



Orchard Therapeutics and SIRION Biotech Announce Licensing Agreement to Enhance Gene Therapy Manufacturing Efficiency

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BOSTON and LONDON and MARTINSRIED, Germany, Dec. 14, 2018 (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, and SIRION Biotech GmbH today announced the entry into a license agreement, pursuant to which Orchard has licensed SIRION's LentiBOOST™ technology to enhance manufacturing efficiency for certain of Orchard's *ex vivo* autologous hematopoietic stem cell gene therapy drug candidates.

"At Orchard Therapeutics, we are establishing manufacturing capabilities to deliver potentially transformative gene therapy products to rare disease patients around the globe. Optimization of lentiviral transduction through a technology such as LentiBOOST™, along with the build-out of our recently announced manufacturing facility, are representative of our continued efforts to increase efficiencies and further streamline our approach to manufacturing gene therapy candidates," said Stewart Craig, Ph.D., chief manufacturing officer of Orchard. "Current studies using LentiBOOST™ in our laboratories have resulted in encouraging data, and we look forward to examining the impact on our portfolio of gene therapy product candidates."

Under the terms of the agreement, SIRION will provide Orchard with a license to its proprietary lentiviral transduction enhancer LentiBOOST™ for development and commercialization activities for select Orchard programs. SIRION will be entitled to upfront and milestone payments and is eligible to receive royalties on net sales of future products that utilize the LentiBOOST™ technology.

"LentiBOOST™ was designed to enhance lentiviral transduction performance for difficult cell types like primary T-cells and hematopoietic stem cells," said Dr. Christian Thirion, founder and chief executive officer of SIRION. "We make our technology available to a wide range of companies and academic agencies – from explorative research hospitals to commercial gene therapy companies. We are delighted that the LentiBOOST™ technology may help Orchard further enhance the efficiency of its lentiviral-based manufacturing processes."

About Orchard Therapeutics

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 primary immune deficiencies, neurometabolic disorders and hemoglobinopathies include three advanced registrational studies for ADA-SCID, metachromatic leukodystrophy (MLD) 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 SIRION Biotech GmbH

SIRION Biotech was founded in 2005 with the goal to spark a new generation of viral vector technologies for gene and cell therapy as well as vaccine development. This meant evolving novel therapeutic viral vectors and technology platforms based on lenti-, adeno-, - and adeno-associated viruses, to expedite its partners' advances in drug development. LentiBOOST™ is applied in a couple of clinical trials from early stage clinical phase I/II up to late stage clinical phase III trials and demonstrated clinical success in improving transduction of the therapeutic vector.

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, the ability of LentiBOOST™ technology to improve efficiencies in drug product manufacturing processes and the results of any pre-clinical and clinical trials of Orchard's product candidates using LentiBOOST™ technology. 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 expected outcomes from the license agreement with SIRION Biotech GmbH, including synergies and improved efficiencies in manufacturing processes, may not be achieved. 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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