

## Industry: Biotech

### Target Indications:

- Inclusion body myositis (IBM)
- Duchenne muscular dystrophy (DMD)

### Future indications:

- Heart failure/reduced ejection fraction
- Heart failure/preserved ejection fraction
- Sarcopenic obesity

## Management

Buel "Dan" Rodgers, PhD  
Founder & CEO

Alexandra "Alex" McPherron, PhD  
CSO

William Mann, PhD/MBA  
COO/SAB

Joe Rininger, PhD  
CMC Director

Sarah Herring, PhD  
Preclinical Director

Tom Lloyd, MD/PhD  
Clinical Director

## Science Advisory Board

William Mann, PhD/MBA (Chair)

Tom Lloyd, MD/PhD

Paul Gregorevic, PhD

Jeff Chamberlain, PhD

Brian O'Rourke, PhD

Dirk Grimm, PhD

## Intellectual Property

- Several issued patents for composition of matter and methods of use
- 1 pending patent

## Funding to Date

- \$5.6M NIH SBIR grants
- \$400K convertible notes
- \$3M towards series A

## 2026 Raises

### Non-diluting:

- \$3.2M NIH/NIA SBIR grant
- \$2M NIH/NIAMS SBIR grant

### Series A:

- \$12M by Q4
- \$3M secured from note investor, Eurofarma Ventures
- Complete AVGN7 preclinical studies and CMC readiness
- File IND for IBM
- Start preclinical studies for DMD combinatorial, AVGN7/AVGND

## Contact Information

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## Executive Summary:

- Aavogen is a preclinical stage biotech startup that develops adeno-associated viral (AAV) gene therapeutics for muscle wasting diseases
- Two therapeutics have been developed to restore muscle mass and function: AVGN7 for inclusion body myositis (IBM) and heart failure (HF) as well as an AVGN7+AVGND combinatorial for Duchenne muscular dystrophy (DMD)
- Aavogen is positioned to acquire an IND and orphan drug status for IBM, and to initiate clinical development ~18 months after program funding
- Potential to pursue multiple disease indications with AVGN7 is the cornerstone of corporate development due to its broad applicability and conserved pathobiology with different muscle and neuromuscular diseases

## Market Opportunity/Unmet Need:

### IBM (a.k.a. sporadic IBM) – lead indication

- Rare disease (160/1M age 50+), typically diagnosed in middle age
- Inflammatory myopathy – not genetic – with severe and systemic muscle wasting, loss of ambulation, dysphagia and premature mortality
- No FDA-approved disease-modifying therapeutics, standard of care involves physical and occupational therapy until death

### HF with preserved or reduced ejection fraction, HFpEF & HFrEF

- Developing indication
- Large chronic disease indication with diverse disease etiologies (HFrEF = aortic stenosis, hypertension, myocardial infarction, etc; HFpEF = aging, hypertension, obesity, etc.)
- Pharmacotherapies for HFrEF include diuretics, RAS inhibitors,  $\beta$ -blockers, etc., none for HFpEF

### DMD – developing indication

- Rare X-linked recessive genetic disease resulting from mutations in *dmd* gene, Becker muscular dystrophy occurs with partial activity
- Typically presents in boys ~5 years old, weakness and muscle dysfunction progresses, resulting in loss of all voluntary muscle use and a median life expectancy of ~25 years
- Mortality results from cardiac and respiratory impairment
- Gene and genetic medicines only recently approved with poor efficacy

## Pipeline:

### AVGN7 (AAVMYO2:CK8e-hSmad7)

- Lead compound, significantly enhances muscle mass and function in a variety of muscle, neuromuscular and inflammatory disease animal models, including DMD, and in cultured muscle cells derived from IBM subjects
- Restores skeletal muscle contractility and  $Ca^{2+}$ -handling dysfunction in 3D engineered muscle tissues derived from IBM subjects
- Prevents cardiac cachexia and enhances cardiac function
- Superior safety due to myotropic AAVMYO2 capsid, the muscle-specific CK8e promoter and a codon-optimized Smad7 with only intracellular targets; avoids serious SAEs of other gene therapeutics and ActRII-attenuators

### AVGN7 + AVGND (AAVMYO2:CK8e- $\mu$ DysH3) combinatorial

- AVGND prevents muscle degeneration by expressing a "micro-dystrophin", AVGN7 enhances muscle mass and function
- Combined titer load well below "toxic ceiling" of other AAV therapeutics
- Address 1<sup>st</sup> mover failures of genetic and gene therapy approaches that only address muscle degeneration, **expands treatment options to older patients with more advanced disease progression**