SAREPTA THERAPEUTICS

# Accelerating...

**DOUG INGRAM**
President and CEO

Sarepta Therapeutics, Inc. (NASDAQ:SRPT)
43rd Annual J.P. Morgan Healthcare Conference
San Francisco, California
**JANUARY 13, 2025**

**BENJAMIN**
Living with Duchenne muscular dystrophy

# This is why we fight

**Untreated**


**Treated**


This patient's results are unique to him and are not reflective of the broader Duchenne patient population.

# Forward-looking statements

This presentation contains "forward-looking statements." Any statements that are not statements of historical fact may be deemed to be forward-looking statements. Words such as “believe,” “anticipate,” “plan,” “expect,” “will,” “may,” “intend,” “prepare,” “look,” “potential,” “possible” and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to future operations, financial performance and projections, including our expected financial results and financial guidance for 2025 and beyond; potential solutions and market opportunities with our RNA technologies, gene therapy, gene editing, and the technologies with our strategic partners, including the siRNA platform; the potential benefits of our technologies and scientific approaches; the potential of gene therapy’s applicability across disease; the potential expansion opportunities for ELEVIDYS; the potential benefits of our collaborations with strategic partners, including the Arrowhead deal and its programs; and expected milestones and plans, including multiple data readouts for ongoing ELEVIDYS studies in 2025, announcing EMERGENE expression data in 2025, starting phase I for SRP-9005 during the first half of 2025, Arrowhead program data readouts, including ARO-DUX4 and ARO-DM1, in the second half of 2025, IND filings (including ARO-HTT and SRP-9010) in the second half of 2025, a potential BLA filing for SRP-9003 in the second half of 2025, and our other 2025 priorities, including a R&D day in 2025.

These forward-looking statements involve risks and uncertainties, many of which are beyond our control and are based on our current beliefs, expectations and assumptions regarding our business. Actual results and financial condition could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties, and such risks and uncertainties could materially and adversely affect our business, results of operations and trading price. Potential known risk factors include, among others, the following: we may not be able to comply with all FDA requests, including post-approval commitments and requirements, in a timely manner or at all; the possible impact of regulations and regulatory decisions by the FDA and other regulatory agencies on our business; our data for our different programs, including gene therapy-based product candidates or programs with our strategic partners, may not be sufficient for obtaining regulatory approval; our product candidates, including those with strategic partners, may not result in viable treatments suitable for commercialization due to a variety of reasons, including the results of future research may not be consistent with past positive results or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; success in preclinical testing and early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful; the expected benefits and opportunities related to our agreements with our strategic partners may not be realized or may take longer to realize than expected due to a variety of reasons, including any inability of the parties to perform their commitments and obligations under the agreements, challenges and uncertainties inherent in product research and development and manufacturing limitations; if the actual number of patients living with the rare diseases we target is smaller than estimated, our revenue may be adversely affected; our dependence on our manufacturers to fulfill our needs for our clinical trials and commercial supply, including any failure on our part to accurately anticipate product demand and timely secure manufacturing capacity to meet product demand, may impair the availability of products to successfully support various programs, including research and development and the potential commercialization of gene therapy product candidates; we may not be able to successfully scale up manufacturing in sufficient quality and quantity or within sufficient timelines; we are subject to uncertainty related to reimbursement policies; we may not be able to execute on our business plans and goals, including meeting our expected or planned regulatory milestones and timelines, clinical development plans, and bringing our product candidates to market, for various reasons including possible limitations of our financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, regulatory, court or agency decisions, such as decisions by the United States Patent and Trademark Office; and those risks identified under the heading “Risk Factors” in Sarepta’s most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) and in its other SEC filings.

For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review Sarepta's filings with the SEC. We caution investors not to place considerable reliance on the forward-looking statements contained in this presentation. The forward-looking statements in this presentation are made as of the date of this presentation only and, other than as required under applicable law, Sarepta does not undertake any obligation to publicly update its forward-looking statements.

# Today's discussion

## Financial Results

Elevidys
delandistrogene moxeparvovec-rokl
suspension for intravenous infusion

EXONDYS 51
(eteplirsen) Injection

VYONDYS 53
(golodirsen) Injection

AMONDYS 45
(casimersen) Injection

## LGMD Franchise

### CLINICAL PROGRAMS

**SRP-9003**
LGMD2E/R4

**SRP-9004**
LGMD2D/R3

**SRP-6004**
LGMD2B/R2
*Dual Vector*

### PRECLINICAL PROGRAMS

**SRP-9005**
LGMD2C/R5

**SRP-9010**
LGMD2A/R1

**SRP-6006**
LGMD2B/R2
*Dual Vector*

## siRNA Programs

- Facioscapulohumerol muscular dystrophy (FSHD1)
- Myotonic Dystrophy Type 1 (DM1)
- Spinocerebellar Ataxia Type 2 (SCA2)
- Idiopathic Pulmonary Fibrosis (IPF)

