PRECISION BIOSCIENCES

PBGENE-DMD gene editing drives safe, efficacious, and durable functional improvement in a humanized Duchenne muscular dystrophy mouse model

ASGCT

May 14, 2026

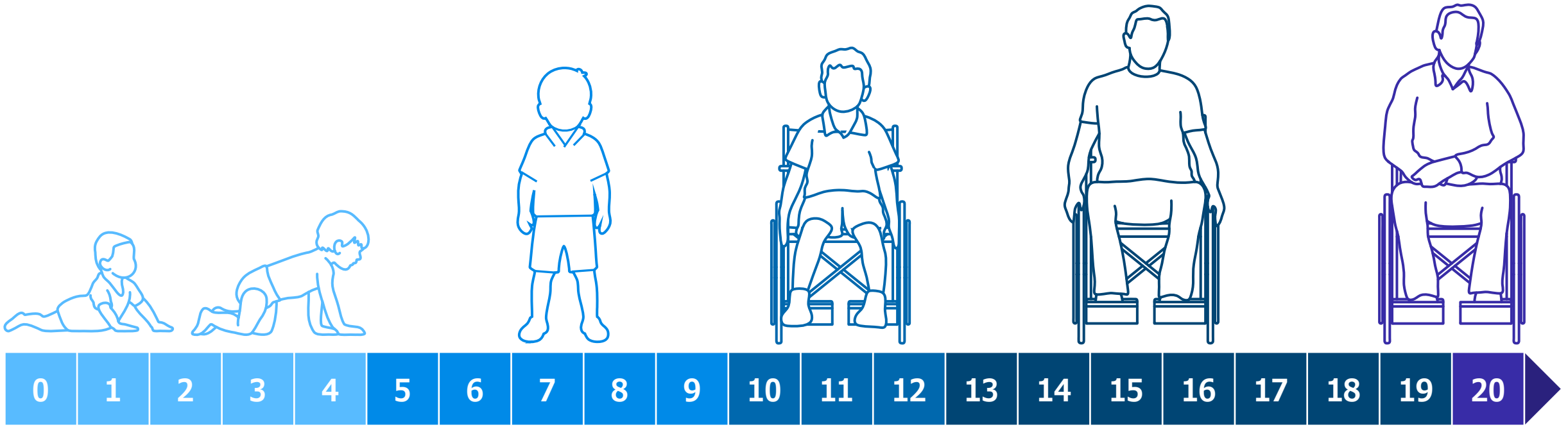
Forward-Looking Statements

This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this presentation that do not relate to matters of historical fact should be considered forward-looking statements, including, without limitation, statements regarding the key advantages of ARCUS and its key capabilities and differentiating characteristics; expectations about operational initiatives, strategies, further development, or timing of additional updates or data releases of PBGENE-DMD, timing and progress of IRB processes and site activations following IND clearance for the PBGENE-DMD program and FUNCTION-DMD trial; the suggestion of broad and robust efficacy in early juvenile mice supporting potential benefit of earlier intervention of PBGENE-DMD in younger DMD patient population; the design of PBGENE-DMD to improve function over time and address 60% of patients with DMD; the design of PBGENE-DMD to excise the 'hot-spot' region between exons 45 and 55 of the dystrophin gene resulting in naturally-expressed, functional dystrophin; translation of results in preclinical studies of ARCUS nucleases (including PBGENE-DMD) to clinical studies in humans; the expectation that ≥5% expression of functional dystrophin protein is needed for therapeutic benefit in DMD patients; and the preclinical and clinical development and demonstrated, potential and expected safety, efficacy, durability, and benefit of PBGENE-DMD, as well as our other product candidates and those being developed by partners. In some cases, you can identify forward-looking statements by terms such as "aim," "anticipate," "approach," "belief", "believe," "contemplate," "could," "design," "designed," "estimate," "expect," "goal," "intend," "look," "may," "mission," "plan," "possible," "potential," "predict," "project," "pursue," "should," "strive," "suggest," "target," "will," "would," or the negative thereof and similar words and expressions.

Forward-looking statements are based on management's current expectations, beliefs, and assumptions and on information currently available to us. These statements are neither promises nor guarantees, and involve a number of known and unknown risks, uncertainties and assumptions, and actual results may differ materially from those expressed or implied in the forward-looking statements due to various important factors, including, but not limited to, our ability to become profitable; our ability to procure sufficient funding to advance our programs; risks associated with our capital requirements, anticipated cash runway, requirements under our current debt instruments and effects of restrictions thereunder, including our ability to raise additional capital due to market conditions and/or our market capitalization; our operating expenses and our ability to predict what those expenses will be; our limited operating history; the progression and success of our programs and product candidates in which we expend our resources; our limited ability or inability to assess the safety and efficacy of our product candidates; the risk that other genome-editing technologies may provide significant advantages over our ARCUS technology; our dependence on our ARCUS technology; the initiation, cost, timing, progress, achievement of milestones and results of research and development activities and preclinical and clinical studies, including clinical trial and investigational new drug applications; public perception about genome editing technology and its applications; competition in the genome editing, biopharmaceutical, and biotechnology fields; our or our collaborators' or other licensees' ability to identify, develop and commercialize product candidates; pending and potential product liability lawsuits and penalties against us or our collaborators or other licensees related to our technology and our product candidates; the U.S. and foreign regulatory landscape applicable to our and our collaborators' or other licensees' development of product candidates; our or our collaborators' or other licensees' ability to advance product candidates into, and successfully design, implement and complete, clinical trials; potential manufacturing problems associated with the development or commercialization of any of our product candidates; delays or difficulties in our and our collaborators' and other licensees' ability to enroll patients; results of preclinical studies and early clinical trials of product candidates may not be predictive of results of later studies or trials; changes in interim "top-line" and initial data that we announce or publish; if our product candidates do not work as intended or cause undesirable side effects; risks associated with applicable healthcare, data protection, privacy and security regulations and our compliance therewith; effects of system failures, cyberattacks, and security breaches; our ability to obtain orphan drug designation, fast track designation, rare pediatric disease designation, or a priority review voucher for our product candidates, or to realize the expected benefits of these designations; our or our licensees' ability to obtain orphan drug designation or fast track designation for our product candidates or to realize the expected benefits of these designations; our or our collaborators' or other licensees' ability to obtain and maintain regulatory approval of our product candidates, and any related restrictions, limitations and/or warnings in the label of an approved product candidate; the rate and degree of market acceptance of any of our product candidates; our ability to effectively manage the growth of our operations; our ability to attract, retain, and motivate executives and personnel; effects of system failures and security breaches; insurance expenses and exposure to uninsured liabilities; effects of tax rules; the success of our existing collaboration and other license agreements, and our ability to enter into new collaboration arrangements; our current and future relationships with and reliance on third parties including suppliers and manufacturers; our ability to obtain and maintain intellectual property protection for our technology and any of our product candidates; potential litigation relating to infringement or misappropriation of intellectual property rights; effects of natural and manmade disasters, public health emergencies and other natural catastrophic events; effects of sustained inflation, supply chain disruptions and major central bank policy actions; market and economic conditions; risks related to ownership of our common stock, including fluctuations in our stock price; our ability to meet the requirements of and maintain listing of our common stock on Nasdaq or other public stock exchanges; and other important factors discussed under the caption "Risk Factors" in our Quarterly Report on Form 10-Q for the quarterly period ended March 31, 2026, as any such factors may be updated from time to time in our other filings with the SEC, which are accessible on the SEC's website at www.sec.gov and the Investors page of our website under SEC Filings at investor.precisionbiosciences.com.

