

Blueprint Medicines Presents Positive Data on BLU-554 for Hepatocellular Carcinoma

- In vivo data support potential clinical benefit of first therapeutic to target a defined subset of liver cancer patients based on genetic profile -

CAMBRIDGE, Mass., Sept. 8, 2014 /PRNewswire/ -- Blueprint Medicines today announced the presentation of preclinical data investigating BLU-554, a first-in-class selective fibroblast growth factor receptor 4 (FGFR4) inhibitor, for the treatment of patients with hepatocellular carcinoma (HCC). Results demonstrate in vivo efficacy, including sustained tumor regression and complete remissions in mice with tumors that represent a genomically defined subset of human HCC. The oral presentation was given at the International Liver Cancer Association 8th Annual Conference in Kyoto, Japan.

"Our preclinical data are early promising proof for the potential clinical benefit of targeting a defined subset of HCC patients based on the genetic profile of their cancers," said Christoph Lengauer, PhD, MBA, chief scientific officer of Blueprint Medicines. "We are pleased by the liver cancer community's interest in our program. We look forward to advancing BLU-554 toward clinical proof of concept studies in 2015."

Results showed dose-dependent tumor regression of BLU-554 in xenograft models with an abnormally activated FGFR4 signaling pathway due to overexpression or amplification of the FGFR4 ligand FGF19. Twenty percent of the mice remained tumor free 30 days after treatment, and BLU-554 was well tolerated.

Blueprint Medicines expects to initiate clinical trials with BLU-554 for the treatment of HCC patients with an abnormally activated FGFR4 signaling pathway in 2015. The targeted patient population is estimated to represent approximately one-third of all HCC patients.[1]

About Hepatocellular Carcinoma

Liver cancer is the sixth most common cancer and is the second most common cause of death from cancer worldwide. More than 780,000 cases of liver cancer are diagnosed worldwide each year.[2] Hepatocellular carcinoma is the most common form of liver cancer and accounts for approximately 80 percent of the primary malignant liver tumors in adults.[3]

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.

[1] Data on file.

[2] Ferlay J, et al., Cancer Incidence, Mortality and Prevalence Worldwide in 2012. GLOBOCAN 2012.

[3] El-Serag, Hashem, Current Concepts in Hepatocellular Carcinoma. *The New England Journal of Medicine*. 2011; 365: 1118-27.

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