

Reshaping Clinician Perceptions of Disease Burden in Becker Muscular Dystrophy: The Impact of Incorporating Patient Perspectives in CME

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Introduction: Healthcare providers (HCPs) often perceive Becker muscular dystrophy (BMD) as milder compared to Duchenne muscular dystrophy (DMD). However, a growing understanding of its variable disease trajectory, along with emerging therapeutic evidence, emphasizes the need for greater recognition of its true burden. We hypothesized that incorporating patient perspectives into continuing medical education (CME) activities presents an opportunity to address misperceptions.

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METHODOLOGY

Educational Interventions:

Title: Becker Muscular Dystrophy: Hope with Emerging Therapeutics to Reduce Contraction-induced Injury

- 60-minute interactive panel discussion, available on-demand for one year
- Segments of a recorded interview with a person diagnosed with BMD were interspersed within the content to provide insights into quality of life, burden of disease, and experiences with HCPs
- Micro-learning segments, each tied to a learning objective, were disseminated to NPI-verified neurologists via LinkedIn



Data Collection and Analysis

- In-activity polling questions evaluated attitudes/beliefs related to BMD
- Pre- and post-activity questions measured knowledge/competence aligned with stated learning objectives, developed in accordance with National Board of Medical Examiner guidelines¹
- Post-activity evaluation questions evaluated intended practice changes
- Statistical tests of significance were applied to comparisons of individual questions (pre- vs post-activity)

Reference:

¹NBME Item-Writing Guide. Constructing written test questions for the health sciences. Nov 2020. www.nbme.org/item-writing-guide.

FACULTY



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Patient Participant



INSIGHTS INTO THE PATIENT EXPERIENCE

Daily Functioning and Loss of Ease

Things like getting ready in the morning probably take three times as long as they did ten years ago—just getting dressed and getting out the door. I'm still driving and able to use a mobility van, but I rely on a scooter or powered wheelchair, even though my lower legs and ankles are still fairly strong. Things that were easy ten or twenty years ago are very difficult now.

I used to like to golf when I was a kid and into my 20s, but I had to give that up. I do a lot of fishing, but I had to sell my boat because it became too much to manage and was hard for me to get in and out of. I've had to give up some of my hobbies over time, and you just learn to deal with what you can do today instead of focusing on what you can't do anymore.

Progressive Mobility Loss and Safety Concerns

About ten years ago, I started walking with a cane, mainly outside the house, because I had a few falls and my balance was off. I was still able to walk, but the cane helped with stability. Then, about seven years ago, I started using a mobility scooter outside the home because anything that required a lot of walking left me too fatigued.

Frustration with Limited Understanding of the Disease

Physical therapy has been a frustrating experience because they don't really understand the condition...one wanted me to start using a walker, which would have been the worst thing for my gait.

It's really helpful to talk with other people who have muscular dystrophy, because they're dealing with things that a lot of people don't understand. Most people just don't get what it's like day to day.

Emotional and Psychological Burden

After the diagnosis, I went into extreme denial. I felt like I was doing okay. I didn't tell anyone, even my own family, for quite a while...the long-term future was a big concern, and it scared me.

