Nurix Announces U.S. FDA Orphan Drug Designation Granted to Bexobrutideg (NX-5948) for the Treatment of Waldenström Macroglobulinemia

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Orphan Drug Designation follows positive Phase 1 data presented at the 12th International Workshop on Waldenström Macroglobulinemia

First-in-class Bruton's tyrosine kinase (BTK) degrader NX-5948 assigned nonproprietary name "bexobrutideg" in newly named degrader class of drugs

SAN FRANCISCO, March 17, 2025 (GLOBE NEWSWIRE) -- Nurix Therapeutics, Inc. (Nasdaq: NRIX), a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of targeted protein degradation medicines, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to bexobrutideg (NX-5948) for the treatment of Waldenström macroglobulinemia (WM). Bexobrutideg is an orally bioavailable, brain penetrant degrader of BTK which is being evaluated in an ongoing Phase 1a/b clinical trial in adults with relapsed or refractory B-cell malignancies.

The FDA's Orphan Drug Designation program provides orphan status to therapies intended for the treatment, diagnosis, or prevention of rare diseases that affect fewer than 200,000 people in the United States. This designation provides certain benefits, including tax credits for qualified clinical testing, waiver or partial payment of FDA application fees and seven years of market exclusivity, if approved.

"The FDA's Orphan Drug Designation for bexobrutideg, also known as NX-5948, represents an important milestone in our regulatory strategy and underscores the significant unmet medical need for improved treatments for Waldenström macroglobulinemia," said Arthur T. Sands, M.D., Ph.D., president and chief executive officer of Nurix. "Granting of the designation highlights bexobrutideg's potential to provide patients with WM a promising new therapeutic option. We are also pleased to announce that our investigational therapy bexobrutideg has been assigned a nonproprietary name reflecting its novel mechanism of action, designated with the unique suffix "deg" for degrader."

In collaboration with the national naming authority, United States Adopted Name (USAN) Council, Nurix's lead BTK degrader, NX-5948, was assigned the nonproprietary name "bexobrutideg." The U.S. and international drug naming convention is designed to select a single name of worldwide acceptability for each active substance that is intended to be marketed as a pharmaceutical. Most notable with bexobrutideg is the designation of a new suffix, "deg," which references bexobrutideg's novel degradation mode of action. Targeted protein degraders are characterized by their bifunctional nature, binding to both a target protein and a ligase to drive ubiquitination and catalytic degradation of the target through the proteasome. The new *deg* suffix is an important recognition that the mechanism of action, pharmacokinetics and pharmacodynamics of targeted protein degraders are fundamentally different than inhibitors, which all use the "ib" suffix. The central stem of the name, "bruti," references the target, Bruton's tyrosine kinase (as used in ibrutinib, zanubrutinib and acalabrutinib). The prefix "bexo" is the unique identifier of a specific agent in the class and is often used for ease of reference to the agent.

"We are excited that bexobrutideg has been recognized by the USAN Council as a unique entity and member of a new class of small molecule drugs, targeted protein degraders," said Gwenn Hansen, Ph.D., chief scientific officer of Nurix. "The catalytic mechanism of action and event driven pharmacology triggering ubiquitination and proteasomal degradation of a target protein is highly differentiated from inhibitors and allows degraders to eliminate the totality of a protein's function. In our BTK degrader clinical program, we have also established that degraders can eliminate mutant oncoproteins that have proven to be resistant to inhibitor therapy."

About Bexobrutideg (NX-5948)
Bexobrutideg is an investigational, orally bioavailable, brain penetrant, small molecule degrader of BTK that is currently being evaluated in a Phase 1 clinical trial in patients with relapsed or refractory B cell malignancies. Nurix has previously reported encouraging safety and efficacy data in patients with WM treated in the ongoing Phase 1a/b clinical trial of bexobrutideg demonstrating early promise of clinical benefit with potential for durable outcomes. Nurix continues to enroll patients with WM in an ongoing Phase 1b expansion cohort and anticipates sharing additional clinical data in 2025. Additional information on the ongoing clinical trial can be accessed at clinicaltrials.gov (NCT05131022). Nurix is also developing bexobrutideg for the potential treatment of inflammatory diseases.

