# 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 31, 2017

# **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.)

38 Sidney Street, Suite 200
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 

☑

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.  $\square$ 

## Item 2.02 Results of Operations and Financial Condition.

On October 31, 2017, Blueprint Medicines Corporation (the "Company") announced its financial results for the quarter ended September 30, 2017. 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 31, 2017								
	2								

# EXHIBIT INDEX

Exhibit No.Description99.1Press release issued by Blueprint Medicines Corporation on October 31, 2017

# **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 31, 2017

By: /s/ Jeffrey W.

Albers

Jeffrey W. Albers Chief Executive Officer



#### **Blueprint Medicines Reports Third Quarter 2017 Financial Results**

- Presented clinical data from ongoing Phase 1 trial of BLU-554 in advanced hepatocellular carcinoma (HCC) at ESMO and ILCA 2017 Congress –
- Updated BLU-285 data in advanced gastrointestinal stromal tumors (GIST) to be presented at CTOS Annual Meeting on November 10, 2017 –
  - Updated BLU-285 data in advanced systemic mastocytosis (SM) to be presented before year-end

CAMBRIDGE, Mass., October 31, 2017 – 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, 2017.

"In the third quarter, we continued to advance Blueprint Medicines' broad and diversified portfolio of compelling clinical and research programs through solid execution, rigorous scientific evaluation and disciplined resource management," said Jeff Albers, Chief Executive Officer of Blueprint Medicines. "In September, we announced new Phase 1 clinical trial results for our highly selective FGFR4 inhibitor BLU-554, which showed encouraging clinical activity in heavily pretreated patients with advanced FGFR4-driven HCC. In addition, we enhanced our wholly-owned research-stage pipeline with the transition of the fibrodysplasia ossificans progressiva program from Alexion and the nomination of an additional program for an undisclosed kinase target. Collectively, we continue to take great pride in our growing drug discovery and development capabilities, as we advance toward our goal of becoming the leading developer of highly selective kinase medicines."

#### **Clinical Programs**

#### BLU-285: Gastrointestinal Stromal Tumors

- Blueprint Medicines continues to enroll patients in the expansion part of the ongoing Phase 1 clinical trial of its highly selective KIT and PDGFRα inhibitor BLU-285 in patients with advanced GIST. Blueprint Medicines expects to complete enrollment of the PDGFRα D842V expansion cohort, which is expected to include approximately 50 patients, by the middle of 2018. Blueprint Medicines also plans to initiate a global, pivotal Phase 3 clinical trial of BLU-285 in third-line patients with KIT-driven GIST in the first half of 2018, with the goal of supporting the registration of BLU-285 in a broader GIST patient population.
- · Updated data from Blueprint Medicines' ongoing Phase 1 clinical trial will be presented at the 22<sup>nd</sup> Connective Tissue Oncology Society (CTOS) Annual Meeting on Friday, November 10, 2017 in Maui, Hawaii. Following the presentation, a copy of the presentation will also be available under the "Abstracts, Posters and Presentations" section of Blueprint Medicines' website at http://www.blueprintmedicines.com/abstracts-posters-and-presentations/.

#### BLU-285: Systemic Mastocytosis

 Blueprint Medicines continues to enroll patients in the expansion part of the ongoing Phase 1 clinical trial of BLU-285 in patients with advanced SM. Blueprint Medicines anticipates presenting updated data from this clinical trial by the end of 2017.

#### BLU-554: Hepatocellular Carcinoma

· In September 2017, Blueprint Medicines presented updated data from its ongoing Phase 1 clinical trial of BLU-554 in patients with advanced HCC at the European Society for Medical Oncology (ESMO) 2017 Congress in Madrid, Spain and the 11<sup>th</sup> International Liver Cancer Association (ILCA) Annual Conference in Seoul, South Korea. In evaluable patients with fibroblast growth factor receptor 4 (FGFR4)-driven HCC, the data showed an objective response rate (ORR) of 16 percent by Response Evaluation Criteria in Solid Tumors (RECIST) version 1.1. In addition, 49 percent of patients with FGFR4-driven HCC experienced

radiographic tumor reduction, and clinical activity was observed regardless of disease etiology, prior treatment with immunotherapy, such as nivolumab, or geography. In contrast, currently approved tyrosine kinase inhibitors (TKIs) provide response rates of less than 10 percent. Preliminary evidence of prolonged disease control was observed in patients with FGFR4-driven HCC who had not received prior treatment with a TKI, such as sorafenib. The data also showed that BLU-554 was well-tolerated, and most adverse events reported by investigators were Grade 1 or 2 (mild or moderate). Read the full data here.

