FATE THERAPEUTICS INC
Form 10-K
March 12, 2015

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Table of Contents

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

(Mark One)

ý **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2014

o **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from to .
Commission file number 001-36076

FATE THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**65-1311552**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
3535 General Atomics Court, Suite 200, San Diego, CA	**92121**
(Address of principal executive offices)	(Zip Code)

(858) 875-1800
(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Name of each exchange on which registered
Common Stock, $0.001 par value	NASDAQ Global Market

Securities registered pursuant to Section 12(g) of the Act: **None**

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes o or No ý

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes o or No ý

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ý or No o

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Website, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§229.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ý No o

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ý

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer o	Accelerated filer ý	Non-accelerated filer o (Do not check if a smaller reporting company)	Smaller reporting company o

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Act). Yes o No ý

The aggregate market value of the common stock held by non-affiliates of the registrant was approximately $111,535,150 as of June 30, 2014 based upon the closing sale price on the NASDAQ Global Market reported for such date. Shares of common stock held by each executive officer and director and certain holders of more than 10% of the outstanding shares of the registrant's common stock have been excluded in that such persons may be deemed to be affiliates. Shares of common stock held by other persons, including certain other holders of more than 10% of the outstanding shares of common stock, have not been excluded in that such persons are not deemed to be affiliates. This determination of affiliate status is not necessarily a conclusive determination for other purposes.

The number of outstanding shares of the registrant's common stock, par value $0.001 per share, as of March 12, 2015 was 20,637,217.

INCORPORATION BY REFERENCE

Portions of the registrant's definitive proxy statement to be filed with the Securities and Exchange Commission, or SEC, pursuant to Regulation 14A in connection with the registrant's 2015 Annual Meeting of Stockholders, to be filed subsequent to the date hereof, are incorporated by reference into Part III of this annual report on Form 10-K. Such proxy statement will be filed with the SEC not later than 120 days after the conclusion of the registrant's fiscal year ended December 31, 2014.

Table of Contents

FATE THERAPEUTICS, INC.

Annual Report on Form 10-K

For the Fiscal Year Ended December 31, 2014

TABLE OF CONTENTS

Table of Contents

FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Such forward-looking statements, which represent our intent, belief or current expectations, involve risks and uncertainties and other factors that could cause actual results and the timing of certain events to differ materially from future results expressed or implied by such forward-looking statements. In some cases, you can identify forward-looking statements by terms such as "may," "will," "expect," "anticipate," "estimate," "intend," "plan," "predict," "potential," "believe," "should" and similar expressions. Forward-looking statements in this Annual Report on Form 10-K include, but are not limited to, statements about:

- the initiation, timing, progress and results of our preclinical and clinical studies, and our research and development programs;
- our ability to obtain and maintain regulatory approval of ProHema and any of our other future product candidates;
- our plans to research, develop and commercialize our product candidates;
- the performance of third parties in connection with the development and manufacture of our product candidates, including third parties conducting our clinical trials as well as third-party suppliers and manufacturers;
- our ability to develop sales and marketing capabilities, whether alone or with potential collaborators, to commercialize our product candidates, if approved;
- our ability to successfully commercialize our product candidates, if approved;
- the potential price and degree of market acceptance of our product candidates;
- the size and growth of the potential markets for our product candidates and our ability to serve those markets;
- regulatory developments and approval pathways in the United States and foreign countries for our product candidates;
- our ability, and the ability of our licensors, to obtain, maintain, defend and enforce intellectual property rights protecting our product candidates, and our ability to develop and commercialize our product candidates without infringing the proprietary rights of third parties;
- our ability to obtain funding for our operations;
- the accuracy of our estimates regarding revenues, expenses and capital requirements; and
- the additional risks and other factors described under the caption "Risk Factors" under Item 1A of this Annual Report on Form 10-K.

The cautionary statements made in this report are intended to be applicable to all related forward-looking statements wherever they may appear in this report. We urge you not to place undue reliance on these forward-looking statements, which speak only as of the date of this report. Except as required by law, we assume no obligation to update our forward-looking statements, even if new information becomes available in the future

In this Annual Report on Form 10-K, unless the context requires otherwise, "Fate Therapeutics," "Company," "we," "our," and "us" means Fate Therapeutics, Inc. and its subsidiaries.

Table of Contents

ITEM 1. Business

General Description of Our Business

We are a clinical-stage biopharmaceutical company engaged in the development of programmed cellular therapeutics for the treatment of severe, life-threatening diseases. We have built a novel platform to program the function and fate of cells *ex vivo* using pharmacologic modulators, such as small molecules. We are focused primarily on developing programmed hematopoietic cellular candidates as therapeutic entities for the treatment of hematologic malignancies, rare genetic disorders, and diseases resulting from the dysregulation of the immune system. We were incorporated in Delaware in 2007, and are headquartered in San Diego, CA.

Our Product Pipeline

The following table summarizes our programmed cellular therapeutic candidates currently in development and those currently in research:

Program	Therapeutic Target	Status
Development Programs		
ProHema *FT1050-modulated UCB*	Hematologic Malignancies	Phase 2 (adults) Phase 1b (pediatric)
ProHema *FT1050-modulated UCB*	Inherited Metabolic Disorders	Phase 1b (pediatric)
Programmed mPB *FT1050+FT4145-modulated mPB*	Hematologic Malignancies	IND enablement
Research Programs		
Programmed Hematopoietic Cells	Immune Regulation	Preclinical
hiPSC-derived Hematopoietic Cells	Not disclosed	Research
hiPSC-derived Myogenic Progenitor Cells	Muscle Regeneration	Research

"UCB" refers to hematopoietic cells within umbilical cord blood.

"mPB" refers to hematopoietic cells within mobilized peripheral blood.