About Waldenström Macroglobulinemia (WM)

WM is a rare, slow growing type of non-Hodgkin's lymphoma that is characterized by the replacement of normal bone marrow cells by malignant lymphocytic cells that produce monoclonal IgM. This replacement leads to anemia, bleeding, and impaired immune function, while the elevated IgM levels may cause neurologic symptoms. The incidence of Waldenström macroglobulinemia ranges from 0.361,2 to 0.573 per 100,000 people in the United States or approximately 1,200 to 1,900 annually. With a median disease duration approaching 10 years, 4 approximately 12,000 to 19,000 patients are living with Waldenstrom's macroglobulinemia in the United States. Recommended first-line treatments including chemoimmunotherapy and BTK inhibitor (BTKi) therapy. There are no therapies approved to treat WM patients after a BTKi.

About Nurix Therapeutics, Inc.
Nurix Therapeutics is a clinical stage biopharmaceutical company focused on the discovery, development and commercialization of targeted protein degradation medicines, the next frontier in innovative drug design aimed at improving treatment options for patients with cancer and inflammatory diseases. Nurix's wholly owned, clinical stage pipeline includes degraders of Bruton's tyrosine kinase (BTK), a B-cell signaling protein, and inhibitors of Casitas B-lineage lymphoma proto-oncogene B (CBL-B), an E3 ligase that regulates activation of multiple immune cell types including T cells and NK cells. Nurix also is advancing multiple potentially first-in-class or best-in-class degraders and degrader antibody conjugates (DACs) in its preclinical pipeline. Nurix's partnered drug discovery pipeline consists of preclinical stage degraders of IRAK4 and STAT6, as well as multiple additional programs under collaboration agreements with Gilead Sciences, Inc., Sanofi S.A. and Pfizer Inc., within which Nurix retains certain options for co-development, co-commercialization and profit sharing in the United States for multiple drug candidates. Powered by a fully AI-integrated discovery engine capable of tackling any protein class, and coupled with unparalleled ligase expertise, Nurix's dedicated team has built a formidable advantage in translating the science of targeted protein degradation into clinical advancements. Nurix aims to establish degrader-based treatments at the forefront of patient care, writing medicine's next chapter with a new script to outmatch disease. Nurix is headquartered in San Francisco, California. For additional information visit http://www.nurixtx.com.

Forward-Looking Statements
This press release contains statements that relate to future events and expectations and as such constitute forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. When or if used in this press release, the words "anticipate," "believe," "could," "estimate," "expect," "intend," "may," "outlook," "plan," "predict," "should," "will," and similar expressions and their variants, as they relate to Nurix, may identify forward-looking statements. All statements that reflect Nurix's expectations, assumptions or projections about the future, other than statements of historical fact, are forward-looking statements, including, without limitation, statements regarding: Nurix's future plans, prospects and strategies, including with respect to bexobrutideg; the potential benefits of Orphan Drug Designation; the tolerability, safety profile, therapeutic potential and other advantages of bexobrutideg; and the planned timing for the provision of updates and findings from Nurix's clinical trials. Forward-looking statements reflect Nurix's current beliefs, expectations, and assumptions. Although Nurix believes the expectations and assumptions reflected in such forward-looking statements are reasonable, Nurix can give no assurance that they will prove to be correct. Forward-looking statements are not guarantees of future performance and are subject to risks, uncertainties and changes in circumstances that are difficult to predict, which could cause Nurix's actual activities and results to differ materially from those expressed in any forward-looking statement. Such risks and uncertainties include, but are not limited to: (i) whether Nurix will be able to advance, obtain regulatory approval of and ultimately commercialize bexobrutideg; (ii) whether Nurix will be able to fund development activities and achieve development goals; (iii) the impact of global business, political and macroeconomic conditions, cybersecurity events, instability in the banking system, and global events, including regional conflicts around the world, on Nurix's business, clinical trials, financial condition, liquidity and results of operations; (iv) whether Nurix will be able to protect intellectual property and (v) other risks and uncertainties described under the heading "Risk Factors" in Nurix's Annual Report on Form 10-K for the year ended November 30, 2024, and other SEC filings. Accordingly, readers are cautioned not to place undue reliance on these forward-looking statements. The statements in this press release speak only as of the date of this press release, even if subsequently made available by Nurix on its website or otherwise. Nurix disclaims any intention or obligation to update publicly any forward-looking statements, whether in response to new information, future events, or otherwise, except as required by applicable law.

Contacts:

Investors
Jason Kantor, Ph.D.
Nurix Therapeutics, Inc.
ir@nurixtx.com

Elizabeth Wolffe, Ph.D.
Wheelhouse Life Science Advisors
lwolffe@wheelhouselsa.com

Media
Aljanae Reynolds
Wheelhouse Life Science Advisors
areynolds@wheelhouselsa.com