Also in September 2017, Blueprint Medicines announced plans to initiate in the first quarter of 2018 an
additional cohort in its ongoing Phase 1 clinical trial to evaluate BLU-554 in TKI-naïve patients with FGFR4driven HCC and to explore opportunities to conduct a clinical trial to evaluate BLU-554 in combination with an
immune checkpoint inhibitor.

#### BLU-667: RET-altered solid tumors

- Blueprint Medicines continues to enroll patients in the dose escalation part of its ongoing Phase 1 clinical trial of its highly selective RET inhibitor BLU-667 in patients with RET-altered non-small cell lung cancer (NSCLC), medullary thyroid cancer (MTC) and other RET-altered solid tumors. On October 29, 2017, new preclinical data supporting the clinical development of BLU-667 in patients with RET-altered solid tumors was presented at the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics in Philadelphia, Pennsylvania. Reported *in vitro* and *in vivo* study results showed that BLU-667 is active against KIF5B-RET fusion NSCLC as well as predicted resistance mutations, including the V804M gatekeeper mutation, without inhibition of off-target kinases with known toxicity profiles, such as vascular endothelial growth factor receptor 2.
- In October 2017, Blueprint Medicines entered into a collaboration with Thermo Fisher Scientific to develop and commercialize a companion diagnostic test to identify NSCLC patients with RET fusions for use with BLU-667

#### **Research Programs**

- Based on a scientific review of discovery-stage portfolio assets, Blueprint Medicines plans to prioritize continued development of its research program targeting the ALK2 kinase for the treatment of fibrodysplasia ossificans progressiva (FOP) with the goal of identifying a potential development candidate. The termination of Blueprint Medicines' collaboration with Alexion Pharma Holding (Alexion) became effective in October 2017, enabling Blueprint Medicines to transition the FOP program into its research portfolio as a wholly-owned program. As a result of the termination, Blueprint Medicines' exclusivity obligations under the Alexion agreement terminated, and Blueprint Medicines will not have any current or future payment obligations to Alexion.
- · Based on a scientific review of its discovery-stage portfolio assets, Blueprint Medicines plans to deprioritize its research program targeting protein kinase cAMP-activated catalytic subunit alpha fusions (PRKACA) for the treatment of fibrolamellar carcinoma (FLC).
- Blueprint Medicines also recently initiated an additional discovery program for an undisclosed kinase target.

#### **Corporate Highlights**

· In October 2017, Blueprint Medicines announced the appointment of Christopher Murray, Ph.D., as Senior Vice President, Technical Operations. In this new role, Dr. Murray will be responsible for technical development, manufacturing and supply chain operations, and he will support the expansion of clinical activities for Blueprint Medicines' lead product candidates and plans to address potential future commercial supply demand.

#### Third Quarter 2017 Financial Results

- Cash Position: As of September 30, 2017, cash, cash equivalents and investments were \$390.7 million, as compared to \$268.2 million as of December 31, 2016. This increase was primarily related to \$215.6 million in net proceeds from the April 2017 follow-on underwritten public offering, partially offset by \$85.1 million in cash used to fund operating activities for the nine months ended September 30, 2017.
- **Collaboration Revenue:** Collaboration revenues were \$8.1 million for the third quarter of 2017, compared to \$6.2 million for the third quarter of 2016. This increase was primarily due to revenue recognized under the Alexion collaboration in connection with the termination of the collaboration and transition of the FOP program.
- **R&D Expenses:** Research and development expenses were \$39.3 million for the third quarter of 2017, including \$1.8 million of stock-based compensation expenses, as compared to \$18.2 million for the third quarter of 2016, including \$0.6 million of stock-based compensation expense. This increase was primarily due to increased clinical and manufacturing expenses associated with advancing Blueprint Medicines' ongoing clinical trials for BLU-285, BLU-554, and BLU-667 and increased personal-related expenses.
- **G&A Expenses:** General and administrative expenses were \$7.4 million for the third quarter of 2017, including \$1.8 million of stock-based compensation expense, as compared to \$4.9 million for the third quarter of 2016, including \$1.3 million of stock-based compensation expense. This increase was primarily due to increased personnel expenses related to an increase in headcount, as well as increased professional fees.
- **Net Loss:** Net loss was \$37.7 million for the third quarter of 2017, or a net loss per share of \$0.96, as compared to a net loss of \$16.8 million for the third quarter of 2016, or a net loss per share of \$0.62.

