

Blueprint Medicines Unveils New Drug Discovery Program for Patients with RET Fusions at 26th EORTC-NCI-AACR Symposium

– Builds on Company’s novel discovery of several kinase fusion genes in cancer –

CAMBRIDGE, Mass., November 20, 2014 – Blueprint Medicines, a leader in discovering and developing highly selective kinase inhibitors for patients with genomically defined cancers, today disclosed a new drug discovery program targeting cancers with RET fusions and predicted resistance mutations. The announcement was made during an oral presentation at the 26th EORTC-NCI-AACR Symposium on Molecular Targets and Cancer Therapeutics in Barcelona, Spain, on Blueprint Medicines’ discovery of several novel kinase fusions implicated in cancer and identification of several new cancer indications for known kinase fusions.

“One of the greatest challenges in treating cancer is addressing cancer cells’ ability to become resistant to therapy. Our new drug discovery program uniquely addresses both the activated wild-type form of RET and its predicted resistance mutations, enabling us to potentially develop a transformative therapy for cancer patients with RET fusions,” said Christoph Lengauer, PhD, MBA, Chief Scientific Officer of Blueprint Medicines. “With the unveiling of this program, we add another proof point for the productivity of Blueprint Medicines’ team and the differentiation of its kinase-focused drug discovery platform, which combines an innovative target discovery engine with a first-of-its-kind fully-annotated chemical library.”

Using proprietary computational tools and techniques, Blueprint Medicines’ scientists identified RET fusions in four of 20 cancer types analyzed, including thyroid, lung, breast and colon cancers, providing a strong rationale for the development of a novel RET inhibitor across multiple patient populations. The identification of RET fusions in colon and breast cancers was one of the novel findings in the research. Combining genomics with structural and cell biology, Blueprint Medicines’ scientists were able to predict future resistance mutations of RET inhibitors currently in clinical studies. Blueprint Medicines’ drug discovery is ongoing.

Blueprint Medicines’ new RET inhibitor program adds to the Company’s existing pipeline, consisting of BLU-285, the first known selective inhibitor of KIT Exon 17 for patients with systemic mastocytosis and gastrointestinal stromal tumors (GIST), and BLU-554, the first known selective FGFR4 inhibitor for patients with hepatocellular carcinoma (HCC). Blueprint Medicines expects to initiate clinical trials for its KIT and FGFR4 programs in 2015.

Fusion genes (or fusions) are known to contribute to the development of cancers. A fusion gene is formed from the abnormal association of two normally separated genes, as a result of a translocation or other chromosomal rearrangements. Fusion genes are proven cancer drug targets, and a number of approved and exploratory drugs target kinase fusions.

About Blueprint Medicines

Blueprint Medicines is a patient-driven oncology company discovering and developing highly selective kinase inhibitors for patients with genomically defined cancers. Led by a management team and advisors with world renowned expertise in cancer genomics, drug discovery and clinical oncology, Blueprint Medicines has developed a platform that combines genomics with a novel small molecule library of kinase inhibitors, enabling Blueprint Medicines to rapidly discover potent and highly selective drugs against clear drivers of diseases. Founded in 2011, Blueprint Medicines is privately held and initially backed by Third Rock Ventures and Fidelity BioSciences. For more information, please visit www.BlueprintMedicines.com.



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