SAREPTA THERAPEUTICS

DOUG INGRAM

President and CEO

Sarepta Therapeutics, Inc. (NASDAQ:SRPT)
JPMorgan Healthcare Conference
San Francisco, California
JANUARY 8, 2024

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; potential solutions and market opportunities with our RNA technologies, gene therapy and gene editing; the potential benefits of our technologies and scientific approaches; the potential benefits of PMO and PPMO; the potential of gene therapy’s applicability across disease; the potential of ELEVIDYS to benefit all patients living with Duchenne; our goal to become a life-transforming big biotech; and expected milestones and plans, including our understanding that, for ELEVIDYS, the FDA will evaluate a labeling expansion to the fullest extent possible based on a review of the data and will do so rapidly, our belief that our confirmatory trial for ELEVIDYS confirms its benefits and our accelerated approval should be converted to traditional approval, announcing clinical data for SRP-5051 in 2024, and our other 2024 priorities, including moving our LGMD programs forward, including SRP-9003, driving innovation in manufacturing and other R&D goals.

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: the FDA may not approve a supplement to expand the approved label for ELEVIDYS; the FDA may not convert accelerated approval into traditional approval; 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 PPMO and gene therapy-based product candidates, 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 Duchenne and LGMD is smaller than estimated, our revenue and ability to achieve profitability 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 our gene therapy product candidates; we may not be able to successfully scale up manufacturing of our product candidates 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 Annual Report on Form 10-K and 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.

The vision set forth in 2017 was clear:

Build a leading biotech enterprise grounded in a patient-first, science-driven philosophy

Where we were as we entered 2017…

1 TECHNOLOGY PLATFORM

PRE-PROFITABILITY

2016 REVENUE
Approx.
$5M

PIPELINE
6

ON-MARKET THERAPIES
1

Market Cap:
$1.50B
12/30/16

The ingredients

1. A passionate mission
2. A well-articulated strategy
3. The ability to attract like-minded professionals
4. A culture that challenges convention and rewards execution

Transformational change

Where we were as we entered 2017…

Where we are today in 2024…

*Based on estimated expected QTD Non-GAAP net profit
**2023 estimated net product revenue (unaudited)

Duchenne

HUDSON & JACOB
Living with Duchenne muscular dystrophy

Duchenne muscular dystrophy affects approximately 1 in 3,500 - 5,000 newborn males worldwide[1]

- Duchenne is a rare, fatal neuromuscular genetic disease inherited in an X-linked recessive pattern[2]
- Muscle weakness becomes increasingly noticeable by 3 to 5 years of age, and most patients use a wheelchair by the time they are 10 to 14 years old[2]
- During adolescence, cardiac and respiratory muscle deterioration lead to serious, life-threatening complications[3]

1. National Institutes of Health. Genetics Home Reference. Duchenne and Becker muscular dystrophy; https://ghr.nlm.nih.gov/condition/duchenne-and-becker-muscular- dystrophy. Accessed Jan 2020.
2. McDonald CM, Abresch RT, Duong T, et al. Long-term effects of glucocorticoids on function, quality of life, and survival in patients with Duchenne muscular dystrophy: a prospective cohort study. *Lancet*. 2018;3(391):451-461.
3. Passamano L, Taglia A, et al. Improvement of survival in Duchenne Muscular Dystrophy: retrospective analysis of 835 patients. *Acta Myologica*. 2012;31(1): 121-125.

