Fortress Biotech, Cyprium Therapeutics and Sentynl Therapeutics Announce the Initiation of Rolling Submission of a New Drug Application for CUTX-101, Copper Histidinate, for Treatment of Menkes Disease

New York, NY, and Solana Beach, CA, December 7, 2021 – Cyprium Therapeutics, Inc. ("Cyprium"), a Fortress Biotech, Inc. (Nasdaq: FBIO) ("Fortress") partner company, with support from its licensing partner Sentynl Therapeutics, Inc. ("Sentynl"), a wholly owned subsidiary of Cadila Healthcare Limited ("Zydus"), today announced the initiation of a rolling submission of a New Drug Application ("NDA") to the U.S. Food and Drug Administration ("FDA") for CUTX-101 ("Copper Histidinate") for the treatment of Menkes disease. The companies expect to complete the submission of the NDA to the FDA in mid-2022.

In October 2021, Cyprium announced 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. These data were presented as a virtual poster at the recent 2021 American Academy of Pediatrics National Conference & Exhibition. CUTX-101 was previously granted FDA Breakthrough Therapy, Fast Track, Rare Pediatric Disease and FDA Orphan Drug Designations. Additionally, the European Medicines Agency previously granted Orphan Drug Designation for CUTX-101.

"We are very pleased to have initiated the rolling submission of our NDA to the FDA for CUTX-101, an important milestone for Cyprium. In clinical trials, CUTX-101 was well tolerated and demonstrated a clinically meaningful efficacy profile and has the potential to be the first FDA-approved treatment for Menkes disease. If approved, CUTX-101 will fill a significant unmet need for children suffering from this rare, fatal pediatric disease," said Lung S. Yam, M.D., Ph.D., President and Chief Executive Officer of Cyprium.

Stephen G. Kaler, M.D., M.P.H., a physician-scientist at the Center for Gene Therapy in the Abigail Wexner Research Institute at Nationwide Children's Hospital, Principal Investigator of the clinical trials and professor of Pediatrics and Genetics at The Ohio State University College of Medicine, said, "I am extremely grateful to the teams at Cyprium and Fortress that prepared and initiated this rolling submission. We especially thank the devoted parents of Menkes disease patients who participated in the clinical studies summarized in this NDA, which represents a major step in the battle against this illness."

Earlier this year, Sentynl and Cyprium executed an asset purchase agreement pursuant to which Sentynl will acquire Cyprium's proprietary rights to CUTX-101 upon FDA approval thereof. Under the terms of the agreement, Cyprium is eligible to receive up to $20 million in upfront development and regulatory cash milestones through NDA approval. Royalties on CUTX-101 net sales ranging from the mid-single digits up to the mid-twenties are also payable, as well as potential sales milestones. Cyprium will retain 100% ownership over any FDA priority review voucher that may be issued at NDA approval for CUTX-101. Cyprium continues to maintain development responsibility for CUTX-101 through approval of the NDA by the FDA, and Sentynl is responsible for commercialization of CUTX-101 as well as progressing newborn screening activities for Menkes disease in the U.S. The development of CUTX-101 is overseen by a Joint Steering Committee consisting of representatives from both companies.

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. 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 that was ranked in Deloitte's 2019 and 2020 Technology Fast 500™, annual rankings of the fastest-growing North American companies in the technology, media, telecommunications, life sciences and energy tech sectors, based on percentages of fiscal year revenue growth over three-year periods. Fortress is focused on acquiring, developing and commercializing high-potential marketed and development-stage drugs and drug candidates. The company has seven marketed prescription pharmaceutical products and over 25 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

Zydus is an innovative, global pharmaceutical company that discovers, develops, manufactures and markets a broad range of healthcare therapies, including small molecule drugs, biologic therapeutics and vaccines. The group employs over 23,000 people worldwide, including 1,400 scientists engaged in R&D, and is dedicated to creating healthier communities globally. For more information, visit www.zyduscadila.com.

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; 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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