Fortress Biotech Announces Breakthrough Therapy Designation for CUTX-101, Copper Histidinate, for the Treatment of Menkes Disease

Rolling submission of New Drug Application to the FDA for CUTX-101 on track to begin in the first quarter of 2021 and to be completed by the end of the second quarter of 2021

New York, NY, December 15, 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 the U.S. Food and Drug Administration ("FDA") has granted Breakthrough Therapy Designation to Cyprium Therapeutics ("Cyprium") for CUTX-101, a potential treatment for Menkes disease. Often lethal if untreated, Menkes disease is an X-linked recessive disorder of copper metabolism caused by mutations in ATP7A, an evolutionarily conserved copper-transporting ATPase. The FDA previously granted Orphan Drug, Fast Track, and Rare Pediatric Disease Designations to CUTX-101 for the treatment of Menkes disease. Additionally, the European Medicines Agency previously granted Orphan Drug Designation to CUTX-101.

"We are very pleased that the FDA has granted Breakthrough Therapy Designation to CUTX-101, a devastating pediatric disease with no FDA-approved treatment options currently available. The positive topline clinical efficacy data reported earlier this year highlight the potential of CUTX-101 to be a safe and effective therapy and fulfill a significant unmet medical need for patients with Menkes disease. We look forward to beginning our rolling submission of a New Drug Application ("NDA") to the FDA for CUTX-101 in the first quarter of next year. Our goal is to bring this treatment to patients as soon as possible," said Lung S. Yam, M.D., Ph.D., President and Chief Executive Officer of Cyprium.

Breakthrough Therapy Designation is intended to expedite the development and review of drugs for serious or life-threatening conditions. The criteria for Breakthrough Therapy Designation require preliminary clinical evidence that demonstrates the drug may have substantial improvement on at least one clinically significant endpoint over available therapy. A Breakthrough Therapy Designation conveys all of the fast-track program features, more intensive FDA guidance on an efficient drug development program, an organizational commitment involving senior managers and eligibility for rolling review and priority review.

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 (Kaler SG, Ferreira CR, Yam LS. Estimated birth prevalence of Menkes disease and ATP7A-related disorders based on the Genome Aggregation Database (gnomAD). Mol Genet Metab Rep. 2020 Jun 5;24:100602). Biochemically, patients with Menkes disease have low levels of copper in their blood and brain, as well as abnormal levels of certain neurochemicals. Definitive diagnosis is 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, failure to thrive, and neurodevelopmental delays. Mortality is high in untreated Menkes disease, with many patients dying before the age of three years. 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") and physiological pH that bypasses the defect in absorption of orally administered copper 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. 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 (HC) 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 Nationwide Children's Hospital (https://www.nationwidechildrens.org/specialties/menkes-disease-clinic) and other US 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 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 pharmaceutical products and 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, 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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