PARTICIPANT DEMOGRAPHICS

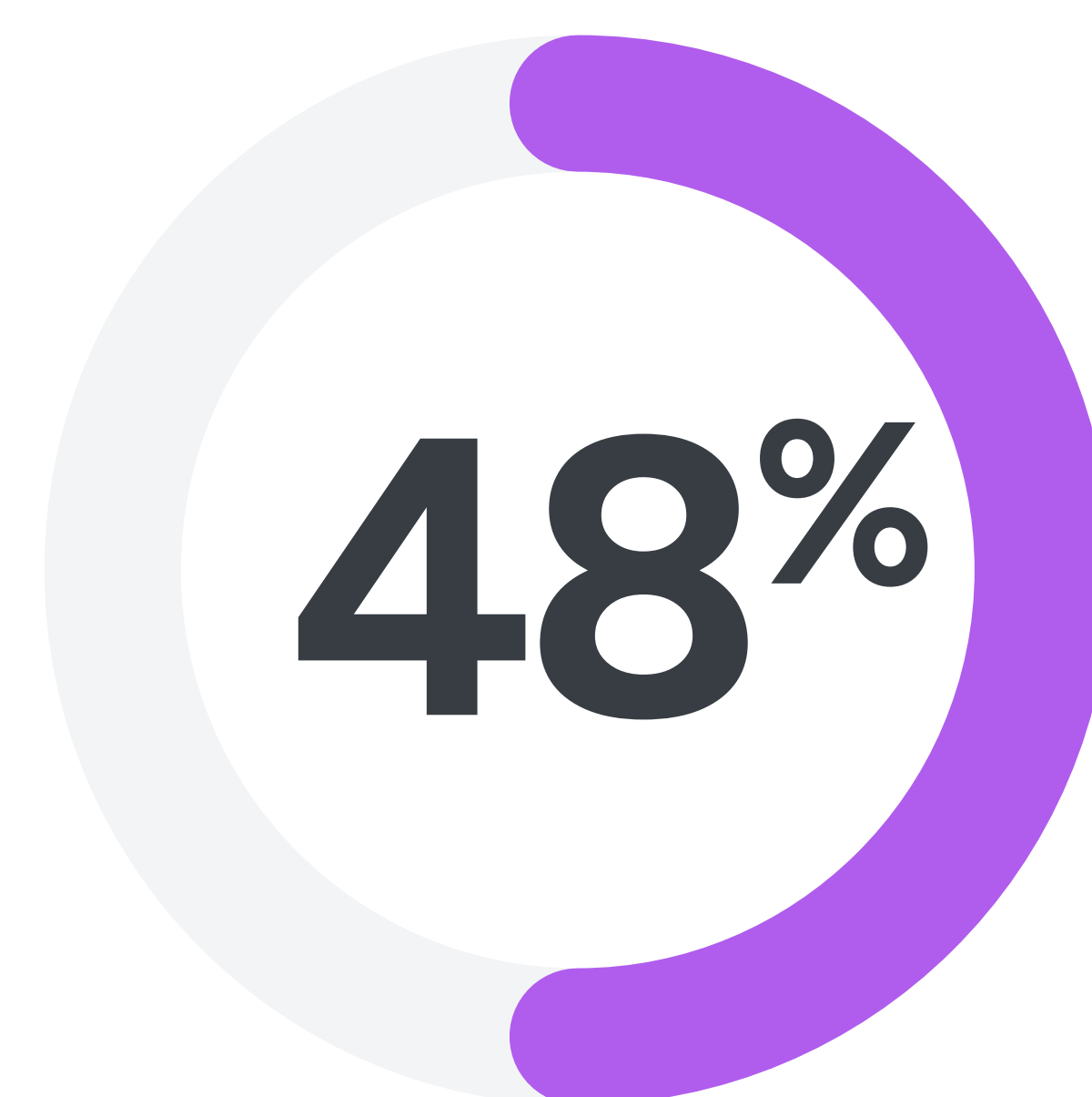
