

Patients can't wait for the next breakthrough  
in medical research.

**So neither will we.**

*Doug Ingram*  
*President and CEO*  
*Sarepta Therapeutics, Inc. (NASDAQ: SRPT)*

June 22, 2023



**BENJAMIN**  
Living with Duchenne  
muscular dystrophy

# 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, without limitation, statements relating to our future operations, business plans, priorities, research and development programs; the potential benefits and risks of ELEVIDYS; the expected demand for ELEVIDYS; our guidance for full-year 2023 net product revenue for our three approved oligonucleotide therapies of \$925 million dollars; our expectation that ELEVIDYS will have some cannibalizing impact on sales; the potential successful launch of ELEVIDYS; the goal of ELEVIDYS to halt the otherwise irreversible muscle damage caused by Duchenne; our belief that we have met our goal of ensuring that the costs to the healthcare system are less than the potential benefits of ELEVIDYS; the belief that nearly all infusions of ELEVIDYS will be subject to a statutory discount; our cost-effectiveness analysis; the potential payer budget impact; our understanding from the FDA that if the read out of EMBARK meets its objectives, the FDA intends to entertain a non-age restricted expansion of the label and that this will be done with maximal speed by the FDA; and expected plans and milestones, including our expectation that EMBARK, if the study meets its objectives, will act as our confirmatory study and for potential label expansion, our plan to submit a BLA supplement as soon as possible post-EMBARK and expand the label to remove any age restrictions or restrictions on the basis of ambulatory status, if the trial is successful, our goal to expand the ELEVIDYS label to treat as much as 95% of the Duchenne population, if successful, and commencing multiple trials to explore the clearance of pre-existing antibodies, in addition to ENVISION.*

*Actual results could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties. Known risk factors include the following: the FDA may not approve a supplement to expand the approved label for ELEVIDYS; continued approval may be contingent upon verification of a clinical benefit in confirmatory trials; we may not be able to comply with all FDA requests 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, as well as the development of our product candidates and our financial and contractual obligations; our dependence on certain manufacturers to produce our products and product candidates, including any inability on our part to accurately anticipate product demand and to secure in a timely manner manufacturing capacity to meet product demand, may impair the availability of product to successfully support various programs; our data may not be sufficient for obtaining regulatory approval; 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; we are subject to uncertainty related to reimbursement policies; success in preclinical and clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful, and the results of future research may not be consistent with past positive results or with advisory committee recommendations, or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; the commencement and completion of our clinical trials and announcement of results may be delayed or prevented for a number of reasons, including, among others, denial by the regulatory agencies of permission to proceed with our clinical trials, or placement of a clinical trial on hold, challenges in identifying, recruiting, enrolling and retaining patients to participate in clinical trials and inadequate quantity or quality of supplies of a product candidate or other materials necessary to conduct clinical trials; different methodologies, assumptions and applications we use to assess particular safety or efficacy parameters may yield different statistical results, and even if we believe the data collected from clinical trials of our product candidates are positive, these data may not be sufficient to support approval by the FDA or other global regulatory authorities; we may not be able to execute on our business plans, including meeting our expected or planned regulatory milestones and timelines, research and clinical development plans, and bringing our product candidates to market, for various reasons, many of which may be outside of our control, including possible limitations of company 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 with respect to patents that cover our product candidates; and those risks identified under the heading “Risk Factors” in our most recent Annual Report on Form 10-K for the year ended December 31, 2022, and Form 10-Q filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by the Company, which you are encouraged to review.*

*Any of the foregoing risks could materially and adversely affect the Company’s business, results of operations and the trading price of Sarepta’s common stock. For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review the SEC filings made by Sarepta. We caution investors not to place considerable reliance on the forward-looking statements contained in this press release. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof, except as required by law.*

# Elevidys

delandistrogene

moxeparvovec-rokl

suspension for intravenous infusion

# An Historic Milestone In Genetic Medicine



**BENJAMIN**  
Living with Duchenne  
muscular dystrophy

# Disease progression in Duchenne<sup>1-3</sup>

**5 TO 7  
YEARS**



- Motor delay
- Enlarged calves
- Toe walking
- Standing from supine, climbing stairs more difficult

**8 TO 11  
YEARS**



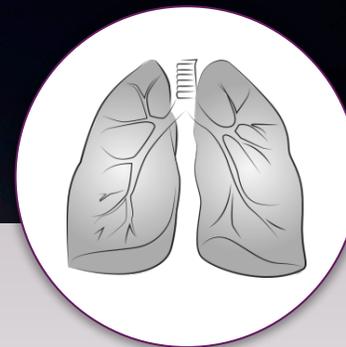
- Increasing loss of walking ability
- Part-time wheelchair use

