

Aavogen

Precision Muscle Gene Therapeutics Across High-Value Rare & Broad Indications

AVGN7 (rAAVMYO2:CK8e-hSmad7)

Multi-Indication Platform Asset

- **Lead Rare Indication:** sporadic inclusion body myositis (IBM)
- **Cardiovascular Expansion:** heart failure with reduced ejection fraction (HFrEF)

AVGND (rAAVMYO2:CK8e-μDysH3)

Duchenne muscular dystrophy (DMD)

- **Targeted Gene Replacement:** restores functional dystrophin, prevents degeneration
- **Combinatorial Synergism:** AVGND + AVGN7 reverses historical necrosis & fibrosis, rebuilds strength

Strategic Investment Highlights

- **Robust IP Moat:** 6 granted patents | 1 pending
- **Significant Market Potential (5 years):** \$11.4B aggregate peak | IBM (\$4.6B) | DMD (\$3.6B) | HFrEF (\$3.2B)

AVGN7 Platform

Scalable Pipeline Across Rare & Cardiovascular Diseases

Inclusion Body Myositis (IBM) | lead orphan indication

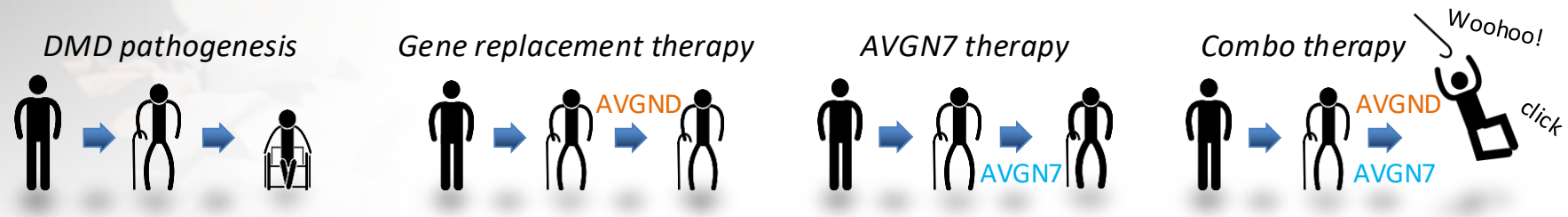
- **Mechanism:** blocks catabolic signaling
- **Clinical value:** halts wasting, restores motor function

Heart Failure with Reduced Ejection Fraction (HFrEF)

- **Mechanism:** reverses ventricular remodeling
- **Clinical value:** restores cardiac function

Duchenne Muscular Dystrophy (DMD)

- **Mechanism:** AVGN7+AVGN7 combination pipeline
- **Clinical value:** overcomes dystrophin-targeting limits



Engineered for superior safety & efficacy



Liver de-targeted AAVMYO2 capsid avoids immune detection & pathologies



Muscle-specific CK8e promoter prevents expression in non-muscle tissues and is clinically validated



