AVIDITY® BIOSCIENCES

explore 44™

Investor & Analyst Event Series – Volume 10

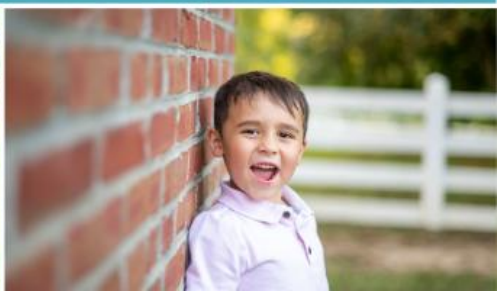
Transforming Duchenne Muscular Dystrophy (DMD)

AOC 1044 EXPLORE44™ Phase 1/2 Initial Data

August 9, 2024

NASDAQ: RNA | aviditybio.com

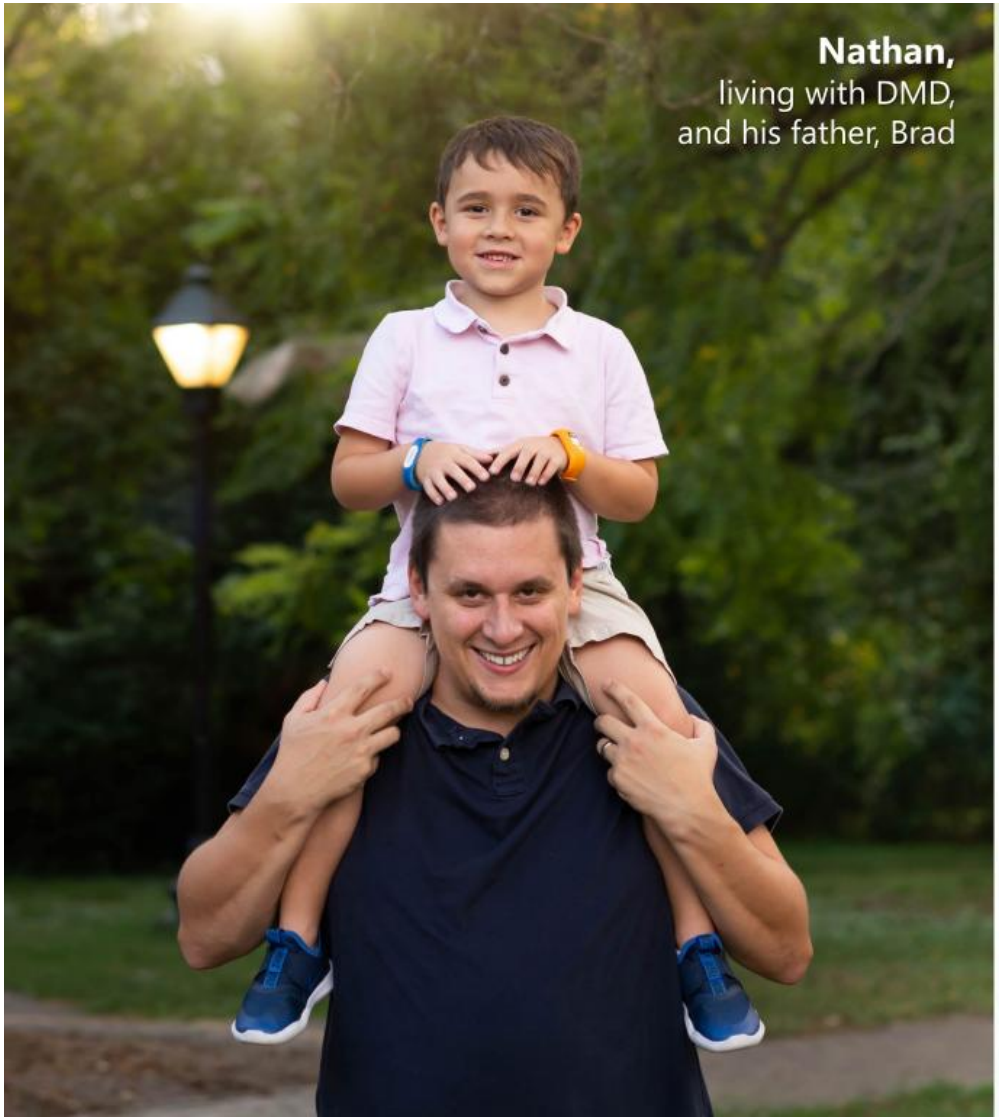


Forward-Looking Statements

We caution the reader that this presentation contains forward-looking statements that involve substantial risks and uncertainties. All statements other than statements of historical fact contained in this presentation are forward-looking statements. Forward-looking statements include, but are not limited to, statements regarding: our business strategy; the anticipated timing, costs, design, goals and conduct of our ongoing and planned preclinical studies and clinical trials; the timing of release of data from our ongoing clinical programs and the announcement of new programs; the timing of additional cohorts to existing clinical trials, including dosage levels and other details thereof; the characterization of data and results from preclinical studies and clinical trials, including the EXPLORE44 trial of delpacibart zotadirsen (*del-zota*, formerly AOC 1044), and conclusions drawn therefrom; research and development plans; plans and projected timelines for delpacibart etedesiran (*del-desiran*, formerly AOC 1001), delpacibart braxlosiran (*del-brax*, formerly AOC 1020) and *del-zota*; safety and tolerability profiles of our product candidates; efficacy data demonstrated by our product candidates; our plans regarding our DMD franchise; the potential of the AOC platform and specific product candidates; the status and potential of our product candidates as first-in-class and/or best-in-class; the ability of our product candidates to treat rare diseases; timing and likelihood of success; product approvals; plans and objectives of management for future operations; collaborations with third parties and expected benefits therefrom; the partial clinical hold related to *del-desiran*; and cash position and our ability to fund our planned operations. In some cases, the reader can identify forward-looking statements by terms such as "may," "will," "should," "expect," "plan," "anticipate," "could," "intend," "target," "project," "contemplates," "believes," "estimates," "predicts," "potential" or "continue" or the negative of these terms or other similar expressions. The inclusion of forward-looking statements should not be regarded as a representation by Avidity that any of our plans will be achieved. Actual results may differ from those set forth in this presentation due to the risks and uncertainties inherent in our business based on factors beyond our control, including, without limitation: we may not be able to fully resolve the partial clinical hold related to *del-desiran*; additional requests for data by the FDA or other regulatory authorities may result in significant additional expense and timing delays; data delivered to the FDA may not be satisfactory to the FDA; additional participant data related to our product candidates that continues to become available may be inconsistent with