"hiPSC" refers to human induced pluripotent stem cells.

Our Cell Programming Approach

The use of human cells as therapeutic entities has disease-transforming potential, and compelling evidence of medical benefit exists across a broad spectrum of severe, life-threatening diseases. One of the most successful and widespread applications of cellular therapeutics is within the setting of hematopoietic stem cell transplantation, or HSCT, with over 60,000 procedures performed worldwide on an annual basis. HSCT holds curative potential for patients afflicted with hematologic malignancies, such as leukemia and lymphoma, and with rare genetic disorders, such as inherited metabolic disorders and immune deficiencies.

Building upon this well-established medical precedent, the clinical investigation of isolated hematopoietic cells, such as CD34+ cells and T cells, as therapeutic entities for the treatment of human diseases is rapidly expanding. In fact, in the United States alone, over 1,200 clinical trials of hematopoietic cellular therapeutics are currently being conducted, including a growing number of trials with genetically-engineered hematopoietic cells. Many of these clinical trials are investigating potentially

Table of Contents

transformative uses of hematopoietic cellular therapeutics for the treatment of hematologic and solid malignancies, genetic disorders and immunological diseases.

While advancements in the isolation, expansion, manufacturing and engineering of hematopoietic cells have opened new avenues for their use as therapeutic entities, we believe the function of hematopoietic cells can be pharmacologically optimized to maximize therapeutic benefit. Since our founding, we have been dedicated to programming the function of cells *ex vivo* to improve their therapeutic potential. We have built a platform that enables us to systematically and precisely modulate *ex vivo* the biological properties of hematopoietic cells. Using advanced molecular characterization tools and technologies, we identify small molecule or biologic modulators that promote rapid and supra-physiologic activation or inhibition of therapeutically-relevant genes and cell-surface proteins, such as those involved in the homing, proliferation and survival of CD34+ cells or those involved in the persistence, proliferation and reactivity of T cells. We apply our deep understanding of the hematopoietic system to rapidly assess and quantify the therapeutic potential of programmed hematopoietic cells *in vivo*. Applying these capabilities in the settings of malignancies and rare genetic disorders, we aim to develop first-in-kind programmed hematopoietic cellular therapeutics with disease-transforming potential.

Additionally, we have worked closely with our scientific founders to pioneer the derivation and differentiation of induced pluripotent stem cells, or iPSCs, a potentially disruptive technology to program the fate of cells *ex vivo*. iPSCs are pluripotent cells that have been reprogrammed through the expression of certain genes and factors, such that the cell's cellular and physiological traits are similar to those of an embryonic stem cell. We use our technology to isolate, genetically engineer, select and characterize iPSCs, at a single-cell level, for clonal expansion. We believe our iPSC platform has the potential to create large quantities of homogeneous cell populations in the hematopoietic lineage, such as CD34+ cells, T cells and natural killer (NK) cells, which can otherwise be limited in quantity, difficult to manufacture, heterogeneous in composition and unoptimized for efficacy. Based on this potential, we believe our iPSC platform may enable the development of a novel class of transformative cellular therapeutics.

Our Strategy

We seek to develop and commercialize first-in-kind hematopoietic cellular therapeutics for the treatment of severe, life-threatening diseases based on our innovative cell programming approach. The key pillars of our strategy are to:

Efficiently develop and commercialize programmed hematopoietic cellular therapeutics addressing key unmet medical needs in allogeneic HSCT. While over one million HSCT procedures have been performed to date with curative intent, we believe hematopoietic cells administered to patients undergoing HSCT can be therapeutically optimized. Using our cell programming approach, we seek to modulate the biological properties of donor-derived CD34+ cells and T cells *ex vivo* to drive long-term therapeutic benefits *in vivo*. We believe our programmed hematopoietic cellular candidates may significantly improve the curative potential of allogeneic HSCT by addressing major complications that currently contribute to the high morbidity and mortality of the procedure, such as delayed neutrophil engraftment and immune reconstitution, viral infections and graft-versus-host disease, or GvHD. We are developing our product candidates across a wide range of patient ages and a broad spectrum of hematologic malignancies and rare genetic disorders, using cell sources most commonly used in HSCT including umbilical cord blood and mobilized peripheral blood. Due to the rare disease nature of our target indications, we believe any pivotal clinical trials which we conduct will generally require relatively small numbers of patients. Additionally, because HSCT is a highly-specialized procedure performed at a limited number of centers, we intend to build our own focused sales

Table of Contents

and marketing capabilities to commercialize in a cost-efficient manner any products that we may successfully develop.

Leverage our scientific, clinical and regulatory expertise to build and advance a pipeline of programmed hematopoietic cells as therapeutic entities beyond the allogeneic HSCT setting. Through the development of our initial product candidates, we have built a leadership position in the identification of pharmacologic modulators that promote rapid and supra-physiologic activation or suppression of therapeutically-relevant genes and cell-surface proteins on CD34+ cells, NK cells, and T cells. Additionally, we have built research, clinical and regulatory affairs teams that are experienced and skilled in the development of novel cellular therapeutics. We are leveraging this expertise to develop a product portfolio of programmed hematopoietic cellular therapeutics for severe, life-threatening diseases, and are currently investigating several attractive product opportunities including programmed CD34+ cells and programmed T cells for the regulation of the immune system. For example, using our screening platform, we have identified a combination of pharmacologic modulators which may enhance immuno-regulatory properties of CD34+ cells by upregulating the gene expression level of PD-L1, a key immunosuppressive protein, by more than 100 fold.

