

**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 26, 2019**

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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**

(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 26, 2019, Blueprint Medicines Corporation announced its financial results for the quarter and year ended December 31, 2018 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 Current Report on Form 8-K shall be deemed to be furnished and not filed:

<u>Exhibit No.</u>	<u>Description</u>
99.1	<a href="#"><u>Press release issued by Blueprint Medicines Corporation on February 26, 2019</u></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 26, 2019

By: /s/ Jeffrey W.

Albers

Jeffrey W. Albers

Chief Executive Officer



## Blueprint Medicines Reports Fourth Quarter and Full Year 2018 Financial Results

- Avapritinib NDA for PDGFRA Exon 18 mutant GIST and fourth-line GIST on track for submission to FDA in second quarter --
- BLU-667 receives Breakthrough Therapy Designation for the treatment of RET mutation-positive medullary thyroid cancer --
- Continued clinical progress across the portfolio with dosing initiated in registration-enabling Phase 2 PIONEER trial of avapritinib in indolent systemic mastocytosis and Phase 1 trial of BLU-782 in healthy volunteers --
- Avapritinib and BLU-667 data submitted for presentation at ASCO --

CAMBRIDGE, Mass., February 26, 2019 – 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 fourth quarter and full year ended December 31, 2018.

“Following a year of remarkable clinical progress across our portfolio in 2018, we are focused on executing our ‘2020 Blueprint’ vision to transform Blueprint Medicines into a fully-integrated global precision therapy company,” said Jeff Albers, Chief Executive Officer of Blueprint Medicines. “The cornerstone of this effort is our planned NDA submission for avapritinib for patients with PDGFRA Exon 18 mutant GIST and fourth-line GIST in the second quarter. As we work to bring this new therapy to GIST patients who currently have no approved treatment options, we are also partnering with treating physicians, the patient community and testing companies to evolve the GIST treatment paradigm toward precision medicine, with the shared goals of maximizing patient outcomes, enabling efficient clinical trials and delivering value to the healthcare system.”

### Fourth Quarter 2018 Highlights and Recent Progress:

#### *Avapritinib: Gastrointestinal stromal tumors (GIST):*

- Locked the registration database and reported top-line results from the Phase 1 NAVIGATOR trial of avapritinib in patients with PDGFRA Exon 18 mutant GIST and fourth-line GIST in preparation for the planned submission of a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) in the second quarter of 2019. Read the top-line data [here](#).
- Presented updated data from the Phase 1 NAVIGATOR trial across treatment lines at the Connective Tissue Oncology Society 2018 Annual Meeting in November 2018 and disclosed plans to conduct the registration-enabling Phase 3 COMPASS-2L precision medicine trial of avapritinib in second-line GIST. Read the full data [here](#).
- Under Blueprint Medicines’ collaboration with CStone Pharmaceuticals, announced the China National Medical Products Administration (NMPA) cleared an Investigational New Drug (IND) application for the ongoing Phase 3 VOYAGER trial of avapritinib in third-line GIST.

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

- Presented updated data from the Phase 1 EXPLORER trial at the American Society of Hematology Annual Meeting and Exposition in December 2018. Read the full data [here](#).
- Initiated patient dosing in two registration-enabling trials: the Phase 2 PATHFINDER trial in advanced SM and the Phase 2 PIONEER trial in indolent and smoldering SM.

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

- Today announced the FDA has granted Breakthrough Therapy Designation to BLU-667 for the treatment of RET-mutation-positive medullary thyroid cancer (MTC) that requires systemic treatment and for which there are no acceptable alternative treatments.

*BLU-554: Hepatocellular carcinoma (HCC):*

- Under Blueprint Medicines' collaboration with CStone Pharmaceuticals, announced the China NMPA cleared an IND application for the ongoing Phase 1 trial of BLU-554 as a monotherapy in advanced HCC.

*BLU-782: Fibrodysplasia ossificans progressiva (FOP):*

- Initiated participant dosing in a Phase 1 trial of BLU-782 in healthy volunteers in the first quarter of 2019.
- Today announced the FDA has granted Fast Track Designation to BLU-782 for the treatment of FOP.

*Corporate:*

- Announced "2020 Blueprint," a two-year global business strategy under which Blueprint Medicines expects to have two marketed products, four pending marketing applications in the United States or Europe, six clinical-stage therapeutic candidates and eight research programs by the end of 2020.
- Announced the promotion of Michael Landsittel to Chief Financial Officer and the promotion of Kate Haviland to Chief Operating Officer in February 2019.

**Key Upcoming Milestones:**

The company expects to achieve the following milestones by the end of the second quarter of 2019.

- Submit an NDA for avapritinib for PDGFRA Exon 18 mutant GIST and fourth-line GIST.
- Present the registration dataset for avapritinib in PDGFRA Exon 18 mutant GIST and fourth-line GIST.
- Present updated data from the Phase 1 EXPLORER trial of avapritinib in advanced SM.
- Present updated data from the Phase 1 ARROW trial of BLU-667 in RET-altered cancers.
- Complete enrollment of previously treated NSCLC and MTC patient cohorts in the Phase 1 ARROW trial of BLU-667.

