



## Orchard Therapeutics Announces First Neurocognitive Data from OTL-201 Study in MPS-III A Accepted for Oral Presentation at ASH 2022

November 3, 2022

**Company to host KOL conference call and webcast on Monday, December 12 at 5:00 p.m. EST**

BOSTON and LONDON, Nov. 03, 2022 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced early clinical findings from its ongoing proof-of-concept study of investigational OTL-201 in MPS-III A will be featured in an oral presentation at the 64th American Society of Hematology (ASH) Annual Meeting & Exposition, taking place December 10-13, 2022, in New Orleans.

The oral presentation at ASH 2022 will showcase the first neurocognitive data for all patients and extend upon previously presented biochemical results, with a median follow-up of 1.5 years.

The presentation details are listed below. Abstracts are available on the [ASH website](#).

- Title: "Biochemical Engraftment and Clinical Outcomes Following Ex-Vivo Autologous Stem Cell Gene Therapy for Mucopolysaccharidosis Type III A"  
Date/Time: Monday, December 12, 2022, at 10:45 a.m. CST  
Presenter: Professor Rob Wynn, consultant pediatric hematologist and director of Pediatric Bone Marrow Transplant Program, Royal Manchester Children's Hospital (RMCH), part of Manchester University NHS Foundation Trust  
Room: Ernest N. Morial Convention Center, 220-222

Orchard is planning to host a virtual KOL investor webcast on Monday, December 12, 2022, at 5:00 p.m. EST to review the data presented at the meeting. A live webcast will be available under "Events" in the Investors & Media section of the company's website at [www.orchard-tx.com](http://www.orchard-tx.com) and a replay of the webcast will be archived following the event.

Five patients with MPS-III A, aged 6 to 24 months, were treated with OTL-201 as part of this ongoing proof-of-concept trial, sponsored by The University of Manchester, conducted at Royal Manchester Children's Hospital, and funded by Orchard Therapeutics. Primary study objectives include safety, tolerability and peripheral expression of SGSH activity in total leukocytes at 12 months. Secondary study objectives include overall survival and neurocognition as measured by the Bayley Scales of Infant and Toddler Development. Patients will be followed for a minimum of three years.

### About MPS-III A

Mucopolysaccharidosis type III A (MPS-III A, also known as Sanfilippo syndrome type A) is a rare and life-threatening metabolic disease. People with MPS-III A are born with a mutation in the *N-sulphoglucosamine sulphohydrolase (SGSH)* gene which, when healthy, helps the body break down sugar molecules called mucopolysaccharides. The buildup of mucopolysaccharides in the brain and other tissues leads to intellectual disability and loss of motor function. MPS-III A occurs in approximately one in every 100,000 live births. Life expectancy of children born with MPS-III A is estimated to be between 10-25 years.

### About OTL-201

OTL-201 is an investigational hematopoietic stem cell gene therapy being developed for the treatment of MPS-III A. It uses a modified virus to insert a functional copy of the *SGSH* gene into a patient's hematopoietic stem cells. OTL-201 has received rare pediatric disease designation from the FDA and is currently being evaluated in an ongoing proof-of-concept clinical trial.

### 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](http://www.orchard-tx.com), and follow us on [Twitter](#) and [LinkedIn](#).

**About Manchester University NHS Foundation Trust (MFT)**

Manchester University NHS Foundation Trust (MFT) is the largest NHS trust in England and a leading provider of specialist healthcare services. Its 10 hospitals are home to hundreds of world class clinicians and academic staff, committed to finding patients the best care and treatments. Its hospitals are Manchester Royal Infirmary, Saint Mary's Hospital, Royal Manchester Children's Hospital, Manchester Royal Eye Hospital, North Manchester General Hospital, University Dental Hospital of Manchester, Trafford General Hospital, Altrincham Hospital, Wythenshawe Hospital and Withington Hospital. More information is available at: [www.mft.nhs.uk](http://www.mft.nhs.uk).

**About The University of Manchester**

The University of Manchester is a member of the prestigious Russell Group and one of the UK's largest single-site universities. We have over 40,000 students, 12,000 staff and, with almost 480,000 former students from more than 190 countries, are home to the largest alumni community of any campus-based university in the UK. We are ranked in the top ten of the [Times Higher Education \(THE\) Impact Rankings](#) globally; are the [top UK University for graduate employability](#) according to The Graduate Market in 2022 and no fewer than 25 Nobel laureates have either worked or studied here. Visit [www.manchester.ac.uk](http://www.manchester.ac.uk) for further information or <https://www.manchester.ac.uk/discover/vision/> for our latest strategic vision.

**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](http://www.orchard-tx.com)), the investor relations website ([ir.orchard-tx.com](http://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. 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. 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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