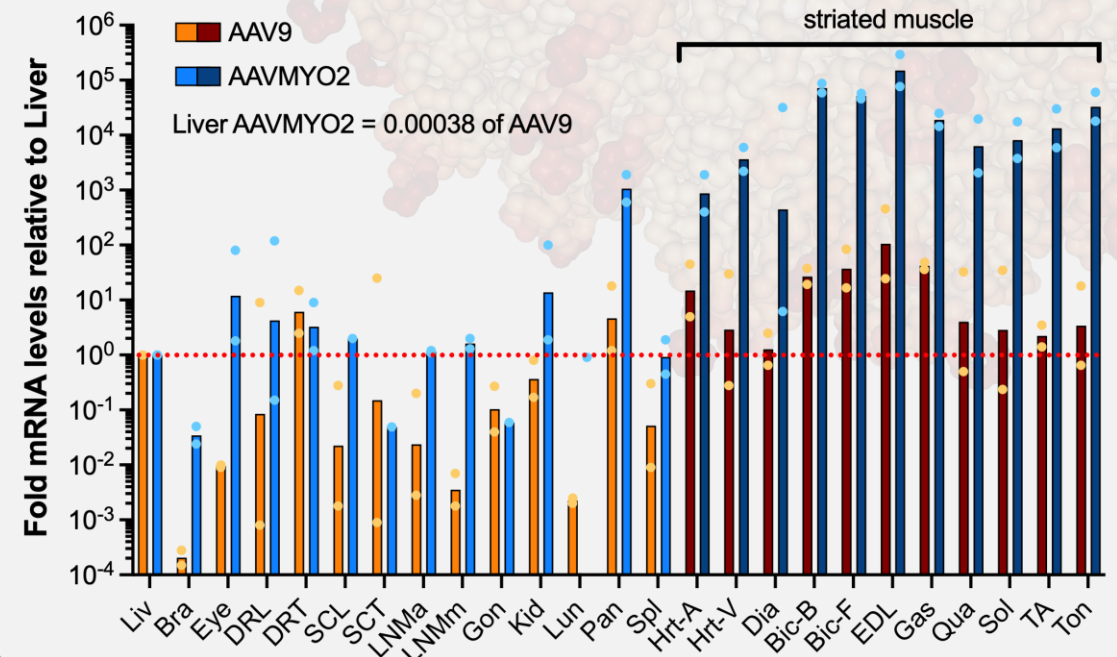
Combinatorial therapy is enabled by low dose requirements



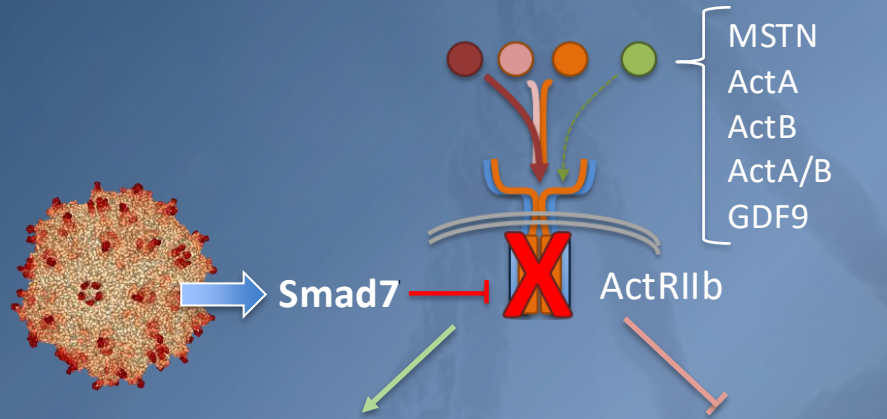
Lower manufacturing burden reduces cost of goods, provides flexible pricing

