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): **May 29, 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 7.01 Regulation FD Disclosure.

On May 29, 2020, Blueprint Medicines Corporation (the "Company") is hosting an investor conference call and webcast to report data from its ongoing ARROW trial of pralsetinib in patients with RET fusion-positive non-small cell lung cancer ("NSCLC"), thyroid cancer and other solid tumors. A copy of the presentation from the investor conference call and webcast is furnished as Exhibit 99.1 to this Current Report on Form 8-K.

The information in Item 7.01 of 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 8.01 Other Events.

On May 29, 2020, the Company issued a press release announcing data from its ongoing ARROW trial of pralsetinib in patients with RET fusion-positive NSCLC, thyroid cancer and other solid tumors. These data are also being presented at the American Society of Clinical Oncology 2020 Virtual Scientific Program. A copy of the press release is filed herewith as Exhibit 99.2 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	Corporate slide presentation of Blueprint Medicines Corporation dated May 29, 2020
99.2	Press release issued by Blueprint Medicines Corporation on May 29, 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: May 29, 2020

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

Pralsetinib data review and registration program update

ASCO 2020 VIRTUAL MEETING

MAY 29, 2020

blueprint MEDICINES

Blueprint Medicines call participants

PREPARED REMARKS

Introduction	**Jeff Albers**, Chief Executive Officer
Pralsetinib clinical data review	**Andy Boral**, MD, PhD, Chief Medical Officer
Pralsetinib commercial strategy	**Christy Rossi**, Chief Commercial Officer
Q&A	**All**

Forward-looking statements

This presentation contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended. 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. In this presentation, forward-looking statements include, without limitation, statements regarding plans and timelines for the development of avapritinib and pralsetinib, including the timing, designs, implementation, enrollment, plans and announcement of results regarding the ongoing and planned clinical trials of Blueprint Medicines Corporation (the "Company"); plans and timelines for submitting marketing applications for avapritinib and pralsetinib and, if approved, for commercializing pralsetinib; the potential benefits of the Company's current and future approved drugs or drug candidates in treating patients; and the Company's strategy, goals and anticipated milestones, business plans and focus.

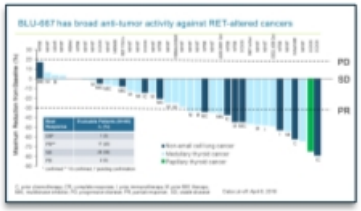
The Company has based these forward-looking statements on management's current expectations, assumptions, estimates and projections. While the Company believes these expectations, assumptions, estimates and projections are reasonable, such forward-looking statements are only predictions and involve known and unknown risks, uncertainties and other important factors, many of which are beyond the Company's control and may cause actual results, performance or achievements to differ materially from those expressed or implied by any forward-looking statements. These risks and uncertainties include, without limitation, risks and uncertainties related to the impact of the COVID-19 pandemic to the Company's business, operations, strategy, goals and anticipated milestones, including the Company's 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 drugs, and launching, marketing and selling current or future approved drugs; the delay of any current or planned clinical trials or the development of the Company's drug candidates or the licensed drug candidate; the Company's advancement of multiple early-stage efforts; the Company's ability to successfully demonstrate the efficacy and safety of its drug candidates and gain approval of its drug candidates on a timely basis, if at all; the preclinical and clinical results for the Company's drug candidates, which may not support further development of such drug candidates; actions or decisions of regulatory agencies or authorities, which may affect the initiation, timing and progress of clinical trials or marketing applications; the Company's ability to obtain, maintain and enforce patent and other intellectual property protection for any drug candidates it is developing or AYVAKIT; the Company' ability and plans for maintaining a commercial infrastructure, and successfully launching, marketing and selling its current or future approved drugs; the Company's ability to develop and commercialize companion diagnostic tests for any of the Company's current or future approved drugs or drug candidates; and the success of the Company's current and future collaborations, partnerships and licenses.

These and other risks and uncertainties are described in greater detail under "Risk Factors" in the Company's filings with the Securities and Exchange Commission ("SEC"), including its most recent Annual Report on Form 10-K, as supplemented by its most recent Quarterly Report on Form 10-Q, and any other filings it has made or may make with the SEC in the future. The Company cannot guarantee future results, outcomes, levels of activity, performance, developments, or achievements, and there can be no assurance that its expectations, intentions, anticipations, beliefs, or projections will result or be achieved or accomplished. The forward-looking statements in this presentation are made only as of the date hereof, and except as required by law, the Company undertakes no obligation to update any forward-looking statements contained in this presentation as a result of new information, future events or otherwise.

This presentation also contains estimates, projections and other statistical data made by independent parties and by the Company relating to market size and growth and other data about the Company's industry. These data involve a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions and estimates of the Company's future performance and the future performance of the markets in which the Company operates are necessarily subject to a high degree of uncertainty and risk.

