Orchard Therapeutics Bolsters R&D and Technical Operations Leadership with New Executive Appointments

September 9, 2021

Gene therapy experts Fulvio Mavilio, Ph.D., and Nicoletta Loggia, Ph.D., join as chief scientific officer and chief technical officer, respectively

Neuroscience and rare disease leader Leslie Meltzer, Ph.D., promoted to chief medical officer

Appointments reinforce company's strategic focus on innovation in the areas of discovery, clinical development and manufacturing

BOSTON and LONDON, Sept. 09, 2021 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced the appointment of two gene therapy and industry experts, Fulvio Mavilio, Ph.D., and Nicoletta Loggia, Ph.D., to serve as chief scientific officer and chief technical officer, respectively. The company also announced the promotion of Leslie Meltzer, Ph.D., who has been serving as senior vice president of medical affairs, clinical operations and diagnostics, to chief medical officer. Together, Drs. Mavilio, Loggia and Meltzer will oversee strategically important functions as Orchard continues to advance its later-stage portfolio and expand its hematopoietic stem cell (HSC) gene therapy pipeline into larger indications.

"We have seen the potentially transformative impact of our HSC gene therapy approach in devastating rare diseases and are now extending our focus on more prevalent conditions where there is a compelling rationale," said Bobby Gaspar, M.D., Ph.D., chief executive officer of Orchard Therapeutics. "Fulvio, Nicoletta and Leslie all have proven track records in their respective areas of expertise, and their leadership in building our next-generation R&D and technical operations capabilities will be instrumental in accelerating our progress, advancing our pipeline and scaling our commercial infrastructure."

"I have worked with many types of genetic medicine technology during my career and believe HSC gene therapy offers immense and distinct promise to correct the underlying cause of many severe diseases with a single treatment," said Dr. Mavilio. "I am eager to begin working with this exceptional team to unlock the full potential of HSC gene therapy in new and larger indications."

"I am excited to join Orchard at this vital juncture as we continue to invest in and scale our technical operations on a global level," said Dr. Loggia. "I look forward to working closely with our CMC teams and CDMO network to put in place innovative and efficient manufacturing solutions that will meet the needs of patients and our organization now and into the future."

"Since joining Orchard more than three years ago, I have been proud to help cultivate the clinical, medical and operational capabilities that support our deep pipeline of investigational and approved HSC gene therapies, including five clinical-stage programs," said Dr. Meltzer. "I look forward to partnering with our development and commercial teams to ensure a seamless integration and advance our portfolio through key anticipated milestones."

Drs. Loggia's and Meltzer's appointments are effective immediately. Dr. Mavilio has initially joined the company in a consulting capacity and will start full-time in January 2022.

About Fulvio Mavilio, Ph.D., chief scientific officer
Dr. Mavilio will lead the company's discovery and translational research activities. In this capacity he will be responsible for expanding the company's pipeline into larger indications.

He joins Orchard Therapeutics from Smart Immune where he serves as chief scientific officer. Previously, Dr. Mavilio was senior vice president of translational science at Audentes Therapeutics in San Francisco, where he was responsible for advancing the company's pipeline from discovery to clinical development overseeing molecular biology, *in vivo* pharmacology, bioinformatics, and bioanalytics. Prior to joining Audentes, Dr. Mavilio was chief scientific officer of Genethon in Évry, France from 2012 to 2017 where he led the development of a robust pipeline of gene therapy programs for blood, liver, and neuromuscular diseases. Earlier in his career, he held various positions at the Center for Regenerative Medicine of the University of Modena, Molmed SpA, Genera SpA, and the San Raffaele-Telethon Institute of Gene Therapy in Milan, Italy.

Dr. Mavilio is a member of the European Molecular Biology Association, past member of the Board of the American Society of Gene and Cell Therapy and a member of the editorial board of many international journals in the fields of genetics, molecular biology and gene therapy. Dr. Mavilio earned a Ph.D. in medical genetics at the University of Rome School of Medicine and has published more than 200 articles in major international journals. He also serves as Professor of Molecular Biology at the University of Modena and Reggio Emilia in Modena, Italy.

About Nicoletta Loggia, Ph.D., chief technical officer
In her role, Dr. Loggia will be responsible for leading all aspects of technical operations, including process and analytical development, manufacturing,

supply chain, engineering and CMC lifecycle management.