+3 PRECLINICAL PROGRAMS

up to 6 DISCOVERY TARGETS

Robust research engine across **Gene Therapy/Gene Editing** and **RNA**

# Q4 2024 total net product revenue $638M*
# Full-year 2024 total $1.79B*

## ELEVIDYS significantly exceeds guidance

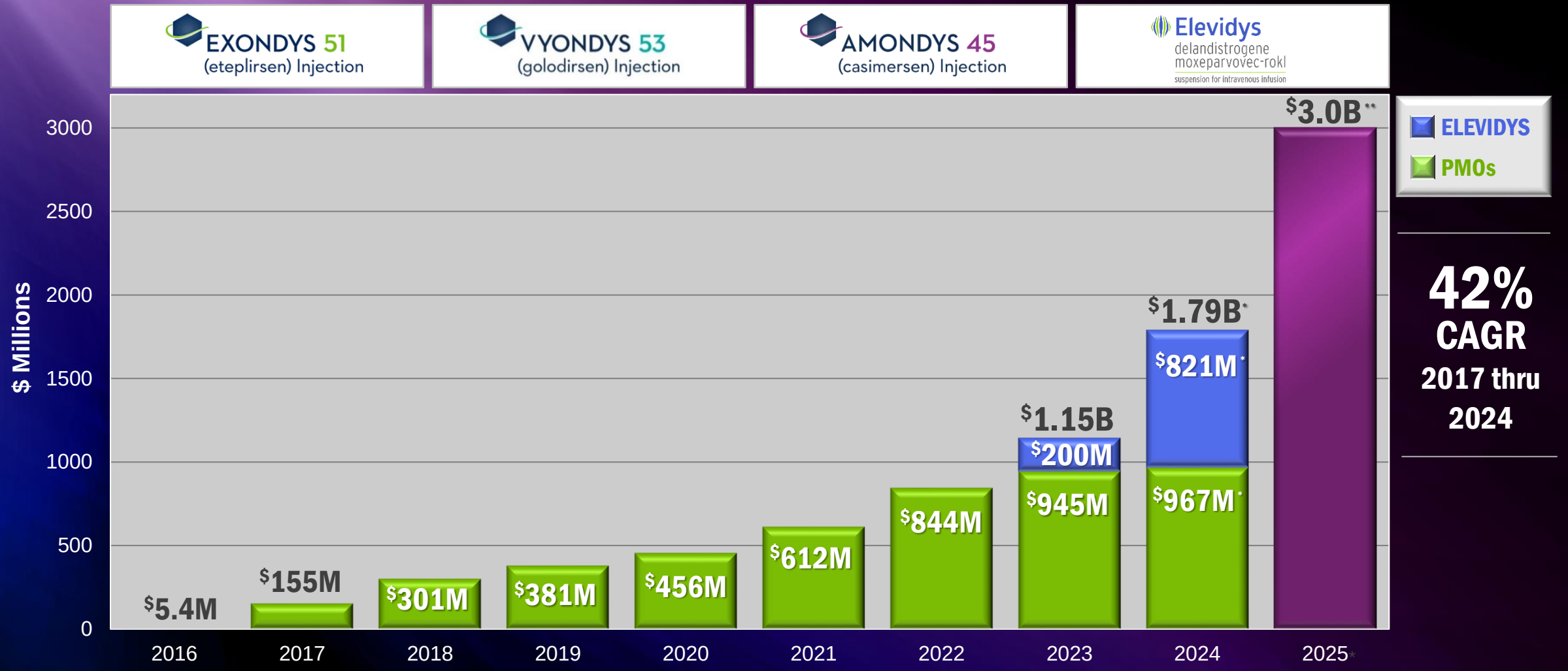
**Q4 net product revenue $384M***
**and full-year total $821M***


## Robust RNA-based PMO revenue

**Q4 net product revenue $254M***
**and full-year total $967M***

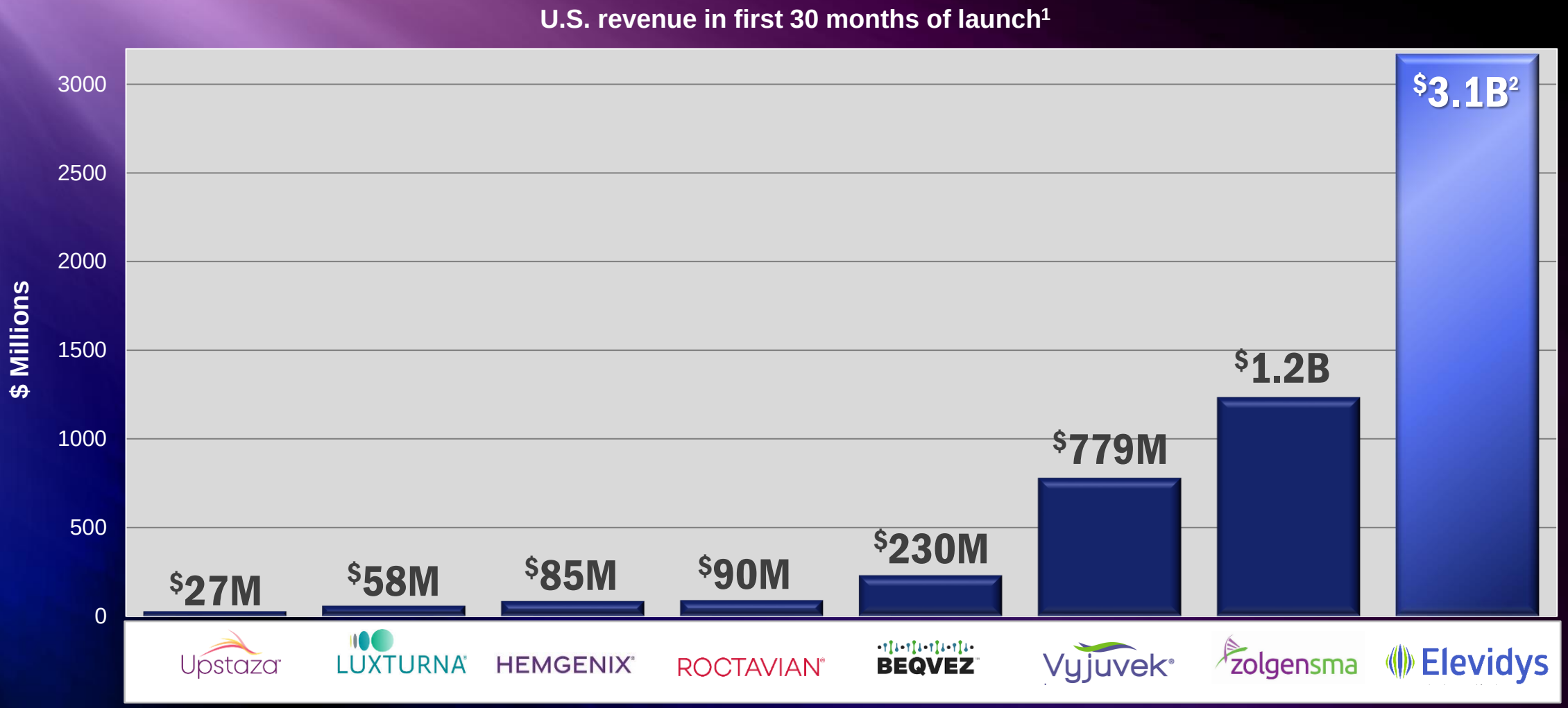
*Q4 QTD and YTD 2024 estimated net product revenue (unaudited)