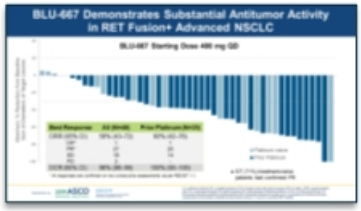
Pralsetinib clinical data have strengthened over time

AACR 2018[1]



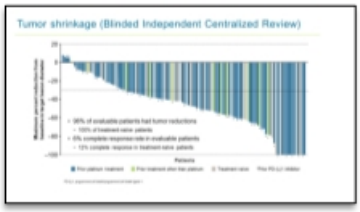
Clinical POC enabling selection of recommended Phase 2 dose

ASCO 2019[2]



Expansion data supporting FDA breakthrough therapy designations for NSCLC and MTC[4]

ASCO 2020[3]



Registration data supporting global marketing applications

Ongoing global development to confirm durability of outcomes and deliver benefit to new populations

~3 years from IND approval to first NDA submission

1. Presented at AACR 2018 Annual Meeting. Data cut off: April 6, 2018. 2. Presented at ASCO 2019 Annual Meeting. Data cut off: April 28, 2019. 3. Presented at ASCO 2020 Virtual Annual Meeting. Data cut off: November 18, 2019. 4. FDA has granted breakthrough therapy designations 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. AACR, American Association for Cancer Research; ASCO, American Society of Clinical Oncology. FDA, U.S. Food and Drug Administration; IND, investigational new drug application; MTC, medullary thyroid cancer; NDA, new drug application; NSCLC, non-small cell lung cancer; POC, proof-of-concept.

Broad pralsetinib registration program continues to rapidly advance

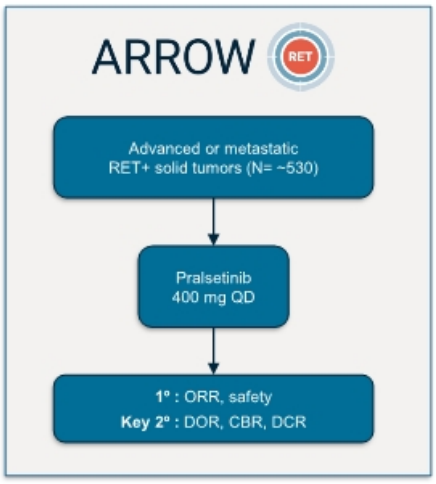
FDA PROGRAMS DESIGNED TO EXPEDITE AND PROMOTE EFFICIENT REVIEW PROCESS

Indication	Breakthrough Therapy[1]	Real-Time Oncology Review	Project Orbis	Priority Review	Regulatory status
RET+ NSCLC	✓		✓	✓	• NDA accepted by FDA with November 23, 2020 PDUFA action date • MAA validated by EMA
RET+ thyroid cancer	✓	✓			• Plan to submit NDA to FDA in June 2020

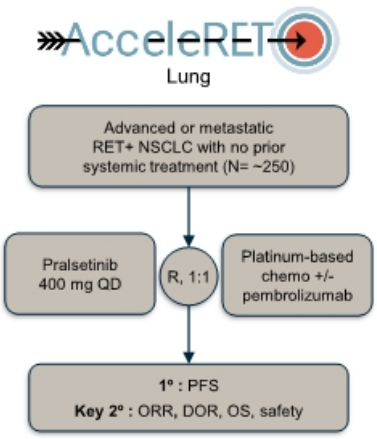
Anticipate U.S. commercial launch in 2H 2020, pending approval

1. FDA has granted breakthrough therapy designations 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. EMA, European Medicines Agency; MAA, marketing authorization application; PDUFA, Prescription Drug User Fee Act.

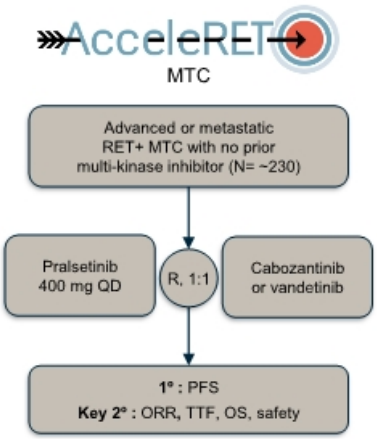
Broad pralsetinib clinical development program in RET+ cancers



Active, enrolling



Active, enrolling



Plan to initiate in 2H 2020

CBR, clinical benefit rate; DCR, disease control rate; DOR, duration of response; ORR, overall response rate; OS, overall survival; PFS, progression free survival; QD, once daily; R, randomized; TTF, time-to-treatment failure; 1°, primary endpoint; 2°, secondary endpoint.