RNA-based PMO revenue exceed 2023 annual guidance of $925M

Q4 net product revenue total $234M*

and full-year total $945M*

Robust launch of ELEVIDYS generates

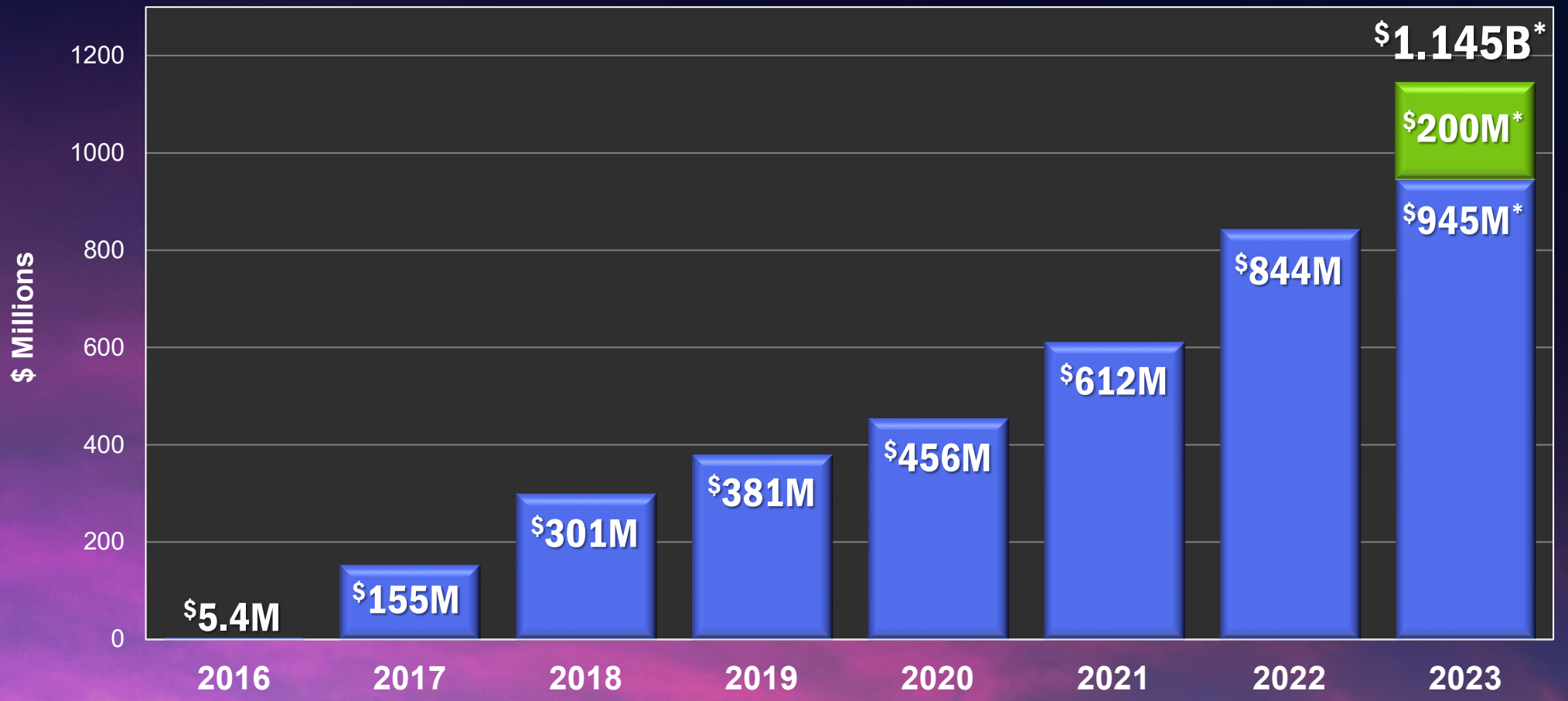
Q4 net product revenue of $131M*

and full-year revenue of $200M*

*Q4 QTD and YTD 2023 estimated net product revenue (unaudited)

Robust total net product revenue for Duchenne franchise (PMOs and gene therapy)

EXONDYS 51 (eteplirsen) Injection | VYONDYS 53 (golodirsen) Injection | AMONDYS 45 (casimersen) Injection | Elevidys delandistrogene moxeparvovec-rokl suspension for intravenous infusion



115%
7-year CAGR

* 2023 estimated net product revenue (unaudited)

ELEVIDYS (delandistrogene moxeparvovec-rokl): Expanding the opportunity for treatment

Granted FDA Approval*

Strong launch trajectory continues

Seeking label expansion

*Accelerated Approval (AA) granted by FDA on June 22, 2023, for Duchenne patients aged 4 through 5 years.

