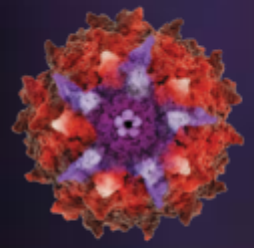

AskBio

Transforming medicine.
Changing lives.

Sheila Mikhail, JD, MBA
Chief Executive Officer | Co-Founder

Jefferies Virtual Healthcare Conference
June 2, 2020

Fully integrated and validated gene therapy company

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Founded in 2001 based on 40+ years of research by pioneers in gene therapy, headquartered in Research Triangle Park, NC

Comprehensive novel technology platform with 500+ patents from discovery through delivery

Three scaled in-house manufacturing facilities with internal/external contract capacity and highest yielding cell line

Robust monogenic and pathway disease pipeline with therapeutic programs in multiple clinical stages

Ongoing milestone and royalty payments with significant near-term cash flow from blue-chip pharma

Proven value and validated technology

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Commercial value

Technology inside every approved gene therapy

Glybera
uniQure

LUXTURNA™
Spark THERAPEUTICS
Roche

zolgensma®
avexis
NOVARTIS

Strategic value

Platform validated by large pharma acquisitions

Takeda
Chatham therapeutics

Pfizer
Bamboo therapeutics

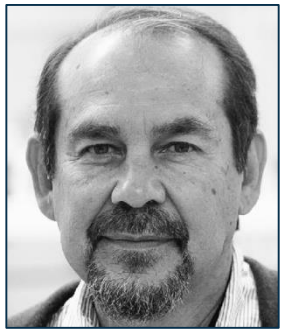
Investor value

Backed by international blue-chip investors

TPG

VIDA ventures

A powerful organization and respected industry leader



R. Jude Samulski, PhD
President & Chief Scientific Officer
Founder & Board Member

Sheila Mikhail JD, MBA
Chief Executive Officer
Founder & Board Member

- 20+ years of partnership launched AskBio, Bamboo Therapeutics and Chatham Therapeutics
- Jude Samulski, PhD, is a gene therapy pioneer who holds 200+ patents
  - **First** to clone AAV for therapeutic purposes
  - **First** to deliver AAV via intrathecal administration
  - **First** to treat patients with DMD and Pompe disease
  - **First** to deliver AAV to the brain
- Developed Pro10™ cell line, the industry gold standard
- 10+ strategic transactions completed since 2001

Seasoned management team

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Sheila Mikhail, JD, MBA
Chief Executive Officer
Founder
Bamboo therapeutics
Chatham therapeutics

Tim Kelly, PhD
President, Manufacturing
KBI BIOPHARMA
Diosynth biotechnologies

Don Haut, PhD
Chief Business Officer
SHERLOCK BIOSCIENCES
The Medicines Company

Michael Roberts, PhD
Chief Technology Officer, Europe
Synpromics the leader in gene control An AskBio Company
MSCA COFUND 2019 MSCA Individual Fellowships

R. Jude Samulski, PhD
President | Chief Scientific Officer
Founder
Bamboo therapeutics
Pfizer
University of North Carolina at Chapel Hill

Joshua Grieger, PhD
Chief Technology Officer
Bamboo therapeutics
Pfizer

David Venables, PhD
President, Europe
Synpromics the leader in gene control An AskBio Company
nightstar THERAPEUTICS

Krys Bankiewicz, MD, PhD
Scientific Founder, BNB
Brain Neurotherapy Bio
Voyager THERAPEUTICS

Tim Trost
Chief Financial Officer
CHIMERIX
Argos THERAPEUTICS

Casey Childers, DO, PhD
Chief Medical Officer
AUDENTES THERAPEUTICS
UNIVERSITY of WASHINGTON

Philippe Moullier, MD, PhD
Chief Scientific Officer, Europe
UNIVERSITÉ DE NANTES
GENETHON