Pralsetinib clinical data review

Andy Boral, MD, PhD, Chief Medical Officer

ARROW trial baseline characteristics: RET fusion+ NSCLC

	Prior platinum (n=92)	Treatment naïve (n=29)
Median age (range), years	60 (28–85)	65 (30–87)
ECOG PS, %		
0	37	38
1	58	59
2†	5	3
Brain metastases‡, %	41	41
RET **fusion partner, %**		
KIF5B	74	69
CCDC6	17	10
Other§	2	0
Unknown*	7	21
Prior therapy type, %		
Chemotherapy	100	0
PD-(L)1 inhibitor	45	0
Chemotherapy + PD-(L)1 inhibitor	45	0

- Prior platinum group consistent with real-world population
- Initial eligibility criteria required treatment-naïve group to be ineligible for standard chemotherapy:
  - 52% >65 years
  - 41% with brain metastases
  - ECOG PS similar to prior platinum group
  - 21% unknown fusion partner

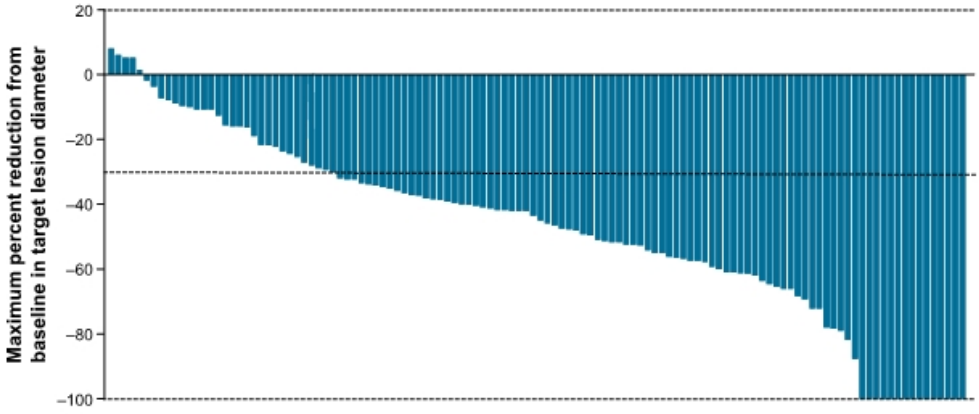
NATURAL HISTORY – RET+ NSCLC REGISTRY

- Median age: 61 years[1]
- 25% incidence of brain metastases in newly diagnosed Stage IV[2]
- 46% life-time incidence of brain metastases in Stage IV[2]
- Fusion partner frequency: 72% KIF5B; 23% CCDC6[1]

Data presented at ASCO 2020 virtual annual meeting. Data cut off: November 18, 2019. †ECOG PS of 2 was permitted prior to a protocol amendment. ‡History of or current. §EML4 or DOCK1. * Fusion present but specific partner unknown. 1. Gautschi, et al. Journal of Clinical Oncology, 2017. 2. Drilon, et al. Journal of Thoracic Oncology, 2018. ECOG PS, Eastern Cooperative Oncology Group (ECOG) Performance Status (PS); PD-(L)1, programmed cell death/programmed cell death ligand-1.

Robust clinical activity in NSCLC patients regardless of prior therapy

TUMOR SHRINKAGE PER CENTRAL RADIOLOGY



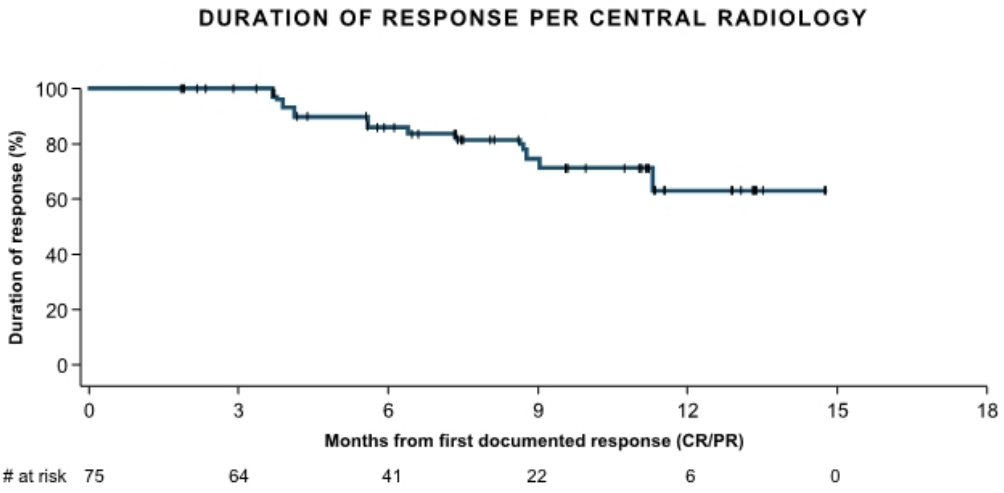
OVERALL RESPONSE RATE

61% (95% CI: 50-72%)	Prior platinum chemo (n=80)[1]
73% (95% CI: 52-88%)	No prior systemic therapy (n=26)

Tumor shrinkage in 96% of patients regardless of prior therapy

Data presented at ASCO 2020 virtual annual meeting. Data cut off: November 18, 2019. 1. Two responses pending confirmation at the time of data cut off were subsequently confirmed. CI, confidence interval.

