



**Cleave Therapeutics Announces Commencement of a Phase 1 Clinical Study of CB-5339, A Valosin-Containing Protein (VCP)/p97 Inhibitor, in Patients with Acute Myeloid Leukemia (AML) and Myelodysplastic Syndrome (MDS)**

**San Francisco—July 28, 2020** - Cleave Therapeutics, Inc., a clinical-stage biopharmaceutical company focused on VCP/p97 as a novel target in oncology, announced today that the first patient has been dosed with CB-5339 in a Phase 1 clinical trial of patients with relapsed/refractory acute myeloid leukemia (AML) or relapsed/refractory intermediate or high-risk myelodysplastic syndrome (MDS). CB-5339 is a potent and selective, second-generation, oral small molecule inhibitor of VCP/p97.

“We are pleased to begin patient enrollment of our clinical study in AML and MDS, which marks an important milestone in evaluating the initial safety and potential therapeutic benefit of VCP inhibitors for patients with cancer,” said Amy Burroughs, president and chief executive officer of Cleave. “This clinical program stems from more than a decade of research by scientists who have identified VCP/p97 as a pan-cancer core fitness target that is essential for cancer cell growth and survival.”

The primary objectives of the Phase 1 study are to characterize the safety and tolerability and determine the maximum tolerated dose (MTD) and/or recommended Phase 2 dose of CB-5339. Additional objectives are to examine the pharmacokinetic parameters and estimate the anti-tumor effects of CB-5339. CB-5339 is administered orally for four days, followed by a three-day treatment-free period weekly in successive 28-day cycles. The trial is expected to enroll 50 to 60 patients in the United States and Australia and does not select or exclude patients based on tumor genetic profile.

“Despite several new drug approvals in the last several years, there remains an urgent need for well-tolerated, effective therapies for patients with myeloid malignancies,” said Courtney DiNardo, MD, MSCE, associate professor, Department of Leukemia, Division of Cancer Medicine, The University of Texas MD Anderson Cancer Center. “We are eager to study CB-5339 as a unique oral therapy that could benefit our AML and MDS patients – perhaps even regardless of their mutational status - by disrupting stress pathways that are critical to cancer cells.”

In addition to the AML/MDS trial, the National Cancer Institute (NCI), part of the National Institutes of Health, is sponsoring a Phase 1 clinical trial with CB-5339 in solid tumors and lymphomas. For more information on both of these trials, please visit the [AML/MDS study](#) or the [Solid Tumors/Lymphomas study](#) at [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

**About Acute Myeloid Leukemia (AML) and Myelodysplastic Syndrome (MDS)**

Acute myeloid leukemia is a type of cancer of the blood and bone marrow, caused by mutations in the genetic material (DNA) of myeloid stem cells which result in the formation of leukemic cells. These cells, also referred to as “AML cells,” cannot mature into fully functional blood cells, and they multiply uncontrollably. Nearly 20,000 newly diagnosed acute myeloid leukemia patients and 12,000 deaths are expected from AML in the U.S this year. Myelodysplastic syndromes are a group of diverse bone marrow disorders in which the bone marrow does not produce enough healthy blood cells. Nearly 15,000 newly diagnosed cases of MDS are expected per year.

**About Cleave Therapeutics**

Cleave Therapeutics is a clinical-stage biopharmaceutical company focused on VCP/p97 as a novel target in protein homeostasis and cellular stress pathways for therapeutic use in cancer. The privately held company, based in San Francisco, is studying CB-5339, its second-generation, small molecule VCP/p97 inhibitor, in a Phase 1 clinical trial in acute myeloid leukemia (AML) and myelodysplastic syndrome (MDS), while the National Cancer Institute (NCI) is sponsoring and evaluating CB-5339 in a Phase 1 clinical trial of patients with solid tumors and lymphomas. Cleave investors include 5AM Ventures, Bristol-Myers Squibb, Orbimed, U.S. Venture Partners (USVP), Arcus Ventures, Astellas Venture Management, and Osage University Partners. For additional information, visit [www.cleavetherapeutics.com](http://www.cleavetherapeutics.com).

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