Selectively establish strategic research and development partnerships that tap our cell programming approach to maximize the therapeutic potential of hematopoietic cellular therapeutics. Over 1,200 clinical trials of hematopoietic cellular therapeutics are currently being conducted in the United States, which include ground-breaking approaches for the treatment of cancer, auto-immune diseases, degenerative diseases and genetic disorders. Most of these clinical trials use CD34+ cells or T cells, including genetically engineered cells, as therapeutic entities which have not been programmed *ex vivo* to optimize their therapeutic potential. Using our *ex vivo* cell programming approach, we believe we have the potential to enhance the *in vivo* homing, proliferation and immuno-regulatory potential of CD34+ cells or the *in vivo* persistence, proliferation and reactivity of T cells, among other properties, to maximize the therapeutic potential of hematopoietic cellular therapeutics. We seek to collaborate with other companies engaged in the development of hematopoietic cellular therapeutics, tapping our cell programming approach to optimize the therapeutic potential of product candidates.

Our Development Programs

We believe that *ex vivo* cell programming can positively affect the biological activity and therapeutic potential of cells *in viv*o, and that severe, life-threatening diseases can be addressed through the development of programmed hematopoietic cellular therapeutics. Our initial clinical product candidates are being developed as therapeutic entities for use in allogeneic HSCT.

Allogeneic HSCT is a well-established procedure that has been performed globally for decades with curative intent in patients with a wide range of hematologic malignancies and rare genetic disorders, including inherited metabolic, immune and blood disorders. The procedure involves transferring donor-derived hematopoietic cells, including hematopoietic stem cells (HSCs) and T cells, to a patient following the administration of chemotherapy and/or radiation therapy. The biological properties of donor-derived CD34+ cells, including HSCs, and T cells each play an essential role in determining outcomes of allogeneic HSCT. Donor-derived HSCs have the unique ability to engraft and reconstitute a new blood and immune system, and donor-derived T cells have an important protective role following a transplant in eliminating residual cancer cells and providing protection against life-threatening infections. The engraftment of donor-derived HSCs is essential for successful reconstitution, and any delay or failure of HSC engraftment leaves a patient severely immuno-compromised and exposed to exceedingly high risk of early morbidity and mortality. Additionally, while the donor-derived T cells impart a critical immunotherapeutic effect, alloreactive T cells can result in a serious complication known as GvHD, where donor-derived T cells recognize antigens on patient's cells as foreign and attack the patient's cells.

Table of Contents

The number of HSCT procedures has increased steadily over the past two decades more than 20,000 allogeneic HSCTs are performed annually worldwide. For most patients undergoing allogeneic HSCT, the procedure represents the only remaining therapeutic option available to achieve long-term disease-free remission and/or a functional cure. Disease-free survival rates of approximately 40-50% at two and five-years following HSCT have been reported in multi-center clinical experiences for the treatment of hematologic malignancies. The highest risk of relapse or death occurs during the initial months following the procedure, where the rate of relapse and non-relapse mortality is approximately 30-40% at six-months following HSCT.

Programmed Umbilical Cord Blood for Allogeneic HSCT (ProHema)

Our lead product candidate, ProHema, is an *ex vivo* programmed hematopoietic cellular therapeutic derived from umbilical cord blood. ProHema is produced by programming the biological properties of CD34+ cells and T cells of umbilical cord blood *ex vivo* using the small molecule modulator FT1050 (16,16 dimethyl prostaglandin E2, or dmPGE2). Our proprietary modulation process induces rapid activation of genes involved in the homing, proliferation and survival of HSCs and in the cell cycle, reactivity and anti-viral properties of T cells.

Prostaglandin E2, or PGE2, was first identified in 2007 as a potent regulator of hematopoiesis by one of our scientific founders, Dr. Leonard Zon of The Children's Hospital Boston. Using a pioneering chemical screening approach in zebrafish embryo, Dr. Zon identified a number of small molecules that regulate processes involved in the development of the hematopoietic system. Dr. Zon subsequently showed that CD34+ cells modulated with dmPGE2 out-compete unmodulated CD34+ cells and preferentially reconstitute the hematopoietic system in a preclinical model of competitive HSCT.

We are developing ProHema to enable the curative potential of HSCT in patients across a wide range of ages and a broad spectrum of life-threatening malignant diseases and rare genetic disorders. The United States Food and Drug Administration (FDA) has granted orphan designation for ProHema for the enhancement of stem cell engraftment to treat neutropenia, thrombocytopenia, lymphopenia and anemia, and the European Commission has granted orphan designation for ProHema for the treatment of acute myeloid leukemia.

Adult Patients with Hematologic Malignancies

Our Phase 2 PUMA Study. We are currently conducting a randomized, controlled, open-label Phase 2 multi-center clinical trial of ProHema in adult subjects undergoing double umbilical cord blood transplantation (dUCBT) for the treatment of hematologic malignancies including acute lymphoblastic leukemia, acute myelogenous leukemia and non-Hodgkin lymphoma, a clinical trial which we refer to as the PUMA (*P*roHema in *UM*bilical cord blood transplant in *A*dults) study. The PUMA study is designed to enroll 60 subjects, age 15 to 65 years, and is currently being conducted at 11 leading allogeneic HSCT centers in the United States. Eligible subjects are being randomized, at a ratio of 2:1, with approximately 40 subjects expected to receive ProHema plus an unmanipulated cord blood unit, and approximately 20 concurrent control subjects expected to receive a standard dUCBT. Based upon physician choice, subjects are being treated with one of two conditioning regimens, an intense myeloablative regimen (MAC) or a reduced-intensity regimen (RIC), to destroy malignant cells and to prevent rejection of the donor hematopoietic cells. Randomization is being stratified by conditioning regimen. An independent Data Monitoring Committee (iDMC) is providing safety oversight during the conduct of the PUMA study. We expect data on the primary efficacy endpoint from the Phase 2 PUMA study to be available in the second half of 2015.