AAVMYO2 biodistribution in cynomolgus macaque

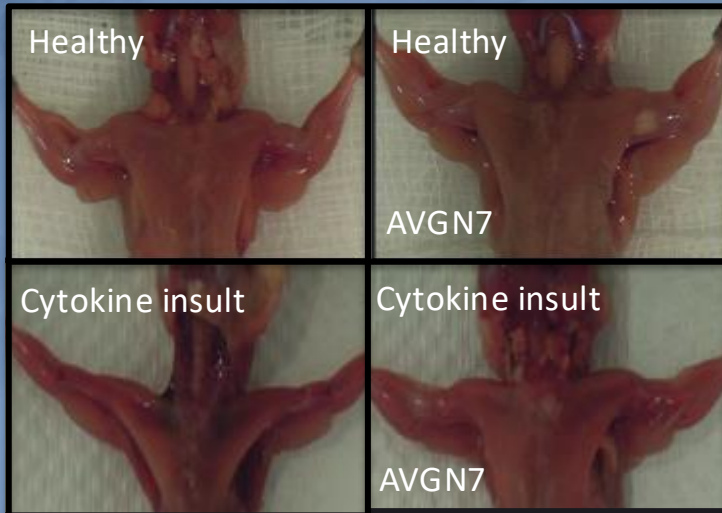
CMV promoter, YFP transgene



AVGN7 inhibits the inhibitors

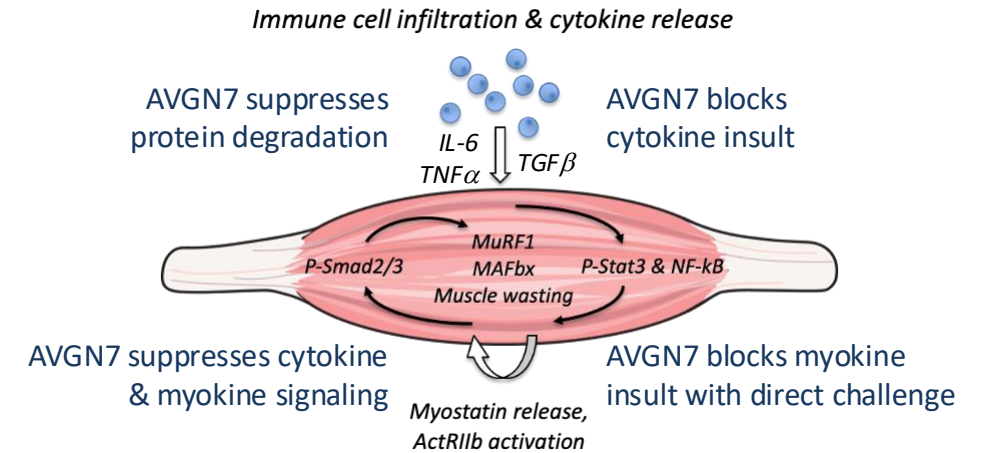


- Protein degradation
- Muscle wasting
- Fibrosis
- **Protein synthesis**
- **Muscle growth**

