



Orchard Therapeutics Outlines Comprehensive Presence at 2021 WORLDSymposium™

January 28, 2021

Nine abstracts accepted demonstrating potential of HSC gene therapy to treat multiple neurodegenerative disorders

New clinical data from all eight patients treated with OTL-203 for Mucopolysaccharidosis type I (MPS I)

Biomarker data from first three patients treated with OTL-201 for Mucopolysaccharidosis type IIIA (MPS-III A or Sanfilippo Syndrome Type A)

Multiple abstracts highlighting clinical and real-world data for OTL-200 and Metachromatic Leukodystrophy (MLD)

Company to host virtual investor webinar to review symposium data on Tuesday, February 9, 2021 at 4:30 p.m. ET

BOSTON and LONDON, Jan. 28, 2021 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today outlined nine upcoming presentations from its neurodegenerative portfolio to be featured at the 17th Annual WORLDSymposium™ being held on February 8-12, 2021. Accepted abstracts include clinical data from three of its hematopoietic stem cell (HSC) gene therapy programs — OTL-200 for MLD, OTL-203 for MPS-I and OTL-201 for MPS-III A — as well as data supporting Orchard's multi-pronged patient identification and market access strategies for eligible MLD patients in Europe.

"Together with our clinical partners, we're proud of our presence at the upcoming WORLD Symposium, which for the first time features clinical data on cognitive function and growth in all eight MPS-I patients treated with gene therapy," said Bobby Gaspar, M.D., Ph.D., chief executive officer of Orchard. "Alongside emerging data in MPS-III A and our extensive body of clinical and real-world data in MLD, our programs are establishing a clear picture of the transformative potential of HSC gene therapy across multiple fatal neurodegenerative conditions."

The presentations are listed below and the full preliminary program is available online on the [WORLDSymposium website](#). The ePosters will open at 2:30 p.m. ET on Monday, February 8, 2021 and will remain open throughout WORLDSymposium 2021.

Orchard is planning to host a virtual investor webinar on Tuesday, February 9th, 2021 at 4:30 p.m. ET to review the data from its neurodegenerative programs presented at the WORLDSymposium. A live webcast will be available under "Events" in the Investors & Media section of the company's website at www.orchard-tx.com and a replay of the webcast will be archived following the event.

Platform Oral Presentation Details:

Ex-vivo autologous stem cell gene therapy clinical trial for mucopolysaccharidosis type IIIA: Update on phase I/II clinical trial

Presenting Author: Jane Kinsella, on behalf of the Clinical Trials Team at Royal Manchester Children's Hospital, part of Manchester University NHS Foundation Trust – 2021 Young Investigator Award Recipient Date/Time: Tuesday, February 9, 2021, 11:12 a.m. ET

Ex vivo hematopoietic stem cell gene therapy for mucopolysaccharidosis type I (Hurler syndrome)

Presenting Author: Bernhard Gentner, San Raffaele Telethon Institute for Gene Therapy
Date/Time: Tuesday, February 9, 2021, 11:24 a.m. ET

Lentiviral hematopoietic stem and progenitor cell gene therapy provides durable clinical benefit in early-symptomatic early juvenile metachromatic leukodystrophy

Presenting Author: Francesca Fumagalli, San Raffaele Telethon Institute for Gene Therapy, IRCCS San Raffaele Scientific Institute
Date/Time: Wednesday, February 10, 2021, 11:36 a.m. ET

ePoster Presentation Details:

Lentiviral haematopoietic stem cell gene therapy for metachromatic leukodystrophy: Results in nine patients treated with a cryopreserved formulation of OTL-200

Abstract Number: 25
Presenting Author: Valeria Calbi, San Raffaele Telethon Institute for Gene Therapy
Date/Time: Wednesday, February 10, 2021, 2:30 – 3:30 p.m. ET

Initial signs and symptoms of metachromatic leukodystrophy: A caregiver perspective

Abstract Number: 64
Presenting Author: Florian Eichler, Massachusetts General Hospital
Date/Time: Thursday, February 11, 2021, 2:30 – 3:30 p.m. ET

Demographic and clinical characteristics of patients with metachromatic leukodystrophy in the United Kingdom: Interim results from an observational real-world study

Abstract Number: 110

Presenting Author: Simon Jones, Manchester Centre for Genomic Medicine

Date/Time: Thursday, February 11, 2021, 2:30 – 3:30 p.m. ET

Quality of life of patients with metachromatic leukodystrophy and their caregivers in the US, UK, Germany and France

Abstract Number: 186

Presenting Author: Francis Pang, Orchard Therapeutics

Date/Time: Thursday, February 11, 2021, 2:30 – 3:30 p.m. ET

Health-related quality of life in metachromatic leukodystrophy based on a societal utility study in the UK

Abstract Number: 187

Presenting Author: Francis Pang, Orchard Therapeutics

Date/Time: Thursday, February 11, 2021, 2:30 – 3:30 p.m. ET

Newborn screening for metachromatic leukodystrophy in Northern Germany - a prospective study

Abstract Number: 269

Presenting Author: Thomas Wiesinger, ARCHIMEDlife

Date/Time: Thursday, February 11, 2021, 2:30 – 3:30 p.m. ET

About Manchester University NHS Foundation Trust

Manchester University NHS Foundation Trust is one of the largest NHS trusts in England and a leading provider of specialist healthcare services. Its nine hospitals are home to hundreds of world class clinicians and academic staff committed to finding patients the best care and treatments. This includes Royal Manchester Children's Hospital (RMCH), one of the largest children's hospitals in the UK with an international reputation as a centre of excellence for research and innovation. More information is available at www.mft.nhs.uk.

About Libmeldy / OTL-200

Libmeldy (autologous CD34+ cell enriched population that contains hematopoietic stem and progenitor cells (HSPC) transduced *ex vivo* using a lentiviral vector encoding the human arylsulfatase-A (ARSA) gene), 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 not approved outside of the European Union, UK, Iceland, Liechtenstein and Norway. OTL-200 is an investigational therapy in the US.

Libmeldy was developed in partnership with the San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget) in Milan, Italy.

About Orchard

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, 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, 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 quarterly report on Form 10-Q for the quarter ended September 30, 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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