**Blueprint Medicines Provides 2023 Portfolio Goals to Achieve Precision at Scale**

*-- Expect to achieve high-end of 2022 total revenue guidance of $180 million to $200 million and AYVAKIT product revenue guidance of $108 million to $111 million --*

*-- Positioned for significant near-term growth with potential FDA approval of AYVAKIT® (avapritinib) in indolent systemic mastocytosis in the middle of 2023 --*

*-- Anticipate multiple clinical data catalysts across mast cell disorder, EGFR-mutant non-small cell lung cancer, and breast cancer programs in 2023 --*

*-- Kate Haviland, Chief Executive Officer, to present company overview and 2023 outlook at 41st Annual J.P. Morgan Healthcare Conference on Tuesday, January 10 at 4:30 p.m. PT / 7:30 p.m. ET --*

CAMBRIDGE, Mass., January 5, 2023 – Blueprint Medicines Corporation (NASDAQ: BPMC) today outlined upcoming portfolio milestones advancing the company's 2027 Blueprint to achieve Precision at Scale, a five-year growth strategy to reach broad patient populations by leveraging its scientific leadership, proven development capability and integrated business infrastructure.

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

"Entering 2023, Blueprint Medicines is poised to achieve significant growth and value creation for patients and shareholders, leveraging our strong enterprise capabilities and infrastructure.

In the coming year, we plan to scale the impact of AYVAKIT by addressing the medical needs of a substantially larger group of patients with an anticipated label expansion in indolent systemic mastocytosis, where AYVAKIT has the potential to be a disease modifying therapy and a major treatment advance. Our experienced team, who is in the field bringing AYVAKIT to patients with advanced systemic mastocytosis, is ready to expand our efforts upon FDA approval.

In addition, the data we plan to generate this year will start to truly characterize the potential of our clinical-stage pipeline to solve complex medical problems in EGFR-mutant lung cancer and CDK2-vulnerable breast cancer, which both represent large established commercial opportunities.

This diverse set of growth drivers, together with our existing commercial portfolio and proven discovery platform, put us squarely on the path to achieve our vision for doubling our impact between now and 2027."

The company's key strategies and upcoming goals are to:

1. **Launch AYVAKIT in indolent systemic mastocytosis (SM), improving treatment options for patients across the spectrum of the disease and expanding the company's leadership position in SM.**

    - Present registrational PIONEER trial data for AYVAKIT in indolent SM at the American Academy of Allergy, Asthma and Immunology Annual Meeting in February 2023
    - Achieve European Medicines Agency validation of a type II variation marketing authorization application for AYVAKYT for indolent SM in the first half of 2023
    - Achieve approval from the Food and Drug Administration (FDA) and initiate the launch of AYVAKIT in indolent SM in the U.S. in the middle of 2023
    - Present HARBOR Part 1 trial data for elenestinib (BLU-263) in indolent SM in the second half of 2023

2. **Advance a robust portfolio of innovative clinical programs, addressing broader patient populations, towards registration.**

EGFR-driven NSCLC:

- Submit an investigational new drug application to the FDA for BLU-525 in the first half of 2023
- Present initial CONCERTO trial dose escalation data for BLU-451 in EGFR exon 20-positive NSCLC in the first half of 2023
- Provide an initial clinical data update on SYMPHONY trial expansion for the combination of BLU-945 and osimertinib in first-line EGFR L858R-positive NSCLC in the second half of 2023

Breast and other CDK2-vulnerable cancers:

- Present initial VELA trial dose escalation data for BLU-222 in CDK2-vulnerable cancers in the first half of 2023

**3. Grow the R&D pipeline with diverse, high-value programs from the company's prolific scientific platform.**

- Nominate a development candidate targeting wild-type KIT for treatment of chronic urticaria in the middle of 2023

**J.P Morgan Healthcare Conference Presentation Information**

Kate Haviland, Chief Executive Officer of Blueprint Medicines, will present a company overview and 2023 outlook at the 41st Annual J.P. Morgan Healthcare Conference on Tuesday, January 10 at 4:30 p.m. PT / 7:30 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 each presentation.

**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 our scientific innovation into a broad pipeline of important approved and investigational precision therapies aimed at addressing difficult-to-treat cancers and blood disorders. Today, we are delivering our approved medicines to patients in the United States, Europe, and in other geographies ourselves or through our partners. In addition, we are globally advancing multiple programs for systemic mastocytosis, lung cancer, breast 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 FDA approval for AYVAKI for the treatment of indolent SM; plans and timing for presenting data from the PIONEER trial of AYVAKIT in patients with indolent SM; statements regarding plans, strategies, timelines and expectations for interactions with the FDA and other regulatory authorities, statements regarding plans and expectations for Blueprint Medicines' current or future approved drugs and drug candidates, including, the results of ongoing and planned clinical trials; plans to expand Blueprint Medicines' scientific platform; Blueprint Medicines' plans, strategies and timelines to nominate development candidates; the potential benefits of any of Blueprint Medicines' current or future approved drugs or drug candidates in treating patients; preliminary selected financial results; and Blueprint Medicines' strategy, goals

and anticipated financial performance, 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 continuing to establish and expand a commercial infrastructure, and successfully launching, marketing and selling current or future approved products; Blueprint Medicines' ability to successfully expand the approved indications for AYVAKIT/AYVAKYT or obtain marketing approval for AYVAKIT/AYVAKYT 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 timing and results of preclinical and clinical studies for Blueprint Medicines' drug candidates, which may not support further development of such drug candidates or may impact the anticipated timing of data, publications or regulatory submissions; actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials; Blueprint Medicines' ability to obtain, maintain and enforce patent and other intellectual property protection for AYVAKIT/AYVAKYT or any drug candidates it is developing; Blueprint Medicines' ability to develop and commercialize companion diagnostic tests for AYVAKIT/AYVAKYT or any of its current and future drug candidates; Blueprint Medicines' ability to successfully expand its operations and scientific platform and the costs thereof; and the success of Blueprint Medicines' current and future collaborations, partnerships, financing 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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