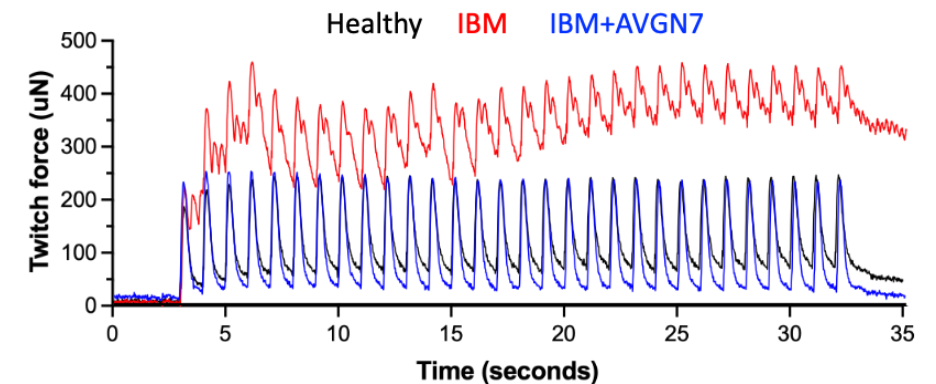


AVGN7 restores IBM muscle

Prevents cytokine-induced muscle atrophy



Restores IBM muscle contractility



AVGN7 in HFrEF models

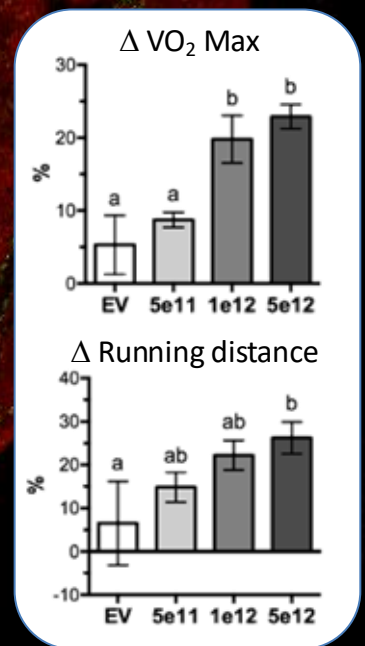
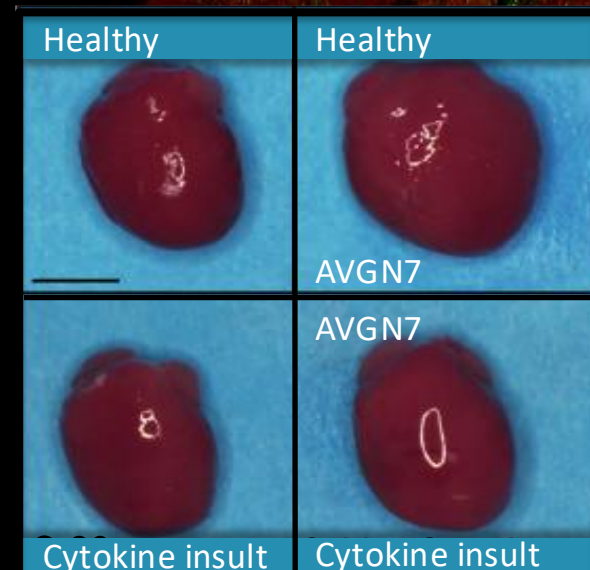
HFrEF is induced by ActRII signaling

- ActRII ligands (MSTN, GDF11, ActA, etc.) stimulate cardiac atrophy
- Circulating ligands are elevated in HFrEF

Attenuating ActRII in the heart

- Enhances excitation-contraction coupling
- Enhances cardiac function in stress tests
- Enhances cellular contractility
- Increases progenitor cell pool
- Increases calcium stores
- **Restores cardiac function in different animal models of HFrEF and HFpEF**

AVGN7 induces *physiological* cardiac hypertrophy – the “good kind”



Synergistic Combination Strategy for DMD

Micro-dystrophin gene therapy

Rodgers et al. *Mol Ther Methods Clin Dev* 2019

Dystrophin 427 kDa



AVGND 137 kDa



$\delta H2-R23+H3/\delta CT$ (a.k.a. $\mu DysH3$)