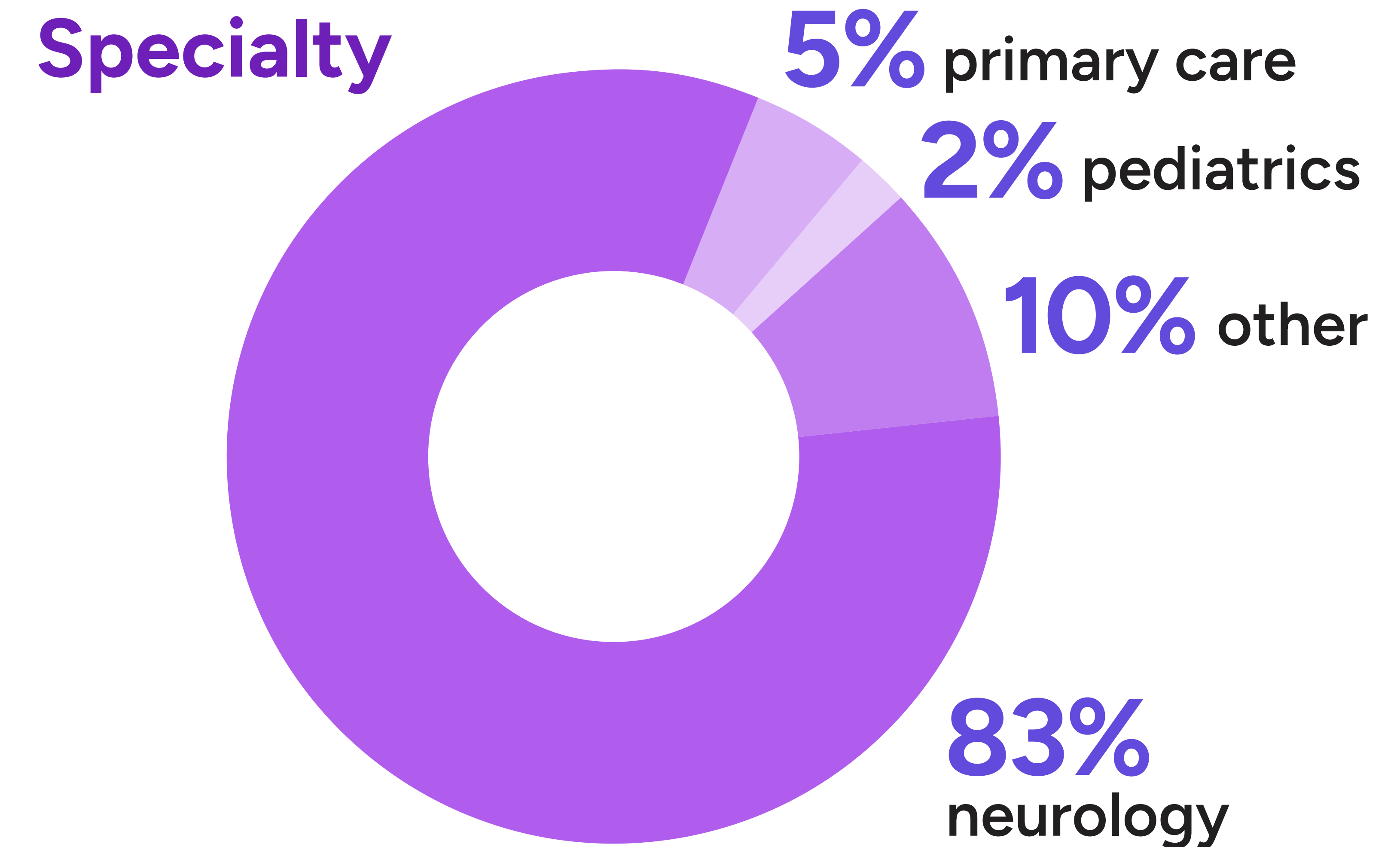
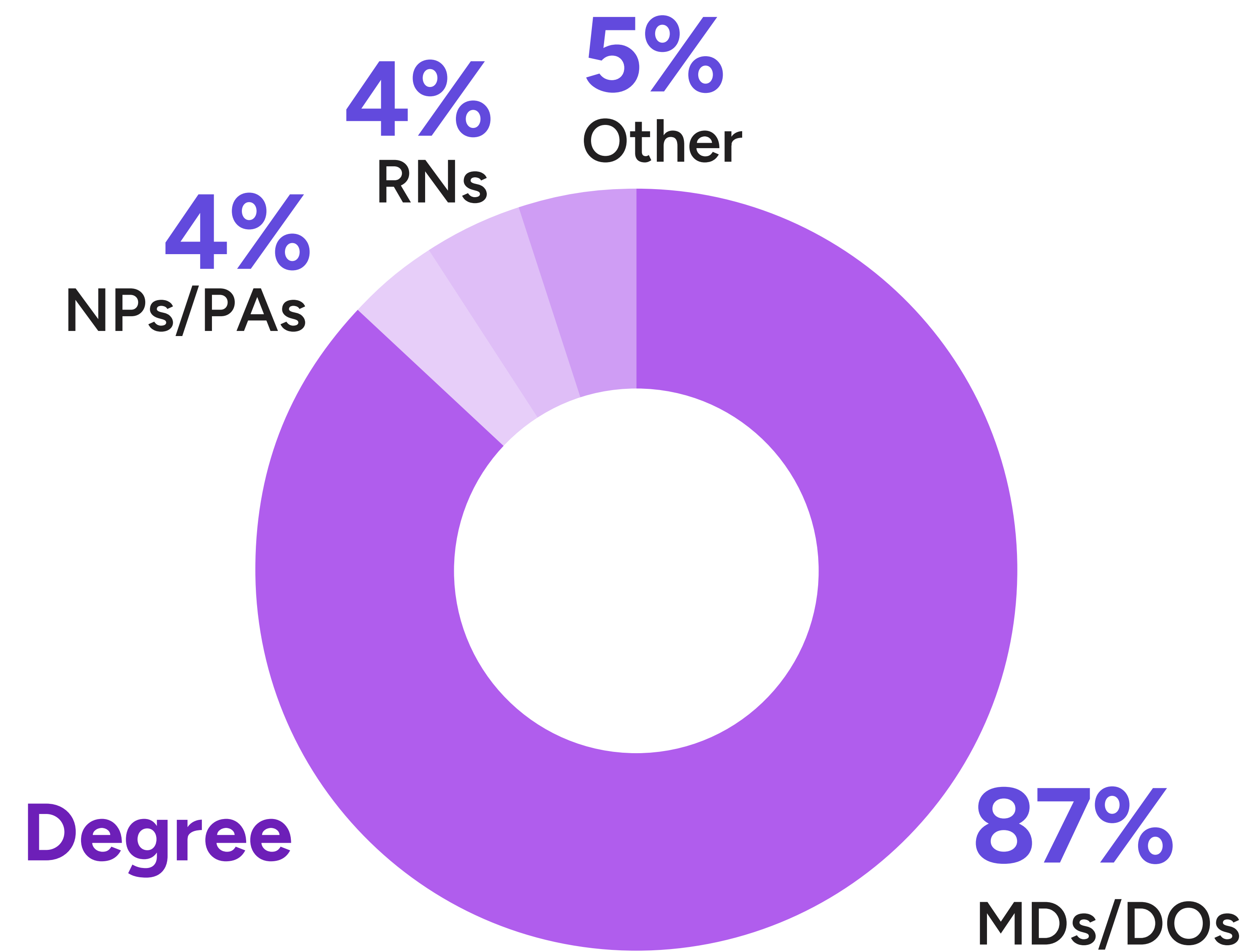