The PUMA study is our first clinical investigation of ProHema where the CD34+ cells and T cells of umbilical cord blood are being programmed in a nutrient-rich media, which we refer to as our NRM formulation. Our prior clinical investigations of ProHema utilized a nutrient-free standard cell

Table of Contents

processing media for cell programming, a media which is commonly used throughout the HSCT setting today for the thawing and washing of umbilical cord blood units. We believe, based on a series of preclinical assessments, that the clinical potency and efficacy profile of ProHema may be significantly improved by programming CD34+ cells and T cells in our NRM formulation.

Multiple clinical endpoints that contribute significantly to the overall morbidity and mortality of allogeneic HSCT are being evaluated in the PUMA study. These clinical endpoints include key measures of the hematopoietic reconstitution and immunotherapeutic potential of ProHema, including time to and incidence of neutrophil and platelet engraftment, rates of engraftment failure, bacterial infections, viral reactivation, GvHD, relapse of underlying disease and overall and disease-free survival. The primary endpoint of the PUMA study is based on a categorical analysis of neutrophil engraftment, and the clinical trial is powered to show with statistical significance that 70% of subjects with neutrophil engraftment in the ProHema treatment arm engraft prior to a pre-specified control day of neutrophil engraftment, which has been established as 26 days for subjects receiving MAC and 21 days for subjects receiving RIC. Complications from delayed or failed neutrophil engraftment following dUCBT are a leading contributor to non-relapse mortality, the risk of which increases several-fold in patients failing to achieve early neutrophil engraftment.

We initiated enrollment of our PUMA study in March 2014. In December 2014, the PUMA study's iDMC conducted a pre-planned interim safety review. A total of 12 subjects that received ProHema were included in the interim review, which assessed safety, time to engraftment, rates of engraftment failure, infection, GvHD and early mortality. These initial data showed that subjects administered ProHema had an improved median time of neutrophil engraftment and an increased incidence of early neutrophil engraftment, as compared to the pre-specified control values of engraftment. Specifically, eight of 10 ProHema subjects receiving MAC achieved neutrophil engraftment, with a median time to engraftment of 20 days, and one of two ProHema subjects receiving RIC achieved neutrophil engraftment on Day 14. Six of the nine engrafting subjects administered ProHema achieved neutrophil engraftment prior to the applicable pre-specified control value of engraftment. Two early deaths prior to engraftment, which were both attributed to the toxicity of the conditioning regimen received by the subjects, were reported in the ProHema arm, and one subject administered ProHema failed to achieve neutrophil engraftment. Based on its consideration of the data available as well as historical outcomes reported from multi-center clinical experiences, the iDMC determined that ProHema had met established safety criteria and the nature and frequency of the adverse events did not show harm and was consistent with this patient population, and supported continuation of the PUMA study.

The pre-specified control values of engraftment are based on multi-center reports published in the literature of historical median times to neutrophil engraftment in adult patients undergoing dUCBT in the United States. We plan to utilize the data from the concurrent control subjects in the PUMA study to provide context for validating the pre-specified control values of engraftment and for interpreting other clinical outcomes. As there is no substantive difference in eligibility or in treatment course between the concurrent control arms of our PUMA study and our initial Phase 2 ProHema-03 study described below, our assessment of the concurrent control subjects will include approximately 20 subjects from the concurrent control arm of the PUMA study and the three subjects from the concurrent control arm of the ProHema-03 study.

If our PUMA trial is successful, we plan to seek additional regulatory guidance with the goal of initiating a registrational trial of ProHema, which may include both adult and pediatric patients undergoing UCBT for hematologic malignancies. Based on the initial regulatory guidance obtained to date, and preliminary statistical power calculations, we believe that a registrational program could consist of a single trial enrolling approximately 200 patients, with time to engraftment of neutrophils, platelets or both, as the primary endpoint to support approval, and that a single trial enrolling both adult and pediatric subjects may be sufficient for approval across both age groups, depending on the results.

Table of Contents

Our ProHema-03 Study. In December 2012, we initiated a randomized, controlled, open-label Phase 2 multi-center clinical trial of ProHema in adult subjects undergoing dUCBT for the treatment of hematologic malignancies, a clinical trial which we refer to as our ProHema-03 study. The ProHema-03 study had the same design, subject population, inclusion criteria, conditioning regimens and schedule as the PUMA study, but used a nutrient-free standard cell processing media for the programming of CD34+ cells and T cells of umbilical cord blood. Eight subjects received ProHema plus an unmanipulated cord blood unit and three concurrent control subjects received a standard dUCBT. All subjects were conditioned using a MAC regimen. Seven of eight ProHema subjects achieved neutrophil engraftment, with a median time of engraftment of 28 days, and one subject failed to achieve neutrophil engraftment. All three concurrent control subjects achieved neutrophil engraftment, with a median time of engraftment of 31 days.

We have continued to follow the subjects in the ProHema-03 study, and we intend to follow such subjects for the two-year period following HSCT. The one-year disease-free survival rate in the ProHema arm was 50%, as compared to 33.3% in the concurrent control arm, and the one-year overall survival rate in the ProHema arm was 50%, as compared to 33.3% in the concurrent control arm. As of January 31, 2015, there was no change in disease-free or overall survival rates from those reported at one-year following HSCT. Additionally, as of January 31, 2015, there are no reports of any subjects experiencing secondary graft failure; one subject in the ProHema arm and one subject in the concurrent control arm experienced Grade III acute GvHD, and one subject in the ProHema arm experienced Grade IV acute GvHD. Adverse events attributed to ProHema were primarily limited to common infusion-related side effects.

