# 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): August 1, 2018

# **Blueprint Medicines Corporation**

(Exact name of registrant as specified in its charter)

**Delaware** (State or other jurisdiction of incorporation) **001-37359** (Commission File Number) **26-3632015** (I.R.S. Employer Identification No.)

45 Sidney Street Cambridge, Massachusetts (Address of principal executive offices)

**02139** (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  $\Box$ 

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 August 1, 2018, Blueprint Medicines Corporation (the "Company") announced its financial results for the quarter ended June 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 August 1, 2018

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## 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: August 1, 2018

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

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#### **Blueprint Medicines Reports Second Quarter 2018 Financial Results**

 Presented Data from Phase 1 EXPLORER Clinical Trial of Avapritinib in Patients with Advanced Systemic Mastocytosis Showing Overall Response Rate of 83% and Durable Ongoing Reponses up to 22 Months –
 Initiated Phase 3 VOYAGER Clinical Trial of Avapritinib in Advanced Gastrointestinal Stromal Tumors –
 Entered into Exclusive Collaboration and License Agreement with CStone Pharmaceuticals to Develop and Commercialize Avapritinib, BLU-554 and BLU-667 in Greater China –

CAMBRIDGE, Mass., August 1, 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 second quarter ended June 30, 2018.

"In the second quarter, Blueprint Medicines continued to advance a broad portfolio, with progress across multiple programs," said Jeff Albers, Chief Executive Officer of Blueprint Medicines. "Importantly, we reported updated data from our Phase 1 EXPLORER trial in patients with advanced systemic mastocytosis that showed profound and durable clinical activity in nearly all patients. These data, combined with previously reported data from our ongoing Phase 1 NAVIGATOR trial in advanced gastrointestinal stromal tumors, reinforce our confidence in avapritinib as a potentially transformative therapy across multiple patient populations. By the end of this year, we expect to have four pivotal clinical trials of avapritinib underway, with the potential to rapidly advance toward approval in defined patient populations."

#### **Clinical Programs:**

#### Avapritinib: Gastrointestinal Stromal Tumors (GIST)

- In June 2018, Blueprint Medicines announced the dosing of the first patient in its Phase 3 VOYAGER clinical trial, which will evaluate the safety and efficacy of avapritinib compared to regorafenib in patients with third- or fourthline advanced GIST.
- In June 2018, Blueprint Medicines presented data from a retrospective natural history study of patients with advanced PDGFRα D842V-driven GIST at the 2018 American Society of Clinical Oncology (ASCO) Annual Meeting. The data confirmed that patients with advanced PDGFRα D842V-driven GIST are unlikely to respond to currently available tyrosine kinase inhibitors (TKIs), illustrating the high unmet need for new therapies in this patient population with a short survival rate.
- Blueprint Medicines continues to evaluate avapritinib in its Phase 1 NAVIGATOR clinical trial and anticipates
  presenting updated data across multiple patient populations, including PDGFRA-driven GIST, third-line or later
  GIST and second-line GIST, in the second half of 2018. Additionally, based on data from this trial, the Company
  plans to submit a new drug application (NDA) to the U.S. Food and Drug Administration (FDA) for avapritinib for
  the treatment of patients with PDGFRA-driven GIST and fourth-line KIT-driven GIST in the first half of 2019.

#### Avapritinib: Advanced Systemic Mastocytosis (SM)

In June 2018, Blueprint Medicines presented updated clinical data from its ongoing Phase 1 EXPLORER clinical trial of avapritinib in patients with advanced SM at the 23<sup>rd</sup> Congress of the European Hematology Association. The data showed an overall response rate of 83 percent and durable ongoing responses up to 22 months. All evaluable patients showed marked decreases on one or more objective measures of mast cell burden, regardless of advanced SM subtype, previous treatment or starting dose level. The data also showed that avapritinib was generally well-tolerated. Most adverse events reported by investigators were Grade 1 or 2, and only three patients discontinued due to a treatment-related adverse event. Read the full data here.

• Blueprint Medicines plans to initiate screening of patients for enrollment in PATHFINDER, a registration-enabling, open-label, single-arm Phase 2 clinical trial in patients with advanced SM, in the third quarter of 2018, and plans to initiate PIONEER, a registration-enabling, randomized, placebo-controlled Phase 2 clinical trial in patients with indolent and smoldering SM, by the end of 2018.

## BLU-667: RET-Altered Solid Tumors

Blueprint Medicines continues to enroll patients in the expansion portion of its ongoing Phase 1 ARROW clinical trial of BLU-667 at a dose of 400 mg once daily. In the expansion, patients are being enrolled in four defined cohorts: RET-altered non-small cell lung cancer (NSCLC) patients previously treated with a TKI; RET-altered NSCLC patients who have not previously received any TKI treatment; patients with medullary thyroid cancer; and patients with other RET-altered solid tumors.

