

# Discovery of lead compounds targeting TDP43 for treatment of Amyotrophic lateral sclerosis (ALS)

KIST (Korea Institute of Science and Technology)



NEUROLOGY	Hit
Product Type	Small molecule
Indication	Amyotrophic lateral sclerosis (ALS)
Target	TDP43 protein
MoA(Mechanism of Action)	Inhibition of TDP43 oligomerization
Competitiveness	First-in-class <ul style="list-style-type: none"> <li>• Target TDP43 cysteine residues to block disulfide-linked oligomerization</li> <li>• Prevents ALS-associated TDP43 aggregation and cytoplasmic accumulation</li> </ul>
Development Stage	Hit
Route of Administration	Oral administration

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