*ELEVIDYS: Clinical development plans

ENDEAVOR

STUDY 103

58 patients

Ages 3+, ambulatory and non-ambulatory

Open-Label

NCT04626674

- Expression and safety
- Enrollment completed for 4 cohorts; enrollment underway for 5th cohort
- Genetic mutation inclusion criteria varies by cohort

Exons 1-79

EMBARK

STUDY 301

125 patients

Ages 4-7, ambulatory

Double-Blind, Placebo-Controlled

NCT05096221

- Pivotal Phase 3 study
- Primary endpoint: NSAA
- Excludes mutations 1 to 17, 45

Exons 18-44, 46-79

ENVISION

STUDY 303

~148 patients

Ambulatory and non-ambulatory

Double-Blind, Placebo-Controlled

NCT05881408

- Phase 3 study
- 128 weeks, 2-part study
- Primary outcome (Part 1): Change from baseline at Week 72 in the total score of PUL**
- Study underway

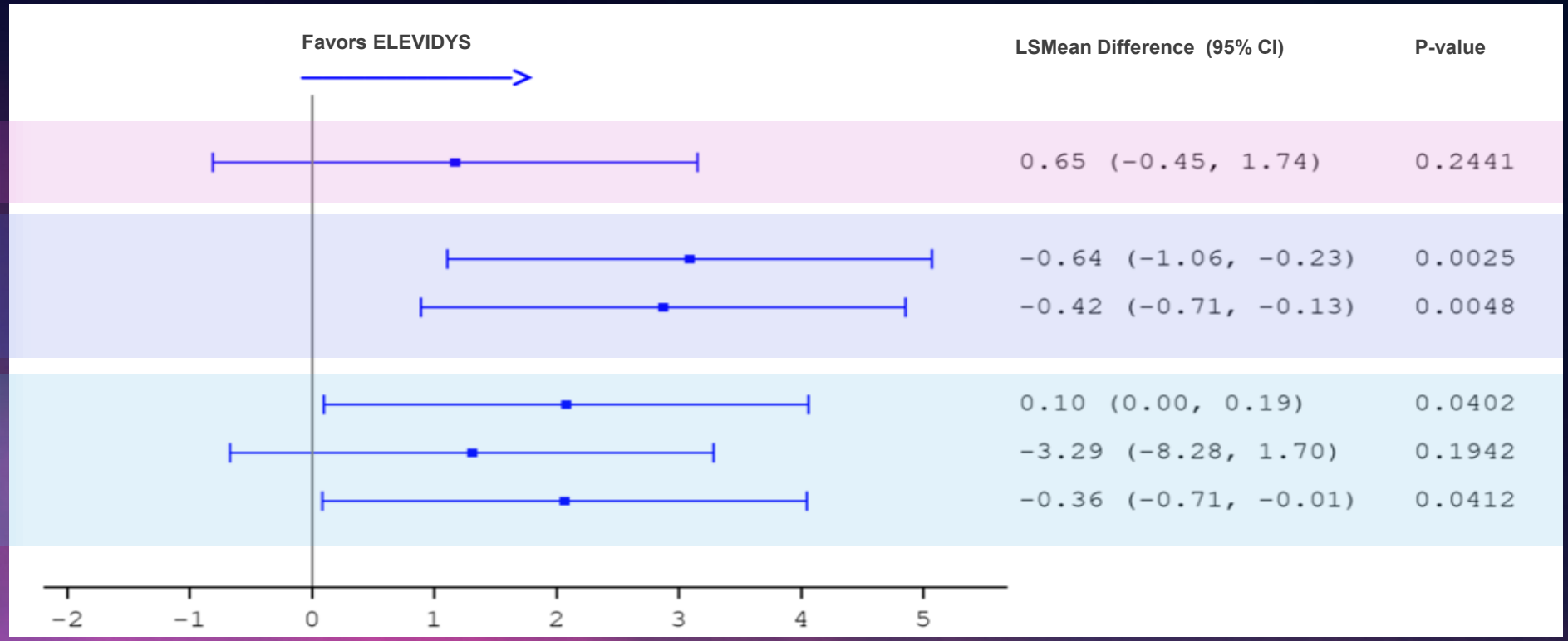
Exons 18-79

**PUL= performance of upper limb

*ELEVIDYS is contraindicated in patients with any deletion in exons 8 and/or 9.

