ADENOVIRUS-ASSOCIATED VIRUS TRANSDUCTION OF PARKIN PROTEIN AS THERAPEUTIC TARGET TO PRESERVE AND IMPROVE SKELETAL MUSCLE FUNCTION

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ALIGO INNOVATION

BACKGROUND

Aging is associated with progressive loss of muscle mass and strength, a biological process called sarcopenia. Strong evidence indicates that mitochondrial dysfunctions occur with aging and are critical in the sarcopenia process. Recent evidence suggests that mitophagy, the process in charge of removing the accumulation of damaged mitochondrial dysfunctions is impaired in aged muscles.

Parkin protein which is implicated in the pathogenesis of the neurodegenerative Parkinson disease, is also prominently expressed in skeletal muscle. Growing evidence suggests that Parkin is playing a central role in the regulation of mitophagy by mediating the turnover of dysfunctional mitochondria.

TECHNOLOGY

The present invention refers to a muscle – specific system for Parkin overexpression. Indeed we have shown that in young mice, Parkin-overexpressing muscles displayed hypertrophy (higher muscle weight and fiber size). In old skeletal muscle, Parkin overexpression significantly attenuated sarcopenia and resulted in increased biogenesis, increased mitochondrial volume density and decreased oxidative stress. These results suggest that Parkin plays a regulatory role allowing the muscle to keep its functions or improve them regardless of age.

COMPETITIVE ADVANTAGES

- Unmet medical need for sarcopenia
- Systemic injection with muscular specificity
- Alternative treatment to anabolic products for muscular increase (avoid secondary effects)

APPLICATIONS

- Attenuation of muscle atrophy related to several medical conditions including sepsis, aging and degenerative diseases (cancer, AIDS/HIV)
- Reduction of oxidative stress in metabolic diseases (Type II diabetes)
- Improvement of muscle performance

TECHNOLOGY DEVELOPMENTAL STAGE

In vivo proof-of-concept.

PATENT STATUS

Will be filed shortly, already talking to patent agent.

BUSINESS OPPORTUNITY

Out licensing or partnering opportunities available.

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