

Preclinical development of an HDAC6 inhibitor targeting Axonal Transport dysfunction in Charcot-Marie-Tooth Disease

NEUROLOGY	Preclinical
Product Type	Small molecule
Indication	Charcot-Marie-Tooth disease
Target	HDAC6 inhibitor, small molecule
MoA(Mechanism of Action)	Restoring of defective Axonal Transport and α -tubulin acetylation mediated Microtubule Stabilization
Competitiveness	<ul style="list-style-type: none"> • Unique mechanism of action <ul style="list-style-type: none"> - Expected differentiated efficacy through a novel target (Axon), distinct from approved and investigational drugs • Overcoming limitations of other HDAC6 inhibitors <ul style="list-style-type: none"> - Superior target tissue exposure (Brain, Sciatic nerve), - High HDAC6 potency and selectivity - Low metabolites, Wide safety margin • Rare + Common disease hybrid strategy <ul style="list-style-type: none"> - Proven robust efficacy in CMT1/2 model mice - Enhanced LTP and Cognitive functions demonstrated in PS19 Tauopathy mice • Excellent drug-like profile <ul style="list-style-type: none"> - Orally suitable physicochemical properties, Favorable pharmacokinetic profiles
Development Stage	Preclinical
Route of Administration	Oral administration

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