All forward-looking statements speak only as of the date of this presentation and, except as required by applicable law, we have no obligation to update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise. Precision consults with various presentation speakers and compensates them for their time and expertise.

Duchenne Muscular Dystrophy is a Progressive Neuromuscular Disorder Leading to Early Mortality



DMD affects roughly **1 in 3500** male births worldwide

By age 5, **muscle strength, stamina, and walking ability** begin to **decline**

Boys typically **lose ability to walk** independently by **age 10-13**

In early 20s, **severe heart and lung complications** are a leading cause of **mortality**

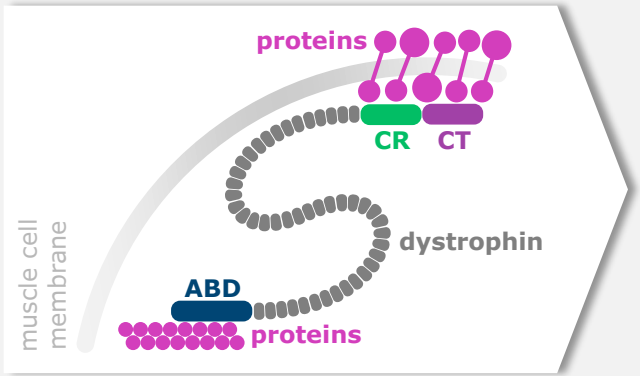
DMD, Duchenne muscular dystrophy.

Duan D, Goemans N, Takeda S, et al. Duchenne muscular dystrophy. *Nat Rev Dis Primers.* 2021;7:13. doi:10.1038/s41572-021-00248-3.

DMD Is Caused by Mutations in the Dystrophin Gene That Prevent Production of Dystrophin Protein Resulting in Muscle Degeneration

Healthy

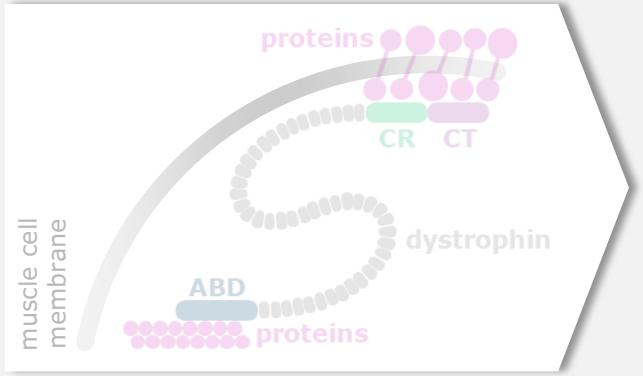
Dystrophin protein is necessary for muscle maintenance and repair following injury

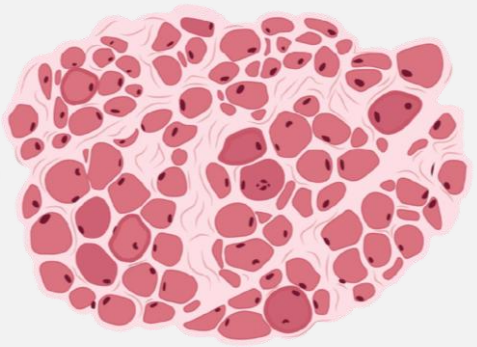


Healthy muscle

DMD

DMD is caused by mutations in the dystrophin gene that prevent the production of functional dystrophin protein