**EARLY  
TEENS**



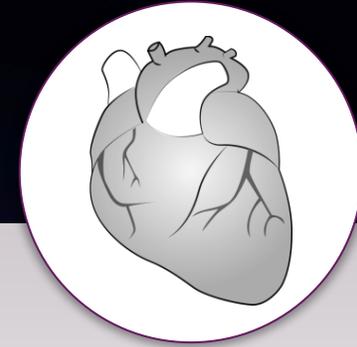
- Loss of ambulation
- Full-time wheelchair use
- Increasing loss of upper limb function

**TEENS**



- Increasing respiratory impairment
- Ventilatory support often required
- Unable to perform activities of daily living

**TEENS TO  
TWENTIES**



- Increasing cardiac dysfunction
- Heart failure
- Death

**EARLY AMBULATORY**

**LATE AMBULATORY**

**EARLY NON-AMBULATORY**

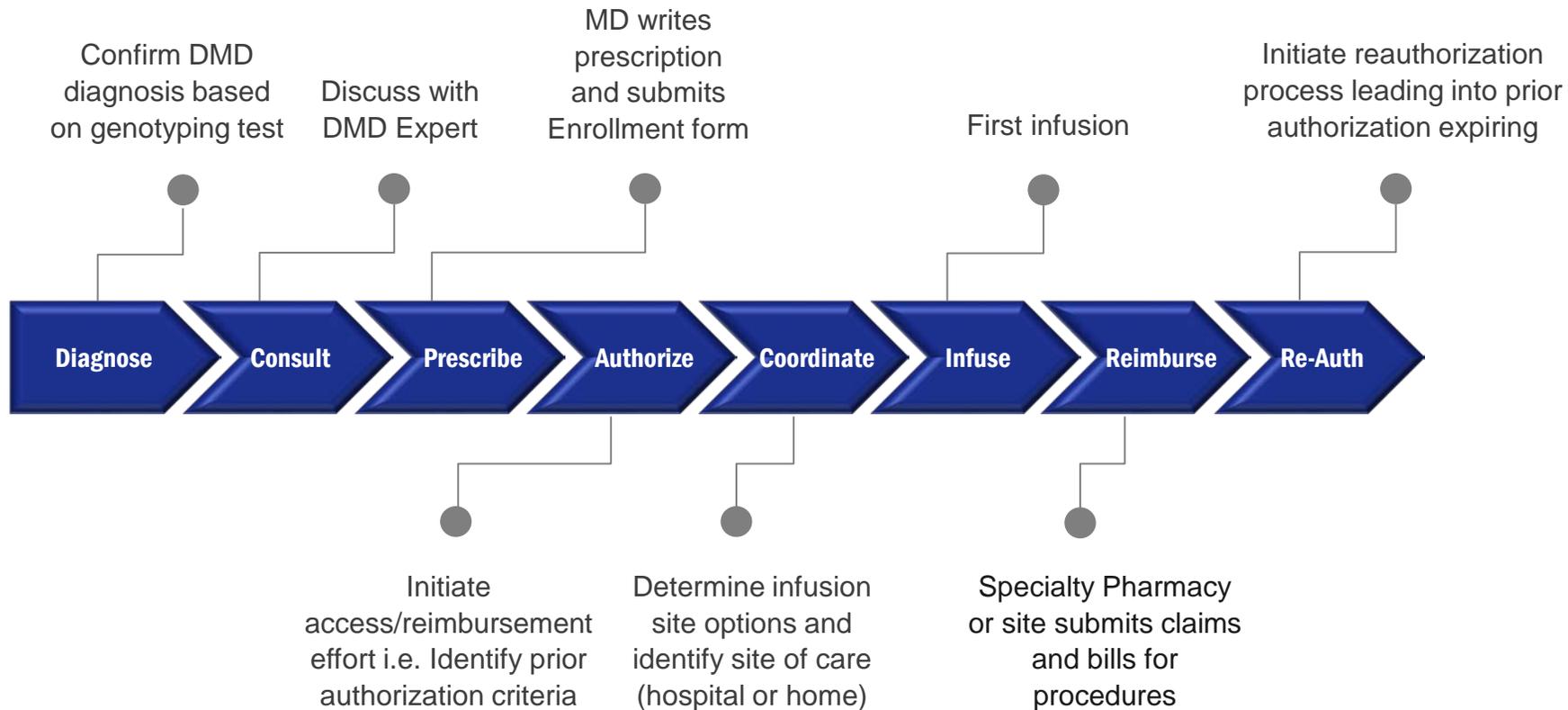
**LATE NON-AMBULATORY**

1. Bushby K, Finkel R, Birnkrant DJ, et al. *Lancet Neurol.* 2010;9:77-93.  
2. Emery AEH. *Lancet.* 2002;359:687-695.  
3. Landfeldt E, Lindgren P, Bell CF, et al. *Neurology.* 2014;83(6):529-536.

