Fortress Biotech Announces Positive Topline Clinical Efficacy Results for CUTX-101, Copper Histidinate, for the Treatment of Menkes Disease

Statistically Significant Improvement in the Primary Endpoint of Overall Survival in Menkes Disease Patients who Received Early Treatment with CUTX-101, Compared to an Untreated Historical Control, with a Nearly 80% Reduction in the Risk of Death (Hazard Ratio = 0.21, p<0.0001)

Rolling Submission of New Drug Application to the FDA for CUTX-101 on Track to Begin in the Fourth Quarter of 2020

New York, NY, August 28, 2020 – Fortress Biotech, Inc. (Nasdaq: FBIO) ("Fortress"), an innovative revenue-generating company focused on acquiring, developing and commercializing or monetizing promising biopharmaceutical products and product candidates cost-effectively, today announced that its partner company, Cyprium Therapeutics, reported positive topline clinical efficacy results for CUTX-101, a potential treatment for Menkes disease. The study demonstrated statistically significant improvement in overall survival for Menkes disease subjects who received early treatment (ET) with CUTX-101, compared to an untreated historical control (HC) cohort, with a nearly 80% reduction in the risk of death (Hazard Ratio = 0.21, p<0.0001). Median survival for the ET cohort was 14.8 years (177.1 months) compared to 1.3 years (15.9 months) for the untreated HC cohort.

"These positive topline clinical efficacy data highlight the potential of CUTX-101 as an effective therapy for Menkes disease patients. With no currently approved U.S. Food and Drug Administration ("FDA") treatments, Menkes disease is a serious condition with a significant unmet medical need. We look forward to presenting these topline data to the FDA at our upcoming pre-new drug application ("pre-NDA") meeting and will begin our rolling submission of a New Drug Application ("NDA") for CUTX-101 in the fourth quarter of this year," said Lung S. Yam, M.D., Ph.D., President and Chief Executive Officer of Cyprium.

As described in the Statistical Analysis Plan (SAP) agreed upon with the FDA, overall survival measured from birth is the primary efficacy endpoint for an Integrated Summary of Effectiveness. The primary efficacy analysis compared overall survival in Menkes disease patients who received daily treatment with CUTX-101 beginning within four weeks of age (adjusted for prematurity) to a historical control (HC) cohort of Menkes disease patients who did not receive copper therapy. Updated survival data and long-term follow-up data were collected under Cyprium-sponsored protocols CYP-001 and CYP-002 for both cohorts. Based on the criteria outlined in the SAP to ensure the two cohorts were comparable, 31 Menkes disease patients who received ET with CUTX-101 and 18 HC Menkes disease patients were included in the primary efficacy analysis. The primary endpoint of overall survival was met.

Stephen G. Kaler, M.D., M.P.H., a physician-scientist in the Center for Gene Therapy in the Abigail Wexner Research Institute at Nationwide Children's Hospital, led the protocols 90-CH-0149 and 09-CH-0059. Dr. Kaler is also the Principal Investigator of Cyprium-sponsored protocols CYP-001 and CYP-002, and a Professor of Pediatrics and Genetics at The Ohio State University College of Medicine.

The topline primary efficacy data and other relevant briefing materials will be presented to the FDA at a pre-NDA meeting scheduled for later in the current quarter. Other pre-specified secondary and sensitivity analyses will be included in the NDA submission. Additional data and analyses will be disclosed in peer-reviewed publications and at future medical or scientific conferences.

"We wish to express our deep gratitude to the Menkes disease patients and their families who have participated in clinical trials of CUTX-101 for this difficult illness over many years," concluded Dr. Yam.

The FDA previously granted Orphan Drug, Fast Track and Rare Pediatric Disease Designations for CUTX-101 for the treatment of Menkes disease. The European Medicines Agency's Committee for Orphan Medicinal Products issued a positive opinion on Cyprium's application for Orphan Drug Designation of CUTX-101 in July 2020.

About the Efficacy Analysis
To ensure the two cohorts were comparable, subjects needed to satisfy the following criteria for inclusion in the primary efficacy analysis:

ET Cohort

- Enrolled in protocols 90-CH-0149 or 09-CH-0059
- Carried a severe pathogenic mutation of the ATP7A gene (deletion/duplication, nonsense, or canonical splice junction)
- Born within the past 20 years (i.e., born after December 31, 1999)
- Initiated treatment with CUTX-101 within four weeks of age (adjusted for prematurity)
- Survived at least four weeks after birth and were asymptomatic for significant neurological signs and symptoms during the first four weeks.

HC Cohort

- Enrolled in a pre-specified HC cohort
- Carried a severe pathogenic mutation of the ATP7A gene (deletion/duplication, nonsense, or canonical splice junction)
- Born within the past 20 years (i.e., born after December 31, 1999)
- Have not received CUTX-101 therapy
- Survived at least four weeks after birth and were asymptomatic for significant neurological signs and symptoms during the first four weeks.

About Menkes Disease and Related Copper Metabolism Disorders
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 males, and potentially as high as 1 in 8,664 live male births, based on recent genome-based ascertainment. Biochemically, Menkes patients have low levels of copper in their blood and brain, as well as abnormal levels of certain neurochemicals. Definitive diagnosis is typically made by sequencing the ATP7A gene. 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, and failure to thrive. Mortality is high in untreated Menkes disease, with many patients dying before the age of three. 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 by replenishing Copper Histidinate, restoring copper homeostasis, and maintaining serum copper levels in the normal age appropriate range. 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 physiological pH and to bypass the oral absorption of copper, which is impaired in patients with Menkes disease. 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. A Phase 3 trial of CUTX-101 in patients with Menkes disease also led by Dr. Kaler has completed enrollment. A Cyprium-sponsored expanded access protocol for Menkes disease patients is ongoing.

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 Menkes patients, and to be used in combination with CUTX-101. CUTX-101 was granted FDA 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.

About Fortress Biotech

Fortress Biotech, Inc. ("Fortress") is an innovative biopharmaceutical company that was recently ranked number 10 in Deloitte's 2019 Technology Fast 500™, an annual ranking of the fastest-growing North American companies in the technology, media, telecommunications, life sciences and energy tech sectors, based on percentage of fiscal year revenue growth over a three-year period. Fortress is focused on acquiring, developing and commercializing high-potential marketed pharmaceutical products and development-stage pharmaceutical product candidates. The company has five 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 Alexion Pharmaceuticals, Inc., AstraZeneca, City of Hope, Fred Hutchinson Cancer Research Center, InvaGen Pharmaceuticals Inc. (a subsidiary of Cipla Limited), St. Jude Children's Research Hospital and Nationwide Children's Hospital. For more information, visit www.fortressbiotech.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. 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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