

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

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**FORM 8-K**

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**CURRENT REPORT  
Pursuant to Section 13 or 15(d)  
of the Securities Exchange Act of 1934**

Date of Report (Date of Earliest Event Reported): **February 21, 2018**

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**Blueprint Medicines Corporation**

(Exact name of registrant as specified in its charter)

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**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**

**38 Sidney Street, Suite 200**  
**Cambridge, Massachusetts 02139**  
(Former name or former address, if changed since last report)

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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.

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**Item 2.02 Results of Operations and Financial Condition.**

On February 21, 2018, Blueprint Medicines Corporation (the “Company”) announced its financial results for the quarter and year ended December 31, 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:

<u>Exhibit No.</u>	<u>Description</u>
99.1	Press release issued by Blueprint Medicines Corporation on February 21, 2018

EXHIBIT INDEX

<u>Exhibit No.</u>	<u>Description</u>
99.1	<a href="#">Press release issued by Blueprint Medicines Corporation on February 21, 2018</a>

**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: February 21, 2018

By: /s/ Jeffrey W.

Albers

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Jeffrey W. Albers

Chief Executive Officer



## Blueprint Medicines Reports Fourth Quarter and Full Year 2017 Financial Results

- Presented clinical data from ongoing Phase 1 trials of avapritinib in patients with advanced gastrointestinal stromal tumors and advanced systemic mastocytosis at CTOS and ASH Annual Meetings –
- BLU-782 selected as development candidate for the treatment of fibrodysplasia ossificans progressiva –
- Completed successful follow-on offering in December 2017 and ended year with \$673.4 million in cash, cash equivalents and investments –
- Plans to present initial clinical data from Phase 1 trial of BLU-667 in patients with RET-altered solid tumors on track for first half of 2018 –

CAMBRIDGE, Mass., February 21, 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 fourth quarter and full year ended December 31, 2017.

“2017 marked a year of significant achievements for Blueprint Medicines as we moved closer to realizing our vision of delivering a new generation of kinase medicines to patients with genomically defined diseases,” said Jeff Albers, Chief Executive Officer of Blueprint Medicines. “We presented transformative data across our full clinical-stage portfolio and expanded our extensive pipeline of highly selective kinase medicines with the nomination of our fourth development candidate, BLU-782. In 2018, we expect to continue this cadence of clinical and preclinical data disclosures, while also working to progress avapritinib quickly toward potential approval for gastrointestinal stromal tumors and systemic mastocytosis and to define the development path for BLU-554. We also plan to devote additional resources to commercial readiness as we progress pivotal clinical trials in multiple patient populations.”

### Clinical Programs:

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

- In November 2017, Blueprint Medicines presented updated data from the dose escalation and expansion portion of its ongoing Phase 1 clinical trial of avapritinib in patients with advanced GIST, called the Navigator trial, at the 22<sup>nd</sup> Connective Tissue Oncology Society (CTOS) Annual Meeting. Among evaluable patients with heavily pretreated KIT-driven GIST treated with 300 to 400mg of avapritinib once daily, the data showed radiographic tumor reductions in 67 percent of these patients, an objective response rate (ORR) of 17 percent and median progression free survival (PFS) of 11.5 months. In evaluable patients with PDGFR $\alpha$  D842V-driven GIST, the data showed an ORR of 71 percent and an estimated 12-month PFS of 78 percent. The data also showed that avapritinib was generally well-tolerated, and most adverse events (AEs) reported by investigators were Grade 1 or 2. Read the full data here.
- Also in November 2017, Blueprint Medicines expanded its ongoing Navigator trial to increase the enrollment target for patients previously treated with imatinib and at least one additional prior tyrosine kinase inhibitor (TKI) from 50 to 100 patients and added a new cohort to evaluate avapritinib in second-line GIST patients. Blueprint Medicines continues to expect to complete enrollment of the PDGFR $\alpha$  D842V expansion cohort by the middle of 2018. Blueprint Medicines also plans to initiate a global, randomized Phase 3 clinical trial evaluating avapritinib compared to regorafenib in third-line patients with KIT-driven GIST, called the Voyager trial, in the first half of 2018, with the goal of supporting the potential approval of avapritinib in a broader population of GIST patients.

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

- In December 2017, Blueprint Medicines presented updated data from the dose escalation portion of its ongoing Phase 1 clinical trial of avapritinib in patients with advanced SM, called the Explorer trial, at the 59th American Society of Hematology Annual Meeting and Exposition (ASH). The data showed an ORR of 72 percent and a disease control rate of 100 percent in patients evaluable for response, based on the

International Working Group – Myeloproliferative Neoplasms Research and Treatment and European Competence Network on Mastocytosis consensus criteria. The data also showed that avapritinib was generally well-tolerated and most AEs reported by investigators were Grade 1 or 2. Read the full data here.

