# Kyowa Kirin successfully completes acquisition of Orchard Therapeutics, a global gene therapy leader for rare diseases

January 24, 2024

**Acquisition enriches Kyowa Kirin's portfolio, enables the development of numerous promising candidates with a clinically differentiated platform, and helps to resource ongoing and future launches of Libmeldy® (atidarsagene autotemcel) in early-onset MLD**

TOKYO and LONDON and BOSTON, Jan. 24, 2024 (GLOBE NEWSWIRE) -- Kyowa Kirin Co., Ltd. (Kyowa Kirin, TSE: 4151) a Japan-based global specialty pharmaceutical company (J-GSP) discovering and developing novel medicines utilizing the latest biotechnology, and Orchard Therapeutics plc (Orchard Therapeutics, Nasdaq: ORTX), a global gene therapy leader, today announced Kyowa Kirin has successfully completed the acquisition of Orchard Therapeutics. The acquisition of all outstanding shares of Orchard Therapeutics by way of a Scheme of Arrangement procedure under the UK Companies Act 2006 was completed on January 24, 2024, and Orchard Therapeutics has become a wholly-owned subsidiary of Kyowa Kirin. The integrated business will now increase its focus on meeting the needs of people living with devastating genetic and other severe diseases where the burden is immense and current treatment options are limited or do not exist.

Orchard Therapeutics' portfolio comprises Libmeldy ® (atidarsagene autotemcel), which is intended for the treatment of eligible patients with early-onset metachromatic leukodystrophy (MLD), a rare and life-threatening inherited disease of the body's metabolic system. In the most severe form of MLD, babies develop normally but in late infancy start to rapidly lose the ability to walk, talk and interact with the world around them. Libmeldy is approved by the European Commission (EC) and UK Medicines and Healthcare products Regulatory Agency (MHRA). Libmeldy is known as OTL-200 in the U.S., where it is currently an investigational drug under Priority Review by the Food and Drug Administration (FDA) with a Prescription Drug User Fee Act (PDUFA) goal date of March 18, 2024.

Using the same hematopoietic stem cell (HSC) gene therapy technology platform, Orchard Therapeutics is also progressing two clinical-stage programs, OTL-203 for the treatment of mucopolysaccharidosis type I Hurler's syndrome (MPS-IH) and OTL-201 in development for mucopolysaccharidosis type IIIA (MPS-IIIA), also known as Sanfilippo syndrome.

"We are truly excited about the acquisition of Orchard Therapeutics, a leading provider of HSC gene therapy. This platform offers significant potential to deliver more innovative treatments and breakthrough therapies and aligns with our purpose to deliver life-changing value for people living with rare and complex diseases," said Masashi Miyamoto, Ph.D., Representative Director, President and CEO of Kyowa Kirin. "Going forward, our companies will build on the extensive experience of Orchard's gene therapy platform and apply it to under-served indications and diseases where we believe it to be scientifically and clinically differentiated."

"We look forward to this next chapter in Orchard Therapeutics' evolution and are eager to partner with our new colleagues at Kyowa Kirin to unlock the full potential of our HSC gene therapy approach," said Bobby Gaspar, co-founder and chief executive officer of Orchard Therapeutics. "The next 12 months have the potential to provide several breakout opportunities that we believe would cement our leadership position in the field, including the potential approval and launch of OTL-200 in the U.S., the acceleration of Libmeldy growth in Europe, the progression of our global registrational trial for OTL-203 in MPS-IH, as well as the advancement of our next-in-line neurometabolic program in MPS-IIIA and earlier-stage research programs."

In the new organizational structure, Bobby Gaspar, M.D., Ph.D., will report to Kyowa Kirin President & CEO Masashi Miyamoto, Ph.D., and become a member of the Kyowa Kirin's senior R&D leadership team, helping the organization evaluate next-generation therapeutic candidates. Members of the Orchard Therapeutics team will continue to operate from its existing facilities in London and Boston. Kyowa Kirin anticipates significant synergies with Orchard Therapeutics and plans to announce its 2024 Financial Guidance in conjunction with its 2023 earnings on 7 February.

**Overview of the transaction**
Under the terms of the agreement, Kyowa Kirin completed the scheme of arrangement to acquire all outstanding shares of Orchard Therapeutics at a price of $16.00 per American Depositary Share (ADS) in cash which represents a premium of 144% to Orchard Therapeutics' volume-weighted average price per ADS over the 30 days ended October 4, 2023, the day before the transaction was announced.

In connection with the transaction, an additional contingent value right (CVR) of $1.00 per ADS is payable to Orchard shareholders for a total of $17.00 per ADS. The additional CVR payments are contingent and payable only on U.S. approval of OTL-200 in 2024 per the terms of the CVR agreement.

**Overview of Orchard Therapeutics**

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| (1) Name | Orchard Therapeutics plc |
| (2) Location | 245 Hammersmith Road, 3rd Floor London W6 8PW United Kingdom |

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| (3) Job title and name of representative | Chief Executive Officer<br>Bobby Gaspar |
| (4) Description of business | Development and commercialization of hematopoietic stem cell gene therapy |
| (5) Share capital | $29,463 thousand (as of September 30, 2023) |
| (6) Date of establishment | 2015 |

**About Libmeldy / OTL-200**

Libmeldy® (atidarsagene autotemcel), also known as OTL-200, has been approved by the European Commission for the treatment of metachromatic leukodystrophy (MLD) in 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 peripheral blood mobilization and apheresis, followed by myeloablative conditioning, which carry their own risks. During the clinical studies of Libmeldy, 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 Kyowa Kirin**

Kyowa Kirin aims to discover novel medicines 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 affected by a severe or rare disease. A shared commitment to our values, to sustainable growth, and to making people smile unites us across our four regions – Japan, Asia Pacific, North America, and EMEA/International. You can learn more about the business of Kyowa Kirin at: https://www.kyowakirin.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.

**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, 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 forward-looking statements, which are made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. All statements that are not statements of historical facts are, or may be deemed to be, forward-looking statements. 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, including signals of safety and efficacy, will not be replicated or will not continue in ongoing or future studies and the risk that long-term adverse safety findings may be discovered. 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 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.

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