Blueprint Medicines' Leadership in Driving Continued Innovation in Systemic Mastocytosis Highlighted at 2023 ASH Annual Meeting

-- HARBOR Part 1 trial data in indolent systemic mastocytosis showed elenestinib was well-tolerated with broad symptom improvement, supporting further development to expand and extend company's SM franchise leadership --

CAMBRIDGE, Mass., December 9, 2023 – Blueprint Medicines Corporation (Nasdaq: BPMC) today announced data showcasing its commitment to advance the scientific understanding and treatment of systemic mastocytosis (SM) at the 65th American Society of Hematology (ASH) Annual Meeting and Exposition being held December 9-12 in San Diego. Data that will be presented include results from the HARBOR Part 1 trial of elenestinib in indolent systemic mastocytosis (ISM) and analyses of real-world data highlighting the burden of and urgency to treat ISM.

"Blueprint Medicines has transformed the standard of care for advanced and indolent systemic mastocytosis with AYVAKIT® (avapritinib), and its proven and compelling clinical profile is redefining what well-controlled disease means for patients living with SM," said Becker Hewes, M.D., Chief Medical Officer at Blueprint Medicines. "Building on the success of AYVAKIT and the clinical expertise amassed during its development, we are strategically advancing our investigational next-generation KIT D816V inhibitor, elenestinib, to expand and extend Blueprint Medicines' SM franchise leadership over the long term."

HARBOR Part 1 trial data in patients with ISM showed elenestinib was well-tolerated and clinically active at all dose levels tested, supporting further development. In patients treated with elenestinib, most adverse events (AEs) were Grade 1 or 2, and there were no discontinuations due to AEs. Elenestinib showed clinically meaningful symptom improvements as assessed by the validated Indolent Systemic Mastocytosis Symptom Assessment Form Total Symptom Score (ISM-SAF TSS), and rapid and profound reductions across multiple measures of mast cell burden.

At ASH, new data on the burden of disease highlight the urgency to treat patients with ISM. A real-world analysis of U.S. health claims data showed patients with ISM had lower survival compared to a matched population cohort ($p<0.0001$), and a model-based analysis assessed that the lifetime risk of progression from ISM to advanced SM was approximately 20 percent. In addition, a data presentation reports on a diagnostic tool to aid in the identification of patients with SM, which was developed based on a real-world analysis at The Quality Cancer Care Alliance (QCCA).

In total, Blueprint Medicines' presence at ASH builds on over a decade of innovative research in the field of SM, and reflects the company's ongoing leadership in transforming care for patients living with the disease.

ASH abstracts are listed below:

Oral Presentations

Presentation Title: Decreased Survival Among Patients with Indolent Systemic Mastocytosis: A Population-Level Retrospective Cohort Analysis Using Healthcare Claims Dataset
Session Title: 634. Myeloproliferative Syndromes: Clinical and Epidemiological: Rare Myeloproliferative Neoplasms: Unveiling Promising Pathways and Novel Therapies
Session Date & Time: Today, December 9 from 9:30 – 11:00 a.m. PT (12:30 – 2:00 p.m. ET)
Presentation Date & Time: Today, December 9 at 10:00 a.m. PT (1:00 p.m. ET)

Abstract Number: 75
Location: San Diego Convention Center, Ballroom 20AB

Presentation Title: Elenestinib, an Investigational, Next Generation KIT D816V Inhibitor, Reduces Mast Cell Burden, Improves Symptoms, and Has a Favorable Safety Profile in Patients with Indolent Systemic Mastocytosis: Analysis of the HARBOR Trial
Session Title: 634. Myeloproliferative Syndromes: Clinical and Epidemiological: Rare Myeloproliferative Neoplasms: Unveiling Promising Pathways and Novel Therapies
Session Date & Time: Today, December 9 from 9:30 – 11:00 a.m. PT (12:30 – 2:00 p.m. ET)
Presentation Date & Time: Today, December 9 at 10:15 a.m. PT (1:15 p.m. ET)
Abstract Number: 76
Location: San Diego Convention Center, Ballroom 20AB

Poster Presentation

Presentation Title: Development and Validation of a Diagnostic Tool for the Timely Diagnosis of Patients with Systemic Mastocytosis
Session Title: 906. Outcomes Research—Myeloid Malignancies: Poster II
Session Date & Time: Sunday, December 10 from 6:00 – 8:00 p.m. PT (9:00 – 11:00 p.m. ET)
Abstract Number: 3800
Location: San Diego Convention Center, Halls G-H

Publication-Only Abstract

Title: A Model of Cumulative Risk of Disease Progression Among Patients with Indolent Systemic Mastocytosis
Abstract Number: 6406

Copies of Blueprint Medicines data presentations from the ASH annual meeting will be available in the "Science—Publications and Presentations" section of the company's website at www.BlueprintMedicines.com.