the data produced as of the most recent respective date cutoffs, and further analysis of existing data and analysis of new data may lead to conclusions different from those established as of such date cutoffs; unexpected adverse side effects or inadequate efficacy of our product candidates may delay or limit their development, regulatory approval and/or commercialization, or may result in additional clinical holds, recalls or product liability claims; we are early in our development efforts; our approach to the discovery and development of product candidates based on our AOC platform is unproven, and we do not know whether we will be able to develop any products of commercial value; the results of preclinical studies and early clinical trials are not necessarily predictive of future results; potential delays in the commencement, enrollment and completion of clinical trials, or of designations conferred by regulatory authorities; our dependence on third parties in connection with preclinical and clinical testing and product manufacturing; we may not realize the expected benefits of our collaborations with third parties, our existing collaborations may terminate earlier than expected or we may not be able to form new collaborations; regulatory developments in the United States and foreign countries, including acceptance of INDs and similar foreign regulatory submissions and our proposed design of future clinical trials; Fast Track or Breakthrough Designation by the FDA may not lead to a faster development or regulatory review or approval process; our ability to obtain and maintain intellectual property protection for our product candidates and proprietary technologies; we may exhaust our capital resources sooner than we expect and fail to raise additional needed funds; and other risks described under the heading "Risk Factors" in our Form 10-K for the year ended December 31, 2023, filed with the SEC on February 28, 2024, and in subsequent filings with the SEC. The reader is cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise. All forward-looking statements are qualified in their entirety by this cautionary statement, which is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995.

This presentation also contains estimates and other statistical data made by independent parties and by us relating to market size and growth and other data about our industry. This data involves a number of assumptions and limitations, and the reader is cautioned not to give undue weight to such estimates. In addition, projections, assumptions, and estimates of our future performance and the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk. These and other factors could cause results to differ materially from those expressed in the estimates made by the independent parties and by us. This presentation shall not constitute an offer to sell or the solicitation of an offer to buy securities, nor shall there be any sale of securities in any state or jurisdiction in which such offer, solicitation or sale would be unlawful prior to registration or qualification under the securities laws of any such state or jurisdiction.