In May 2013, we elected to pause enrollment in our ProHema-03 study, and we notified the FDA of our intent to generate data qualifying an optimized manufacturing process for ProHema using our NRM formulation. In August 2013, we submitted to the FDA an amendment to our Investigational New Drug (IND) application and an amended protocol defining how we planned to resume our Phase 2 clinical investigation of ProHema using our NRM formulation. Specifically, we stated that we planned to enroll approximately 40 subjects using our NRM formulation for the manufacture of ProHema. In March 2014, we submitted to the FDA manufacturing and product data incorporating our NRM formulation for the manufacture of ProHema, and we commenced enrollment of our Phase 2 PUMA study.

Our ProHema-01 Study. In September 2011, we completed a controlled, open-label Phase 1b clinical trial of ProHema in adult subjects undergoing dUCBT for the treatment of hematologic malignancies, a clinical trial which we refer to as our ProHema-01 study. All subjects were conditioned using a RIC regimen, and a nutrient-free standard cell processing media was utilized for the programming of CD34+ cells and T cells of umbilical cord blood.

The ProHema-01 trial consisted of two cohorts of patients with acute leukemia, non-Hodgkin lymphoma and myelodysplastic syndrome: (1) an inactive cohort of nine subjects who received an unmanipulated cord blood unit and a cord blood unit modulated with FT1050 under biologically inactive conditions; and (2) the ProHema cohort of 12 subjects who received ProHema and an unmanipulated cord blood unit. The trial was conducted at the Dana Farber Cancer Institute and the Massachusetts General Hospital, and the results were compared against patient outcomes from a then-current historical control group of 53 adult patients with hematologic malignancies undergoing dUCBT with the same conditioning regimen at these same institutions.

The primary objective was to evaluate the safety of allogeneic HSCT using ProHema plus an unmanipulated cord blood unit. Secondary objectives of the trial included the assessment of time to

Table of Contents

engraftment and 100-day survival. We observed the following potential clinical benefits in our ProHema-01 trial:

- The ProHema cohort exhibited a statistically-significant improvement in time to neutrophil engraftment as compared to the historical control group (p=0.043);
- The disease-free survival rate at Day 100 following HSCT was 100% in the ProHema cohort, as compared to 76% in the historical control group;
- The cumulative incidence of neutrophil engraftment and the cumulative incidence of platelet engraftment in the ProHema cohort compared favorably to both the inactive cohort and the historical control group; and
- Cytomegalovirus (CMV) reactivation occurred in only two of 12 subjects (17%) in the ProHema cohort during the one-year period following HSCT, which compares favorably to rates of CMV reactivation reported in the literature.

The following table shows the results observed in the ProHema-01 trial with respect to the key measures of time to engraftment and rate of failure to achieve neutrophil engraftment:

Cohort	Median Time to Engraftment	Rate of Failure to Achieve Neutrophil Engraftment
ProHema (n=12)	17.5 days [range 14 - 31 days]	0%
Inactive (n=9)	22.0 days [range 14 - 40 days]	11%
Historical (n=53)	20.5 days [range 13 - 70 days]	6%

We also evaluated the incidence of GvHD and observed, during the first 100-days following HSCT, there was an 8% incidence of Grade II-IV acute GvHD in the ProHema cohort, as compared to 17% in the historical control group. One subject in the ProHema cohort experienced mild chronic GvHD. The trial met all established safety criteria and demonstrated that ProHema was well tolerated. Adverse events attributed to ProHema consisted of mild to moderate infusion-related events consisting of rash, nausea, chills, flushing, abdominal pain, and cough, all of which are considered common transplant-related side effects. One subject with known coronary artery disease experienced transient myocardial ischemia that resolved promptly after completion of the infusion.

We followed all subjects in the ProHema cohort for a two-year period following HSCT in accordance with the study protocol, at which time the study was concluded. During the two-year period following HSCT, there were no reports of any subject in the ProHema cohort experiencing secondary graft failure. In addition, the one-year and two-year disease-free survival rates in the ProHema cohort were 58.3% and 41.7%, respectively. The corresponding one and two-year overall survival rates in the ProHema cohort were 75.0% and 58.3%, respectively.

Additionally, a retrospective analysis of the T cell compartment of subjects from our ProHema-01 study was conducted by the clinical investigators. The assessment revealed that, at Day 100 following HSCT, subjects who received ProHema showed a two-fold increase in the percentage of naïve and early memory T cell fraction within the CD8+ T cell compartment, as compared to subjects who received two unmanipulated cord blood units. Naïve and early memory CD8+ T cell populations are believed to play a key role in promoting immune reconstitution and viral immunity following HSCT. Consistent with these reported immuno-modulatory effects on CD8+ T cells, low rates of viral reactivation were observed in our ProHema-01 study. We believe these findings suggest that *ex vivo* programming using

Table of Contents

FT1050 may also enhance the immuno-modulatory properties of T cells, and promote viral immunity and immune reconstitution following HSCT.

Pediatric Patients with Rare Genetic Disorders

The transformative effect of allogeneic HSCT, and umbilical cord blood transplantation in particular, across a broad spectrum of rare genetic disorders has been demonstrated and published in numerous clinical studies, case series and retrospective analyses of multi-national patient registries. It is estimated that over 50 rare genetic disorders, many of which are life-threatening and lack alternative therapeutic options, have been treated with allogeneic HSCT to date, including lysosomal storage disorders, such as Hurler syndrome, Krabbe disease and metachromatic leukodystrophy; peroxisomal storage disorders, such as adrenoleukodystrophy; hemoglobinopathies, such as sickle cell disease and certain thalassemias; inherited bone marrow failure syndromes, such as Fanconi anemia and Diamond-Blackfan anemia; and inherited immune deficiencies, such as Wiskott-Aldrich syndrome. Since allogeneic hematopoietic cells are sourced from healthy donors, we believe our product candidates have the inherent potential to correct genetic defects across a wide range of rare genetic disorders, whether they are caused by defective genes encoding enzymes, hemoglobin or other essential proteins.