About AYVAKIT (avapritinib)

AYVAKIT (avapritinib) is a precision therapy approved by the U.S. Food and Drug Administration (FDA) for the treatment of three indications: adults with ISM, adults with advanced SM, including aggressive SM (ASM), SM with an associated hematological neoplasm (SM-AHN) and mast cell leukemia (MCL), and adults with unresectable or metastatic gastrointestinal stromal tumor (GIST) harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations. For more information, visit AYVAKIT.com. This medicine is approved by the European Commission (AYVAKYT®) for the treatment of adults with ASM, SM-AHN or MCL, after at least one systemic therapy, and adults with unresectable or metastatic GIST harboring the PDGFRA D842V mutation. Please click here to see the full U.S. Prescribing Information for AYVAKIT, and click here to see the European Summary of Product Characteristics for AYVAKYT.

In November 2023, the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) issued a positive opinion recommending the approval of AYVAKYT for the treatment of adult patients with ISM with moderate to severe symptoms inadequately controlled on symptomatic treatment.

To learn about ongoing or planned clinical trials, contact Blueprint Medicines at medinfo@blueprintmedicines.com or 1-888-BLU-PRNT (1-888-258-7768). Additional information is available at blueprintclinicaltrials.com or clinicaltrials.gov.

Important Safety Information

Intracranial Hemorrhage—Serious intracranial hemorrhage (ICH) may occur with AYVAKIT treatment; fatal events occurred in <1% of patients. Overall, ICH (eg, subdural hematoma, ICH, and cerebral hemorrhage) occurred in 2.9% of 749 patients who received AYVAKIT in clinical trials. In Advanced SM patients who received AYVAKIT at 200 mg daily, ICH occurred in 2 of 75 patients (2.7%) who had platelet counts ≥50 x 10^9/L prior to initiation of therapy and in 3 of 80 patients (3.8%) regardless of platelet counts. In ISM patients, no events of ICH occurred in the 246 patients who received any dose of AYVAKIT in the PIONEER study.

Monitor patients closely for risk factors of ICH, which may include history of vascular aneurysm, ICH or cerebrovascular accident within the prior year, concomitant use of anticoagulant drugs, or thrombocytopenia.

Symptoms of ICH may include headache, nausea, vomiting, vision changes, or altered mental status. Advise patients to seek immediate medical attention for signs or symptoms of ICH.

Permanently discontinue AYVAKIT if ICH of any grade occurs. In Advanced SM patients, a platelet count must be performed prior to initiating therapy. AYVAKIT is not recommended in Advanced SM patients with platelet counts <50 x 10^9/L. Following treatment initiation, platelet counts must be performed every 2 weeks for the first 8 weeks. After 8 weeks of treatment, monitor platelet counts every 2 weeks or as clinically indicated based on platelet counts. Manage platelet counts of <50 x 10^9/L by treatment interruption or dose reduction.

Cognitive Effects—Cognitive adverse reactions can occur in patients receiving AYVAKIT and occurred in 33% of 995 patients overall in patients who received AYVAKIT in clinical trials including: 28% of 148 Advanced SM patients (3% were Grade ≥3), and 7.8% of patients with ISM who received AYVAKIT + best supportive care (BSC) versus 7.0% of patients who received placebo + BSC (<1% were Grade 3). Depending on the severity and indication, withhold AYVAKIT and then resume at same dose or at a reduced dose upon improvement, or permanently discontinue.

Photosensitivity—AYVAKIT may cause photosensitivity reactions. In all patients treated with AYVAKIT in clinical trials (n=1049), photosensitivity reactions occurred in 2.5% of patients. Advise patients to limit direct ultraviolet exposure during treatment with AYVAKIT and for one week after discontinuation of treatment.

Embryo-Fetal Toxicity—AYVAKIT can cause fetal harm when administered to a pregnant woman. Advise pregnant women of the potential risk to a fetus. Advise females and males of reproductive potential to use an effective method of contraception during treatment with AYVAKIT and for 6 weeks after the final dose of AYVAKIT. Advise women not to breastfeed during treatment with AYVAKIT and for 2 weeks after the final dose.

Adverse Reactions—The most common adverse reactions (≥20%) in patients with Advanced SM were edema, diarrhea, nausea, and fatigue/asthenia.

The most common adverse reactions (≥10%) in patients with ISM were eye edema, dizziness, peripheral edema, and flushing.

Drug Interactions—Avoid coadministration of AYVAKIT with strong or moderate CYP3A inhibitors. If coadministration with a moderate CYP3A inhibitor cannot be avoided in patients with Advanced SM, reduce dose of AYVAKIT. Avoid coadministration of AYVAKIT with strong or moderate CYP3A inducers.

To report suspected adverse reactions, contact Blueprint Medicines Corporation at 1-888-258-7768 or FDA at 1-800-FDA-1088 or http://www.fda.gov/medwatch.

Please click here to see the full Prescribing Information for AYVAKIT.

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' expectations regarding the potential benefits of AYVAKIT for the treatment of patients with ISM, including with respect to the burden of and urgency to treat ISM; 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.

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