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): **September 20, 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 September 20, 2020, Blueprint Medicines Corporation issued a press release reporting updated data from its ongoing Phase 1/2 ARROW clinical trial of pralsetinib in patients with advanced RET-mutant medullary thyroid cancer. These data are also being presented at the European Society for Medical Oncology Virtual Congress 2020. A copy of the press release is filed herewith as Exhibit 99.1 to this Current Report on Form 8-K and incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits.

Exhibit No.	Description
99.1	Press release issued by Blueprint Medicines Corporation on September 20, 2020
104	Cover Page Interactive Data File (embedded within the Inline XBRL document and incorporated as Exhibit 101)

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: September 21, 2020

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

Blueprint Medicines Reports ARROW Trial Data at ESMO Virtual Congress 2020 Demonstrating Durable Clinical Benefits of GAVRETO™ (Pralsetinib) in Patients with Advanced RET-Mutant Medullary Thyroid Cancer

-- 60% ORR in patients previously treated with vandetanib or cabozantinib, and 74% ORR in treatment-naïve patients --

-- Median DOR and median PFS not reached across lines of therapy --

-- New drug application under review for RET-mutant medullary thyroid cancer and RET fusion-positive thyroid cancer --

-- NCCN guidelines now include GAVRETO as a preferred treatment option for RET fusion-positive NSCLC --

CAMBRIDGE, Mass., September 20, 2020 – Blueprint Medicines Corporation (NASDAQ: BPMC), a precision therapy company focused on genomically defined cancers, rare diseases and cancer immunotherapy, today announced results from the ongoing ARROW clinical trial showing durable responses and a well-tolerated safety profile for GAVRETO™ (pralsetinib) in patients with advanced RET-mutant medullary thyroid cancer (MTC). In these registrational data, GAVRETO demonstrated consistent clinical activity in patients across lines of therapy and regardless of RET mutation genotypes, including a high response rate in patients with gatekeeper mutations resistant to multi-kinase inhibitors. The results are being presented today in a proffered paper session during the European Society for Medical Oncology (ESMO) Virtual Congress 2020.

“For patients with RET-mutant medullary thyroid cancer, there is an important need for targeted therapies like pralsetinib (GAVRETO) that are highly active across RET genotypes, including gatekeeper resistance mutations,” said Mimi Hu, M.D., professor in the Department of Endocrine Neoplasia and Hormonal Disorders at The University of Texas MD Anderson Cancer Center. “The reported data highlight the robust clinical activity and safety of GAVRETO, with most patients remaining on treatment for prolonged periods of time. These results are a promising advancement for RET-mutant medullary thyroid cancer across both systemic treatment-naïve and previously treated patients.”

“By selectively inhibiting RET alterations, GAVRETO has broad potential to address the limitations of multi-kinase inhibitors and enable transformative outcomes for patients with RET-mutant medullary thyroid cancer,” said Andy Boral, M.D., Ph.D., Chief Medical Officer at Blueprint Medicines. “Across first-line and previously treated settings, GAVRETO has shown durable clinical benefits and a well-tolerated safety profile that has remained consistent over time. With the recent FDA approval of GAVRETO in patients with RET fusion-positive metastatic non-small cell lung cancer, these data further support our efforts to bring this once-daily treatment to patients across multiple RET-driven tumor types.”

As previously announced, the U.S. Food and Drug Administration (FDA) has accepted a new drug application (NDA) for GAVRETO for the treatment of patients with advanced or metastatic RET-mutant MTC and RET fusion-positive thyroid cancer. This NDA was accepted for priority review under the FDA’s Real-Time Oncology Review (RTOR) pilot program, which aims to explore a more efficient review process to ensure safe and effective treatments are available to patients as early as possible.