Inherited metabolic disorders, or IMDs, 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 traditional enzyme replacement therapy. Long-term follow up of children with LSDs and peroxisomal storage disorders who underwent allogeneic HSCT has shown that the progressive worsening of many clinical manifestations can be prevented or substantially reduced through early allogeneic HSCT intervention. These effects have been attributed to the ability of donor-derived HSCs to home to and engraft within the central nervous system (CNS), where they give rise to microglia cells that become a permanent source of enzyme supply through a process called cross-correction.

We believe the programming of CD34+ cells has the potential to significantly improve the homing of donor-derived cells across the blood-brain barrier, arresting degenerative neurological manifestations and improving the course of disease progression in pediatric patients with rare genetic disorders, such as IMDs. We have demonstrated in sub-lethally irradiated NSG mice that the modulation of human CD34+ cells with FT1050, as compared to unmanipulated CD34+ cells, significantly increases the number of human cells that home to and migrate across the blood-brain barrier into the CNS at 20 hours following administration. Additionally, in *in vivo* murine models of allogeneic HSCT, we have demonstrated that the use of FT1050-programmed donor CD34+ cells, as compared to unmanipulated donor CD34+ cells, led to a statistically-significant increase both in the engraftment of donor CD34+ cells (p=0.008) and in the donor-derived expression of iduronidase, the gene that is defective in patients with Hurler syndrome, in the brain (p=0.018) at eight weeks following administration.

CNS Engraftment **Enzyme mRNA in CNS**

Table of Contents

Our Phase 1b PROVIDE Study. During the first half of 2015, we plan to initiate an open-label Phase 1b multi-center clinical trial of ProHema in pediatric subjects undergoing single umbilical cord blood transplantation (sUCBT) for the treatment of IMDs, a clinical trial which we refer to as the PROVIDE (*PRO*Hema e*V*aluation for the treatment of *I*nherited metabolic *D*isord*E*rs) study. The PROVIDE trial is designed to enroll up to 12 subjects with various forms of IMDs, between the ages of 1 and 18, at up to three leading pediatric HSCT centers in the United States. The study inclusion criteria allow for the enrollment of pediatric subjects with sixteen different types of IMDs, including Hurler and Hunter syndromes, Krabbe disease and various other leukodystrophies, among others. While the primary endpoint of the PROVIDE study is safety as assessed by neutrophil engraftment, we plan to follow subjects for a two-year period following HSCT and regularly conduct a series of neuro-imaging and neuro-cognitive assessments to explore the potential of the programmed hematopoietic cells to provide long-term replacement of the otherwise deficient enzyme to the CNS. Subject to commencing enrollment in accordance with our plans, we expect to report initial topline data from our PROVIDE study in 2015.

Pediatric Patients with Hematologic Malignancies

Each year, over 3,500 children in the United States are diagnosed with leukemia, many of whom may ultimately require HSCT. For pediatric patients, the standard of care in umbilical cord blood transplantation for the treatment of hematologic malignancies utilizes a single cord blood unit. While the cell dose received by a pediatric patient from a single cord blood unit can be sufficient, data suggest that pediatric patients undergoing sUCBT are at high risk for experiencing delayed engraftment, graft failure and transplant-related morbidity and mortality.

Our Phase 1b PROMPT Study. In April 2014, the FDA permitted our IND amendment to go into effect for the clinical development of ProHema using our NRM formulation in pediatric patients undergoing sUCBT following myeloablative conditioning for the treatment of various hematologic malignancies, such as acute lymphoblastic leukemia and acute myeloid leukemia, a clinical trial which we refer to as the PROMPT (*PRO*Hema for the treatment of hematologic *M*alignancies in *P*edia*T*ric patients) study. The PROMPT study is designed to enroll up to 18 subjects, between the ages of 1 and 18, at three leading pediatric HSCT centers in the United States. The primary endpoint of the PROMPT study is safety as assessed by neutrophil engraftment. The study will also evaluate various parameters of efficacy, including additional measures of neutrophil engraftment, platelet engraftment, rates of engraftment failure, GvHD, serious infections, and disease-free and overall survival. We are currently screening subjects for enrollment in our Phase 1b PROMPT study, and data on the primary endpoint is expected in the second half of 2015.

Our ProHema-02 Study. Our decision to conduct a Phase 1b clinical trial of ProHema in pediatric subjects undergoing sUCBT for hematologic malignancies was supported by a Phase 1 clinical trial that we conducted to determine safety in the setting of sUCBT in adult subjects with hematologic malignancies, a clinical trial which we refer to as the ProHema-02 trial. Qualifying subjects received a single ProHema cord blood unit following reduced-intensity conditioning. The primary endpoint of the trial was safety, and we analyzed a range of engraftment measures as well as rates of GvHD, relapse and survival. Of the eight subjects enrolled, six subjects, ages 39-63 years (median 43.5 years), were evaluable. Four of the six evaluable subjects engrafted at Days 17, 19, 22 and 37, and two subjects experienced primary graft failure. We followed all evaluable subjects for a one-year period following HSCT, at which time the study was concluded. During the one-year period following HSCT, there were no reports of any subject experiencing secondary graft failure. All four engrafting subjects were alive at Day 100, and two of the four engrafting subject were alive at one year, following HSCT. There were no reported incidents of acute or chronic GvHD. Adverse events attributed to ProHema were limited to common transplant-related side effects.

