December 15, 2025

Fortress Biotech and Cyprium Therapeutics Announce FDA Acceptance of CUTX-101 NDA Resubmission

New PDUFA Target Action Date of January 14, 2026 set by FDA

MIAMI, Dec. 15, 2025 (GLOBE NEWSWIRE) -- Fortress Biotech, Inc. (Nasdaq: FBIO) ("Fortress") and its majority-owned subsidiary, Cyprium Therapeutics, Inc. ("Cyprium"), today announced that the U.S. Food and Drug Administration ("FDA") has accepted the resubmission of the New Drug Application ("NDA") for CUTX-101 (copper histidinate), intended to treat Menkes disease in pediatric patients.

The resubmission has been accepted as a Class 1 resubmission and as a result, the new Prescription Drug User Fee Act (PDUFA) target action date for the NDA is January 14, 2026.

In December 2023, Sentynl Therapeutics, Inc. ("Sentynl"), a U.S.-based biopharmaceutical company wholly owned by Zydus Lifesciences, Ltd. ("Zydus Group"), assumed full responsibility for the development and commercialization of CUTX-101 from Cyprium. On November 14, 2025, Sentynl resubmitted the revised NDA after receiving a complete response letter ("CRL") from the FDA on September 30, 2025, which CRL cited observations regarding the manufacturing site's cGMP compliance. The CRL did not cite any other approvability concerns, nor did it identify any deficiencies in CUTX-101's efficacy and safety data, which demonstrate significant improvement in overall survival for Menkes disease subjects who received early treatment with the therapy.

Pursuant to the transaction with Sentynl, Sentynl will transfer to Cyprium, if issued upon approval, a Rare Pediatric Disease Priority Review Voucher ("PRV"), and Cyprium will also be eligible to receive royalties on net sales of CUTX-101 and up to $129 million in aggregate development and sales milestones from Sentynl.

The CUTX-101 NDA was initially granted Priority Review by the FDA and is supported by positive topline clinical efficacy results for CUTX-101, demonstrating significant improvement in overall survival for Menkes disease subjects who received early treatment with CUTX-101.

About Menkes Disease
Menkes disease is a rare X-linked recessive pediatric disease caused by gene mutations of the copper transporter *ATP7A*. The minimum birth prevalence for Menkes disease is believed to be 1 in 34,810 live male births, and potentially as high as 1 in 8,664 live male

births, based on recent genome-based ascertainment (Kaler SG, Ferreira CR, Yam LS. Estimated birth prevalence of Menkes disease and *ATP7A*-related disorders based on the Genome Aggregation Database (gnomAD). Molecular Genetics and Metabolism Reports 2020 June 5;24:100602). 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, failure to thrive, and neurodevelopmental delays. Mortality is high in untreated Menkes disease, with many patients dying between 2-3 years of age. 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") 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 with the Eunice Kennedy Shriver National Institute of Child Health and Human Development ("NICHD"), part of the NIH, to advance the clinical development of CUTX-101 (Copper Histidinate injection) for the treatment of Menkes disease. CUTX-101 is an investigational drug currently under FDA review to treat patients with Menkes disease. In 2023, Cyprium completed the transfer of its proprietary rights and assigned its FDA documents pertaining to CUTX-101 to Sentynl Therapeutics, Inc. Cyprium and NICHD also have an ongoing 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 patients with Menkes disease, and to be used in combination with CUTX-101; AAV-ATP7A gene therapy is currently in pre-clinical development and has received FDA Orphan Drug Designation. Cyprium was founded by, and is a majority-owned subsidiary of, Fortress Biotech, Inc. (Nasdaq: FBIO). For more information, visit www.cypriumtx.com.

About Fortress Biotech

Fortress Biotech, Inc. ("Fortress") is an innovative biopharmaceutical company focused on acquiring and advancing assets to enhance long-term value for shareholders through product revenue, equity holdings and dividend and royalty income. The company has eight marketed prescription pharmaceutical products and multiple programs in development at Fortress, at its majority-owned and majority-controlled partners and subsidiaries and at partners and subsidiaries it founded and in which it holds significant minority ownership positions. Fortress' portfolio is being commercialized and developed for various therapeutic areas including oncology, dermatology, and rare diseases. Fortress' model is focused on leveraging its significant biopharmaceutical industry expertise and network to further expand and advance 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 AstraZeneca, City of Hope, Nationwide Children's Hospital, Columbia University, Dana Farber Cancer Center and Sentynl Therapeutics. For more information, visit www.fortressbiotech.com.

Forward-Looking Statements

Statements in this press release that are not descriptions of historical facts are "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. The words “anticipates,” “believes,” “can,” “continue,” “could,” “estimates,” “expects,” “intends,” “may,” “might,” “plans,” “potential,” “predicts,” “should,” or “will” or the negative of these terms or other comparable terminology are generally intended to identify forward-looking statements. These 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: the possibility that the NDA for CUTX-101 is not approved or that such NDA is approved but no PRV is issued in connection therewith; our growth strategy, financing and strategic agreements and relationships; our need for substantial additional funds and uncertainties relating to financings; uncertainty related to the timing and amounts expected to be realized from future milestone, contingent value right, royalty or similar future revenue streams, if at all; our ability to identify, acquire, close and integrate product candidates successfully and on a timely basis; our ability to attract, integrate and retain key personnel; the early stage of products under development; the results of research and development activities; uncertainties relating to preclinical and clinical testing; our ability to obtain regulatory approval for products under development; our ability to successfully commercialize products or other marketable assets for which we receive regulatory approval or receive royalties or other distributions from third parties; our ability to secure and maintain third-party manufacturing, marketing and distribution of our and our partner companies’ products and product candidates; 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, and we claim the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995. 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 Contact:
Jaclyn Jaffe
Fortress Biotech, Inc.
(781) 652-4500
ir@fortressbiotech.com

Media Relations Contact:
Tony Plohoros
6 Degrees
(908) 591-2839
tplohoros@6degreespr.com

Source: Fortress Biotech, Inc.