- Also in December 2017, Blueprint Medicines announced plans to engage global regulatory authorities in the first half of 2018 to obtain input on registration pathways for avapritinib in patients with advanced SM and patients with indolent and smoldering SM. Blueprint Medicines expects to initiate a registration-enabling Phase 2 clinical trial in patients with advanced SM in the first half of 2018 and a dose-finding and proof-of-concept Phase 2 clinical trial in patients with indolent and smoldering SM in the second half of 2018. Blueprint Medicines continues to enroll patients in the expansion portion of its ongoing Explorer trial, with the goal of generating additional data in 2018.

#### *Avapritinib: Recent Scientific Publications*

- In November 2017, Blueprint Medicines announced the publication of preclinical data and clinical case studies for avapritinib, highlighting the potent activity of avapritinib against activation loop mutations, as well as a broad spectrum of additional clinically relevant mutations, with a selectivity profile minimizing inhibition of other kinases. The paper, titled “A precision therapy against cancers driven by KIT/PDGFRA mutations” was published online in *Science Translational Medicine*.

#### *BLU-554: Hepatocellular Carcinoma*

- Blueprint Medicines continues to enroll patients in the expansion portion of its ongoing Phase 1 clinical trial of BLU-554 in patients with advanced hepatocellular carcinoma (HCC). In November 2017, Blueprint Medicines added a new cohort to this trial to enroll approximately 40 TKI-naïve patients with FGFR4-driven HCC. Blueprint Medicines is also exploring opportunities to evaluate BLU-554 in combination with an immune checkpoint inhibitor. Blueprint Medicines plans to report updated data from the expansion portion of its ongoing Phase 1 clinical trial for advanced HCC, including from the new TKI-naïve cohort, in the second half of 2018.

#### *BLU-667: RET-Altered Solid Tumors*

- Blueprint Medicines continues to enroll patients in the dose escalation portion of its ongoing Phase 1 clinical trial of BLU-667 in patients with RET-altered non-small cell lung cancer (NSCLC), medullary thyroid cancer (MTC) and other advanced solid tumors. In December 2017, Blueprint Medicines provided an update on its ongoing Phase 1 clinical trial. As previously reported, as of December 1, 2017, 30 patients were enrolled in the trial, and BLU-667 was generally well-tolerated. The majority of adverse events reported by investigators were Grade 1, and the maximum tolerated dose or recommended part 2 dose had not yet been established. Investigators reported preliminary evidence of clinical activity in patients with RET-altered NSCLC, including patients with KIF5B and other RET fusions, and RET-altered MTC. Blueprint Medicines plans to report preliminary clinical data and initiate the expansion portion of this Phase 1 clinical trial in the first half of 2018.

#### **Research Programs:**

- In December 2017, Blueprint Medicines announced the selection of BLU-782 as its development candidate for the treatment of patients with fibrodysplasia ossificans progressiva, a rare genetic disease caused by mutations in the ALK2 gene. Blueprint Medicines plans to initiate investigational new drug (IND) application-enabling studies for BLU-782 in the first half of 2018 and plans to report preclinical data for this program in 2018.

#### **Corporate Highlights:**

- In December 2017, Blueprint Medicines announced the closing of an underwritten public offering of 4,259,259 shares of its common stock at a public offering price of \$81.00 per share, including the exercise in full by the underwriters of their option to purchase additional shares of common stock. Blueprint
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Medicines received net proceeds from the offering of approximately \$325.7 million, after deducting underwriting discounts and commissions and offering expenses.

