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Blueprint Medicines wins FDA Approval of AYVAKIT to treat GIST

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AYVAKIT is the first approved precision therapy for GIST and the only highly active treatment for PDGFRA exon 18 mutant Gastrointestinal Stromal Tumor (GIST)

Blueprint Medicines Corporation, a precision therapy company focused on genomically defined cancers, rare diseases and cancer immunotherapy, has announced that the U.S. Food and Drug Administration (FDA) has approved AYVAKIT™ (avapritinib) for the treatment of adults with unresectable or metastatic gastrointestinal stromal tumor (GIST) harboring a platelet-derived growth factor receptor alpha (PDGFRA) exon 18 mutation, including PDGFRA D842V mutations. AYVAKIT is the first precision therapy approved to treat a genomically defined population of patients with GIST.

The FDA granted a full approval to AYVAKIT based on efficacy results from the Phase 1 NAVIGATOR clinical trial, as well as combined safety results from multiple clinical trials for avapritinib. In patients with PDGFRA exon 18 mutant GIST, AYVAKIT had an overall response rate (ORR) of 84 percent (95% CI: 69%, 93%), and a median duration of response (DOR) was not reached. Blueprint Medicines plans to make AYVAKIT available in the U.S. within a week.

GIST is a rare, genomically driven sarcoma of the gastrointestinal (GI) tract. Approximately 6 percent of patients with newly diagnosed GIST have PDGFRA exon 18 mutations. The most common PDGFRA exon 18 mutation is the D842V mutation, which is resistant to all other approved therapies. A retrospective study showed that when these patients were treated with imatinib, they had an ORR of 0 percent.

Blueprint Medicines is dedicated to helping patients with PDGFRA exon 18 mutant GIST access treatment with AYVAKIT and providing robust support throughout their treatment journey. As part of this commitment, Blueprint Medicines is introducing YourBlueprint™, a patient support program that offers access and affordability solutions for individuals receiving AYVAKIT.