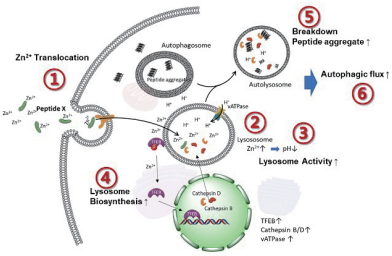
RS-2023-00218770

Discovery the preclinical candidate for amyotrophic lateral sclerosis (ALS) based on the activation of lysosomes using small-molecular proteins.

Zincure

NEUROSCIENCE | **Candidate**

Product Type	Small Peptide (unnatural)
Indication	Amyotrophic lateral sclerosis (ALS)
Target	Lysosome Activation
MoA(Mechanism of Action)	1. Receptor-Mediated Endocytosis of Peptide X 2. Increase in Zn2+ within Lysosomes 3. Lysosomal Acidification 4. Stimulation of Lysosome Biogenesis 5. Degradation of Toxic Protein Aggregates 6. Enhancement of Autophagic Flux 
Competitiveness	The Zincure's drug candidate represents a unique approach, being a first-in-class medication with a distinct mechanism of action. It directly targets toxic protein aggregates implicated in the disease's pathogenesis by activating lysosomes. Notably, TDP-43 aggregate accumulation has been observed in over 90% of ALS patients. This remarkable drug, a small peptide containing unnatural amino acids, possesses numerous outstanding qualities. It exhibits exceptional properties, including metabolic stability and the absence of CYP inhibition, making it a highly promising therapeutic candidate. Furthermore, it demonstrates an impressive ability to penetrate the blood-brain barrier. Considering the prevalence of toxic protein aggregate accumulation in various neurodegenerative diseases, such as beta-amyloid for Alzheimer's disease, and Lewy bodies for Parkinson's disease, the potential therapeutic applications of Zincure's drug candidate extend beyond ALS, presenting exciting possibilities for a broader impact.
Development Stage	Candidate
Route of Administration	Subcutaneous

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