

# ChromaDex Receives Exclusive U.S. FDA Orphan Drug Designation (ODD) and Rare Pediatric (RPD) Disease Designation for Nicotinamide Riboside Chloride (NRC) for the Treatment of Ataxia Telangiectasia (AT)

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ChromaDex plans to conduct additional studies on NRC in anticipation of filing for an Investigational New Drug Application (IND) for the treatment of AT

LOS ANGELES--(BUSINESS WIRE)-- **ChromaDex Corp.** (NASDAQ:CDXC), the global authority on nicotinamide adenine dinucleotide (NAD+) and healthy aging research, today announced that the U.S. Food & Drug Administration (FDA) granted Orphan Drug Designation (ODD) and Rare Pediatric Disease (RPD) Designation for NRC, the company's product candidate for the treatment of Ataxia Telangiectasia (AT). Plans are underway to file an Investigational New Drug (IND) application with the U.S. FDA in anticipation of conducting human clinical trials, which will be guided by Dr. Vilhelm (Will) Bohr, Prof., University of Copenhagen and Scientific Advisor to ChromaDex.

**"Over 30 million people in the U.S.** are impacted by more than 7,000 rare diseases, many of which are life-threatening and lack effective treatments," said Rob Fried, CEO of ChromaDex. "We believe NRC has potential as a treatment for AT."

"AT is a condition where children suffer from the adverse effects of premature aging and face a very limited life expectancy," said Dr. Andrew Shao, ChromaDex Senior Vice President of Global Scientific & Regulatory Affairs. "We are excited to continue supporting the AT community and eagerly anticipate the results from future research."

About Ataxia Telangiectasia (AT)

Ataxia Telangiectasia (AT) is a rare, progressive disease that typically presents in early childhood and is characterized by neurological and immunological symptoms. Those with AT often exhibit an unsteady gait (ataxia), impaired coordination of eye movements (oculomotor apraxia), and involuntary movements (choreoathetosis). AT leads to cerebellar degeneration and many affected children become wheelchair-dependent. Currently, there is no cure or FDA-approved treatment to slow the progression of AT, with the average life expectancy being around 25 years for those diagnosed in childhood.

There are many types of ataxia. Another form of ataxia is Friedreich's ataxia (FA), which is being addressed by Biogen and Larimar Therapeutics. FA impacts 1 in 50,000 people in the U.S. ( **NIH** ). Ataxia Telangiectasia (AT) impacts roughly 1 in 40,000 people in the U.S. (Riboldi et al., 2023; Tieve et al., 2015).

## Clinical Research on NRC and AT

To date, NRC has been investigated in two third-party funded, peer-reviewed published clinical trials for the treatment of AT. The first study published in **Movement Disorders** demonstrated that supplementation with NRC improved AT scores and increased immunoglobulins, or antibodies, in the immune-compromised patients, with AT score improvements reversing once supplementation concluded (Veenhius et al., 2021).

The second phase II two-year long study, also published in **Movement Disorders** , demonstrated that long-term NRC supplementation increased whole blood NAD+ levels up to fourfold, and improved neuromotor coordination and eye movements in 90% of participants while maintaining biomarkers of stable liver and kidney function, as compared to historical disease progression (Presterud et al., 2023). Both studies reported no serious adverse events, with NRC being generally well-tolerated.

Not associated with ChromaDex's future NRC IND filing, there are two additional investigator-initiated ongoing registered clinical trials that will examine NR supplementation in AT patients. The first is a continuation of Presterud et al. 2023 and will track AT patients over the course of 8-10 years. Recently registered, the second will be a single-arm open-label clinical trial scheduled to commence this year in Australia.

## Significance of Orphan Drug Designation (OOD) and Rare Pediatric Disease Designation (RPD)

**According to the FDA**, there are too few treatments for rare diseases because of high research and development costs, which companies often cannot recoup as a result of small patient populations. To incentivize companies to invest in bringing treatments to market for rare diseases, in 1983, Congress passed the Orphan Drug Act, which makes **Orphan Drug Designation (ODD)** candidates eligible for tax credits, waives their user fees, and may provide a period of exclusivity should the orphan drug be subsequently approved by FDA.

