

# Development of first-in-class drugs to treat Leber's hereditary optic neuropathy (LHON)

UNIST (Ulsan National Institute of Science and Technology)



OPHTHALMOLOGY	Hit
Product Type	Small molecule
Indication	Leber Hereditary Optic Neuropathy (LHON)
Target	Mitochondrial chaperone
MoA (Mechanism of Action)	Activating the mitochondrial chaperone stabilizes and restores impaired mitochondrial electron transport chain complexes, leading to increased ATP production and reduced ROS generation.
Competitiveness	<ul style="list-style-type: none"> <li>• <b>First-in-class drug</b> targeting a mitochondrial chaperone, addressing the root cause of mitochondrial dysfunction in LHON and Parkinson's disease.</li> <li>• <b>Disease-modifying mechanism:</b> Unlike current symptomatic therapies such as Idebenone for LHON or L-DOPA and dopamine agonists for Parkinson's disease, Activation of the mitochondrial chaperone directly restores mitochondrial ETC stability and function, leading to sustained recovery of cellular energetics and inhibition of neuronal cell death.</li> <li>• <b>Broad therapeutic potential:</b> Applicable to a wide spectrum of mitochondrial and neurodegenerative diseases, including MELAS and Parkinson's disease, caused by mitochondrial ETC defects.</li> </ul>
Development Stage	Hit
Route of Administration	oral

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