Results favor treatment with ELEVIDYS on all endpoints

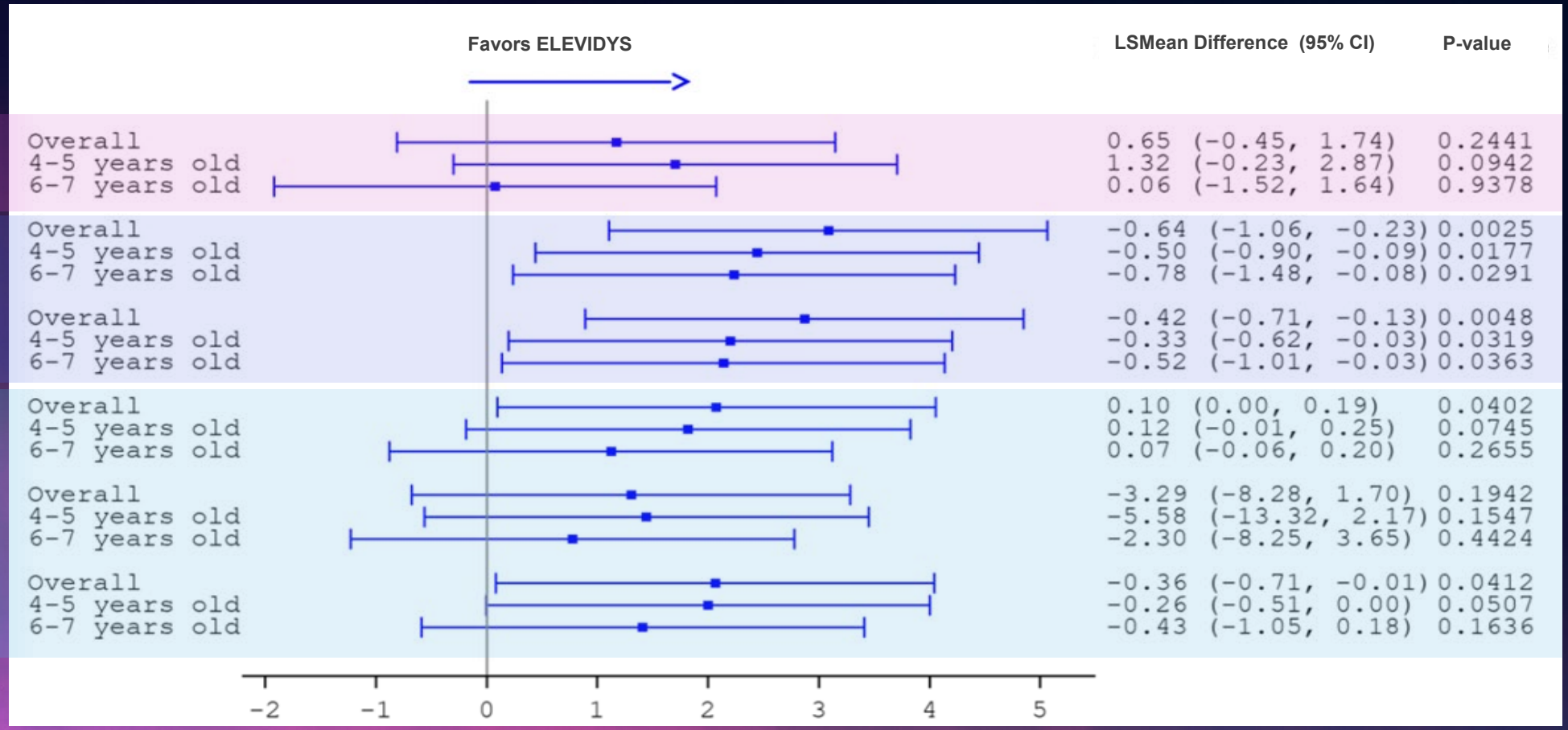
EMBARK achieved statistical significance on all pre-specified key secondary endpoints



* Timed function tests sign reversed to align favorable directions among effect endpoints

** Blue lines plot standardized test statistic (+/- 1.96) after dividing LSMean (95% CI) by standard error

Functional benefits of ELEVIDYS are not limited to a particular age group



* Timed function tests sign reversed to align favorable directions among effect endpoints
**Blue lines plot standardized test statistic (+/- 1.96) after dividing LSMean (95% CI) by standard error

Summary

- We believe the data from EMBARK exceeded the threshold for substantial evidence of effectiveness and the risk/benefit of ELEVIDYS remains favorable
- We are pleased with the consistency, the magnitude of response and the clinical meaningfulness of the results from EMBARK and from the body of evidence supporting ELEVIDYS
- The data support ELEVIDYS as a disease-modifying therapy and therefore we believe all patients with Duchenne can benefit from treatment
- Following positive discussions with FDA leadership, they are committed to evaluating a labeling expansion to the fullest extent possible based on a review of the data and will do so rapidly
- No new safety signals were observed