OUR VISION

To profoundly improve people's lives by revolutionizing the delivery of RNA therapeutics

Purpose of Update

Present initial data from Phase 1/2 EXPLORE44
Del-zota 5mg/kg at 4-month timepoint

- Unsurpassed delivery to muscle
- Statistically significant robust exon skipping
- Statistically significant dystrophin production
- Profound reduction in creatine kinase
- Favorable safety and tolerability

What's next for *del-zota*

- Regulatory interactions on expediting path to accelerated approval

Global DMD Franchise – beyond DMD44

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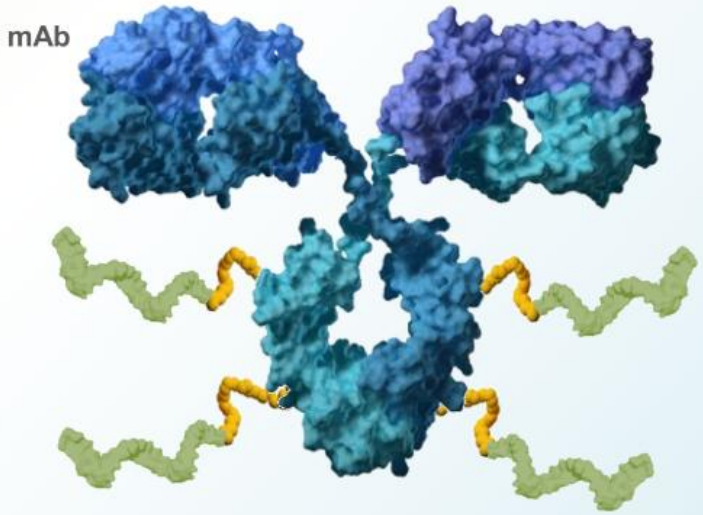
delpacibart zotadirsen
abbreviation: *del-zota*
(formerly known as AOC 1044)



Del-zota: Potential New Treatment for People Living with DMD44

Unsurpassed delivery; significant increase of 25% of normal in dystrophin production

Unsurpassed Delivery to Muscle

- Consistent delivery of PMO of 200 nM in skeletal muscle

Significant Exon Skipping

- Robust 37% increase in exon skipping
- Up to 66% increase in exon skipping

Significant Dystrophin Production

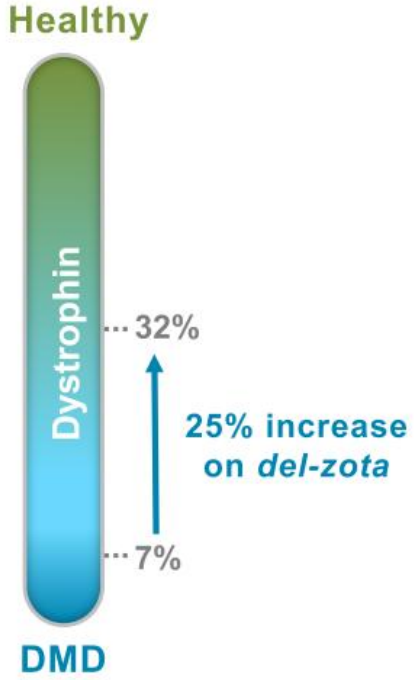
- Substantial increase of 25% of normal in dystrophin production
- Restored total dystrophin up to 54% of normal

Profound Reduction in Creatine Kinase Biomarker

- Creatine kinase levels were reduced to near normal with greater than 80% reduction compared to baseline

Favorable Safety and Tolerability

Safety data from EXPLORE44™ as of 25 July 2024



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Chief Scientific & Technical Officer

Michael MacLean
Chief Financial & Business Officer

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Transforming the Treatment of DMD

