



Orchard Therapeutics Announces Presentations at ASGCT 2022 Showcasing Potential of HSC Gene Therapy in Neurodegenerative Disorders and Beyond

May 10, 2022

BOSTON and LONDON, May 10, 2022 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today outlined seven presentations from across its platform to be featured at the American Society of Gene & Cell Therapy (ASGCT) 25th Annual Meeting taking place May 16-19 in Washington, D.C. Featured presentations include updated results on the OTL-203 clinical program for mucopolysaccharidosis type I Hurler syndrome (MPS-IH), as well as several accepted abstracts highlighting preclinical work demonstrating the applicability of HSC gene therapy to potentially address other neurodegenerative and CNS-related conditions, including frontotemporal dementia (FTD).

"The breadth of data to be presented at ASGCT represent notable progress in our efforts to realize the full potential of our HSC gene therapy platform, particularly for neurodegenerative and CNS-related disorders," said Leslie Meltzer, Ph.D., chief medical officer of Orchard Therapeutics. "Our programs continue to demonstrate the unique ability of the HSC gene therapy approach to enable broad distribution of gene-corrected cells and localized delivery of therapeutic enzymes and proteins at clinically relevant concentrations not achievable by other modalities."

The full presentation details are as follows:

- Monday, May 16
 - Presentation title: **Up to 10.5 years of follow-up in 17 subjects treated with hematopoietic stem and progenitor cell lentiviral gene therapy for Wiskott-Aldrich syndrome**
Time: 2:15-2:30 p.m. EDT
Lead Author: Dr. Francesca Ferrua
Type: Oral Abstract Session
Session Title: Gene and Cell Therapy Trials in Progress
 - Presentation title: **Targeting CX3CR1 gene to improve microglia reconstitution and transgene delivery into the CNS upon hematopoietic stem and progenitor cell transplant**
Time: 4:00-4:15 p.m. EDT
Lead Author: Dr. Annita Montepeloso
Type: Oral Abstract Session
Session Title: Hematopoietic Stem Cell Gene Therapy
 - Presentation title: **Hematopoietic reconstitution and lineage commitment in HSC-GT patients are influenced by the disease background**
Time: 5:30-6:30 p.m. EDT
Lead Author: Dr. Andrea Calabria
Type: Poster Session
Session Title: Hematologic and Immunologic Diseases I
 - Presentation title: **Development of an ex vivo gene therapy for frontotemporal dementia (FTD)**
Time: 5:30-6:30 p.m. EDT
Lead Author: Dr. Yuri Ciervo
Type: Poster Session
Session Title: Neurologic Diseases I
- Wednesday, May 18
 - Presentation title: **Clinical trial results of hematopoietic stem cell gene therapy for mucopolysaccharidosis type I Hurler**
Time: 8:00-8:26 a.m. EDT
Lead Author: Maria Ester Bernardo
Type: Scientific Symposium
Session Title: Inborn Metabolic Issues

- Presentation title: **Pathophysiological mechanisms of bone damage and bone cross correction in MPSIH gene therapy**
Time: 5:30-6:30 p.m. EDT
Lead Author: Dr. Ludovica Santi
Type: Poster Session
Session Title: Musculo-skeletal Diseases
- Presentation title: **Innovative and regulated lentiviral promoter for the gene therapy of neurodegenerative diseases**
Time: 5:30-6:30 p.m. EDT
Lead Author: Dr. Yuri Ciervo
Type: Poster Session
Session Title: Neurologic Diseases III

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 is advancing a 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.

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 "potential," "continue to," and "future" or similar expressions that are intended to identify forward-looking statements. Except for statements of historical fact, information contained herein constitutes forward-looking statements and may include, but is not limited to, Orchard's expectations regarding the safety and efficacy of its products and product candidates. 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, these risks and uncertainties include, without limitation, the risk that prior results, such as signals of safety, activity or durability of effect, observed from preclinical studies or clinical trials of Orchard's product candidates will not be repeated or continue in ongoing or future studies or trials involving its product candidates and the severity of the impact of the COVID-19 pandemic on Orchard's business, including on preclinical and clinical development, its supply chain and its commercial programs. Given these uncertainties, the reader is advised not to place 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 most recent annual or quarterly report filed with the U.S. Securities and Exchange Commission (SEC), 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.

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