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): **January 8, 2020**

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:

☐ 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. ☐

Securities registered pursuant to Section 12(b) of the Exchange Act:

Title of each class	Trading symbol(s)	Name of each exchange on which registered
Common stock, par value $0.001 per share	BPMC	Nasdaq Global Select Market

Item 8.01 Other Events.

On January 8, 2020, Blueprint Medicines Corporation (the "Company") issued a press release announcing top-line data from its Phase 1/2 ARROW clinical trial evaluating pralsetinib for the treatment of patients with RET fusion-positive non-small cell lung cancer ("NSCLC") and that the Company has initiated the submission of a rolling new drug application ("NDA") to the U.S. Food and Drug Administration for the treatment of patients with RET fusion-positive NSCLC. The Company expects to complete the NDA submission in the first quarter of 2020. A copy of the press release is filed herewith as Exhibit 99.1 to this Current Report on Form 8-K.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits.

Exhibit No.	Description
99.1	Press release issued by Blueprint Medicines Corporation on January 8, 2020
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

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: January 8, 2020

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

Blueprint Medicines Announces Top-line Data for Pralsetinib and Initiates Rolling NDA Submission to FDA for the Treatment of Patients with RET Fusion-Positive Non-Small Cell Lung Cancer

-- 61% ORR and median DOR not reached in patients with RET fusion-positive NSCLC previously treated with platinum-based chemotherapy --

-- 73% ORR and 12% CR rate in patients with treatment-naïve RET fusion-positive NSCLC --

-- Expect to complete NDA rolling submission for RET fusion-positive NSCLC in Q1 2020 --

-- Planned NDA submission for previously treated RET-mutant medullary thyroid cancer on track for submission to FDA in Q2 2020 --

CAMBRIDGE, Mass., January 8, 2020 – Blueprint Medicines Corporation (NASDAQ: BPMC), a precision therapy company focused on genomically defined cancers, rare diseases and cancer immunotherapy, today announced independent centrally reviewed top-line data for pralsetinib in patients with RET fusion-positive non-small cell lung cancer (NSCLC). The data from the ongoing Phase 1/2 ARROW clinical trial of pralsetinib showed a 61 percent objective response rate (ORR) and prolonged durability, with a median duration of response (DOR) not reached, in patients with RET fusion-positive NSCLC previously treated with platinum-based chemotherapy. Designed by Blueprint Medicines, pralsetinib is a potent and highly selective once-daily oral inhibitor of RET fusions and mutations, including predicted resistance mutations.

In addition, Blueprint Medicines announced it has initiated the submission of a rolling New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for pralsetinib for the treatment of patients with RET fusion-positive NSCLC. The company expects to complete the NDA submission in the first quarter of 2020. Blueprint Medicines also plans to submit an NDA to the FDA for pralsetinib for the treatment of patients with medullary thyroid cancer (MTC) previously treated with an approved multi-kinase inhibitor in the second quarter of 2020.

“As the clinical data for pralsetinib have matured, with deep and durable responses along with robust evidence of activity against brain metastases, our confidence has continued to grow in the potential of pralsetinib to provide lasting benefit to a broad population of patients with RET fusion-positive NSCLC, including those with newly diagnosed unresectable or metastatic disease,” said Andy Boral, M.D., Ph.D., Chief Medical Officer of Blueprint Medicines. “Now, with strong, centrally reviewed top-line data, we feel a profound sense of urgency and have taken the first step toward making pralsetinib broadly available to patients by initiating a rolling NDA submission to the FDA.”

Top-line Data from Phase 1/2 ARROW Trial in RET Fusion-Positive NSCLC

Results from the Phase 1/2 ARROW clinical trial of pralsetinib will be used to support the NDA submission for pralsetinib for the treatment of patients with RET fusion-positive NSCLC. The registration endpoints are ORR and DOR based on independent central radiology and Response Evaluation Criteria in Solid Tumors version 1.1 (RECIST 1.1) criteria.

Top-line efficacy data were reported for patients treated with pralsetinib who were evaluable for response assessment per RECIST 1.1, as determined by blinded independent central review. All patients received the proposed indicated dose of 400 mg once daily (QD).

In 80 patients with RET fusion-positive NSCLC previously treated with platinum-based chemotherapy, the ORR was 61 percent (95% CI: 50-72%) per independent central review (two responses pending confirmation) as of a data cutoff date of November 18, 2019. Overall, 95 percent of patients had tumor shrinkage, including 14 percent of patients with complete regression of target tumors. The median DOR was not reached (95% CI: 11.3 months, not estimable).

In 26 patients with treatment-naïve RET fusion-positive NSCLC, the ORR was 73 percent (95% CI: 52-88%) per independent central review (all responses confirmed), with 12 percent of patients achieving a complete response. All patients had tumor shrinkage.

Top-line safety data were consistent with those previously reported. Pralsetinib was well-tolerated, and most adverse events (AEs) were Grade 1 or 2. Across all patients enrolled in the ARROW trial treated with the proposed indicated dose of 400 mg QD (N=354), only four percent of patients discontinued treatment with pralsetinib due to treatment-related AEs.

