



Orchard Therapeutics Appoints Robin Kenselaar to Chief Commercial Officer and Announces Multiple Business Updates Pertaining to its Approved HSC Gene Therapy for MLD

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First U.S. patients being treated with Lenmeldy™ (atidarsagene autotemcel) in a commercial setting; other launch efforts progressing well following Food and Drug Administration approval last year

Reimbursement agreement for Libmeldy® reached with Spanish National Health System enabling access to all eligible patients in the country

Application to include MLD in its national newborn screening program submitted by patient advocates in Germany

TOKYO and LONDON and BOSTON, Feb. 12, 2025 (GLOBE NEWSWIRE) -- Orchard Therapeutics, a Kyowa Kirin company, today announced the appointment of Robin Kenselaar as chief commercial officer. In this role, Mr. Kenselaar will oversee all aspects of commercial strategy, planning and operations globally, including sales, marketing, market access, and government affairs.

He joined Orchard in 2018, serving as senior vice president and general manager of EMEA, where he led the commercialization strategy and execution in Europe and the Middle East for Libmeldy® following its approval by the European Commission (EC) in December 2020.

"Robin has a wealth of experience and a strong track-record of successfully launching rare disease therapies, including playing a central role in making Libmeldy available to eligible children with metachromatic leukodystrophy following its commercial introduction in Europe," said Frank Thomas, president and chief operating officer of Orchard Therapeutics. "He is an ideal candidate to continue building our commercial team, infrastructure and capabilities as we look to accelerate growth in the U.S. and Europe while exploring future potential regulatory approvals in other regions and territories for MLD and the rest of our portfolio."

Previously, Mr. Kenselaar held positions of increasing responsibility for nearly 15 years at Sanofi Genzyme, where he built and led commercial operations throughout Europe for therapies addressing significant medical needs in rare genetic diseases, oncology and immunology.

"I am honored to be appointed chief commercial officer at a pivotal time for the company," said Robin Kenselaar. "Our hematopoietic stem cell gene therapy approach continues to demonstrate great promise in addressing diseases for which current treatments are limited or do not exist. I look forward to the opportunity to play an even greater role in leading Orchard's efforts to bring these potentially curative therapies to eligible patients and their families around the world."

In addition to the appointment, the company announced multiple updates pertaining to its hematopoietic stem cell (HSC) gene therapy for the treatment of patients with early-onset metachromatic leukodystrophy (MLD)—recently approved as Lenmeldy™ (atidarsagene autotemcel) in the United States (U.S.) and known as Libmeldy® in Europe.

Robin Kenselaar added, "The U.S. launch is building on Orchard's success delivering personalized gene therapies to eligible children throughout Europe and the Middle East by utilizing a similar commercial strategy. Commercial activities in the U.S. are proceeding well with notable progress being made across several key areas pertaining to accelerating diagnosis, treatment delivery and reimbursed access. Ultimately, our goal is to ensure every child diagnosed with MLD who may be eligible for treatment with HSC gene therapy has the access to care he or she deserves."

First U.S. patients being treated in a commercial setting

Multiple U.S. patients with confirmed eligibility for treatment are in the process of receiving therapy with the first infusion of commercial therapy occurring recently. In addition, several children are currently scheduled for infusion or being evaluated for treatment eligibility.

Since the launch, more than 50 patients in the U.S. with early-onset MLD have been considered for treatment; however, most of these patients were too symptomatic to benefit from HSC gene therapy underscoring the need for early detection and diagnosis through newborn screening.

Qualifying specialized treatment centers across the U.S.

In the U.S., Lenmeldy is available to eligible patients through a network of Qualified Treatment Centers (QTCs) across the country. Initially, five treatment centers—with specialized expertise in transplantation and the treatment of neurometabolic diseases, like MLD, as well as experience gaining cross-state reimbursement for Medicaid patients—have been activated.

To date, all five initial QTCs are fully qualified and currently evaluating patient cases. Concurrently, Orchard Therapeutics is assessing additional QTCs in the U.S. with the goal of further minimizing the travel burden on patients and their families.

Enabling timely access and reimbursement

Since its approval, Orchard Therapeutics has been working collaboratively with commercial and government payers to offer outcomes- and value-based agreements intended to ensure timely access by sharing risk between the payer and manufacturer. To date, several commercial and government payers—representing approximately 180 million covered lives—have published favorable coverage policies that are aligned with the U.S. Prescribing Information.

