Opna Bio Receives Orphan Drug Designation for OPN-6602, an Oral EP300/CBP Bromodomain Inhibitor, for Multiple Myeloma

OPN-6602 is Currently in a Phase 1 Clinical Trial for Relapsed/Refractory Multiple Myeloma

South San Francisco, CA – February 12, 2025 - Opna Bio, a clinical-stage biopharmaceutical company focused on the discovery and development of novel oncology therapeutics, announced today that the FDA has granted orphan drug designation (ODD) to one of its lead programs, OPN-6602, for the treatment of multiple myeloma (MM). OPN-6602 is an oral, small molecule inhibitor of the E1A binding protein (EP300) and CREB-binding protein (CBP) currently being tested in a Phase 1 trial in patients with relapsed or refractory MM.

Multiple myeloma is a rare and aggressive cancer of the plasma cells in the bone marrow that often leads to serious complications such as bone damage, kidney failure and immune suppression. The disease is typically diagnosed in older adults, with limited treatment options available for patients with relapsed or refractory disease.

"We are pleased to have received ODD for OPN-6602 for the treatment of multiple myeloma, a further validation of the drug's therapeutic potential in patients with this disease who have limited treatment options once they have relapsed," said Gideon Bollag, PhD, chief scientific officer.

Orphan Drug Designation is granted by the FDA to encourage the development of therapies for rare diseases, which are defined as those affecting fewer than 200,000 people in the U.S. The designation provides several benefits, including tax credits for clinical trial costs, a waiver of certain FDA fees, and eligibility for seven years of market exclusivity upon approval.

Opna recently presented data at the American Society of Hematology (ASH) meeting in December 2024 showing that in human-derived multiple myeloma models, OPN-6602 suppresses tumor growth, while downregulating key MM driving genes. Synergistic effects were observed with OPN-6602 in combination with dexamethasone, pomalidomide and mezigdomide. OPN-6602's distinct pharmacokinetic profile allows for continuous daily dosing that potentially results in a lower incidence of toxicities and improved efficacy.

The Phase 1 study in patients with relapsed or refractory multiple myeloma (NCT06433947) is taking place at multiple sites in the U.S. Opna Bio expects to complete the single agent, dose-escalation phase of the trial in 2026. Further development of OPN-6602 in combination with other standard-of-care agents in multiple myeloma is planned.

About Opna Bio

Opna Bio is a clinical-stage biopharmaceutical company focused on the discovery and development of novel oncology therapeutics. The company's broad portfolio targets multiple

drivers of cancer, including a novel oncology discovery program focused on the fragile-X multifunctional RNA-binding protein (FMRP) and a diversified pipeline of promising oncology assets. The Opna team has a proven track record of scientific expertise and commercial value creation, having advanced multiple FDA-approved drugs to market. Opna's lead clinical compounds include OPN-2853, a potentially best-in-class BET bromodomain inhibitor, being evaluated in patients with myelofibrosis in combination with ruxolitinib, and OPN-6602, a dual EP300/CBP inhibitor, currently being studied in a first-in-human Phase 1 clinical trial in patients with multiple myeloma. For more information, please visit opnabio.com.

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