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Fortress Biotech Announces Rare Pediatric Disease Designation for CUTX-101 for the Treatment of Menkes Disease

Cyprium Therapeutics, a Fortress partner company, on track to begin submitting rolling New Drug Application for CUTX-101 to the FDA in the second half of 2020

NEW YORK, Jan. 16, 2020 (GLOBE NEWSWIRE) -- Fortress Biotech, Inc. (Nasdaq: FBIO) ("Fortress"), an innovative biopharmaceutical company focused on identifying, in-licensing and developing high-potential marketed and development-stage drugs and drug candidates, today announced that the U.S. Food and Drug Administration ("FDA") has granted Rare Pediatric Disease Designation to Cyprium Therapeutics' ("Cyprium") Copper Histidinate, also referred to as CUTX-101, for the treatment of Menkes disease. Menkes disease is a rare X-linked recessive pediatric disease caused by genetic mutations of the copper transporter, ATP7A. The FDA previously granted Orphan Drug and Fast Track Designations to CUTX-101 for the treatment of Menkes disease.

The FDA grants Rare Pediatric Disease Designation for serious and life-threatening diseases that primarily affect children ages 18 years or younger and fewer than 200,000 people in the United States. If Cyprium's new drug application ("NDA") is approved, the company may be eligible to receive a priority review voucher, which can be redeemed to obtain priority review for any subsequent marketing application and may be sold or transferred. This program is intended to encourage development of new drugs and biologics for the prevention and treatment of rare pediatric diseases.

"The FDA's Rare Pediatric Disease Designation of CUTX-101 for the treatment of Menkes disease, after granting Orphan Drug and Fast Track Designations to CUTX-101 previously, highlights the significant unmet medical needs for patients with this devastating and life-threatening disease. Currently, there is no FDA-approved treatment for Menkes disease," said Lung S. Yam, M.D., Ph.D., President and Chief Executive Officer of Cyprium. "The entire Cyprium team is encouraged by this designation and will continue to work diligently toward submitting the NDA for CUTX-101."

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, which affects approximately one in 100,000 newborns per year.

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, with many patients dying before the age of three years, if untreated. 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 supplement blood and brain copper levels in patients diagnosed with classic Menkes disease who have not demonstrated significant clinical progression. 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 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 is ongoing at the *Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD).

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 identifying, in-licensing and developing high-potential marketed and development-stage drugs and drug 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 therapeutic areas, including oncology, rare

diseases and gene therapy, which allow it to create value while mitigating risk 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., 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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