Nathalie Cartier, MD
Scientific Founder, BrainVectis
BRAINVECTIS
Inserm

Accomplished board of directors

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Sheila Mikhail, JD, MBA
Founder | Board Member
AskBio

R. Jude Samulski, PhD
Founder | Board Member
AskBio

Fred Cohen, MD, DPhil
Board Member
VIDA ventures
TPG

Javier Garcia, MBA
Board Member
Columbus Venture Partners
Lilly

Bill Hawkins, MBA
Board Member
Medtronic

Jon Salveson, MMgt
Board Member
PIPER | SANDLER

Jon Schilling, MD
Board Member
TPG McKinsey &Company Abbott

Katherine Wood, MBA
Board Member
TPG Goldman Sachs

Introduction to gene therapy

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Durable expression of the **intact gene** and stable production of a **functional protein** cures disease

1 *Therapeutic Development*

A functional version of a **gene** and a **promoter** are inserted into a **viral vector**

2 *Therapeutic Delivery*

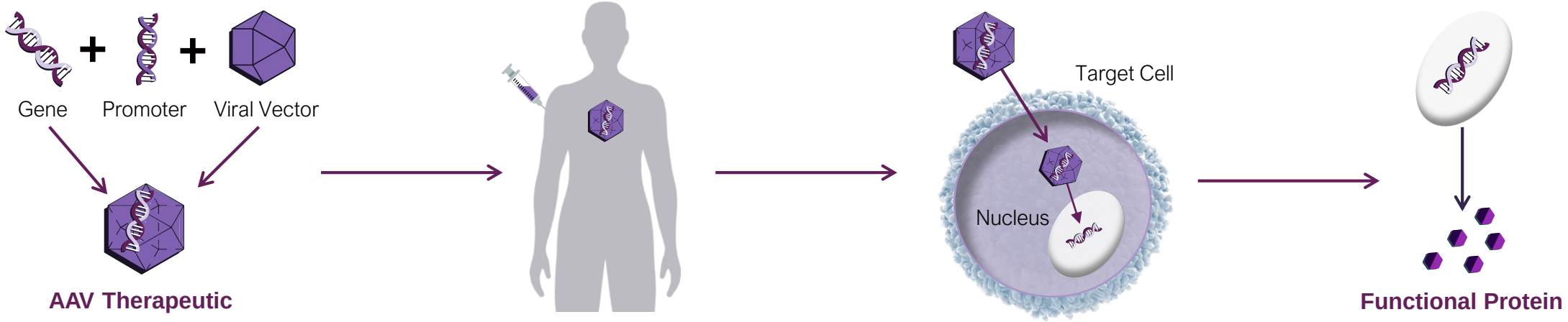
The **AAV therapeutic** is administered to the patient