# The midpoint of our 2025 guidance represents robust +67% growth over 2024




*2024 estimated net product revenue (unaudited)
**Midpoint of guidance $2.9 - 3.1B

# Sarepta set a new standard for gene therapy launches with ELEVIDYS

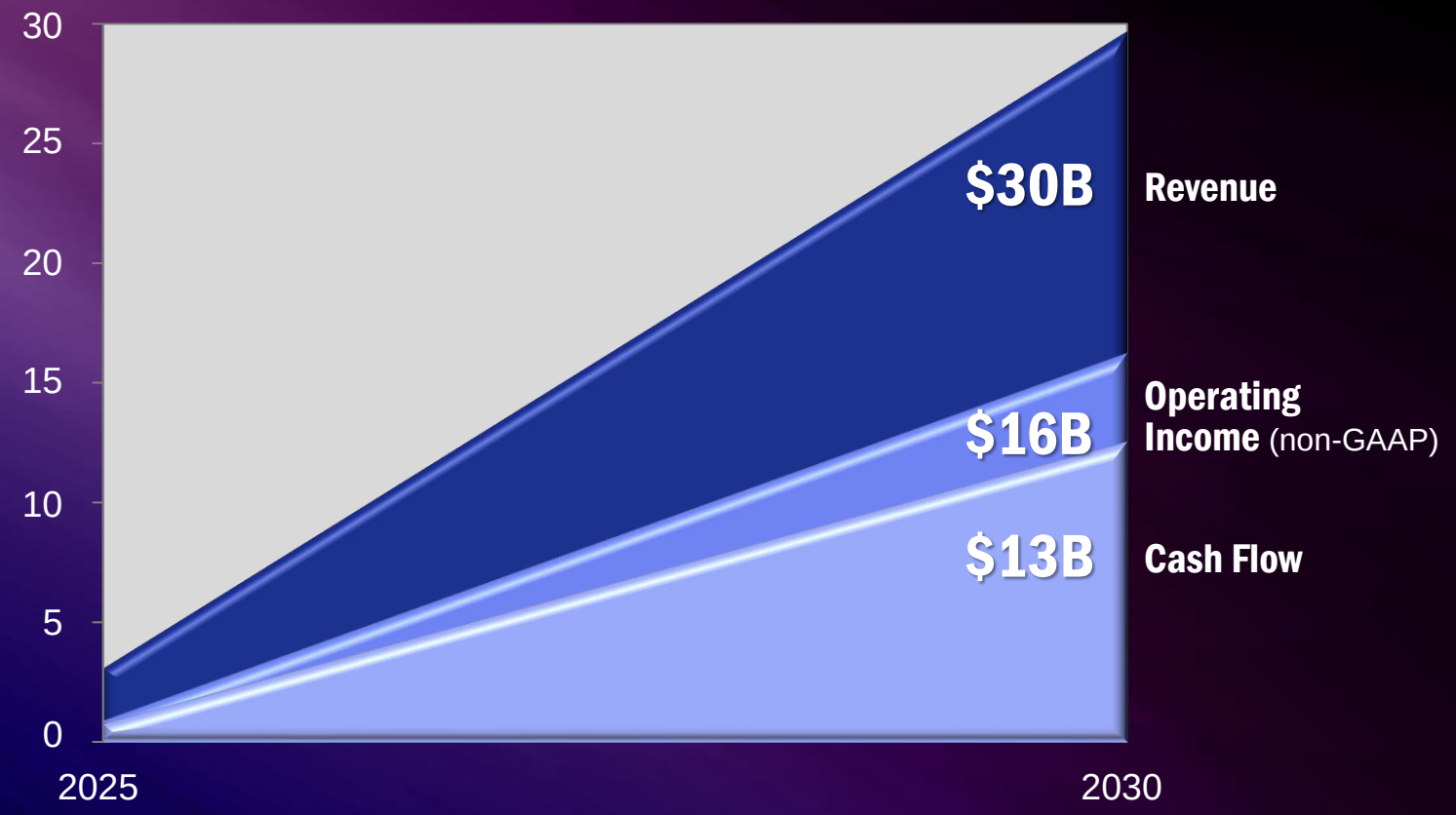



1. U.S. 30 month revenue figures from launch include a combination of actuals, forecasts and consensus estimates
2. To complete first 30 months, the last 4 quarters are forward-looking projections based upon external guidance

