

Utilizing Gene Therapy in Duchenne Muscular Dystrophy: Impact of Continuing Medical Education on Participant Knowledge and Behavior

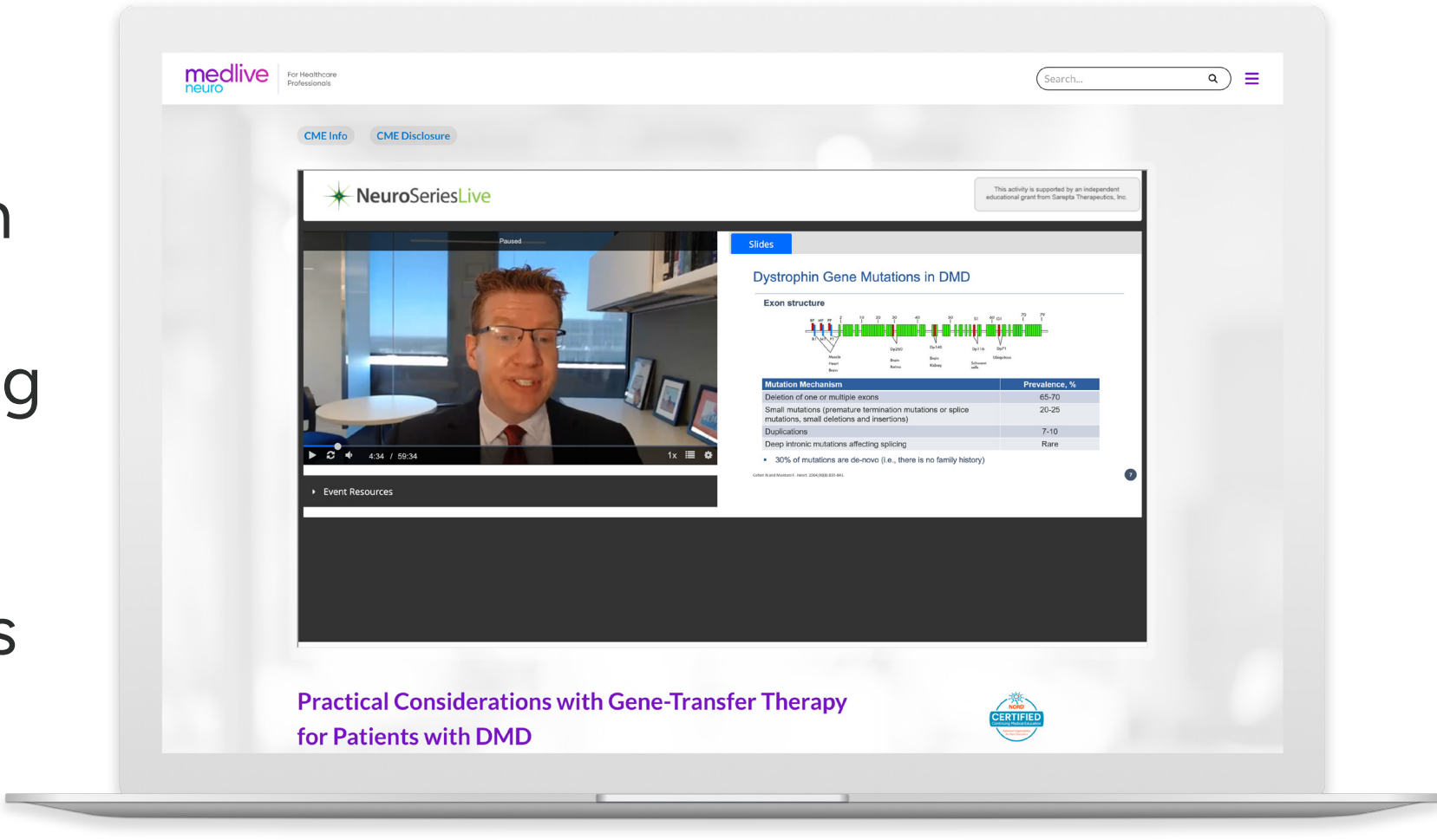


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INTRODUCTION

In June 2023, the US Food and Drug Administration (FDA) approved the first gene therapy (GT) for the treatment of patients with Duchenne muscular dystrophy (DMD). We sought to characterize the impact of continuing medical education (CME) on healthcare professionals' (HCPs') awareness of the rationale for and data related to GT for DMD, as well as their ability to recognize and counsel patients who are candidates for GT.