Prolonged duration of response in NSCLC patients regardless of prior therapy



NR (95% CI: 11.3 m - NR)	Median DOR
86%	6-month DOR
74%	Responders on treatment

Data presented at ASCO 2020 virtual annual meeting. Data cut off: November 18, 2019. CR, complete response; PR, partial response. NR, not reached.

Pralsetinib is well-tolerated in patients with advanced cancer

- Only 4% discontinued due to treatment-related AEs
- Low ≥Grade 3 hypertension
- Low ≥Grade 3 AST/ALT elevations
- No clinically or statistically significant QT prolongation observed in QT sub-study
- No treatment-related hypersensitivity

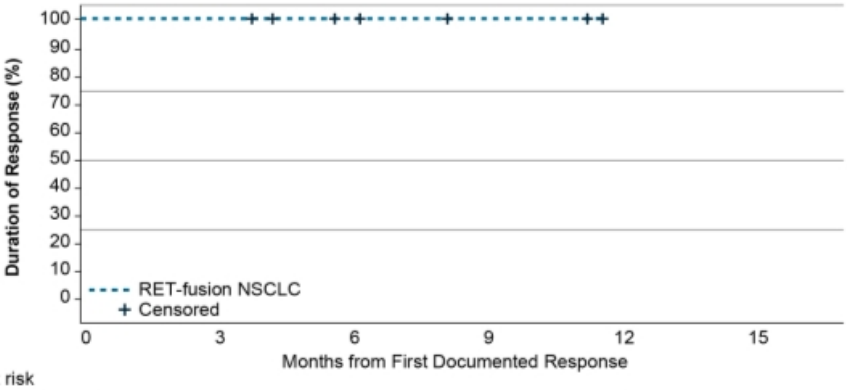
Treatment-related adverse events in ≥15% of patients	All patients (N=354)*	
AE preferred term	**Any grade (%)**	**Grade ≥3 (%)**
AST increased	31	2
Anemia	22	8
ALT increased	21	1
Constipation	21	1
Hypertension	20	10
Neutropenia	19	10

Natural history data highlight importance of drug safety profile in advanced cancer patients:
67% of NSCLC patients have ≥1 CV comorbidity, with ~10% experiencing a CV event[1]

Data presented at ASCO 2020 virtual annual meeting. Data cut off: November 18, 2019. 1. Kocher, et al. Clinical Lung Cancer, 2014. AE, adverse event; ALT, alanine aminotransferase; AST, aspartate aminotransferase; CV, cardiovascular; QT, interval between Q and T electrical waves on an electrocardiogram.

Complete responses lead to highly durable benefit in NSCLC patients

DURATION OF RESPONSE PER CENTRAL RADIOLOGY



Number at risk

RET-fusion NSCLC	8	8	5	3	0

COMPLETE RESPONSE RATE

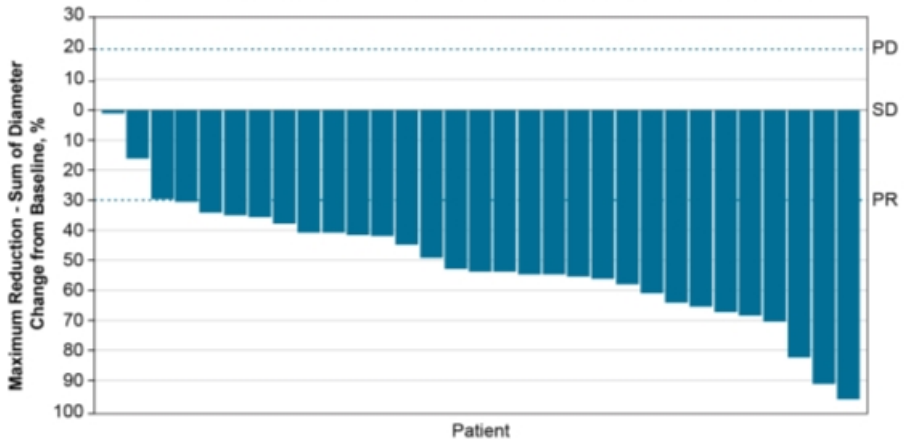
5%	Prior platinum chemo (n=80)
12%	No prior systemic therapy (n=26)

All complete responses ongoing with treatment durations up to ~15 months

Data cut off: November 18, 2019.

