July 2, 2018

Fortress Biotech Announces Cyprium Therapeutics' CUTX-101 (Copper Histidinate) Granted FDA Fast Track Designation for Treatment of Classic Menkes Disease

NEW YORK, July 02, 2018 (GLOBE NEWSWIRE) -- Fortress Biotech, Inc. (NASDAQ:FBIO) ("Fortress"), a biopharmaceutical company dedicated to acquiring, developing and commercializing novel pharmaceutical and biotechnology products, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track Designation to Cyprium Therapeutics' ("Cyprium") Copper Histidinate, also referred to as CUTX-101, for patients diagnosed with classic Menkes disease who have not demonstrated significant clinical progression.

As a subsidiary of Fortress, Cyprium is developing CUTX-101 for Menkes disease under 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). The CRADA was established in March 2017. Stephen G. Kaler, M.D., Senior Investigator and Head, Section on Translational Neuroscience and former NICHD Clinical Director, leads the Menkes disease research program at NICHD and serves as principal investigator of the CUTX-101 Phase 3 clinical trial.

"We are pleased that the FDA granted Fast Track Designation for Copper Histidinate based on the promising clinical data observed to date," stated Lung S. Yam, M.D., Ph.D., President and Chief Executive Officer of Cyprium. "Menkes disease is a rare and fatal pediatric disease, and patients with classic Menkes disease currently have no approved therapeutic options. This designation signifies the FDA's recognition of the significant unmet medical need that Copper Histidinate has potential to address. We appreciate our ongoing discussions with the FDA and look forward to working closely with the agency and Dr. Kaler as we advance the development of Copper Histidinate, which is currently being evaluated in a Phase 3 study."

The FDA's Fast Track program facilitates the development of drugs intended to treat serious conditions and that have the potential to address unmet medical needs. A drug program with Fast Track status is afforded greater access to the FDA for the purpose of expediting the drug's development, review and potential approval. In addition, the Fast Track program

allows for eligibility for Accelerated Approval and Priority Review, if relevant criteria are met, as well as for Rolling Review, which means that a drug company can submit completed sections of its New Drug Application (NDA) for review by the FDA, rather than waiting until every section of the NDA is completed before the application is submitted for review.

About CUTX-101 (Copper Histidinate)

CUTX-101 is in development to supplement blood and brain copper levels in patients with Menkes disease and related disorders. CUTX-101 is a subcutaneous injectable formulation of Copper Histidinate manufactured under cGMP that is intended to improve tolerability due to physiological pH and bypass the oral absorption of copper, which is impaired in patients with Menkes disease. In a Phase 1/2 clinical study conducted by Dr. Kaler at NIH, early treatment of Menkes patients with CUTX-101 led to an improvement in neurodevelopmental outcomes and survival. A Phase 3 study of CUTX-101 in Menkes disease is ongoing.

Cyprium and NICHD are also collecting natural history data on classic Menkes disease patients who were not treated with copper supplements. Data from this natural history study will serve as a historical control cohort to further demonstrate the efficacy and safety of CUTX-101.

CUTX-101 previously has been granted orphan drug designation by the U.S. Food and Drug Administration.

About Menkes Disease and Related Copper Metabolism Disorders

Menkes disease is a rare X-linked pediatric disease caused by gene mutations of copper transporter ATP7A, which affects approximately one in 100,000 newborns per year. Biochemically, Menkes patients may have low levels of copper in their blood and brain, as well as abnormal levels of catecholamines, but 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”), failure to thrive, connective tissue problems and severe neurological symptoms such as seizures. Mortality is high, 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 Cyprium Therapeutics

Cyprium Therapeutics, Inc. (Cyprium), a Fortress Biotech company, 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 Phase 3 candidate 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. AAV-ATP7A gene therapy was also previously granted orphan drug

designation by the FDA. Cyprium is a majority-owned subsidiary of Fortress Biotech (NASDAQ:FBIO) and is based in New York City.

About Fortress Biotech

Fortress Biotech, Inc. ("Fortress") is a biopharmaceutical company dedicated to acquiring, developing and commercializing novel pharmaceutical and biotechnology products. Fortress develops and commercializes products both within Fortress and through certain subsidiary companies, also known as Fortress Companies. In addition to its internal development programs, Fortress leverages its biopharmaceutical business expertise and drug development capabilities and provides funding and management services to help the Fortress Companies achieve their goals. Fortress and the Fortress Companies may seek licensings, acquisitions, partnerships, joint ventures and/or public and private financings to accelerate and provide additional funding to support their research and development programs. 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. 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 required by law.

Contacts:

Cyprium Therapeutics, Inc.
Lung Yam, M.D., Ph.D., President and CEO
ir@cypriumtx.com

Fortress Biotech, Inc.
Jaclyn Jaffe, Investor Relations
(781) 652-4500
ir@fortressbiotech.com

Fortress Biotech Media Relations

Tony Plohoros
6 Degrees
(908) 940-0135
tplohoros@6degreespr.com

Source: Fortress Biotech, Inc.