Orchard Therapeutics Announces Agreement Enabling Access and Reimbursement for Libmeldy for All Eligible MLD Patients in Germany

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BOSTON and LONDON, May 02, 2022 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced it has reached an agreement with Gesetzliche Krankenversicherung Spitzenverband (GKV-SV) which will result in reimbursed access to Libmeldy® (atidarsagene autotemcel) for all metachromatic leukodystrophy (MLD) patients in Germany who fall within the scope of the European marketing authorization.

The agreement follows the completion of a comprehensive assessment during which Libmeldy received the highest possible therapeutic benefit rating for presymptomatic, early-onset patients with MLD by the Federal Joint Committee, also known as Gemeinsame Bundesausschuss (G-BA). To date, only four other medicines have ever received this rating.

“Our agreement with GKV-SV for Libmeldy marks a significant milestone for children with MLD, their caregivers and the entire community,” said Braden Parker, chief commercial officer. “Following similar agreements in the UK and Italy, Germany is the third major European market to recognize the value of Libmeldy commensurate with its clinical impact. We are encouraged by the continued progress we are making across the region to help eligible patients gain access to this therapy.”

As previously disclosed, one German patient has already received Libmeldy at Tübingen University Hospital through an interim reimbursed access arrangement.

Expansion of Patient Identification Efforts
To help identify other eligible patients within the treatment window, Orchard Therapeutics is supporting several newborn screening and diagnostic testing initiatives in the country, including a recently initiated prospective newborn screening study in Hannover, Germany in collaboration with ARCHIMEDlife. This study will expand to include Weiden, Germany.

The company also partnered with ARCHIMEDlife to support its leukodystrophy testing program, which provides no-charge testing to patients in Germany to confirm the underlying cause of symptoms suggestive of specific neurodegenerative diseases, including MLD. This program also offers no-charge testing to siblings of patients who receive a definitive disease diagnosis.

About MLD
MLD is a rare and life-threatening inherited disease of the body’s metabolic system occurring in approximately one in every 100,000 live births. MLD is caused by a mutation in the *arylsulfatase-A* (*ARSA*) gene that results in the accumulation of sulfatides in the brain and other areas of the body, including the liver, gallbladder, kidneys, and/or spleen. Over time, the nervous system is damaged, leading to neurological problems such as motor, behavioral and cognitive regression, severe spasticity and seizures. Patients with MLD gradually lose the ability to move, talk, swallow, eat and see. In its late infantile form, mortality at five years from onset is estimated at 50 percent and 44 percent at 10 years for juvenile patients.[i]

About Libmeldy / OTL-200
Libmeldy (atidarsagene autotemcel), 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 EMA website.

Libmeldy is approved in 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 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 is advancing a 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. Such forward-looking statements may be identified by words such as "anticipates," "believes," "expects," "plans," "intends," "projects," and "future" or similar expressions that are intended to identify forward-looking statements. Except for statements of historical fact, information contained herein constitutes forward-looking statements and may include, but is not limited to, Orchard's expectations regarding the safety and efficacy of Libmeldy, Orchard's ability to establish the infrastructure necessary to enable the treatment of eligible MLD patients, Orchard's ability to identify eligible MLD patients, and the adequacy of Orchard's supply chain and ability to commercialize Libmeldy. 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 Libmeldy will not be successfully commercialized; the risk that any one or more of Orchard's product candidates, including OTL-200, will not be approved, successfully developed or commercialized; the risk that prior results, such as signals of safety, activity or durability of effect, observed from preclinical studies or clinical trials of Orchard's product candidates will not be repeated or continue in ongoing or future studies or trials involving its product candidates; the risk that the market opportunity for Libmeldy or its product candidates may be lower than estimated; and the severity of the impact of the COVID-19 pandemic on Orchard's business, including on preclinical and clinical development, its supply chain and commercial programs. Given these uncertainties, the reader is advised not to place 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 most recent annual or quarterly report 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.

[i] *Mahmood et al. Metachromatic Leukodystrophy: A Case of Triplets with the Late Infantile Variant and a Systematic Review of the Literature. Journal of Child Neurology 2010, DOI:* http://doi.org/10.1177/0883073809341669

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