Orchard Therapeutics Announces Clinical Data Presentations at the 46th Annual Meeting of the European Society for Blood and Marrow Transplantation (EBMT)

August 24, 2020

BOSTON and LONDON, Aug. 24, 2020 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced presentations at the upcoming 46th Annual Meeting of the European Society for Blood and Marrow Transplantation (EBMT), taking place virtually from August 29 - September 1, 2020. New interim data from OTL-203, an investigational gene therapy for the treatment of mucopolysaccharidosis type I (MPS-I), will be shared as part of an invited oral presentation titled 'Gene Therapy in Leucodystrophies and Other Metabolic Disorders'.

The presentations are listed below and the full preliminary program is available online at the EBMT Annual Meeting website. Presentations will be available to registered attendees for virtual viewing throughout the duration of the live meeting and content will be accessible online following the close of the meeting.

Invited Oral Presentation Details

E7-2: Gene Therapy in Leucodystrophies and Other Metabolic Disorders
Session: Gene Therapy for Inherited Disorders 2020
Presenter: M. Ester Bernardo, M.D., Ph.D., San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), Italy
Date and time: Monday, August 31, 2020, 4:50-5:10pm CET/10:50-11:10am ET

ePoster Details

Ex-vivo* Autologous Haematopoietic Stem Cell Gene Therapy in Mucopolysaccharidosis Type IIIA
Poster Session & Number: Gene Therapy; ePoster A214

Lentiviral Hematopoietic Stem and Progenitor Cell Gene Therapy (HSPC-GT) For Metachromatic Leukodystrophy (MLD): Clinical Outcomes From 33 Patients
Poster Session & Number: Gene Therapy; ePoster O075

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 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 quarterly report on Form 10-Q for the quarter ended June 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.

**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 ongoing 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.*

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