High responses rates in treatment-naïve NSCLC populations consistent with real-world patients

TUMOR SHRINKAGE PER INVESTIGATOR ASSESSMENTS



OVERALL RESPONSE RATE

TREATMENT-NAÏVE PATIENTS

88% **Eligible for standard therapy (n=32)**

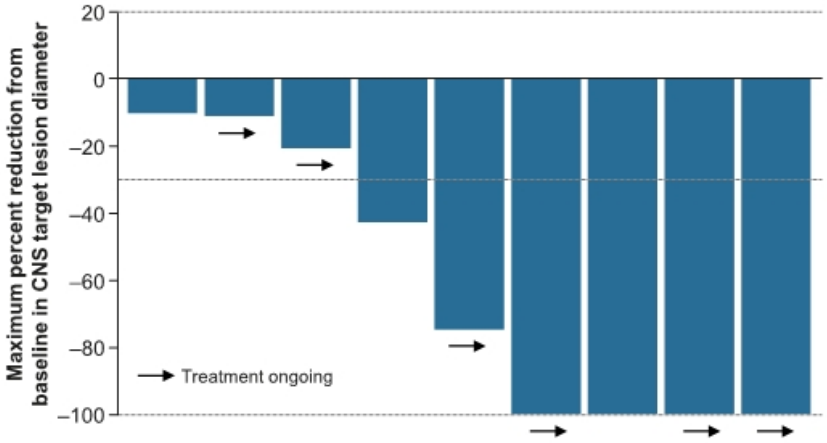
Target population for actively enrolling AcceleRET Lung Phase 3 trial in treatment-naïve NSCLC

Patients evaluable for response and enrolled after July 11, 2019. Data cut off: April 24, 2020. PD, progressive disease; SD, stable disease.

High CNS CR rate and prolonged CNS duration of response in NSCLC patients

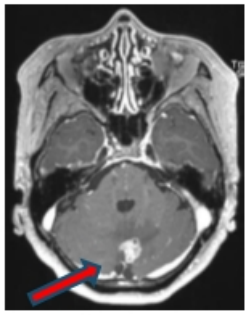
- 33% intracranial CR rate
- 100% with shrinkage of CNS metastases
- Median CNS DOR not reached
- No CNS progressive events among CNS responders

SHRINKAGE OF CNS METASTASES IN PATIENTS WITH MEASURABLE CNS LESIONS AT BASELINE (N=9)

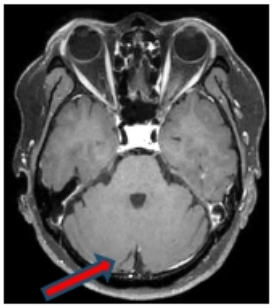


Data presented at ASCO 2020 virtual annual meeting. Data cut off: November 18, 2020. CNS, central nervous system.

Patient case: durable complete CNS response



Baseline



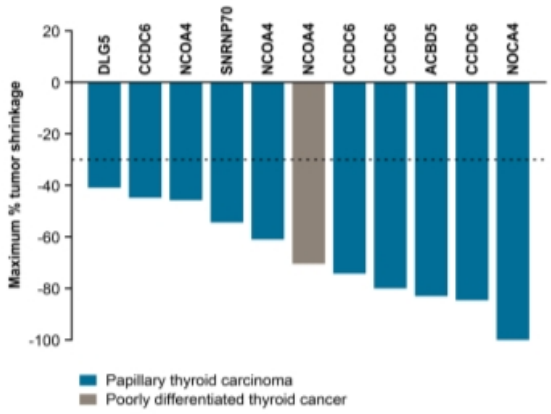
After 16 months

- 56 year-old female never smoker with RET-KIF5B fusion-positive NSCLC
- Previously received adjuvant therapy with carboplatin/paclitaxel
- Metastatic disease in brain, pleura, lymph nodes at study entry
- 20 mm brain target lesion with rapid shrinkage and complete resolution by 7.5 months on pralsetinib
- As of May 1, 2020, continues pralsetinib for 16+ months with ongoing overall partial response

Data presented at ASCO 2020 virtual annual meeting. Case courtesy of D.W. Kim, Seoul National University Hospital, Seoul, South Korea.

Deep and durable responses in patients with RET fusion+ thyroid cancer

TUMOR SHRINKAGE PER CENTRAL RADIOLOGY



RET FUSION+ THYROID CANCER ALL DOSES (N=11)

91% (95% CI: 59-100%)	**ORR**
100%	**6-month DOR**

10/11 PATIENTS PREVIOUSLY TREATED WITH SYSTEMIC THERAPY

Additional pralsetinib data in patients with other RET fusion+ solid tumors to be presented during virtual ASCO Special Clinical Science Symposium on Sunday, May 31

Data presented at ASCO 2020 virtual annual meeting. Data cut off: February 13, 2020.