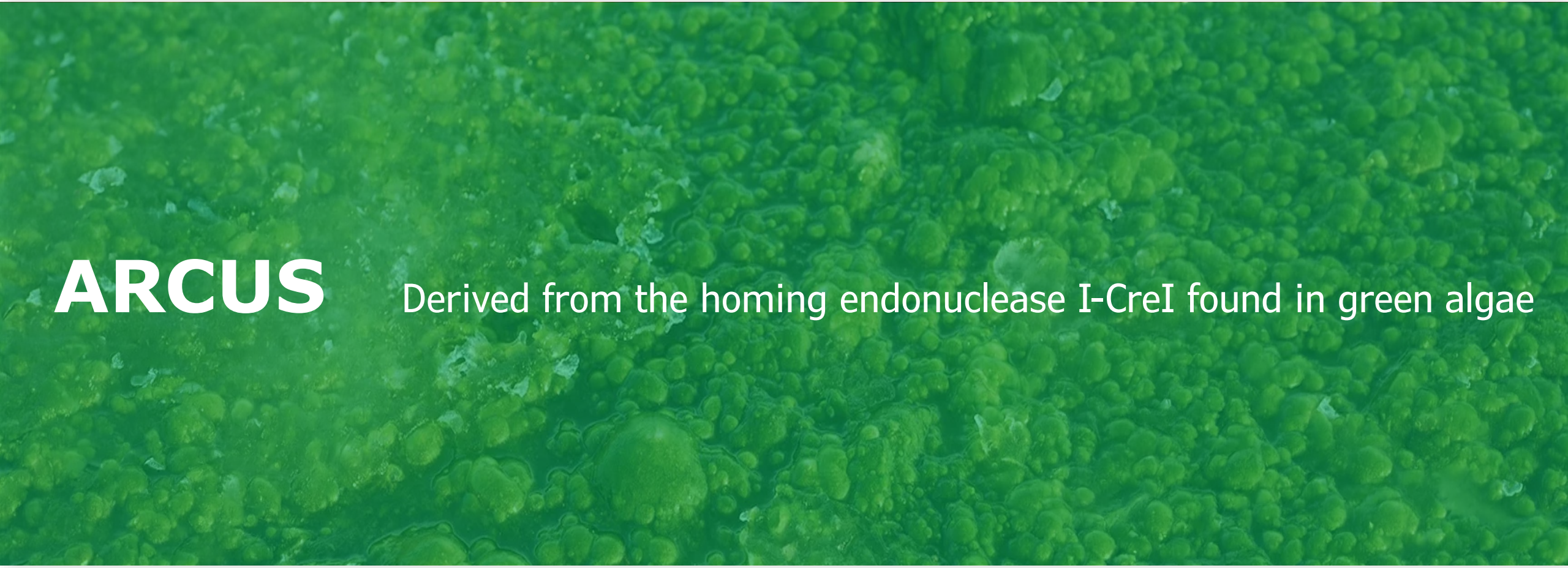
Degenerating muscle

Lack of dystrophin protein triggers progressive loss of muscle integrity and function
Despite advancements in DMD therapies, there remains an unmet need for novel therapies

DMD, Duchenne muscular dystrophy.

Duan D, Goemans N, Takeda S, et al. Duchenne muscular dystrophy. *Nat Rev Dis Primers.* 2021;7:13. doi:10.1038/s41572-021-00248-3.

ARCUS Is Our Proprietary Gene-Editing Platform: Naturally Evolved to Drive High-Efficiency Editing

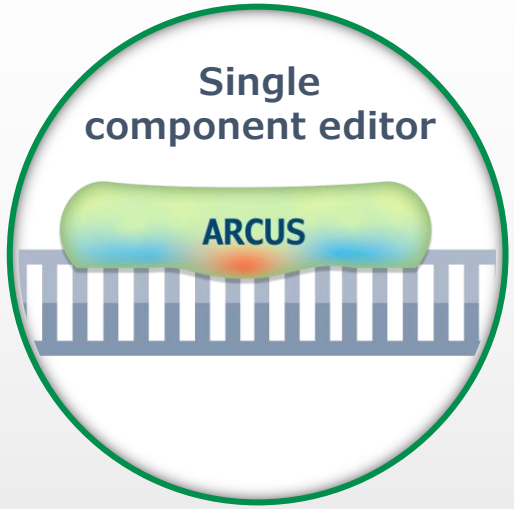


ARCUS Is Our Proprietary Gene-Editing Platform: Naturally Evolved to Drive High-Efficiency Editing

ARCUS Derived from the homing endonuclease I-CreI found in green algae

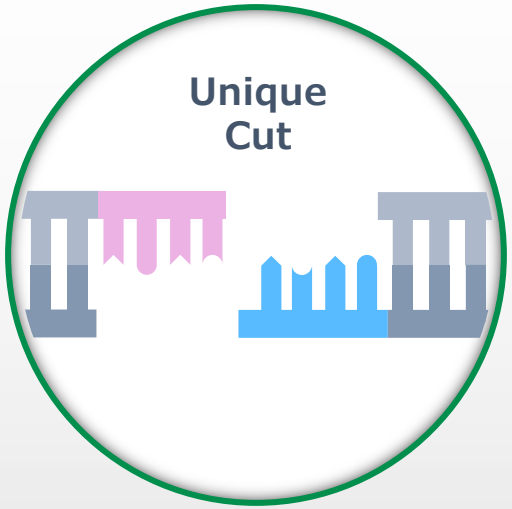
Safety and Efficacy

Two nucleases fit within a single AAV, enabling lower doses and higher efficiency



Efficacy

Iterative protein engineering enabled co-evolution of two ARCUS nucleases, facilitating efficient excision