## BLU-554: Hepatocellular Carcinoma (HCC)

In June 2018, Blueprint Medicines announced plans to initiate a proof-of-concept clinical trial with CStone Pharmaceuticals in China to evaluate BLU-554 in combination with CS1001, a clinical-stage anti-programmed death ligand-1 (PD-L1) immunotherapy being developed by CStone Pharmaceuticals, as a first-line treatment for patients with HCC. Additionally, the companies plan to expand Blueprint Medicines' ongoing Phase 1 clinical trial of BLU-554 as a monotherapy to include new sites in Mainland China. The companies expect to submit an investigational new drug (IND) application for BLU-554 to the Chinese health authorities by the end of 2018, and plan to initiate the clinical trial evaluating BLU-554 in combination with CS1001 and the expansion of Blueprint Medicines' ongoing clinical trial for BLU-554 as a monotherapy in Mainland China in 2019.

## **Corporate:**

- In June 2018, Blueprint Medicines announced an exclusive collaboration and license agreement with CStone Pharmaceuticals to develop and commercialize avapritinib, BLU-554 and BLU-667 in Mainland China, Hong Kong, Macau and Taiwan, either as a monotherapy or as part of a combination therapy. Under the terms of the agreement, Blueprint Medicines received an upfront cash payment of \$40.0 million, will be eligible to receive up to approximately \$346.0 million in potential milestone payments and tiered percentage royalties in the mid-teens to low twenties on annual net sales of each licensed product in the territory.
- Blueprint Medicines recently received a \$10.0 million milestone payment from Roche following the achievement of a research milestone.

#### **Second Quarter Financial Results:**

- **Cash Position:** As of June 30, 2018, cash, cash equivalents and investments were \$616.7 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 Blueprint Medicines entering into the collaboration with CStone Pharmaceuticals and the \$10.0 million milestone payment received from Roche.
- **Collaboration Revenues**: Collaboration revenues were \$41.4 million for the second quarter of 2018, as compared to \$5.9 million for the second quarter of 2017. This increase was primarily due to revenue recognized under the collaboration agreement with CStone Pharmaceuticals.
- R&D Expenses: Research and development expenses were \$58.6 million for the second quarter of 2018, as compared to \$33.3 million for the second quarter of 2017. This increase was primarily attributable to increased clinical and manufacturing expenses associated with advancing avapritinib, BLU-554 and BLU-667 further through clinical trials and increased personnel-related expenses. Research and development expenses included \$4.3 million in stock-based compensation expenses for the second quarter of 2018.
- **G&A Expenses**: General and administrative expenses were \$12.3 million for the second quarter of 2018, as compared to \$6.8 million for the second quarter of 2017. This increase was primarily attributable to increased personnel-related expenses and increased professional fees, including pre-commercial planning activities. General and administrative expenses included \$3.5 million in stock-based compensation expenses for the second quarter of 2018.

• **Net Loss:** Net loss was \$27.0 million the second quarter of 2018, or a net loss per share of \$0.62, as compared to a net loss of \$33.4 million for the second quarter of 2017, or a net loss per share of \$0.86.

#### **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.

#### **Conference Call Information:**

Blueprint Medicines will host a live conference call and webcast today at 8:30 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 5597837. A webcast of the conference call will be available in the Investors section of Blueprint Medicines' website at <u>http://ir.blueprintmedicines.com</u>. 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 multiple 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 <u>www.blueprintmedicines.com</u>.

## **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 presenting preclinical and clinical data for Blueprint Medicines' current or future clinical trials; plans and timelines for initiating Blueprint Medicines' PATHFINDER and PIONEER trials; plans and timelines for submitting an NDA to the FDA for avapritinib or an IND to the Chinese health authorities for BLU-554; 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 D842Vdriven 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 March 31, 2018, as filed with the Securities and Exchange Commission (SEC) on May 2, 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.

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

	June 30		December 31,	
		2018		2017
Cash, cash equivalents and investments	\$	616,651	\$	673,356
Working capital <sup>(1)</sup>		571,880		642,615
Total assets		665,113		715,737
Deferred revenue		48,295		35,373
Term loan payable		692		1,518
Lease incentive obligation		15,474		16,331
Total stockholders' equity		552,932		623,970

<sup>(1)</sup> 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 June 30,		Six Months Ended June 30,		
	2018	2017	2018	2017	
Collaboration revenue	\$ 41,439	\$ 5,890	\$ 42,393	\$ 11,730	
Operating expenses:					
Research and development	58,573	33,271	108,527	61,758	
General and administrative	12,333	6,833	22,244	12,516	
Total operating expenses	70,906	40,104	130,771	74,274	
Other income (expense):					
Other income, net	2,442	861	4,836	1,286	
Interest expense	(23)	(59)	(55)	(131)	
Total other income	2,419	802	4,781	1,155	
Net loss	\$ (27,048)	\$ (33,412)	\$ (83,597)	\$ (61,389)	
Net loss per share applicable to common stockholders — basic and					
diluted	\$ (0.62)	\$ (0.86)	\$ (1.91)	\$ (1.71)	
Weighted-average number of common shares used in net loss per share applicable to common stockholders — basic and diluted	43,856	38,775	43,779	35,998	

## Investor and Media Relations Contacts:

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