Orchard Therapeutics Announces Global License Agreements for Stable Cell Line Technology from GSK

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BOSTON and LONDON, July 15, 2020 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced that the company has entered into two worldwide royalty-bearing license agreements with GlaxoSmithKline plc (GSK) (LSE/NYSE: GSK) for use of their proprietary lentiviral stable cell line technology (LV-SCLT) for Orchard's investigational hematopoietic stem cell gene therapies for Wiskott Aldrich syndrome (OTL-103 for WAS) and transfusion-dependent beta thalassemia (OTL-300 for TDT).

"Utilization of a stable cell line provides an opportunity to generate lentiviral vector of consistently high titer and eliminates the need to purchase plasmids prior to the production of each viral vector batch, providing more efficient production processes and shorter lead times," said Bobby Gaspar, M.D., Ph.D., chief executive officer of Orchard. "By increasing the efficiency of the vector manufacturing process, this technology can provide a key competitive advantage and supports our focus on manufacturing innovations that enable commercial scalability."

The LV-SCLT permanently and stably enables all the lentiviral vector components to be introduced into a cell line in one step. Selection and expansion of a resulting clonal producer line in either suspension or adherent culture can deliver consistent levels of high titer lentiviral production comparable to those seen using conventional methods. An overview of this technology, co-authored by Orchard and GSK scientists, was presented at the European Society of Gene & Cell Therapy (ESGCT) Annual Congress in October 2019 using work done in the OTL-300 program for TDT*. Under the licenses, GSK has granted patents and pending patent applications related to its LV-SCLT**.

Gaspar continued, "We believe the work completed with OTL-300 utilizing a stable cell line can be leveraged and applied to future development plans in OTL-103 for WAS, which will be especially useful in a commercial setting given the large number of patients diagnosed and living with the disease."

Orchard plans to submit a biologics license application (BLA) and marketing authorization application (MAA) for OTL-103 for the treatment of WAS in the U.S. and EU, respectively, in 2021.

The terms of the license are not expected to have a material impact on Orchard's financial position or near-term cash needs.

About OTL-103 and WAS
OTL-103 is an *ex vivo* autologous hematopoietic stem cell gene therapy in development for the treatment of Wiskott Aldrich syndrome (WAS). WAS is a life-threatening inherited immune disorder that primarily affects males and is characterized by recurrent and severe infections, autoimmunity, eczema and severe bleeding episodes. It is caused by a mutation in the gene that produces the WAS protein, which results in abnormal function of white blood cells and low platelets. Without treatment, the median survival for children born with WAS is 14 years of age. The global incidence of WAS is estimated to be about one in every 100,000 male births per year***.

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, Orchard's expectations regarding the timing of regulatory submissions for its product candidates, the size of the potential markets for Orchard's product candidates, and the effectiveness, efficiencies expected from and expected use of stable cell line technology. 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; the risk of delays in Orchard's ability to commercialize its product candidates, if approved; the risk that the market opportunity for its product candidates may be lower than estimated; and the risk that stable cell line technology is less effective than anticipated or does not yield the expected manufacturing efficiencies. 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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**Zeguer JL, Acors S, Rana B, et al. A BAC-cloning platform for development of stable producer cell lines for commercial scale lentiviral vector manufacture. Hum Gene Ther 2019; 30:11, P056*

***PCT/EP2016/078336 and PCT/EP2016/078334*

****Source: Malik MA, Masab M. Wiskott-Aldrich Syndrome. [Updated 2019 Jun 22]. In: StatPearls [Internet]. Treasure Island (FL): StatPearls Publishing; 2019 Jan-. Available from: https://www.ncbi.nlm.nih.gov/books/NBK539838/*

Source: Orchard Therapeutics (Europe) Limited