3 *Gene Transfer*

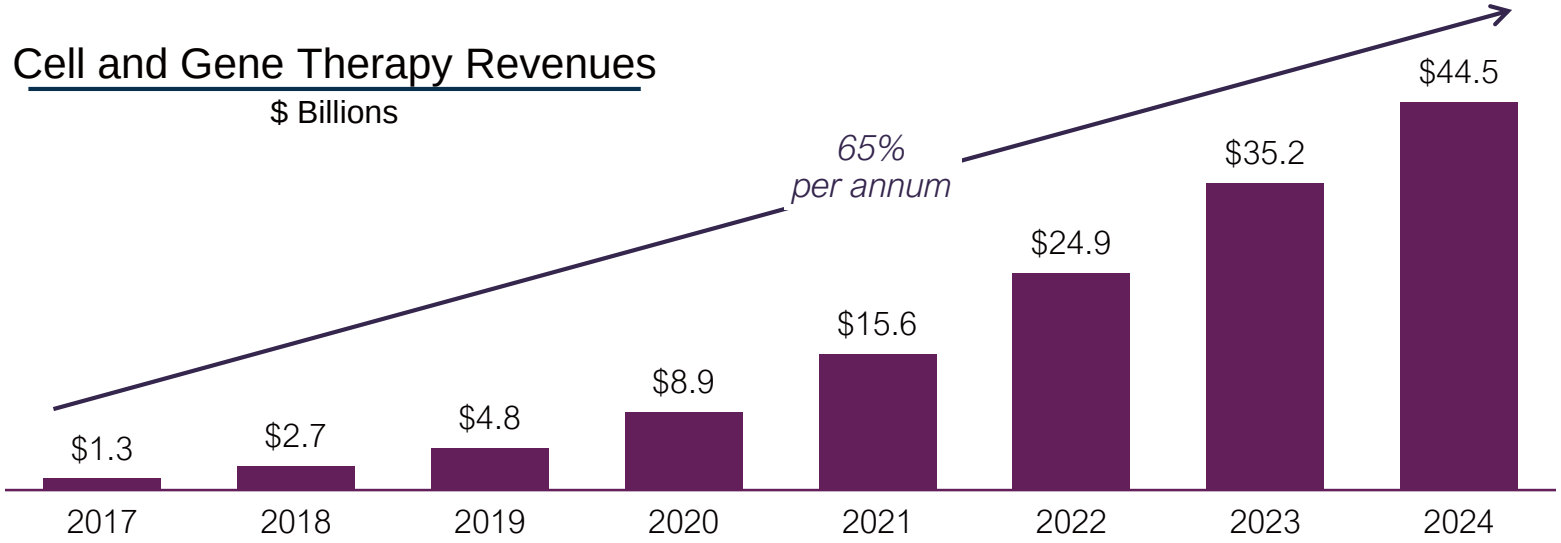
The **AAV therapeutic** infects the **target cells** and transfers the intact **gene** into the **nucleus**

4 *Therapeutic Effect*

Expression of the **intact gene** within the **nucleus** leads to **functional protein** production



A new class of medicine projected to grow 10x by 2024



Clinical Trials Approved to Begin Enrollment	135 2014	352 2019
Approved Drugs Cumulative	1 2014	5 2019
Drug Approvals Per Annum	2 2019	20 2025

> "The promise of gene therapy is very much becoming a reality. These recent product approvals represent just the tip of the iceberg."
>
> Scott Gottlieb, Former FDA Commissioner
> May 2018

Note: Forecasts are inherently uncertain and subject to change. Actual results may vary.
Source: PwC, EvaluatePharma, Journal of Gene Medicine, company filings, websites, press releases

Valuations reflect scarcity value and disruptive potential

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AskBio's value is elevated by revenue generation across all categories

Therapeutics		Manufacturing		Cash Flows & Royalties	
avexis / NOVARTIS	**$8.7B** April 2018	caldevron / IEQT	**$3B+** July 2019	REGENXBIO	**$1.7B**
Spark THERAPEUTICS / Roche	**$4.8B** Feb. 2019	brammer bio / ThermoFisher SCIENTIFIC	**$1.7B** March 2019		
AUDENTES THERAPEUTICS / astellas	**$3.0B** Dec. 2019	PARAGON BIOSERVICES / Catalent	**$1.2B** April 2019		
SAREPTA THERAPEUTICS	**$9.6B**				
bluebirdbio	**$5.0B**				
uniQure	**$3.1B**				

Market caps as of January 10, 2020
Note: The comparables presented above may not represent all relevant comparables. There can be no assurance that any similar transactions will ultimately be available or consummated
Source: Capital IQ, Filings, Press Releases, TPG Estimates

