatment of mastocytosis.
- Enrollment in the expansion portion of the Phase 1 EXPLORER clinical trial for advanced SM is ongoing. Blueprint Medicines plans to present data from this trial at the 60th American Society for Hematology (ASH) Annual Meeting and Exposition in December 2018.

Avapritinib: Gastrointestinal Stromal Tumors (GIST)

- Blueprint Medicines continues to evaluate avapritinib in its Phase 1 NAVIGATOR clinical trial and will present updated data across multiple patient populations, including PDGFRA-driven GIST, third-line or later GIST, and second-line GIST, at the 2018 Connective Tissue Oncology Society (CTOS) Annual Meeting on November 15, 2018. Based on data from this trial, Blueprint Medicines plans to submit a new drug application (NDA) to the FDA for avapritinib for the

treatment of patients with PDGFRα Exon 18 mutant GIST and fourth-line GIST in the first half of 2019.

BLU-667: RET-Altered Solid Tumors

- Blueprint Medicines recently received written feedback from the FDA supporting expedited development of BLU-667 and plans to submit an NDA for BLU-667 in the first half of 2020 based on additional data from the ongoing Phase 1 ARROW trial. Based on the feedback from the FDA, Blueprint Medicines currently expects the NDA submission will be for separate potential indications: (1) patients with RET-fusion positive NSCLC and papillary thyroid cancer (PTC) who have progressed following prior systemic therapy and (2) patients with RET-mutant medullary thyroid cancer (MTC) who have progressed following treatment with a tyrosine kinase inhibitor.
- In October 2018, Blueprint Medicines presented updated data from its ongoing Phase 1 ARROW clinical trial of BLU-667 in patients with MTC and PTC at the 88th Annual Meeting of the American Thyroid Association. The data showed that BLU-667 is highly active and well-tolerated in these patient populations, with increased activity observed at higher dose levels and longer treatment durations. Ninety percent of evaluable patients with MTC and PTC experienced radiographic tumor reductions, regardless of RET alteration or prior multi-kinase inhibitor therapy. The response rate was 62 percent in patients with MTC in the 300 and 400 milligram once daily dose groups who were treated for at least 24 weeks. The data also showed that BLU-667 was well-tolerated, and most adverse events reported by investigators were Grade 1. Read the full data here.
- In September 2018, Blueprint Medicines presented two clinical case studies demonstrating proof-of-concept for BLU-667 in combination with Tagrisso® (osimertinib) in patients with treatment-resistant, EGFR-mutant NSCLC harboring an acquired RET fusion. The data showed that the combination of BLU-667 and osimertinib overcame resistance to standard therapy, and both patients achieved a partial response with a 78 percent reduction in target tumors per RECIST version 1.1. In these two patients, the combination was well-tolerated, and all reported adverse events were Grade 1 or 2. Read the full data here.

BLU-554: Hepatocellular Carcinoma (HCC)

- In September 2018, Blueprint Medicines and its partner, CStone Pharmaceuticals, submitted an investigational new drug (IND) application for BLU-554 to Chinese health authorities. Subject to approval of the IND application, the companies plan to expand Blueprint Medicines' ongoing Phase 1 clinical trial of BLU-554 as a monotherapy for the treatment of advanced HCC to include clinical sites in Mainland China. Additionally, the companies plan to initiate a proof-of-concept clinical trial evaluating BLU-554 in combination with CS1001, a clinical-stage anti-PD-L1 immunotherapy, in 2019.

Research Programs:

BLU-782: Fibrodysplasia Ossificans Progressiva (FOP)

- In September 2018, Blueprint Medicines presented preclinical proof-of-concept data for BLU-782, an investigational precision therapy specifically designed to target the underlying cause of FOP, at the 2018 American Society for Bone and Mineral Research Annual Meeting. The data showed that BLU-782 prevented injury- and surgery-induced heterotrophic ossification, reduced edema and restored healthy tissue response to muscle injury in a well-characterized FOP mouse model. Read the full data here.

- Blueprint Medicines expects to submit an IND application to the FDA for BLU-782 by the end of 2018, and subject to review of the IND application, plans to initiate a Phase 1 clinical trial in healthy volunteers in the first quarter of 2019. Upon completion of the Phase 1 clinical trial, Blueprint Medicines plans to advance BLU-782 into a registration-enabling Phase 2 clinical trial in patients with FOP.

Corporate:

- In October 2018, Blueprint Medicines announced the expansion of its leadership team with the appointment of Christina Rossi as Chief Commercial Officer. Blueprint Medicines also announced the appointment of Paul Beresford as General Manager, International.

Third Quarter Financial Results:

- **Cash Position:** As of September 30, 2018, cash, cash equivalents and investments were $559.6 million, as compared to $673.4 million as of December 31, 2017. This decrease was primarily related to cash used in operating activities, partially offset by the $40.0 million upfront payment received in connection with entering into the collaboration with CStone Pharmaceuticals and the $10.0 million milestone payment achieved under the Roche collaboration in June 2018.
- **Collaboration Revenues**: Collaboration revenues were $1.1 million for the third quarter of 2018, as compared to $8.1 million for the third quarter of 2017. This decrease was primarily due to the termination of the Alexion agreement in 2017.
- **R&D Expenses**: Research and development expenses were $64.6 million for the third quarter of 2018, as compared to $39.3 million for the third quarter of 2017. This increase was primarily attributable to increased clinical and manufacturing expenses associated with advancing avapritinib and BLU-667 further through clinical trials and increased personnel-related expenses. Research and development expenses included $4.8 million in stock-based compensation expenses for the third quarter of 2018.
- **G&A Expenses**: General and administrative expenses were $12.0 million for the third quarter of 2018, as compared to $7.4 million for the third quarter of 2017. This increase was primarily due to increased personnel-related expenses and increased professional fees, including pre-commercial planning activities. General and administrative expenses included $3.6 million in stock-based compensation expenses for the third quarter of 2018.
- **Net Loss:** Net loss was $72.7 million for the third quarter of 2018, or a net loss per share of $1.66, as compared to a net loss of $37.7 million for the third quarter of 2017, or a net loss per share of $0.96.