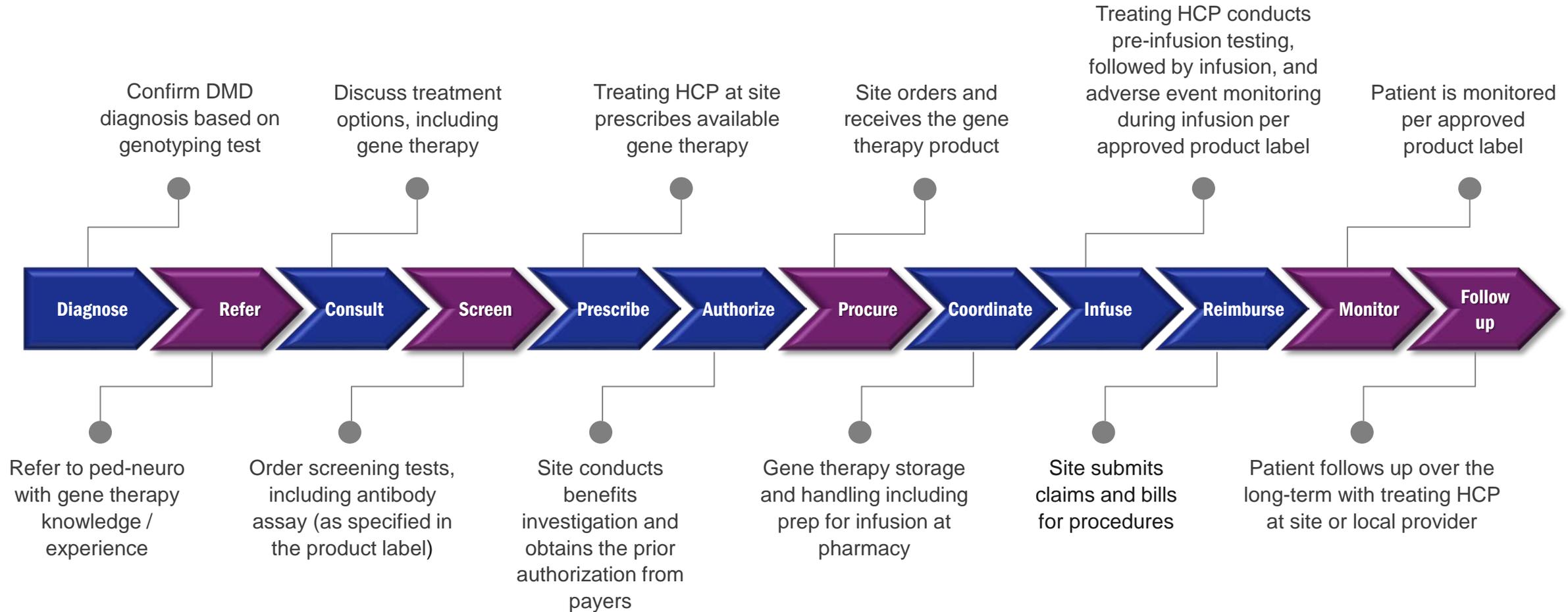
**Sarepta is the recognized leader in  
Duchenne research and commercialization....**