The only fully integrated gene therapy company

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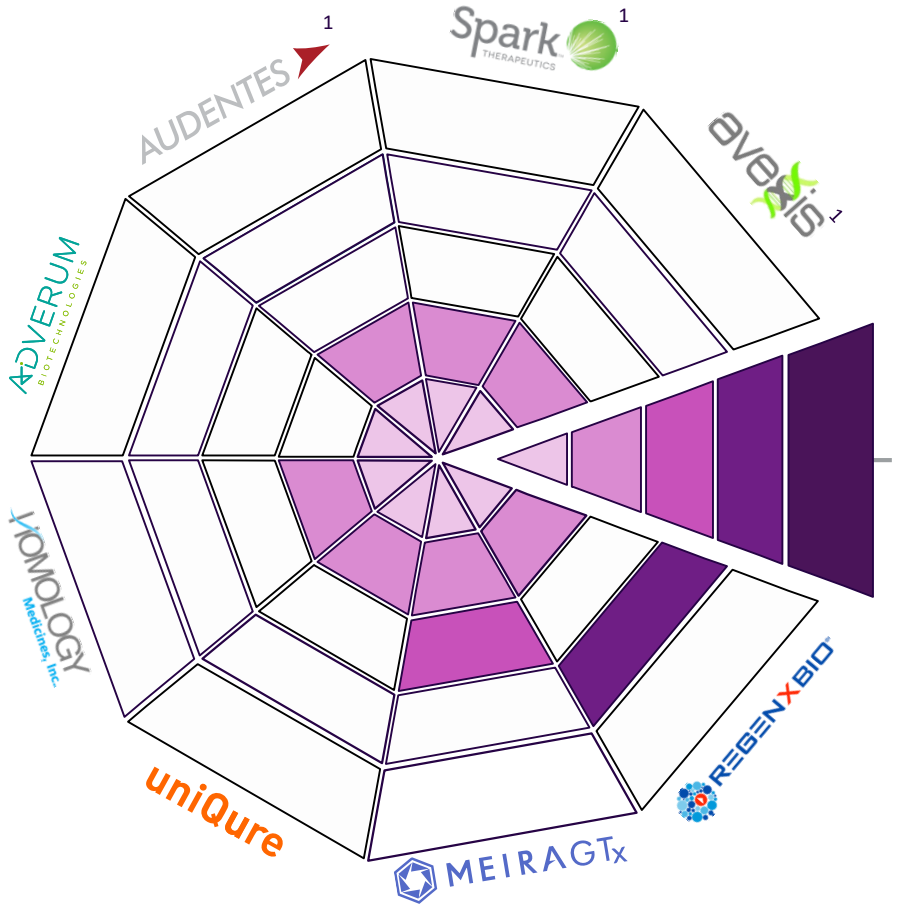

1. **Robust therapeutic pipeline**
   Programs targeting monogenic and pathway diseases
2. **In-house manufacturing capabilities**
   3 manufacturing facilities totaling 350,000 square feet with GLP and GMP capabilities and scaled cell line
3. **Contract manufacturing capabilities**
   Extra gene therapy manufacturing capacity available for contract to meet growing industry demand
4. **Cash flows from commercial royalties**
   Near-term milestones and royalties from past transactions with Takeda, Pfizer and Novartis
5. **Comprehensive scientific platform**
   Novel technologies utilized from discovery to delivery based on over 500+ patents

1. Audentes, Spark, and AveXis acquired by Astellas, Roche and Novartis, respectively
Company Inclusion Criteria: Primary focus on AAV gene therapy, No approved products (public companies only), Market cap > $700M as of January 10, 2020 or M&A value > $1B

Platform built on novel IP portfolio of 500+ patents

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A deep portfolio of novel technologies across the gene therapy value chain

Therapeutics

- Extensive capsid library
- Engineered chimeric & haploid capsids
- Synthetic promoters
- Self-complementary vectors[1]
- Targeted transduction vectors

Manufacturing

- Pro10™ cell line
- Synthetic promoters
- Plasmid alternative (dbDNA)
- Captive CDMO
- Thin film technology

Delivery

- Nanotech cargo delivery
- Regulated gene expression
- Repeat dosing
- Gene editing