#### **Financial Guidance**

Based on its current plans, Blueprint Medicines believes its existing cash, cash equivalents and investments, excluding any potential option fees and milestone payments under its existing collaboration with Roche, will be sufficient to enable it to fund its operating expenses and capital expenditure requirements into the second half of 2019.

#### **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 2042388. A webcast of the conference call will be available in the Investors section of 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 diseases 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.

#### **Availability of Other Information About Blueprint Medicines**

Investors and others should note that Blueprint Medicines communicates with its investors and the public using its company website (www.blueprintmedicines.com), including but not limited to investor presentations and scientific presentations, Securities and Exchange Commission filings, press releases, public conference calls and webcasts. You can also connect with Blueprint Medicines on Twitter (@BlueprintMeds) or LinkedIn. The information that Blueprint Medicines posts on these channels and websites could be deemed to be material information. As a result, Blueprint Medicines encourages investors, the media and others interested in Blueprint Medicines to review the information that it posts on these channels, including Blueprint Medicines' investor relations website, on a regular basis. This list of channels may be updated from time to time on Blueprint Medicines' investor relations website and may include other social media channels than the ones described above. The contents of Blueprint Medicines' website or these channels, or any other website that may be accessed from its website or these channels, shall not be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended.

#### **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 BLU-285, BLU-554 and BLU-667; the timing of updated clinical data for Blueprint Medicines' Phase 1 clinical trials for BLU-285 and the timing of initial clinical data for Blueprint Medicines' Phase 1 clinical trial for BLU-667; plans to prioritize continued development of Blueprint Medicines' research program targeting the ALK2 kinase for the treatment of FOP and to deprioritize its research program targeting PRKACA for the treatment of FLC; 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 BLU-285, BLU-554 and BLU-667; Blueprint Medicines' advancement of multiple early-stage efforts; Blueprint Medicines' ability to successfully demonstrate the efficacy and safety of its drug candidates; the preclinical and clinical results for Blueprint Medicines' drug candidates, which may not support further development of such drug candidates; and 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, BLU-285 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. 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, 2017, as filed with the Securities and Exchange Commission (SEC) on August 2, 2017, and other filings that Blueprint Medicines 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. Blueprint Medicines explicitly disclaims any obligation to update any forward-looking statements.

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

	September 30,		 December 31,
		2017	2016
Cash, cash equivalents and investments	\$	390,652	\$ 268,218
Unbilled accounts receivable		3,435	3,577
Working capital (1)		364,789	191,913
Total assets		420,118	282,795
Deferred revenue		36,772	47,235
Term loan payable		2,095	4,069
Lease incentive obligation		9,900	3,370
Total stockholders' equity		341,240	213,078

(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		<b>Nine Months Ended</b>					
	September 30,			September 30,				
		2017		2016		2017		2016
Collaboration revenue	\$	8,068	\$	6,160	\$	19,798	\$	20,081
Operating expenses:								
Research and development		39,300		18,150		101,058		57,058
General and administrative		7,378		4,893		19,894		14,227
Total operating expenses		46,678		23,043		120,952		71,285
Other income (expense):								
Other income (expense), net		954		158		2,240		350
Interest expense		(47)		(109)		(178)		(378)
Total other income (expense)		907		49		2,062		(28)
Net loss		37,703)	\$(	16,834)	\$ (	(99,092)	\$	(51,232)
Net loss per share applicable to common stockholders — basic and		_						
diluted	\$	(0.96)	\$	(0.62)	\$	(2.67)	\$	(1.89)
Weighted-average number of common shares used in net loss per share applicable to common stockholders — basic and diluted								
		39,130		27,251		37,053		27,170

# **Media and Investor Relations Contacts**

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