Orchard Announces Publication by San Raffaele-Telethon Institute for Gene Therapy of OTL-300 Clinical Data for the Treatment of Transfusion-Dependent Beta-Thalassemia in Nature Medicine

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Publication Highlights Encouraging Evidence of OTL-300's Safety and Efficacy in Transfusion-Dependent Beta-Thalassemia Patients

BOSTON and LONDON, Jan. 22, 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 the San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget) has published encouraging results from a clinical trial of OTL-300, an autologous *ex vivo* lentiviral gene therapy program being studied in individuals with transfusion-dependent beta-thalassemia. The paper, published in *Nature Medicine,* provides an interim analysis of long-term efficacy outcomes in seven of the nine treated patients with more than one year of follow up from this ongoing trial.

"Orchard recognizes the pioneering work of researchers and clinicians from SR-Tiget that has led to this peer-reviewed publication of promising clinical data for the treatment of transfusion-dependent beta-thalassemia patients in *Nature Medicine*," said Andrea Spezzi, M.D., chief medical officer of Orchard Therapeutics. "Current treatments for these patients are limited and often require lifelong blood transfusions, which severely affect quality of life, or allogeneic bone marrow transplants, which have a risk of mortality. We are encouraged by this early data and by the potential for OTL-300 to transform the lives of patients suffering from transfusion-dependent beta-thalassemia, and we anticipate reporting data from all nine patients in this proof of concept trial later this year."

The clinical trial conducted at SR-Tiget in Milan is led by Giuliana Ferrari, professor at The Vita-Salute San Raffaele University and made possible by the strategic alliance between San Raffaele Hospital, Telethon Foundation and Orchard Therapeutics, following the transfer of GSK's gene therapy portfolio to Orchard in April 2018. The publication of the trial results was the result of the collaboration between basic researchers and clinicians, and in partnership with the Pediatric Immunohematology Unit headed by Alessandro Aiuti, the Hematology and Hematopoietic Stem Cell Transplantation Unit headed by Fabio Ciceri, and the Rare Diseases Centre at the Policlinico Hospital headed by Maria Domenica Cappellini. The trial, with the clinical coordination of doctor Sarah Marktel of SR-Tiget, has involved other Italian centers specializing in thalassemia and the cooperation of patient organizations.

About Beta-Thalassemia
Beta-thalassemia is a genetic blood disorder caused by a mutation in the beta-globin gene, a fundamental protein required for red blood cells to work correctly. Over 300 mutations in the beta-globin gene are known, which give rise to many different forms of beta-thalassemia, with variable severity. The most damaging mutations cause the almost complete absence of the protein in a patient's blood, causing them to rely on frequent blood transfusions to survive or a bone marrow transplant from a compatible donor.

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 gene therapies includes Strimvelis, the first autologous ex vivo gene therapy approved by the European Medicines Agency for adenosine deaminase severe combined immunodeficiency (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.

About the Telethon Foundation, San Raffaele Hospital and Orchard Therapeutics Alliance
The San Raffaele Telethon Institute of Gene Therapy in Milan (SR-Tiget) was born in 1995 through a joint venture between San Raffaele Hospital, a highly specialized internationally renowned scientific, clinical and academic facility, and the Telethon Foundation, one of the main biomedical charities in Italy. SR-Tiget is now an international point of reference for gene therapy research and cell transplantation for many genetic disorders. To transform basic research results into therapies for patients, in 2010 the Telethon Foundation and San Raffaele Hospital signed a strategic partnership with GSK, to complement their research with the development capacity of a pharmaceutical industry. The goal of this alliance was to develop a therapy for seven rare diseases, including ADA-SCID (Strimvelis, the first ex vivo gene therapy for this condition, received approval in 2016) and beta-thalassemia. In 2018 GSK transferred its rare disease gene therapy portfolio to Orchard Therapeutics. This included Strimvelis, two late-stage clinical programs for metachromatic leukodystrophy (OTL-200) and Wiskott-Aldrich syndrome (OTL-103), and one clinical program for transfusion-dependent beta-thalassemia (OTL-300).

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-300. 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-300, 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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