1. Licensed to AveXis (Novartis) for use in Zolgensma

Recent strategic investments in platform expansion

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Doggybone™ DNA

TOUCHLIGHT

- 50% JV - State-of-the-art manufacturing San Sebastian, Spain
- Safest, most efficient, scalable DNA

Synthetic Promoters

Synpromics
the leader in gene control
An AskBio Company

- Acquired market leader promoters and gene expression
- 10+ blue-chip therapy customers
- Built-in royalties/milestones

Re-dosing / Neutralizing Antibodies

Selecta Biosciences

Licensing and collaboration agreement to enable repeat administration of therapies

RoverMed BioSciences

Acquisition of nanotech cargo delivery of therapeutic portfolio

SQZBIOTECH

- Collaboration agreement to enable repeat administration with SQZ red blood cell tolerizing antigen carriers
- AskBio capsid design and manufacturing with SQZ red blood cell tolerizing antigen carrier technology

Broader Indications

editas MEDICINE

- Licensing and collaboration agreement combines AskBio AAV and promoter technology with Editas CRISPR technology
- Gene editing approach for CNS indications

BRAINVECTIS

- Acquisition to broaden CNS clinical pipeline with Huntington's disease indication
- Expertise with CYP46A1, the key enzyme of brain cholesterol metabolism

Best-in-class AAV therapeutics and novel technology

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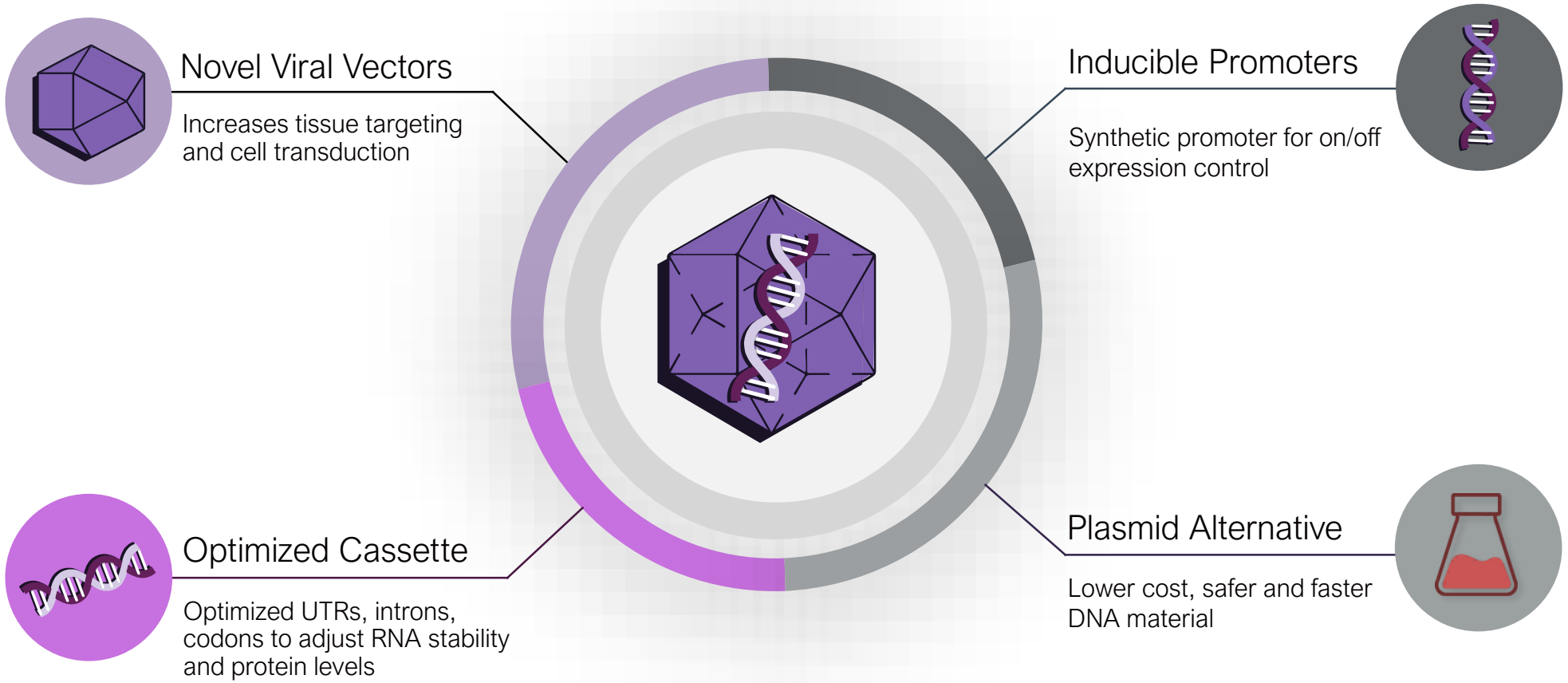

