January 4, 2025

BSE Limited **Code: 532321** P J Towers, Dalal Street, Mumbai-400001	**National Stock Exchange of India Limited** **Code: Zyduslife** Exchange Plaza, C/1, Block G, Bandra-Kurla Complex, Bandra (East), Mumbai-400051

Re.: **Press Release**

Dear Sir / Madam,

Please find attached Press Release with title "**Sentynl Therapeutics Announces U.S. FDA Acceptance and Priority Review of New Drug Application for CUTX-101 (Copper Histidinate) Product Candidate for Treatment of Menkes Disease"**.

Please bring the aforesaid news to the knowledge of public at large.

Thanking you,

Yours faithfully,
For, **Zydus Lifesciences Limited**

DHAVAL NARENDRA SONI
Digitally signed by DHAVAL NARENDRA SONI
Date: 2025.01.06 23:57:48 +05'30'

Dhaval N. Soni
Company Secretary and Compliance Officer
Membership No. FCS7063

Encl.: As above

Sentynl Therapeutics Announces U.S. FDA Acceptance and Priority Review of New Drug Application for CUTX-101 (Copper Histidinate) Product Candidate for Treatment of Menkes Disease

Six-month priority review granted for CUTX-101 copper histidinate with PDUFA target action date set for June 30, 2025

CUTX-101 has potential to be the first FDA-approved treatment for Menkes disease, a rare and fatal pediatric disease

Solana Beach, CA, Ahmedabad, India, and Miami, FL – January 6, 2025 — Sentynl Therapeutics, Inc. ("Sentynl"), a U.S.-based biopharmaceutical company wholly-owned by Zydus Lifesciences, Ltd. ("Zydus Group"), and Fortress Biotech, Inc. (Nasdaq: FBIO) ("Fortress") announced that the U.S. Food and Drug Administration (FDA) has accepted for filing and Priority review Sentynl's New Drug Application (NDA) for CUTX-101, the product candidate for the treatment of Menkes disease. Menkes disease is a rare X-linked recessive pediatric disease caused by gene mutations of the copper transporter ATP7A. Recent estimates suggest a prevalence of 1 in 34,810 to as high as 1 in 8,664 live male births.

Sentynl's NDA is supported by positive topline clinical efficacy results for CUTX-101, demonstrating statistically significant improvement in overall survival for Menkes disease subjects who received early treatment with CUTX-101, with a nearly 80% reduction in the risk of death compared to an untreated historical control cohort. Median overall survival was 177.1 months for CUTX-101 early treatment cohort compared to 16.1 months for the untreated historical control cohort.

"The NDA acceptance of CUTX-101 marks an important step towards our vision to transform lives and meaningfully impact patients, caregivers, and the rare disease community at large," said Punit Patel, President and CEO, Zydus Americas. "We remain committed to providing access to path-breaking discoveries that can address unmet needs globally, as evident in the potential impact of CUTX-101 on Menkes disease patients and their families."

"Menkes disease presents a difficult journey for patients and their caregivers, as *ATP7A* mutations impact the transport of copper to a range of organs and systems, such as the lungs, brain and heart. With no known cure or current FDA-approved treatments, death typically occurs between 2 to 3 years of age," said Matt Heck, President & Chief Executive Officer of Sentynl. "We are eager for the FDA to review our application for CUTX-101, which has the potential to be the first FDA-approved therapy for this devastating condition."

CUTX-101 was granted Breakthrough Therapy, Fast Track, Rare Pediatric Disease and Orphan Drug Designations by the FDA and Orphan Designation by the European Medicines Agency (EMA). Under the Prescription Drug User Fee Act (PDUFA), the FDA set a six-month period with a target action date of June 30, 2025.

In December 2023, Sentynl assumed full responsibility for the development and commercialization of CUTX-101 from Cyprium Therapeutics ("Cyprium"), a Fortress subsidiary company focused on the development of novel therapies for the treatment of Menkes disease and related copper metabolism disorders.

"We congratulate Sentynl on the NDA acceptance for filing and review of CUTX-101, a program that Cyprium advanced and proudly supports," said Lindsay A. Rosenwald, M.D., Chairman, President and

Chief Executive Officer of Fortress and Chairman of Cyprium. “The drug has a demonstrated safety and efficacy profile for the treatment of Menkes disease and, if approved, CUTX-101 will fill a significant unmet need for children suffering from this rare, fatal pediatric disease.”

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About CUTX-101 (Copper Histidinate)
CUTX-101 is an investigational drug currently under NDA review with the U.S. FDA to treat patients with Menkes disease. CUTX-101 is a subcutaneous injectable formulation of copper histidinate manufactured under current good manufacturing practice (“cGMP”) that is intended to improve tolerability due to its physiological pH. 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. Cyprium previously reported positive topline clinical efficacy results for CUTX-101, demonstrating statistically significant improvement in overall survival for Menkes disease subjects who received early treatment (ET) with CUTX-101, compared to an untreated historical control cohort, with a nearly 80% reduction in the risk of death. Median overall survival (OS) was 177.1 months for CUTX-101 ET cohort compared to 16.1 months for the untreated historical control cohort. CUTX-101 has been granted FDA Breakthrough Therapy, Fast Track, Rare Pediatric Disease and FDA Orphan Drug Designations. Additionally, the European Medicines Agency granted Orphan Designation for CUTX-101. An expanded access protocol for patients with Menkes disease is ongoing at multiple U.S. medical centers.

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 Sentynl Therapeutics
Sentynl Therapeutics, Inc. (“Sentynl”) is a U.S.-based biopharmaceutical company focused on bringing innovative therapies to patients suffering from rare diseases. The company was acquired by the Zydus Group in 2017. Sentynl’s experienced management team has previously built multiple successful pharmaceutical companies. With a focus on commercialization, Sentynl looks to source effective and well-differentiated products across a broad spectrum of therapeutic areas to address unmet needs. Sentynl is committed to the highest ethical standards and compliance with all applicable laws, regulations and industry guidelines. For more information, visit https://sentynl.com.

About Zydus Group
Zydus Lifesciences Ltd. with an overarching purpose of empowering people with freedom to live healthier and more fulfilled lives, is an innovative, global lifesciences company that discovers, develops, manufactures, and markets a broad range of healthcare therapies. The group employs over 27,000 people worldwide, including 1,400 scientists engaged in R & D, and is driven by its mission to unlock new possibilities in lifesciences through quality healthcare solutions that impact lives. The group aspires to

transform lives through path-breaking discoveries. For more information, visit https://www.zyduslife.com/zyduslife.

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 revenue. The company has seven marketed prescription pharmaceutical products and over 20 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. 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 focused on leveraging its significant biopharmaceutical industry expertise and network 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 AstraZeneca, City of Hope, Fred Hutchinson Cancer Center, Nationwide Children's Hospital and Sentynl. For more information, visit www.fortressbiotech.com.

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