



Orchard Therapeutics Announces Multiple Presentations at WORLDSymposium™ 2022

February 7, 2022

BOSTON and LONDON, Feb. 07, 2022 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today outlined five upcoming presentations from across its neurodegenerative portfolio to be featured at the 18th Annual WORLDSymposium™ being held on February 7-11, 2022.

The company will feature encores clinical data presentations from two of its investigational hematopoietic stem cell (HSC) gene therapy programs —OTL-203 for MPS-I and OTL-201 for MPS-IIIA—as well as data supporting its patient identification and market access initiatives for Libmeldy®/OTL-200 in Europe for children with early-onset metachromatic leukodystrophy (MLD).

The presentation details are as follows:

- Wednesday, February 9 at 8:00 a.m. PST
 - **Clinical trial update: Ex-vivo autologous HSC gene therapy in MPS-IIIA**
Lead Author: Simon Jones, MBChB BSc MRCPCH, Manchester University NHS Foundation Trust (MFT)
Presenter: Brian Bigger, Ph.D., University of Manchester
Type: Oral (also being presented as a virtual poster #144)
Program: OTL-201
- Thursday, February 10 from 3:00 to 5:00 p.m. PST
 - **Newborn screening for metachromatic leukodystrophy in northern Germany**
Poster Number: 222
Presenting Author: Petra Oliva, Ph.D., ARCHIMED Life Science GmbH
Type: Poster (in-person)
Program: Libmeldy / OTL-200
 - **The cost-effectiveness of atidarsagene autotemcel for the treatment of metachromatic leukodystrophy in France**
Poster Number: 227
Presenting Author: Francis Pang, MBA, Orchard Therapeutics
Type: Poster (virtual)
Program: Libmeldy / OTL-200
 - **Quality of life and caregiver burden in metachromatic leukodystrophy: Results from a cross-national study of 6 countries**
Poster Number: 228
Presenting Author: Francis Pang, MBA, Orchard Therapeutics
Type: Poster (virtual)
Program: Libmeldy® / OTL-200
- Friday, February 11 at 8:00 a.m. PST
 - **First-in-human Phase I/II clinical trial of hematopoietic stem and progenitor cell gene therapy for Hurler syndrome: Favorable safety profile and extensive metabolic correction**
Lead Author: Francesca Tucci, M.D., San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget)
Presenter: Su Syonmez, MSc, Orchard Therapeutics
Type: Oral
Program: OTL-203

About Libmeldy / OTL-200

Libmeldy (atidarsagene autotemcel), also known as OTL-200, has been approved by the European Commission for the treatment of MLD in eligible

early-onset patients characterized by biallelic mutations in the ARSA gene leading to a reduction of the ARSA enzymatic activity in children with i) late infantile or early juvenile forms, without clinical manifestations of the disease, or ii) the early juvenile form, with early clinical manifestations of the disease, who still have the ability to walk independently and before the onset of cognitive decline. Libmeldy is the first therapy approved for eligible patients with early-onset MLD.

The most common adverse reaction attributed to treatment with Libmeldy was the occurrence of anti-ARSA antibodies. In addition to the risks associated with the gene therapy, treatment with Libmeldy is preceded by other medical interventions, namely bone marrow harvest or peripheral blood mobilization and apheresis, followed by myeloablative conditioning, which carry their own risks. During the clinical studies, the safety profiles of these interventions were consistent with their known safety and tolerability.

For more information about Libmeldy, please see the [Summary of Product Characteristics \(SmPC\)](#) available on the EMA website.

Libmeldy is approved in the European Union, UK, Iceland, Liechtenstein and Norway. OTL-200 is an investigational therapy in the U.S.

Libmeldy was developed in partnership with the SR-Tiget in Milan, Italy.

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 has a deep 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. 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. 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 quarterly report on Form 10-Q for the quarter ended September 30, 2021, 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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