METHODOLOGY

Educational Program and Evaluation Details

Title: Practical Considerations with Gene-Transfer Therapy for Patients with DMD

Topics Covered:

- 1) Rationale for using GT in DMD
- 2) Clinical trial findings
- 3) Pre-, peri-, and post-GT practical considerations, such as patient preparation, adverse-event management, and multidisciplinary care delivery

Interventions

- A live, online, 1-hour, CME-certified activity launched on September 15, 2023, featuring key opinion leaders experienced with newly available GT for DMD. The activity will remain on-demand for 1 year.

- Activity components: downloadable resources, live polling, and an audience real-time question-and-answer session.

- Four, short (3-6 minute) educational videos, each tied to a learning objective from the 60-minute CME activity, will also be posted to LinkedIn targeting NPI-verified neurologists.

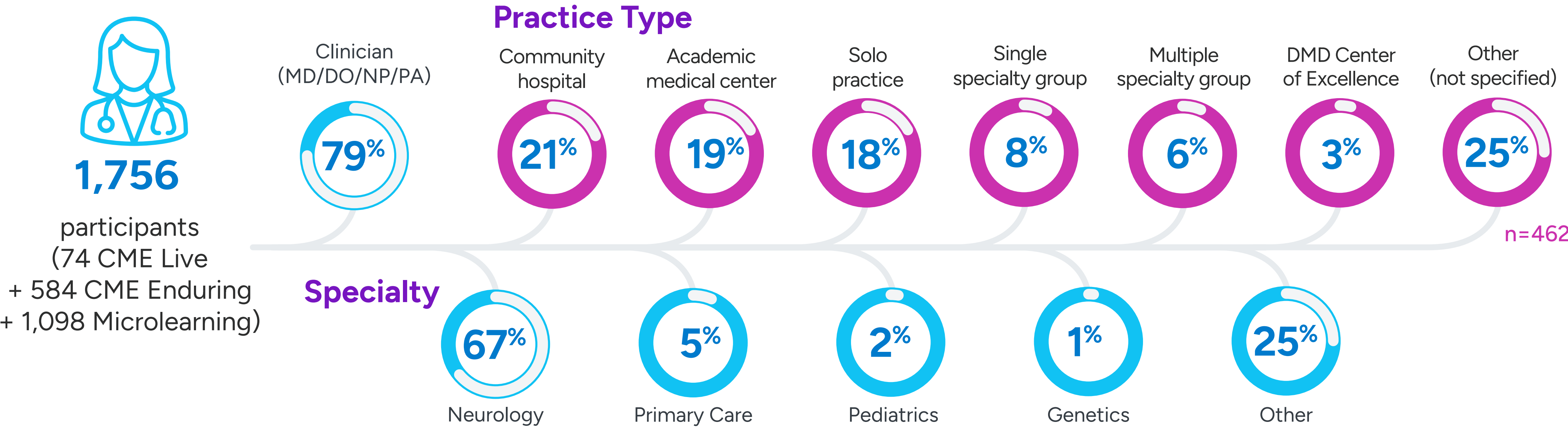
Faculty

John Brandsema, MD
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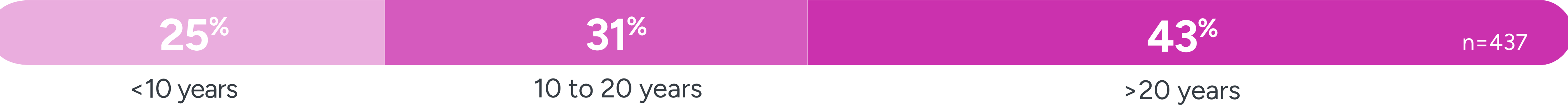
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RESULTS

