



Orchard Therapeutics Announces New Clinical and Pre-clinical Data at ASGCT 2023

May 3, 2023

Six presentations showcase the differentiated profile and broad applicability of the company's HSC gene therapy platform

Oral presentations in MPS-IH and MPS-IIIA and the progranulin form of frontotemporal dementia highlight leadership in neurometabolic and CNS diseases

Pre-clinical data in NOD2-Crohn's disease and HSC-Tregs demonstrate potential in larger indications

BOSTON and LONDON, May 03, 2023 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced six presentations from across its hematopoietic stem cell (HSC) gene therapy platform will be featured at the 26th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) taking place May 16-20, 2023, in Los Angeles.

Featured presentations include updated biochemical and early neurocognitive results from the company's ongoing proof-of-concept (PoC) study of OTL-201 in mucopolysaccharidosis type IIIA (MPS-IIIA), also known as Sanfilippo syndrome type A, as well as updated skeletal outcomes from the PoC study of OTL-203 in the Hurler subtype of mucopolysaccharidosis type I (MPS-IH). In addition, several abstracts from the company's pre-clinical research programs demonstrate the therapeutic potential of HSC gene therapy to address genetic sub-types of frontotemporal dementia (FTD) and Crohn's disease, as well as chronic autoimmune disorders.

"Our presence at ASGCT highlights the transformative impact and broad applicability of our platform," said Leslie Meltzer, Ph.D., chief medical officer of Orchard Therapeutics. "Taken together, the data from our MPS programs continues to show the potential of a one-time HSC gene therapy to address some of the most severe manifestations of this complex group of multi-system diseases. In addition, we continue to apply the differentiated delivery mechanism of our platform to advance a strategic research pipeline in difficult-to-treat diseases such as NOD2-Crohn's disease, where we will share updated pre-clinical PoC results. With our research collaborators, we also look forward to providing a first-look at pre-clinical results from OTL-204, which is being developed for the potential treatment of FTD caused by heterozygous mutations in the progranulin gene."

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

- Title: Early skeletal outcome after hematopoietic stem cell gene therapy for Mucopolysaccharidosis Type I Hurler
Date/Time: Thursday, May 18 at 8:15 a.m.
Lead Author: Maurizio De Pellegrin
- Title: Development of an ex vivo Gene Therapy for Frontotemporal Dementia (FTD)
Date/Time: Friday, May 19 at 5:15 p.m.
Lead Author: Yuri Ciervo
- Title: Supraphysiological enzyme and early neurocognitive outcomes after autologous hematopoietic stem cell gene therapy in patients with Mucopolysaccharidosis IIIA
Date/Time: Saturday, May 20 at 11:00 a.m.
Lead Author: Brian W. Bigger

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

- Title: CAR-Treg cells differentiated in vivo by transduced hematopoietic stem cells: a new approach to treatment of autoimmune disorders
Date/Time: Wednesday, May 17 from noon to 2:00 p.m.
Lead Author: Greg Crawford
- Hematopoietic Stem Cell Gene Therapy as a Novel Therapeutic Approach for Severe NOD2-Deficient Crohn's Disease
Date/Time: Friday, May 19 from noon to 2:00 p.m.
Lead Author: Florence Enjalbert

- Title: Mucopolysaccharidosis type I skeletal alterations and cross correction in an ex vivo gene therapy approach

Date/Time: Friday, May 19 from noon to 2:00 p.m.

Lead Author: Ludovica Santi

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 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.

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