Orchard Therapeutics and Er-Kim Announce Partnership to Broaden Access to Libmeldy to Eligible Patients in Turkey and Certain Eurasian Countries

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ISTANBUL and LONDON, and BOSTON, Oct. 07, 2024 (GLOBE NEWSWIRE) -- Orchard Therapeutics, recently acquired by Kyowa Kirin with the goal of accelerating the delivery of new gene therapies to patients around the globe and Er-Kim, an international pharmaceutical company specializing in the commercialization of novel therapies, today announced the companies have signed an exclusive distribution agreement to commercialize Libmeldy® (atidarsagene autotemcel), the only approved therapy for eligible children with early-onset metachromatic leukodystrophy (MLD).

Libmeldy aims to correct the underlying genetic cause of MLD, an ultra-rare, rapidly progressive, irreversible and ultimately fatal neurometabolic disease that affects approximately one in 100,000 live births based on existing literature.

Under the agreement, Er-Kim will serve as the exclusive representative of Orchard to identify children eligible for treatment with Libmeldy in international markets across Turkey, Russia, Ukraine, and the Commonwealth of Independent States. Er-Kim will collaborate with Orchard Therapeutics to refer eligible children with early-onset MLD identified in these countries to an established European-based qualified treatment center.

“The pharmaceutical and biotech industries have made notable strides in advancing gene therapies and we are thrilled to partner with Orchard Therapeutics to enable access to this important treatment in the region,” said Cem Zorlular, chief executive officer of Er-Kim. “Libmeldy is a potentially transformative therapy for eligible children with early-onset MLD who previously had no approved treatment options beyond supportive and end-of-life care. We are looking forward to working with Orchard and our counterparts in these territories that we specialize in to further implement bespoke approaches to deliver genetic medicines to eligible patients as soon as possible.”

“MLD is a devastating disease for which there is evidence of a higher incidence rate in Turkish and Eurasian populations than those in the U.S. and Europe, which heightens our urgency to expand access mechanisms for Libmeldy in these regions,” said Robin Kenselaar, senior vice president and general manager of EMEA at Orchard Therapeutics. “With its extensive regional footprint and deep expertise in delivering specialty medicines to patients in need, we look forward to working with our new partners at Er-Kim to broaden access for eligible children and their families.”

About MLD
MLD is a rare and life-threatening inherited disease of the body’s metabolic system estimated to occur in approximately one in every 100,000 live births based on existing literature. 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
Libmeldy® (atidarsagene autotemcel) aims to correct the underlying genetic cause of metachromatic leukodystrophy (MLD) by inserting one or more functional copies of the human *ARSA* gene *ex vivo* (outside the body) into the genome of a patient’s own hematopoietic stem cells (HSCs) using a lentiviral vector. The genetically repaired cells are infused back into the patient, where, once engrafted, they differentiate into multiple cell types, some of which migrate across the blood-brain barrier into the central nervous system and express the functional enzyme.

Prior to treatment, patients must undergo high-dose chemotherapy, a process that removes cells from the bone marrow so they can be replaced with the modified cells in Libmeldy. This approach has the potential to restore enzymatic function to stop or slow disease progression with a single treatment.

Libmeldy is approved by the European Commission (EC), UK Medicines and Healthcare products Regulatory Agency (MHRA), and Swiss Agency for Therapeutic Products (Swissmedic). For more information, please see the Summary of Product Characteristics (SmPC) available on the EMA website.

The program was originated by and developed in partnership with the San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget) in Milan, Italy.

About Er-Kim
Established in 1981, Er-Kim Pharmaceuticals stands at the forefront of biopharmaceutical innovation, partnering with over 40 global leaders to revolutionize patient care in key international markets. Our pioneering business models, tailored for sustainability and flexibility, have positioned us as a full-service solution, extending our reach to over 600 million patients through our fully-owned affiliates. With a dedicated team of over 280 professionals worldwide and revenues exceeding EUR 200M, Er-Kim is not just a partner but a trailblazer in healthcare, continually setting new standards in commercialization and patient access. For more information, please visit http://www.er-kim.com/.

About Orchard Therapeutics
Orchard Therapeutics, a Kyowa Kirin company, is a global gene therapy leader focused on ending the devastation caused by genetic and other severe diseases 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 with a single treatment.

Founded in 2015, Orchard's roots go back to some of the first research and clinical developments involving HSC gene therapy. Our team has played a central role in the evolution of this technology from a promising scientific idea to a potentially life-transforming reality. Today, Orchard is advancing a pipeline of 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.

For more information, please visit www.orchard-tx.com.

About Kyowa Kirin
Kyowa Kirin aims to discover and deliver novel medicines and treatments with life-changing value. As a Japan-based Global Specialty Pharmaceutical Company, we have invested in drug discovery and biotechnology innovation for more than 70 years and are currently working to engineer the next generation of antibodies and cell and gene therapies with the potential to help patients with high unmet medical needs, such as bone & mineral, intractable hematological diseases/hemato oncology, and rare diseases. A shared commitment to our values, to sustainable growth, and to making people smile unites us across the globe. You can learn more about the business of Kyowa Kirin at www.kyowakirin.com.

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[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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