



Orchard Therapeutics Announces Multiple Presentations at ESGCT 2023

October 24, 2023

Eight presentations highlight the differentiated profile and broad applicability of the company's HSC gene therapy platform

Data from proof-of-concept study of OTL-203 in MPS-IH demonstrate favorable outcomes for clinical manifestations of the disease not well addressed by the current standard-of-care

Chief Technical Officer Nicoletta Loggia, Ph.D., to give invited talk on manufacturing strategies and innovations aimed at enabling the application of HSC gene therapy to address more prevalent diseases

BOSTON and LONDON, Oct. 24, 2023 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced eight presentations from across its hematopoietic stem cell (HSC) gene therapy platform will be featured at the European Society of Gene and Cell Therapy (ESGCT) 30th Annual Congress taking place October 24-27, 2023, in Brussels.

Featured presentations include a range of interim clinical outcomes in addition to the neurological and skeletal results previously reported from the company's proof-of-concept study of OTL-203 in the Hurler subtype of mucopolysaccharidosis type I (MPS-IH), as well as a first look at pre-clinical data demonstrating the use of vectorized HSCs as a delivery vehicle for monoclonal antibodies. In addition, several abstracts from the company's pre-clinical research programs demonstrate the therapeutic potential of HSC gene therapy to address larger indications, including a genetic sub-type of frontotemporal dementia (FTD) and Crohn's disease, as well as chronic autoimmune disorders.

The company will also host a sponsored symposium on Thursday, October 26, 2023, from 1:30 to 2:50 p.m. CEST featuring expert speakers discussing the potential of HSC gene therapy as a platform to deliver therapeutic genes to target organs such as the brain, bone and GI tract for the treatment of more prevalent diseases where current therapies are limited or do not exist.

Additionally, Nicoletta Loggia, Ph.D., chief technical officer, will give an invited talk on Friday, October 27 at 8:30 a.m. CEST, titled "Meeting the moment: Overcoming manufacturing bottlenecks and technical challenges to usher a new era of genetic medicines," exploring strategies and innovations critical to enabling the application of HSC gene therapy to address larger indications.

"Together with our clinical collaborators, we're proud of our significant presence at ESGCT which continues to underscore the transformative impact and broad applicability of our HSC gene therapy platform," said Fulvio Mavilio, Ph.D., chief scientific officer of Orchard Therapeutics. "Collectively, these data demonstrate the potential of our approach to correct the underlying genetic defects responsible for several difficult-to-treat diseases, furthering our efforts toward unlocking the full promise of our portfolio."

Details of the oral presentations are follows (all times in CEST):

- Title: Lentiviral ex-vivo HSC gene therapy as a tool to deliver therapeutic antibodies beyond the blood-brain barrier
Date/Time: Tuesday, October 24 at 6:15 p.m.
Lead Author: Chiara Recchi
- Title: Interim skeletal outcome after hematopoietic stem and progenitor cell-gene therapy for Mucopolysaccharidosis type I Hurler
Date/Time: Wednesday, October 25 at 3:30 p.m.
Lead Author: Maria Ester Bernardo
- Title: Development of an ex vivo hematopoietic stem cell Gene Therapy for Frontotemporal Dementia (FTD)
Date/Time: Friday, October 27 at 9:45 a.m.
Lead Author: Yuri Ciervo

Details of the poster presentations are as follows (all times in CEST):

- Title: Hematopoietic Stem Cell Gene Therapy as a Novel Gene Therapy Approach for Severe Crohn's Disease Associated with NOD2-Deficiency
Date/Time: Wednesday, October 25 at 5:00 p.m.
Lead Author: Florence Enjalbert

- Title: HSC-derived CAR-Treg gene therapy: a novel approach for the treatment of chronic autoimmune disease
Date/Time: Wednesday, October 25 at 5:00 p.m.
Lead Author: Greg Crawford
- Title: Interim non-neurological, non-skeletal outcomes after hematopoietic stem and progenitor cell-gene therapy for Mucopolysaccharidosis type I Hurler
Date/Time: Wednesday, October 25 at 6:15 p.m.
Lead Author: Francesca Tucci
- Title: Dissecting the mechanisms of myeloid-to-neural enzymatic cross-correction in the context of hematopoietic stem cell gene therapy for Metachromatic Leukodystrophy
Date/Time: Wednesday, October 25 at 5:00 p.m.
Lead Author: Vasco Meneghini
- Title: Downstream strategies for concentration and purification of LV Vectors from suspension cell-based systems
Date/Time: Wednesday, October 25 at 6:15 p.m.
Lead Author: Luca Crippa

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 [X \(Twitter\)](#) 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 ([X: 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 forward-looking statements, which are made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. All statements that are not statements of historical facts are, or may be deemed to be, forward-looking statements. Forward-looking statements include express or implied statements relating to, among other things, the therapeutic potential of Orchard's 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 products will not be successfully commercialized, and the risk that long-term adverse safety findings may be discovered. 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 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.