#### **Fourth Quarter and Year End 2017 Financial Results:**

- **Cash Position:** As of December 31, 2017, cash, cash equivalents and investments were \$673.4 million, as compared to \$268.2 million as of December 31, 2016. This increase was primarily due to an aggregate of \$541.3 million in net proceeds from Blueprint Medicines' underwritten public offerings in April and December 2017, partially offset by \$119.9 million in cash used to fund operating activities for the year ended December 31, 2017.
- **Collaboration Revenues:** Collaboration revenues were \$1.6 million for the fourth quarter of 2017 and \$21.4 million for the year ended December 31, 2017, as compared to \$7.7 million for the fourth quarter of 2016 and \$27.8 million for the year ended December 31, 2016. This decrease was primarily due to the termination of the Alexion agreement during the fourth quarter of 2017.
- **R&D Expenses:** Research and development expenses were \$43.6 million for the fourth quarter of 2017 and \$144.7 million for the year ended December 31, 2017, as compared to \$24.1 million for the fourth quarter of 2016 and \$81.1 million for the year ended December 31, 2016. This increase was primarily attributable to increased clinical and manufacturing expenses associated with advancing avapritinib, BLU-554, and BLU-667 further through Phase 1 clinical trials and increased personnel-related expenses. Research and development expenses included \$1.9 million in stock-based compensation expenses for the fourth quarter of 2017 and \$6.3 million in stock-based compensation expenses for the year ended December 31, 2017.
- **G&A Expenses:** General and administrative expenses were \$8.1 million for the fourth quarter of 2017 and \$28.0 million for the year ended December 31, 2017, as compared to \$5.0 million for the fourth quarter of 2016 and \$19.2 million for the year ended December 31, 2016. This increase was primarily attributable to increased personnel-related expenses and professional fees, including market research and public relation costs. General and administrative expenses included \$1.8 million in stock-based compensation expenses for the fourth quarter of 2017 and \$6.2 million in stock-based compensation expenses for the year ended December 31, 2017.
- **Net Loss:** Net loss was \$49.0 million for the fourth quarter of 2017 and \$148.1 million for the year ended December 31, 2017, or a net loss per share of \$1.23 and \$3.92, respectively, as compared to a net loss of \$21.3 million for the fourth quarter of 2016 and \$72.5 million for the year ended December 31, 2016, or a net loss per share of \$0.75 and \$2.64, respectively.

#### **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 collaboration with Roche, will be sufficient to enable it to fund its operating expenses and capital expenditure requirements into the middle 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 1-855-728-4793 (domestic) or 1-503-343-6666 (international) and referring to conference ID 3391675. 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 diseases driven by the abnormal activation of kinases. Blueprint Medicines is advancing four

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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](http://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](http://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 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' Voyager trial; plans and timelines for initiating a registration-enabling clinical trial in patients with advanced SM; plans and timelines for initiating a proof-of-concept Phase 2 clinical trial in patients with indolent and smoldering SM; plans and timelines for engaging regulatory authorities to obtain input on registration pathways for avapritinib and BLU-554; the timing of initial clinical data for Blueprint Medicines' Phase 1 clinical trial for BLU-667; plans and timelines for initiating the expansion portion of Blueprint Medicines' Phase 1 clinical trial for BLU-667; plans and timelines for initiating IND-enabling studies for BLU-782; the timing for reporting preclinical data for the BLU-782 program; plans to devote additional resources to commercial readiness; expectations regarding plans and timelines for pivotal clinical trials in multiple patient populations; expectations regarding potential milestones; 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 PDGFR $\alpha$  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 September 30, 2017, as filed with the Securities and Exchange Commission (SEC)

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on October 31, 2017, 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.

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

	<u>December 31,</u>		<u>December 31,</u>	
	<u>2017</u>		<u>2016</u>	
Cash, cash equivalents and investments	\$	673,356	\$	268,218
Unbilled accounts receivable		—		3,577
Working capital (1)		642,615		191,913
Total assets		715,737		282,795
Deferred revenue		35,373		47,235
Term loan payable		1,518		4,069
Lease incentive obligation		16,331		3,370
Total stockholders' equity		623,970		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)

	<u>Three Months Ended</u>		<u>Years Ended</u>	
	<u>December 31,</u>		<u>December 31,</u>	
	<u>2017</u>	<u>2016</u>	<u>2017</u>	<u>2016</u>
Collaboration revenue	\$ 1,628	\$ 7,691	\$ 21,426	\$ 27,772
Operating expenses:				
Research and development	43,629	24,073	144,687	81,131
General and administrative	8,092	4,991	27,986	19,218
Total operating expenses	51,721	29,064	172,673	100,349
Other income (expense):				
Other income, net	1,108	201	3,349	551
Interest expense	(42)	(91)	(221)	(469)
Total other income	1,066	110	3,128	82
Net loss	\$ (49,027)	\$ (21,263)	\$ (148,119)	\$ (72,495)
Net loss per share applicable to common stockholders — basic and diluted	\$ (1.23)	\$ (0.75)	\$ (3.92)	\$ (2.64)
Weighted-average number of common shares used in net loss per share applicable to common stockholders — basic and diluted	39,988	28,450	37,793	27,492

**Media and Investor Relations Contacts:**

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