Table of Contents

Programmed Mobilized Peripheral Blood for Allogeneic HSCT

Mobilized peripheral blood (mPB) is the predominant cell source used in HSCT. While the use of mPB is associated with faster rates of neutrophil and platelet engraftment compared to other cell sources, approximately 35-50% of patients develop severe viral infections, such as CMV infection, within the first 100 days following HSCT and approximately 50% of patients develop acute GvHD within the first 180 days following HSCT. We believe our cell programming approach has the potential to mitigate these T cell-mediated complications and improve outcomes in patients undergoing HSCT with mPB as a cell source.

At the 56th Annual Meeting and Exposition of the American Society of Hematology in December 2014, we presented data showing that a newly-identified small molecule modulator, referred to as FT4145, synergizes with FT1050 to promote the supra-physiologic activation of genes implicated in the cell cycle, immune tolerance and anti-viral properties of T cells, as well as in the survival, proliferation and engraftment potential of CD34+ cells. Specifically, the programming of CD34+ cells with FT1050 and FT4145 resulted in a 60-fold increase in CXCR4 gene expression levels and a statistically-significant increase in engraftment as compared to unmodulated cells. Additionally, T cells programmed with FT1050 and FT4145 were found to have a 66% reduction of cell-surface protein expression of ICOS, a key T cell activation marker, and a statistically-significant reduction in proliferation rates as compared to unmodulated cells. We are currently preparing for an IND application for FT1050-FT4145 programmed mobilized peripheral blood, which we plan to submit to the FDA in 2015, to support the initiation of a clinical trial to assess our programmed mobilized peripheral blood candidate in adult subjects undergoing allogeneic HSCT for the treatment of hematologic malignancies.

Nutrient-Rich Media Formulation

We have incorporated our NRM formulation into all of our clinical development programs for ProHema, including our PUMA, PROMPT and PROVIDE studies. In the conduct of our ProHema-01, ProHema-02 and ProHema-03 clinical trials of ProHema, we utilized a nutrient-free standard cell processing media for cell programming, a media which is commonly used throughout the HSCT setting today for the thawing and washing of umbilical cord blood units. During the second quarter of 2013, we completed *in vitro* and animal studies demonstrating that the clinical potency and efficacy profile of ProHema may be significantly improved by programming the biological properties of CD34+ cells and T cells of umbilical cord blood in a nutrient-rich processing media. Using our NRM formulation, as compared to the use of nutrient-free standard cell processing media, we have shown that CD34+ cells programmed with FT1050 had an 8-fold increase in CXCR4 gene expression and a statistically significant increase in cell-surface protein expression of CXCR4, a key receptor implicated in the homing of HSCs to the bone marrow niche ($p<0.05$); and *ex vivo* programmed CD34+ cells exhibited a more than two-fold improvement in HSC engraftment at 12-weeks post-transplant in a xenograft mouse study ($p=0.0005$). We believe that the clinical potency and efficacy profile of ProHema may be significantly improved by programming CD34+ cells and T cells in our NRM formulation.

Our Research Programs

We seek to leverage our scientific, clinical and regulatory expertise with ProHema to build a pipeline of programmed CD34+ cells and programmed T cells as therapeutic entities for use beyond the HSCT setting. We have built a leadership position in the identification of pharmacologic modulators, including combinations of modulators, that promote rapid and supra-physiologic activation or inhibition of therapeutically-relevant genes and cell-surface proteins on CD34+ cells and T cells. Additionally, our patent-protected iPSC technology allows us to engineer and program the fate of cells *ex vivo*, and we have demonstrated the potential to create large quantities of homogeneous cell populations, including hematopoietic cells and myogenic progenitor cells, that can otherwise be limited in quantity, difficult to manufacture, heterogeneous in composition and unoptimized for efficacy.

Table of Contents

Programmed Hematopoietic Cells

We are currently investigating several attractive opportunities for programmed hematopoietic cellular candidates with disease-transforming potential, including programmed CD34+ cells and programmed T cells for the regulation of the immune system. Using our screening platform, we have identified a triple modulator combination of pharmacologic modulators that programs human CD34+ cells to express high levels of PD-L1, a key immunosuppressive protein. The role of the PD-1/PD-L1 pathway is being explored in the field of cancer immunotherapy and the recent clinical success of PD-1 checkpoint inhibitors to dramatically enhance the ability of T cells to eliminate cancer cells provides support for the potent immunosuppressive potential of PD-L1 expression. We are exploiting PD-L1 expression to limit the activity of activated T cells arising from an inflammatory or auto-immune response. Using a combination of three modulators, we have increased by greater than 100-fold the gene expression levels of PD-L1 on CD34+ cells during a transient ex vivo modulation. Additionally, CD34+ cells programmed with the three-modulator combination have been shown to significantly reduce the proliferation rates of activated T-cells using in vitro assays, as compared to unmodulated HSCs. The Company is currently investigating the *in vivo* therapeutic potential of PD-L1 programmed CD34+ cells to selectively home to sites of, and suppress, T cell proliferation and cytokine production. We aim to nominate an additional programmed hematopoietic cellular candidate for further development in 2015.

iPSC-derived Cellular Therapeutics

We believe iPSC technology has the potential to enable the next frontier in the development of cellular therapeutics. The seminal discovery that it is possible to reprogram the fate of fully-differentiated human cells *ex vivo* through the expression of certain genes and factors, such that the reprogrammed cell's cellular and physiological traits are similar to those of an embryonic stem cell, is one of the most remarkable scientific breakthroughs of the past decade and was recognized with the 2012 Nobel Prize in Science and Medicine. The advent of iPSCs, with their capacity to be cultured and expanded indefinitely *in vitro* and to serve as a potentially unlimited cell source for differentiation into specialized cell types, introduces a new and potentially disruptive strategy for modeling human disease and developing innovative cellular therapeutics.

