

Blueprint Medicines Announces Global Collaboration with Roche to Develop and Commercialize Pralsetinib for Patients with RET-Altered Cancers

-- Blueprint Medicines and Genentech, a member of the Roche Group, to co-commercialize pralsetinib and equally share profits in the U.S. --

-- Roche will obtain an exclusive license to commercialize pralsetinib outside the U.S., excluding Greater China --*

-- Blueprint Medicines will receive \$775 million in upfront payments, comprising \$675 million in cash and \$100 million equity investment priced at \$96.57 per share, and is eligible to receive up to \$927 million in potential milestones, plus royalties on net product sales outside the U.S. --

-- Transformative partnership creates path for Blueprint Medicines to achieve a self-sustainable financial profile --

-- Blueprint Medicines to host conference call today at 8:00 a.m. ET --

CAMBRIDGE, Mass., July 14, 2020 – Blueprint Medicines Corporation (NASDAQ: BPMC), a precision therapy company focused on genomically defined cancers, rare diseases and cancer immunotherapy, today announced that it has entered into a global collaboration with Roche and Genentech, a member of the Roche Group, to develop and commercialize pralsetinib, an investigational once-daily oral precision therapy for the treatment of people with cancer driven by oncogenic RET alterations, including non-small cell lung cancer (NSCLC), medullary thyroid cancer (MTC), other thyroid cancers and other solid tumors. Under the collaboration, Blueprint Medicines and Genentech will co-commercialize pralsetinib in the U.S. and Roche will obtain exclusive commercialization rights for pralsetinib outside of the U.S., excluding Greater China. The companies also plan to expand development of pralsetinib in multiple treatment settings and explore development of a next-generation RET inhibitor as part of this collaboration.

The collaboration combines Blueprint Medicines' pralsetinib and precision therapy expertise with Roche's global reach, integrated personalized healthcare capabilities and portfolio of cancer therapies. Marketing applications for pralsetinib are submitted or planned for RET fusion-positive NSCLC, RET mutation-positive MTC and RET fusion-positive thyroid cancer in the U.S., Europe and other geographies.

"With Roche's global reach and unparalleled expertise in personalized healthcare, this collaboration will accelerate our ability to bring pralsetinib to patients with significant medical needs around the world and expand development of pralsetinib across multiple treatment settings where there is potential to benefit even broader patient populations," said Jeff Albers, Chief Executive Officer of Blueprint Medicines. "In addition, the collaboration is transformative for Blueprint Medicines and our efforts to build the leading precision medicine company, as it enables us to continue to build best-in-class commercial capabilities, further invest in our rapidly growing pipeline including our systemic mastocytosis programs and fortify our strong financial position to bridge the company to a self-sustaining future."

"We are very excited to enter into this collaboration with Blueprint Medicines, a partner we have already been working with for four years, with the goal of bringing a potentially transformative treatment option to patients with rare RET-driven cancers as quickly as possible," said James Sabry, Head of Roche Pharma Partnering. "In bringing pralsetinib to patients, we will leverage our global reach and expertise in oncology, as well as our capabilities in diagnostics and the use of real-world data toward our aim of providing personalised treatments for patients."

Based on its current operating plans, Blueprint Medicines anticipates its existing cash and investments, together with the upfront payments and anticipated product revenues, will provide sufficient capital to enable the company to achieve a self-sustainable financial profile as the company continues to commercialize and expand development of its approved medicine AYVAKIT™ (avapritinib) and advance new innovative research programs.

Under the terms of the agreement, Blueprint Medicines will grant Roche an exclusive worldwide license excluding Greater China and the U.S., and a co-exclusive license in the U.S. to develop and commercialize pralsetinib. In addition, Roche will have the right to opt in to a next-generation RET compound co-developed under the collaboration.

Blueprint Medicines will receive \$775 million in upfront payments, including a cash payment of \$675 million and an equity investment by Roche of \$100 million in Blueprint Medicines' common stock at a purchase price of \$96.57 per share. Blueprint Medicines will be eligible to receive up to an additional \$927 million in contingent payments, including specified development, regulatory and sales-based milestones for pralsetinib and any licensed product containing a next-generation RET compound.

In the U.S., Blueprint Medicines and Genentech will work together to co-commercialize pralsetinib, with the companies equally sharing responsibilities, profits and losses. In addition, Blueprint Medicines is eligible to receive tiered royalties ranging from high-teens to mid-twenties on annual net sales of pralsetinib outside the U.S.

Blueprint Medicines and Roche have agreed to co-develop pralsetinib globally in RET-altered solid tumors, including NSCLC, MTC and other thyroid cancers, as well as other solid tumors. The companies will share global development expenses based on pre-specified cost-sharing.

The closing of a minority portion of the equity investment is subject to the expiration or termination of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, and other customary closing conditions.

CStone Pharmaceuticals will retain all rights to the development and commercialization of pralsetinib in Greater China under its existing collaboration with Blueprint Medicines.

