HN22C0723

Drug development for Duchenne muscular dystrophy by increasing mRNA stability

RiboTech

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METABOLIC **Lead**

Product Type	Chemical drug
Indication	Duchenne muscular dystrophy
Target	Dystrophin
MoA(Mechanism of Action)	20% of Duchenne muscular dystrophy patients have nonsense mutations in the dystrophin gene resulting in nonsense-mediated mRNA decay (NMD) of dystrophin mRNA. Administration of NMD inhibitors may restore dystrophin expression in those patients.
Competitiveness	- Most drugs for DMD(Duchenne muscular dystrophy) are supportive therapy aiming to reduce symptoms and do not affect the underlying cause. - We discovered several NMD-inhibiting compounds by high-throughput screening. - MOA(mechanism of action) studies of the compounds should facilitate drug development for DMD.
Development Stage	Lead
Route of Administration	Oral administration

kddf_bd@kddf.org
KDDF Business Development Team

KDDF
Korea Drug Development Fund