In addition, Blueprint Medicines today announced that the National Comprehensive Cancer Network has updated its Clinical Practice Guidelines in Oncology (NCCN Guidelines®) for Non-Small Cell Lung Cancer (NSCLC) to include GAVRETO as a preferred treatment option (category 2A) for patients with RET fusion-positive NSCLC as a first-line or subsequent therapy. This rating indicates that there is uniform NCCN consensus that the intervention is appropriate. The NCCN Guidelines are the recognized clinical standard for cancer care by U.S. healthcare providers and payers, and are maintained by a committee of expert physicians from leading U.S. cancer centers.

GAVRETO is being jointly commercialized by Genentech, a wholly owned member of the Roche Group, and Blueprint Medicines in the U.S. and will be commercialized by Roche outside of the U.S., excluding Greater China (Mainland China, Hong Kong, Macau and Taiwan).

Highlights from the ARROW Trial in Patients with RET-Mutant MTC

The presented data included response-evaluable patients with RET-mutant MTC who were previously treated with cabozantinib or vandetanib, or naïve to systemic treatment. Tumor response was assessed by blinded, independent central review using Response Evaluation Criteria in Solid Tumors (RECIST) version 1.1. All patients received a GAVRETO starting dose of 400 mg once daily (QD), and results were reported as of a data cutoff date of February 13, 2020.

GAVRETO demonstrated broad clinical activity in patients with RET-mutant MTC with or without prior systemic therapy. In 53 patients previously treated with cabozantinib or vandetanib, the overall response rate (ORR) was 60 percent (95% CI: 46%, 74%) with one response pending confirmation, and the disease control rate (DCR) was 96 percent (95% CI: 87%, 100%). The median duration of response (DOR) was not reached (95% CI: not reached, not reached), with 94 percent of responders remaining on treatment. The median progression-free survival (PFS) was not reached (95% CI: not reached, not reached) in patients previously treated with cabozantinib or vandetanib.

In 19 systemic treatment-naïve patients who were ineligible for standard therapy per the study protocol, the confirmed ORR was 74 percent (95% CI: 49%, 91%), and the DCR was 100 percent (95% CI: 82%, 100%). The median DOR was not reached (95% CI: 7 months, not reached), with 93 percent of responders remaining on treatment. The median PFS was not reached (95% CI: not reached, not reached) in systemic treatment-naïve patients.

Five of six patients whose tumors had a RET V804M or V804L gatekeeper mutation achieved a clinical response. Three patients previously treated with multi-kinase inhibitors had a RET M918T activating mutation and a RET V804M or V804L gatekeeper resistance mutation at baseline, and all three of these patients had a clinical response following GAVRETO treatment.

The reported safety data included a total of 438 patients enrolled in the ARROW trial at a GAVRETO starting dose of 400 mg QD, regardless of tumor type. GAVRETO was well-tolerated with safety results consistent with previously reported data. Overall, treatment-related adverse events (AEs) were primarily Grade 1 or 2. The most common treatment-related AEs reported by investigators (≥15 percent) were increased aspartate aminotransferase, anemia, increased alanine aminotransferase, hypertension, constipation, decreased white blood cell count, neutropenia, decreased neutrophil count and hyperphosphatemia. Investigator-reported Grade 3 or higher treatment-related AEs (≥5 percent) were hypertension, neutropenia, anemia and decreased neutrophil count. Four percent of patients discontinued GAVRETO due to treatment-related AEs.

These data for GAVRETO are being reported in a proffered paper (Abstract Number: 1913O) at the ESMO Virtual Congress 2020. A copy of the data presentation is available in the “Science—Publications and Presentations” section of Blueprint Medicines’ website at www.BlueprintMedicines.com.

About RET-Altered Solid Tumors

RET activating fusions and mutations are key disease drivers in many cancer types, including NSCLC and multiple types of thyroid cancer. 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, while RET mutations are implicated in approximately 90 percent of patients with advanced MTC. In addition, oncogenic RET fusions are observed at low frequencies in colorectal, breast, pancreatic and other cancers, as well as in patients with treatment-resistant EGFR-mutant NSCLC.

