Orchard Therapeutics Outlines Comprehensive Presence at the European Society of Gene & Cell Therapy Congress

October 13, 2021

Nine accepted abstracts demonstrate broad potential of the company's HSC gene therapy approach to treat severe neurodegenerative diseases and immunological disorders

BOSTON and LONDON, Oct. 13, 2021 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced the acceptance of nine abstracts at the upcoming European Society of Gene & Cell Therapy Congress (ESGCT) taking place virtually from October 19-22.

Clinical and pre-clinical data from across the company's hematopoietic stem cell (HSC) gene therapy portfolio will be featured in two oral and seven poster presentations, including an update on the ongoing proof-of-concept study of OTL-201 for the treatment of Mucopolysaccharidosis type IIIA (MPS-IIIA, also known as Sanfilippo syndrome type A), pre-clinical data from OTL-204 in frontotemporal dementia (FTD), as well as proof-of-principle for longitudinal monitoring of vector integration sites using Liquid Biopsy Integration Site sequencing (LiBIS-seq).

Additionally, Orchard's scientific advisory board member and clinical collaborator Alessandra Biffi, M.D., professor of pediatrics, University of Padua and chief of the Pediatric Onco-hematology Unit of Padua Hospital, will be giving an invited presentation on the HSC gene therapy landscape for the treatment of neurodegenerative disorders, which will include an overview of several of the company's investigational programs.

The presentations are listed below, and the full program is available online on the ESGCT website. All times are Central European Summer Time (CEST).

Oral Presentation Details:

Haematopoietic reconstitution dynamics of mobilized peripheral blood- and bone marrow-derived haematopoietic stem/progenitor cells after gene therapy
Presenting Author: Andrea Calabria, Ph.D., San Raffaele Telethon Institute for Gene Therapy
Abstract Number: OR049
Date/Time: Friday, October 22, 2021 at 10:01 CEST

Longitudinal monitoring of vector integration sites in *in vivo* GT approaches by Liquid-Biopsy-Integration-Site-Sequencing
Presenting Author: Daniela Cesana, Ph.D., San Raffaele Telethon Institute for Gene Therapy
Abstract Number: OR058
Date/Time: Friday, October 22, 2021 at 12:46 CEST

Poster Presentation Details:

All posters will be available on demand starting October 19, 2021 on the ESGCT website.

Development of an ex vivo Gene Therapy for Frontotemporal Dementia (FTD)
Presenting Author: Yuri Ciervo, Ph.D., division of pediatric Hematology,Oncology and Stem Cell Transplantation, Woman's and Child Health Department, University of Padova, Padova, Italy
Abstract Number: P077

Optimized Lentiviral Transduction Process for ex vivo CD34+ Hematopoietic Stem Cell Gene Therapy Drug Product Manufacture
Presenting Author: Saranya Elavazhagan, Orchard Therapeutics
Abstract Number: P271

Clinical Trial Update: Ex-vivo autologous stem cell gene therapy in MPSIIIA
Presenting Author: Brian Bigger, Ph.D., University of Manchester
Abstract Number: P361

Dissecting bone remodelling mechanisms and hematopoietic stem cell gene therapy impact in Mucopolysaccharidosis type I Hurler bone defects
Presenting Author: Ludovica Santi, Ph.D., San Raffaele Telethon Institute for Gene Therapy
Abstract Number: P157

Hematopoietic reconstitution and lineage commitment in HSC GT patients are influenced by the disease background
Presenting Author: Andrea Calabria, Ph.D., San Raffaele Telethon Institute for Gene Therapy
Abstract Number: P181

Kinetics and composition of haematopoietic stem/progenitors mobilized cells upon G-CSF and Plerixafor administration in transplant donor or patients undergoing autologous gene therapy
Presenting Author: Luca Basso-Ricci, San Raffaele Telethon Institute for Gene Therapy
Abstract Number: P174

Role of peripheral blood circulating haematopoietic stem/progenitor cells during physiological haematopoietic maturation and after gene therapy
Presenting Author: Pamela Quaranta, San Raffaele Telethon Institute for Gene Therapy
Abstract Number: P186

About Orchard Therapeutics
At Orchard Therapeutics, our vision is to end the devastation caused by genetic and other severe diseases. We aim to do this by discovering, developing and commercializing new treatments that tap into the curative potential of hematopoietic stem cell (HSC) gene therapy. In this approach, a patient's own blood stem cells are genetically modified outside of the body and then reinserted, with the goal of correcting the underlying cause of disease in a single treatment.

In 2018, the company acquired GSK's rare disease gene therapy portfolio, which originated from a pioneering collaboration between GSK and the San Raffaele Telethon Institute for Gene Therapy in Milan, Italy. Today, Orchard has a deep pipeline spanning pre-clinical, clinical and commercial stage HSC gene therapies designed to address serious diseases where the burden is immense for patients, families and society and current treatment options are limited or do not exist.

Orchard has its global headquarters in London and U.S. headquarters in Boston. For more information, please visit www.orchard-tx.com, and follow us on Twitter and LinkedIn.

Availability of Other Information About Orchard
Investors and others should note that Orchard communicates with its investors and the public using the company website (www.orchard-tx.com), the investor relations website (ir.orchard-tx.com), and on social media (Twitter and LinkedIn), including but not limited to investor presentations and investor fact sheets, U.S. Securities and Exchange Commission filings, press releases, public conference calls and webcasts. The information that Orchard posts on these channels and websites could be deemed to be material information. As a result, Orchard encourages investors, the media, and others interested in Orchard to review the information that is posted on these channels, including the investor relations website, on a regular basis. This list of channels may be updated from time to time on Orchard's investor relations website and may include additional social media channels. The contents of Orchard's website or these channels, or any other website that may be accessed from its website or these channels, shall not be deemed incorporated by reference in any filing under the Securities Act of 1933.

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