

NEWS RELEASE

Niagen Bioscience Receives Exclusive U.S. FDA Rare Pediatric Disease (RPD) Designation and European Medicines Agency Orphan Medicinal Product Designation (OMPD) for NB4168 for the Treatment of Ataxia Telangiectasia (A-T)

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Regulatory recognition in the United States and Europe supports the development of NB4168, a novel small molecule candidate for a rare pediatric disease with no approved treatments

LOS ANGELES--(BUSINESS WIRE)-- **Niagen Bioscience, Inc.** (NASDAQ: NAGE), the global authority on NAD+ (nicotinamide adenine dinucleotide) with a focus on the science of healthy aging, today announced that the U.S. Food & Drug Administration (FDA) granted Rare Pediatric Disease (RPD) Designation for its proprietary lead small molecule drug candidate NB4168 for the treatment of Ataxia Telangiectasia (A-T). NB4168 is an oral small molecule therapy engineered to deliver substantially greater nicotinamide riboside (NR) exposure than conventional NR while maintaining a differentiated pharmacokinetic and safety profile. In addition, the European Medicines Agency (EMA) has granted Orphan Medicinal Product Designation (OMPD) to NB4168 for the treatment of A-T, providing regulatory recognition in the European Union and further supporting the Company's plans to advance the program globally.

The FDA granted RPD Designation based on its determination that A-T is a serious and life-threatening disease that primarily affects individuals from birth through adolescence and meets the statutory definition of a rare disease. The EMA's Committee for Orphan Medicinal Products similarly concluded that NB4168 met the criteria for orphan designation for the treatment of A-T. Together, these regulatory designations recognize the significant unmet medical need in A-T and provide development incentives intended to support and accelerate the advancement of

promising therapies for rare diseases.

NB4168 is the first investigational therapeutic candidate to emerge from Niagen Bioscience's **recently announced** wholly owned subsidiary focused on developing therapies for rare genetic diseases and age-related disorders, **NAD Pharmaceuticals Corp**. NB4168 is designed to have significantly higher bioavailability and increase NAD⁺, a coenzyme essential for DNA repair, mitochondrial function, cellular energy production, and stress responses—biological pathways disrupted in A-T. As A-T is categorized as a rare genetic premature aging disease, NB4168 may translate to other age-related diseases.

“Receiving RPD Designation from the U.S. FDA and OMPD from the EMA represents meaningful regulatory validation of NB4168 and our strategy to develop therapies for patients with serious rare diseases,” said Rob Fried, CEO of Niagen Bioscience. “These milestones strengthen our path toward clinical development and reinforce the opportunity to extend our leadership in NAD⁺ science into regulated medicines.”

About Ataxia Telangiectasia (A-T)

A-T is a rare genetic disease caused by mutations in the ATM gene. The disease typically presents in early childhood and is characterized by progressive loss of motor coordination, impaired immune function, increased susceptibility to infections, pulmonary complications, a substantially elevated risk of cancer, and premature aging. Children living with A-T often experience worsening neurological disability over time, with many requiring wheelchair assistance as the disease progresses. There are currently no FDA-approved therapies for A-T, and treatment is largely limited to supportive care. A-T impacts roughly 1 in 40,000 people in the U.S. (Riboldi et al., 2023; Teive et al., 2015) and 1 in 150,000 people in Europe (Bhatt et al., 2015).

About the U.S. FDA RPD and EMA OMPD

The FDA's RPD Designation is intended to encourage the development of therapies for serious and life-threatening diseases that primarily affect children. The designation provides certain regulatory and development incentives intended to support the advancement of promising therapies for rare pediatric conditions.

The EMA's OMPD is granted to therapies intended to diagnose, prevent, or treat life-threatening or chronically debilitating rare diseases affecting fewer than five in 10,000 people in the European Union. Orphan designation provides access to regulatory support and other development incentives designed to facilitate treatment development for rare diseases.

About NB4168

NB4168 is a distinct, proprietary small molecule designed for oral pharmaceutical development. It is not commercially available as a supplement or approved drug and has robust coverage by Niagen Bioscience's patent portfolio, including a composition-of-matter patent. After oral administration, NB4168 is designed to deliver significantly increased doses of NR to the bloodstream. NR enters cells directly, where it is converted through the nicotinamide riboside kinase pathway into NAD⁺. Because NAD⁺ supports DNA repair, mitochondrial function and cellular resilience, increasing intracellular NAD⁺ may represent a novel therapeutic approach for rare genetic diseases such as A-T in which these biological pathways are impaired.

The compound was designed to build upon Niagen Bioscience's extensive expertise in NR and NAD⁺ biology. The Company is currently advancing preclinical development activities and plans to submit an Investigational New Drug (IND) application to the FDA in anticipation of initiating human clinical studies, representing another step in Niagen Bioscience's strategy to translate decades of NAD⁺ science into proprietary medicines for serious rare genetic diseases.

"A-T is characterized by defects in DNA damage repair, mitochondrial dysfunction and chronic cellular stress, all biological processes that rely on adequate NAD⁺ availability," said Vilhelm Bohr, M.D., Ph.D., D.Sc., formerly at the National Institute on Aging, NIH, and currently a Professor (AFL) in Molecular Aging at the University of Copenhagen. "The absence of effective treatment options underscores the urgent need for new therapeutic approaches. It is encouraging to see scientific advances in NAD⁺ biology translated into investigational medicines such as NB4168, as this intervention has implications for similar accelerated aging diseases."