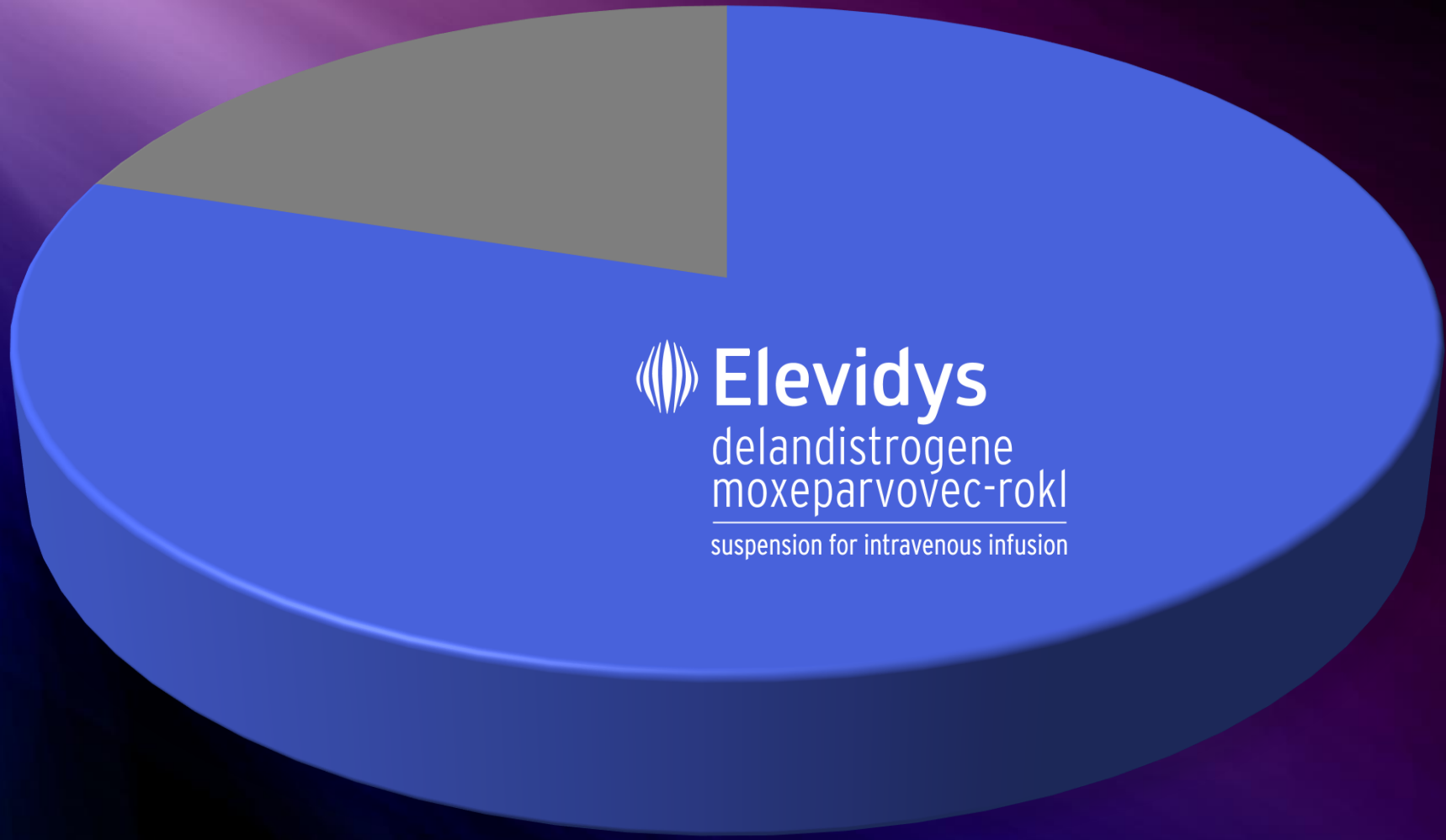
# Our largely de-risked portfolio is poised to deliver substantial revenue and cash flow through 2030

## Through 2030 our aspirational goal is

- 10 approved therapies on the market
- Revenue of ~$30B
- Non-GAAP Operating Income ~$16B
- Cash Flow ~$13B



# ELEVIDYS can treat 80% of Duchenne patients



# Clinical studies expanding reach and providing support of ELEVIDYS

## STUDY 101

Ages 4-7, ambulatory
Open-Label

**4**

PARTICIPANTS

## STUDY 102

Ages 4-7, ambulatory
Placebo-Controlled

**41**

PARTICIPANTS

## ENDEAVOR

Ages 3+, ambulatory and non-ambulatory
Open-Label

**58**

PARTICIPANTS

## EMBARK

Ages 4-7, ambulatory
Double-Blind, Placebo-Controlled

**126**

PARTICIPANTS

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## EMBARK

Ages 4-7, ambulatory
Double-Blind, Placebo-Controlled

**126**
PARTICIPANTS

## ENVISION

Double-blind placebo-controlled safety and efficacy in ambulatory and non-ambulatory participants

**148**
PARTICIPANTS

## ENVOL

Safety and expression in participants under 4 years of age

**~21**
PARTICIPANTS

## SRP-9001-104

Safety, tolerability and expression of delandistrogene moxeparvovec in association with imlifidase in participants with pre-existing antibodies to rAAVrh74

Up to **6**
AMBULATORY PARTICIPANTS

## SRP-9001-105

Safety, tolerability and expression of delandistrogene moxeparvovec following plasmapheresis in participants with pre-existing antibodies to rAAVrh74

Up to **16**
AMBULATORY PARTICIPANTS

## EXPEDITION

Safety and efficacy in subjects who have previously received delandistrogene moxeparvovec in a clinical study (long-term follow-up study)

**400**
PARTICIPANTS

## ENDURE

Comparative effectiveness and safety of delandistrogene moxeparvovec vs. standard of care under conditions of routine clinical practice