4,532
Participants

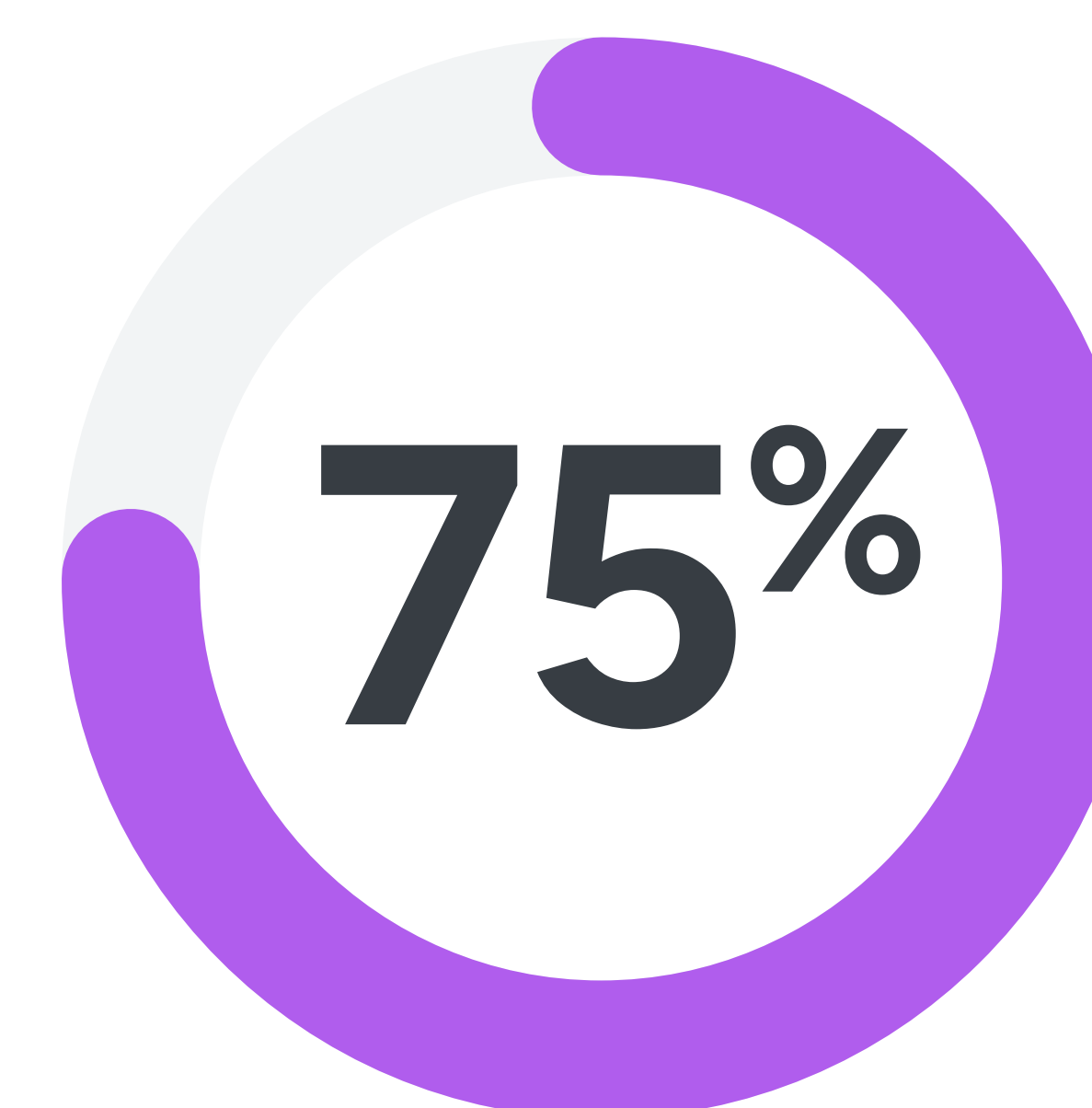
31 CME Live

914 live + enduring

3,618 micro-learning



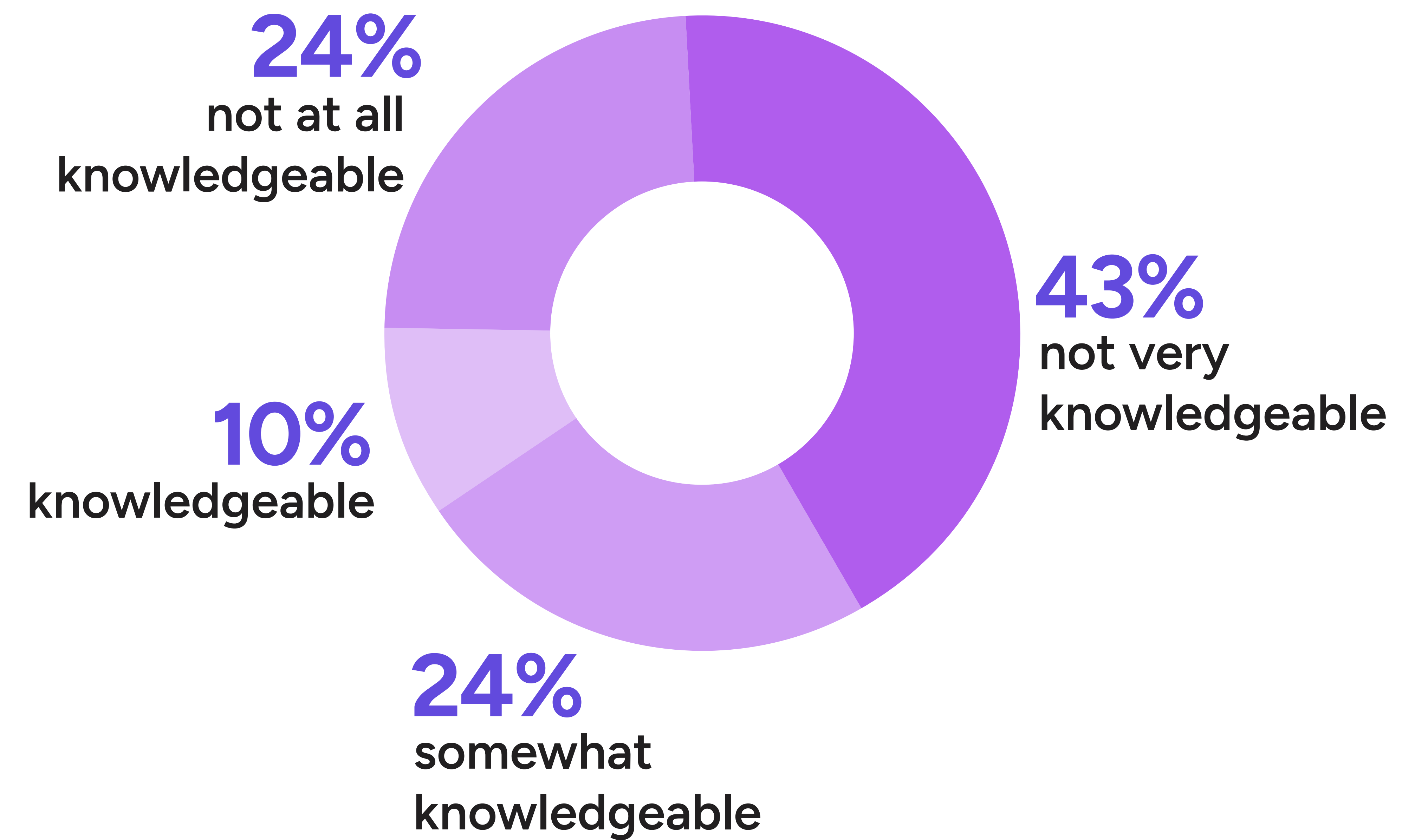
of CME learners
identified as
current treaters



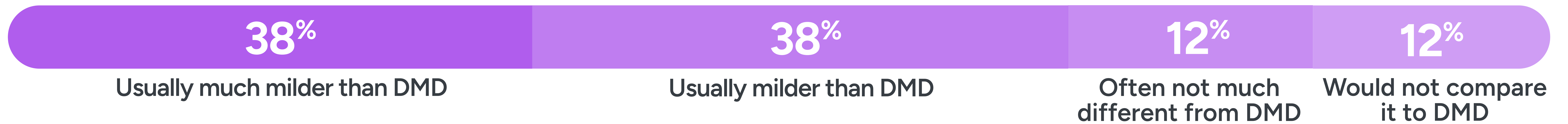
of current treaters
see 1 to 5 patients
with BMD each
week

RESULTS

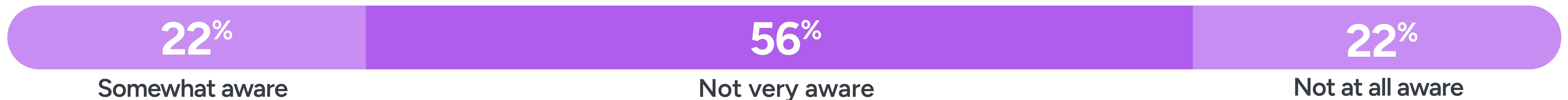
Knowledge of Cellular and Molecular Mechanisms Leading to Contraction-induced Injury in BMD (polling)



Perception of BMD (polling)



Awareness of Agents in Phase II or Phase III Clinical Trials for Treatment of BMD (polling)