Increased **potency and efficacy** | **Better safety** profile | **Optimized tissue targeting** and payload

350,000 cumulative square feet of scaled-up manufacturing

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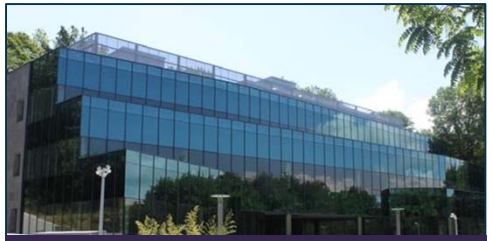
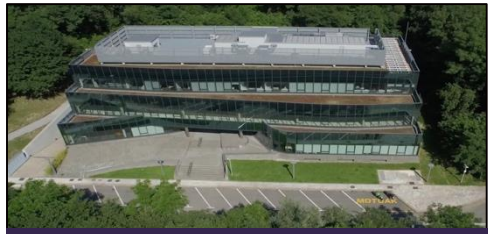


Viralgen Clinical
San Sebastian, Spain

Viralgen Commercial
San Sebastian, Spain



Touchlight Joint Venture
San Sebastian, Spain/Hampton, UK

- Serum free
- Scaled up to 2,000 liters
- Yields of 10^17

Pro10™ Cell Line

- Shorter cycle time
- Fewer bacterial contaminants
- Lower cost

Plasmid Alternative

- Superior manufacturability
- Reduces burden and increases throughput

Novel Viral Vectors

Novel manufacturing technologies at every stage

- ✓ Improved yields
- ✓ Higher throughput
- ✓ In-house control
- ✓ Unmatched batch purity
- ✓ Reliable consistency