Pralsetinib commercial strategy

Christy Rossi, Chief Commercial Officer

blueprint™ MEDICINES

Our plan to deliver a best-in-class selective RET inhibitor to patients

DIFFERENTIATED CLINICAL PROFILE

Data showing deep responses, long-lasting benefit, tolerability and convenience

PATIENT- AND HEALTHCARE PROVIDER-CENTERED APPROACH

Tailored support enabling patient identification, ease of prescribing and ongoing patient management

HIGHLY EXPERIENCED, NIMBLE TEAM

Fully-integrated launch-ready team in place, 2/3 with prior lung cancer experience


blueprint MEDICINES
Questions & answers

Key anticipated corporate milestones through 2H 2020

JUNE 2020

- Submit pralsetinib NDA to FDA for RET+ thyroid cancer
- Present updated Part 1 data from PIONEER for avapritinib in ISM at EAACI Congress

Q3 2020

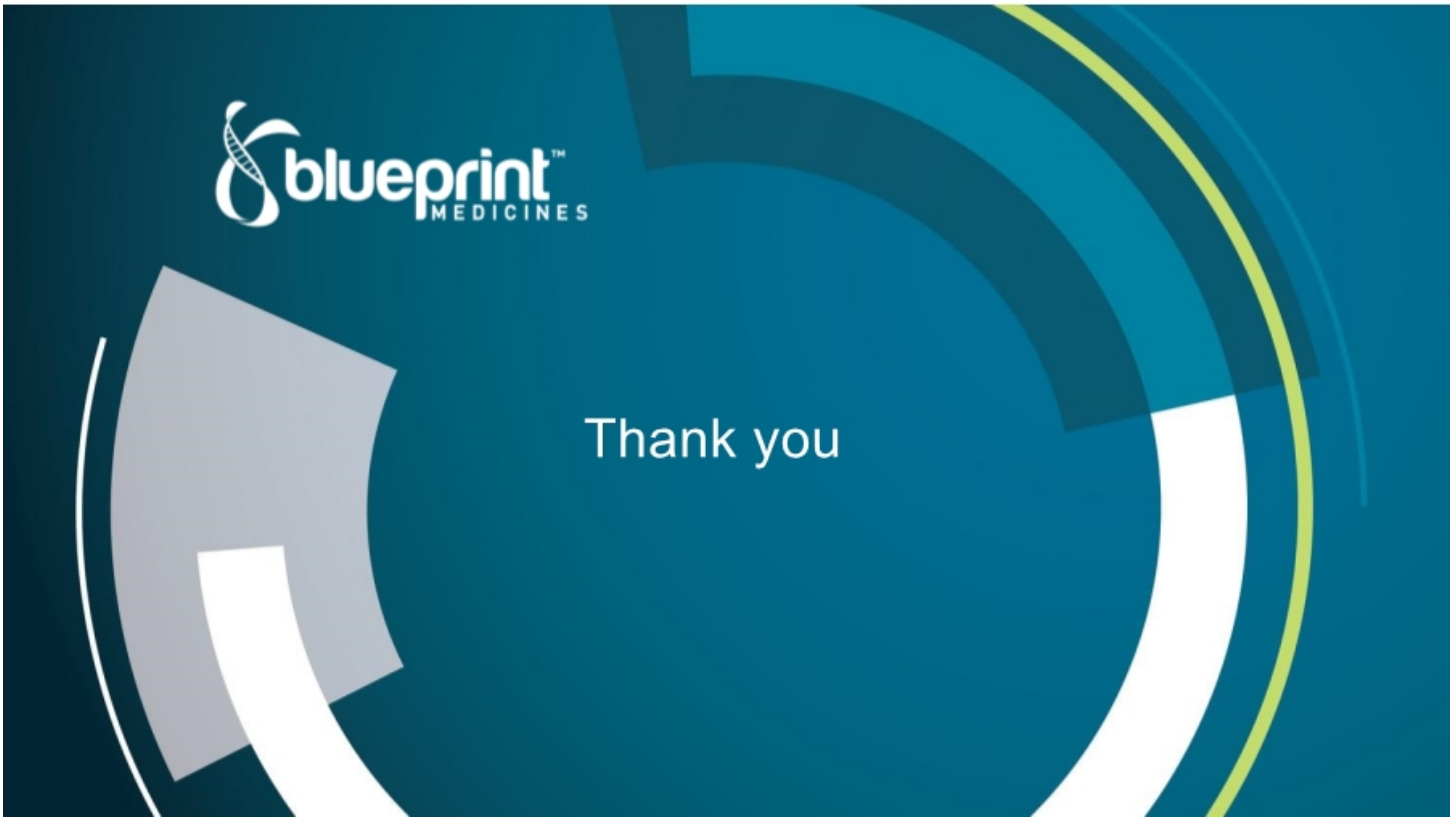
- Obtain CHMP opinion from EMA for avapritinib in PDGFRA GIST
- Report top-line EXPLORER and PATHFINDER data for avapritinib in advanced SM

Q4 2020

- Pralsetinib PDUFA action date for RET+ NSCLC NDA
- Submit avapritinib NDA to FDA for advanced SM

Additional medical conference presentations planned throughout 2020

CHMP; EMA Committee for Medicinal Products for Human Use; EAACI, European Academy of Allergy and Clinical Immunology; GIST, gastrointestinal stromal tumor; ISM, indolent systemic mastocytosis; SM, systemic mastocytosis.


blueprint
MEDICINES
Thank you

Blueprint Medicines Announces Data Presentations at ASCO20 Highlighting Deep, Durable Clinical Activity and Well-Tolerated Safety Profile of Pralsetinib Across Broad Range of RET Fusion-Positive Tumors

