Orchard Therapeutics Showcases Clinical Data at the 61st American Society of Hematology Annual Meeting

December 8, 2019

BOSTON and LONDON, Dec. 08, 2019 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a leading commercial-stage biopharmaceutical company dedicated to transforming the lives of patients with serious and life-threatening rare diseases through innovative gene therapies, will be presenting new registrational data from multiple programs at the 61st American Society of Hematology (ASH) Annual Meeting being held December 7-10, 2019 in Orlando, FL.

On Sunday, December 8, 2019, investigators will describe ongoing clinical progress for two lead development programs in the company's primary immune deficiencies portfolio: OTL-103, an investigational gene therapy in development for the treatment of Wiskott-Aldrich syndrome (WAS) at the San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget) in Milan, Italy; and OTL-101, an investigational gene therapy in development for the treatment of adenosine deaminase severe combined immunodeficiency (ADA-SCID).

In addition, on Monday, December 9, 2019, investigators will deliver an oral presentation featuring updated data from the ongoing clinical proof-of-concept study of OTL-203, an investigational gene therapy in development for the treatment of mucopolysaccharidosis type I (MPS-I) at SR-Tiget.

To learn more about Orchard's approach to *ex vivo*, autologous, hematopoietic stem cell (HSC) based gene therapy, conference attendees can visit booth #2228 in the Exhibition Hall.

Full presentation details are below:

Poster Presentation Details

Lentiviral Hematopoietic Stem and Progenitor Cell Gene Therapy for Wiskott-Aldrich Syndrome (WAS): Up to 8 Years of Follow up in 17 Subjects Treated Since 2010
Publication Number: 3346
Session: 801. Gene Therapy and Transfer: Poster II
Date and time: Sunday, December 8, 6:00-8:00pm ET

Lentiviral Gene Therapy with Autologous Hematopoietic Stem and Progenitor Cells (HSPCs) for the Treatment of Severe Combined Immune Deficiency Due to Adenosine Deaminase Deficiency (ADA-SCID): Results in an Expanded Cohort
Publication Number: 3345
Session: 801. Gene Therapy and Transfer: Poster II
Date and time: Sunday, December 8, 6:00-8:00pm ET

Oral Presentation Details

Extensive Metabolic Correction of Hurler Disease by Hematopoietic Stem Cell-Based Gene Therapy: Preliminary Results from a Phase I/II Trial
Publication Number: 607
Session: 801. Gene Therapy and Transfer: Gene Therapies for Non-Malignant Disorders
Date and time: Monday, December 9, 7:00am ET

About ADA-SCID and OTL-101
Severe combined immune deficiency due to adenosine deaminase deficiency (ADA-SCID) is a rare, life-threatening, inherited disease of the immune system caused by mutations in the ADA gene resulting in a lack of, or minimal, immune system development.[1-4] The first symptoms of ADA-SCID typically manifest during infancy with recurrent severe bacterial, viral and fungal infections and overall failure to thrive, and without treatment the condition can be fatal within the first two years of life. The incidence of ADA-SCID is currently estimated to be one in 500,000 live births in the United States and between one in 200,000 and one in 1 million in Europe.[3] OTL-101 is an autologous, *ex vivo,* hematopoietic stem cell-based gene therapy for the treatment of patients diagnosed with ADA-SCID being investigated in multiple clinical trials in the United States and Europe, including a registrational trial at the University of California, Los Angeles (UCLA). OTL-101 has received orphan drug designation from the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) for the treatment of ADA-SCID, and Breakthrough Therapy Designation from the FDA.

About WAS and OTL-103
Wiskott-Aldrich Syndrome (WAS) is a life-threatening inherited immune disorder characterized by autoimmunity and abnormal platelet function and manifests with recurrent, severe infections and severe bleeding episodes, which are the leading causes of death in this disease. Without treatment, the median survival for WAS patients is 14 years of age. Treatment with stem cell transplant carries significant risk of mortality and morbidities. OTL-103 is an *ex vivo,* autologous, hematopoietic stem cell-based gene therapy developed for the treatment of WAS that Orchard acquired from GSK in April 2018 and has been developed at the San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget) in Milan, Italy. The global incidence of WAS is estimated to be about 100-260 births per year, with a global prevalence of 2,900-4,700 patients.

