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Development of a Sarcopenia Therapeutic Based on New-Modality Protein Degradation Technology

Mitos Therapeutics Inc.

OTHERS | Hit

Product Type	TPD
Indication	Treatment of Sarcopenia
Target	XXXXX(Master regulator of myogenic differentiation, chromatin-binding transcription regulator)
MoA(Mechanism of Action)	First-in-class Targeted Protein Degradation (TPD) of an undruggable nuclear regulator. Modality: Small-molecule Molecular Glue Degrader (2nd-generation TPD, linker-free) Mechanism: Recruits an endogenous E3 ligase to the target protein, inducing a ternary complex → poly-ubiquitination → proteasomal degradation. Releases the repression on myogenic transcription, restoring MyoD / Myogenin / MHC.
Competitiveness	**First-in-class — no FDA-approved sarcopenia therapeutic exists worldwide.** • **Opposite strategy:** Existing programs (myostatin/activin antibodies, SARMs, mTOR modulators) activate muscle growth and have failed Phase II. Our approach inhibits an upstream repressor of myogenesis. • **New modality advantage:** Linker-free Molecular Glue — small MW, oral, low off-target risk vs. PROTAC. Selectively degrades target in muscle tissue. • **Proprietary discovery platform:** Target promoter-GFP reporter for HTS; validated in C2C12 and target-overexpressing mouse models; 4 distinct *in vivo* sarcopenia models (CTX-, HFD-, Velcro-, age-induced). • **Confirmed aging biomarker:** Target expression rises with age in skeletal muscle (4W → 24M); Reversed *in vivo* by lead compound at 10 mg/kg.
Development Stage	Hit
Route of Administration	**Oral, once-daily (small molecule)** Designed for chronic outpatient use in elderly patients — high compliance, no injection burden.