

Blueprint Medicines Discovers Novel Cancer Drug Targets

- Findings and research approach published in *Nature Communications* –

CAMBRIDGE, Mass., Sept. 10, 2014 /PRNewswire/ -- Blueprint Medicines, a leader in discovering and developing highly selective kinase inhibitors for genomically defined cancers, today announced the identification of several new cancer drug targets known as fusion genes. These findings were made through a proprietary computational approach and led to two new drug discovery programs at Blueprint Medicines. Blueprint Medicines' research and scientific approach were published in the September 10, 2014 edition of *Nature Communications* (Stransky N. *et al.*, [The landscape of kinase fusions in cancer](#)).

"This research represents an important observation in cancer genomics, with potential clinical trial implications in oncology," said personalized medicine pioneer Levi Garraway, MD, PhD, associate professor at Dana-Farber Cancer Institute and Harvard Medical School in the Department of Medical Oncology, the co-director of the Cancer Genetics Program at Dana-Farber/Harvard Cancer Center, and a senior associate member at the Broad Institute of MIT and Harvard. "It also shows how thoughtful computational algorithms can engender novel discoveries through focused applications to the treasure trove of existing cancer genome data."

Leveraging proprietary computational tools, Blueprint Medicines uncovered 14 known fusions in new cancer types and identified 18 novel fusions that likely play a key role in cancer. Fusion genes are known to contribute to the development of cancers. A fusion gene is formed from the 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 or exploratory drugs currently exist for many of the newly discovered fusions.

"The genomic discoveries highlighted in this publication are significant. They not only offer the potential to lead to truly novel therapeutics but also have immediate implications for the diagnosis and treatment of cancer patients," said Brian Druker, MD, director of the Knight Cancer Institute and JELD-WEN Chair of Leukemia Research at Oregon Health & Science University and scientific founder of Blueprint Medicines. "These findings provide important insights in the fight against cancer. I am pleased they were published quickly in the interest of patients."

Blueprint Medicines is currently advancing two drug discovery programs against kinase fusion targets. In addition, Blueprint Medicines expects to initiate clinical trials in 2015 with its more advanced programs:

- BLU-285: The first known selective inhibitor of KIT Exon 17 mutants for the underserved systemic mastocytosis patient population, as well as a genomically defined subset of patients with gastrointestinal stromal tumors (GIST), and
- BLU-554: The first known paralog-selective FGFR4 inhibitor for patients suffering from hepatocellular carcinoma with FGFR4 pathway activation.

"We are proud of our target identification capabilities, which coupled with our proprietary chemical library of carefully crafted compounds tailored to the kinome, enable us to uniquely fulfill our mission of consistently discovering and developing highly selective medicines for patients with genomically defined cancers," said Christoph Lengauer, PhD, MBA, chief scientific officer of Blueprint Medicines. "The work published in *Nature Communications* underlines the productivity of the scientists in our target discovery team who yielded several important results just three years since the Company's inception."

About Blueprint Medicines

Blueprint Medicines is a patient-driven oncology company discovering and developing highly selective kinase inhibitors for genomically defined cancer patients. 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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