Safety and Efficacy

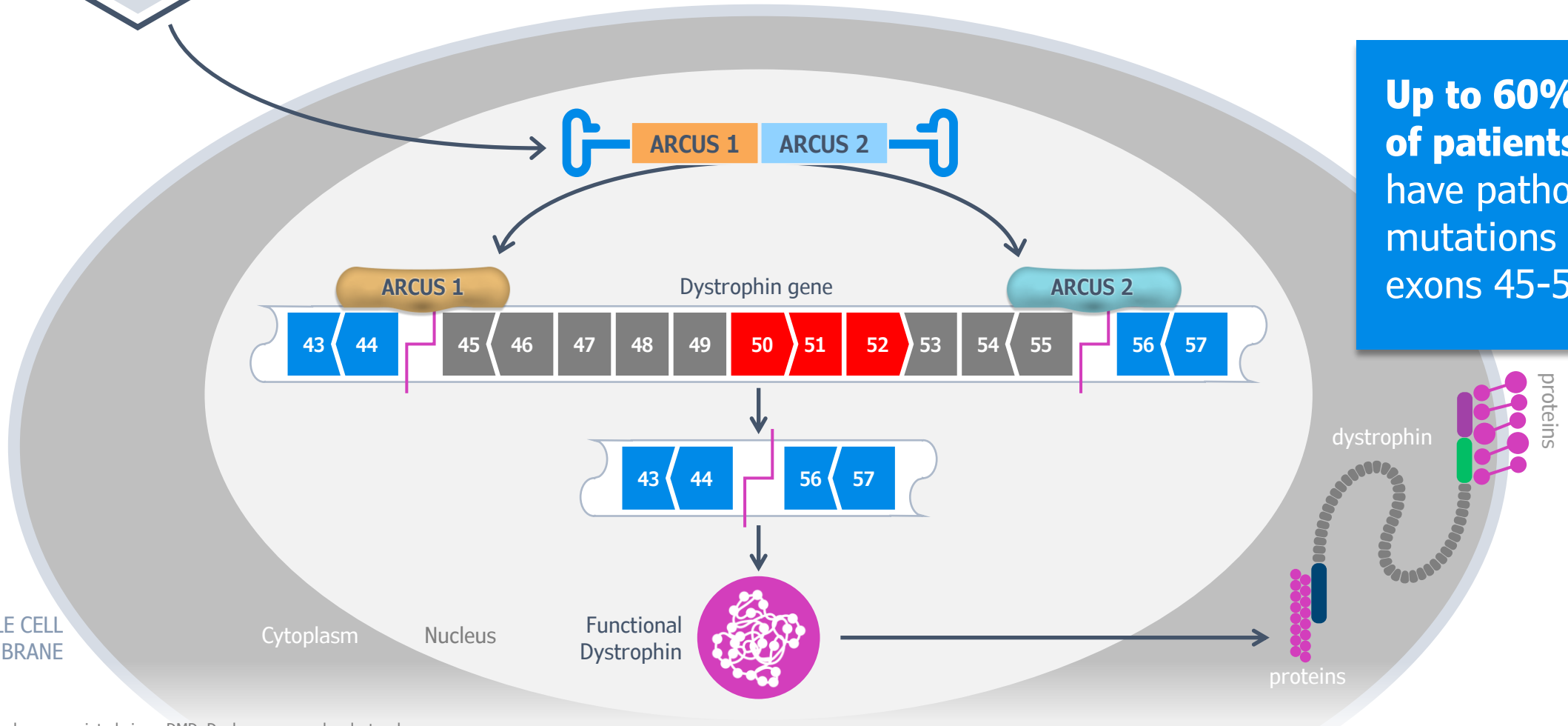
Unique cut allows for superior characterization of specificity and high efficiency of excision

These unique features of ARCUS are fundamental to PBGENE-DMD

AAV, adeno-associated virus.

PBGENE-DMD Designed to Provide Durable Functional Improvement for 60% of Patients With DMD

PBGENE-DMD: A single AAV encodes two ARCUS proteins designed to permanently excise exons 45-55 of the dystrophin gene, resulting in naturally expressed, functional dystrophin



Up to 60% of patients have pathogenic mutations in exons 45-55[1]

AAV, adeno-associated virus; DMD, Duchenne muscular dystrophy.

1. Beroud C, Tuffery-Giraud S, Matsuo S, et al. *Hum Mutat.* 2007;28(2):196-202. doi:10.1002/humu.20428.

PBGENE-DMD Designed to Produce a Near-Full–Length Dystrophin Protein, Demonstrated to Be Functional in Humans

Full-length Dystrophin

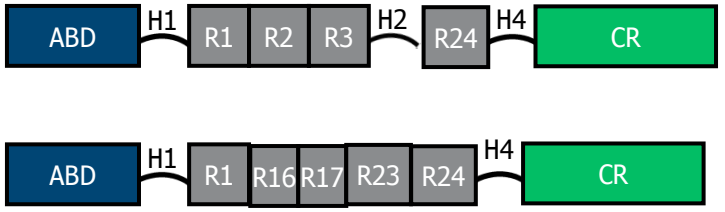
Full-length Dystrophin

Functional Dystrophin

PBGENE-DMD Dystrophin Gene Excision

~80%
of full-length dystrophin

Truncated Synthetic Microdystrophins



Synthetic Microdystrophins

~34%
of full-length dystrophin

> "The truncated dystrophin apparently does not function quite as well as the full-length gene, even when overexpressed…"
> — Phelps, et a"

Expected that **≥5% expression of functional dystrophin** protein is needed for therapeutic benefit in DMD patients

ABD, actin-binding domain; CR, cysteine-rich domain; CT, C-terminal domain; DMD, Duchenne muscular dystrophy.

Phelps SF, Hauser MA, Cole NM, et al. *Hum Mol Genet.* 1995;4(8):1251-1258. doi:10.1093/hmg/4.8.1251.

PBGENE-DMD: Long-Term Efficacy and Durability in Late Juvenile Mice

Functional & Durability Study Design – 9-Month Study

Study Design

- Disease mice are from a humanized DMD mouse model (hDMDdel52/mdx)
- Mice dosed at **3 weeks of age, late juvenile** (equivalent to target patient population of 4-7 years old)
- **Evaluated mice at 3 and 9 months** post PBGENE-DMD dosing

