

July 29, 2014

## **Fate Therapeutics Announces FDA Clearance of IND for Clinical Development of PROHEMA in Inherited Metabolic Disorders**

### **Phase 1b PROVIDE Study in Pediatric Patients with Inherited Metabolic Disorders Expected to Begin in 4Q14**

SAN DIEGO, July 29, 2014 (GLOBE NEWSWIRE) -- Fate Therapeutics, Inc. (Nasdaq:FATE), a biopharmaceutical company engaged in the discovery and development of adult stem cell modulators to treat orphan diseases, announced today that the U.S. Food and Drug Administration (FDA) has cleared its Investigational New Drug Application (IND) for the clinical development of PROHEMA® in pediatric patients undergoing hematopoietic stem cell (HSC) transplantation for the treatment of inherited metabolic disorders (IMDs). The FDA's clearance of the IND allows the Company to begin expanding its clinical investigation of PROHEMA into rare, non-malignant disorders. The Company plans to initiate enrollment of the "PROVIDE" trial (PROHEMA eValuation for the treatment of Inherited metabolic Disorders) in pediatric patients with IMDs in the fourth quarter of 2014.

"For many severe inherited metabolic disorders, including those with central nervous system involvement, cellular enzyme replacement through unrelated donor cord blood transplantation has emerged as a potentially transformative therapeutic intervention," commented Christian Weyer, M.D., M.A.S., President and Chief Executive Officer of Fate Therapeutics. "We believe pharmacologically-optimized HSC therapeutics, such as PROHEMA, hold significant promise for improving outcomes in patients with inherited metabolic and other rare genetic disorders. FDA clearance of the PROVIDE study marks an important step towards our goal of clinically evaluating this novel treatment paradigm in the non-malignant disease setting."

The PROVIDE trial is designed to enroll up to 12 patients with various forms of IMDs, between the ages of 1 and 18, at up to three leading U.S. pediatric HSC transplant centers. The trial's inclusion criteria allows for the enrollment of patients with different types of lysosomal and peroxisomal storage diseases such as Hurler and Hunter syndromes, Krabbe disease and various other leukodystrophies, among others. The primary endpoint of the PROVIDE trial is safety as assessed by neutrophil engraftment.

PROHEMA (16, 16-dimethyl prostaglandin E2, or dmPGE2, modulated cord blood), the Company's lead pharmacologically-modulated HSC therapeutic, is currently in Phase 2 clinical development for the treatment of adult patients with hematologic malignancies undergoing allogeneic HSC transplantation, and the Company intends to initiate enrollment of a Phase 1b clinical trial in pediatric patients with hematologic malignancy in mid-2014. In 2010, the FDA granted PROHEMA orphan designation for the enhancement of stem cell engraftment in patients undergoing allogeneic cord blood HSC transplantation. In *in vivo* murine models of allogeneic HSC transplantation, Fate Therapeutics demonstrated that the use of PROHEMA, as compared to unmodulated HSCs, led to a significant increase both in the engraftment of donor HSCs and in the donor-derived expression of enzyme in the brain.

#### **About Inherited Metabolic Disorders (IMDs) and Allogeneic HSC Transplantation (HSCT)**

Inherited metabolic disorders include a range of genetic enzyme deficiencies that interfere with critical metabolic pathways necessary to maintain normal organ function. In many of these disorders, the enzyme deficiency leads to cellular accumulation of toxic intermediates within the brain, causing progressive neurological damage that cannot be addressed with enzyme replacement therapy. For those inherited metabolic disorders, which include over 20 lysosomal and peroxisomal storage diseases such as Hurler and Hunter syndromes, Krabbe disease and multiple leukodystrophies, allogeneic HSCT holds potential as a one-time, definitive therapy. Following allogeneic HSCT, donor-derived cells can migrate to and engraft in the brain, providing a long-term supply of an otherwise deficient enzyme to the central nervous system in a process known as cross-correction.

#### **About PROHEMA®**

PROHEMA is a pharmacologically-modulated, cord blood-derived hematopoietic stem cell (HSC) therapeutic. 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 clinical trial in adult patients with hematologic malignancies undergoing double umbilical cord blood transplant (dUCBT), the median time to neutrophil recovery (> 500 cells/ $\mu$ L) with PROHEMA was 17.5 days, which compares favorably to historical norms for patients undergoing dUCBT. In that trial, 100-day survival with

PROHEMA was 100%, and 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 hematologic reconstitution.

### **About Fate Therapeutics, Inc.**

Fate Therapeutics is a clinical-stage biopharmaceutical company engaged in the discovery and development of pharmacologic modulators of adult stem cells, including small molecules and therapeutic proteins, to treat orphan diseases. The Company has built two adult stem cell modulation platforms: a hematopoietic stem cell (HSC) modulation platform, which seeks to optimize the therapeutic potential of HSCs for treating patients with hematologic malignancies and rare genetic disorders, and a muscle satellite stem cell modulation platform, which seeks to activate the regenerative capacity of muscle for treating patients with degenerative muscle disorders. The Company is presently advancing its lead HSC product candidate, PROHEMA, in Phase 2 clinical development for hematologic malignancies, while also advancing its proprietary Wnt7a protein analogs in preclinical development for the treatment of muscular dystrophies. Fate Therapeutics is headquartered in San Diego, CA. For more information, please visit [www.fatetherapeutics.com](http://www.fatetherapeutics.com).

### **Forward-Looking Statements**

This release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding the therapeutic potential of PROHEMA®, and our clinical development plans for PROHEMA®, including the timing of, and our ability to conduct, the PROVIDE and PROMPT studies. These and any other forward-looking statements in this release are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to, the risks that the results of PROHEMA® observed in prior preclinical and clinical development may not be replicated in our PROVIDE clinical trial, or other current or subsequent clinical trials, of PROHEMA®, and that PROHEMA® may not produce the therapeutic benefits suggested by the results observed in our prior clinical development, or may cause other unanticipated adverse effects, in current or subsequent clinical trials, the risk of cessation or delay of any ongoing or planned preclinical or clinical development activities for a variety of reasons, including additional information that may be requested or additional obligations that may be imposed by the FDA as a condition to our commencement and continuation of clinical trials with PROHEMA®, any difficulties or delays in patient enrollment in the PROVIDE study, or any adverse events or other negative results that may be observed in the PROVIDE study. For a discussion of other risks and uncertainties, and other important factors, any of which could cause our actual results to differ from those contained in the forward-looking statements, see the risks and uncertainties detailed in the company's periodic filings with the Securities and Exchange Commission, including but not limited to the company's Form 10-Q for the quarter ended March 31, 2014, and from time to time the company's other investor communications. Fate Therapeutics is providing the information in this press release as of this date and does not undertake any obligation to update any forward-looking statements contained in this press release as a result of new information, future events or otherwise.

CONTACT: Paul Cox, Stern Investor Relations, Inc.

212.362.1200, [paul@sternir.com](mailto:paul@sternir.com)