Orchard Announces Multiple Presentations at 2022 ESGCT Annual Congress Showing the Potential of HSC Gene Therapy in Several Therapeutic Areas

October 10, 2022

Clinical and pre-clinical data from across the company's portfolio will be featured in four oral and six poster presentations

Co-founder and Chief Executive Officer Bobby Gaspar to give invited talk exploring the curative potential of HSC gene therapy when coupled with widespread newborn screening

BOSTON and LONDON, Oct. 10, 2022 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced multiple presentations at the 29th Annual Congress of the European Society of Gene & Cell Therapy (ESGCT), taking place October 11-14, 2022, in Edinburgh.

Clinical and pre-clinical data from across the company's hematopoietic stem cell (HSC) gene therapy portfolio will be featured in four oral and six poster presentations, including an invited talk from Bobby Gaspar, M.D., Ph.D., co-founder and chief executive officer of Orchard Therapeutics exploring the curative potential of HSC gene therapy when coupled with widespread newborn screening.

Additionally, Orchard's clinical collaborator Bernhard Gentner M.D., Ph.D., group leader of the Translational Stem Cell and Leukemia Research Unit at the San Raffaele-Telethon Institute for Gene Therapy (SR-TIGET) in Milan and staff hematologist in the Hematology and Bone Marrow Transplantation Unit of San Raffaele Hospital will be giving an invited presentation on the company's investigational HSC gene therapy OTL-203 for MPS-IH.

Other featured presentations include several accepted abstracts highlighting the company's newborn screening and early research efforts, including a poster on its internally discovered HSC-CAR Treg gene therapy as a potential treatment for severe chronic autoimmune disorders.

"Together with our clinical and research partners, we're proud of our presence at the upcoming ESGCT Annual Congress," said Leslie Meltzer, Ph.D., chief medical officer of Orchard Therapeutics. "Alongside our extensive body of clinical data and real-world evidence in MLD, our programs are showing the transformative potential of HSC gene therapy across many different genetic diseases with high unmet medical need."

The presentations are listed below, and the full program is available online on the ESGCT website. All times are British Summer Time (BST).

The oral presentation details are as follows:

- Title: "Ending the devastation caused by severe genetic diseases through HSC gene therapy and newborn screening: a case study in metachromatic leukodystrophy"
  Date/Time: Friday, October 14 at 14:30 – 17:00
  Presenter: Bobby Gaspar
  Room: Pentland Suite

- Title: "Genetically-modified hematopoietic stem cells as a one-time, systemic treatment for non-hematologic disorders"
  Date/Time: Wednesday, October 12 at 11:10 – 13:15
  Presenter: Bernhard Gentner
  Room: Pentland Suite

- Title: "Skeletal damage and cross-correction in MPSIH HSPC-gene therapy"
  Date/Time: Tuesday, October 11 at 17:00 – 19:15
  Presenter: Ludovica Santi
  Room: Fintry

- Title: "Safety and clinical benefit of lentiviral hematopoietic stem and progenitor cell gene therapy in 23 patients with Wiskott-Aldrich Syndrome up to 10.5 years of follow-up"
  Date/Time: Tuesday, October 11 at 17:00 – 19:15
  Presenter: Francesca Ferrua
  Room: Fintry

The poster presentation details are as follows:

- Title: "Quantification of hexadecanoylsulphatide in dried blood spots using liquid chromatography tandem mass spectrometry"
  Lead Author: Heather Brown
  Poster #: P565

- Title: “Bloodspot sulphatide concentration in suspected lysosomal storage diseases including metachromatic leukodystrophy”
  Lead Author: Heather Brown
  Poster #: P552

- Title: “HSC-CAR Treg gene therapy as a treatment for severe chronic autoimmune disorders through delivery of stable antigen-specific suppression of autoimmunity”
  Lead Author: Greg Crawford
  Poster #: P405

- Title: “Optimized lentiviral transduction process for ex vivo CD34+ hematopoietic stem cell gene therapy drug product manufacture”
  Lead Authors: Saranya Elavazhagan and Maria del Mar Masdeu
  Poster #: P277

- Title: “Development of an ex vivo Gene Therapy for Frontotemporal Dementia (FTD)”
  Lead Author: Yuri Ciervo
  Poster #: P200

- Title: “Innovative and Regulated Lentiviral Promoter for the Gene Therapy of Neurodegenerative Diseases”
  Lead Author: Yuri Ciervo
  Poster #: P190

About Libmeldy / OTL-200
Libmeldy (atidarsagene autotemcel), also known as OTL-200, has been approved by the European Commission for the treatment of metachromatic leukodystrophy (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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