Groups

PBGENE-DMD ($1x10^{14}$ vg/kg) – Diseased mice

Untreated – Healthy mice

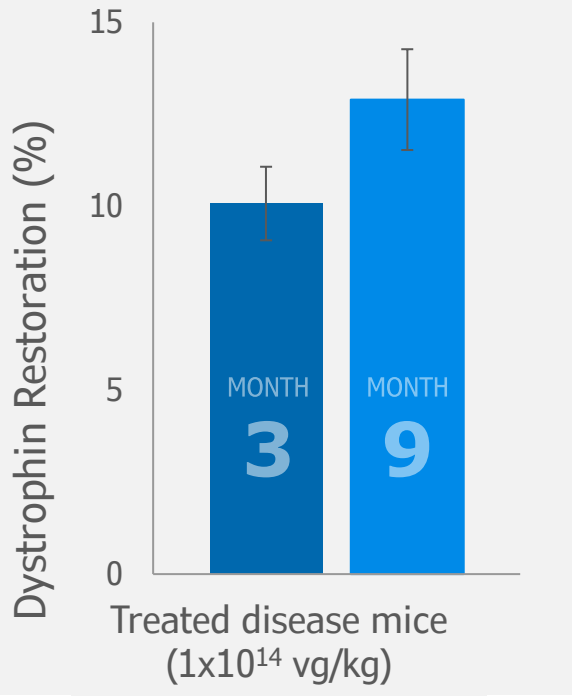
Untreated – Diseased mice

Key Readouts

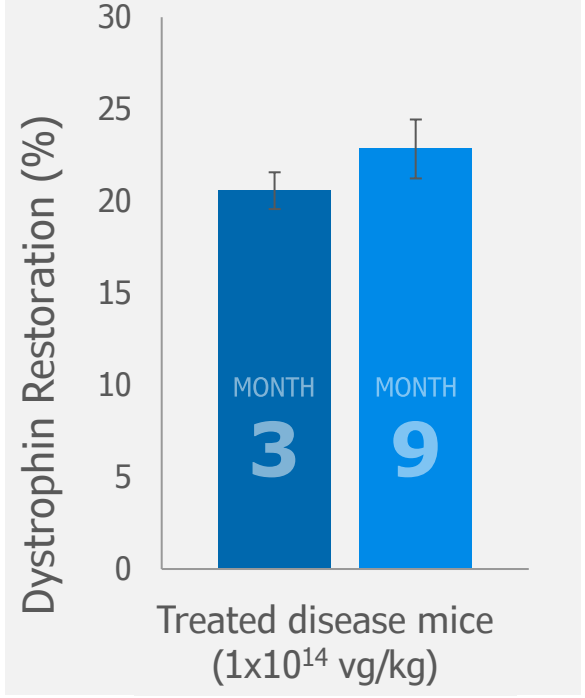
- All functional readouts were conducted in vivo through Myologica
- Dystrophin Protein Restoration (WES)
- Dystrophin Positive Fibers (IF)
- Muscle Force Output

PBGENE-DMD Resulted in Increased Dystrophin Protein Levels Over Time, Exceeding Expected Therapeutic Threshold

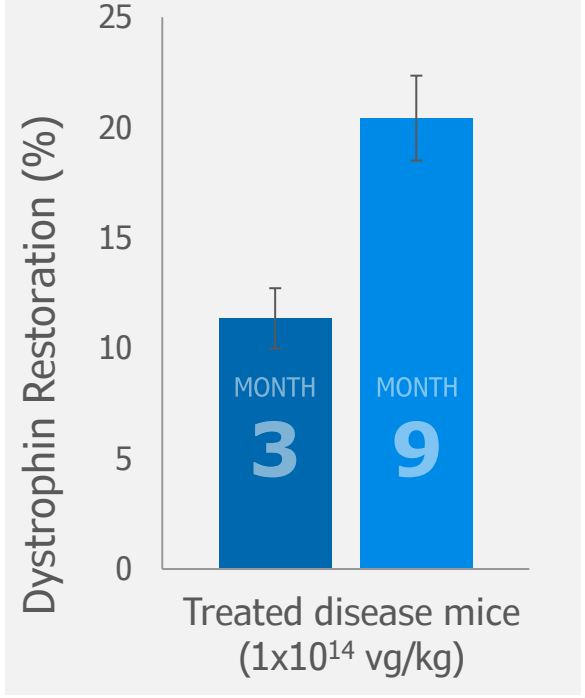
Heart



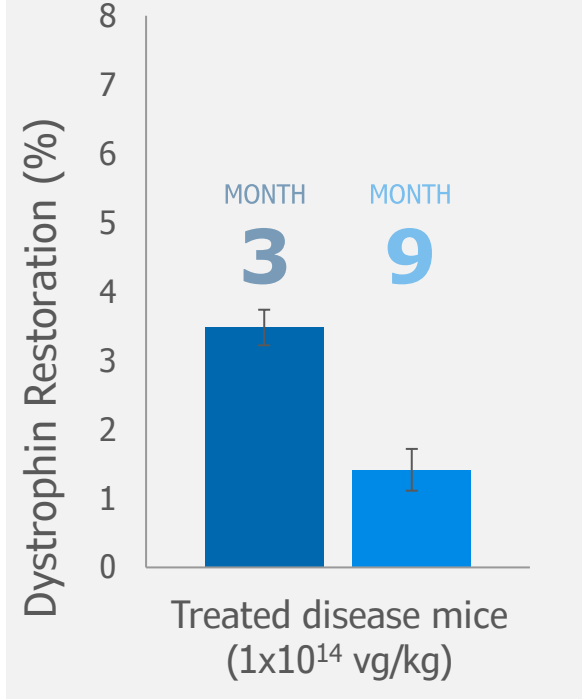
Calf



Quadriceps



Diaphragm

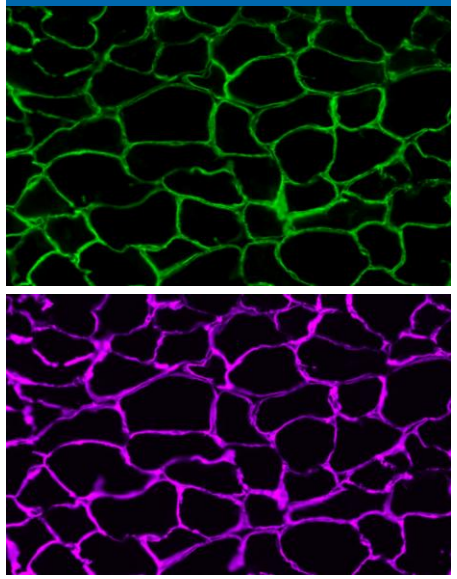


Endogenously produced, near-full–length functional dystrophin protein increases through 9 months in mice

DMD, Duchenne muscular dystrophy; vg/kg, vector genomes per kilogram.