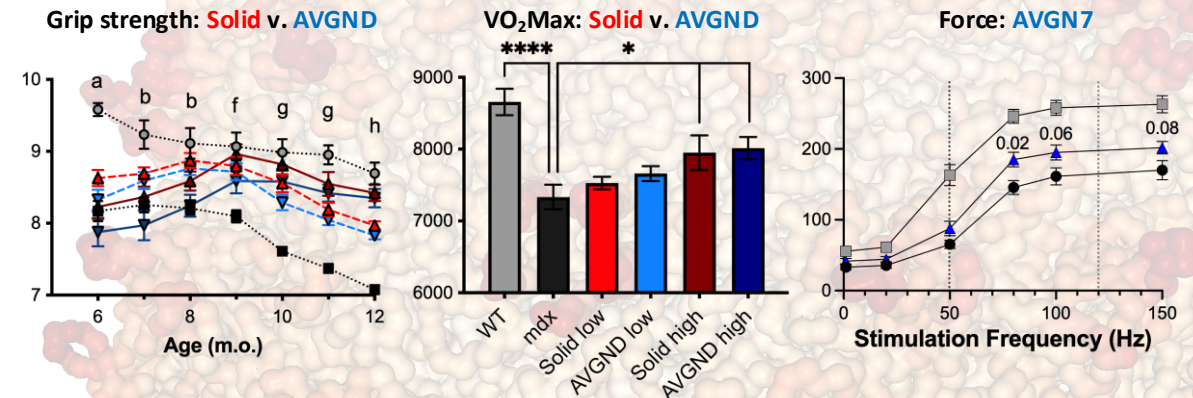
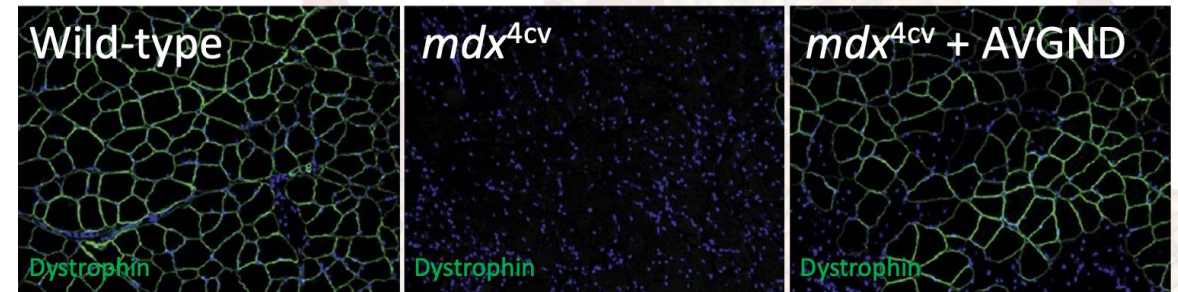
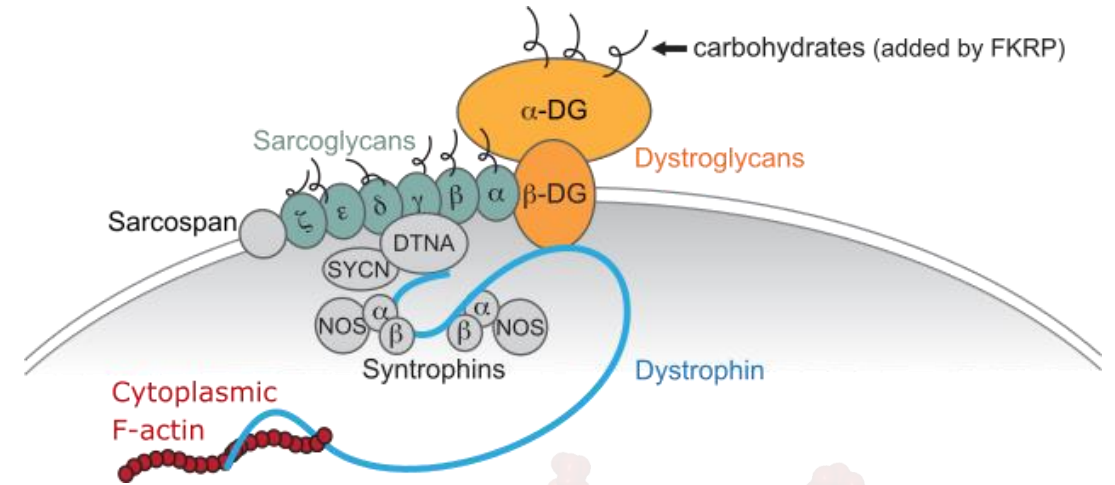
- ## AVGND mechanism of action

- Restores muscle structure
- Prevents degeneration & loss of function

AVGND & Solid's μ Dys GTx are equally effective

AVGN7 builds muscle mass & strength in dystrophic mice

AVGND + AVGN7 restores healthy muscle



IBM Competition

Abcuro Ulviprubarb & Pfizer Sirolimus

- Anti-inflammatory
- Prevents degeneration **ONLY**
- Sirolimus failed Phase III, discontinued
- Ulviprubarb failed in Phase III

AVGN7

- Muscle-specific
- Durable, prevents degeneration
- Restores muscle mass
- Restores muscle function

