RS-2025-02213047

Nonclinical efficacy and safety evaluation of an LRRK2 inhibitor candidate for the development of a disease-modifying Parkinson's disease therapy and subsequent IND approval

Novorex Inc.

NEUROLOGY	Preclinical

Product Type	Small molecules
Indication	Parkinson's disease
Target	LRRK2
MoA(Mechanism of Action)	Through its inhibitory effect on LRRK2, NRX02067 restores lysosomal and mitochondrial functions, thereby promoting the degradation of α-synuclein aggregates in the brain and ultimately preventing the degeneration of dopaminergic neurons—a pathological hallmark of Parkinson's disease
Competitiveness	• A novel fragment-based drug discovery approach has yielded a promising lead compound. With a small molecular size (~250 Da), it demonstrates excellent blood-brain barrier (BBB) penetration (Kp,uu,brain: 0.40). • It shows superior efficacy over competitors in restoring lysosomal and mitochondrial function. • Notably, NRX02067 exhibits significant efficacy in an α-synuclein-induced Parkinson's disease model using LRRK2 wild-type mice (C57BL/6J), without observed toxicity.
Development Stage	Preclinical
Route of Administration	Oral

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