Related to ODD, the **FDA's Rare Pediatric Disease (RPD) designation**, further incentivizes companies to invest in rare childhood diseases by providing a voucher program to applicants approved by September 30th, 2024. Through this program, companies with RPD designation that ultimately obtain successful drug approval for a rare pediatric disease are provided a voucher, which can be used to expedite the FDA review of another drug candidate or sold to other companies.

## Future Clinical Trials on NRC and AT

With both designations granted, ChromaDex plans to file an Investigational New Drug (IND) application for future human clinical trials for the use of NRC in the treatment of AT. As a leading expert on the cell biology and biochemistry of AT, Dr. Bohr will serve ChromaDex as an advisor through this process.

Dr. Bohr remarked, "This is a significant step towards providing treatment for AT, a disease with no cure. It is an honor to work as an advisor on such pivotal research, and we are committed to advancing the science behind NRC to meet the urgent needs of this rare disease community."

"We are excited at the thought of embarking on these important future clinical trials for NRC as we are committed to advancing this to provide hope and relief for those suffering from this debilitating disease," commented Dr. Susan Perlman, MD, Clinical Professor of Neurology and Director of the Ataxia Center at the UCLA Medical Center in Los Angeles, and ChromaDex Clinical Consultant.

For additional information on the science supporting NRC and for future updates visit [www.chromadex.com](http://www.chromadex.com).

## Forward-Looking Statements:

This release contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities and 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" or the negative of such terms or other similar expressions, and include the statements regarding the potential benefits and development of NRC as a treatment for AT or other diseases, including statements regarding clinical trials and obtaining IND Designation from the FDA. These forward-looking statements are based on the Company's current expectations and are subject to risks and uncertainties that may cause actual results to differ materially, including unanticipated developments in and risks related to: the ability to continue to pursue additional studies, human trials, and to obtain an IND Designation from the FDA; 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; our ability to maintain sales, marketing and distribution capabilities; changing consumer perceptions of our products; our reliance on a single or limited number of third-party suppliers; and the risks and uncertainties associated with our business and financial condition. More detailed information about ChromaDex and the risk factors that may affect the realization of forward-looking statements is set forth in ChromaDex's Annual Report on Form 10-K for the fiscal year ended December 31, 2023, ChromaDex's Quarterly Reports on Form 10-Q and other filings submitted by ChromaDex to the SEC, copies of which may be obtained from the SEC's website at [www.sec.gov](http://www.sec.gov). 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 ChromaDex undertakes no obligation to revise or update this release to reflect events or circumstances after the date hereof.

## About ChromaDex:

**ChromaDex Corp.** (NASDAQ:CDXC) is the global authority on nicotinamide adenine dinucleotide (NAD+), with a focus on the science of healthy aging. The ChromaDex team, comprised 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 other everyday stressors. NAD+ depletion is a contributor to age-related changes in health and vitality.

Setting the benchmark as the gold standard in scientific rigor, safety, quality, and transparency, ChromaDex is the innovator behind its clinically proven flagship ingredient, Niagen® (patented nicotinamide riboside, or NR), the most efficient and superior-quality NAD+ booster available.

ChromaDex's robust patent portfolio protects NR or nicotinamide riboside chloride (NRC) and other NAD+ precursors. ChromaDex maintains a website at [www.chromadex.com](http://www.chromadex.com), to which ChromaDex regularly publishes copies of its press releases, news, and financial information.

## ChromaDex Media Contact:

Kendall Knysch, Senior Director of Media Relations & Partnerships

310-388-6706 ext. 689

[kendall.knysch@chromadex.com](mailto:kendall.knysch@chromadex.com)

## ChromaDex Investor Relations Contact:

Ben Shamsian

Lytham Partners

646-829-9701

**[shamsian@lythampartners.com](mailto:shamsian@lythampartners.com)**

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