- Increases productivity
- Stable packaged cell line

Inducible Promoters

Thin Film Technology

- Extends shelf-life
- Eliminates need for -80°C cold storage

Robust monogenic and pathway disease pipeline

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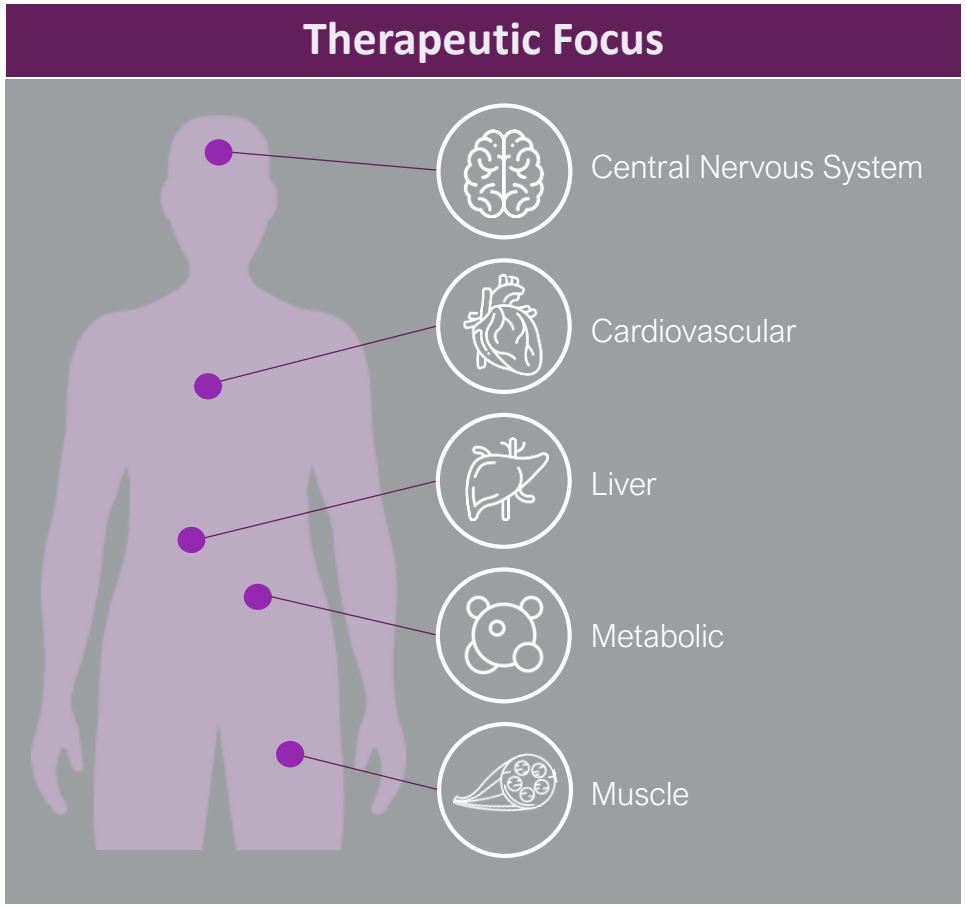
Neuromuscular	Discovery	Preclinical	Phase I/II
Pompe disease			
Limb-girdle muscular dystrophy 2i			
CNS	**Discovery**	**Preclinical**	**Phase I/II**
Huntington's disease			
Parkinson's disease			
Angelman syndrome			
Cardiovascular	**Discovery**	**Preclinical**	**Phase I/II**
Congestive heart failure			
Metabolic	**Discovery**	**Preclinical**	**Phase I/II**
Methylmalonic acidemia (MMA)			
Divestitures	**Discovery**	**Preclinical**	**Phase I/II**
DMD (Bamboo therapeutics)	Acquired by Pfizer 2016		
Hemophilia A/B (Chatham therapeutics)	Acquired by Takeda 2014		



Pompe program success

Pompe disease is an autosomal recessive metabolic disorder caused by an accumulation of glycogen in the lysosome due to deficiency of **acid α-glucosidase (GAA).**

Six patients successfully treated in two cohorts and clinically stable

Cohort 1
Low dose

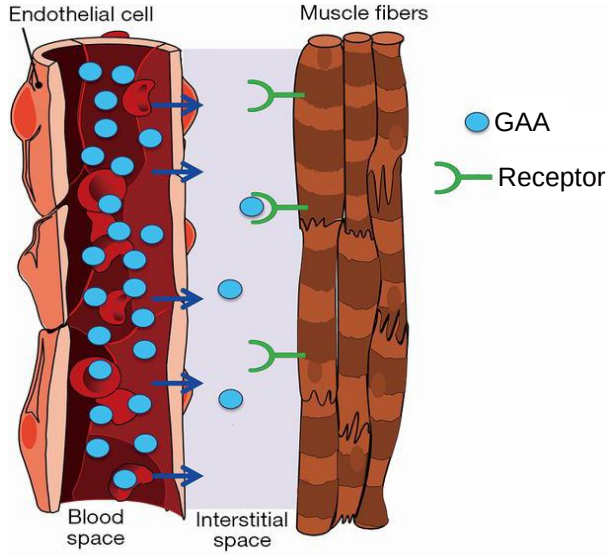
Cohort 2
High dose

1

ERT no longer required
Patients stopped bi-weekly infusions at an annual cost that can exceed $500,000