About the ARROW Trial

The Phase 1/2 ARROW trial (ClinicalTrials.gov Identifier: NCT03037385) is designed to evaluate the safety, tolerability and efficacy of GAVRETO in adults with RET-altered cancers. The trial consists of two parts: a dose escalation portion, which is complete, and an expansion portion in patients treated at 400 mg QD. The study's objectives include assessing response, pharmacokinetics, pharmacodynamics and safety. The trial is enrolling patients at multiple sites in the United States, European Union and Asia.

Patients and physicians interested in the ARROW trial can contact the Blueprint Medicines study director at medinfo@blueprintmedicines.com or 1-888-BLU-PRNT (1-888-258-7768) in the U.S., or medinfoeurope@blueprintmedicines.com or +31 85 064 4001 in Europe. Additional information is available at www.BlueprintClinicalTrials.com/ARROW and www.clinicaltrials.gov.

About GAVRETO (pralsetinib)

GAVRETO (pralsetinib) is a once-daily oral targeted therapy approved by the FDA for the treatment of adults with metastatic RET fusion-positive NSCLC as detected by an FDA approved test. It is designed to selectively and potently target oncogenic RET alterations. In pre-clinical studies, GAVRETO inhibited RET at lower concentrations than other pharmacologically relevant kinases, including VEGFR2, FGFR2 and JAK2. For more information, visit GAVRETO.com.

GAVRETO is not approved for the treatment of any other indication in the U.S. by the FDA or for any indication in any other jurisdiction by any other health authority.

Blueprint Medicines and Roche are co-developing GAVRETO globally (excluding Greater China) for the treatment of patients with RET-altered NSCLC, various types of thyroid cancer and other solid tumors. The FDA has accepted an NDA for GAVRETO for the treatment of RET-mutant MTC and RET fusion-positive thyroid cancer, and the European Medicines Agency has validated a marketing authorization application for GAVRETO for the treatment of RET fusion-positive NSCLC. The FDA has granted breakthrough therapy designation to GAVRETO for the treatment of RET fusion-positive NSCLC that has progressed following platinum-based chemotherapy and for RET mutation-positive MTC that requires systemic treatment and for which there are no acceptable alternative treatments.

Blueprint Medicines has an exclusive collaboration and license agreement with CStone Pharmaceuticals for the development and commercialization of GAVRETO in Greater China.

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 have two FDA-approved precision therapies and are currently advancing multiple investigational medicines in clinical development, along with a number of 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 and, if approved, commercialization of GAVRETO for the treatment of patients with RET-mutant MTC, RET fusion-positive thyroid cancer and other RET-altered cancers; the potential benefits of the FDA's RTOR

program; the potential benefits of Blueprint Medicines' current and future approved drugs or drug candidates in treating patients; and Blueprint Medicines' strategy, goals and anticipated milestones, business plans and focus. The words "aim," "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 impact of the COVID-19 pandemic to Blueprint Medicines' business, operations, strategy, goals and anticipated milestones, including Blueprint Medicines' ongoing and planned research and discovery activities, ability to conduct ongoing and planned clinical trials, clinical supply of current or future drug candidates, commercial supply of current or future approved products, and launching, marketing and selling current or future approved products; Blueprint Medicines' ability and plans in establishing a commercial infrastructure, and successfully launching, marketing and selling current or future approved products, including AYVAKIT™ (avapritinib) and GAVRETO; Blueprint Medicines' ability to successfully expand the approved indications for AYVAKIT and GAVRETO or obtain marketing approval for AYVAKIT and GAVRETO in additional geographies in the future; the delay of any current or planned clinical trials or the development of Blueprint Medicines' current or future drug candidates; 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, partnerships or licensing arrangements, including Blueprint Medicines' global collaboration with Roche for the development and commercialization of GAVRETO. 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 Annual Report on Form 10-K, as supplemented by its 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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