Importantly, to date, all U.S. patients that have been determined to be clinically eligible for HSC gene therapy have been able to access treatment.

Reimbursement agreement reached in Spain

Beyond the U.S. launch, the company continues to expand reimbursed access and support efforts to accelerate diagnosis throughout Europe.

Following similar arrangements made with reimbursement authorities in the United Kingdom (UK), Ireland, Germany, Italy, Sweden, Iceland, Finland, Norway, Belgium and The Netherlands, Orchard Therapeutics has reached an agreement with the Spanish National Health System (SNS) which will result in reimbursed access to Libmeldy for all MLD patients in the country who fall within the scope of the European marketing authorization.

With reimbursed access now secured, the company is working to qualify a seventh European treatment center in Spain.

Advancing newborn screening efforts globally

As with many rare, life-threatening pediatric diseases, early detection and diagnosis is key to ensuring the best possible outcomes for patients, and Orchard Therapeutics supports efforts to expand newborn screening (NBS) for diseases like MLD which meet widely accepted inclusion criteria.

Recently, the Federal Joint Committee, also known as Gemeinsame Bundesausschuss (G-BA), in Germany has accepted an application to consider adding MLD to the country's national NBS program. The application was based, in part, on findings from an investigator-initiated pilot study funded by Orchard Therapeutics and conducted in Hannover, Germany which demonstrated MLD can be reliably screened and diagnosed through NBS.

With the acceptance of the application, a consultation procedure has been initiated by the G-BA, with an initial evaluation by the Institute for Quality and Efficiency in Health Care (IQWiG) ongoing and a final decision expected in December 2027.

The submission of the application in Germany follows news last year that [Norway became the first country in the world](#) to implement national NBS for MLD, which commenced in January 2025.

In the U.S., the Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC), part of the Health Resources and Services Administration (HRSA) within the U.S. Department of Health and Human Services (HHS) unanimously recommended to move the previously submitted nomination to add MLD to the Recommended Uniform Screening Panel (RUSP) to full evidence-based review. As part of this review, the committee will analyze the information presented about the benefit of NBS for MLD. Once a new condition is added to the RUSP, adoption and implementation is then carried out at the state level. As of today, 13 states have RUSP alignment legislation intended to expedite the process of adding new conditions to state NBS panels once approved by the HHS Secretary.

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.¹

About Lenmeldy / Libmeldy

Lenmeldy™ (atidarsagene autotemcel), formerly known as OTL-200, is the only approved therapy in the U.S. for the treatment of children with pre-symptomatic late infantile (PSLI), pre-symptomatic early juvenile (PSEJ) or early-symptomatic early juvenile (ESEJ) metachromatic leukodystrophy (MLD).

For additional details about Lenmeldy, please refer to the full [Prescribing Information](#).

In Europe, Lenmeldy is known as Libmeldy®, where it has been approved by the European Commission (EC) and UK Medicines and Healthcare products Regulatory Agency (MHRA). For more information about Libmeldy, 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.

INDICATION

LENMELDY™ (atidarsagene autotemcel) is an autologous hematopoietic stem cell-based gene therapy indicated for the treatment of children with pre-symptomatic late infantile (PSLI), pre-symptomatic early juvenile (PSEJ), or early symptomatic early juvenile (ESEJ) metachromatic leukodystrophy (MLD).

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS

Thrombosis and Thromboembolic Events:

Treatment with LENMELDY may increase the risk of thrombosis and thromboembolic events. A child with PSEJ MLD died after experiencing a left hemisphere cerebral infarction secondary to a thrombotic event in a large blood vessel approximately 1 year after treatment with LENMELDY. Evaluate the risk factors for thrombosis prior to and after LENMELDY infusion according to best clinical practice.

Encephalitis:

Treatment with LENMELDY may increase the risk of encephalitis. A child with ESEJ developed a serious event of encephalitis after treatment with

LENMELDY. The etiology of this event is unclear but attribution to LENMELDY cannot be ruled out. Treatment with LENMELDY may trigger a relapsing-remitting pattern of disease progression. No other events related to encephalitis have been reported during the clinical development of LENMELDY. Monitor children for signs or symptoms of encephalitis after LENMELDY treatment.

