Fortress Biotech Announces Publication of Study on Estimated Birth Prevalence of Menkes Disease in *Molecular Genetics and Metabolism Reports*

Cyprium Therapeutics, a Fortress partner company, is developing CUTX-101 for Menkes disease and is on track to begin submitting a rolling New Drug Application to the FDA in the fourth quarter of 2020

New York, NY, June 11, 2020 – Fortress Biotech, Inc. (Nasdaq: FBIO) ("Fortress"), an innovative biopharmaceutical company focused on acquiring, developing and commercializing high-potential marketed pharmaceutical products and development-stage pharmaceutical product candidates, today announced the publication of a study, "Estimated birth prevalence of Menkes disease and ATP7A-related disorders based on the Genome Aggregation Database (gnomAD)," in *Molecular Genetics and Metabolism Reports*. The study was published online in June 2020 and will be published in the print edition of the journal in September 2020.

The study evaluated the prevalence of Menkes disease, an often lethal, if untreated, X-linked recessive disorder of copper metabolism caused by mutations in ATP7A, an evolutionarily conserved copper-transporting ATPase. Previous estimates of Menkes disease were based on confirmed clinical cases ascertained from specific populations and varied from 1 in 40,000 to 1 in 354,507.

Led by 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, the authors reviewed the canonical ATP7A transcript in the current version of gnomAD (v2.1.1) to evaluate frequencies of loss-of-function and pathogenic missense variants in a diverse normal control population. Assuming Hardy-Weinberg genetic equilibrium, the allelic frequency of loss-of-function variants suggests a minimum birth prevalence for Menkes disease of 1 in 34,810 males, higher than previously recognized. If likely pathogenic missense variants are included, the estimated birth prevalence could potentially be as high as 1 in 8,664 live male births.

"Based on these findings, it appears that Menkes disease is under-reported in the population. This may reflect disparities in access to health care or tertiary care genetics clinics, challenges in distinguishing the Menkes phenotype from other conditions, and deaths of affected subjects before diagnosis," said Dr. Kaler, also a professor of Pediatrics and Genetics at The Ohio State University College of Medicine.

"Our latest study suggests that a newborn screening pilot study for Menkes disease would confirm a higher than previously estimated prevalence, as we have seen with other rare inherited disorders such as Pompe disease," said Lung S. Yam, M.D., Ph.D., President and Chief Executive Officer of Cyprium. "Earlier diagnosis of Menkes disease through newborn screening would likely increase the number of Menkes disease patients identified at birth and allow for institution of early treatment during the asymptomatic phase of the condition."

The study can be accessed here.

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. 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 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 National Institutes of Health (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 U.S. Food and Drug Administration ("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; 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 SEC 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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