Niagen Bioscience, Inc. is a publicly traded bioscience company focused on NAD⁺ science and healthy aging research. The Company's product portfolio includes its flagship patented NR ingredient, Niagen[®], Tru Niagen[®], Niagen[™] Plus and a pharmaceutical development effort focused on proprietary NAD⁺ precursors. Niagen Bioscience maintains a portfolio of over 50 patents protecting NR and other NAD⁺ precursors.

For additional information on the Pharmaceutical Program for rare genetic diseases and age-related disorders and NB4168, visit www.niagenbioscience.com/nad-pharmaceuticals.

About Niagen Bioscience

Niagen Bioscience, Inc. (NASDAQ: NAGE) is the global authority in NAD⁺ (nicotinamide adenine dinucleotide) science and healthy-aging research. As a trusted pioneer of NAD⁺ discoveries, Niagen Bioscience[™] is dedicated to advancing healthspan through precision science and innovative NAD⁺-boosting solutions.

The Niagen Bioscience team, composed of world-renowned scientists, works with independent investigators from esteemed universities and research institutions around the globe to uncover the full potential of NAD⁺. A vital

coenzyme found in every cell of the human body, NAD⁺ declines with age and exposure to everyday lifestyle stressors. NAD⁺ depletion is a key contributor to age-related changes in health and vitality.

Distinguished by state-of-the-art laboratories, rigorous scientific and quality protocols, and collaborations with leading research institutions worldwide, Niagen Bioscience sets the gold standard for research, quality, and innovation. There's a better way to age.

At the heart of its clinically proven product portfolio is Niagen® (patented nicotinamide riboside, or NR), the most efficient, well-researched, and high-quality NAD⁺ booster available. Niagen powers the Company's consumer supplement, Tru Niagen®, the number one NAD⁺ boosting oral supplement in the United States[†] (available at www.truniagen.com), and Niagen™ Plus, featuring pharmaceutical-grade intravenous (IV) and injectable Niagen products (www.niagenplus.com). Pharmaceutical-grade Niagen IV and injections are compounded and distributed by U.S. FDA-registered 503B outsourcing facilities and are available exclusively at clinics with a prescription. NAD Pharmaceuticals Corp., the Company's wholly owned subsidiary focused on developing therapies for rare genetic diseases and age-related disorders, is conducting research on NB4168, a differentiated molecule.

Niagen Bioscience's robust patent portfolio protects NR and other NAD⁺ precursors. Niagen Bioscience maintains a website at www.niagenbioscience.com, where copies of press releases, news, and financial information are regularly published.

[†] Based on revenue per largest U.S. e-commerce marketplace (Jan. 2025 – Dec. 2025)

Forward-Looking Statements

This release contains 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. Statements that are not a description of historical facts constitute forward-looking statements and may often, but not always, be identified by the use of such words as "expects," "anticipates," "intends," "estimates," "plans," "potential," "possible," "probable," "believes," "seeks," "may," "will," "should," "could," "predicts," "projects," "continue," "would" or the negative of such terms or other similar expressions.

Forward-looking statements are based on current expectations and assumptions and are subject to risks and uncertainties that could cause actual results to differ materially from those described. These risks and uncertainties include, but are not limited to, statements regarding Niagen Bioscience's NB4168 pharmaceutical development program; planned preclinical, IND-enabling and clinical development activities; the potential timing of an IND submission or first-in-human study; the potential bioavailability, exposure, safety, tolerability, efficacy, pharmacodynamic or clinical profile of NB4168; and the Company's ability to translate its NAD⁺ platform into

pharmaceutical products; inflationary conditions and adverse economic conditions; our history of operating losses; the growth and profitability of our product sales; our ability to maintain and grow sales, marketing and distribution capabilities; changing consumer perceptions of our products; our reliance on a single or limited number of third-party suppliers; risks of conducting business in China; including unanticipated developments in and risks related to the Company's ability to secure adequate quantities of pharmaceutical-grade Niagen in a timely manner; the Company's ability to obtain appropriate contracts and arrangements with U.S. FDA-registered 503B outsourcing facilities required to compound and distribute pharmaceutical-grade Niagen to clinics; the Company's ability to remain on the U.S. FDA Bulk Drug Substances Nominated for Use in Compounding Under Section 503B of the Federal Food, Drug, and Cosmetic Act Category 1 list; the Company's ability to maintain and enforce the Company's existing intellectual property and obtain new patents; whether the potential benefits of NRC can be further supported; further research and development and the results of clinical trials possibly being unsuccessful or insufficient to meet applicable regulatory standards or warrant continued development; the ability to enroll sufficient numbers of subjects in clinical trials; determinations made by the FDA and other governmental authorities, including with respect to products seeking to compete in our market; mislabeling or other misleading marketing practices by competitors; economic and market instability, including as a result of tariffs or trade conflicts; and the risks and uncertainties associated with our business and financial condition in general, described in our filings with the Securities and Exchange Commission (SEC), including, without limitation, our most recent Annual Report on Form 10-K and Quarterly Report on Form 10-Q as filed with the SEC.

Readers are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof, and actual results may differ materially from those suggested by these forward-looking statements. All forward-looking statements are qualified in their entirety by this cautionary statement and Niagen Bioscience undertakes no obligation to revise or update this release to reflect events or circumstances after the date hereof.

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