Orchard Therapeutics Announces Presentation of Clinical Data from Neurometabolic Franchise at 16th Annual WORLD Symposium

February 10, 2020

BOSTON and LONDON, Feb. 10, 2020 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced upcoming presentations from its neurometabolic franchise at the 16th Annual WORLD Symposium on February 10-13 in Orlando, FL. Accepted abstracts include encore clinical presentations for OTL-200 and emerging data quantifying metachromatic leukodystrophy (MLD) caregiver-reported quality of life experiences, as well as clinical data for investigational treatments in mucopolysaccharidosis type I (MPS-I) and mucopolysaccharidosis type IIIA (MPS-IIIA).

“Neurometabolic disorders such as MLD can have a devastating, lifelong impact, not only on children but on their caregivers, support systems and the broader community,” said Mark Rothera, president and chief executive officer of Orchard. “We look forward to showcasing both real-world and clinical study data from our neurometabolic portfolio at the upcoming WORLD Symposium as we strive to bring about a brighter future for all those affected by rare disease.”

The presentations are listed below and the full preliminary program is available online at the conference website.

Oral presentation details:

Case report of the first patient treated with *ex-vivo* autologous haematopoietic stem cell gene therapy transplant in mucopolysaccharidosis type IIIA*
Presenter: Jane Kinsella, Royal Manchester Children’s Hospital
Session: Translational Research II
Date: Wednesday, February 12
Time: 9:15-9:30 a.m. ET

Lentiviral hematopoietic stem and progenitor cell gene therapy (HSPC-GT) for metachromatic leukodystrophy (MLD): Clinical outcomes from 33 patients
Presenter: Francesca Fumagalli, San Raffaele Telethon Institute for Gene Therapy
Session: Clinical Trials II: Clinical Outcomes
Date: Thursday, February 13
Time: 8:15-8:30 a.m. ET

Poster presentation details:

Lentiviral hematopoietic stem and progenitor cell gene therapy (HSPC-GT) for metachromatic leukodystrophy (MLD): Clinical outcomes from 33 patients
Poster abstract #: P126
Presenter: Francesca Fumagalli, San Raffaele Telethon Institute for Gene Therapy
Session: Poster Reception (Exhibit Hall)
Date: Monday, February 10
Time: 4:30-6:30 p.m. ET

Caregiver-reported impact on quality of life and disease burden in patients diagnosed with metachromatic leukodystrophy: Results of an online survey and a qualitative interview
Poster abstract #: P320
Presenter: Francis Pang, Orchard Therapeutics
Session: Poster Reception (Exhibit Hall)
Date: Tuesday, February 11
Time: 4:30-6:30 p.m. ET

Extensive metabolic correction of mucopolysaccharidosis type I (MPS IH, Hurler syndrome) by hematopoietic stem and progenitor cell (HSPC) based gene therapy (GT): Preliminary results from a phase I/II trial
Poster abstract #: LB-15
Presenter: Francesca Tucci, San Raffaele Telethon Institute for Gene Therapy
Session: Poster Reception (Exhibit Hall)
Date: Wednesday, February 12

Time: 4:30-6:30 p.m. ET

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.

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. Such forward-looking statements may be identified by words such as "anticipates," "believes," "expects," "plans," "intends," "projects," and "future" or similar expressions that are intended to identify forward-looking statements. Forward-looking statements include express or implied statements relating to, among other things, the therapeutic potential of Orchard's product candidates, including the product candidate or candidates referred to in this release, Orchard's expectations regarding the timing of regulatory submissions for approval of its product candidates, including the product candidate or candidates referred to in this release, the timing of interactions with regulators and regulatory submissions related to ongoing and new clinical trials for its product candidates, the timing of announcement of clinical data for its product candidates and the likelihood that such data will be positive and support further clinical development and regulatory approval of these product candidates, and the likelihood of approval of such product candidates by the applicable regulatory authorities. 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, the risks and uncertainties include, without limitation: 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 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, 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 20-F for the year ended December 31, 2018, as filed with the U.S. Securities and Exchange Commission (SEC) on March 22, 2019, 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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Source: Orchard Therapeutics (Europe) Limited

*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.

Source: Orchard Therapeutics (Europe) Limited