Blueprint Medicines Highlights 2024 Corporate Strategy and Business Priorities at 42nd Annual J.P. Morgan Healthcare Conference

-- AYVAKIT® (avapritinib) launch in indolent systemic mastocytosis to drive strong revenue growth in 2024, with ongoing U.S. launch and recent EU approval --

-- Expanding mast cell disease leadership with oral wild-type KIT inhibitor, BLU-808, advancing into clinical development; IND submission planned in Q2 2024 --

-- Maintaining durable cash position through focused investment and global commercial execution --

-- Kate Haviland, Chief Executive Officer, to present at J.P. Morgan conference today at 10:30 a.m. PT (1:30 p.m. ET) --

SAN FRANCISCO, Calif., Jan. 8, 2024 /PR Newswire/ -- Blueprint Medicines Corporation (Nasdaq: BPMC) today outlined its 2024 corporate strategy to deliver accelerated revenue growth, sustainable research and development, and a clear path to profitability.

Kate Haviland, Chief Executive Officer of Blueprint Medicines, said:

“As we enter 2024, AYVAKIT’s early launch success in indolent systemic mastocytosis has grown our conviction that AYVAKIT has the potential to be a multi-billion-dollar therapy that will drive long-term growth into the next decade. We know that the first few quarters of a launch are critical in defining the trajectory of a new medicine, and we have built a strong foundation for success with AYVAKIT as we continue to drive growth in the U.S**.** and expand our launch in Europe this year.

Throughout 2023, we also made significant progress across our research and development pipeline enabling us to focus investments on our most promising programs. A core component of our growth strategy is to build on our leadership position in SM by expanding to other allergic-inflammatory diseases where mast cells play a core role in the biology, and we have integrated infrastructure that we can efficiently scale. Across our portfolio, we are investing in our most compelling opportunities to deliver innovative, life-changing medicines to patients, while maintaining a strong and sustainable financial profile.”

Focused investment strategy in 2024 to drive long-term growth and maintain durable cash position

1. Prioritized programs for investment

Blueprint Medicines is building portfolio scale in therapeutic areas where there are significant medical needs in large patient populations and the company has a deep understanding of biological pathways, a potential to drive best-in-class efficacy and an ability to leverage expertise and infrastructure.

Mast cell diseases

- Extend the company’s leadership position in systemic mastocytosis with the ongoing launch of AYVAKIT in the U.S. and EU and continued development of the next-generation KIT D816V inhibitor elenestinib.
- Expand into larger patient populations with allergic-inflammatory diseases with BLU-808, an oral wild-type KIT inhibitor, including chronic urticaria and other diseases where mast cells are core to the biology.

Breast cancer and other solid tumors

- Advance combination development of BLU-222, a highly selective CDK2 inhibitor with best-in-class potential, in HR+/HER2- breast cancer based on positive previously reported monotherapy clinical data.

45 Sidney Street

Cambridge, MA 02139

- Progress ongoing strategic partnership discussions to maximize the potential of BLU-222 as a backbone combination therapy in HR+/HER2- breast cancer and other CDK2-vulnerable cancers.
- Advance additional programs including BLU-956, a next-generation CDK2 inhibitor development candidate nominated in 2023, and targeted protein degrader research programs for CDK2 and an undisclosed target to support long-term lifecycle management.

2. De-prioritized programs

Blueprint Medicines is discontinuing investment in specific programs, based on the evolving external landscape, emerging clinical data and partnering considerations.

Lung cancer

- Discontinue further investment in the early clinical-stage therapies BLU-945 and BLU-451 for EGFR-mutant NSCLC and explore strategic options, including potential out-licensing, based on the evolving external landscape and emerging clinical data.
- In February 2023, Blueprint Medicines announced Roche's decision to terminate the global collaboration agreement for GAVRETO® (pralsetinib). Given Blueprint Medicines' lack of global infrastructure in lung and thyroid cancer, the company has decided to discontinue global development and marketing of GAVRETO in territories excluding the U.S. and Greater China. The companies will continue working on transition and wind-down activities anticipated to begin in the first quarter of 2024; further information on product discontinuation timing to be provided in the near future.
- Blueprint Medicines has identified a potential alternate partner for GAVRETO in the U.S. and is continuing to work with the involved parties to define a scenario that enables continued availability of GAVRETO in the U.S.
- Blueprint Medicines expects the wind-down of the Roche collaboration for GAVRETO will result in significantly lower year-over-year operating expenses related to GAVRETO in 2024 and will not affect the $175 million upfront payment received under a 2022 financing agreement with Royalty Pharma.

As a result of continued strategic portfolio prioritization, Blueprint Medicines expects a year-over-year decline in operating expenses in 2024. The company plans to provide financial guidance for 2024, including anticipated AYVAKIT revenue, when it reports fourth quarter and full-year 2023 financial results in February 2024.

2024 Corporate Milestones

The company's anticipated 2024 corporate milestones include:

Mast cell diseases

- Present long-term safety and efficacy data from the PIONEER trial of AYVAKIT in indolent SM (ISM) in the first half of 2024.
- Submit an investigational new drug (IND) application for BLU-808 in the second quarter of 2024.
- Initiate the registration-enabling Part 2 of the HARBOR trial of elenestinib in ISM in the second half of 2024.

Breast cancer and other solid tumors

- Continue ongoing strategic business development discussions.
- Present data for BLU-222 in combination with ribociclib and fulvestrant in patients with HR+/HER2- breast cancer in the first half of 2024.
- Provide update on BLU-222 registration plan in HR+/HER2- breast cancer in the second half of 2024.

J.P. Morgan Healthcare Conference Presentation Information

Kate Haviland, Chief Executive Officer of Blueprint Medicines, will present a company overview and 2024 outlook at the 42nd Annual J.P. Morgan Healthcare Conference on Monday, January 8 at 10:30 a.m. PT / 1:30 p.m. ET. A live webcast of the presentation and Q&A breakout session will be available by visiting the "Events and Presentations" section of Blueprint Medicines' website at http://ir.blueprintmedicines.com. A replay of the webcast will be archived on Blueprint Medicines' website for 30 days following the presentation.

About Blueprint Medicines

Blueprint Medicines is a global precision therapy company that invents life-changing medicines. Applying an approach that is both precise and agile, we create therapies that selectively target the root cause of disease, 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 solid tumors. For more information, visit www.BlueprintMedicines.com and follow us on X (formerly 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 AYVAKIT's potential to be a multi-billion-dollar therapy; the continued growth of AYVAKIT in the U.S. and the expansion of AYVAKYT's launch in Europe; the expansion of Blueprint Medicines' mast cell disease franchise with the development of BLU-808; the advancement of its clinical development of BLU-222, preclinical development of BLU-956 and progression of targeted protein degrader research programs for CDK2; 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, outlook 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. 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.

Trademarks

Blueprint Medicines, AYVAKIT, AYVAKYT, GAVRETO and associated logos are trademarks of Blueprint Medicines Corporation.

Investor Relations Contact

Jenna Cohen
857-209-3147
ir@blueprintmedicines.com

Media Relations Contact

Andrew Law
617-844-8205
media@blueprintmedicines.com