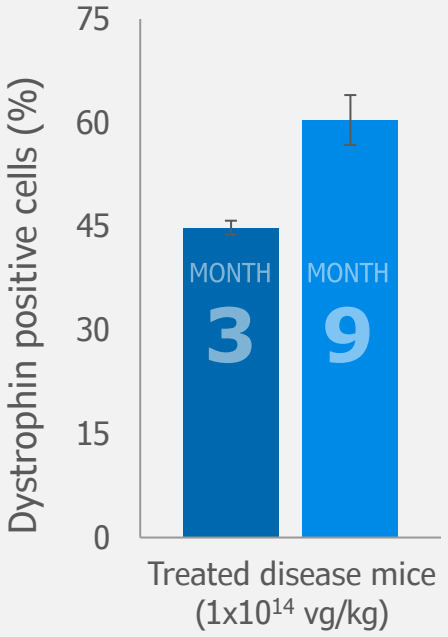
Broad Distribution of Dystrophin Positive Cells Across Skeletal, Cardiac, and Respiratory Muscles After PBGENE-DMD Treatment

PBGENE-DMD
Disease Model

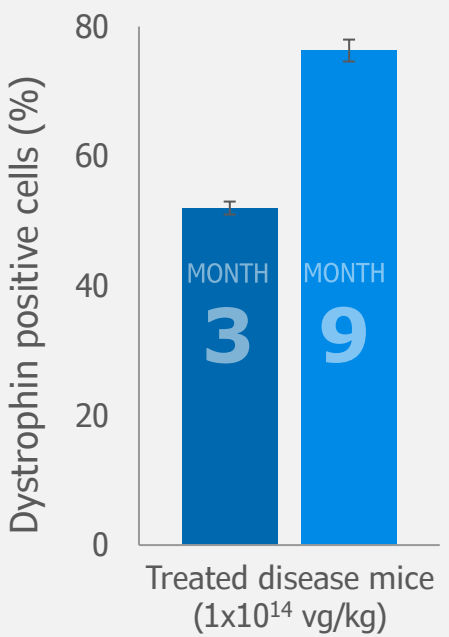


Dystrophin Laminin

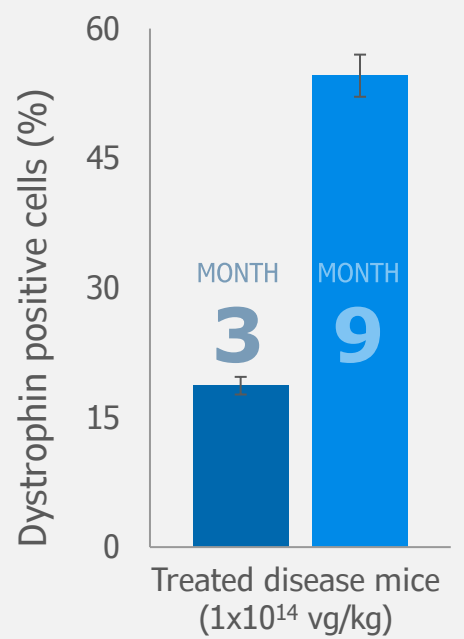
Heart



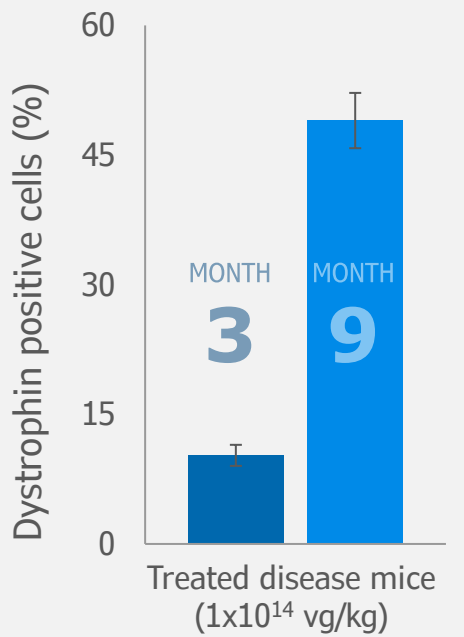
Calf



Quadriceps



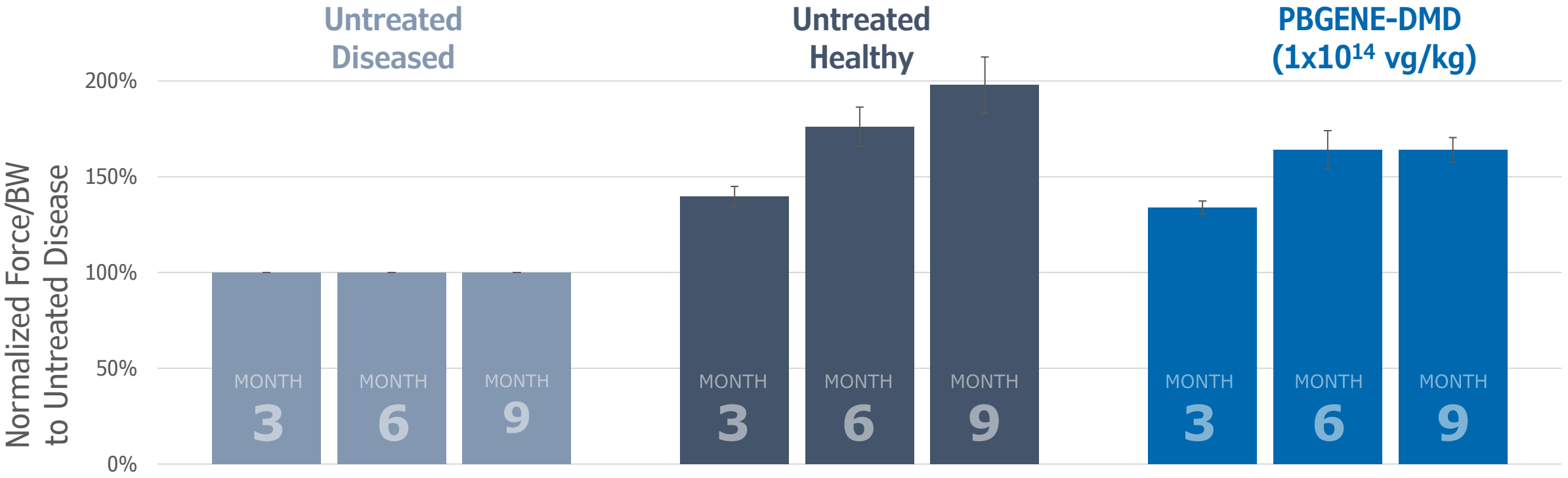
Diaphragm



Percentage of cells expressing near-full–length dystrophin protein increases over time after treatment with PBGENE-DMD

DMD, Duchenne muscular dystrophy; vg/kg, vector genomes per kilogram.

