



## Orchard Therapeutics Announces Multiple Presentations at the 20th Annual WORLDSymposium

February 2, 2024

### Three oral presentations and seven posters highlight transformative potential of HSC gene therapy and newborn screening to address severe neurometabolic diseases

BOSTON and LONDON, Feb. 02, 2024 (GLOBE NEWSWIRE) -- Orchard Therapeutics, a global gene therapy leader recently acquired by Kyowa Kirin with the goal of accelerating the delivery of new gene therapies to patients around the globe, today announced 10 presentations from across its neurometabolic portfolio will be featured at the 20th Annual WORLDSymposium™ taking place February 4-9, 2024, in San Diego, California.

Featured presentations include updated neurocognitive and biochemical results from an ongoing proof-of-concept study of investigational OTL-201 in mucopolysaccharidosis type IIIA (MPS-III A), also known as Sanfilippo syndrome type A, with median follow-up now extending to 2.75 years (ranging from 24 to 39 months). Additional data highlights include several accepted abstracts detailing newborn screening efforts to support the timely and accurate diagnosis of metachromatic leukodystrophy (MLD), as well as an encore presentation showcasing long-term results from an updated integrated analysis of 39 patients with MLD treated with investigational OTL-200, which comprises key components of the clinical data package of the Biologics License Application (BLA) currently under Priority Review by the U.S. Food and Drug Administration.

In addition to the data presentations outlined below, the company will also host a sponsored symposium on Monday, February 5, at 11:45 a.m., titled "Momentum, Leadership & Diagnosis: Changing the paradigm for MLD," featuring expert speakers from different regions sharing their knowledge and experience to establish new standards and advance newborn screening through interdisciplinary collaboration.

Oral presentation details are as follows (all times in PST; \* denotes corresponding poster):

- Title: Clinical outcomes and sustained biochemical engraftment following ex-vivo autologous stem cell gene therapy for Mucopolysaccharidosis Type IIIA\*  
Date/Time: Wednesday, February 7 at 8:12 a.m.  
Presenter: Simon Jones
- Title: Atidarsagene autotemcel (autologous hematopoietic stem cell gene therapy) preserves cognitive and motor development in early-onset metachromatic leukodystrophy with up to 12 years follow-up\*  
Date/Time: Wednesday, February 7 at 8:24 a.m.  
Presenter: Francesca Fumagalli
- Title: Results of Prospective Newborn Screening for Metachromatic leukodystrophy in Germany and Austria  
Date/Time: Friday, February 9 at 8:36 a.m.  
Presenter: Petra Oliva

Poster presentation details are as follows (all times in PST; \* denotes corresponding oral presentation):

- Title: A UK-based pre-pilot newborn screening study for Metachromatic Leukodystrophy identified a late infantile case  
Date/Time: Tuesday, February 6 from 3:00-5:00 p.m.  
Presenting Author: Teresa Wu  
Poster #: 349
- Title: Atidarsagene autotemcel (autologous hematopoietic stem cell gene therapy) preserves cognitive and motor development in early-onset metachromatic leukodystrophy with up to 12 years follow-up\*  
Date/Time: Wednesday, February 7 from 3:00-5:00 p.m.  
Presenting Author: Francesca Fumagalli  
Poster #: 092
- Title: Lentiviral hematopoietic stem cell gene therapy (atidarsagene autotemcel) for late juvenile Metachromatic leukodystrophy (MLD)  
Date/Time: Wednesday, February 7 from 3:00-5:00 p.m.  
Presenter: Francesca Fumagalli  
Poster #: 096
- Title: Compassionate use of lentiviral gene therapy for metachromatic leukodystrophy  
Date/Time: Wednesday, February 7 from 3:00-5:00 p.m.  
Presenting Author: Paul Orchard

Poster #: 250

- Title: Design of a multi-center randomized active controlled phase 3 clinical trial (HURCULES) evaluating the safety and efficacy of OTL-203 in patients with MPS-IH versus standard of care with allogeneic hematopoietic stem cell transplantation  
Date/Time: Wednesday, February 7 from 3:00-5:00 p.m.  
Presenting Author: Paul Orchard  
Poster #: 249
- Title: Clinical outcomes and sustained biochemical engraftment following ex-vivo autologous stem cell gene therapy for Mucopolysaccharidosis Type IIIA\*  
Date/Time: Wednesday, February 7 from 3:00-5:00 p.m.  
Presenting Author: Simon Jones  
Poster #: 166
- Title: Cost-effectiveness framework by tandem mass spectrometry (TMS) for newborn screening of Metachromatic Leukodystrophy (MLD) in the United States (US)  
Date/Time: Thursday, February 8 from 3:00-5:00 p.m.  
Presenting Author: Karen Bean  
Poster #: 030

#### **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](http://www.orchard-tx.com).

#### **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 and rare diseases. 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>.

Contact Benjamin Navon +1 857-248-9454 Benjamin.Navon@orchard-tx.com