



## Orchard Therapeutics Announces Multiple Presentations at the 24th Annual Meeting of the American Society of Gene & Cell Therapy

April 28, 2021

*Seven abstracts accepted show the potential of HSC gene therapy to transform the treatment of multiple severe rare disorders*

*First peer-reviewed presentation from company's work in transduction enhancers showcases promise to improve manufacturing efficiency of lentiviral-based gene therapy*

BOSTON and LONDON, April 28, 2021 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced the acceptance of seven abstracts at the 24th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT) taking place virtually from May 11-14. Accepted abstracts include clinical data from several of its hematopoietic stem cell (HSC) gene therapy programs, including: OTL-203, being investigated for the treatment of mucopolysaccharidosis type I (MPS-I), and OTL-101, being investigated for the treatment of adenosine deaminase severe combined immunodeficiency (ADA-SCID), as well as the company's efforts to improve efficiency of HSC gene therapy manufacturing through the development of proprietary transduction enhancers.

"Our significant presence at ASGCT this year showcases not only the depth and breadth of our HSC gene therapy pipeline and the strength of our clinical data but also how we are using innovation to improve the way our HSC gene therapies will be manufactured in the future," said Bobby Gaspar, M.D., Ph.D., chief executive officer of Orchard Therapeutics. "These data demonstrate the transformative potential of HSC gene therapy and, along with our partners, we look forward to sharing results with the broader scientific and medical communities."

The presentations are listed below, and the full preliminary program is available online on the ASGCT [website](#).

### Oral Presentation Details:

#### **Development of an optimized lentiviral transduction process for *ex vivo* CD34+ hematopoietic stem cell gene therapy drug product manufacture**

Abstract Number: 1

Presenting Author: Pervinder Sagoo, Ph.D., Orchard Therapeutics

Date/Time: Tuesday, May 11 at 5:30 p.m. EDT

#### **Autologous *ex vivo* lentiviral gene therapy for the treatment of ADA-SCID**

Abstract Number: 40

Presenting Author: Claire Booth, Ph.D., UCL GOSH Institute of Child Health

Date/Time: Tuesday, May 11 at 6:30 p.m. EDT

#### **Immune reconstitution in transfusion dependent beta-thalassemia patients treated with hematopoietic stem cell gene therapy**

Abstract Number: 79

Presenting Author: Samantha Scaramuzza, Ph.D., San Raffaele Telethon Institute for Gene Therapy

Date/Time: Wednesday, May 12 at 5:45 p.m. EDT

#### **Hematopoietic reconstitution and lineage commitment in HSC gene therapy patients are influenced by the disease background**

Abstract Number: 82

Presenting Author: Andrea Calabria, Ph.D., San Raffaele Telethon Institute for Gene Therapy

Date/Time: Wednesday, May 12 at 6:30 p.m. EDT

#### **Liquid-biopsy-integration-site-sequencing allows safety studies and longitudinal monitoring of vector integration sites in LV and AAV-based *in-vivo* GT applications**

Abstract Number: 250

Presenting Author: Daniela Cesana, Ph.D., San Raffaele Telethon Institute for Gene Therapy

Date/Time: Friday, May 14 at 12:15 p.m. EDT

#### ***Ex vivo* hematopoietic stem cell gene therapy for mucopolysaccharidosis type I (Hurler syndrome): An interim analysis with a median follow up of 19 months**

Abstract Number: 219

Presenting Author: Bernhard Gentner, M.D., San Raffaele Telethon Institute for Gene Therapy

Date/Time: Friday, May 14 at 1:15 p.m. EDT

### Poster Presentation Details:

#### **Dissecting bone remodeling mechanisms and hematopoietic stem cell gene therapy impact in mucopolysaccharidosis type I Hurler bone defects**

Abstract Number: 609

Presenting Author: Ludovica Santi, Ph.D., San Raffaele Telethon Institute for Gene Therapy

Date/Time: Tuesday, May 11 at 8:00 a.m. EDT

## **About Orchard Therapeutics**

Orchard Therapeutics is a global gene therapy leader dedicated to transforming the lives of people affected by rare diseases through the development of innovative, potentially curative gene therapies. Our *ex vivo* autologous gene therapy approach harnesses the power of genetically modified blood stem cells and seeks to correct the underlying cause of disease in a single administration. In 2018, Orchard 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. Orchard now has one of the deepest and most advanced gene therapy product candidate pipelines in the industry spanning multiple therapeutic areas where the disease burden on children, families and caregivers is immense 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](#).

## **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 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. Forward-looking statements include express or implied statements relating to, among other things, Orchard's business strategy and goals, and the therapeutic potential of Orchard's product candidates, including the product candidate or candidates referred to in this release. 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 will not be replicated or will not continue in ongoing or future studies or trials involving Orchard's product candidates, will be insufficient to support regulatory submissions or marketing approval in the US or EU, as applicable, or that long-term adverse safety findings may be discovered; the risk that any one or more of Orchard's product candidates, including the product candidates referred to in this release, will not be approved, successfully developed or commercialized; the risk of cessation or delay of any of Orchard's ongoing or planned clinical trials; the risk that Orchard may not successfully recruit or enroll a sufficient number of patients for its clinical trials; the delay of any of Orchard's regulatory submissions; the failure to obtain marketing approval from the applicable regulatory authorities for any of Orchard's product candidates or the receipt of restricted marketing approvals; the inability or risk of delays in Orchard's ability to commercialize its product candidates, if approved, or Libmeldy in the EU; the risk that the market opportunity for Libmeldy, or any of Orchard's product candidates, may be lower than estimated; and the severity of the impact of the COVID-19 pandemic on Orchard's business, including on clinical development, its supply chain and commercial programs. 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 Annual Report on Form 10-K for the year ended December 31, 2020, as 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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