In collaboration with two of our Scientific Founders, Dr. Rudolf Jaenisch of the Whitehead Institute for Biomedical Research and Dr. Sheng Ding of the Gladstone Institute at UCSF, we have developed a proprietary, small molecule-enhanced iPSC platform. We believe our iPSC platform can enable the development of entirely new classes of autologous, allogeneic, and genome-edited cellular therapeutics with disease-transforming potential. Our patent-protected iPSC technology enables the isolation, genetic-engineering, selection and characterization of pluripotent cells, at the single-cell level, for clonal expansion. Additionally, we have demonstrated the potential to create large quantities of homogenous cell populations in the hematopoietic lineage, such as CD34+ cells, T cells and NK cells, which can otherwise be limited in quantity, difficult to manufacture, heterogeneous in composition and unoptimized for efficacy. We are currently applying our iPSC platform to the research and development of iPSC-derived cellular therapeutics for the treatment of hematologic, immunologic and skeletal muscle diseases and disorders.

Our Intellectual Property

Overview

We seek to protect our product candidates and our cell programming technology through a variety of methods, including seeking and maintaining patents intended to cover our products and compositions, their methods of use and processes for their manufacture, our platform technologies and any other inventions that are commercially important to the development of our business. We seek to

Table of Contents

obtain domestic and international patent protection and, in addition to filing and prosecuting patent applications in the United States, we typically file counterpart patent applications in additional countries where we believe such foreign filing is likely to be beneficial, including Europe, Japan, Canada, Australia and China. We continually assess and refine our intellectual property strategy in order to best fortify our position, and we are prepared to file additional patent applications if our intellectual property strategy warrants such filings. We also rely on know-how, continuing technological innovation and in-licensing opportunities to develop and maintain our proprietary position. We have entered into exclusive license agreements with various academic and research institutions to obtain the rights to use certain patents for the development and commercialization of our product candidates.

As of March 6, 2015, our intellectual property portfolio is currently composed of 107 issued patents, 148 patent applications that we license from academic and research institutions and 53 patent applications that we own. These patents and patent applications generally provide us with the rights to develop our product candidates in the United States and worldwide. This portfolio covers our product candidates, including ProHema, our cell programming approach and our iPSC technology. We believe that we have a significant intellectual property position and substantial know-how relating to the programming of hematopoietic cells and to iPSC technology.

We cannot be sure that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications we may own or license in the future, nor can we be sure that any of our existing patents or any patents we may own or license in the future will be useful in protecting our technology. Please see "Risk Factors Risks Related to Our Intellectual Property" for additional information on the risks associated with our intellectual property strategy and portfolio.

Intellectual Property Relating to the Programming of Hematopoietic Cells

As of March 6, 2015, we own eight families of pending U.S. and foreign patent applications covering the programming of hematopoietic cells. This portfolio includes 24 pending applications relating to ProHema and other therapeutic compositions of hematopoietic cells that have been pharmacologically-modulated to enhance their therapeutic properties, and methods of manufacturing their cellular compositions. Applications in this portfolio include claims covering (i) therapeutic compositions of human hematopoietic cells that have been programmed *ex vivo* with one or more agents, such as a prostaglandin agonist, to guide their fate and optimize their therapeutic function *in vivo* and (ii) methods of improving HSCT and methods of treating patients requiring hematopoietic reconstitution, as well as disclosures of methods for preparing cell populations for HSCT. Our portfolio also includes applications relating to cell culture media, including our NRM formulation, for improved processing and programming of cells *ex vivo* and a cell potency assay for rapidly assessing and quantifying the biological function and therapeutic potential of programmed cell populations. Any U.S. patents issued from these applications will have statutory expiration dates between 2030 and 2034.

Additionally, we have an exclusive license to an intellectual property portfolio consisting of two families of issued patents and pending patent applications co-owned by the Children's Medical Center Corporation and The General Hospital Corporation. As of March 6, 2015, we currently have exclusive rights to 20 issued patents and 23 pending patent applications in the United States and worldwide relating to methods for promoting tissue growth or regeneration (including the reconstitution of the hematopoietic system) using modulators that up-regulate the prostaglandin signaling pathway or its downstream mediators. These patent rights consist of issued U.S. patents (including U.S. Patents 8,168,428 and 8,563,310) claiming methods for promoting HSC engraftment and reconstitution through the *ex vivo* modulation of HSCs using FT1050, including HSCs obtained from cryopreserved cord blood, bone marrow and mobilized peripheral blood. Pending applications in the United States and foreign jurisdictions are directed to therapeutic compositions of HSCs derived from cord blood, wherein the cells have been modulated by increasing prostaglandin activity, methods of preparing these

Table of Contents

compositions, and methods of promoting hematopoietic reconstitution, expansion and self-renewal using modulators that increase prostaglandin signaling activity. Any patents within this portfolio that have issued or may yet issue will have a statutory expiration date in 2027.

We have also licensed exclusive rights to two families of patent applications from the Indiana University Research and Technology Corporation claiming methods of enhancing HSCT procedures by altering prostaglandin activity in HSCs and methods of enhancing viral transduction efficiency in the genetic engineering of stem cells including HSCs. These applications describe methods of increasing mobilization of stem cells from a stem cell donor, and methods for increasing HSC homing and engraftment in a stem cell transplant recipient. One family of applications is directed to preferentially modulating certain receptors present on HSCs to increase the therapeutic potential of such cells for homing and engraftment. Claims in these applications specifically cover the modulation of umbilical cord blood by altering prostaglandin activity and methods for increasing viral transduction efficiency for gene therapy. These applications are currently pending in the United States and in certain foreign jurisdictions, and U.S. patents, if issued, from the applications could have terms expiring in 2029 or 2030.