Up to **500**
PARTICIPANTS

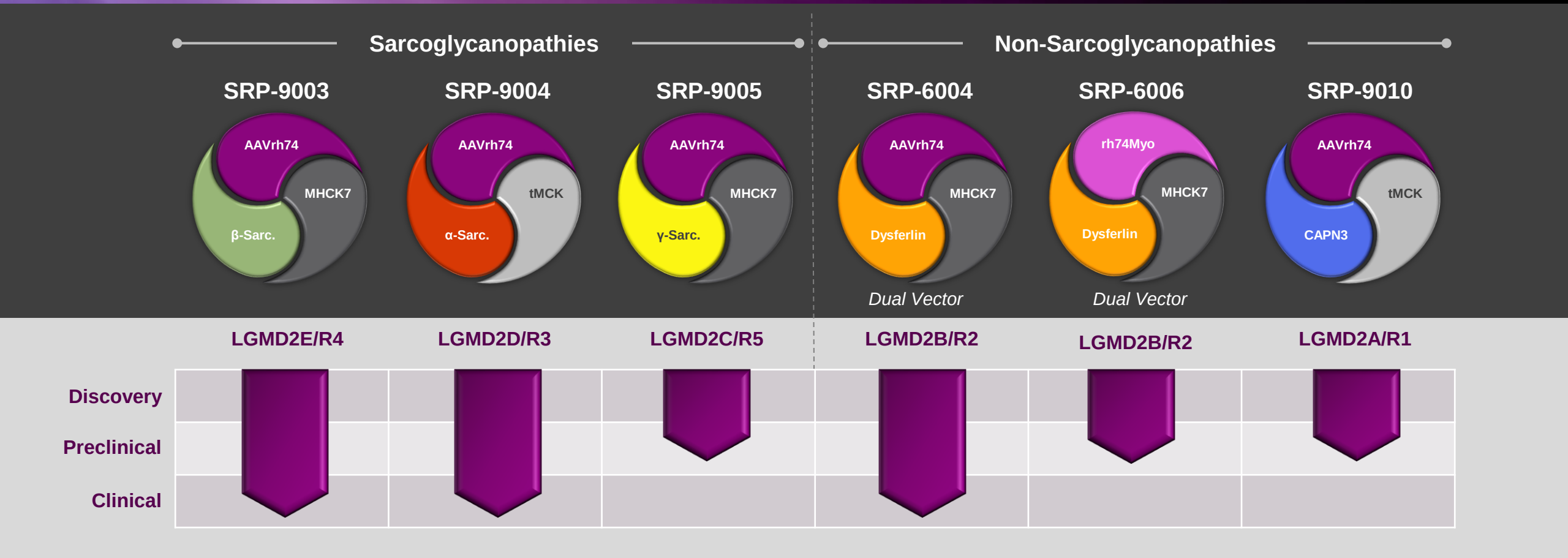
# Robust ELEVIDYS expansion opportunities are still ahead of us

Reduction in COGS / margin expansion from suspension manufacturing technology — ~90%