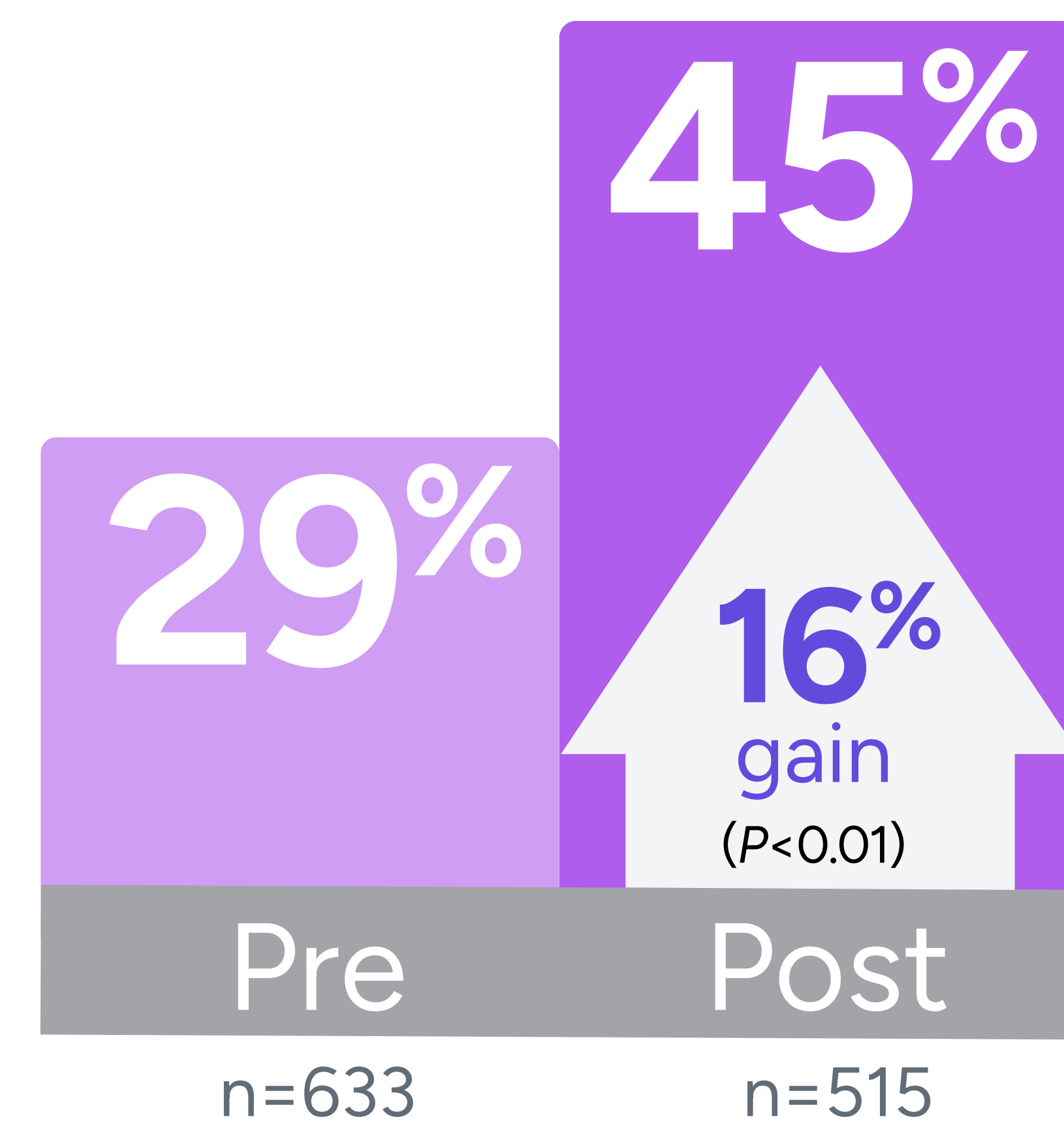


RESULTS

Learning gains:

Mechanisms underlying contraction-induced muscle injury

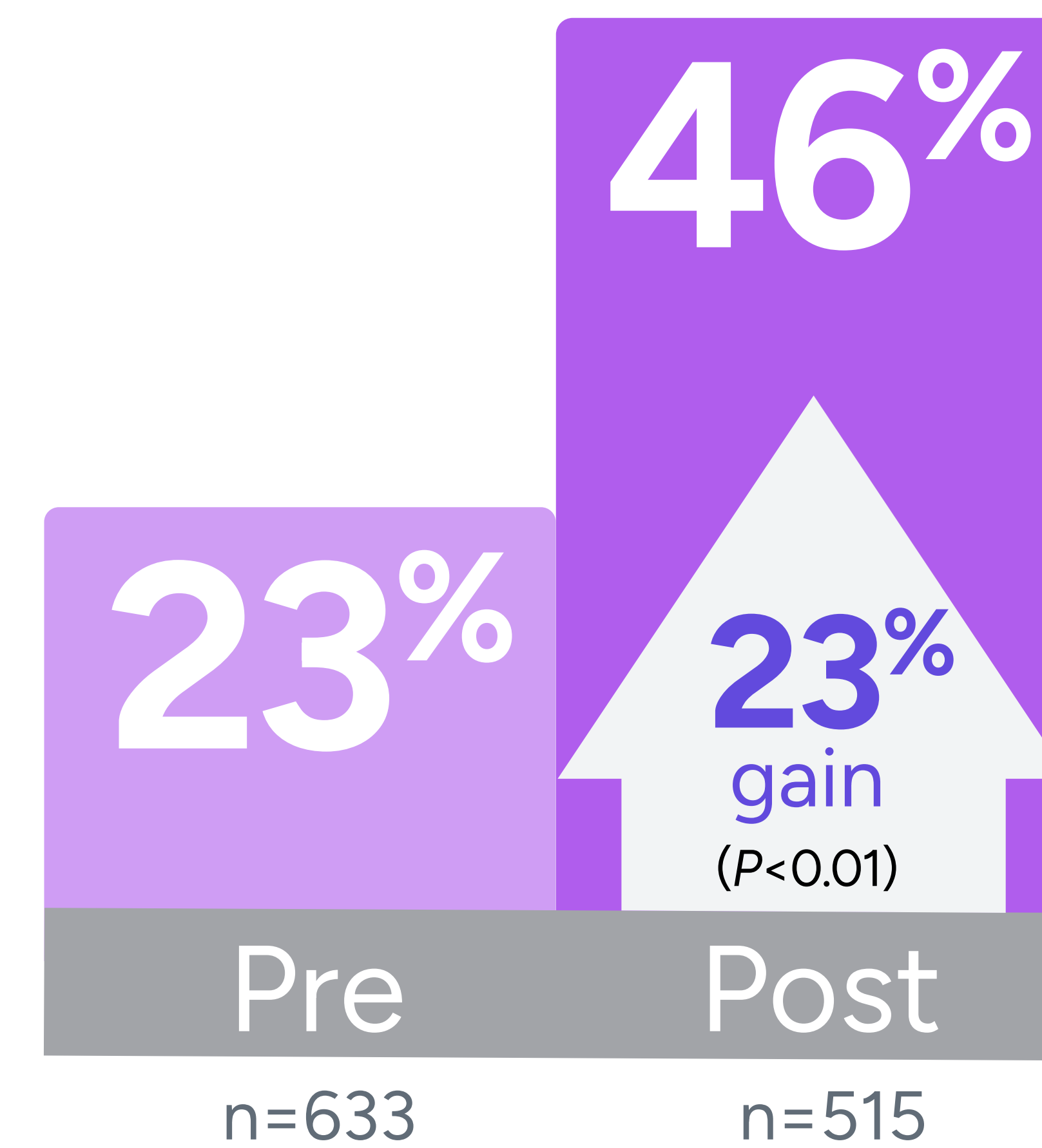
Which component of muscle is most susceptible to contraction-related damage in patients with BMD and DMD?



