Fate Therapeutics
Guiding cells to improve life

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Fate Therapeutics Initiates Phase 2 Clinical Study of ProHema for the Treatment of Hematologic Malignancies

San Diego, CA – Fate Therapeutics, Inc., a biopharmaceutical company engaged in the discovery and development of adult stem cell modulators, announced today the initiation of a randomized, controlled, Phase 2 multi-center study of its investigational hematopoietic stem cell therapy, ProHema, in adult patients undergoing double umbilical cord blood transplantation (dUCBT) for hematologic malignancy. The advancement of ProHema into later-stage development builds upon positive results from a Phase 1b single-center study, interactions with the U.S. Food and Drug Administration and refinements to the product manufacturing process. The previously completed Phase 1b study achieved its primary objective of demonstrating safety and tolerability. The study also established early clinical proof-of-concept – trends of accelerated neutrophil recovery, improved 100-day survival and low rates of graft-versus-host disease were evident, and durable and preferential reconstitution with ProHema occurred in 10 of 12 evaluable patients. ProHema is produced through a proprietary, two-hour, ex vivo modulation process, which has been shown to significantly activate key biological pathways involved in hematopoietic stem cell homing, proliferation and survival in preclinical models.

“Allogeneic umbilical cord blood transplantation holds great promise as a potentially curative treatment for children and adults with hematologic malignancies and many other life-threatening, non-malignant disorders,” said Christian Weyer, M.D., M.A.S., President and Chief Executive Officer of Fate Therapeutics. “ProHema is being developed with the intent to improve outcomes in patients undergoing cord blood transplantation by facilitating both accelerated engraftment and durable reconstitution using a simple, point-of-care, *ex vivo* modulation process. The initiation of Phase 2 marks an important milestone for the company and brings us one step closer towards achieving this objective.”

The Phase 2 study is expected to enroll at least 45 adult patients undergoing dUCBT for the treatment of hematologic malignancies. Patients will be randomized, with a ratio of 2:1, to receive either ProHema plus an unmanipulated cord blood unit or two unmanipulated cord blood units. The study will evaluate time to neutrophil and platelet recovery, incidence of serious infections and graft-versus-host disease, 100-day mortality and relative dominance of ProHema over the unmanipulated cord in contributing to reconstitution. Results are expected in 2013.

“The preclinical and clinical data obtained to date suggest that ProHema may address several of the unmet medical needs in the evolving field of hematopoietic stem cell transplantation,” said Steven Devine, M.D., Professor of Medicine and Program Director of the Blood and Marrow Transplant Program at the Ohio State University and a principal investigator of the Phase 2 clinical study. “While further investigation is required, an intervention that enables early and durable reconstitution of the best-HLA-matched cord blood unit has the potential to improve patient outcomes and substantially enhance the therapeutic value proposition of cord blood transplant.”

About ProHema
ProHema is an innovative cord blood-derived cell therapy containing pharmacologically-modulated hematopoietic stem cells (HSCs). ProHema is produced through a proprietary, two-hour, *ex vivo* cell modulation process that results in rapid activation of key biological pathways involved in homing, proliferation and survival of HSCs. In preclinical testing, ProHema has demonstrated the potential to accelerate engraftment and to drive durable hematopoietic reconstitution, without the need for multi-week expansion protocols. In an initial Phase 1b study in adult patients with hematologic malignancies undergoing double umbilical cord blood transplant (dUCBT), the median time to neutrophil recovery (> 500 cells/µL) with ProHema was 17.5 days, which compares favorably to historical norms for patients undergoing dUCBT. In that study, ProHema provided the dominant source of hematopoiesis in 10 of 12 evaluable subjects, suggesting that treatment with ProHema may accelerate engraftment and drive durable and preferential reconstitution.