Blueprint Medicines' AYVAKYT® (avapritinib) Receives European Commission Approval for the Treatment of Adults with Advanced Systemic Mastocytosis

-- AYVAKYT is the first approved therapy in the European Union designed to selectively target the KIT D816V mutation, the primary driver of disease --

-- Advanced Systemic Mastocytosis (AdvSM) is a debilitating disease characterized by damage across multiple organ systems, reduced overall survival and poor quality of life –

-- Initial commercial launch is planned for Germany immediately following the EC approval --

CAMBRIDGE, Mass., March 25, 2022 -- Blueprint Medicines Corporation (NASDAQ: BPMC) today announced that the European Commission (EC) has expanded the current indication for AYVAKYT® (avapritinib) to include monotherapy for the treatment of adult patients with aggressive systemic mastocytosis (ASM), systemic mastocytosis with an associated hematological neoplasm (SM-AHN) or mast cell leukemia (MCL), after at least one systemic therapy.

"Today we are incredibly proud to bring an innovative new treatment option to individuals who have been impacted by advanced systemic mastocytosis," said Georg Pirmin Meyer, M.D., Senior Vice President, International at Blueprint Medicines. "We believe that AYVAKYT has the potential to shift the treatment paradigm to a precision therapy approach in advanced forms this disease, and we look forward to working closely with national reimbursement bodies across Europe to bring AYVAKYT to patients."

In Europe, Blueprint Medicines plans to initiate its first commercial launch in Germany following the EC approval, and the timing of AYVAKYT commercial availability will vary for other countries based on local reimbursement and access pathways. AYVAKYT will be available in 25 mg, 50 mg, 100mg and 200mg dose strengths, and the recommended starting dose in advanced SM is 200 mg once daily.

The EC decision follows the positive opinion by the Committee for Medicinal Products for Human Use (CHMP) and is based on results from the Phase 1 EXPLORER trial and Phase 2 PATHFINDER trial, in which AYVAKYT showed durable clinical efficacy in advanced SM patients across all disease subtypes after at least one systemic therapy and a generally well-tolerated safety profile.

About AYVAKYT® (avapritinib)
AYVAKYT® (avapritinib) is a kinase inhibitor approved by the European Commission for the treatment of adults with unresectable or metastatic gastrointestinal stromal tumors (GIST) harboring the PDGFRA D842V mutation and for the treatment of adult patients with aggressive systemic mastocytosis (ASM), systemic mastocytosis with an associated hematological neoplasm (SM-AHN) or mast cell leukemia (MCL), after at least one systemic therapy. Under the brand name AYVAKIT, the medicine is approved in the U.S. for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations, and for the treatment of adults with Advanced SM, including aggressive SM (ASM), SM-AHN and mast cell leukemia (MCL).[1]

It is also approved under the brand name AYVAKIT in Mainland China for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V

mutations, and in Hong Kong and Taiwan for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA D842V mutation.[2-4]

AYVAKYT/AYVAKIT is not approved for the treatment of any other indication in the European Union, UK, U.S., or Greater China, or for any indication in any other jurisdiction by any other health authority.

Blueprint Medicines is developing AYVAKYT/AYVAKIT globally for the treatment of advanced and non-advanced SM. The European Commission granted orphan medicinal product designation for AYVAKYT for the treatment of GIST and mastocytosis. The U.S. Food and Drug Administration (FDA) granted breakthrough therapy designation to AYVAKIT for the treatment of moderate to severe indolent SM.

To learn about ongoing or planned clinical trials, contact Blueprint Medicines at medinfoeurope@blueprintmedicines.com and +31 85 064 4001. Additional information is available at blueprintclinicaltrials.com and clinicaltrials.gov.

Please click here to see the Summary of Product Characteristics for AYVAKYT.

About Systemic Mastocytosis
Systemic mastocytosis (SM) is a rare disease driven by the KIT D816V mutation. Uncontrolled proliferation and activation of mast cells result in chronic, severe and often unpredictable symptoms for patients across the spectrum of SM. The vast majority of those affected have non-advanced (indolent or smoldering) SM, with debilitating symptoms that lead to a profound, negative impact on quality of life. A minority of patients have advanced SM, which encompasses a group of high-risk SM subtypes including ASM, SM-AHN and MCL. In addition to mast cell activation symptoms, advanced SM is associated with organ damage due to mast cell infiltration and poor survival. Across advanced SM subtypes, the median overall survival is approximately 3.5 years in ASM, approximately two years in SM-AHN and less than six months in MCL.[5] In Europe, there are about 40,000 patients with SM, and advanced SM represents about 5 to 10 percent of this patient population.[6]

Debilitating symptoms, including anaphylaxis, maculopapular rash, pruritis, diarrhea, brain fog, fatigue and bone pain, often persist across all forms of SM despite treatment with a number of symptomatic therapies. Patients often live in fear of severe, unexpected symptoms, have limited ability to work or perform daily activities, and isolate themselves to protect against unpredictable triggers. Historically, there had been no approved therapies for the treatment of SM that selectively inhibit D816V mutant KIT.[7,8]

About Blueprint Medicines
Blueprint Medicines is a global precision therapy company that invents life-changing therapies for people with cancer and blood 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 systemic mastocytosis, lung cancer and other genomically defined cancers, 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' views with respect to the approval of AYVAKYT and the implications of such approval for 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'

views with respect to the approval of AYVAKYT and the implications of such approval for patients, caregivers and healthcare professionals; expectations regarding the potential benefits of AYVAKYT in treating patients with advanced SM; expectations concerning launch timing and when AYVAKYT will be commercially available in Europe; 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® (pralsetinib); Blueprint Medicines' ability to successfully expand the approved indications for AYVAKIT and GAVRETO or obtain marketing and reimbursement approvals 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 and marketing applications; Blueprint Medicines' ability to develop and commercialize companion diagnostic tests for its current and future drug candidates; Blueprint Medicines' ability to successfully expand its operations, research platform and portfolio of therapeutic candidates and to increase the output of its discovery engine, and the timing and costs thereof; Blueprint Medicines' ability to realize the anticipated benefits of its executive leadership transition plan; and the success of Blueprint Medicines' current and future acquisitions, collaborations, partnerships 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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