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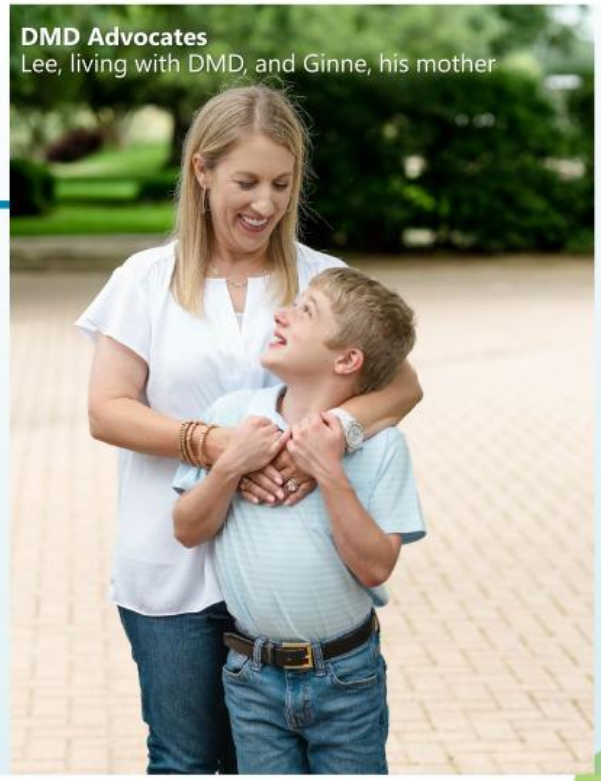
Transforming the Treatment of DMD44

DMD: Hereditary Disorder Causing Progressive, Debilitating Muscle Damage and Significantly Reduced Life Expectancy

~10,000 - 15,000
PEOPLE WITH DMD IN THE US
SIMILAR PREVALENCE IN EUROPE

~900
PEOPLE WITH DMD44 IN THE US

- Monogenic, X-linked, recessive condition characterized by progressive muscle damage and weakness
- Primarily affects males, loss of ambulation by teenage years
- Significantly reduces life expectancy
- Caused by mutations in the DMD gene, which encodes for the dystrophin protein
  - **~ 7%** of DMD skip-amenable patients have mutations amenable to exon 44 skipping (DMD44)
  - **~900** patients with DMD44 in US
- *Del-zota*: designed to specifically skip exon 44 of dystrophin gene to enable dystrophin production


DMD Advocates
Lee, living with DMD, and Ginne, his mother

EXPLORE44 Phase 1/2 DMD44 Cohorts: Overview & Objectives

Key Information

- Multiple dose
- N=24; Ages 7-27
- Ambulatory and non-ambulatory
- Biopsies in all cohorts
- Participants eligible to roll-over into extension

Primary Objective

- Safety and tolerability of multiple doses in DMD patients amenable to exon 44 skipping

Secondary Objectives

- Pharmacokinetics
- Pharmacodynamics
- Exon 44 skipping
- Dystrophin protein levels

Key Exploratory Objectives

- Measures of muscle function
- Patient-reported outcomes (PRO)
- Quality of life

Demographics and Baseline Characteristics

	Placebo N=3 mean (SD)	*Del-Zota* 5.0 mg/kg N=9 mean (SD)
Age (yrs)	13.7 (2.5)	16.0 (4.7)
BMI (kg/m^2)	23.1 (5.5)	24.2 (5.6)
Age of Symptom Onset (yrs)	3.8 (2.9)	3.6 (2.3)
Creatine Kinase, U/L	7,118 (1659)	5,033 (3246)
Corticosteroid Use	100%	100%

