Orchard Therapeutics Receives EMA PRIME Designation for OTL-203 for the Treatment of MPS-I

September 28, 2020

BOSTON and LONDON, Sept. 28, 2020 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced that the European Medicines Agency (EMA) has granted Priority Medicines (PRIME) designation to OTL-203, an investigational *ex vivo* autologous hematopoietic stem cell (HSC) gene therapy in development for the treatment of mucopolysaccharidosis type I (MPS-I) at the San Raffaele Telethon Institute for Gene Therapy (SR-Tiget) in Milan, Italy.

"We are encouraged by EMA's decision to grant PRIME designation to OTL-203, which was based on an initial clinical assessment of data supporting the potential benefit of our HSC gene therapy for patients with MPS-IH beyond the current standard of care," said Anne Dupraz-Poiseau, PhD., chief regulatory officer of Orchard. "In 2021, we look forward to building upon the promising early data in the ongoing proof-of-concept study and plan to initiate a registrational trial to advance a potential new treatment for patients."

The PRIME program is designed to enhance regulatory support in the EU for the development of promising investigational medicines that, based on early clinical data, may offer a major therapeutic advantage over existing treatments or benefit patients without treatment options. PRIME aims to provide multiple benefits so that these medicines can reach patients earlier: enhanced interaction and early dialogue with EMA, guidance on the overall development plan and regulatory strategy, and the potential for accelerated assessment of the marketing authorization application. For more information please visit the EMA website at www.ema.europa.eu.

Additional interim data was recently presented from the ongoing proof-of-concept clinical trial evaluating the safety and efficacy of OTL-203 in the severe Hurler subtype of MPS-I. Eight patients have been treated in the study, which completed enrollment in December 2019. As of July 2020, all patients had been followed for a minimum of six months, with the longest follow-up extending out to 24 months. Orchard expects to release full proof-of-concept results at one year and initiate a registrational study for OTL-203 in 2021.

About OTL-203 and MPS-I

Mucopolysaccharidosis type I (MPS-I) is a rare, inherited neurometabolic disease caused by a deficiency of the alpha-L-iduronidase (IDUA) lysosomal enzyme, which is required to break down sugar molecules called glycosaminoglycans (also known as GAGs). The accumulation of GAGs across multiple organ systems results in symptoms including neurocognitive impairment, skeletal deformity, loss of vision and hearing, and cardiovascular and pulmonary complications. MPS-I occurs at an overall estimated frequency of one in every 100,000 live births. There are three subtypes of MPS-I; approximately 60 percent of children born with MPS-I have the most severe subtype, called Hurler syndrome, and rarely live past the age of 10 when untreated.
Treatment options for MPS-I include hematopoietic stem cell transplant and chronic enzyme replacement therapy, both of which have significant limitations. Though early intervention with enzyme replacement therapy has been shown to delay or prevent some clinical features of the condition, it has only limited efficacy on neurological symptoms. OTL-203 is an investigational *ex vivo* autologous hematopoietic stem cell gene therapy being studied for the treatment of MPS-I. Orchard was granted an exclusive worldwide license to intellectual property rights to research, develop, manufacture and commercialize the gene therapy program for the treatment of MPS-I developed by the San Raffaele Telethon Institute for Gene Therapy in Milan, Italy.

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. 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, Orchard's business strategy and goals, the therapeutic potential of Orchard's product candidates, including the product candidate referred to in this release, Orchard's expectations regarding the timing of clinical trials for its product candidates, including the product candidate 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. 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, its supply chain and commercial programs; the risk that Orchard will not realize the anticipated benefits of its new strategic plan or the expected cash savings associated with such plan; the risk that any one or more of Orchard's product candidates, including the product candidate referred to in this release, will not be successfully developed, approved 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 or that long-term adverse safety findings may be discovered; 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.

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

orchard_logo.jpg

Source: Orchard Therapeutics (Europe) Limited