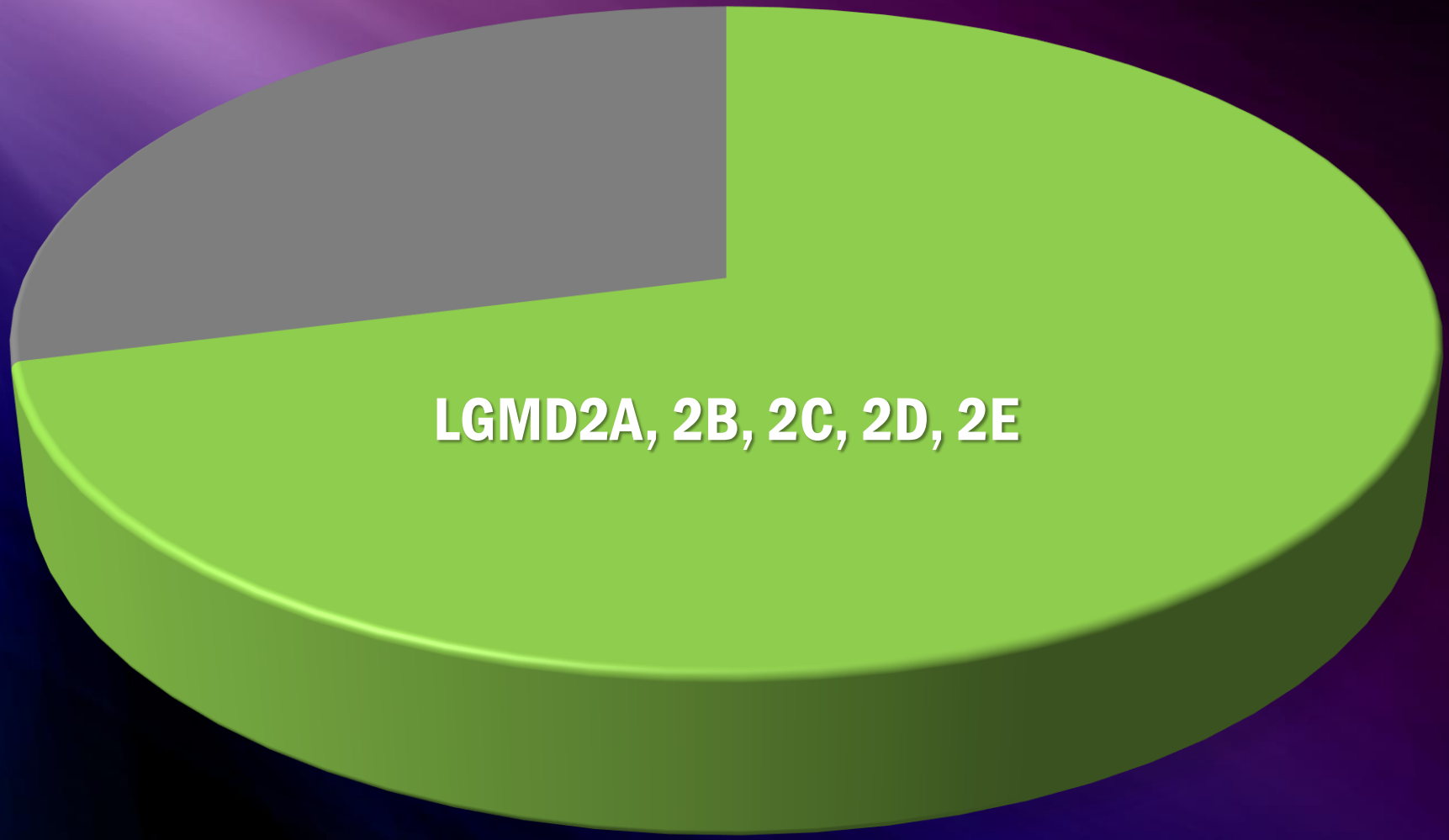
Ability to treat AB+ patients — 15%

Recognize royalties from sales in currently approved and potential new ex-U.S. markets — $500M+ peak year

# Market leading gene therapy portfolio in LGMD

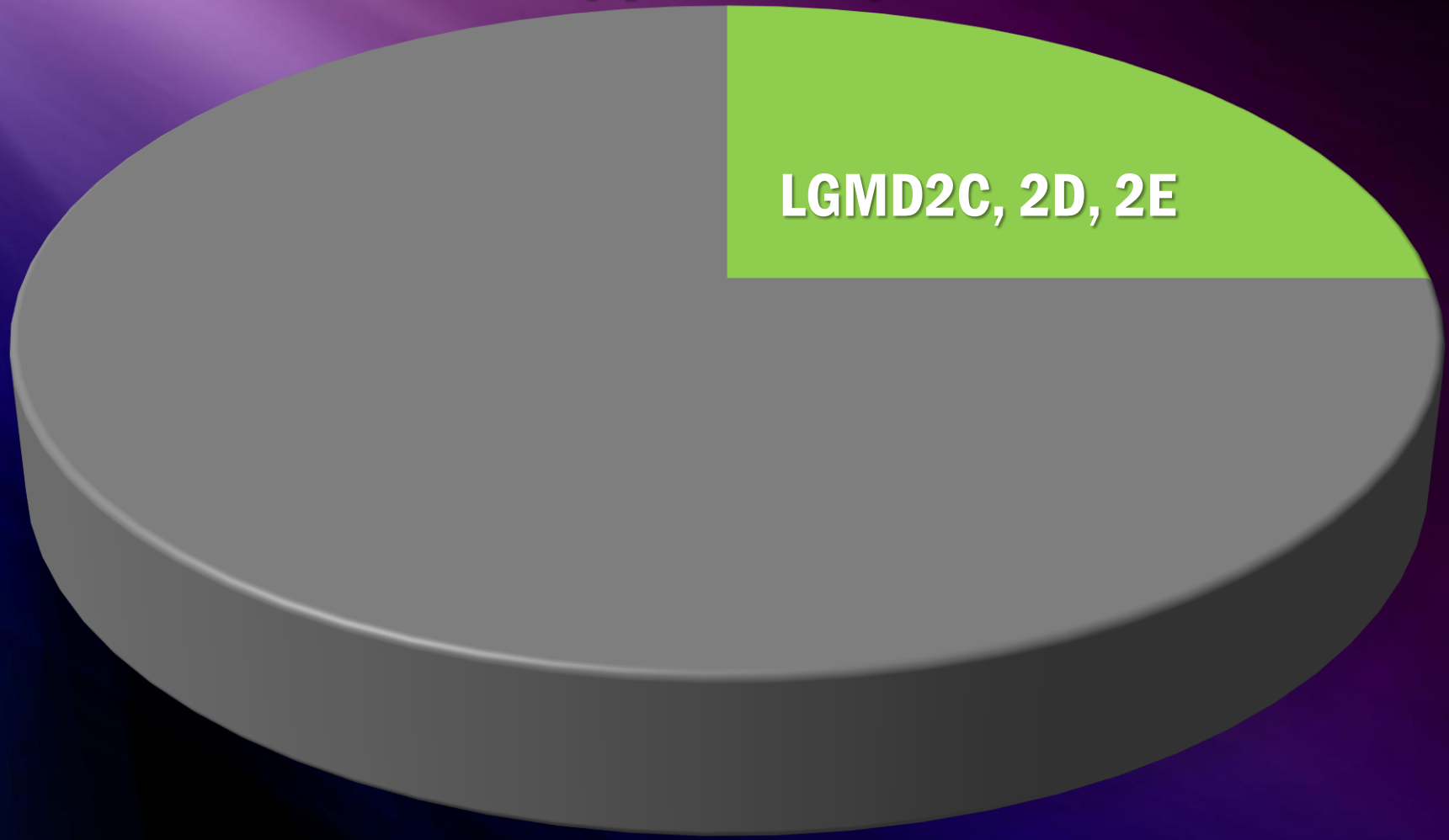


# Sarepta's LGMD therapies represent 71%* of the ELEVIDYS commercial opportunity



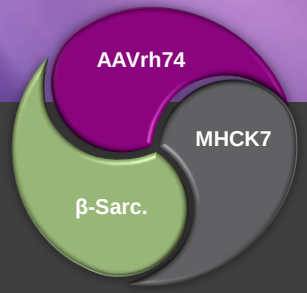

*U.S. patient population

# Sarepta's programs to treat LGMD2C/R5, 2D/R3 and 2E/R4 (sarcoglycanopathies) represent 25%* of the ELEVIDYS commercial opportunity