Blueprint Medicines plans to present the full registration dataset at a scientific meeting later this year.

Planned Phase 3 AcceleRET Lung Trial in Treatment-Naïve RET Fusion NSCLC

In addition, Blueprint Medicines plans to initiate the first clinical trial site for its Phase 3 AcceleRET Lung clinical trial in January 2020. The primary objective of the AcceleRET trial is to evaluate the potential of pralsetinib to extend progression free survival (PFS) compared to platinum-based chemotherapy with or without pembrolizumab in patients with first-line RET fusion-positive NSCLC.

The global, randomized AcceleRET trial will enroll approximately 250 patients with advanced or metastatic RET fusion-positive NSCLC who have received no prior systemic therapy for metastatic disease. Participants will be randomized to receive either pralsetinib or the investigator’s choice of platinum-based chemotherapy regimen with or without pembrolizumab. The trial’s primary endpoint is PFS and secondary endpoints include overall survival, ORR and DOR. Patients may receive local testing to identify a RET fusion. In addition, patients randomized to the control arm may crossover upon progression to receive pralsetinib. Multiple trial sites are planned in North America, Europe and Asia.

About RET-Altered Solid Tumors

RET activating fusions and mutations are key disease drivers in many cancer types, including NSCLC and MTC. RET fusions are implicated in approximately 1 to 2 percent of patients with NSCLC and approximately 10 to 20 percent of patients with papillary thyroid cancer (PTC), while RET mutations are implicated in approximately 90 percent of patients with advanced MTC. In addition, oncogenic RET alterations are observed at low frequencies in colorectal, breast, pancreatic and other cancers, and RET fusions have been observed in patients with treatment-resistant, EGFR-mutant NSCLC.

Currently, there are no approved therapies that selectively target RET-driven cancers, although there are several approved multi-kinase inhibitors (MKIs) with RET activity being evaluated in clinical trials. To date, clinical activity attributable to RET inhibition has been uncertain for these approved MKIs, likely due to insufficient inhibition of RET and off-target toxicities. There is a need for precision therapies that provide durable clinical benefit by selectively targeting RET alterations and anticipated resistance mutations.

About Pralsetinib

Pralsetinib is an investigational, once-daily oral precision therapy specifically designed for highly potent and selective targeting of oncogenic RET alterations. Blueprint Medicines is developing pralsetinib for the treatment of patients with RET-altered NSCLC, MTC and other solid tumors. The FDA has granted Breakthrough Therapy Designation to pralsetinib for the treatment of RET-fusion positive NSCLC that has progressed following platinum-based chemotherapy, and RET-mutant MTC that requires systemic treatment and for which there are no acceptable alternative treatments.

Pralsetinib was designed by Blueprint Medicines' research team, leveraging the company's proprietary compound library. In preclinical studies, pralsetinib consistently demonstrated sub-nanomolar potency against the most common RET fusions, activating mutations and predicted resistance mutations. In addition, pralsetinib

demonstrated markedly improved selectivity for RET compared to pharmacologically relevant kinases, including approximately 90-fold improved potency for RET versus VEGFR2. By suppressing primary and secondary mutants, pralsetinib has the potential to overcome and prevent the emergence of clinical resistance. Blueprint Medicines believes this approach will enable durable clinical responses across a diverse range of RET alterations, with a favorable safety profile.

Blueprint Medicines has an exclusive collaboration and license agreement with CStone Pharmaceuticals for the development and commercialization of pralsetinib, avapritinib and fisogatinib in Mainland China, Hong Kong, Macau and Taiwan. Blueprint Medicines retains development and commercial rights for all three drug candidates in the rest of the world.

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 three investigational medicines in clinical development, along with multiple research programs. For more information, visit 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 its drug candidates, including the timing, design, implementation, enrollment, plans and announcement of results regarding Blueprint Medicines' ongoing and planned clinical trials for pralsetinib; plans, timelines and expectations for full data from the ARROW clinical trial; expectations regarding the potential benefits of pralsetinib in treating patients with RET-altered NSCLC, MTC and other solid tumors; plans and timelines for submitting and completing ongoing and planned marketing applications for pralsetinib; plans, timelines and expectations for interactions with the FDA and other regulatory authorities; and Blueprint Medicines' strategy, goals and anticipated milestones, 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 or licensed product candidate; Blueprint Medicines' advancement of multiple early-stage efforts; Blueprint Medicines' ability to successfully demonstrate the safety and efficacy of its drug candidates and gain approval of its drug candidates on a timely basis, if at all; 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; and the success of Blueprint Medicines' current and future collaborations or licensing arrangements, including its cancer immunotherapy collaboration with F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc., its collaboration with CStone Pharmaceuticals and its license to Clementia Pharmaceuticals. These and other risks and uncertainties are described in greater detail in the section entitled "Risk Factors" in Blueprint Medicines' filings with the Securities and Exchange Commission (SEC), including Blueprint Medicines' most recent Quarterly Report on Form 10-Q 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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