5 yr IBM US Market

- Rare disease indication
- 10% penetration = 2.3K
- \$2M/patient

\$4.6B

HFrEF Competition

Sardocor **SRD-001** & AskBio **AB1002**

- Increases **SERCA2A** levels
- Increases **PLN** activation
- Off-target toxicity risks (AAV1 & 8)

AVGN7

- Increases SERCA2a levels
- Increases PLN activation
- Muscle-specific
- Durable, prevents degeneration
- Restores cardiac mass & function

5 yr HFrEF US Market

- Large-market indication
- IC delivery for 2% = 63K
- \$50K/patient

\$3.2B

DMD Competition

Sarepta Elevidys, REGENXBIO RGX-202 & Solid SGT-003 μ Dys GTXs

- Durable, prevents degeneration **ONLY**
- Serious off-target toxicity risks

Sarepta exon skipping drugs

- Questionable efficacy
- Limited market penetration

AVGND + AVGN7

- Muscle-specific
- Durable, prevents degeneration
- Restores muscle mass
- Restores muscle function

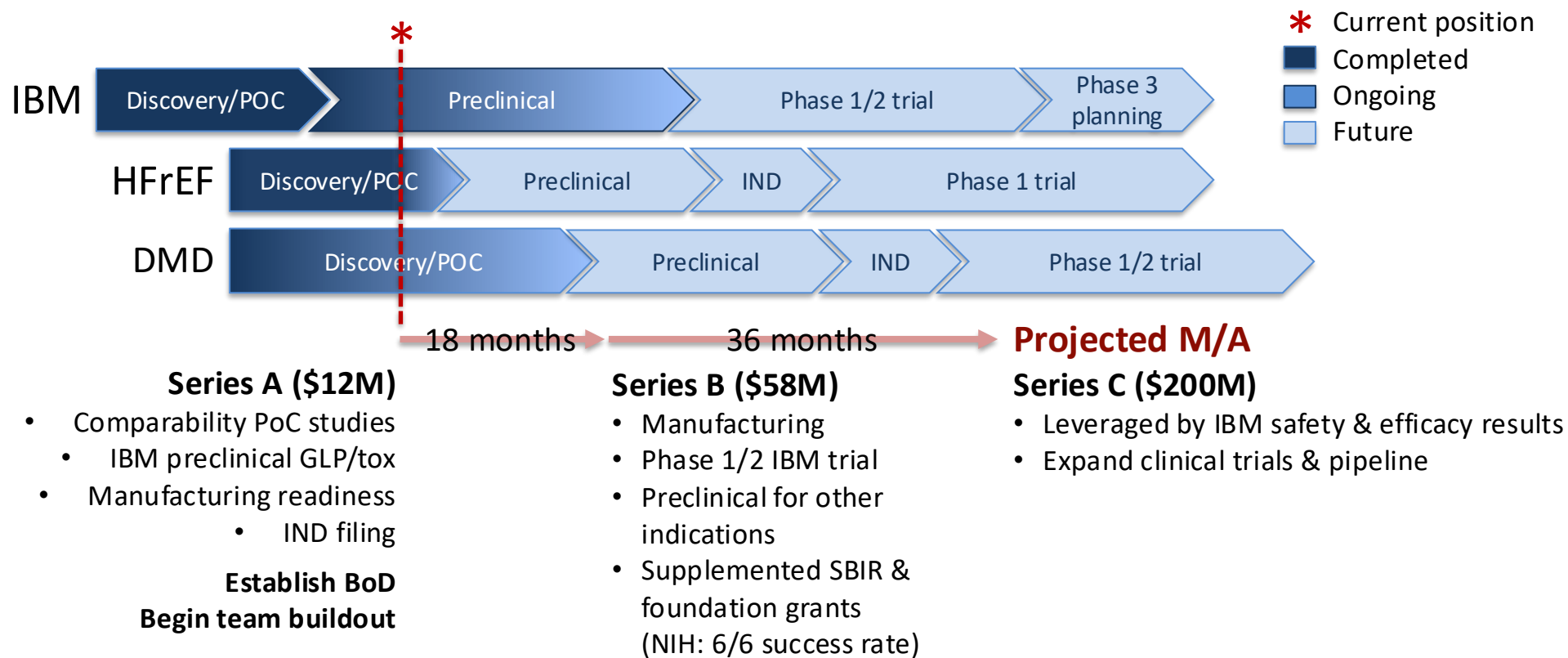
5 yr DMD US Market

- Rare disease indication
- 10% = 1.6K
- \$3M/patient

\$4.8B

Business Model

Begin buildout with Series A, expand R&D focus capitalizing on core technologies & licensing



Development path/capital strategy

Current raise, \$12M Series A
\$3M anchored by Eurofarma Ventures

Year 0: IND-enabling/Venture & Non-diluting

- Series A: GLP/tox, CMC readiness & IND-filing for IBM
- NIH SBIR/Catalyze grants for DMD/HFrEF P-o-C

Year 1.5: Clinical/Venture & Non-diluting

- Series B: Phase I/II clinical trial for IBM
- NIH SBIR/Catalyze grants for DMD/HFrEF preclinical

Year 4.5: Growth & Commercialization/M&A or IPO

- Phase III & BLA for IBM
- Initiate DMD & HFrEF clinical trials

Building value without dilution

