Orchard Therapeutics Announces Eight Presentations Across Gene Therapy Portfolio at the American Society of Gene & Cell Therapy (ASGCT) 23rd Annual Meeting

April 29, 2020

Most Comprehensive Presentation of Orchard Clinical Data to Date; Spans Five Disease Areas and Highlights Broad Applicability of Hematopoietic Stem Cell Gene Therapy Approach

Accepted Abstracts Include Data on Investigational Gene Therapies for Neurometabolic Conditions, Including Updated Interim Data from Proof-of-Concept Trial in OTL-203 for Mucopolysaccharidosis Type I

BOSTON and LONDON, April 29, 2020 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced eight upcoming presentations from across its neurometabolic, primary immune deficiency and blood disorder franchises at the American Society of Gene & Cell Therapy (ASGCT) 23rd Annual Meeting, which will take place online May 12-15, 2020, marking the company's most comprehensive presentation of clinical data at a medical meeting to date. Accepted abstracts include data on three investigational treatments to address various neurometabolic disorders, including mucopolysaccharidosis type I (MPS-I), with high unmet need.

"Data to be presented at ASGCT represent tremendous progress in our mission and continued momentum across our clinical programs," said Bobby Gaspar, M.D., Ph.D., chief executive officer of Orchard. "Collectively, these data demonstrate the potential of our hematopoietic stem cell gene therapy approach to correct the underlying genetic defects responsible for five different rare diseases, furthering our efforts toward realizing the full promise of this approach."

Gaspar continues, "These programs are a result of tireless work by our research partners, and I want to thank them, the clinicians and patients involved in the clinical trials for showing how hematopoietic stem cell gene therapy can potentially change the lives of individuals with these devastating conditions."

Of note, two of the eight presentations at ASGCT focus on new interim data from OTL-203, Orchard's investigational gene therapy for the treatment of MPS-I, a rare, inherited neurometabolic disease caused by a deficiency of the alpha-L-iduronidase (IDUA) lysosomal enzyme that results in the accumulation of complex carbohydrates called glycosaminoglycans and is characterized by neurological, skeletal and cardiovascular problems.

The presentations also include the full integrated analysis of the registrational data set of OTL-101 for the treatment of adenosine deaminase severe combined immunodeficiency (ADA-SCID), which includes all patients treated in the U.S. with 24 months of follow-up; full results of the clinical proof-of-concept clinical trial for OTL-300 for the treatment of transfusion-dependent beta thalassemia, which achieved its primary outcome measures; and long-term outcomes in patients with ADA-SCID treated with Strimvelis®.

The presentations are listed below and the full abstracts for the oral and poster presentations are available on the ASGCT meeting website. All times listed are Eastern Time (ET).

Invited Oral Presentation Details

Long-term Outcomes of Strimvelis (*ex vivo* gene therapy for ADA-SCID)
Session: The Long-term View: Extended follow up of Patients in Pivotal Gene Therapy Trials
Date and time: Friday, May 15, 2020, 8:52 – 9:18 a.m.

HSC-based Strategies to Treat CNS Disorders: Focus Hurler/MPS1H
Session: Gene and Cell Therapeutic Strategies to Treat Disorders of the CNS – An International Perspective - Organized by the International Committee
Date and time: Friday, May 15, 2020, 9:10 – 9:45 a.m.

Oral Presentation Details

Preliminary Outcomes of Haematopoietic Stem Cell Gene Therapy in a Patient with Mucopolysaccharidosis IIIA*
Abstract number: 525
Session: Clinical Gene Therapies for Inborn Errors of Metabolism
Date and time: Wednesday, May 13, 2020, 4:00 – 4:15 p.m.

Pathophysiological Mechanisms of Bone Defects and Impact of *Ex Vivo* Hematopoietic Stem Cell Gene Therapy in Mucopolysaccharidosis Type I Hurler

Abstract number: 528
Session: Clinical Gene Therapies for Inborn Errors of Metabolism
Date and time: Wednesday, May 13, 2020, 4:45 – 5:00 p.m.

Late Phases of Hematopoietic Reconstitution in Metachromatic Leukodystrophy Gene Therapy Patients are Characterized by Lineage Commitment of Individual HSC Clones
Abstract number: 530
Session: Clinical Gene Therapies for Inborn Errors of Metabolism
Date and time: Wednesday, May 13, 2020, 5:15 – 5:30 p.m.

Lentiviral Gene Therapy with Autologous Hematopoietic Stem and Progenitor Cells (HSPCs) for the Treatment of Severe Combined Immune Deficiency Due to Adenosine Deaminase Deficiency (ADA-SCID): Two Year Follow-up Results
Abstract number: 1300
Session: Clinical Trials Spotlight Symposium
Date and time: Friday, May 15, 2020, 8:45 – 9:00 a.m.

Poster Presentation Details

Clinical Outcomes from a Phase I/II Gene Therapy Trial for Patients Affected by Severe Transfusion Dependent Beta-Thalassemia: Two-year Follow Up
Abstract number: 376
Session: Hematologic and Immunologic Diseases
Date and time: Tuesday, May 12, 2020, 5:30 – 6:30 p.m.

Liquid-Biopsy-Integration-Site-Sequencing for the Retrieval of Vector Integrations from Cell-Free DNA and Detection of Early Premalignant Expansions Hidden in Solid Tissues
Abstract number: 391
Session: Hematologic and Immunologic Diseases
Date and time: Tuesday, May 12, 2020, 5:30 – 6:30 p.m.

**Patient was treated by the Royal Manchester Children's Hospital (RMCH) under a "Specials" license, granted by the UK government for the use of an unlicensed pharmaceutical product in situations of high unmet need when there is no other treatment option available. Orchard holds the license to the MPS-IIIA investigational gene therapy product (OTL-201) and is funding the proof-of-concept clinical trial being conducted at RMCH, which utilizes the same technology and procedures that were used to treat this first MPS-IIIA patient.*

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. The company has one of the deepest gene therapy product candidate pipelines in the industry and is advancing seven clinical-stage programs across multiple therapeutic areas, including inherited neurometabolic disorders, primary immune deficiencies and blood disorders, 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.com/orchard_tx and www.linkedin.com/company/orchard-therapeutics), 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 severity of the impact of the COVID-19 pandemic on Orchard's business, including on clinical development and commercial programs; the risk that any one or more of Orchard's product candidates, including the product candidate or 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 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; 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; and the risk of delays in Orchard's ability to commercialize its product candidates, if approved. 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, 2019, as filed with the U.S. Securities and Exchange Commission (SEC) on February 27, 2020, 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.

Contacts

Investors
Renee Leck
Director, Investor Relations
+1 862-242-0764
Renee.Leck@orchard-tx.com

Media
Molly Cameron
Manager, Corporate Communications
+1 978-339-3378
media@orchard-tx.com

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