Financial Guidance:

Based on its current plans, Blueprint Medicines expects that its existing cash, cash equivalents and investments, excluding any potential option fees and milestone payments under its existing collaborations with Roche and CStone Pharmaceuticals, will be sufficient to enable it to fund its operating expenses and capital expenditure requirements into the second half of 2020.

Earnings Conference Call Information:

Blueprint Medicines will host a live conference call and webcast at 8:30 a.m. ET today to discuss third quarter 2018 financial results and recent business activities. The conference call may be accessed by dialing (855) 626-8618 (domestic) or (531) 289-2784 (international) and referring to conference ID 7598866. 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.

CTOS Conference Call Information:

Blueprint Medicines will host a live conference call and webcast to discuss data being presented at the 2018 CTOS Annual Meeting on November 15th at 7:30 a.m. ET. The conference call may be accessed by dialing (855) 728-4793 (domestic) or (503) 343-6666 (international) and referring to conference ID 3479587. 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 developing a new generation of targeted and potent kinase medicines to improve the lives of patients with genomically defined diseases. Its approach is rooted in a deep understanding of the genetic blueprint of cancer and other disease driven by the abnormal activation of kinases. Blueprint Medicines is advancing four programs in clinical development for subsets of patients with gastrointestinal stromal tumors, hepatocellular carcinoma, systemic mastocytosis, non-small cell lung cancer, medullary thyroid cancer and other advanced solid tumors, as well as multiple programs in research and preclinical development. For more information, please visit www.blueprintmedicines.com.

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 clinical development of avapritinib, BLU-554, BLU-667 and BLU-782; the potential benefits of Blueprint Medicines' current and future drug candidates in treating patients; plans and timelines for initiating Blueprint Medicines' PIONEER trial; the potential benefits of receiving Breakthrough Therapy Designation for avapritinib for the treatment of patients with advanced SM, including the subtypes of aggressive SM, SM with an associated hematologic neoplasm and mast cell leukemia; plans to present data from the Phase 1 EXPLORER clinical trial; plans and timelines for submitting an NDA to the FDA for avapritinib; plans and timelines for submitting an NDA to the FDA for BLU-667; plans and timelines for expanding Blueprint Medicines' ongoing Phase 1 clinical trial of BLU-554 monotherapy to include clinical sites in Mainland China; plans and timelines for initiating a proof-of-concept clinical trial evaluating BLU-554 in combination with CS1001; plans and timelines for submitting an IND application to the FDA for BLU-782; plans and timelines for initiating a Phase 1 clinical trial for BLU-782 in healthy volunteers; plans to work with clinical experts and the patient community to design a potential Phase 2 clinical trial of BLU-782 in patients with FOP; expectations regarding Blueprint Medicines' existing cash, cash equivalents and investments; and Blueprint Medicines' strategy, business

plans and focus. The words “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 delay of any current or planned clinical trials or the development of Blueprint Medicines’ drug candidates, including avapritinib, BLU-554, BLU-667 and BLU-782; Blueprint Medicines’ advancement of multiple early-stage efforts; Blueprint Medicines’ ability to successfully demonstrate the safety and efficacy of its drug candidates; 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, including companion diagnostic tests for BLU-554 for FGFR4-driven HCC, avapritinib for PDGFRα D842V-driven GIST and BLU-667 for RET-driven NSCLC; and the success of Blueprint Medicines’ cancer immunotherapy collaboration with F. Hoffmann-La Roche Ltd. and Hoffmann-La Roche Inc. and Blueprint Medicines’ collaboration with CStone Pharmaceuticals. These and other risks and uncertainties are described in greater detail in the section entitled “Risk Factors” in Blueprint Medicines’ Quarterly Report on Form 10-Q for the quarter ended June 30, 2018, as filed with the Securities and Exchange Commission (SEC) on August 1, 2018, 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:

Tagrisso® is a registered trademark of AstraZeneca plc. All other trademarks and trade names in this press release are the property of Blueprint Medicines Corporation.

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

	September 30 2018	December 31, 2017
Cash, cash equivalents and investments	$ 559,636	$ 673,356
Working capital [1]	509,378	642,615
Total assets	606,110	715,737
Deferred revenue	47,200	35,373
Term loan payable	277	1,518
Lease incentive obligation	15,046	16,331
Total stockholders' equity	489,296	623,970

[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,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Collaboration revenue	$ 1,095	$ 8,068	$ 43,488	$ 19,798
Operating expenses:				
Research and development	64,562	39,300	173,089	101,058
General and administrative	12,041	7,378	34,285	19,894
Total operating expenses	76,603	46,678	207,374	120,952
Other income (expense):				
Other income, net	2,799	954	7,635	2,240
Interest expense	(14)	(47)	(69)	(178)
Total other income	2,785	907	7,566	2,062
Net loss	$ (72,723)	$ (37,703)	$ (156,320)	$ (99,092)
Net loss per share — basic and diluted	$ (1.66)	$ (0.96)	$ (3.57)	$ (2.67)
Weighted-average number of common shares used in net loss per share — basic and diluted	43,915	39,130	43,825	37,053

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