Orchard Therapeutics Highlights Recent Accomplishments and 2019 Strategic Priorities as a Global Leader in Gene Therapy

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Preparing Three Lead Programs for MLD, ADA-SCID and WAS for Regulatory Filings Over the Next Three Years

Recently Announced Clinical Proof-of-Concept in X-CGD Demonstrates Platform's Transformative Potential

Advancing Earlier Stage Pipeline with Potential Clinical Proof-of-Concept for TDBT and Clinical Trial Application for MPS-IIIA

Entering 2019 in a Strong Financial Position with $340M in Cash and Investments

BOSTON and LONDON, Jan. 07, 2019 (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, today summarized recent accomplishments and 2019 strategic priorities in conjunction with its attendance at the 37th Annual J.P. Morgan Healthcare Conference in San Francisco. Mark Rothera, president and chief executive officer, will present a business overview outlining the company's progress as a global leader in gene therapy on Wednesday, January 9, 2019 at 3:00 p.m. PT that will be webcast at ir.orchard-tx.com.

"2018 was a momentous year for Orchard, marked by the success of our acquisition and integration of GSK's rare disease *ex-vivo* gene therapy portfolio, initial scaling of our manufacturing capabilities and completion of our initial public offering," said Mr. Rothera. "2019 will continue the company's evolution as a leader in gene therapy, with multiple clinical milestones supporting three regulatory filings over the next three years and growing manufacturing capabilities. We have a bold vision and are well on our way to delivering gene therapies that have the potential to transform the lives of patients with rare, life-threatening diseases worldwide with a single treatment."

2019 Strategic Priorities

Neurometabolic Disorders

- Release two and three-year follow-up data in 20 patients from the fresh formulation registrational trial of OTL-200 for metachromatic leukodystrophy (MLD)
- Release engraftment data in the first three patients from the cryopreserved formulation clinical trial of OTL-200 for MLD
- Submit clinical trial application (CTA) for OTL-201 for mucopolysaccharidosis type IIIA (MPS-IIIA) and support initiation of a clinical trial

Primary Immune Deficiencies

- Release two-year follow-up data in 20 patients from the fresh formulation registrational trial of OTL-101 in adenosine deaminase severe combined immune deficiency (ADA-SCID)
- Release engraftment data in 10 patients from a cryopreserved formulation clinical trial of OTL-101 in ADA-SCID
- Release three-year follow-up data in eight patients from the fresh formulation registrational trial of OTL-103 in Wiskott-Aldrich syndrome (WAS)
- Initiate cryopreservation formulation clinical trial for OTL-103 in WAS
- Design and engage regulators on registrational trial for OTL-102 in X-linked chronic granulomatous disease (X-CGD), which recently achieved clinical proof-of-concept (link to full release here)

Hemoglobinopathies

- Report clinical proof-of-concept data for OLT-300 in transfusion-dependent beta-thalassemia (TDBT)

Major 2018 Accomplishments

Pipeline Expansion and Advancement

- Completed the strategic acquisition and subsequent integration of GSK's rare disease *ex-vivo* gene therapy portfolio, including Strimvelis®, the only treatment for patients with ADA-SCID approved in the EU, along with clinical programs in MLD, WAS and TDBT
- Completed pre-biologics license application (BLA) and CMC specific meetings with the U.S. Food and Drug Administration (FDA) for OTL-101 for ADA-SCID, following which the program remains on track for a BLA filing in the U.S. in 2020
- Achieved clinical proof of concept for OTL-102 in X-CGD, demonstrating sustained levels of functioning neutrophils in patients after 12 months
- Obtained Rare Pediatric Disease Designations from the FDA for OTL-200 for the treatment of MLD and OTL-201 for the

treatment of MPS-IIIA
- Obtained priority medicines (PRIME) designation from the European Medicines Agency (EMA) for OTL-300 for the treatment of TDBT

Corporate & Manufacturing Developments

- Raised approximately $375 million in gross proceeds in 2018 from a Series C financing and underwritten initial public offering
- Leased a manufacturing site in Fremont, CA and opened a Boston, MA corporate office. The manufacturing facility will enhance the company's capacity to develop and deliver *ex-vivo* lentiviral vector and gene-corrected hematopoietic stem cells for a wide range of rare diseases on a global scale and will complement the existing network of partner CMOs that will underpin the launches for the first three programs. (Link to full release here)

Cash Guidance
The company ended 2018 with approximately $340 million of cash and investments. The company expects that its cash, cash equivalents and marketable securities as of December 31, 2018 will enable the company to fund its currently anticipated operating expenses and capital expenditure requirements into the second half of 2020.

Presentation at 37th Annual J.P. Morgan Healthcare Conference

Orchard will webcast its corporate presentation from the 37th Annual J.P. Morgan Healthcare Conference in San Francisco on Wednesday, January 9, 2019 at 3:00 p.m. PT. A live webcast of the presentation will be available under "News & Events" in the Investors & Media section of the company's website at orchard-tx.com. A replay of the webcast will be archived on the Orchard website following the presentation.

About Orchard
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 neurometabolic disorders, primary immune deficiencies and hemoglobinopathies include three advanced registrational studies for metachromatic leukodystrophy (MLD), ADA-SCID 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.

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. Forward-looking statements include express or implied statements relating to, among other things, Orchard's expectations regarding the timing of regulatory submissions for approval of its product candidates, 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, the likelihood of approval of such product candidates by the applicable regulatory authorities, and Orchard's guidance that its existing cash, cash equivalents and marketable securities as of December 31, 2018 will enable the company to fund its anticipated operating expenses and capital expenditure requirements into the second half of 2020. 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 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, the receipt of restricted marketing approvals, or 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. 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, including in the final prospectus related to Orchard's initial public offering filed with the Securities and Exchange Commission pursuant to Rule 424(b) of the Securities Act of 1933, as amended, as well as subsequent filings and reports filed by Orchard with the Securities and Exchange Commission. These forward-looking statements speak only as of the date of publication of this document. 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.

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