Orchard Therapeutics Announces First Patient Dosed with OTL-201 Gene Therapy in Proof-of-Concept Clinical Trial for Sanfilippo Syndrome (MPS-IIIA)

April 27, 2020

BOSTON and LONDON, April 27, 2020 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced that the first patient has been dosed in an open-label, proof-of-concept investigational study of OTL-201, an *ex vivo* autologous hematopoietic stem cell (HSC) gene therapy for the treatment of mucopolysaccharidosis type IIIA (MPS-IIIA). The study is designed to evaluate safety, tolerability and clinical efficacy and is intended to enroll up to five patients between three months and 24 months of age who will be followed for three years. The study also contains a number of key secondary outcome measures such as overall survival, cognition and behavior to help inform future clinical development of HSC gene therapy in this indication.

MPS-IIIA, also known as Sanfilippo syndrome type A, is a rare, inherited neurometabolic disorder caused by genetic mutations that leads to the buildup of sugar molecules called mucopolysaccharides in the body, resulting in progressive intellectual disability and loss of motor function. Children born with MPS-IIIA rarely live past adolescence or early adulthood, and no approved therapies currently exist to treat the disease.

"I am very encouraged that we, together with our research and clinical collaborators in Manchester, could achieve this important milestone in our efforts to develop a gene therapy for MPS-IIIA despite the current, challenging global health circumstances," said Bobby Gaspar, M.D., Ph.D., chief executive officer of Orchard. "It is a testament to the dedication of our collective teams and underscores the truly dire, life-limiting nature of the disease for affected children and their families. This study adds to Orchard's clinical pipeline of HSC gene therapies for the treatment of severe neurometabolic disorders and further demonstrates the potential of our platform approach."

About MPS-IIIA
Mucopolysaccharidosis type IIIA (MPS-IIIA, also known as Sanfilippo syndrome type A) is a rare and life-threatening metabolic disease. People with MPS-IIIA are born with a mutation in the *N-sulphoglucosamine sulphohydrolase (SGSH)* gene, which, when healthy, helps the body break down sugar molecules called mucopolysaccharides. The buildup of mucopolysaccharides in the brain and other tissues leads to intellectual disability and loss of motor function. MPS-IIIA occurs in approximately one in every 100,000 live births. Life expectancy of children born with MPS-IIIA is estimated to be between 10-25 years.

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.

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.com/orchard_tx and www.linkedin.com/company/orchard-therapeutics), 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 and the therapeutic potential of Orchard's product candidates, including the product candidate or candidates referred to in this release. 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 annual report on Form 10-K for the year ended December 31, 2019, as filed with the U.S. Securities and Exchange Commission (SEC) on February 27, 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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