



Fortress Biotech Forms New Subsidiary, Cyprium Therapeutics, Inc., to Develop Novel Therapies for Menkes Disease and Related Copper Metabolism Disorders

Cyprium, NICHD establish CRADA to advance clinical development of Phase 3 candidate CUTX-101 (Copper Histidinate injection) in Menkes disease, a rare and fatal pediatric disease

Cyprium licenses preclinical AAV-ATP7A gene therapy from NICHD to develop in combination with CUTX-101

Lung S. Yam, M.D., Ph.D., named Chief Executive Officer

New York, NY, March 14, 2017 -- Fortress Biotech, Inc. (Nasdaq: FBIO) today announced the launch of a new subsidiary, Cyprium Therapeutics, Inc. (Cyprium), to develop novel therapies for the treatment of Menkes disease and related copper metabolism disorders. As part of its formation, Cyprium has 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 have 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.

Under the terms of the CRADA, Cyprium is authorized to reference the Investigational New Drug application currently held by NICHD and any data generated at NICHD to advance the development of CUTX-101, including the filing of a New Drug Application and commercialization in the U.S. and other territories. In addition, Cyprium will be responsible for the manufacturing of CUTX-101 under Current Good Manufacturing Practice ("cGMP") regulations to support ongoing clinical studies at NICHD. Cyprium will also provide financial and logistical support for clinical and basic science research on Menkes disease and other related copper metabolism disorders at NICHD.

Stephen G. Kaler, M.D., Senior Investigator and Head, Section on Translational Neuroscience, Molecular Medicine Branch, and former NICHD Clinical Director, leads the Menkes disease research program at NICHD and developed the AAV-ATP7A gene therapy technology. Dr. Kaler will serve as principal investigator of the CUTX-101 clinical trials and studies of AAV-ATP7A gene therapy.

Lindsay A. Rosenwald, M.D., Fortress Biotech's Chairman, President and Chief Executive Officer, said, "Fortress Biotech's mission is to develop products that improve the lives of patients across multiple diseases. We are pleased to launch another subsidiary company, Cyprium Therapeutics, which we believe will further broaden our rare disease platform through the development of novel therapies for the treatment of Menkes disease and other related disorders."

CUTX-101 Development Pathway

CUTX-101 is in development to supplement 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 to bypass the oral absorption of copper, which is impaired in patients with Menkes disease. In Phase 1/2 clinical studies conducted at NICHD, early treatment of Menkes patients with CUTX-101 led to an improvement in neurodevelopmental outcomes and survival.

Cyprium and NICHD plan to conduct a retrospective natural history study to collect data on Menkes disease patients who have not been treated with copper supplements. Data from this natural history study may serve as a historical control to demonstrate the efficacy and safety of CUTX-101. Cyprium expects to request a meeting with the U.S. Food and Drug Administration (FDA) in 2017 to determine a regulatory pathway for CUTX-101. CUTX-101 has been granted orphan drug designation by the FDA.

AAV-ATP7A Gene Therapy Development Pathway

AAV-ATP7A gene therapy, which is currently in preclinical development at NICHD, has demonstrated the ability to rescue neurological phenotypes and improve survival when co-administered with copper injections in a mouse model of Menkes disease. Cyprium will work with NICHD to determine optimal vector design and expects to nominate a candidate for clinical development in 2018. AAV-ATP7A gene therapy has been granted orphan drug designation by the FDA.

Lung S. Yam, M.D., Ph.D., Named Cyprium Chief Executive Officer

Fortress also announced the appointment of Lung S. Yam, M.D., Ph.D., as Chief Executive Officer of Cyprium. Dr. Yam co-founded Cyprium as part of his Business Development Consultant role at Fortress, where he identified multiple opportunities across diverse therapeutic areas leading to the in-licensing of multiple assets, including CUTX-101 and the AAV-ATP7A gene therapy program. In addition to his roles at Cyprium and Fortress, Dr. Yam serves as Senior Analyst at Opus Point Partners, LLC, an affiliated life-science focused asset management firm. Prior to joining Opus Point Partners, Dr. Yam was an equity research associate at Rodman & Renshaw, LLC. Dr. Yam earned M.D. and Ph.D. degrees from New York University School of Medicine, and a B.A. in biology from Johns Hopkins University where he graduated Phi Beta Kappa.

Dr. Yam stated, "We are thrilled to collaborate with Dr. Kaler and his team at NICHD to develop novel therapies for patients with Menkes disease and related disorders. Dr. Kaler has dedicated much of his career to improving treatment options for Menkes patients, including leading clinical studies of Copper Histidinate injections and developing an AAV-based gene therapy that has demonstrated encouraging preclinical results. The Cyprium team looks forward to continuing this important work with the goal of generating the first approved therapies for the treatment of Menkes disease."

Cyprium was founded by Dr. Rosenwald, Dr. Yam and Michael S. Weiss, Executive Vice Chairman, Strategic Development at Fortress. Dr. Rosenwald will serve as the Executive Chairman of Cyprium's Board of Directors, and Dr. Yam and Mr. Weiss will be additional members of the Board.

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 of the ATP7A gene. The condition is characterized by distinctive clinical features, including sparse and depigmented hair ("kinky hair"), failure to thrive, connective tissue disorders 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 diseases, including Occipital Horn Syndrome and ATP7A-related Distal Motor Neuropathy. Currently, there is no FDA-approved treatment for Menkes disease and its variants.

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 of its subsidiary companies, also known as Fortress Companies. Additionally, Fortress recently acquired a controlling interest in National Holdings Corporation (NASDAQ: NHLD), a diversified independent brokerage company (together with its subsidiaries, "NHLD"). 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. 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 related to our growth strategy; risks relating to the results of research and development activities; our ability to obtain, perform under and maintain financing and strategic agreements and relationships; uncertainties relating to preclinical and clinical testing; 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.

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