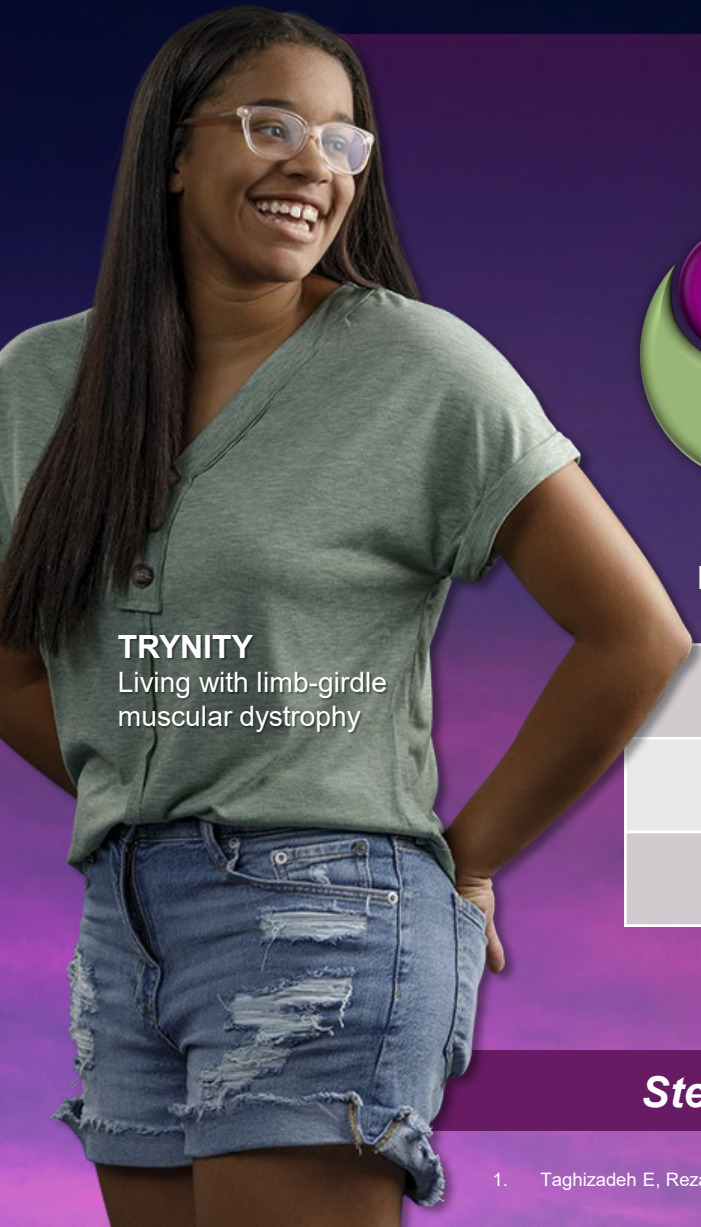
Path forward for ELEVIDYS

ELEVIDYS trials remain ongoing as Sarepta pursues label expansion

2024 priorities

- Serve the Duchenne community with approved therapies
- Announce clinical data for next-generation RNA-based therapy/PPMO (SRP-5051) to treat Duchenne
- Execute R&D productivity, including a novel new capsid and approaches to clearing pre-existing antibodies
- Drive innovation in manufacturing

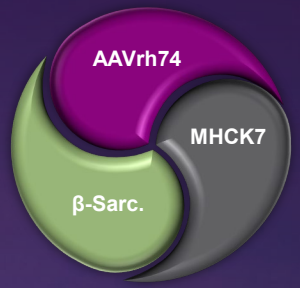
Market leading gene therapy portfolio in 6 LGMDs