*U.S. patient population

# Catalysts across the LGMD clinical programs

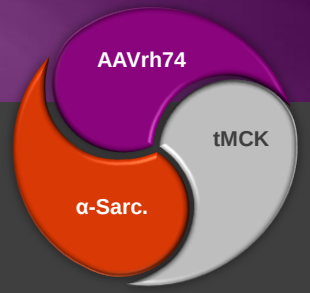



## SRP-9003 LGMD2E/R4

### Phase 3 Registrational Study (EMERGENE)

- Ages 4+
- 5-year, single-arm, open-label study
- 17 participants (11 ambulatory; 6 non-ambulatory)
- Global, confirmatory study evaluating change from baseline expression
- Enrollment complete
- **Biomarker data 1H of 2025**

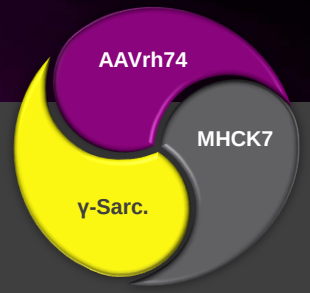
***BLA filing before end of 2025***




## SRP-9004 LGMD2D/R3

### Phase 1 (DISCOVERY)

- Ages 4+
- 5-year single-arm, open-label study
- Proof-of-concept study evaluating safety and change from baseline expression
- **Enrolling**




## SRP-9005 LGMD2C/R5

### Phase 1

- Ages 4+
- 5-year single-arm, open-label study
- First-in-human study evaluating safety and change from baseline expression
- **Anticipated to start Q1 2025**

2E EMERGENE

DISCOVERY
LGMD2D/R3 GENE THERAPY TRIAL

# Arrowhead deal elevates Sarepta to an enduring, fully integrated genetic medicines company

**Expansion into chronic therapies**

**Leverages neuromuscular expertise**

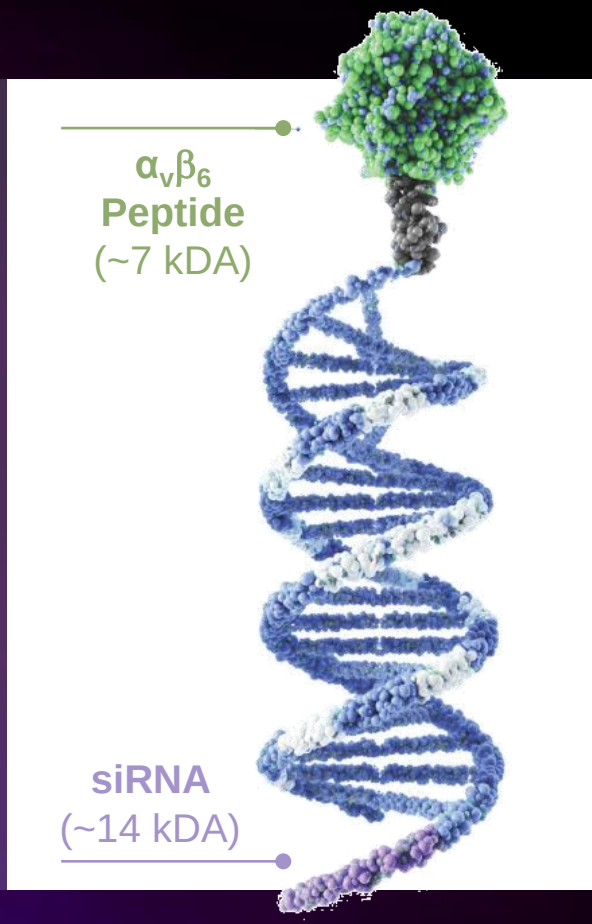
**Diversification into CNS, cardiomyopathy and pulmonary**

**Blockbuster mid-term commercial opportunities**

**Drives value and growth for near-, mid- and long-term**

# Best-in-class profile with Arrowhead's siRNA platform

| | |
|---|---|
| **siRNA Design** | Effective and efficient target knock-down, improved durability, specificity, and reduction of off-target effects |
| **Delivery** | Superior tissue targeting |
| **Dosing/Safety** | Less frequent dosing; favorable safety profile |



# Facioscapulohumerol muscular dystrophy (FSHD1)

**A rare genetic disease that causes weakness in the skeletal muscles.**
Progressively spreads from the face into other areas, including scapular girdle, upper limb, pelvic girdle, abdominal and leg muscles. [1]

- FSHD1 is linked to deletions of D4Z4 units on chromosome 4.[1]
- The average age of diagnosis is age 20. [1]
- ~50% of FSHD patients will require a wheelchair after ~20 years.
- There is currently no cure and there are no disease-modifying treatments.

**~13,000**
Diagnosed patients in the U.S.

**70%**
patients experience debilitating pain and fatigue[2]

**PROGRAM:**
**ARO-DUX4** is an RNA interference (RNAi) conjugate designed to specifically target the gene that encodes human double homeobox 4 (DUX4) protein.

**STAGE:**
**Phase 1/2**

1. https://www.mda.org/disease/facioscapulohumeral-muscular-dystrophy/signs-and-symptoms
2. https://www.fshdsociety.org/what-is-fshd/

# Myotonic Dystrophy Type 1 (DM1)

**A form of muscular dystrophy that affects muscles and many other organs in the body.[1]**

- Myotonic dystrophy (DM) is the most common form of muscular dystrophy. [2]
- There are two types of DM: DM1 is caused by mutations in the DMPK gene and is generally more severe than DM2.[1]
- DM1 impacts the respiratory muscle and significant breathing problems can result. [3] As DM1 progresses, the heart can develop an abnormal rhythm and weaken. [1]
- Life expectancy is shortened. [4]
- There is currently no cure and there are no disease-modifying treatments for DM1.

