Blueprint Medicines' AYVAKYT® (avapritinib) Receives European Commission Approval as the First and Only Treatment for Indolent Systemic Mastocytosis

-- In the European Union, patients with indolent systemic mastocytosis now have an approved medicine that treats the primary driver of disease --

-- Approval based on data from PIONEER trial, in which AYVAKYT achieved significant improvements across a broad range of symptoms with a safety profile comparable to placebo[1] --

CAMBRIDGE, Mass., Dec. 12, 2023 /PRNewswire/ -- Blueprint Medicines Corporation (Nasdaq: BPMC) today announced the European Commission has approved AYVAKYT® (avapritinib) for the treatment of adult patients with indolent systemic mastocytosis (ISM) with moderate to severe symptoms inadequately controlled on symptomatic treatment. AYVAKYT is the first and only approved therapy for people living with ISM in Europe.

Systemic mastocytosis (SM) is a rare hematologic disorder that can lead to a range of debilitating symptoms with a significant impact on patients' quality of life. The majority of patients living with SM have ISM, and there are approximately 40,000 people living with ISM in the European Union.[2,3*] AYVAKYT was designed to potently and selectively target KIT D816V, the primary underlying driver of the disease.

"Today's approval represents an important step toward delivering a new global standard of care for patients with ISM and builds on years of collaboration with the SM community," said Georg Pirmin Meyer, M.D., Senior Vice President, International at Blueprint Medicines. "For the first time in Europe ISM patients have an approved therapy, marking a new era in the treatment of this disease. AYVAKYT is the first approved medicine for both ISM and advanced SM, and our team is committed to bringing this transformative therapy to patients across the spectrum of disease."

"Indolent systemic mastocytosis can be characterized by significant symptom burden across multiple organ systems, which can profoundly impact patients' ability to perform activities of daily living in a relevant proportion of patients," said Jens Panse, M.D., Deputy Director of the Department of Hematology/Oncology of the University Hospital RWTH Aachen. "AYVAKYT represents an important treatment breakthrough as the first medicine approved for patients living with ISM, and the only therapy designed to selectively target the primary genetic driver of the disease. In the PIONEER trial, AYVAKYT showed statistically significant and durable clinical benefits across all measured ISM symptoms with a well-tolerated safety profile. Based on these practice-changing data, AYVAKYT has the potential to advance treatment for a broad range of patients living with ISM."

The approval follows the positive opinion by the Committee for Medicinal Products for Human Use (CHMP), and this EC decision is based on data from the double-blind, placebo-controlled PIONEER trial – the largest study ever conducted in ISM. AYVAKYT showed clinically meaningful improvements versus placebo in the primary and all key secondary endpoints, including overall symptoms and measures of mast cell burden. AYVAKYT was well-tolerated with a favorable safety profile, and most adverse events (AEs) were reported as mild (Grade 1). The most common AEs were flushing, edema, increased blood alkaline phosphate and insomnia.[1]

"Many people living with indolent systemic mastocytosis face unpredictable and severe symptoms, which significantly impair their ability to work or spend quality time with their family, friends and communities," said Patrizia Marcis, President of the Associazione Italiana Mastocitosi (ASIMAS) ODV. "Today's approval offers a new sense of hope to the ISM

community, and we are proud to collaborate with clinical researchers, patients and companies like Blueprint Medicines to advance care for all those living with the disease."

In Europe, Blueprint Medicines plans to initiate its first commercial launch in Germany, followed by additional markets based on local healthcare technology assessment and reimbursement process timelines.

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About AYVAKYT® (avapritinib)

AYVAKYT® (avapritinib) is a kinase inhibitor approved by the European Commission for the treatment of three indications: adults with indolent systemic mastocytosis (ISM), adults 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, and adults with unresectable or metastatic gastrointestinal stromal tumors (GIST) harboring the PDGFRA D842V mutation.[4] Under the brand name AYVAKIT®, the medicine is approved in the U.S. for the treatment of adults with ISM, adults with advanced SM, including ASM, SM-AHN and MCL, and adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations.[5]

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 in about 95 percent of cases.[6] Uncontrolled proliferation and activation of mast cells result in chronic, severe and often unpredictable symptoms for patients across the spectrum of SM. In the European Union, approximately 40,000 people live with indolent systemic mastocytosis.[2,3*] A broad range of symptoms, including anaphylaxis, maculopapular rash, pruritis, diarrhea, brain fog, fatigue and bone pain, frequently persist in patients with ISM despite treatment with multiple symptom-directed therapies. This burden of disease can lead to a profound, negative impact on quality of life. 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.

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 have brought our approved medicines to patients in the United States and Europe, and we are globally advancing multiple programs for mast cell disorders, including systemic mastocytosis and chronic urticaria, breast cancer and other cancers vulnerable to CDK2 inhibition, as well as

EGFR-mutant lung cancer. 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 implications of the approval of AYVAKYT for people living with ISM in Europe; plans for Blueprint Medicines' first commercial launch in Germany, followed by additional markets; plans, strategies, timelines and expectations for Blueprint Medicines' current or future approved drugs and drug candidates; the potential benefits of any of Blueprint Medicines' current or future approved drugs or drug candidates in treating patients; and Blueprint Medicines' financial performance, 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: preliminary activity and safety data may not be representative of more mature data; the risk of delay of any current or planned clinical trials or the development of Blueprint Medicines' current or future drug candidates; risks related to 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; preclinical and clinical results for Blueprint Medicines' drug candidates may not support further development of such drug candidates either as monotherapies or in combination with other agents or may impact the anticipated timing of data or regulatory submissions; the timing of the initiation of clinical trials and trial cohorts at clinical trial sites and patient enrollment rates may be delayed or slower than anticipated; actions of regulatory agencies may affect the initiation, timing and progress of clinical trials; the success of Blueprint Medicines' current and future collaborations, financing arrangements, partnerships or licensing arrangements may impact Blueprint Medicines' ability to capitalize on the market potential of its approved drugs and drug candidates; and risks related to Blueprint Medicines' ability to obtain, maintain and enforce patent and other intellectual property protection for its products and current or future drug candidates it is developing. 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.

References

*Based on Cohen 2014 study of 548 adults with SM diagnosed from 1997 to 2010 in linked Danish national health registries, with a 14-year limited-duration prevalence estimated at 9.59 per 100,000 as of 1 January 2011

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