Del-zota: Favorable Safety and Tolerability in DMD44 Patients

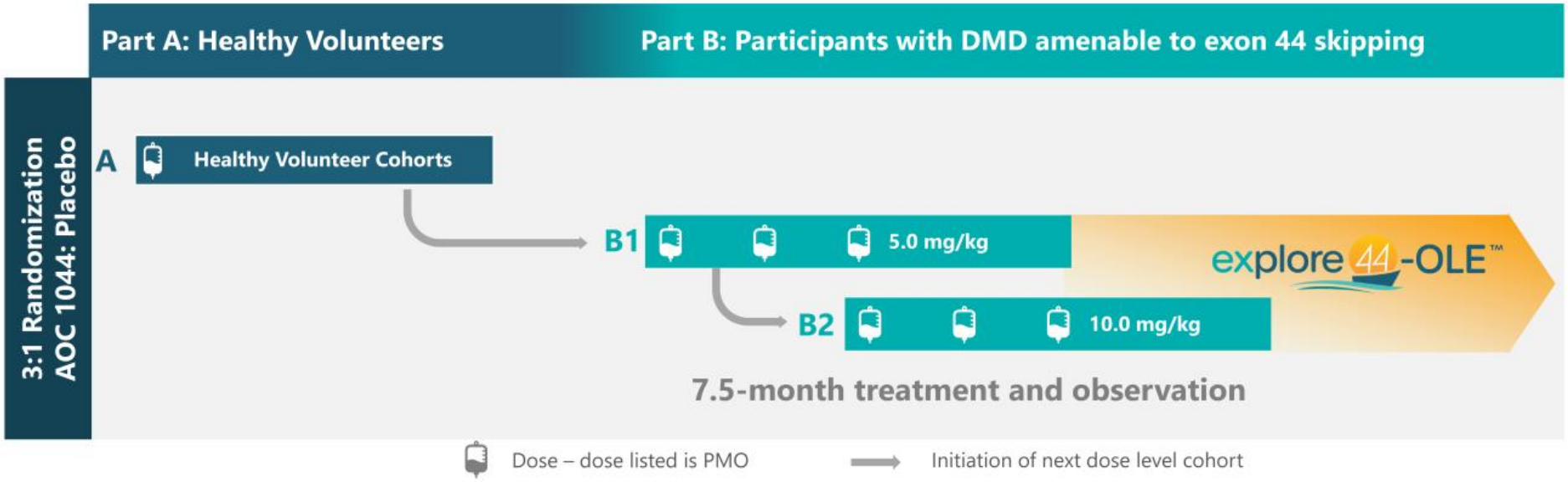
Subjects with ≥ 1 TEAE n (%)	Placebo N=7	5.0 mg/kg N=9	10 mg/kg N=9
Any AE	4 (57%)	8 (89%)	4 (44%)
Related to study drug	0	2 (22%)	1 (11%)
Serious AE (SAE)	0	1 (11%)	0
AE leading to study discontinuation	0	2 (22%)	0
AE leading to death	0	0	0

Data from EXPLORE44™ as of 25 July 2024

Most treatment emergent adverse events (TEAEs) were mild or moderate

- No related AE occurred in >1 patient
- 1 participant discontinued due to serious AE of anaphylaxis
- 1 participant discontinued due to moderate infusion related reaction
- No symptomatic hemoglobin changes, hypomagnesemia or renal events

Expediting *Del-zota* Registrational Plan



- EXPLORE44™ enrollment complete; dose-escalation to 20 mg/kg not necessary
- Plan to enroll additional patients in the EXPLORE44™ Open-Label Extension study
- Regulatory interactions to discuss the most expeditious path to accelerated approval

Del-zota: Initial Data from 5mg/kg Cohort

Del-zota: Transforming the Treatment of DMD44

Unsurpassed Delivery to Muscle	Statistically Significant Robust Exon Skipping	Statistically Significant Dystrophin Production	Profound Decrease in Creatine Kinase Biomarker
• Consistent delivery of PMO of 200 nM in skeletal muscle • Once again, reinforcing the disruptive and broad potential of our AOC platform	• 37% increase in exon 44 skipping • Up to 66% increase in exon 44 skipping	• Increase of 25% of normal in dystrophin production • Restored total dystrophin up to 54% of normal	• Creatine kinase levels reduced to near normal with greater than 80% reduction compared to baseline

