



Orchard Therapeutics Secures Partnerships to Broaden Access to Libmeldy™ for Eligible Patients in Middle East & Turkey

January 19, 2021

BOSTON and LONDON, Jan. 19, 2021 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced plans to extend the company's commercial reach in the Middle East and Turkey through exclusive agreements with GenPharm Services and GEN, two leading regional specialty pharmaceutical companies with extensive experience in rare genetic disease. GenPharm and GEN will collaborate with Orchard to facilitate access to treatment with Libmeldy in European-based qualified treatment centers for eligible patients living in the Middle East and Turkey.

Libmeldy was approved in the EU in December 2020 as the first gene therapy for the treatment of early-onset metachromatic leukodystrophy (MLD), an inherited, neurodegenerative disorder. Patients from the Middle East and Turkey were among those who participated in the clinical studies and/or compassionate use programs that supported the marketing authorization for Libmeldy in the EU.

"MLD is a devastating disease found at a higher rate in Middle Eastern and Turkish populations than those in the U.S. and Europe, which heightens our urgency to establish access mechanisms for Libmeldy in support of eligible patients in these regions," said Frank Thomas, president and chief operating officer, Orchard Therapeutics. "Orchard's agreements with GenPharm and GEN represent an innovative way to scale through collaboration, leveraging the pre-existing infrastructure, expertise and relationships of our partners to quickly and efficiently extend our reach to serve patients in these areas of the world."

Under terms of the agreements, GenPharm and GEN will be responsible for all activities related to identifying patients, driving disease awareness, and ensuring market access for Libmeldy in their respective territories. Combined, these partnerships will expand access to Libmeldy for patients living in the following seven additional markets: Saudi Arabia, Kuwait, UAE, Qatar, Bahrain, Oman and Turkey.

"We launched GenPharm Services nearly nine years ago to address significant gaps we saw in meeting rare disease patients' needs in the Middle East region," said Karim Smaira, co-founder of GenPharm Services. "Given the severity of MLD and the lack of any other approved treatment options, we are honored to partner with Orchard Therapeutics to support eligible young MLD patients and their families in getting to Europe for treatment with such an innovative and promising therapy as Libmeldy."

"As a leading specialty pharmaceutical company in Turkey, GEN is excited by the partnership opportunity with Orchard to help Turkish citizens access this groundbreaking gene therapy. We are committed to collaborating with physicians and the government to support the country's eligible MLD patients in navigating a path to receive treatment with Libmeldy," said Abidin Gülmüş, CEO of GEN.

About Libmeldy / OTL-200

Libmeldy (autologous CD34⁺ cell enriched population that contains hematopoietic stem and progenitor cells (HSPC) transduced *ex vivo* using a lentiviral vector encoding the human *arylsulfatase-A* (ARSA) gene), also known as OTL-200, has been approved by the European Commission for the treatment of MLD in eligible early-onset patients characterized by biallelic mutations in the ARSA gene leading to a reduction of the ARSA enzymatic activity in children with i) late infantile or early juvenile forms, without clinical manifestations of the disease, or ii) the early juvenile form, with early clinical manifestations of the disease, who still have the ability to walk independently and before the onset of cognitive decline. Libmeldy is the first therapy approved for eligible patients with early-onset MLD.

The most common adverse reaction attributed to treatment with Libmeldy was the occurrence of anti-ARSA antibodies. In addition to the risks associated with the gene therapy, treatment with Libmeldy is preceded by other medical interventions, namely bone marrow harvest or peripheral blood mobilization and apheresis, followed by myeloablative conditioning, which carry their own risks. During the clinical studies, the safety profiles of these interventions were consistent with their known safety and tolerability.

For more information about Libmeldy, please see the [Summary of Product Characteristics \(SmPC\)](#) available on the European Medicines Agency (EMA) website.

Libmeldy is not approved outside of the European Union, UK, Iceland, Liechtenstein, and Norway. OTL-200 is an investigational therapy in the U.S.

Libmeldy was developed in partnership with the San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget) in Milan, Italy.

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.

About GenPharm Services

GenPharm Services is a regional pharmaceutical company, based in the Dubai Science Park (DSP), UAE, with operations across the Middle East and North Africa (MENA) region. GenPharm is the leading partner in the region in Rare Diseases and Specialty Care.

With an experienced senior management team and a dedicated team of experts, GenPharm provides its partners with market access strategies and sustainable commercial solutions for MENA. For more information, visit .

About GEN

GEN is the leading specialty pharmaceutical company in Turkey with more than 20 years of experience. We partner with global pharmaceutical companies to bring innovative therapies and rare solutions to our community. We work compliant with ethical and scientific principles and strive to set the best standards for quality, safety, and value in the manufacture and access to health care products. With our GMP certificated production facility and R&D center based in Ankara, we offer solutions around the globe in the treatment of rare diseases and disorders. In addition to its HQ and offices in Turkey, GEN has offices in Germany, Russia, Kazakhstan, Uzbekistan, and Azerbaijan. For more information please visit our website at <https://en.genilac.com.tr/>. You can also follow GEN on [LinkedIn](#), [Twitter](#), [Instagram](#), and [Facebook](#).

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 expectations regarding the size of the potential markets for Libmeldy, including its expectations to extend its commercial reach into certain regions through agreements with GenPharm and GEN. 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 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 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; the risk that Orchard's agreement with GenPharm or GEN will not yield the expected results, including that GenPharm or GEN will not be successful at identifying patients, driving disease awareness, or facilitating market access to Libmeldy for eligible individuals in one or more of their respective territories; 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 September 30, 2020, 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.

Contacts

Investors

Renee Leck
Director, Investor Relations
+1 862-242-0764
Renee.Leck@orchard-tx.com

Media

Christine Harrison
Vice President, Corporate Affairs
+1 202-415-0137
media@orchard-tx.com