MENU

NIA Small Business Showcase: Ridgeline Therapeutics, LLC

Sarcopenia is one of the most widespread chronic diseases affecting older adults. The disease is characterized by progressive age-associated skeletal muscle degeneration, resulting in significant loss of muscle strength, function, and regenerative capacity. Sarcopenia greatly reduces overall health and quality of life in the elderly. Moreover, sarcopenia is a core component of debilitating frailty, which severely affects an individual's independence and longevity. There are no FDA-approved treatments to prevent or reverse sarcopenia. Current recommendations for preventing and treating sarcopenia include resistance training and a protein-rich diet, although there is limited evidence to suggest that these methods are effective.

Ridgeline Therapeutics is developing first-in-class oral drugs that improve muscle strength, function, and regeneration in the elderly, thus increasing mobility, well-being, and independence. Ridgeline's novel therapeutic selectively targets and inhibits a methyltransferase enzyme upregulated in aged skeletal muscle and recently found to regulate cellular metabolic pathways, particularly NAD biosynthesis, which is highly compromised in aged muscle.

This drug will be introduced into clinical trials and the market in a phased manner, initially as a drug to improve hip fracture recovery outcomes in the elderly, next as a treatment for sarcopenia, and finally as a medication to reverse muscle decline in the elderly. Ridgeline's novel once-daily pill increases the growth, regenerative capacity, and function of aged muscles, thereby promoting better health and well-being in older adults. Advantages of a daily pill include high patient adoption and low manufacturing and distribution costs.

Since each year 300,000 elderly Americans suffer a hip fracture, the market for treatments is conservatively estimated at $1.1 billion annually. Additionally, 12 million Americans over age 65 have sarcopenia, which suggests the market for the first-in-human sarcopenia drug is $3 billion to $4 billion annually. Global markets, specifically the European Union (EU) and Japan, are comparable.

Company Milestones

Scientific

- 2020: Chemistry, manufacturing, and control scale-up completed. Proof-of-concept (POC) animal efficacy studies completed and published showing that treatment of aged mice resulted in increased muscle stem cell proliferation and myofiber regeneration, twofold larger muscle fiber growth, and 70% greater peak muscle contractile function.
- 2020: Testing second-generation clinical leads in ongoing POC sarcopenia trials.
- 2020: Non-GLP (good laboratory practice) safety/toxicity studies completed in two species.
- 2021: Planned GLP safety/toxicity studies on target for Quarter 1 of 2021.

Business

- 2019: Raised nondilutive funding in excess $5 million in 12 months.
- 2019: Completed filing of national (e.g., United States, Canada, EU, Japan, Australia) patent applications under the Patent Cooperation Treaty.
- 2019: Obtained exclusive licensing for composition-of-matter and use for technologies used in first-generation clinical candidates.
- 2020: Additional $2 million in nondilutive funding pending.
- 2021: Anticipating investigational new drug (IND) filing in Quarter 3 of 2021.

Financial Overview

Ridgeline has raised more than $5 million in funds from nondilutive (e.g., U.S. Department of Defense, NIH) sources. An additional $2 million in nondilutive funding is expected in 2021 for further POC and GLP safety studies. A seed round of $6 million is planned to close Q3 of 2020, with funds allocated to an IND filing and a Phase I clinical trial.

Intellectual Property

Ridgeline has established licensing agreements with the University of Texas Medical Branch that provide the company with exclusive rights to all NNMT inhibitor–related technologies and freedom to operate, with a lead position in pursuing additional NNMT patents as the company continues to commercialize this technology.

Product Development and Regulatory Strategy

Ridgeline’s leading drug candidate has entered regulated GMP (good manufacturing practice) production and GLP safety/toxicity studies. Ongoing studies are focused on finalizing formulation, manufacturing, and packaging specifications for clinical use, assembling and submitting an IND application to FDA, and delivering a clinical candidate to medical sites for FDA-regulated first-in-human Phase I and II clinical trials. These trials are the most significant value-added inflection points along the company’s strategic path to commercialization. To add substantial value to the drug product and company, Phase I and II clinical trials must unambiguously demonstrate the safety of the company’s drug and superior efficacy in increasing muscle strength relative to current treatments, such as resistance training.

Commercialization Strategy

Ridgeline envisions partnership with a pharmaceutical company, outlicensing of its clinical lead, or venture financing as potential strategies for completing Phase III clinical trials. Marketing would occur throughout licensing to pharmaceutical companies.

Company Details

Ridgeline Therapeutics, LLC website

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Industry: Therapeutics

Management Team:

- Executive Director for Research & Development: Harshini Neelakantan, Ph.D.
- Executive Chair: Neil Warma, M.B.A.
- Founder and Chief Executive Officer: Stanley Watowich, Ph.D.

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Conference Selected for Showcase: BIO Digital (June 2020)

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