



## Executive Summary

### Corporate History

Aavogen (Ay-voh-jen) is a preclinical stage biotech company that develops gene therapeutics for muscle wasting. *Our mission is to make patients stronger.* The company was founded by a single family directly impacted by Duchenne muscular dystrophy (DMD), spinocerebellar ataxia, cancer cachexia and chronic obstructive pulmonary disease. We are, therefore, deeply committed to patient advocacy and affected families.

### Problem Statement

Muscle wasting is a chronic condition affecting over 2% of the world's population. It occurs in myriad conditions including muscle and neuromuscular diseases with treatment costs exceeding \$600B annually. *Such treatments, however, cannot restore muscle mass or function and focus instead on preventing future degeneration.*

### Solution/Pipeline

1. **AVGN7 (AAVMYO2:CK8e-hSmad7):** Adeno-associated viral gene therapeutic that enhances muscle mass and function. Industry-leading muscle specificity is directed by the combined effects of the liver de-targeted muscle tropic capsid, AAVMYO2, and the CK8e promoter that is optimized to only activate in striated muscle. Self-complementary genomes enrich expression of the patented codon-optimized human Smad7 gene, reducing dose requirements and manufacturing burden. Smad7 attenuates processes that normally inhibit muscle growth, thereby enhancing muscle mass and strength as well as exercise capacity and cardiac function. Moreover, it attenuates multiple inhibitory signals, has lasting effects with a single injection and can be used systemically or locally to treat a variety of chronic and rare diseases.
2. **AVGND (AAVMYO2:CK8e- $\mu$ DysH3):** This "micro-dystrophin" gene therapeutic shares the same capsid, promoter and muscle-specificity as AVGN7. The  $\mu$ DysH3 gene restores dystrophin function, stabilizes skeletal and cardiac muscle and prevents degeneration in subjects with DMD. Its biological activity is identical to other micro-dystrophin gene therapeutics, yet it does not produce ringbinden and myotendinous tears caused by competing "H2-containing" constructs (Sarepta, Insmed & Novartis/Kate).

### Opportunity

The lead indication is sporadic inclusion body myositis (IBM). This inflammatory myopathy is an FDA-classified rare disease and results from inflammatory insult rather than genetic mutations. Muscle degeneration develops progressively and requires durable treatments, like AVGN7, that can also attenuate the inflammatory signals underlying muscle wasting. An AVGN7+AVGND combinatorial will address a market weakness in treating DMD; no technology (i.e. gene replacement/editing or exon skipping) has ever restored dystrophic muscle to normal healthy levels due to the accumulative pathologies like muscle necrosis and fibrosis. In fact, Aavogen's highly innovative approach is the *industry's only "one-two combo punch" that compensates for these pathologies* by preventing muscle degeneration with AVGND and restoring muscle mass and function with AVGN7. It also expands treatment options to older patients with advanced disease progression.

### Market Summary

IBM occurs at a prevalence of 160/1M people over 50 years of age, equal to ~23K US subjects. Assuming a typical 10% penetration for rare disease markets, and conservative pricing (\$2M/patient therapy) based on muscle disease genetic medicines (e.g., Zolgensma, Spinraza, Elevidys, etc.), sales are estimated to reach \$4.6B in year 1. DMD occurs with 1 in every 4.3K male births, equal to ~16K US subjects. Similar penetration and pricing assumptions (\$3M/patient) estimate year 1 sales at \$4.8B.

### Business/Funding Model

A virtual structure minimizes development costs through IND filing. Fund raising is linked to detailed milestones and coordinated exit positions. It is also leveraged by \$5.6M of non-dilutive funding raised to date, an additional \$3M towards a current \$12M series A raise, established partnerships and a Maryland tax incentive. Out-licensing and "option-to-acquire" deals will support manufacturing and development into additional disease indications, most notably heart failure as well as combinatorial approaches for AVGN7 with other genetic medicines.