



Sentynl Therapeutics Completes Asset Transfer of CUTX-101 Copper Histidinate Product Candidate for Treatment of Menkes Disease from Cyprium Therapeutics

Sentynl receives worldwide proprietary rights and rolling New Drug Application with U.S. FDA pertaining to CUTX-101 copper histidinate

CUTX-101 has potential to be the first FDA-approved treatment for Menkes disease; rolling submission of New Drug Application to FDA is ongoing and expected to be completed in 2024

Solana Beach, CA and Ahmedabad, India – December 6, 2023 — [Sentynl Therapeutics, Inc.](#) (Sentynl), a U.S.-based biopharmaceutical company wholly-owned by Zydus Lifesciences, Ltd. (Zydus Group), today announced the execution of an Assignment and Assumption Agreement with Cyprium Therapeutics, Inc. (Cyprium), a Fortress Biotech, Inc. (Nasdaq: FBIO) (Fortress) subsidiary company. Under the agreement, Cyprium completed the transfer of its worldwide proprietary rights and rolling New Drug Application (NDA) pertaining to CUTX-101, the copper histidinate product candidate for the treatment of Menkes disease, to Sentynl.

Sentynl now assumes full responsibility for the development and commercialization of CUTX-101. In 2021, Sentynl and Cyprium reported positive results from a safety and efficacy analysis of data integrated from two completed pivotal studies in patients with Menkes disease treated with CUTX-101. A rolling submission of CUTX-101 to the FDA is ongoing, with expected completion in 2024.

Speaking on the development, Dr. Sharvil Patel, Managing Director, Zydus Lifesciences said, “We have been committed to providing access to path-breaking discoveries that can bridge unmet healthcare needs, globally. The rights for CUTX-101 is a significant milestone towards our vision to transform lives and meaningfully impact patients, healthcare providers and the rare disease community at large. This novel, breakthrough therapy could unlock possibilities for the treatment of the life threatening Menkes disease.”

“Menkes disease has a devastating impact on patients and their caregivers. With no current approved treatments, death usually occurs between 2-3 years of age,” said Matt Heck, President & Chief Executive Officer of Sentynl. “We are committed to advancing CUTX-101, which has the potential to not only become the first FDA-approved treatment for Menkes disease, but also positively impact the lives of the patients and their caregivers affected by this rare, fatal condition.”

“We know first-hand the difficult journey that patients and families of Menkes disease face,” commented Drew and Jamie Eckman, Founders of the Menkes Foundation and parents of Wesley, who was diagnosed with Menkes disease at 8.5 months old and passed away three months later. “The development of CUTX-101 represents incredible progress in the fight against Menkes disease. We hold a strong amount of hope for a time when there is an option available to treat this condition, providing a path forward for caregivers and their children.”

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About CUTX-101 (Copper Histidinate)

CUTX-101 is an investigational drug currently under a rolling NDA submission with FDA to treat patients with Menkes disease. CUTX-101 is a subcutaneous injectable formulation of copper histidinate manufactured under current good manufacturing practice (“cGMP”) that is intended to improve tolerability due to its physiological pH. In a Phase 1/2 clinical trial conducted by Stephen G. Kaler, M.D., M.P.H., at the National Institutes of Health (“NIH”), early treatment of patients with Menkes disease with CUTX-101 led to

an improvement in neurodevelopmental outcomes and survival. Cyprium previously reported positive topline clinical efficacy results for CUTX-101, demonstrating statistically significant improvement in overall survival for Menkes disease subjects who received early treatment (ET) with CUTX-101, compared to an untreated historical control cohort, with a nearly 80% reduction in the risk of death. Median overall survival (OS) was 177.1 months for CUTX-101 ET cohort compared to 16.1 months for the untreated historical control cohort. CUTX-101 has been granted FDA Breakthrough Therapy, Fast Track, Rare Pediatric Disease and FDA Orphan Drug Designations. Additionally, the European Medicines Agency granted Orphan Designation for CUTX-101. An [expanded access](#) protocol for patients with Menkes disease is ongoing at multiple U.S. medical centers and will be managed by Sentyln after the transfer.

About Menkes Disease

Menkes disease is a rare X-linked recessive pediatric disease caused by gene mutations of the copper transporter ATP7A. The minimum birth prevalence for Menkes disease is believed to be 1 in 34,810 live male births, and potentially as high as 1 in 8,664 live male births, based on recent genome-based ascertainment (Kaler SG, Ferreira CR, Yam LS. Estimated birth prevalence of Menkes disease and ATP7A-related disorders based on the Genome Aggregation Database (gnomAD). *Molecular Genetics and Metabolism Reports* 2020 June 5;24:100602). The condition is characterized by distinctive clinical features, including sparse and depigmented hair (“kinky hair”), connective tissue problems, and severe neurological symptoms such as seizures, hypotonia, failure to thrive, and neurodevelopmental delays. Mortality is high in untreated Menkes disease, with many patients dying between 2-3 years of age. Milder versions of *ATP7A* mutations are associated with other conditions, including Occipital Horn Syndrome and ATP7A-related Distal Motor Neuropathy. Currently, there is no FDA-approved treatment for Menkes disease and its variants.

About Sentyln Therapeutics

Sentyln Therapeutics is a U.S.-based biopharmaceutical company focused on bringing innovative therapies to patients living with rare diseases. The company was acquired by the Zydus Group in 2017. Sentyln's experienced management team has previously built multiple successful pharmaceutical companies. With a focus on commercialization, Sentyln looks to source effective and well-differentiated products across a broad spectrum of therapeutic areas to address unmet needs. Sentyln is committed to the highest ethical standards and compliance with all applicable laws, regulations and industry guidelines. For more information, visit <https://sentyln.com>.

About Zydus

Zydus Lifesciences Ltd. with an overarching purpose of empowering people with freedom to live healthier and more fulfilled lives, is an innovative, global lifesciences company that discovers, develops, manufactures, and markets a broad range of healthcare therapies. The group has a significant presence in cancer related therapies and offers a wide range of solutions with cytotoxic, supportive & targeted drugs. The group employs nearly 25,000 people worldwide, including 1,400 scientists engaged in R & D, and is driven by its mission to unlock new possibilities in lifesciences through quality healthcare solutions that impact lives. The group aspires to transform lives through path-breaking discoveries. For more information, visit <https://www.zyduslife.com/zyduslife/>

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