Pipeline Therapeutics Announces First Subject Dosed with PIPE-791 in Phase 1 Study

August 1, 2023

-Trial to evaluate PIPE-791 for safety and pharmacokinetics in healthy volunteers-

– Expects enrollment to be completed by YE 2023-

SAN DIEGO, August 1, 2023 – Pipeline Therapeutics, a clinical-stage biopharmaceutical company pioneering precision neuroregeneration, today announced that the first subject was dosed in the Phase 1 clinical trial of PIPE-791 in healthy volunteers. PIPE-791 is a potent, selective and brain-penetrant small molecule antagonist of the lysophosphatidic acid 1 receptor (LPA1).

"Initiating dosing in this first in human study with PIPE-791 is a significant milestone for Pipeline that brings us one step closer to transforming the treatment of CNS indications by addressing both remyelination and neuroinflammation," said Carmine Stengone, President and CEO of Pipeline. "PIPE-791 is our lead, wholly owned program, and we look forward to evaluating its ability to inhibit the LPA1 receptor to bring benefit to people with MS and other neurological and systemic diseases."

Last month, Pipeline announced that it received clearance from the U.S. Food and Drug Administration (FDA) to initiate the Phase 1 clinical trial of PIPE-791, which is a randomized, double-blind, placebo-controlled dose-ranging study expected to enroll approximately 80 healthy volunteer subjects.

Stephen Huhn, M.D., Chief Medical Officer and Senior Vice President of Clinical Development of Pipeline, added, "The goal of this trial is to characterize the safety, tolerability and pharmacokinetic profile of PIPE-791 in healthy volunteers, and to inform dose selection in future clinical studies. We expect to fully enroll the study by the end of 2023."

About PIPE-791

The Company's wholly owned program, PIPE-791, is in Phase 1 development for remyelination and neuroinflammation.

PIPE-791 is a potent, selective, brain-penetrant small molecule antagonist of the LPA1 receptor that has demonstrated activity in *in vitro* and *in vivo* models and was tolerated in IND-enabling studies. Lysophosphatidic acid (LPA) is a pro-inflammatory lipid, upregulated in the plasma, peripheral blood mononuclear cells and cerebrospinal fluid of MS patients. LPA activates the G-protein coupled LPA1 receptor on both oligodendrocyte precursor cells (OPCs) and microglia resulting in a local neuroinflammatory response limiting remyelination.

PIPE-791 has demonstrated *in vitro* evidence of oligodendrocyte differentiation, myelination, and protection from cytokine-induced oligodendrocyte cell death. In pre-clinical *in vivo* models, PIPE-791 has demonstrated evidence of central nervous system LPA1 receptor occupancy, increased remyelination, and inhibition of neuroinflammation.

About Pipeline Therapeutics

Pipeline Therapeutics is a clinical-stage biopharmaceutical company pioneering the development and commercialization of first-in-class therapies for precision neuroregeneration including myelin restoration, synaptogenesis and axonal repair. The Company has a broad pipeline of programs to address multiple CNS disorders. In April 2023, Pipeline announced that it entered into a global license and development agreement with Janssen Pharmaceutica NV (Janssen) for its lead program, PIPE-307, a highly selective M1 antagonist, in central nervous system disorders. The Company's lead wholly-owned program, PIPE-791, an LPA1 receptor antagonist, is advancing through Phase 1 testing to support treatment of neurological diseases.

For more information, please visit www.pipelinetherapeutics.com and engage with us on LinkedIn.

Company Contact:
Peter Slover
CFO
ir@pipeline-tx.com

Investor Contact:
Amy Conrad
858-914-1962
amy@juniper-point.com