

Press release

Inverna Therapeutics receives grant from The Michael J. Fox Foundation for Parkinson's Research to advance novel LRRK2-targeted RNA therapy for Parkinson's Disease

Odense, Denmark – October 09, 2025 – Inverna Therapeutics (Inverna), a Danish biotech company, today announced it has been awarded a research grant from The Michael J. Fox Foundation for Parkinson's Research (MJFF) through its <u>LRRK2 Investigative Therapeutics Exchange</u> (LITE) program. The LITE program supports the development of new therapies targeting LRRK2 for the treatment of Parkinson's disease patients. The grant will support the development of a novel LRRK2 RNA therapeutics program, offering fresh hope for a potential precision medicine treatment for Parkinson's disease patients.

Parkinson's disease affects an estimated 6 million people worldwide, a number projected to double by 2040. The LRRK2 gene plays a central role in regulating neuronal health, and mutations in LRRK2 are the most common inherited cause of Parkinson's disease. Furthermore, abnormal LRRK2 kinase activity is observed even in patients with sporadic Parkinson's disease. Excessive LRRK2 activity disrupts normal brain cell function, leading to the progressive movement and cognitive symptoms characteristic of the disease.

Inverna will utilize its proprietary RNA therapeutics platform for screening and identification of LRRK2 pseudo-exon activator oligonucleotides. These oligonucleotides are designed to potently and selectively remove LRRK2 RNA from cells, aiming to downregulate LRRK2 activity and thereby halt Parkinson's disease progression through a novel, first-in-class RNA-based medicine. Inverna's novel pseudo-exon activators have already shown significant promise within CNS disorders due to their unprecedented target specificity.

"We are honored to receive this support from The Michael J. Fox Foundation, which enables us to advance a highly innovative therapeutic strategy for Parkinson's," said Poul Sørensen, PhD, CEO at Inverna Therapeutics. "Our splice-switching platform is uniquely designed to modulate gene expression with exceptional specificity, and applying it to LRRK2 represents a major step toward disease-modifying RNA medicines in neurodegeneration".

"The supported project will allow us to develop highly specific treatments that enable either removal of total LRRK2 protein or allele-specific removal of only the disease-associated mutant LRRK2 protein. We hope that this can be developed into an effective, long-lasting therapy, where LRRK2 activity is decreased in a specific and controllable manner to alleviate LRRK2-associated neuronal dysfunction and cause a decline of disease progression in patients," said PI Brage S. Andresen, CSO of Inverna Therapeutics.

Under the terms of the 18-month grant, Inverna will conduct a set of initial in vitro screening activities, lead optimization, and subsequent testing in animal models of Parkinson's disease. These preclinical studies will use patient-derived cellular systems and *in vivo* Parkinson's disease mouse models to evaluate the efficacy, selectivity, and safety of their proprietary oligonucleotide candidates. The goal is to advance this therapeutic approach toward clinical development.

"The Michael J. Fox Foundation is steadfast in its mission to accelerate the development of better treatments and move us even closer to a cure for Parkinson's disease," said Shalini Padmanabhan, PhD, senior vice president and head of translational research at MJFF. "Through the LRRK2 Investigative Therapeutics Exchange, we are advancing LRRK2 drug development while reducing barriers to investment through open-science policies and expert collaboration. We welcome Inverna to this international effort and look forward to working together to advance progress on potential disease-modifying therapies for Parkinson's."

About Inverna Therapeutics

Inverna Therapeutics is a Danish biotech company, founded in 2024 and based in Odense. With its proprietary drug discovery platform, Inverna is dedicated to developing transformative RNA therapeutics based on RNA splicing modulation. The company aims to rapidly design, test, and develop highly specific activators of pseudo-exons in disease-relevant target RNA. With a strong scientific foundation and an experienced, talented team, Inverna is committed to advancing novel RNA therapeutics and bringing hope to patients.

Learn more at https://www.invernatx.com/.

Contact: Poul Sørensen, CEO of Inverna Therapeutics

Email: psorensen@invernatx.com

About the LRRK2 Investigative Therapeutics Exchange (LITE) Program

The Michael J. Fox Foundation for Parkinson's Research (MJFF) launched LITE in 2024 to pave the way for new therapeutic approaches for LRRK2, connect companies that are developing LRRK2-targeting therapies with pharma and biotech opinion leaders, and provide preclinical and clinical resources to establish best practices for advancing LRRK2 targeted therapeutics. Mutations in the LRRK2 gene linked to Parkinson's disease were first discovered in 2004 and are now understood to be the most common cause of inherited PD. Built on MJFF's dedication to open science, LITE fosters international collaboration across more than 30 academic and clinical centers and more than a dozen companies. The initiative is governed by an active steering committee consisting of MJFF staff and field leaders and is implemented by the University of Dundee in the United Kingdom. The LITE program also will benefit from collaboration with the Aligning Science Across Parkinson's (ASAP) initiative-supported programs including the Collaborative Research Network (CRN), the Parkinson's Progression Markers Initiative (PPMI) and the Global Parkinson's Genetics Program (GP2). Learn more here.

Learn more at https://www.michaeljfox.org/grant/lrrk2-investigative-therapeutics-exchange-lite