Serious Infection:

In the period between start of conditioning and within 1 year after LENMELDY treatment, severe Grade 3 infections occurred in 39% of all children (21% bacterial, 5% viral, 5% bacterial and viral or bacterial and fungal, and 8% unspecified). Grade 3 febrile neutropenia developed within 1 month after LENMELDY infusion in 82% of children. In the event of febrile neutropenia, monitor for signs and symptoms of infection and manage with broad-spectrum antibiotics, fluids, and other supportive care as medically indicated. Monitor children for signs and symptoms of infection after myeloablative conditioning and LENMELDY infusion and treat appropriately. Administer prophylactic antimicrobials according to best clinical practice.

Veno-Occlusive Disease:

Three children (8%) treated in clinical trials of LENMELDY developed veno-occlusive disease (VOD) with one Grade 4 SAE and two Grade 3 AEs. None of these three events met Hy's Law criteria. Monitor children for signs and symptoms of VOD including liver function tests in all children during the first month after LENMELDY infusion. Consider prophylaxis for VOD with anti-thrombotic agents based on risk factors for VOD and best clinical practice.

Delayed Platelet Engraftment (DPE):

DPE has been observed with LENMELDY treatment. Bleeding risk is increased prior to platelet engraftment and may continue after engraftment in children with prolonged thrombocytopenia. In clinical trials of LENMELDY, 4 (10%) children had delayed platelet engraftment after day 60 (range day 67-109), with 3 children requiring platelet transfusions until engraftment occurred. Patients should be informed of the risk of bleeding until platelet recovery has been achieved. Monitor patients for thrombocytopenia and bleeding until platelet engraftment and recovery are achieved.

Neutrophil Engraftment Failure:

There is a potential risk of neutrophil engraftment failure after treatment with LENMELDY. Monitor neutrophil counts until engraftment has been achieved. If neutrophil engraftment failure occurs in a child treated with LENMELDY, provide rescue treatment with the unmanipulated back-up collection of CD34⁺ cells.

Insertional Oncogenesis:

There is a potential risk of LVV-mediated insertional oncogenesis after treatment with LENMELDY. Children treated with LENMELDY may develop hematologic malignancies and should be monitored life-long. Monitor for hematologic malignancies with a complete blood count (with differential) annually and integration site analysis as warranted for at least 15 years after treatment with LENMELDY. In the event that a malignancy occurs, contact Orchard Therapeutics at 1-888-878-0185 for reporting and to obtain instructions on collection of samples for testing.

Hypersensitivity Reactions:

The dimethyl sulfoxide (DMSO) in LENMELDY may cause hypersensitivity reactions, including anaphylaxis which is potentially life-threatening and requires immediate intervention. Hypersensitivity including anaphylaxis can occur in children with and without prior exposure to DMSO. Monitor for hypersensitivity reactions during infusion and after infusion.

Anti-Retroviral Use:

Children should not take prophylactic HIV anti-retroviral medications for at least one month prior to mobilization, or for the expected duration of time needed for the elimination of the medications. Anti-retroviral medications may interfere with the manufacturing of LENMELDY. If a child requires antiretrovirals for HIV prophylaxis, initiation of LENMELDY treatment should be delayed until confirmation of a negative test for HIV.

Interference With Serology Testing:

Due to the likelihood of a false-positive test for HIV, children who have received LENMELDY should not be screened for HIV infection using a PCR-based assay.

USE IN SPECIFIC POPULATIONS

Females and Males of Reproductive Potential

Pregnancy Testing

As a precautionary measure, a negative serum pregnancy test must be confirmed prior to the start of mobilization, and reconfirmed prior to conditioning procedures, and before administration of LENMELDY in females of childbearing potential.

Contraception

Consult the Prescribing Information of the mobilization and conditioning agents for information on the need for effective contraception. Males capable of fathering a child and females of childbearing age should use an effective method of contraception from start of mobilization through at least 6 months after administration of LENMELDY.

Infertility

There are no data on the effects of LENMELDY on fertility.

Data are available on the risk of infertility with myeloablative conditioning. In clinical trials of LENMELDY, seven children (50% of females) developed ovarian failure. Advise children of the option to cryopreserve semen or ova before treatment, if appropriate.

For additional safety information, please see the full [Prescribing Information](#).

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.

¹Mahmood 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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