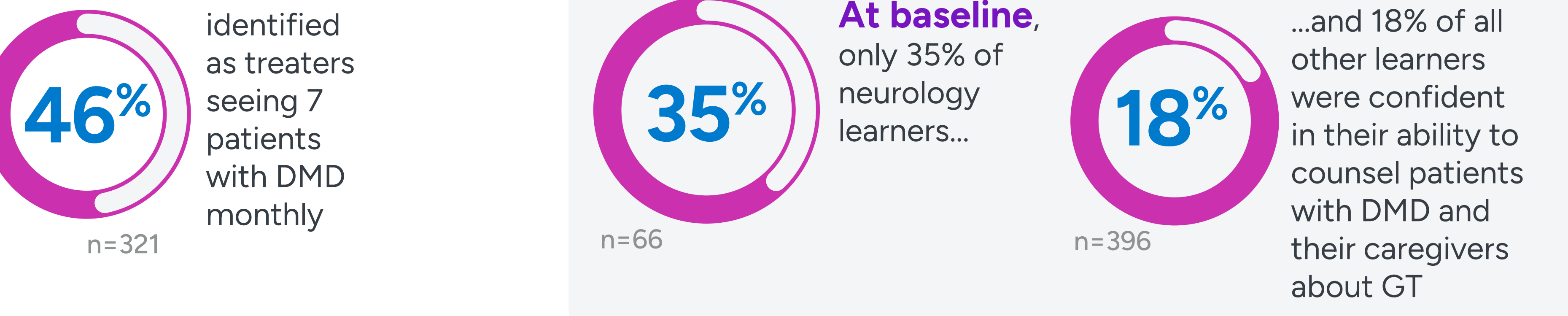
Learner Demographics



Years in Practice



Patient Experience



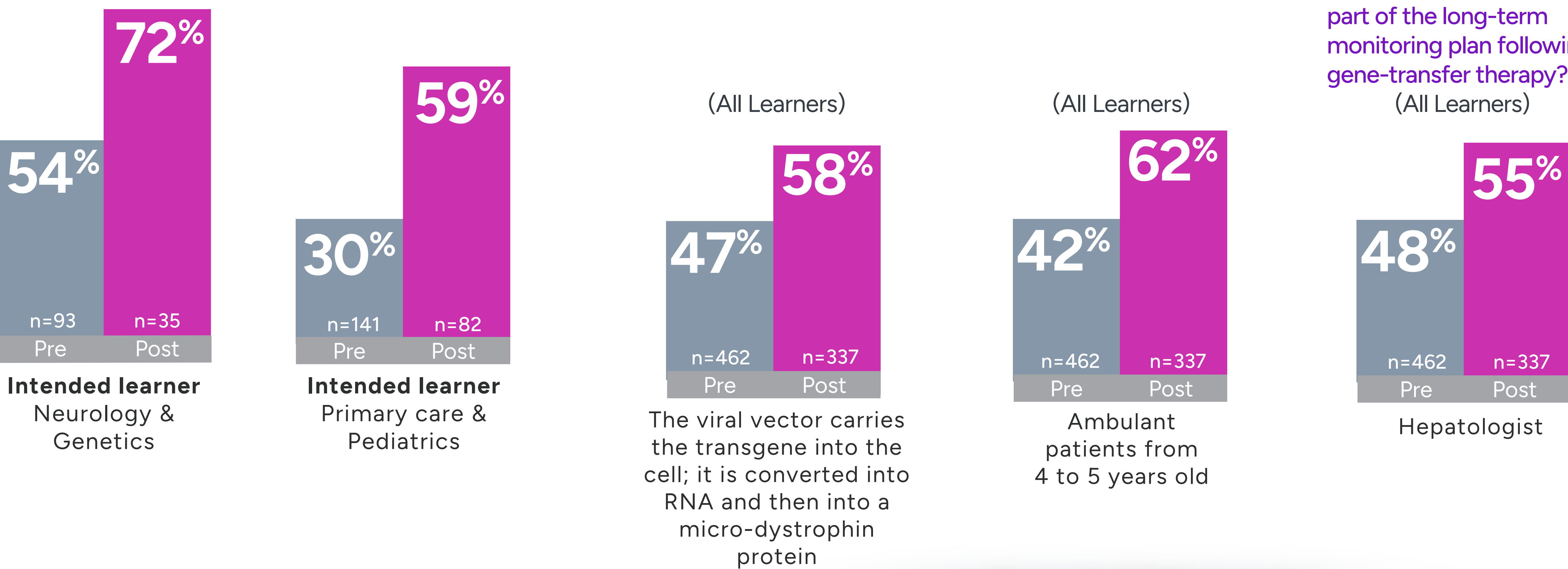
Expected Greatest Challenges

Greatest challenge in managing a patient with DMD in the era of gene therapy	Percentage
Unfamiliarity with gene-transfer therapies	20%
Late diagnosis	19%
Finding support and resources	11%
Incorporating genetic testing into decision-making	9%
Managing adverse events and side effects	8%
Difficulty matching patient to therapy	8%
Managing patient/family anxiety	6%
Other (not specified)	19%

n=333

Changes in Knowledge/Competence

Average score (% correct) on 3-item test linked to learning objectives



Post activity, 61% of learners indicated that they intended to implement a practice change related to treatment

Intended Practice Changes	Percentage
Apply latest guidelines	45%
Choice of treatment/management approach	45%
Change in current practice for referral	25%

n=196

CONCLUSION

CME was successful in improving the ability of HCPs of various specialties to discuss the topic of GT with their patients by enhancing their knowledge and competence in critical areas related to the implementation of GT. Data also highlight a continued need for education related to DMD diagnosis, which is an important issue based on the number of GTs on the horizon for DMD and other neuromuscular diseases.