About MPS-I and OTL-203
Mucopolysaccharidosis type I (MPS-I) is a rare inherited neurometabolic disease caused by a deficiency of the IDUA (alpha-L-iduronidase) lysosomal

enzyme required to break down glycosaminoglycans (also known as GAGs or mucopolysaccharides). The accumulation of GAGs across multiple organ systems results in the symptoms of MPS-I including neurocognitive impairment, skeletal deformity, loss of vision and hearing, hydrocephalus, and cardiovascular and pulmonary complications. MPS-I occurs at an overall estimated frequency of one in every 100,000 live births.[5] There are three subtypes of MPS-I; approximately 60 percent of MPS-I patients have the severe Hurler subtype and, when untreated, these patients rarely live past the age of 10.[Id] Treatment options for MPS-I include hematopoietic stem cell transplant and chronic enzyme replacement therapy, both of which have significant limitations. Though early intervention with enzyme replacement therapy has been shown to delay or prevent some clinical features of the condition, it has only limited efficacy on neurological symptoms. OTL-203 is an *ex vivo*, autologous, hematopoietic stem cell-based gene therapy being studied for the treatment of MPS-I. Orchard was granted an exclusive worldwide license to intellectual property rights to research, develop, manufacture and commercialize the gene therapy program for the treatment of MPS-I developed by the San Raffaele-Telethon Institute for Gene Therapy in Milan, Italy.

About Orchard
Orchard Therapeutics is a fully integrated commercial-stage biopharmaceutical company dedicated to transforming the lives of patients with serious and life-threatening rare diseases through innovative gene therapies.

Orchard's portfolio of *ex vivo*, autologous, hematopoietic stem cell (HSC) based gene therapies includes Strimvelis®, a gammaretroviral vector-based gene therapy and the first such treatment approved by the European Medicines Agency for severe combined immune deficiency due to adenosine deaminase deficiency (ADA-SCID). Additional programs for neurometabolic disorders, primary immune deficiencies and hemoglobinopathies are all based on lentiviral vector-based gene modification of autologous HSCs and include three advanced registrational studies for metachromatic leukodystrophy (MLD), ADA-SCID and Wiskott-Aldrich syndrome (WAS), clinical programs for X-linked chronic granulomatous disease (X-CGD), transfusion-dependent beta-thalassemia (TDT) and mucopolysaccharidosis type I (MPS-I), as well as an extensive preclinical pipeline. Strimvelis, as well as the programs in MLD, WAS and TDT were acquired by Orchard from GSK in April 2018 and originated from a pioneering collaboration between GSK and the San Raffaele Telethon Institute for Gene Therapy in Milan, Italy initiated in 2010.

Orchard currently has offices in the UK and the U.S., including London, San Francisco and Boston.

Forward-Looking Statements
This press release contains certain forward-looking statements about Orchard's strategy, future plans and prospects, which are made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements may be identified by words such as "anticipates," "believes," "expects," "intends," "projects," and "future" or similar expressions that are intended to identify forward-looking statements. Forward-looking statements include express or implied statements relating to, among other things, the therapeutic potential of Orchard's product candidates, including the product candidate or candidates referred to in this release, Orchard's expectations regarding the timing of regulatory submissions for approval of its product candidates, including the product candidate or candidates referred to in this release, the timing of announcement of clinical data for its product candidates and the likelihood that such data will be positive and support further clinical development and regulatory approval of these product candidates, including any cryopreserved formulations of such product candidates, and the likelihood of approval of such product candidates by the applicable regulatory authorities. These statements are neither promises nor guarantees and are subject to a variety of risks and uncertainties, many of which are beyond Orchard's control, which could cause actual results to differ materially from those contemplated in these forward-looking statements. In particular, the risks and uncertainties include, without limitation: the risk that any one or more of Orchard's product candidates, including the product candidate or candidates referred to in this release, will not be successfully developed or commercialized, the risk of cessation or delay of any of Orchard's ongoing or planned clinical trials, the risk that prior results, such as signals of safety, activity or durability of effect, observed from preclinical studies or clinical trials will not be replicated or will not continue in ongoing or future studies or trials involving Orchard's product candidates, the delay of any of Orchard's regulatory submissions, the failure to obtain marketing approval from the applicable regulatory authorities for any of Orchard's product candidates, the receipt of restricted marketing approvals, and the risk of delays in Orchard's ability to commercialize its product candidates, if approved. Given these uncertainties, the reader is advised not to place any undue reliance on such forward-looking statements.

Other risks and uncertainties faced by Orchard include those identified under the heading "Risk Factors" in Orchard's annual report on Form 20-F for the year ended December 31, 2018 as filed with the U.S. Securities and Exchange Commission (SEC) on March 22, 2019, as well as subsequent filings and reports filed with the SEC. The forward-looking statements contained in this press release reflect Orchard's views as of the date hereof, and Orchard does not assume and specifically disclaims any obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events or otherwise, except as may be required by law.

[1]Orphanet. SCID due to ADA deficiency. [2]Whitmore KV, Gaspar HB. Front Immunol. 2016;7:314. [3]Kwan A, et al. JAMA. 2014;312:729-738. [4]Sauer AV, et al. Front Immunol. 2012;3:265. [5]Beck et al. The Natural History of MPS I: Global Perspectives from the MPS I Registry. Genetics in Medicine 2014, 16(10), 759.

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