Orchard Therapeutics Announces Multiple Presentations at 2022 SSIEM Annual Symposium Highlighting Neurometabolic Disease Portfolio

August 29, 2022

BOSTON and LONDON, Aug. 29, 2022 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced seven presentations from across its neurometabolic portfolio will be featured at the Society for the Study of Inborn Errors of Metabolism (SSIEM) Annual Symposium, taking place from August 30 to September 2, 2022, in Freiburg, Germany.

Featured presentations include an oral presentation on Libmeldy® (atidarsagene autotemcel) from clinical development through approval by the European Commission and treatment of the first patients in a commercial setting in Europe, several accepted abstracts highlighting newborn screening efforts to support the timely and accurate diagnosis of metachromatic leukodystrophy (MLD), as well as an encore clinical data presentation from the company's investigational hematopoietic stem cell (HSC) gene therapy OTL-203 for MPS-IH.

The oral presentation details are as follows:

- Title: LC-MSMS sulfatides measurement in dried blood spots for the diagnosis of metachromatic leukodystrophy
  Date/Time: Wednesday, August 31 at 3:30 p.m. CEST
  Type: Parallel Session 2A
  Session: Mechanisms and Markers in Lysosomal Disorders
  Lead Author: Dr. Magali Pettazzoni
  Abstract #: 2378

- Title: From academic clinical development to an approved commercial drug administered in multiple highly specialised centres: arsa-cel, a lentiviral haematopoietic stem-cell gene therapy for early-onset metachromatic leukodystrophy (MLD)
  Date/Time: Thursday, September 1 at 12:15 p.m. CEST
  Type: Parallel Session 3A
  Session: Gene Therapy Clinical Trials
  Lead Author: Dr. Francesca Fumagalli
  Abstract #: 2118

- Title: Hematopoietic stem & progenitor cell gene therapy for Hurler syndrome: interim clinical results and extensive metabolic correction
  Date/Time: Thursday, September 1 at 2:30 p.m. CEST
  Type: Parallel Session 3A
  Session: Gene Therapy Clinical Trials
  Lead Author: Dr. Francesca Tucci
  Abstract #: 2040

The poster presentation details are as follows:

- Title: Blood spot hexadecanoyl sulphatide concentration in metachromatic leukodystrophy and age-matched, ARSA pseudodeficiency, and unaffected controls
  Lead Author: Dr. Heather Brown
  Abstract #: 2810

- Title: Quantification of hexadecanoylsulphatide in dried blood spots using liquid chromatography tandem mass spectrometry
  Lead Author: Dr. Heather Brown
  Abstract #: 2885

- Title: Newborn Screening for Metachromatic leukodystrophy in Germany - A prospective Study
  Lead Author: Dr. Berthold Streubel
  Abstract #: 2776

- Title: Pilot program MLD screening of high-risk population
  Lead Author: Dr. Berthold Streubel
  Abstract #: 2929

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 of Libmeldy, 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 San Raffaele-Telethon Institute for Gene Therapy (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 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. 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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