TRYNITY
Living with limb-girdle muscular dystrophy

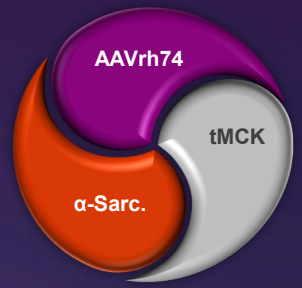
Sarcoglycanopathies

SRP-9003



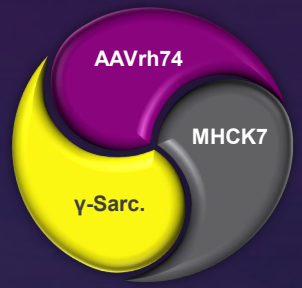
LGMD2E/R4

SRP-9004



LGMD2D/R3

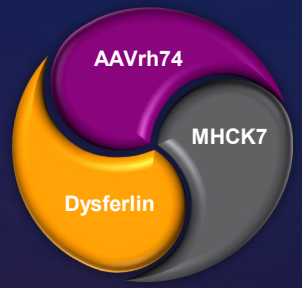
SRP-9005



LGMD2C/R5

Non-Sarcoglycanopathies

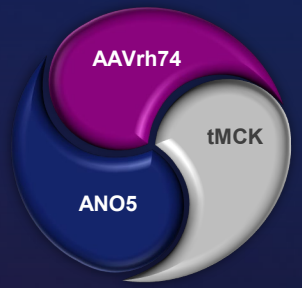
SRP-6004



Dual Vector

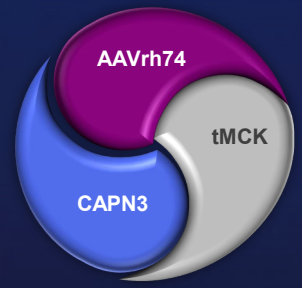
LGMD2B/R2

SRP-9006

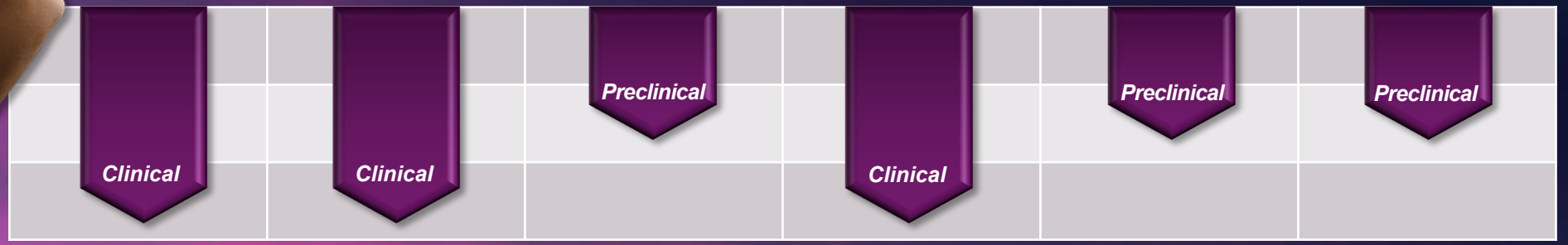


LGMD2L/R12

SRP-9010



LGMD2A/R1



Steady stream of gene therapy candidates covering >70% of known LGMD patients[1]

1. Taghizadeh E, Rezaee M et al. *J Cell Physiol.* 2019;234(6):7874-7884.

2024 will be monumental

Transformational change, unparalleled opportunity

Where we were as we entered 2017…

1 TECHNOLOGY PLATFORM

PRE-PROFITABILITY

2016 REVENUE Approx. $5M

PIPELINE 6

ON-MARKET THERAPIES 1

Market Cap: $1.50B (12/30/16)

Where we are today in 2024…

3 TECHNOLOGY PLATFORMS

PROFITABLE*

REVENUE** $1.145B

PIPELINE 40+

ON-MARKET THERAPIES 4

Market Cap: $9.02B (12/29/23)

*Based on estimated expected QTD Non-GAAP net profit

**2023 estimated net product revenue (unaudited)

Transformational change, unparalleled opportunity

Where we were as we entered 2017…

Where we are today in 2024…

By 2030: Sarepta is poised to become a big biotech, focusing on cutting-edge genetic medicine to improve the human condition

*Based on estimated expected QTD Non-GAAP net profit
**2023 estimated net product revenue (unaudited)

From the words of someone who drives our mission and passion

> *There is more to do.*
>
> **But now let us pause and bask in the glow of our achievement.**
> **Let us pause in gratitude.**
> **Let us pause in celebration.**
> **Let us watch as dawn rises and brings in the day.**

BUDDY CASSIDY
Living with Duchenne and an FDA advisory committee member for ELEVIDYS
May 2023

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President and CEO

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