Correct answer:
Fast myofibril

Emerging agents for BMD

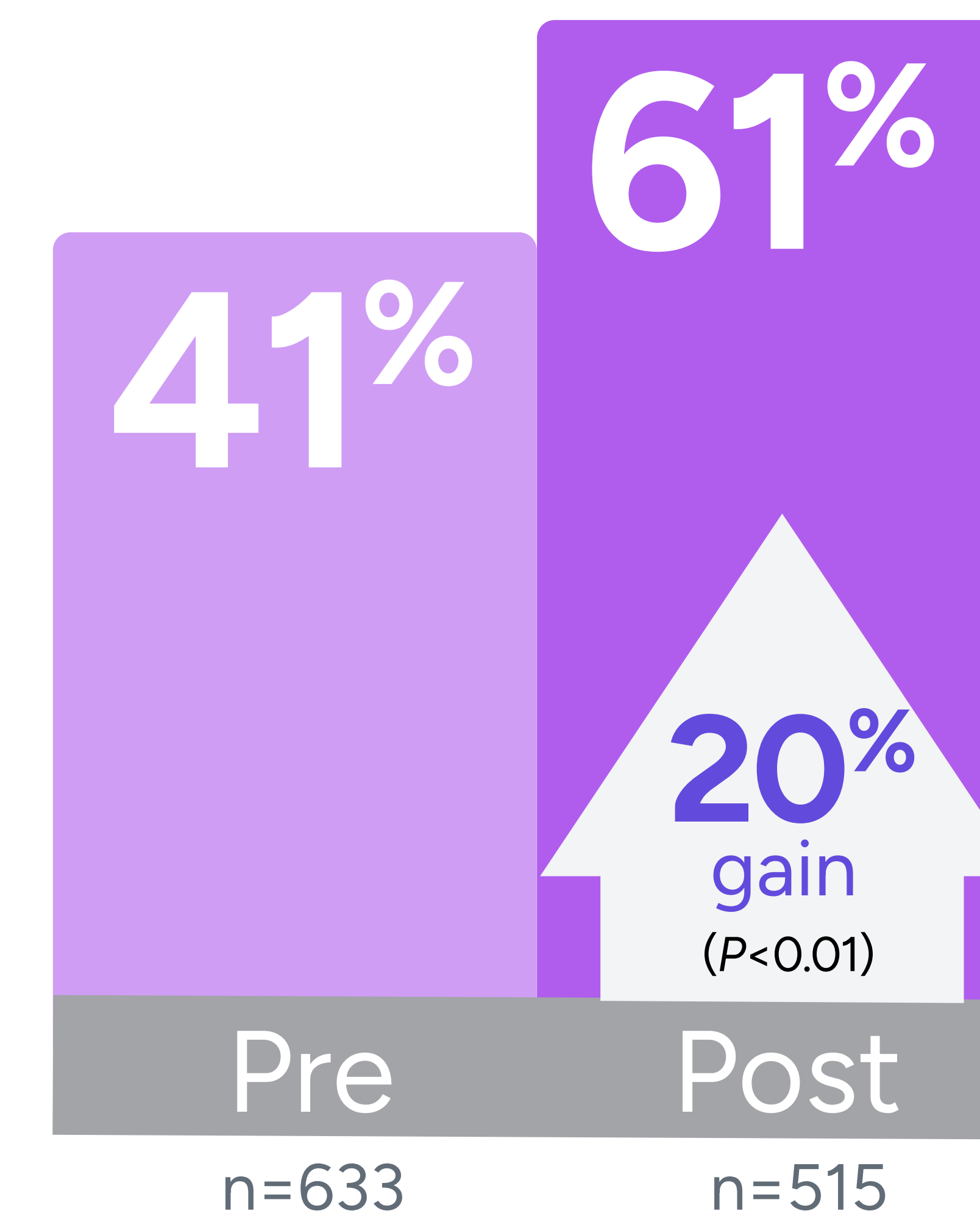
Which of the following is a selective type II myosin inhibitor showing promise in early phase trials for treatment of BMD?



