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## **Fortress Biotech Announces Publication of Study on Targeted Next Generation Sequencing for Newborn Screening of Menkes Disease in Molecular Genetics and Metabolism Reports**

***Cyprium Therapeutics, a Fortress partner company, is developing CUTX-101 for Menkes disease***

***A rolling submission of a New Drug Application to the FDA is expected to begin in the fourth quarter of 2020***

NEW YORK, July 29, 2020 (GLOBE NEWSWIRE) -- 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, "Targeted Next Generation Sequencing for Newborn Screening of Menkes Disease" in *Molecular Genetics and Metabolism Reports*. The study was published online in July 2020.

The study assessed the analytic validity of an ATP7A targeted next generation DNA sequencing assay as a potential newborn screen for Menkes disease, a X-linked recessive disorder of copper metabolism caused by mutations in ATP7A, an evolutionarily conserved copper-transporting ATPase. Left undetected and untreated, Menkes disease is often fatal by three years of age. Population-based newborn screening (NBS) allows early detection and treatment of inherited disorders. For certain medically-actionable conditions, however, NBS is limited by the absence of reliable biochemical signatures amenable to detection by current platforms.

In the study, supported in part by The Menkes Foundation (<https://themenkesfoundation.org/>) and 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, researchers blindly analyzed dried blood spots from control or Menkes disease subjects (n=22) for pathogenic variants in the copper transporter gene, *ATP7A*. The analytical method was optimized to minimize cost and provide rapid turnaround time. The algorithm correctly identified pathogenic ATP7A variants, including missense, nonsense, small insertions/deletions, and large copy number variants, in 21/22 (95.5%) of

subjects, one of whom had inconclusive diagnostic sequencing previously. For one false negative that also had not been detected by commercial molecular laboratories, researchers identified a deep intronic variant that impaired ATP7A mRNA splicing.

“The results of our study support proof-of-concept that primary DNA-based NBS would accurately detect Menkes disease, a disorder for which biochemical detection in the newborn period is currently unavailable. Targeted next generation sequencing for NBS would enable improved Menkes disease clinical outcomes through early detection, and eliminate the lengthy, expensive, and uncomfortable diagnostic odysseys endured by many affected infants and their parents,” said Dr. Kaler, who is also a professor of Pediatrics and Genetics at The Ohio State University College of Medicine.

Lung S. Yam, M.D., Ph.D., President and Chief Executive Officer of Cyprium, added, “This study suggests that Menkes disease could be accurately detected by a quick NBS method that is also cost effective. NBS could potentially increase the number of Menkes disease patients identified at birth allowing for earlier treatment, a critical component correlated with clinical outcome.”

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. The minimum birth prevalence for Menkes disease is believed to be 1 in 34,810 males, but could potentially be as high as 1 in 8,664 live male births, higher than previously recognized. 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](http://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](http://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 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.

**Company Contacts:**

Jaclyn Jaffe and William Begien  
Fortress Biotech, Inc.  
(781) 652-4500  
[ir@fortressbiotech.com](mailto:ir@fortressbiotech.com)

Lung Yam, M.D., Ph.D.  
Cyprium Therapeutics, Inc.  
[ir@cypriumtx.com](mailto:ir@cypriumtx.com)

**Company Investor Relations Contact:**

Daniel Ferry  
LifeSci Advisors, LLC  
(617) 430-7576  
[daniel@lifesciadvisors.com](mailto:daniel@lifesciadvisors.com)

**Company Media Relations Contact:**

Tony Plohoros  
6 Degrees  
(908) 591-2839  
[tplohoros@6degreespr.com](mailto:tplohoros@6degreespr.com)

**Nationwide Children's Hospital Media Relations Contact:**

Mary Ellen Fiorino  
[MaryEllen.Fiorino@nationwidechildrens.org](mailto:MaryEllen.Fiorino@nationwidechildrens.org)



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