# Orchard Therapeutics Announces Orphan Drug and Rare Pediatric Disease Designations for OTL-203 for the Treatment of MPS-I

July 20, 2020

BOSTON and LONDON, July 20, 2020 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced that the company has received both orphan drug designation and rare pediatric disease designation from the U.S Food and Drug Administration (FDA) for OTL-203, an *ex vivo* autologous hematopoietic stem cell (HSC) gene therapy in development for the treatment of mucopolysaccharidosis type I (MPS-I).

"We are pleased by the FDA's acknowledgement of the critical and urgent need to develop additional treatments for MPS-I given the severe, life-limiting nature of the disease," said Bobby Gaspar, M.D., PhD., chief executive officer of Orchard. "The underlying causes of lysosomal storage disorders such as MPS-I have been notably difficult to address, and we are encouraged by the early evidence of our hematopoietic stem cell gene therapy's approach to potentially treating this condition. The orphan drug and rare pediatric disease designations provide important momentum for the OTL-203 clinical program, which we remain committed to advancing as quickly as possible for patients in need."

The FDA grants orphan designation, also referred to as orphan status, to drugs intended for the treatment of rare diseases that affect fewer than 200,000 people in the US.[1] This designation affords Orchard certain benefits, including tax credits for qualified clinical testing, waiver or partial payment of FDA application fees and seven years of market exclusivity, if approved.[2] Separately, rare pediatric disease designations are granted for rare diseases that primarily affect children under 18 years old with recipients of this designation being awarded a priority review voucher, upon approval.[3] The priority review voucher may be redeemed, transferred, or sold.[4]

Orchard recently announced new interim data from an ongoing proof-of-concept clinical trial evaluating the safety and efficacy of OTL-203. The first primary outcome measure was met with all eight patients achieving hematologic engraftment. Additionally, improved motor skills compared to baseline, stable cognitive scores, and normal growth was seen in the first two patients with at least one year of follow-up. Orchard expects to release full proof-of-concept results and initiate the 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 *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. 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 or candidates referred to in this release, and Orchard's expectations regarding the timing of clinical trials and 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 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 March 31, 2020, as filed with the U.S. Securities and Exchange Commission (SEC) on May 7, 2020, 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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[1] §316 Orphan Drug Act & §316.20-21: Verification of orphan-drug status (https://www.accessdata.fda.gov/scripts/cdrh/cfdocs/cfcfr/CFRSearch.cfm?fr=316.20)

[2] FDA Industry Guidance: Designating an Orphan Product: Drugs and Biological Products (https://www.fda.gov/industry/developing-products-rare-diseases-conditions/designating-orphan-product-drugs-and-biological-products)

[3] FDA Rare Pediatric Disease Designation Voucher Programs (https://www.fda.gov/industry/developing-products-rare-diseases-conditions/rare-pediatric-disease-rpd-designation-and-voucher-programs)

[4] §360ff Title 21 – Food and Drugs (https://www.govinfo.gov/content/pkg/USCODE-2012-title21/pdf/USCODE-2012-title21-chap9-subchapV-partB-sec360ff.pdf)

**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

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