Correct answer:
Sevasemten

Common cause of mortality for people with BMD

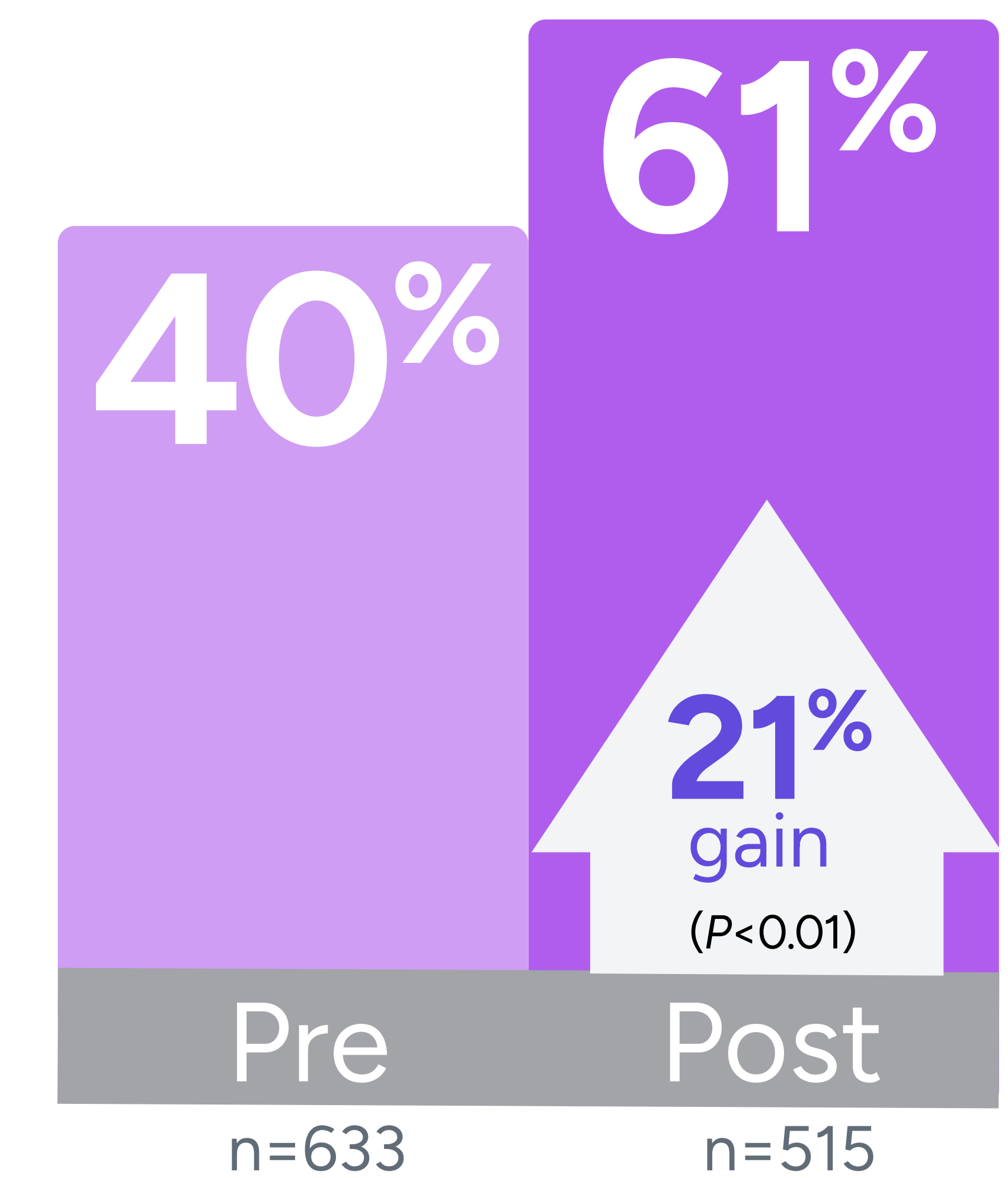
What is the most common cause of death in patients with BMD?



Correct answer:
Cardiomyopathy

Grading disease severity

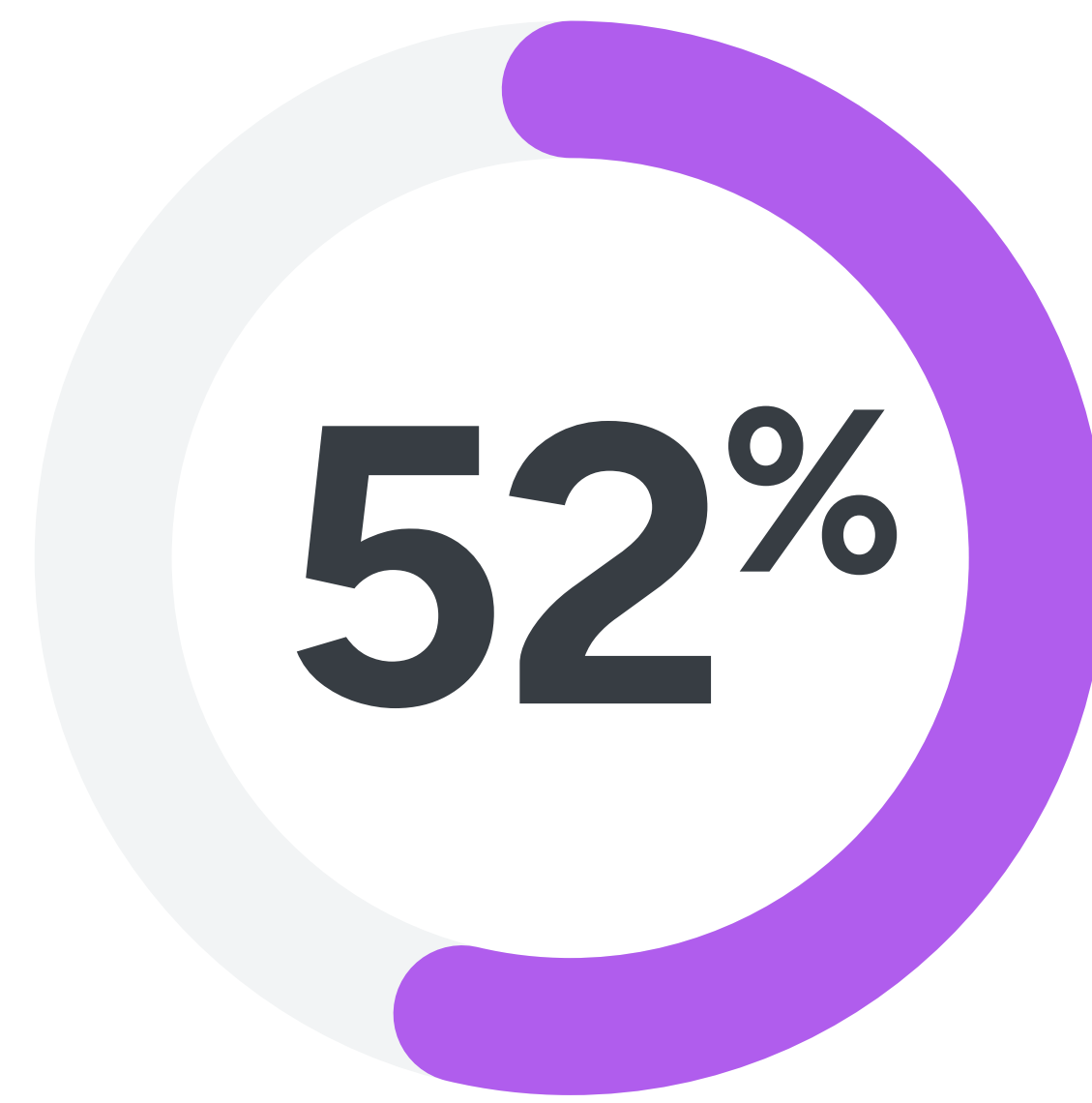
Ray is an 8-year-old patient who has recently been diagnosed with BMD based on the finding of an in-frame deletion of exon 48 in the dystrophin gene. He is experiencing mild muscle weakness and fatigue during physical activities. Based on Ray's genetic mutation and current symptoms, what severity of disease do you anticipate over his lifetime?