-- Registrational data in RET fusion-positive NSCLC show 61% ORR in patients previously treated with platinum-based chemotherapy and 73% ORR in treatment-naïve patients --

-- 12% complete response rate in patients with treatment-naïve RET fusion-positive NSCLC --

-- Median DOR not reached across lines of therapy in RET fusion-positive NSCLC, with 6-month DOR of 86% --

-- NDA accepted by FDA and MAA validated by EMA for pralsetinib in RET fusion-positive NSCLC --

-- Blueprint Medicines to host investor conference call and webcast today at 8:30 a.m. ET --

CAMBRIDGE, Mass., May 29, 2020 – Blueprint Medicines Corporation (NASDAQ: BPMC), a precision therapy company focused on genomically defined cancers, rare diseases and cancer immunotherapy, today announced data from the ongoing ARROW clinical trial of pralsetinib in patients with RET fusion-positive non-small cell lung cancer (NSCLC), thyroid cancer and other solid tumors. Registrational data for pralsetinib in patients with RET fusion-positive NSCLC showed deep and durable clinical responses, with a median duration of response (DOR) not reached. Additional results showed the broad clinical activity of pralsetinib across other RET fusion-positive tumors, including thyroid cancer. Pralsetinib was well-tolerated and safety results were consistent with prior data, with no new safety signals observed. These results are being presented during the American Society of Clinical Oncology 2020 (ASCO20) Virtual Scientific Program.

In addition, Blueprint Medicines today announced that the U.S. and EU marketing applications for pralsetinib for the treatment of locally advanced or metastatic RET fusion-positive NSCLC have been accepted by the U.S. Food and Drug Administration (FDA) and validated by the European Medicines Agency (EMA), respectively. The FDA granted priority review and set an action date of November 23, 2020 under the Prescription Drug User Fee Act. Blueprint Medicines plans to submit an NDA for pralsetinib for advanced RET mutant and RET fusion-positive thyroid cancers in June 2020, under the FDA's Oncology Center of Excellence Real-Time Oncology Review pilot program.

"The use of targeted therapies for molecularly defined subsets of patients is fundamentally altering the treatment of non-small cell lung cancer and, similar to oncogenes like EGFR and ALK, RET is a proven driver and promising therapeutic target," said Justin Gainor, M.D., Director of the Center for Thoracic Cancers and Targeted Immunotherapy at Massachusetts General Hospital Cancer Center and an investigator on the ARROW trial. "The ARROW trial results presented today during the ASCO virtual meeting showed that patients with RET fusion-positive lung cancer treated with the selective RET inhibitor pralsetinib had durable responses. In addition to supporting the development of pralsetinib across a broad population, these data highlight the urgency to test lung cancer patients with next-generation sequencing so that eligible patients may be identified for treatment."

"Building on a unique preclinical profile characterized by selectivity for RET and equipotent activity against predicted resistance mutations, the clinical data for pralsetinib is showing high complete response rates, prolonged durability and a favorable safety profile as a convenient once-daily oral treatment. With this differentiated profile, pralsetinib has the potential to change the standard of care for patients with RET-altered non-small cell lung cancer and thyroid cancer," said Andy Boral, M.D., Ph.D., Chief Medical Officer of Blueprint Medicines. "More broadly, data presented during the ASCO virtual meeting highlight the clinical activity of pralsetinib across ten distinct RET-altered tumor types. These results strongly support continued development of pralsetinib across all RET-altered cancers, regardless of a tumor's tissue of origin, with the goal of delivering transformative benefit to the broadest possible patient population."

Clinical Activity Data

The reported data included response-evaluable populations comprising 116 patients with NSCLC who received a starting dose of 400 mg once daily (QD), including 80 patients with NSCLC previously treated with platinum-based chemotherapy and 26 patients with treatment-naïve NSCLC, 11 patients with RET fusion-positive thyroid cancer, and 12 patients with other RET fusion-positive cancers. Tumor response was assessed by blinded, independent central review using Response Evaluation Criteria in Solid Tumors (RECIST) version 1.1.

RET Fusion-Positive NSCLC

As of a data cutoff of November 18, 2019, pralsetinib demonstrated consistent and robust clinical activity in RET fusion-positive NSCLC, regardless of prior therapy, RET fusion partner or central nervous system (CNS) involvement.

In 80 patients who previously received platinum-based chemotherapy, the ORR was 61 percent (95% CI: 50-72%). Two partial responses (PR) were pending confirmation at the time of the data cut off and were subsequently confirmed. Five percent of patients had a confirmed response (CR) and 14 percent of patients had complete regression of target tumors.

In 26 patients with no prior systemic therapy, the confirmed ORR was 73 percent (95% CI: 52-88%), and the CR rate was 12 percent.