**Fourth Quarter and Year End 2018 Financial Results:**

- **Cash Position:** As of December 31, 2018, cash, cash equivalents and investments were \$494.0 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 \$1.0 million for the fourth quarter of 2018 and \$44.5 million for the year ended December 31, 2018, as compared to \$1.6 million for the fourth quarter of 2017 and \$21.4 million for the year ended December 31, 2017. This increase for the year was primarily due to revenue recognized under the collaboration agreement with CStone Pharmaceuticals, partially offset by the termination of the Alexion agreement in 2017.
  - **R&D Expenses:** Research and development expenses were \$70.5 million for the fourth quarter of 2018 and \$243.6 million for the year ended December 31, 2018, as compared to \$43.6 million for the fourth quarter of 2017 and \$144.7 million for the year ended December 31, 2017. This increase was primarily due to increased clinical and manufacturing expenses driven by Blueprint Medicines' lead development candidates and increased personnel-related expenses. Research and development expenses included \$4.9 million in stock-based compensation expenses for the fourth quarter of 2018 and \$17.0 million in stock-based compensation expenses for the year ended December 31, 2018.
  - **G&A Expenses:** General and administrative expenses were \$13.6 million for the fourth quarter of 2018 and \$47.9 million for the year ended December 31, 2018, as compared to \$8.1 million for the fourth quarter of 2017 and \$28.0 million for the year ended December 31, 2017. This increase was primarily due to increased personnel-related expenses and increased professional fees, including pre-commercial planning activities. General and administrative
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expenses included \$3.9 million in stock-based compensation expenses for the fourth quarter of 2018 and \$13.5 million in stock-based compensation expenses for the year ended December 31, 2018.

• **Net Loss:** Net loss was \$80.3 million for the fourth quarter of 2018 and \$236.6 million for the year ended December 31, 2018, or a net loss per share of \$1.83 and \$5.39, respectively, as compared to a net loss of \$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.

#### **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 at 8:30 a.m. ET today to discuss fourth quarter and full year 2018 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 26735762. 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 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 are currently advancing four investigational medicines in clinical development, along with multiple research programs. For more information, visit [www.BlueprintMedicines.com](http://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 plans and timelines for the development of avapritinib, BLU-667, BLU-554 and BLU-782; the potential benefits of Blueprint Medicines' current and future drug candidates in treating patients; Blueprint Medicines' "2020 Blueprint" strategy, key goals and anticipated milestones; plans and timelines for submitting an NDA to the FDA for avapritinib; plans and timelines for presenting the registration dataset for avapritinib in PDGFRA Exon 18 mutant GIST and fourth-line GIST; plans and timelines for presenting updated data from the Phase 1 EXPLORER trial of avapritinib in advanced SM; plans and timelines for presenting updated data from the Phase 1 ARROW trial of BLU-667 in RET-altered cancers; plans and timelines for completing previously treated NSCLC and MTC patient cohorts in the Phase 1 ARROW trial of BLU-667; 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-667, BLU-554 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

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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 avapritinib for PDGFR $\alpha$  D842V-driven GIST, BLU-667 for RET-driven NSCLC and BLU-554 for FGFR4-driven HCC; the success of Blueprint Medicines' current and future collaborations, including its cancer immunotherapy collaboration with F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc. and its 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 September 30, 2018, as filed with the Securities and Exchange Commission (SEC) on October 30, 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.

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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>2018</u>		<u>2017</u>	
Cash, cash equivalents and investments	\$	494,012	\$	673,356
Working capital <sup>(1)</sup>		439,464		642,615
Total assets		540,124		715,737
Deferred revenue		46,167		35,373
Term loan payable		-		1,518
Lease incentive obligation		14,617		16,331
Total stockholders' equity		419,009		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)

	<u>Three Months Ended</u>		<u>Year Ended</u>	
	<u>December 31,</u>		<u>December 31,</u>	
	<u>2018</u>	<u>2017</u>	<u>2018</u>	<u>2017</u>
Collaboration revenue	\$ 1,033	\$ 1,628	\$ 44,521	\$ 21,426
Operating expenses:				
Research and development	70,532	43,629	243,621	144,687
General and administrative	13,643	8,092	47,928	27,986
Total operating expenses	84,175	51,721	291,549	172,673
Other income (expense):				
Other income, net	2,825	1,108	10,459	3,349
Interest expense	(5)	(42)	(73)	(221)
Total other income (expense)	2,820	1,066	10,386	3,128
Net loss	\$ (80,322)	\$ (49,027)	\$ (236,642)	\$ (148,119)
Net loss per share — basic and diluted	\$ (1.83)	\$ (1.23)	\$ (5.39)	\$ (3.92)
Weighted-average number of common shares used in net loss per share — basic and diluted	43,994	39,988	43,867	37,793



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