Del-zota Produces Near Full-Length Dystrophin

Naturally occurring dystrophin

Representative dystrophin produced by *del-zota*

Micro-dystrophin produced by gene therapies

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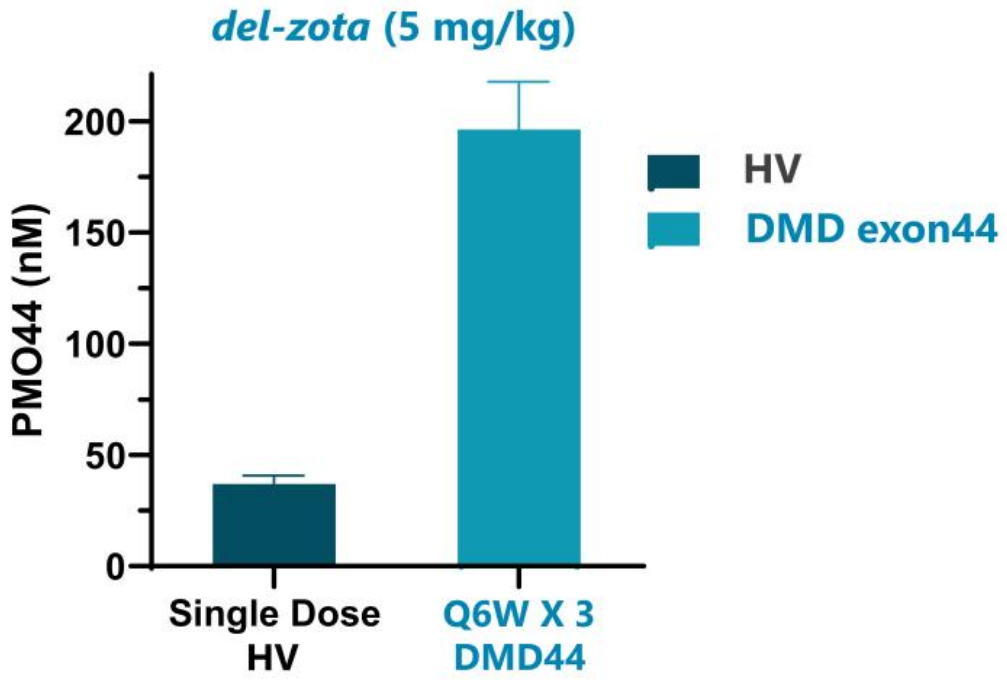
Del-zota: Unsurpassed Delivery to Muscle in DMD Participants

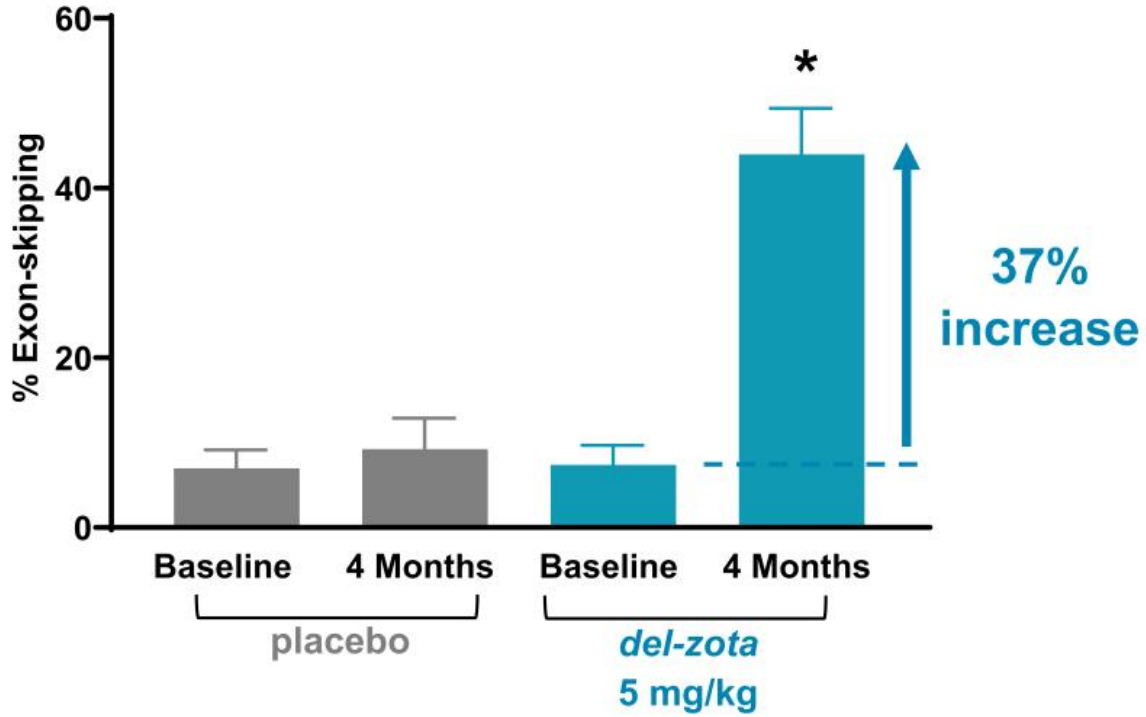
PMO tissue concentration of 200 nM after 3 doses



PMO44 muscle tissue concentrations were determined utilizing HPLC. Muscle biopsy collected from vastus lateralis (HV) or biceps brachii (DMD44) at 1 month post last dose. Mean +/- SEM. N=8 healthy volunteers and N=7 *del-zota*. Doses expressed as PMO component.

