



Orchard Therapeutics Announces Acceptance of Late-Breaking Abstracts of OTL-101 for ADA-SCID and OTL-102 for X-CGD at the 2019 Transplantation and Cellular Therapy Meetings of ASBMT and CIBMTR

February 6, 2019

BOSTON and LONDON, Feb. 06, 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, today announced that new clinical data for OTL-101 and OTL-102 will be presented in oral and poster presentations at the Transplantation and Cellular Therapy (TCT) Meetings of the American Society of Blood and Marrow Transplantation (ASBMT) and Center for International Blood and Marrow Transplant Research (CIBMTR) to be held on February 20-24, 2019 in Houston, TX.

Donald B. Kohn, M.D., professor of Microbiology, Immunology & Molecular Genetics at the University of California, Los Angeles, will be presenting data from an ongoing registrational clinical trial of OTL-101 for the treatment of adenosine deaminase severe combined immunodeficiency (ADA-SCID). Dr. Kohn will also be presenting detailed clinical proof-of-concept data for OTL-102 for the treatment of X-linked chronic granulomatous disease (X-CGD), which was highlighted during the Presidential Symposium at the 2018 American Society of Hematology Annual Meeting & Exposition in December.

The presentations are listed below and registration to access to a live stream of the President's Symposia is available online via the TCT website: <https://www.eiseverywhere.com/ereg/index.php?eventid=350979&>

Oral presentation details for OTL-101 and OTL-102:

Title: Gene Therapy for ADA-SCID
Session: President's Symposia: Cell and Gene Therapy: The Next Big Challenges
Date: Friday, February 22, 2019
Time: 9:30-10:00 a.m. CT
Location: George R. Brown Convention Center - Grand Ballroom ABC

Title: *LBA1: Effective Lentiviral Gene Therapy for X-linked Chronic Granulomatous Disease (X-CGD)*
Session: Late Breaking Abstracts
Date: Sunday, February 24, 2019
Time: 12:00-12:15 p.m. CT
Location: Hilton Americas Houston - Grand Ballroom A

Poster presentation details for OTL-101:

Title: *LBA12: Autologous Ex Vivo Lentiviral Gene Therapy for the Treatment of Severe Combined Immune Deficiency Due to Adenosine Deaminase Deficiency (ADA-SCID)*
Session: Poster Session II: Late Breaking Abstracts
Date: Saturday, February 23, 2019
Time: 6:45-7:45 p.m. CT
Location: George R. Brown Convention Center - Exhibit Hall B3

About ADA-SCID and OTL-101

Adenosine deaminase-severe combined immunodeficiency (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 in the United States is currently estimated to be between one in 200,000 and one in 1 million live births.³ Higher incidence rates are reported in geographies of higher consanguinity, such as Turkey and the Middle-East. OTL-101 is an autologous *ex vivo* lentiviral gene therapy for the treatment of 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 EMA for the treatment of ADA-SCID, and Breakthrough Therapy Designation from the FDA. The studies are supported by multiple institutions including the National Institute of Allergy and Infectious Diseases (NIAID), the National Institutes of Health (NIH), the National Gene Vector Biorepository, the California Institute of Regenerative Medicine, Medical Research Council and the National Institute for Health Research Biomedical Research Centre at Great Ormond Street Hospital for Children NHS Foundation Trust and University College London.

About X-CGD and OTL-102

X-linked chronic granulomatous disease (X-CGD) is a rare, life-threatening, inherited disease of the immune system caused by mutations in the cytochrome B-245 beta chain (CYBB) gene. Because of the underlying genetic defect in the CYBB gene, the neutrophils of patients with X-CGD are unable to kill bacteria and fungi, leading to repeated chronic infections. The main clinical manifestations of X-CGD are pyoderma; pneumonia; colitis; lymphadenitis; brain, lung and liver abscesses; and osteomyelitis. Patients with X-CGD typically start to develop infections in the first decade of life and mortality has been estimated at approximately 40% by the age of 35 years.⁵ The incidence of X-CGD is currently estimated to be between 2.6 in 1 million and 10 in 1 million male live births. OTL-102 is an autologous *ex vivo* lentiviral gene therapy being studied for the treatment of X-CGD. The studies are supported by multiple institutions including the California Institute of Regenerative Medicine, the Gene Therapy Resource Program from the National Heart, Lung, and Blood Institute, the NIH, the NIAID Intramural Program, the Wellcome Trust and the National Institute for Health Research Biomedical Research Centre at Great Ormond Street Hospital for Children NHS Foundation Trust and University College London.

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 autologous *ex vivo* gene therapies includes Strimvelis, the first autologous *ex vivo* gene therapy approved by the European Medicines Agency for adenosine deaminase severe combined immunodeficiency (ADA-SCID). Additional programs for neurometabolic disorders, primary immune deficiencies and hemoglobinopathies 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) and transfusion-dependent beta-thalassemia (TDBT), as well as an extensive preclinical pipeline.

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

Forward-Looking Statements

This press release contains certain forward-looking statements 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," "anticipates," 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, Orchard's programs, including the therapeutic potential of its product candidates, including OTL-101 and OTL-102. These statements are neither promises nor guarantees, but 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 OTL-101 and OTL-102, 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, and the risk of delays in Orchard's ability to commercialize its product candidates, if approved. Orchard undertakes no 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. For additional disclosure regarding these and other risks faced by Orchard, see the disclosure contained in Orchard's public filings with the Securities and Exchange Commission.

¹Flinn AM, Gennery AR. *Orphanet J Rare Dis.* 2018;13(1):65 ²Whitmore KV, Gaspar HB. *Front Immunol.* 2016;7:314. ³Kwan A, et al. *JAMA.* 2014;312:729-738. ⁴Sauer AV, et al. *Front Immunol.* 2012;3:265. ⁵van den Berg et. al, *PLoS One.*2009;4(4):e5234

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