Fortress Biotech, Cyprium Therapeutics and Sentynl Therapeutics Announce CUTX-101, Copper Histidinate, Data to be Presented at 2022 American College of Medical Genetics Annual Clinical Genetics Meeting

Cyprium Therapeutics, a subsidiry of Fortress Biotech, is developing CUTX-101 for the treatment of Menkes disease

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

Miami, FL, and Solana Beach, CA, March 21, 2022 – Cyprium Therapeutics, Inc. ("Cyprium"), a Fortress Biotech, Inc. (Nasdaq: FBIO) ("Fortress") subsidiary, with support from its licensing partner Sentynl Therapeutics, Inc. ("Sentynl"), 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 Sentynl 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 Sentynl will be responsible for commercialization of CUTX-101 as well as progressing newborn screening activities.

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 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. Additionally, the European Medicines Agency previously granted Orphan Drug Designation to CUTX-101. Cyprium was founded by Fortress Biotech, Inc. (Nasdaq: FBIO) and is based in New York City. For more information, visit 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 Sentynl Therapeutics, Inc. For more information, visit www.fortressbiotech.com.

About Sentynl Therapeutics

Sentynl 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. Sentynl's highly experienced management team has previously built multiple successful pharmaceutical companies. With a focus on commercialization, Sentynl looks to source effective and well differentiated products across a broad spectrum of therapeutic areas to address unmet needs. Sentynl is committed to the highest ethical standards and compliance with all applicable laws, regulations, and industry guidelines. For more information, visit www.sentynl.com.

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 pharmaceutical 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 life-sciences through quality healthcare solutions that impact lives. The group aspires to become a global life-sciences company transforming lives through pathbreaking discoveries. For more information, visit https://www.zyduslife.com/zyduslife/

Forward-Looking Statements

This press release may contain "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934, as amended. As used below and throughout this press release, the words "we", "us" and "our" may refer to Fortress individually or together with one or more partner companies, as dictated by context. Such statements include, but are not limited to, any statements relating to our growth strategy and product development programs and any other statements that are not historical facts. Forward-looking statements are based on management's current expectations and are subject to risks and uncertainties that could negatively affect our business, operating results, financial condition and stock price. Factors that could cause actual results to differ materially from those currently anticipated include: risks relating to our growth strategy; our ability to obtain, perform under and maintain financing and strategic agreements and relationships; risks relating to the results of research and development activities; uncertainties relating to preclinical and clinical testing; risks relating to the timing of starting and completing clinical trials, including the possible disruption of trials due to the hostilities in Europe; our dependence on third-party suppliers; risks relating to the COVID-19 outbreak and its potential impact on our employees' and consultants' ability to complete work in a timely manner and on our ability to obtain additional financing on favorable terms or at all; our ability to attract, integrate and retain key personnel; the early stage of products under development; our need for substantial additional funds; government regulation; patent and intellectual property matters; competition; as well as other risks described in our Securities and Exchange Commission filings. We expressly disclaim any obligation or undertaking to release publicly any updates or revisions to any forward-looking statements contained herein to reflect any change in our expectations or any changes in events, conditions or circumstances on which any such statement is based, except as may be required by law, and we claim the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995. The information contained herein is intended to be reviewed in its totality, and any stipulations, conditions or provisos

that apply to a given piece of information in one part of this press release should be read as applying *mutatis mutandis* to every other instance of such information appearing herein.

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