Orchard Therapeutics Strongly Supports Enactment of Newborn Metabolic Screening Act in Illinois, Adding Metachromatic Leukodystrophy to Statewide Panel

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BOSTON and LONDON, Aug. 02, 2023 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, expresses its strong appreciation for the passage of the Newborn Metabolic Screening Act in Illinois, also known as SB67. The law, anticipated to go into effect in January 2024, mandates the expansion of the Illinois Department of Public Health's Newborn Screening program to include metachromatic leukodystrophy (MLD) for the estimated 130,000 newborns in the state each year. Illinois becomes the first state in the U.S. to add newborn screening for MLD to its statewide panel.

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. These children may require 24-hour care, and the majority pass away within five years of disease onset, creating an enormous burden on patients, their families and health care systems. There are currently no approved treatment options in the U.S. for MLD beyond supportive care.

"The enactment of SB67 represents the culmination of a multi-stakeholder initiative working together to expand screening for MLD and represents a significant milestone for newborn screening in the U.S.," said Leslie Meltzer, Ph.D., chief medical officer of Orchard Therapeutics. "As with many rare, life-threatening diseases, early detection and diagnosis is key to ensuring the best possible outcomes for patients. This significant milestone by Illinois will ensure the state keeps up the pace of diagnoses to coincide with biomedical innovation. We are highly encouraged by this progress and look forward to continuing to work with families, physicians and the broader community to actively lay the groundwork for the implementation of universal MLD screening in other states."

Newborn screening is widely considered one of the most successful public health programs worldwide. In the U.S., approximately 1 in 300 newborns have a condition that can be diagnosed through newborn screening, and more than 12,000 infants receive life-saving treatment due to this public health program annually, according to the Centers of Disease Control and Prevention.

"Today is a monumental day for the MLD community and I commend Illinois for being the first state in the nation to initiate screening for this devastating disease," said Barbara Burton, M.D., attending physician, genetics, genomics and metabolism at the Ann & Robert H. Lurie Children's Hospital of Chicago. "I am proud to have played a small role in making this happen and envision a day, not too far away, when, much like polio, physicians will no longer know what MLD looks like because all babies will be screened and those who test positive can be diagnosed and referred for appropriate treatment before the onset of symptoms."

Orchard Therapeutics is proud to support newborn screening research to generate the data necessary to enable the implementation of universal screening for MLD. In addition to SB67, the company is supporting several newborn screening initiatives, including:

- Nine prospective newborn screening studies are active throughout Europe, the U.S. and the Middle East. By the end of 2023, more than 200,000 newborns are expected to have been screened.
    - Four confirmed cases of MLD have been identified following the screening of approximately 150,000 newborns globally as of June 30.
    - These preliminary findings suggest an incidence rate closer to one in 50,000 live births versus prior estimates in literature of one in 100,000.[i]
- Orchard is a founding member of BeginNGS at Rady Children's Institute for Genomic Medicine, San Diego, where cutting-edge genomic technology will be used to continue to advance the science of newborn screening.
- In Germany, following the positive identification of three newborns with MLD from a prospective study, progress has been made toward an application for nationwide screening.

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.[ii]

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's 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 (SEC) 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. Such forward-looking statements may be identified by words such as "anticipated," "expected," and other similar expressions. Forward-looking statements include express or implied statements relating to, among other things: the anticipated timing of MLD's inclusion in the Illinois Department of Public Health's Newborn Screening program; the number of newborns expected to be screened by the end of 2023; the timing of additional newborn screening initiatives; the incidence rate of MLD suggested by newborn screening; and, the therapeutic potential of OTL-200.

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 the incidence rate of MLD is lower than estimated, which additional newborn screening may demonstrate; the risk that additional newborn screening efforts are delayed or do not occur; and the risk that long-term adverse safety findings may be discovered for OTL-200. 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 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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[i] *von Figura K, Jaeken J. Metachromatic leukodystrophy. In: Scriver CR, Valle D, WS S, eds. The metabolic and molecular bases of inherited diseases. Mac Graw-Hill; 2001:3695-3724, chap. 148.*

[ii] *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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