She joins Orchard Therapeutics from Novartis, where she held positions of increasing responsibility since 2004, most recently serving as global head of cell and gene therapies. In this role, Dr. Loggia led multidisciplinary international teams responsible for the end-to-end technical development, manufacturing and project management of several gene and cell therapies modalities, including lentiviral, adeno-associated virus (AAV), stem and CAR-T cell processes, from concept to commercialization. She also oversaw the integration of the CAR-T teams and AveXis' AAV technical capabilities into Novartis' technical R&D. Previously, Dr. Loggia was global head of technical development novel biologic entities and cell and gene therapies where she was responsible for the early phase development of biologics and gene therapies. Her broad industry experience encompasses global leadership in the development and manufacturing of sterile drug products and devices and approvals of several commercial assets.

A medicinal chemist by training, Dr. Loggia began her career in the biopharmaceutical industry as a formulation scientist at Pfizer. She earned her Ph.D. in pharmaceutical technologies from the University of Pavia in Italy. She is a registered pharmacist and member of the UK Royal and Italian Pharmaceutical societies.

About Leslie Meltzer, Ph.D., chief medical officer
Dr. Meltzer joined Orchard Therapeutics in June 2018 bringing extensive experience leading medical affairs for several premier biopharmaceutical companies. Throughout her career, Dr. Meltzer led integration of medical functions across brands including publication planning, field team training, advisory boards, patient registry and identification efforts, investigator-initiated trial prioritization and post-marketing planning. A neuroscientist by training, Dr. Meltzer has dedicated much of her career to advancing new therapies for difficult-to-treat brain diseases. During her tenure at the company, Dr. Meltzer has held positions of increasing responsibility, most recently serving as senior vice president of global medical affairs, diagnostics, clinical operations and data management.

She joined Orchard Therapeutics from Keryx Biopharmaceuticals (prior to its merger with Akebia Therapeutics) where she was the vice president of medical affairs supporting Auryxia® (ferric citrate) for the treatment of chronic kidney disease. She previously served in various positions of increasing seniority in medical affairs at Biogen, where she led key elements of the U.S. launch and post-launch plan for Tecfidera® (dimethyl fumarate) for the treatment of relapsing forms of multiple sclerosis. In recognition of her success and contributions, Dr. Meltzer was promoted to leadership roles in Biogen's multiple sclerosis and then its hemophilia franchises, comprising the company's approved and investigational therapies. Dr. Meltzer began her career at Actelion (now part of Janssen) where she supported four approved and eight investigational therapies in a variety of therapeutic areas, including respiratory, cardiovascular, rheumatology, neurology and rare disease.

Dr. Meltzer earned her Ph.D. in neuroscience from Stanford University School of Medicine.

About Orchard Therapeutics
At Orchard Therapeutics, our vision is to end the devastation caused by genetic and other severe diseases. We aim to do this by discovering, developing and commercializing new treatments that tap into the curative potential of hematopoietic stem cell (HSC) gene therapy. In this approach, a patient's own blood stem cells are genetically modified outside of the body and then reinserted, with the goal of correcting the underlying cause of disease in a single treatment.

In 2018, the company 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. Today, Orchard has a deep pipeline spanning pre-clinical, clinical and commercial stage HSC gene therapies designed to address serious diseases where the burden is immense for patients, families and society 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, including its plans and expectations for the commercialization of Libmeldy, the therapeutic potential of Libmeldy (OTL-200) and Orchard's product candidates, including the product candidates referred to in this release, Orchard's expectations regarding its ongoing preclinical and clinical trials, including the timing of enrollment for clinical trials and release of additional preclinical and clinical data, the likelihood that data from clinical trials will be positive and support further clinical development and regulatory approval of Orchard's product candidates, and Orchard's financial condition and cash runway into the first half of 2023. 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 risk that prior results, such as signals of safety, activity or durability of effect, observed from clinical trials of Libmeldy will not continue or be repeated in our ongoing or planned clinical trials of Libmeldy, will be insufficient to support regulatory submissions or marketing approval in the US or to maintain marketing approval in the EU, or that long-term adverse safety findings may be discovered; the risk that any one or more of Orchard's product candidates, including the product 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 inability or risk of delays in Orchard's ability to commercialize its product candidates, if approved, or Libmeldy, including the risk that Orchard may not secure adequate pricing or reimbursement to support continued development or commercialization of Libmeldy; the risk that the market opportunity for Libmeldy, or any of Orchard's product candidates, may be lower than estimated; and the severity of the impact of the COVID-19 pandemic on Orchard's business, including on clinical development, its supply chain and commercial programs. 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 June 30, 2021, as filed with the U.S. Securities and Exchange Commission (SEC), 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.

Editor's note: Auryxia and Tecfidera are registered trademarks of Akebia Therapeutics, Inc. and Biogen Inc., respectively.

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