** Greater China encompasses Mainland China, Hong Kong, Macau and Taiwan.*

Conference Call Information

Blueprint Medicines will host a live webcast today beginning at 8:00 a.m. ET to discuss the collaboration. To access the live call, please dial (855) 728-4793 (domestic) or (503) 343-6666 (international) and refer to conference ID 9477613. A webcast of the conference call will be available under "Events and Presentations" in the Investors & Media section of Blueprint Medicines' website at <http://ir.blueprintmedicines.com>. The archived webcast will be available on Blueprint Medicines' website approximately two hours after the conference call and will be available for 30 days following the call.

About RET-Altered Solid Tumors

RET activating fusions and mutations are key disease drivers in many cancer types, including NSCLC and multiple types of thyroid cancer. RET fusions are implicated in approximately 1 to 2 percent of patients with NSCLC and approximately 10 to 20 percent of patients with papillary thyroid cancer, while RET mutations are implicated in approximately 90 percent of patients with advanced MTC. In addition, oncogenic RET alterations are observed at low frequencies in colorectal, breast, pancreatic and other cancers, and RET fusions have been observed in patients with treatment-resistant EGFR-mutant NSCLC.

About Pralsetinib

Pralsetinib is an investigational, once-daily oral precision therapy designed to selectively target oncogenic RET alterations. Blueprint Medicines is developing pralsetinib for the treatment of patients with RET-altered NSCLC, various types of thyroid cancer and other solid tumors.

Blueprint Medicines has submitted new drug applications (NDAs) to the U.S. Food and Drug Administration (FDA) for pralsetinib for the treatment of RET fusion-positive NSCLC, RET mutation-positive MTC and RET fusion-positive thyroid cancer. For the NDA for RET fusion-positive NSCLC, the FDA granted priority review and assigned a November 23, 2020 action date under the Prescription Drug User Fee Act. In addition, Blueprint Medicines has submitted a marketing authorization application to the European Medicines Agency for pralsetinib for the treatment of RET fusion-positive NSCLC. The FDA has granted breakthrough therapy designation to pralsetinib for the treatment of RET fusion-positive NSCLC that has progressed following platinum-based chemotherapy and for RET mutation-positive MTC that requires systemic treatment and for which there are no acceptable alternative treatments. The FDA has accepted the MTC NDA for its Real-Time Oncology Review (RTOR) pilot program, which aims to explore a more efficient review process to ensure safe and effective treatments are available to patients as early as possible.

Pralsetinib was designed by Blueprint Medicines' research team, leveraging the company's proprietary compound library. In preclinical studies, pralsetinib consistently demonstrated sub-nanomolar potency against the most common RET fusions, activating mutations and predicted resistance mutations. In addition, pralsetinib demonstrated markedly improved selectivity for RET compared to pharmacologically relevant kinases, including approximately 80-fold improved potency for RET versus VEGFR2. By suppressing primary and secondary mutants, pralsetinib has the potential to overcome and prevent the emergence of clinical resistance. Blueprint Medicines believes this approach will enable durable clinical responses across a diverse range of RET alterations, with a favorable safety profile.

About Blueprint Medicines

Blueprint Medicines is a precision therapy company striving to improve human health. With a focus on genomically defined cancers, rare diseases and cancer immunotherapy, we are developing transformational medicines rooted in our leading expertise in protein kinases, which are proven drivers of disease. Our uniquely targeted, scalable approach empowers the rapid design and development of new treatments and increases the likelihood of clinical success. We have one FDA-approved precision therapy and are currently advancing multiple investigational medicines in clinical development, along with a number of research programs. For more information, visit www.BlueprintMedicines.com and follow us on [Twitter](https://twitter.com/BlueprintMeds) (@BlueprintMeds) and [LinkedIn](https://www.linkedin.com/company/blueprintmedicines).

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 the collaboration agreement among Blueprint Medicines, Roche and Genentech, including anticipated upfront, milestone and other payments, equity investment and other financial terms of the collaboration agreement; plans and timelines for the development of pralsetinib, including plans to expand development of pralsetinib in multiple treatment settings; plans to develop a next-generation RET inhibitor; plans and timelines for submitting additional marketing applications for pralsetinib and, if approved, commercializing pralsetinib; the potential benefits of pralsetinib in treating patients; plans to expand development of AYVAKIT™ (avapritinib) and advance new innovative research programs; expectations regarding Blueprint Medicines' existing cash, cash equivalents and investments, including expectations for achieving a self-sustainable financial profile; 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 plan in establishing a commercial infrastructure, and successfully launching, marketing and selling current or future approved products; the delay of any current or planned clinical trials or the development of Blueprint Medicines' drug candidates or licensed product candidate; 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; Blueprint Medicines' ability to develop and commercialize companion diagnostic tests for its current and future drug candidates; and the success of Blueprint Medicines' current and future collaborations 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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