



**Blueprint Medicines Debuts Comprehensive Preclinical Data Set on First Selective Drug
for Patients with Systemic Mastocytosis
at 2014 American Society of Hematology Annual Meeting**

SAN FRANCISCO, Calif., December 8, 2014 – Blueprint Medicines, a leader in discovering and developing selective kinase drugs for patients with genetically defined diseases, today announced the presentation of its comprehensive set of preclinical data for BLU-285, the Company’s lead program and the first drug to selectively block KIT D816V, the driver mutation in more than 95 percent of patients with systemic mastocytosis. These data, unveiled for the first time, highlight the potent activity, *in vivo* efficacy, and exceptional selectivity of BLU-285 and were presented at the 56th American Society of Hematology Annual Meeting & Exposition in San Francisco, California. Blueprint Medicines plans to initiate clinical trials with BLU-285 in 2015.

“These data provide evidence for the exciting promise of BLU-285 to benefit patients with systemic mastocytosis,” said Christoph Lengauer, Chief Scientific Officer of Blueprint Medicines. “No therapy exists currently for these patients due to the challenge to selectively and sustainably inhibit the driver mutation of this debilitating disease. BLU-285 is the first drug to inhibit the mutation selectively and potently. The discovery of BLU-285 demonstrates the power of Blueprint Medicines’ platform and team to understand the genetic blueprints of diseases and craft highly selective drugs with the aim of rapidly developing medicines that eradicate diseases.”

In the poster, “First Selective KIT D816V Inhibitor for Patients with Systemic Mastocytosis,” Blueprint Medicines shows the body of evidence supporting the decision to advance BLU-285 into clinical studies. These data for BLU-285 include:

- Superior biochemical and cellular potency and kinome selectivity relative to other kinase inhibitors tested in systemic mastocytosis;
- Highly potent (>90 percent) KIT D816V target inhibition sustained for a minimum of 24 hours in a KIT D816 mutant-driven disease model after one dose; and
- Dose dependent tumor regression and good tolerability in a mouse mastocytoma model.

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. For more information, please visit www.BlueprintMedicines.com.

About Systemic Mastocytosis

Systemic mastocytosis (SM) is a disease characterized by the abnormal proliferation and accumulation of mast cells. In aggressive cases, these mast cells accumulate in organs such as the bone marrow, spleen and liver, and result in compromised organ function with average patient survival of only 3 to 5 years after diagnosis. The mast cells of nearly all patients with SM harbor a heterozygous D816V mutation in the activation loop of KIT which confers constitutive, ligand-independent activation of the kinase, suggesting that the mutation is the driver of disease in these patients. Approximately 5,000 patients in the developed markets have advanced SM. Approximately 20,000 patients in the developed markets have the indolent form of the disease.

Contact:

Investor Relations:

Beth DelGiacco

Stern Investor Relations, Inc.

212 362 1200

beth@sternir.com

Media Relations:

David Polk

Chandler Chicco Companies

310 309 1029

dpolk@chandlerchiccocompanies.com

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