Orchard Therapeutics Announces Acceptance of OTL-200 Abstract for MLD at the 45th Annual Meeting of the European Society for Blood and Bone Marrow Transplantation (EBMT)

March 11, 2019

BOSTON and LONDON, March 11, 2019 (GLOBE NEWSWIRE) -- Orchard Therapeutics (NASDAQ: ORTX), a leading commercial-stage biopharmaceutical company dedicated to transforming the lives of patients with serious and life-threatening rare diseases through innovative gene therapies, today announced that new clinical data from the registrational trial of OTL-200 in metachromatic leukodystrophy (MLD) will be featured in an oral presentation at the 45th Annual Meeting of the European Society for Blood and Bone Marrow Transplantation (EBMT) to be held on March 24-27, 2019 in Frankfurt, Germany. The presentation will feature updated safety and efficacy results from patients with a minimum of three years of follow-up following treatment with gene therapy.

Oral presentation details for OTL-200:

Title: *Lentiviral Hematopoietic Stem Cell Gene Therapy (HSC-GT) for Metachromatic Leukodystrophy (MLD) Provides Sustained Clinical Benefit*
Presenter: Valeria Calbi, San Raffaele Scientific Institute and San Raffaele-Telethon Institute for Gene Therapy (SR-TIGET)
Session: Pediatrics 4, Inborn Errors Oral Session
Date: Tuesday, March 26, 2019
Time: 4:55 - 5:06 p.m. CET
Location: Messe Frankfurt Congress Center, Panorama 3

About MLD and OTL-200
Metachromatic leukodystrophy (MLD) is a rare and life-threatening inherited disease of the body's metabolic system occurring in approximately one in every 100,000 live births. MLD is caused by a mutation in the arylsulfatase-A gene (ARSA) gene that results in the accumulation of sulfatides in the brain and other areas of the body, including the liver, the gall bladder, kidneys, and/or spleen. Over time, the nervous system is damaged and patients with MLD will experience neurological problems such as motor, behavioral and cognitive regression, severe spasticity and seizures finding it more and more difficult to move, talk, swallow, eat and see**.** Currently, there are no effective treatments for MLD. In its late infantile form, mortality at 5 years from onset is estimated at 50% and 44% at 10 years for juvenile patients.[1] OTL-200 is an *ex vivo*, autologous, hematopoietic stem cell based gene therapy that Orchard acquired from GSK in April 2018, being studied for the treatment of MLD. OTL-200 originated from a pioneering collaboration between GSK and the Hospital San Raffaele and the Telethon Foundation, acting through their joint San Raffaele-Telethon Institute for Gene Therapy in Milan, initiated in 2010.

About Orchard
Orchard Therapeutics is a fully integrated commercial-stage biopharmaceutical company dedicated to transforming the lives of patients with serious and life-threatening rare diseases through innovative gene therapies.

Orchard's portfolio of autologous, *ex vivo,* hematopoietic stem cell gene therapies includes Strimvelis, the first such treatment approved by the European Medicines Agency for severe combined immune deficiency due to adenosine deaminase deficiency (ADA-SCID). Additional programs for neurometabolic disorders, primary immune deficiencies and hemoglobinopathies include three advanced registrational studies for metachromatic leukodystrophy (MLD), ADA-SCID and Wiskott-Aldrich syndrome (WAS), clinical programs for X-linked chronic granulomatous disease (X-CGD) and transfusion-dependent beta-thalassemia (TDBT), as well as an extensive preclinical pipeline.

Orchard currently has offices in the U.K. and the U.S., including London, San Francisco and Boston.

Forward-Looking Statements
This press release contains certain forward-looking statements 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," "intends," "projects," "anticipates," 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, Orchard's programs, including the therapeutic potential of its product candidates, including OTL-200. These statements are neither promises nor guarantees, but 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 OTL-200, will not be 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, and the risk of delays in Orchard's ability to commercialize its product candidates, if approved. Orchard undertakes no 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. For additional disclosure regarding these and other risks faced by Orchard, see the disclosure contained in Orchard's public filings with the Securities and Exchange Commission.

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[1]Mahmood et al. Metachromatic Leukodystrophy: A Case of Triplets with the Late Infantile Variant and a Systematic Review of the Literature. Journal of Child Neurology 2010, DOI: http://doi.org/10.1177/0883073809341669

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