2

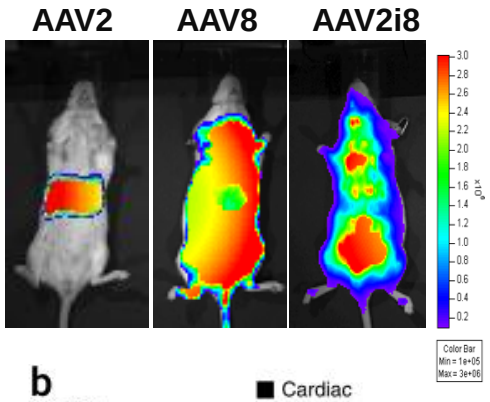
GAA in blood and muscle
Muscle function maintained due to healthy production of GAA



CHF program success

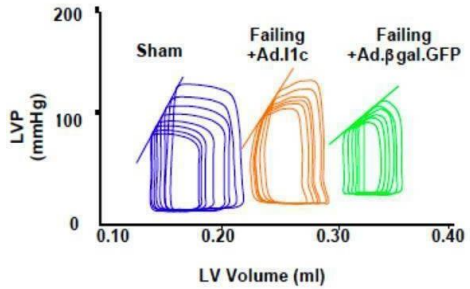
Novel single-dose gene transfer intra-coronary infusion enhances cardiac contractility, restores heart function and reverses pump failure.

De-targets the liver and allows effective uptake in the heart



1

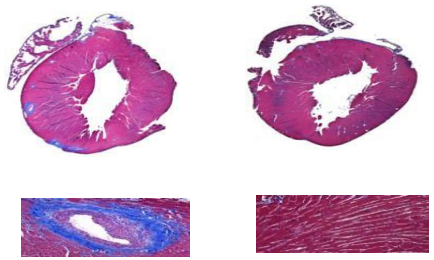
Improves contractility & relaxation
Increased calcium cycling and healthy heart muscle function



I1c gene increases phosphorylation of phospholamban

2

Reduces enlarged heart muscle (Hypertrophy) and reduces scar tissue (Fibrosis)

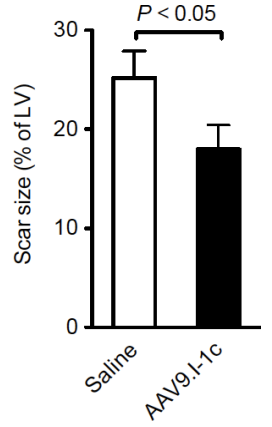


Wild Type Banded

I-1c gene Banded

3

Increases cell survival
Prevents cell death referred to as apoptosis



Milestone and royalty payments from large pharma

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Milestone Payments

Chatham therapeutics

Acquired by:

Takeda

- $70M upfront and undisclosed milestones
- Resulted from collaboration in Hemophilia A/B

Bamboo therapeutics

Acquired by:

Pfizer

- $150M upfront and potential future payments up to $495M
- DMD/FA programs and gene therapy manufacturing facility

Royalties

AskBio

Technology licensed to:

avexis

NOVARTIS

- Non-exclusive right to sell self-complementary (SC) technology for Zolgensma®

Market-leader across the gene therapy landscape

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- Highly attractive market with broad range of transformative clinical applications
- Robust therapeutic pipeline
- Large-scale GMP manufacturing, supporting internal and contract needs
- Comprehensive IP portfolio and only end-to-end technology platform
- Significant milestone and royalty streams and operational cash flow
- Scalable platform for continued growth and innovation