\$5.6M raised to date, 6-for-6 NIH SBIR grant success



National Institute on Aging
National Institute of Arthritis, Musculoskeletal and Skin Diseases
National Institute of Diabetes and Digestive and Kidney Diseases
National Heart, Lung and Blood Institute
National Cancer Institute

Pending grants in review

NIA R44 Phase IIB: \$3,246,993

- *Preclinical Development of AVGN7 for Inclusion Body Myositis*
- Supported by The Myositis Association

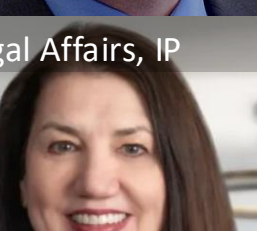
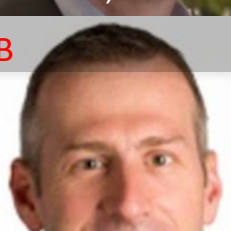
NIAMS R44 Phase II: \$2,299,433

- *Preclinical Development of a Combinatorial Gene Therapy for Duchenne Muscular Dystrophy*
- Supported by The Muscular Dystrophy Association & CureDuchenne

NHLBI R61/R33 Catalyze Phase I/II: \$1,301,693

- *AVGN7 to Prevent Heart Failure with Reduced Ejection Fraction*

Our Team

CEO  Dan Rodgers, PhD	CSO  Alex McPherron, PhD	COO/ SAB Chair  Bill Mann, PhD	CFO  Gary Carswell, CPA	Reg. Affairs Dir.  Chris Novashinski RareMoon
CMC Dir.  Joe Rininger, PhD Latham BioPharm	Pre-clin. Dir.  Sarah Herring, PhD Latham BioPharm	BD Dir.  Jade Brown, MBA	Clin. Dir./ SAB  Tom Lloyd, MD/PhD Baylor Coll. of Med.	Legal Affairs, BD  Francis Mosley, JD Cooley LLP
Legal Affairs, IP  Lisa Hillman, PhD/JD Lathrop GPM	SAB  Jeff Chamberlain, PhD Univ. of Washington	SAB  Paul Gregorevic, PhD Univ. of Melbourne	SAB  Brian O'Rourke, PhD Johns Hopkins Univ.	SAB  Dirk Grimm, PhD Univ. of Heidelberg

Our Partners

Animal research CROs

- Myologica
- Noble Life Science

CMC

- Andelyn Biosciences
- CHOP Vector Core

Assay Development CROs

- Smithers
- InfinixBio

University Collaborators

- Johns Hopkins
- Baylor
- Columbia