Del-zota: Increase of 37% in Exon 44 Skipping

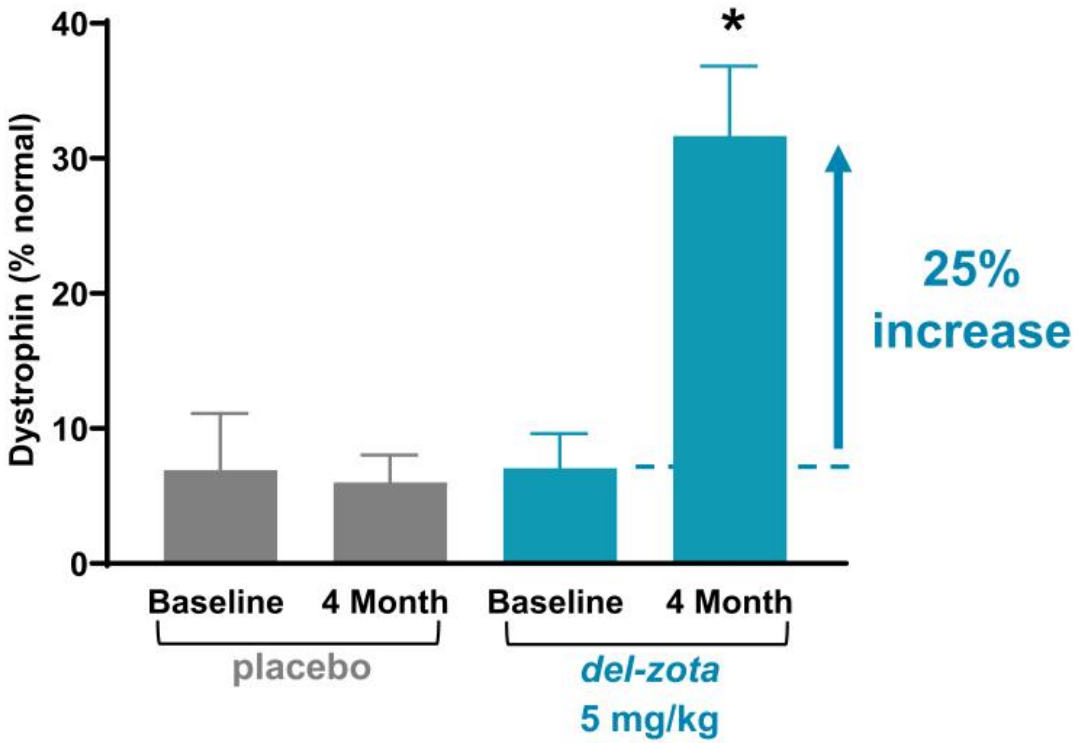
Statistically significant increase in exon 44 skipping of up to 66%

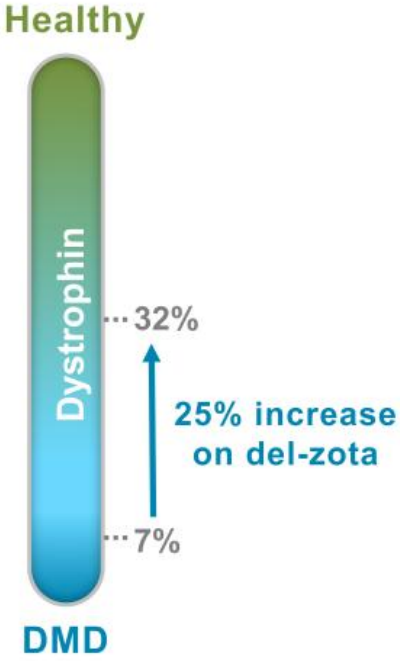


Muscle biopsy collected from biceps brachii at 1 month post last dose (q6W x 3). Mean +/- SEM.
N=3 placebo and N=7 *del-zota*. Exon 44 skipping determined using ddPCR. *p<0.05 by Wilcoxon test