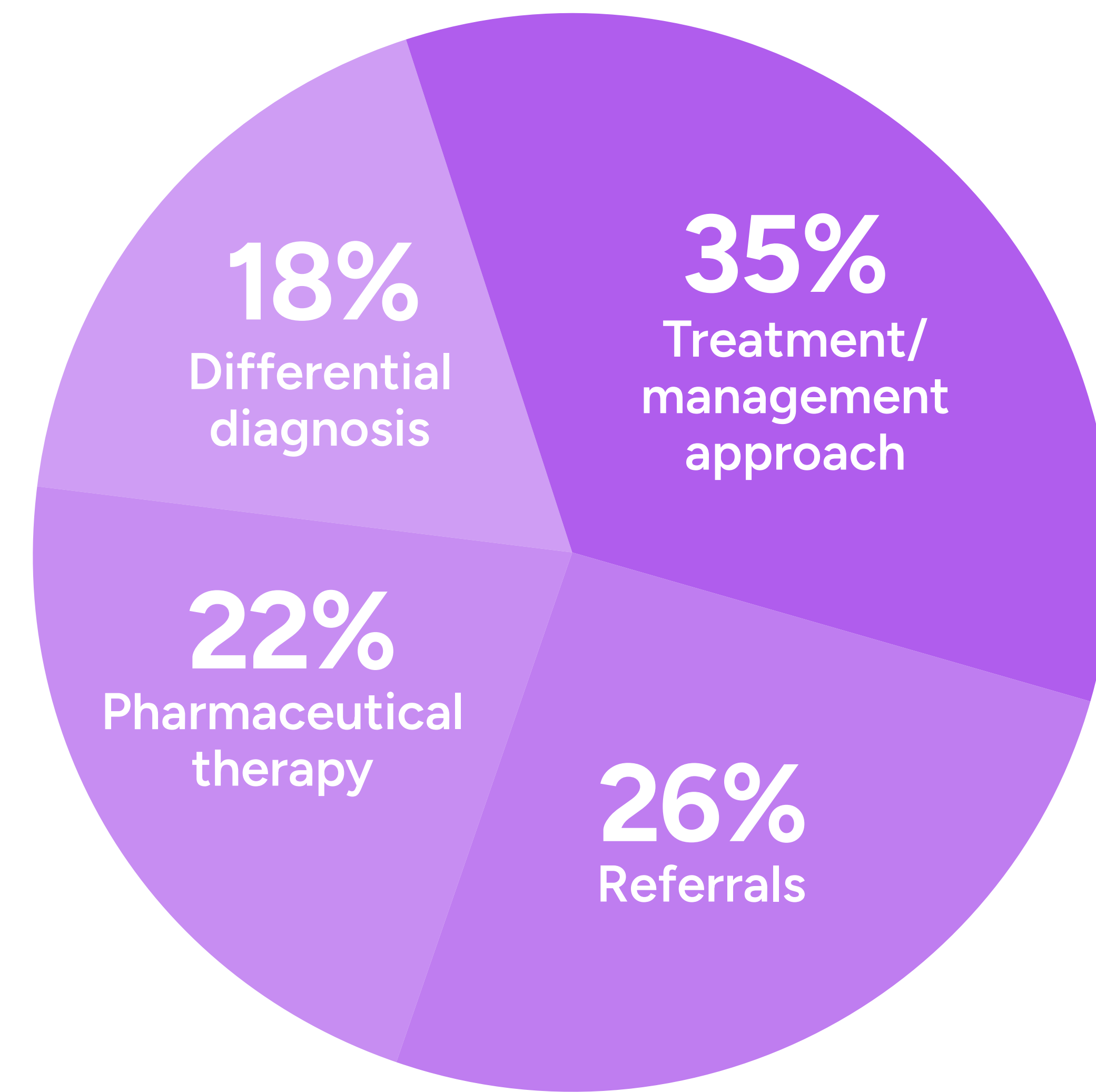
Correct answer:
Mild, maintaining ambulation and ability to perform daily activities with minimal assistance well into adulthood

CONCLUSION

Practice impact:



of participating clinicians, intended to change practice in the following areas:



Integrating patient perspectives into BMD education improved HCPs' knowledge of pathophysiology and emerging therapies and helped reshape perceptions of disease burden. Outcomes data highlighted the value of patient-centered CME in preparing HCPs to deliver more empathetic care for individuals with BMD.