**~30,000**
Diagnosed patients in the U.S.

**58 years**
Mean age at death[5]

**PROGRAM:**
**ARO-DM1** is an RNAi conjugate designed to specifically silence DMPK mRNA in skeletal muscle.

**STAGE:**
**Phase 1/2**

1. https://www.mda.org/disease/myotonic-dystrophy
2. https://www.nichd.nih.gov/health/topics/musculardys/conditioninfo/types
3. https://www.myotonic.org/what-dm/how-dm-affects-your-body/respiratory-system
4. https://www.ncbi.nlm.nih.gov/books/NBK1165/
5. Bassez et al, Neuromuscular Disorders 2024

# Spinocerebellar Ataxia Type 2 (SCA2)

**Spinocerebrellar ataxia (SCA) is a group of rare, genetic neurodegenerative disorders** leading to severe disability and premature death.[1]

- In SCA, the nerve fibers carrying messages to and from the brain are affected, resulting in degeneration of the cerebellum (the coordination center of the brain). [1]
- There are more than 40 types of SCA.[2] SCA2 is caused by mutations in the ATXN2 gene.[3]
- SCA2 symptoms include movement, vision, speech and swallowing problems, as well as peripheral neuropathy, tremor and muscle wasting; and may include short-term memory problems and dementia.[1]
- There is currently no cure and there are no disease-modifying treatments.

**~2,000**
Diagnosed SCA2 patients in the U.S. [4]

**10-20 years**
After diagnosis, patients become dependent on a wheelchair [1]

**PROGRAM:**
**ARO-ATXN2** RNAi targets production of toxic ATXN2 protein that causes the disease.

**STAGE:**
**Phase 1**


1. https://www.ninds.nih.gov/health-information/disorders/spinocerebellar-ataxias-including-machado-joseph-disease
2. https://my.clevelandclinic.org/health/diseases/24077-spinocerebellar-ataxia
3. https://medlineplus.gov/genetics/condition/spinocerebellar-ataxia-type-2/#causes
4. Ruano et al, Neuroepidemiology 2014

# Idiopathic Pulmonary Fibrosis (IPF)

**A chronic lung disease that develops when the lung tissue becomes scarred or fibrotic over time.** The scarring progresses differently in everyone, as some people's disease stays the same for years, and in others, the condition can worsen rapidly. [1]

- Though the cause is relatively unknown, the risk for IPF is higher amongst smokers or have a family history of IPF. The risk also increases with age, most often impacting people over age 50. [1]
- The most common symptoms of IPF are shortness of breath and dry cough that get worse over time. Complications of IPF include pulmonary hypertension and respiratory failure. [1]
- There is a great unmet clinical need for this disease, as IPF patients have few options to help slow the progression of the disease.

**~60,000**

Diagnosed patients in the U.S.

**~5 years**

is the average life expectancy from the time of diagnosis [2]

**PROGRAM:**

**ARO-MMP7** is designed to reduce expression of matrix metalloproteinase 7 (MMP7) as a potential treatment for idiopathic pulmonary fibrosis (IPF).

**STAGE:**

**Phase 1/2**


1. https://www.nhlbi.nih.gov/health/idiopathic-pulmonary-fibrosis/causes
2. https://pmc.ncbi.nlm.nih.gov/articles/PMC9779053/

# Track record of successfully delivering across the business

- Successfully executed strategic plan set forth in 2017
- Achieved approval of 4 therapies, significantly growing revenue
- Advanced ELEVIDYS to the market, obtaining the broadest possible label
- Achieved profitability
- Sustainably cash flow positive
- Poised to deliver robust revenue through this decade
- Built robust gene therapy and siRNA pipeline that addresses large, unmet markets, generating multiple clinical data readouts
- Attracted and retained industry-leading team at all levels of the organization

# Numerous 2025 data and program milestones

## Late 2024 (Completed)

### LGMD2E/R4

**SRP-9003:**

- EMERGENE enrollment completed

### LGMD2D/R3

**SRP-9004:**

- Phase 1 initiated

### SCA2

**ARO-ATXN2:**

- Phase 1 initiated

## 1H 2025

### Duchenne

**ELEVIDYS:**

- EMBARK 2-year topline data vs. EC
- 3-year pooled functional analysis vs. EC
- SRP-9001-101, 102, 103 and 301 pooled cardiac data
- ENVISION study enrollment completion

### LGMD2C/R5

**SRP-9005:**

- Start phase 1

### LGMD2E/R4

**SRP-9003:**

- EMERGENE expression data

## 2H 2025

### FSHD1

**ARO-DUX4:**

- Preliminary results from phase 1

### DM1

**ARO-DM1:**

- Preliminary results from phase 1

### Duchenne

**ELEVIDYS:**

- Study 104 (imlifidase) expression data
- Study 105 (plasmapheresis) expression data
- ENDEAVOR Cohort 6 expression data
- BLA supplement <4 years old

### LGMD2E/R4, LGMD2D/R3, LGMD2C/R5

- JOURNEY data in sarcoglycanopathies

### LGMD2E/R4

**SRP-9003:**

- BLA filing
- VOYAGENE data (expression, function, safety)

### LGMD2A/R1

**SRP-9010:**

- IND filing

### Huntington's Disease

**ARO-HTT:**

- IND filing


# R&D Day 2025

*details to come...*

# Delivering on our goals

# Sarepta 2030

SAREPTA THERAPEUTICS

(NASDAQ:SRPT)

**DILLON**
Living with Duchenne muscular dystrophy

**AMANDA**
Living with limb-girdle muscular dystrophy