Del-zota: Increase of 25% of Normal in Dystrophin Production

Significantly restored total dystrophin up to 54% of normal

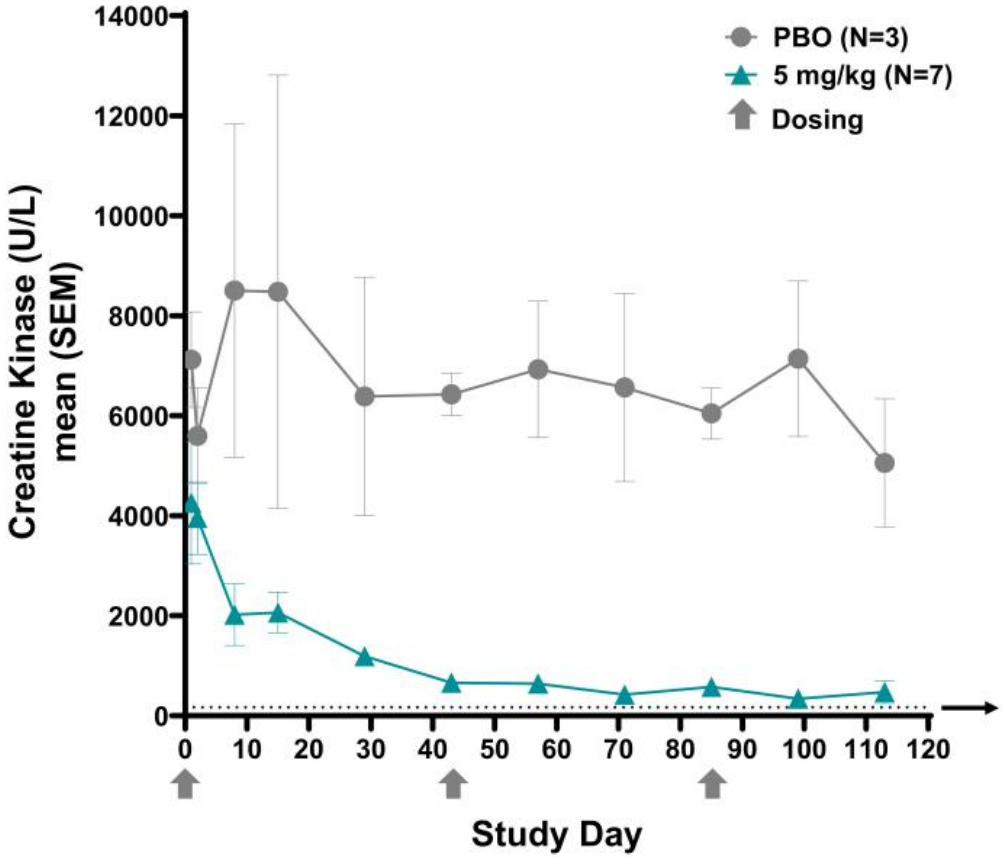




Dystrophin protein determined in biceps brachii muscle biopsy at 1 month post 3rd dose by Western Blot. Data normalized to myosin heavy chain. Mean +/- SEM. N=3 (Placebo), N=7 (*del-zota*). Dose expressed as PMO component. *p<0.05 by Wilcoxon test

Del-zota: Creatine Kinase Levels Decrease to Near Normal

Creatine kinase reduced by greater than 80% compared to baseline



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Del-zota: First of Multiple AOCs in DMD Franchise

Data support expediting advancement of additional exon-skipping candidates

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Revolutionizing the Delivery of RNA Therapeutics

Avidity is Committed to Partnering with the DMD Community to Efficiently Advance Treatment Options

We understand the responsibility to get treatments to patients as quickly as possible

- FDA has granted *del-zota* Orphan Drug, Fast Track and Rare Pediatric Disease designations. EMA has granted *del-zota* Orphan designation
- EXPLORE44™ enrollment complete; plan to enroll additional patients in the EXPLORE44™ open-label extension study
- Looking forward to regulatory interactions to discuss the most expeditious path to accelerated approval

OUR VISION

To profoundly improve people's lives by revolutionizing the delivery of RNA therapeutics

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Q&A

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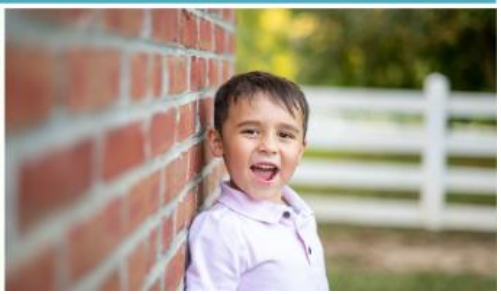

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