nLife Therapeutics receives USD 350,000 (EUR 271,000) from The Michael J. Fox Foundation for Parkinson’s Research

The funding will help characterize the mechanism of action of nLife’s lead compound NLF-PD-1233 in a Parkinson disease model and find a proper dose to engage clinical development

Granada, Spain – September 23, 2014 – nLife Therapeutics, an early-stage biopharmaceutical company leading developments in nOligos (neuronal specific oligonucleotides) as therapeutic agents for CNS disorders, today announces that The Michael J. Fox Foundation for Parkinson’s Research (MJFF) has awarded the company a grant of USD 350,000 (EUR 271,000).

nLife will conduct a research study entitled: “Pharmacokinetic and Pharmacodynamic characterization of a novel therapy to silence selectively alpha-synuclein in monoaminergic neurons of rat.”

The money will be used to characterize the pharmacokinetic (distribution of the molecule) and pharmacodynamic (effect on target protein) profiles of NLF-PD-1233, nLife’s lead compound.

This is only the second grant from the MJFF to a Spanish company (as opposed to academia). Since its inception in 2000, the MJFF has awarded more than 1,750 grants worldwide, with 24 of them involving investigators from Spanish organizations.

nLife Therapeutics designed the NLF-PD-1233 molecule to target specific neurons in the brain to reduce expression of the alpha-synuclein protein, a major constituent of Lewy bodies (protein clumps that are the pathological hallmark of Parkinson’s disease). The MJFF-funded project aims to characterize the dose and efficacy of the molecule in an alpha-synuclein overexpression model of Parkinson’s disease (PD) vs. a healthy model. The results are due in November 2015.

The funding enables nLife to study the processes of absorption, distribution, elimination and efficacy of the compound. The primary endpoint of the study is to find a suitable treatment dose for intranasal and direct brain administration. nLife will compare the drug concentrations achieved when given through the nose to those when given directly to the brain or intravenously.

The secondary endpoint will be to characterize the efficacy of NLF-PD-1233, analyzing the knockdown of alpha-synuclein mRNA expression and alpha-synuclein protein changes. Then, the company intends to characterize the concentration-response relationship for NLF-PD-1233 and alpha-synuclein mRNA knockdown. The resulting data will be used to model the projected dose-effect relationships for larger animal models and for humans. Clinical trials could start in December 2015.

“Based on our preclinical findings, we believe NLF-PD-1233 may be able to slow down the progression of PD and relieve motor and non-motor symptoms,” said Andres Montefeltro, CEO at nLife Therapeutics. “If the results are sustained in humans, it could be the first disease-modifying treatment for PD.”
About Parkinson’s disease

Parkinson’s disease is a progressive disorder of the central nervous system, characterized by neuron loss and symptoms including tremor, rigidity, cognitive decline and autonomic dysfunction. Parkinson’s disease has no cure at present, but drugs help to control the symptoms.

The motor symptoms of Parkinson’s are due to a loss of dopaminergic neurons. Current medications aim to supplement dopamine. The most effective medication is levodopa (which is converted into dopamine once it reaches the brain), however this drug loses its efficacy after years of use and dyskinesia (involuntary movements) are observed at high dose.

Another approach is based on the direct administration of dopamine agonists. Although this medication lasts longer, it is less effective than levodopa. More recently, alternative strategies have been marketed focused on the regulation of enzymes directly involved in the dopamine metabolism, but many side effects are observed (e.g. increased dyskinesia, hallucination, headaches).

About alpha-synuclein and Parkinson’s disease

Alpha-synuclein is a protein present in neurons. Its normal function is not fully understood, but researchers believe this protein plays a role regulating synaptic vesicles and on the release of specific neurotransmitters. This protein is a major component of Lewy bodies, which are aggregates characteristic of Parkinson’s disease. Reducing accumulation of alpha-synuclein is one of the main goals for new therapies in Parkinson’s disease.

About nLife’s nOligos technology

nLife has a unique neuronal specific oligonucleotide delivery technology (nOligos). Oligonucleotides are short nucleic acid polymers designed to bind to specific complementary messenger RNA strands promoting its degradation, which produces a decrease in the levels of specific proteins that become toxic when they accumulate. The targeting technology allows decreasing levels of toxic proteins in only the affected cells, which spares the healthy ones. Targeting is achieved by attaching a neuronal specific chemical ligand to the oligonucleotide. Thus nOligos have the potential to become a disease modifying therapy, and can be applied to un-druggable target proteins, allowing the development of therapies for diseases that might otherwise have no cure.

About nLife Therapeutics

nLife Therapeutics is an early-stage biopharmaceutical company leading developments in nOligos (neuronal specific oligonucleotides) as therapeutic agents for CNS disorders. nLife has a unique oligonucleotide delivery platform with strong IP protection to target several neurodegenerative conditions.

Its lead candidate (NLF-PD-1233) is a First in Class disease modifying oligonucleotide treatment for Parkinson’s disease (PD). The company has
outstanding preclinical data that validates the nOligos Mode of Action in Major Depressive Disorder and PD. The company also has programs targeting Huntington and Alzheimer disease underway.

Founded in 2010 and based in Granada (Spain), nLife has raised a total of USD 11 million since inception. The investors are private Angels and three Spanish VCs: Crossroad Biotech, Caixa Capital Biomed and HealthEquity.

For more information - [http://www.n-life.es](http://www.n-life.es)

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