BioTime Initiates Dosing in Phase I/IIa Clinical Study of OpRegen for Treatment of Dry-AMD Utilizing Orbit Subretinal Delivery System

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ALAMEDA, Calif.--(BUSINESS WIRE)--Jul. 11, 2019-- BioTime, Inc. (NYSE American and TASE: BTX), a clinical-stage biotechnology company developing cellular therapies for unmet medical needs, today announced that it has dosed its first patient with the Orbit Subretinal Delivery System (Orbit SDS) as well as with a new Thaw-and-Inject formulation of OpRegen®, the Company's retinal pigment epithelium (RPE) transplant therapy, in its ongoing Phase I/IIa clinical study for the treatment of dry age-related macular degeneration, a leading cause of adult blindness in the developed world.

"We are excited to be evaluating the FDA-cleared Orbit SDS in the next six patients of our Phase I/IIa study," stated Brian M. Culley, Chief Executive Officer of BioTime. "We believe that precise administration of OpRegen cells to the back of the eye utilizing the Orbit SDS will lead to better dose control and an overall safer procedure, with an increased likelihood of positive clinical outcomes. In preparation for potential future commercialization, we also are introducing our new Thaw-and-Inject formulation, enabling rapid off-the-shelf administration of RPE cells upon thawing, which we believe will streamline the use of OpRegen by retinal surgeons."

The primary objective of the Phase I/IIa study is to evaluate the safety and tolerability of OpRegen as assessed by the incidence and frequency of treatment emergent adverse events (AEs). Secondary objectives are to evaluate the preliminary efficacy of OpRegen treatment by assessing the changes in ophthalmological parameters measured by various methods of primary clinical relevance. Additionally, for the patients in Cohort 4 that receive subretinal delivery of OpRegen utilizing the Orbit SDS, objectives will include the evaluation of the safety of delivery of OpRegen using the Orbit SDS.

About the Phase I/IIa Clinical Study

This is a Phase I/IIa open-label, dose escalation safety and efficacy study of human embryonic stem cell-derived retinal pigment epithelium cells transplanted subretinally in patients with advanced dry age-related macular degeneration with geographic atrophy. The study will enrollapproximately 24 subjects, divided into 4 cohorts. The first 2 cohorts, each consisting of 3 legally blind subjects with best corrected visual acuity (BCVA) of 20/200 or less, received a single subretinal implantation of OpRegen. The third cohort included 6 subjects with BCVA of 20/200 or less, who received a single subretinal implantation of OpRegen. Staggered intervals within and between cohorts are applied to ensure subject safety and welfare. The fourth cohort will include approximately 12 subjects with BCVA between 20/64 and 20/250, who will receive a single subretinal implantation of OpRegen. Cohort 4 includes two formulations of OpRegen; the first 3 subjects were treated with an ophthalmic Balanced Salt Solution Plus (BSS Plus) version of OpRegen. Remaining subjects will be treated with an "off-the-shelf" or "thaw and inject" (TAI) formulation. OpRegen TAI can be shipped directly to sites and used upon thawing, which removes the complications and logistics of having to use a dose preparation facility. Staggered intervals will be applied between at least the first two subjects of each delivery modality to ensure subject safety and welfare.

About OpRegen®

OpRegen is a retinal pigment epithelium (RPE) transplant therapy in Phase I/IIa development for the treatment of dry-AMD, a leading cause of adult blindness in the developed world. OpRegen consists of a suspension of RPE cells delivered subretinally as an intraocular injection. RPE cells are essential components of the back lining of the retina and function to help nourish the retina including photoreceptors. OpRegen has been granted Fast Track designation from the U.S. Food and Drug Administration. OpRegen is a registered trademark of Cell Cure Neurosciences Ltd., a majority-owned subsidiary of BioTime, Inc.

About BioTime, Inc.

BioTime is a clinical-stage biotechnology company developing new cellular therapies for unmet medical needs. BioTime's programs are based on its proprietary cell-based therapy platform and associated development and manufacturing capabilities. With this platform BioTime develops and manufactures specialized, terminally-differentiated human cells from its pluripotent and progenitor cell starting materials. These differentiated cells are developed either to replace or support cells that are dysfunctional or absent due to degenerative disease or traumatic injury or administered as a means of helping the body mount an effective immune response to cancer. BioTime's clinical assets include (i) OpRegen ®, a retinal pigment epithelium transplant therapy in Phase I/IIa development for the treatment of dry age-related macular degeneration, a leading cause of blindness in the developed world; (ii) OPC1, an oligodendrocyte progenitor cell therapy in Phase I/IIa development for the treatment of acute spinal cord injuries; and (iii) VAC2, an allogeneic cancer immunotherapy of antigen-presenting dendritic cells currently in Phase I development for the treatment of non-small cell lung cancer. For more information, please visit www.biotimeinc.com or follow the Company on Twitter @BioTimeBTX.

About the Orbit Biomedical Subretinal Delivery System

The Orbit Biomedical Surgical Delivery System (Orbit SDS) was designed to help surgeons deliver therapies to the retina with precision and accuracy. To deliver a therapy to the retina a surgeon would traditionally perform a vitrectomy and remove the gel-like substance (the vitreous) that fills the eye. Then the therapy is injected into the eye through the retina, a process called a retinotomy. The Orbit SDS, which received 510K clearance from the U.S. Food and Drug Administration, is designed to avoid the need for a vitrectomy and perforation of the retina. The goal is to simplify surgical procedures to make the administration safe and consistent to give the medicine the best chance of working. The Orbit SDS was developed by Orbit Biomedical. In 2019 Orbit Biomedical merged with Gyroscope Therapeutics creating the only retinal gene therapy company to combine discovery research, drug development, a manufacturing platform and surgical delivery capabilities. In addition to developing the Orbit SDS technology for its own medicines, Gyroscope also licenses the Orbit SDS technology to other companies who are developing gene and cell therapies to treat eye disease.

Forward-Looking Statements

BioTime cautions you that all statements, other than statements of historical facts, contained in this press release, are forward-looking statements. Forward-looking statements, in some cases, can be identified by terms such as “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “design,” “intend,” “expect,” “could,” “plan,” “potential,” “predict,” “seek,” “should,” “would,” “contemplate,” “project,” “target,” “tend to,” or the negative version of these words and similar expressions. Such statements include, but are not limited to, statements relating to BioTime’s ability to advance its product candidates and BioTime’s expectations regarding its delivery systems and formulations. Forward-looking statements involve known and unknown risks, uncertainties and other factors that may cause BioTime’s actual results, performance or achievements to be materially different from future results, performance or achievements expressed or implied by the forward-looking statements in this press release, including risks and uncertainties inherent in BioTime’s business and other risks described in BioTime’s filings with the Securities and Exchange Commission (SEC). BioTime’s forward-looking statements are based upon its current expectations and involve assumptions that may never materialize or may prove to be incorrect. All forward-looking statements are expressly qualified in their entirety by these cautionary statements. Further information regarding these and other risks is included under the heading “Risk Factors” in BioTime’s periodic reports filed with the SEC, including BioTime’s Annual Report on Form 10-K filed with the SEC on March 14, 2019 and its other reports, which are available from the SEC’s website. You are cautioned not to place undue reliance on forward-looking statements, which speak only as of the date on which they were made. BioTime undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made, except as required by law.

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