

Blueprint Medicines Announces Strategic Collaboration with Alexion to Advance Kinase Drug Candidates in Rare Genetic Disease

- *Collaboration combines Blueprint Medicines' kinase-focused drug discovery platform with Alexion's experience developing and commercializing therapies for severe and life-threatening disorders –*
- *Blueprint Medicines to receive \$15 million upfront payment, research reimbursement, milestone payments and royalties -*

CAMBRIDGE, Mass., March 3, 2015 – Blueprint Medicines today announced a strategic collaboration with Alexion to discover, develop and commercialize novel drug candidates for an undisclosed activated kinase target, which is the cause of a rare genetic disease. Blueprint Medicines will apply its kinase-focused drug discovery platform to identify and optimize drug candidates and will conduct all research activities prior to the filing of an Investigational New Drug (IND) application with the Food and Drug Administration. Alexion will be responsible for the development and commercialization of these Blueprint Medicines' drug candidates under the collaboration.

“Our kinase-focused platform, which integrates a novel target discovery engine and a proprietary compound library, enables us to craft highly selective kinase drugs for genomic drivers of disease across many therapeutic areas. Alexion is the ideal partner for our rare genetic disease program with their successful track record developing and commercializing therapies for severe and life-threatening disorders,” said Jeffrey Albers, Chief Executive Officer of Blueprint Medicines. “Working with Alexion on this target will allow the team at Blueprint Medicines to focus on our primary strategic area of oncology, while we leverage our platform in additional therapeutic areas.”

Under the terms of the agreement, Blueprint Medicines will receive an upfront payment of \$15 million and will be reimbursed for all research expenses. Blueprint Medicines is eligible to receive over \$250 million in payments upon the successful achievement of pre-specified preclinical, clinical, regulatory and commercial milestones. In addition, Blueprint Medicines will be eligible to receive royalty payments following the commercialization of the product.

“Blueprint Medicines' unique discovery platform enables it to create drug candidates for extremely challenging kinase targets. Even in these early stages, Blueprint Medicines' compounds show impressive selectivity toward the mutant kinase, thereby sparing other kinases and delivering drug to the specified target,” said Martin Mackay, Ph.D., Executive Vice President, Global Head of Research and Development at Alexion. “We look forward to partnering with the talented Blueprint Medicines' team to advance a highly innovative therapy for patients suffering from a devastating rare genetic disease.”

About Blueprint Medicines

Blueprint Medicines makes kinase drugs to treat patients with genomically defined diseases. Led by a team of industry innovators, Blueprint Medicines integrates a novel target discovery engine and a proprietary compound library to understand the blueprint of cancer and craft highly selective therapies. This empowers the Blueprint Medicines team to develop patient-defined medicines aimed at eradicating cancer. Blueprint Medicines is privately held and raised \$115 million in financing since its 2011 inception.

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