PBGENE-DMD Significantly Improved Muscle Function and Demonstrated Potential for Long-Term Durability



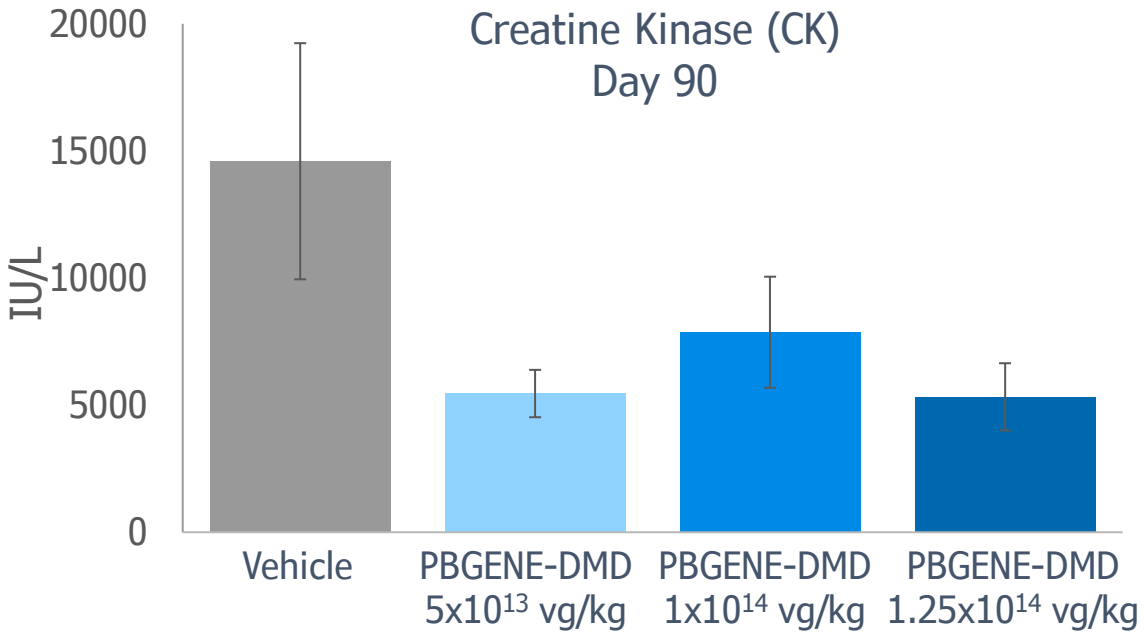
- Improved muscle function observed from 3 to 6 months
- Durable functional improvements maintained out to 9 months
- Significant improvement in resistance to eccentric injury

Force was measured in the calf across multiple stimulation frequencies. Averaged force normalized to body weight is shown. Statistically significant ($p<0.001$) increases in force were observed in both doses of PBGENE-DMD compared with untreated diseased animals at all three time points. N=10 mice per cohort.
DMD, Duchenne muscular dystrophy; vg/kg, vector genomes per kilogram.

PBGENE-DMD Reduces Injury Biomarkers and Improves Muscle Pathology

In a 90-day **late-juvenile** mouse GLP Tox/PK study, PBGENE-DMD was evaluated across multiple dose levels (5×10^{13}–1.25×10^{14} vg/kg), with assessments including clinical chemistry and histopathology.

Reduction in Serum Injury Biomarkers (CK) Following PBGENE-DMD Treatment



>45% decrease in CK across dose levels

PBGENE-DMD Improved Muscle Pathology After Treatment

	Vehicle		PBGENE-DMD ($1.25x10^{14}$ vg/kg)	
Day	30	90	30	90
Biceps Brachii	0.7	1.1	0.7	0.4
Diaphragm	1	1	0.8	0.9
Gastrocnemius	1.1	1.1	0.6	0.5
Quadriceps	1.3	1.3	0.9	0.8
Tibialis Anterior	0.4	0.6	0.3	0.3
Heart	0.4	0.1	0.1	0.1
Skeletal Muscle (total)	0.9	1	0.7	0.6

Histopathologic findings for each tissue were graded subjectively and semi-quantitatively by a pathologist on a scale 0–5: **0=unremarkable; **1**=minimal; **2**=mild; **3**=moderate; **4**=marked; **5**=severe.*

