



## **Fortress Biotech and Cyprium Therapeutics alongwith ZyduS' Sentyln Therapeutics Announce CUTX-101, Copper Histidinate Data to be Presented at 2022 American College of Medical Genetics Annual Clinical Genetics Meeting**

*CUTX-101 has potential to be first FDA-approved treatment for Menkes disease; rolling submission of New Drug Application to FDA is ongoing and expected to be completed in mid-year 2022*

*Cyprium Therapeutics, a subsidiary of Fortress Biotech, with support from Sentyln Therapeutics is developing CUTX-101 for the treatment of Menkes disease*

*Ahmedabad, India, March 21, 2022*

Cyprium Therapeutics, Inc. (“Cyprium”), a Fortress Biotech, Inc. (Nasdaq: FBIO) (“Fortress”) subsidiary, with support from its licensing partner Sentyln Therapeutics, Inc. (“Sentyln”), a wholly owned subsidiary of ZyduS Lifesciences Ltd. (formerly known as Cadila Healthcare Ltd.), today announced positive data on CUTX-101, copper histidinate (CuHis), in patients with Menkes disease. The data will be presented as a “Top-Rated Abstract” and Poster at the 2022 American College of Medical Genetics and Genomics (“ACMG”) Annual Clinical Genetics Meeting taking place March 22-26, 2022, virtually and at Music City Center in Nashville, TN. The [previously reported results](#) are from an efficacy and safety analysis of data integrated from two completed pivotal studies in patients with Menkes disease treated with CUTX-101.

Details of the poster are as follows:

**Poster Title:** Safety and Efficacy of Copper Histidinate (CUTX-101) Treatment for Menkes Disease Caused by Severe Loss-of-Function Variants in ATP7A

**Poster Number:** eP195

**Authors:** Stephen G. Kaler, M.D., M.P.H., Shama Munim, M.S., Michael Chen, Ph.D., Robert Niecestro, Ph.D., Lung S. Yam, M.D., Ph.D.

**Dates / Times:** Posters will be available for viewing on Wednesday, March 23, 5:00 p.m. – 7:00 p.m., Thursday, March 24, 9:30 a.m. - 4:30 p.m. and Friday, March 25, 10:00 a.m. – 1:00 p.m. in the Exhibit Hall. Dr. Kaler will formally present the poster on Thursday, March 24 from 10:00 a.m. – 11:30 a.m. CT.

The abstract can be viewed [here](#).

“The positive data that will be presented at the 2022 ACMG Annual Clinical Genetics meeting demonstrate the efficacy and safety of CUTX-101 and its potential to be the first treatment approved by the U.S. Food and Drug Administration (“FDA”) for patients with Menkes disease. We continue to make progress with our rolling submission of a new drug application (“NDA”) for CUTX-101 which we anticipate to be completed in the middle of this year,” said Lung S. Yam, M.D., Ph.D., President and Chief Executive Officer of Cyprium. “We welcome the opportunity to

present the positive efficacy and safety data of CUTX-101 to medical geneticists who are often involved in the diagnosis and treatment of Menkes disease, a rare, fatal pediatric disease.”

In 2021, Cyprium partnered with Sentyln Therapeutics, Inc., a U.S.-based specialty pharmaceutical company owned by the Zydus Group, to bring CUTX-101 to market. Cyprium will retain development responsibility of CUTX-101 through approval of the NDA by the FDA, and Sentyln will be responsible for commercialization of CUTX-101 as well as progressing newborn screening activities.

Zydus’ wholly owned subsidiary Sentyln Therapeutics is focused on empowering patients suffering from rare diseases. The positive data on CUTX-101, copper histidinate (CuHis) is a positive step in this direction. The group company recently entered into an asset purchase agreement for the purchase of BridgeBio’s NULIBRY™ (Fosdenopterin) for Injection to reduce the risk of mortality in patients with molybdenum cofactor deficiency (MoCD) Type A, an ultra-rare, life-threatening pediatric genetic disorder.

### **About Menkes Disease**

Menkes disease is a rare X-linked recessive pediatric disease caused by gene mutations of 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 before the age of three years old. 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 CUTX-101 (Copper Histidinate)**

CUTX-101 is in clinical development to treat patients with Menkes disease. CUTX-101 is a subcutaneous injectable formulation of Copper Histidinate manufactured under current good manufacturing practice (“cGMP”) and 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. In August 2020, Cyprium 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. 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 Drug Designation for CUTX-101. A Cyprium-sponsored [expanded access](#) protocol for patients with Menkes disease is ongoing at multiple U.S. medical centers.

## **About Zydus**

The Zydus Group, 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 employs over 23000 people worldwide and is driven by its mission to unlock new possibilities in lifesciences through quality healthcare solutions that impact lives. The group aspires to become a global lifesciences company transforming lives through path-breaking discoveries. For more information, visit <https://www.zyduslife.com/zyduslife/>

## **About Sentyln Therapeutics**

Sentyln Therapeutics is a U.S.-based biopharmaceutical focused on bringing innovative therapies to patients living with rare diseases. The company was acquired by the Zydus Group in 2017. Sentyln's highly 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 [www.sentyln.com](http://www.sentyln.com).

## **About Cyprium Therapeutics**

Cyprium Therapeutics, Inc. ("Cyprium") is focused on the development of novel therapies for the treatment of Menkes disease and related copper metabolism disorders. In March 2017, Cyprium entered into a Cooperative Research and Development Agreement ("CRADA") with the Eunice Kennedy Shriver National Institute of Child Health and Human Development ("NICHD"), part of the NIH, to advance the clinical development of CUTX-101 (Copper Histidinate injection) for the treatment of Menkes disease. In addition, Cyprium and NICHD entered into a worldwide, exclusive license agreement to develop and commercialize adeno-associated virus (AAV)-based gene therapy, called AAV-ATP7A, to deliver working copies of the copper transporter that is defective in patients with Menkes disease, and to be used in combination with CUTX-101. CUTX-101 was granted FDA Breakthrough Therapy, Fast Track and Rare Pediatric Disease Designations, and both CUTX-101 and AAV-ATP7A have received FDA Orphan Drug Designation previously. Cyprium was founded by Fortress Biotech, Inc. (Nasdaq: FBIO) and is based in New York City. For more information, visit [www.cypriumtx.com](http://www.cypriumtx.com).

## **About Fortress Biotech**

Fortress Biotech, Inc. ("Fortress") is an innovative biopharmaceutical company focused on acquiring, developing and commercializing high-potential marketed and development-stage drugs and drug candidates. The company has nine marketed prescription pharmaceutical products and over 30 programs in development at Fortress, at its majority-owned and majority-controlled partners and at partners it founded and in which it holds significant minority ownership positions. Such product candidates span six large-market areas, including oncology, rare diseases and gene therapy, which allow it to create value for shareholders. Fortress advances its diversified pipeline through a streamlined operating structure that fosters efficient drug development. The Fortress model is driven by a world-class business development team that is focused on leveraging its significant biopharmaceutical industry expertise to further expand the company's portfolio of product opportunities. Fortress has established partnerships with some of the world's leading academic research institutions and biopharmaceutical companies to maximize each opportunity to its full potential, including AstraZeneca plc, City of Hope, Fred Hutchinson Cancer Research Center, St. Jude Children's Research Hospital, Nationwide Children's Hospital and Sentyln Therapeutics, Inc. For more information, visit [www.fortressbiotech.com](http://www.fortressbiotech.com).