Across all 116 patients, regardless of prior therapy, the median DOR was not reached (95% CI: 11 months, not reached), and the 6-month DOR was 86 percent. Overall, 74 percent of confirmed responders, including all patients with CRs, were on treatment as of the data cutoff.

Robust and durable intracranial activity was shown in nine patients with measurable CNS metastases at baseline. All patients had shrinkage of CNS metastases, with an intracranial CR rate of 33 percent. No CNS responders experienced CNS progressive events. The median CNS DOR was not reached, with ongoing treatment durations up to 12 months in patients with measurable CNS metastases. Among patients without a history of CNS metastases, none have developed new CNS metastases on study as of the data cutoff date.

Other RET Fusion-Positive Cancers

As of a data cutoff of February 13, 2020, pralsetinib demonstrated robust clinical activity in a range of additional RET fusion-positive cancers. In 11 patients with RET fusion-positive thyroid cancer (10 previously treated with systemic therapy), the centrally confirmed ORR was 91 percent (95% CI: 59-100%), and the disease control rate was 100 percent (95% CI: 72-100%). Overall, 70 percent of responders remain on therapy with ongoing treatment durations up to 22 months as of the data cutoff. Across 12 patients with other RET fusion-positive cancers previously treated with systemic therapy, the investigator-assessed ORR was 50 percent (95% CI: 21–79), with one PR pending confirmation. Responses were observed in all evaluable patients with pancreatic adenocarcinoma (n=3) and cholangiocarcinoma (n=2), tumor types with a typically poor prognosis.

Safety Data

As previously reported, as of the data cutoff date of November 18, 2019, a total of 354 patients were enrolled in the ARROW trial at a starting dose of 400 mg QD. Overall, safety results were consistent with previously reported data. Pralsetinib was well-tolerated across tumor types, and most treatment-related adverse events (AEs) were Grade 1 or 2.

The most common treatment-related AEs reported by investigators (≥15 percent) were increased aspartate aminotransferase (AST), anemia, increased alanine aminotransferase (ALT), constipation, hypertension and neutropenia. Investigator-reported Grade 3 or higher treatment-related AEs (≥5 percent) were hypertension, neutropenia and anemia. Only 4 percent of patients discontinued pralsetinib due to treatment-related AEs.

These updated data for pralsetinib are being reported in two presentations at the ASCO20 Virtual Scientific Program Annual Meeting, including a poster discussion presentation on trial results in RET fusion-positive NSCLC (Abstract Number: 9515) and an oral presentation on trial results in other RET fusion-positive cancers (Abstract Number: 109). Copies of the data presentations are available in the “Science—Publications and Presentations” section of Blueprint Medicines' website at www.BlueprintMedicines.com.

Conference Call Information

Blueprint Medicines will host a live webcast today beginning at 8:30 a.m. ET to discuss updated data from the ARROW trial of pralsetinib in RET fusion-positive cancers. To access the live call, please dial (855) 728-4793 (domestic) or (503) 343-6666 (international) and refer to conference ID 8585078. A webcast of the conference call will be available under “Events and Presentations” in the Investors & Media 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 the Clinical Development Program in RET-Altered Cancers

Blueprint Medicines is pursuing a broad development program for pralsetinib in patients with RET fusion-positive NSCLC, RET-mutant medullary thyroid cancer (MTC), RET-fusion thyroid cancer and other advanced solid tumors. The Phase 1/2 ARROW trial and the Phase 3 AcceleRET Lung trial are currently ongoing.

ARROW is designed to evaluate the safety, tolerability and efficacy of pralsetinib 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.

The primary objective of the AcceleRET Lung trial is to evaluate the potential of pralsetinib to extend progression-free survival compared to platinum-based chemotherapy, with or without pembrolizumab, as a first-line treatment for RET fusion-positive NSCLC. The trial is designed to enroll approximately 250 patients randomized to receive either pralsetinib or the investigator's choice of platinum-based chemotherapy regimen with or without pembrolizumab. Patients randomized to the control arm may crossover upon progression to receive pralsetinib. Additional endpoints include overall survival, ORR and DOR. Multiple trial sites are active or planned in North America, Europe and Asia.

Patients and physicians interested in the ARROW or AcceleRET Lung trial can contact the Blueprint Medicines study director at studydirector@blueprintmedicines.com or 1-617-714-6707. Additional information is available at www.BlueprintClinicalTrials.com/ARROW and www.clinicaltrials.gov.

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, 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.

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, thyroid cancer 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 mutation-positive 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 80-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 and certain other drug candidates in Mainland China, Hong Kong, Macau and Taiwan. Blueprint Medicines retains development and commercial rights for pralsetinib 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 have one FDA-approved precision therapy 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 of pralsetinib, including the timing, designs, implementation, enrollment, plans and announcement of results regarding Blueprint Medicines' ongoing and planned clinical trials; plans and timelines for submitting additional marketing applications for pralsetinib and, if approved, commercializing pralsetinib; 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 plan in establishing a commercial infrastructure, and

successfully launching, marketing and selling current or future approved products; 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. 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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