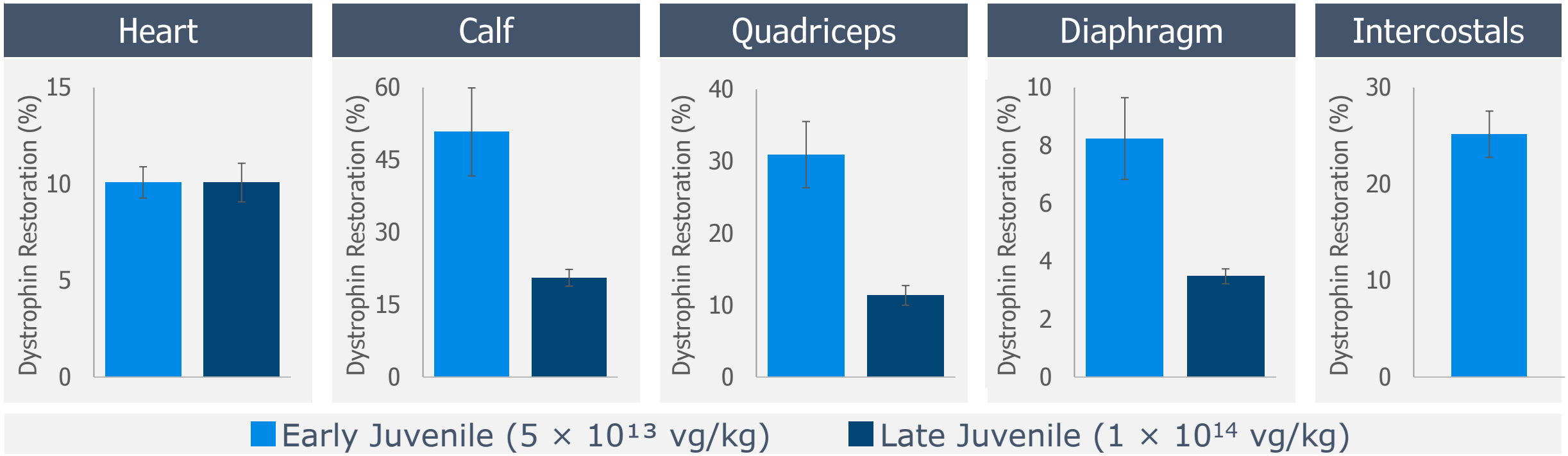
Mice dosed at 3-4 weeks old.
Mice are hDMDdel52/mdx.
CK, creating kinase; vg/kg, vector genomes per kilogram.

PBGENE-DMD Delivers Broad Dystrophin Restoration in Early Juvenile Mice

In a **3-month early juvenile (2-week-old)** mouse study, PBGENE-DMD (5×10^{13} vg/kg) demonstrated increased dystrophin protein restoration and dystrophin-positive fibers versus vehicle.

Dystrophin protein restoration in early juvenile mice was up to 2.5-12X higher in skeletal and respiratory muscle compared to late juvenile mice at 3 months

Strong efficacy in respiratory skeletal muscle

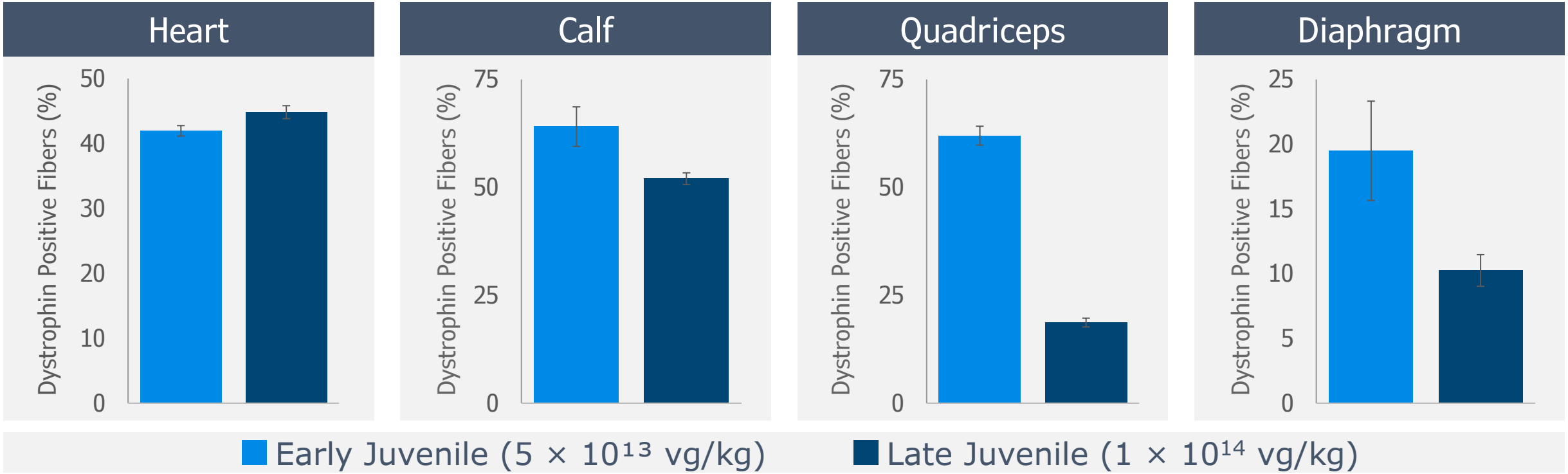


*12X higher dystrophin protein restoration observed in respiratory muscle was at equivalent dose levels (5×10^{13} vg/kg)

DMD, Duchenne muscular dystrophy; vg/kg, vector genomes per kilogram.

PBGENE-DMD Drives High Dystrophin-Positive Fiber Levels in Early Juvenile Mice

Dystrophin positive fibers in early juvenile mice were 2–3X higher in quadriceps and diaphragm compared to late juvenile mice at 3 months post PBGENE-DMD administration



Broad and robust efficacy in early juvenile mice supports potential benefit of earlier intervention of PBGENE-DMD in younger DMD patient population

DMD, Duchenne muscular dystrophy; vg/kg, vector genomes per kilogram.

Comprehensive Preclinical Data to Support PBGENE-DMD Safety and Efficacy

ARCUS promotes high frequency and fidelity excision for **predictable repair outcomes**

Durable muscle function improvements in DMD mice out to 9 months post dosing

Well tolerated for ≥9 months in both DMD diseased and healthy mice

Increased dystrophin protein and up to 85% dystrophin-positive myofibers over time in DMD mice

Reduced serum biomarkers in DMD disease mouse model after treatment

Strong efficacy in respiratory skeletal muscle in DMD mice

Signs of **improved muscle pathology** in DMD disease mouse model after treatment

Efficacy in early juvenile mice supports PBGENE-DMD treatment in younger patient population

Robust Preclinical
Safety & Efficacy
Data Package

DMD, Duchenne muscular dystrophy.

functionDMD

To connect with one of our study sites, please reach out to:
patientadvocacy@precisionbiosciences.com