# The Patient Journey

# PMO patient journey



# Gene therapy patient journey...



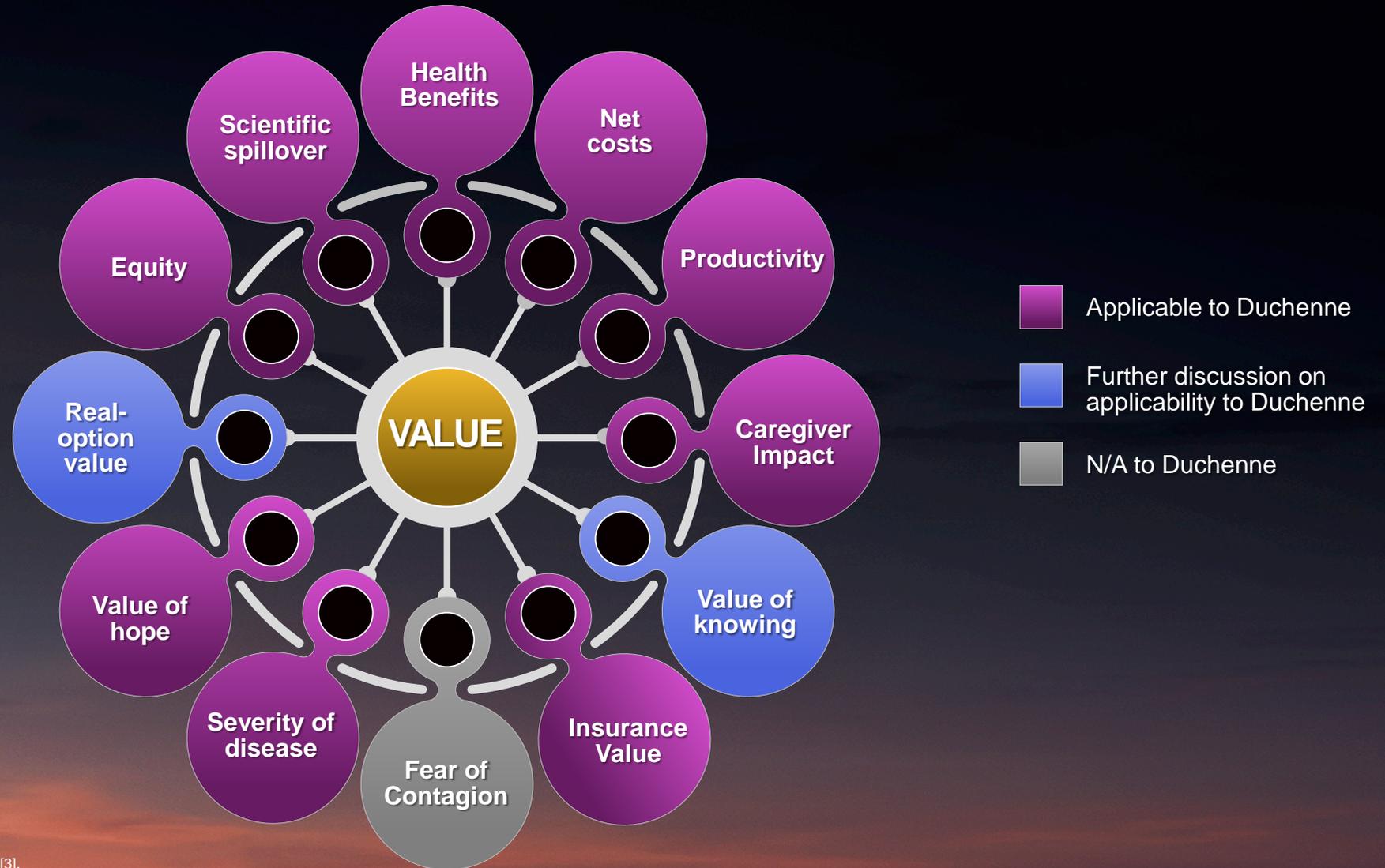
*...is more complex and requires engaging a multitude of stakeholders along the way*

# *Journal of Market Access and Health Policy*

---

*Assessing the value of delandistrogene moxeparvovec (SRP-9001) gene therapy in patients with Duchenne muscular dystrophy in the United States*

# A holistic approach to assessing innovative treatments



# The cost-effectiveness analysis shows the value of ELEVIDYS in treating Duchenne

**ELEVIDYS has the potential to be...**

cost-effective in a range of **\$5 million to \$13 million**  
compared to standard-of-care alone

# The cost-effectiveness analysis shows the value of ELEVIDYS in treating Duchenne

**ELEVIDYS has the potential to be...**

cost-effective in a range of **\$5 million to \$13 million**  
compared to standard-of-care alone

**At a price of \$3.2 million**  
**ELEVIDYS is**

below the cost-effectiveness range

# Expanding the Label

# Reaching More Patients

# Thank You



# Q&A

Patients can't wait for the next breakthrough  
in medical research.

**So neither will we.**

*Doug Ingram*  
*President and CEO*  
*Sarepta Therapeutics, Inc. (NASDAQ: SRPT)*

June 22, 2023



**BENJAMIN**  
Living with Duchenne  
muscular dystrophy