Blueprint Medicines Reports Portfolio Milestones and Outlines 2021 Roadmap for Precision Medicine Leadership

-- AYVAKIT™ (avapritinib) granted FDA breakthrough therapy designation for the treatment of moderate to severe indolent systemic mastocytosis --

-- Positive top-line results from Phase 1 healthy volunteer trial of BLU-263 support plans to initiate Phase 2 HARBOR trial in non-advanced systemic mastocytosis in mid-2021 --

-- Nominated potential first-in-class development candidate targeting double-mutant EGFR, deepening leadership in lung cancer --

-- Nominated potential best-in-class development candidate targeting MAP4K1 under cancer immunotherapy collaboration with Roche --

CAMBRIDGE, Mass., January 11, 2021 – Blueprint Medicines Corporation (NASDAQ: BPMC) today provided an update on key portfolio milestones and outlined a strategic roadmap to become the world's leading precision therapy company.

"For the first time, we enter a new year as a fully integrated, global biopharmaceutical company, with four regulatory approvals in the United States and Europe in 2020, a pipeline of eight wholly owned or partnered precision therapies, and the strongest financial position since our inception," said Jeff Albers, Chief Executive Officer of Blueprint Medicines. "With this solid foundation, we are now scaling our ambition and aim to make real the promise of precision medicine to improve and extend life for as many people with cancer and hematologic disorders as possible. We will do this by bringing our medicines to more patients globally, rapidly advancing a wave of new therapeutic candidates to clinical proof-of-concept, and further expanding our platform-enabled research pipeline."

In addition, Blueprint Medicines today announced the achievement of several portfolio milestones:

- AYVAKIT received breakthrough therapy designation from the U.S. Food and Drug Administration (FDA) for the treatment of moderate to severe indolent systemic mastocytosis (SM), which encompasses the majority of patients with SM, highlighting the medical need in this population as well as the clinical potential of AYVAKIT to demonstrate substantial improvement over the current standard of care.
- Positive top-line results from a Phase 1 trial in healthy volunteers showed BLU-263 was well-tolerated across a range of single- and multiple-ascending doses predicted to potently inhibit D816V mutant KIT, the underlying SM disease driver. These data support development of BLU-263 as a potential treatment for patients with SM and other mast cell disorders.
- The company nominated a selective, brain-penetrant development candidate for treatment-resistant double-mutant EGFR-driven non-small cell lung cancer (NSCLC), with the potential to be first-in-class, showing potent activity against the activating L858R or exon 19 deletion mutations and the acquired C797S mutation, the most common on-target resistance mutation to osimertinib.
- The company nominated a development candidate targeting MAP4K1, a kinase believed to play a role in T-cell regulation, with the potential to be best-in-class. The program was developed under the company's cancer immunotherapy collaboration with Roche. In addition, Blueprint Medicines and Roche have amended their agreement to focus on MAP4K1 and one additional undisclosed target, collectively identified as the most promising targets of the collaboration to date.

Entering 2021, the company's key strategies and goals include:

1. Accelerate global adoption of AYVAKIT and GAVRETO™ (pralsetinib)

AYVAKIT, a selective KIT and PDGFRA inhibitor, is approved in the U.S. and Europe for the treatment of patients with unresectable or metastatic gastrointestinal stromal tumor driven by certain PDGFRA mutations.

- Obtain FDA approval and launch AYVAKIT in advanced SM in the U.S. in the second half of 2021.
- Submit a Type II variation marketing authorization application (MAA) to the European Medicines Agency (EMA) for AYVAKYT® (avapritinib) for advanced SM in the first quarter of 2021.

- Present registrational data from the PATHFINDER trial of AYVAKIT in advanced SM in the first half of 2021.
- Complete enrollment of the registration-enabling Part 2 of the PIONEER trial of AYVAKIT in non-advanced SM in mid-2021.

GAVRETO, a selective RET inhibitor, is approved in the U.S. for the treatment of patients with certain advanced or metastatic RET fusion-positive NSCLC, RET-mutant medullary thyroid cancer (MTC) and RET fusion-positive thyroid cancer. Under a global collaboration, Blueprint Medicines and Roche are developing and commercializing GAVRETO for the treatment of RET-altered cancers.

- Obtain regulatory approval from the European Commission and launch GAVRETO in RET fusion-positive NSCLC in Europe in the first half of 2021.
- Submit a Type II variation MAA to the EMA for GAVRETO for RET-altered thyroid cancers in the second half of 2021.
- Initiate a GAVRETO cohort in Roche's TAPISTRY tumor-agnostic platform trial in the second half of 2021.
- Submit marketing applications for GAVRETO for RET-altered NSCLC and thyroid cancers across multiple additional global geographies in 2021.

2. Advance a new wave of innovative therapeutic candidates into clinical development, with plans to achieve rapid proof-of-concept and regulatory approval.

BLU-263, a next-generation selective KIT inhibitor

- Initiate the Phase 2 HARBOR trial of BLU-263 in patients with non-advanced SM in mid-2021.

Development candidates for treatment-resistant EGFR-driven NSCLC

- Initiate a Phase 1 trial of BLU-945, a triple-mutant EGFR inhibitor, in patients with treatment-resistant EGFR-driven NSCLC in the first half of 2021.
- Initiate a Phase 1 trial of the company's double-mutant EGFR inhibitor in patients with treatment-resistant EGFR-driven NSCLC by the end of 2021.
- Present foundational preclinical data for the company's double-mutant EGFR inhibitor in the first half of 2021.
- Present preclinical data supporting combination of the company's wholly owned double- and triple-mutant EGFR inhibitors in treatment-naïve EGFR-driven NSCLC in the second half of 2021.

Development candidate targeting MAP4K1, under the cancer immunotherapy collaboration with Roche

- Present foundational preclinical data in the first half of 2021.

3. Further expand the company's precision medicine pipeline with a focus on delivering transformational benefit to patients with cancer and hematologic disorders.

- Expand pipeline with one or more development candidates in 2021.
- Pursue external opportunities to complement the company's precision medicine pipeline.

Financial Guidance

Based on its current operating plans, Blueprint Medicines continues to anticipate its existing cash, cash equivalents and investments, together with anticipated future product revenues, will provide sufficient capital to enable the company to achieve a self-sustainable financial profile.

About Blueprint Medicines

Blueprint Medicines is a global precision therapy company that invents life-changing therapies for people with cancer and hematologic disorders. Applying an approach that is both precise and agile, we create medicines that selectively target genetic drivers, with the

goal of staying one step ahead across stages of disease. Since 2011, we have leveraged our research platform, including expertise in molecular targeting and world-class drug design capabilities, to rapidly and reproducibly translate science into a broad pipeline of precision therapies. Today, we are delivering approved medicines directly to patients in the United States and Europe, and we are globally advancing multiple programs for genomically defined cancers, systemic mastocytosis, and cancer immunotherapy. 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 Blueprint Medicines' 2021 goals and anticipated milestones; plans, strategies, timelines and expectations for Blueprint Medicines' current or future approved drugs and drug candidates, including timelines for marketing applications and approvals, the initiation of clinical trials or the results of ongoing and planned clinical trials; the potential benefits of any of Blueprint Medicines' current or 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 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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