

Recognition and Management of Duchenne Muscular Dystrophy: Knowledge and Behavior Changes from Continuing Education

Authors: Carole Drexel, PhD¹, John Brandsema, MD², Elizabeth del Nido¹, Wendy Turell, DrPH¹

1 - PlatformQ Health, Needham, MA; 2 - Perelman School of Medicine, U of Penn.



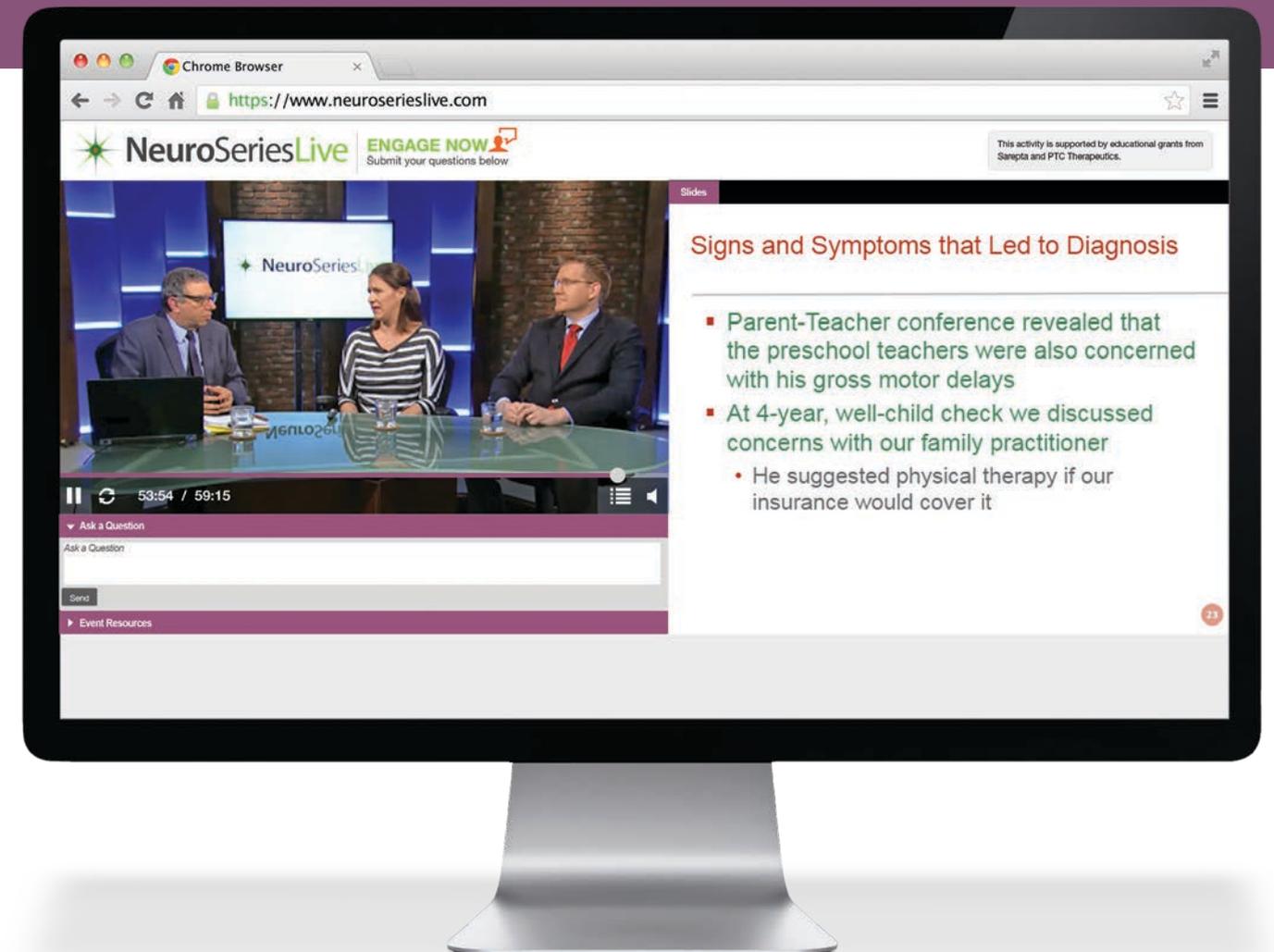
Postgraduate Institute
for Medicine



PLATFORMQ HEALTH
EDUCATION

INTRODUCTION

Duchenne muscular dystrophy (DMD) poses a significant challenge to clinicians due to its progressive nature and lack of a cure. However, recent advances in therapeutic options have spurred updated care guidelines focused on the evolving nature of the diagnosis and management of DMD. The innovation in guidelines and treatment options created an urgent need for clinician education. We sought to assess the influence of online continuing medical education (CME) on addressing clinician gaps in knowledge regarding emerging therapeutic strategies, biomarkers and outcome measures, multidisciplinary care coordination, disease complications, and transition to adult care.



This activity is supported by educational grants from Sarepta and PTC Therapeutics.

METHODS

Educational Program and Evaluation Details

Title

Overall Initiative Title: Duchenne Muscular Dystrophy – An Expanding Lifespan

Format

- 2, 1-hour CME sessions produced and broadcast live-online on NeuroSeriesLive.com, and immediately on-demand 12 mos.
- Downloadable slides, panel discussions, live polling, pre-program and live Q&A.

Accredited Provider

Postgraduate Institute for Medicine

Learning Objectives

- Summarize the differential diagnosis of DMD and applicable diagnostic approaches
- Adopt a multidisciplinary approach to diagnosing patients with DMD
- Describe signs and symptoms that would raise a high index of suspicion of DMD
- Identify mutations that may be associated with DMD and how that relates to making treatment decisions
- Assess the therapeutic potential of available and emerging treatment options for the management of DMD
- Adopt a multidisciplinary approach to managing patients with DMD
- Describe elements for a successful transition from childhood to adulthood care in patients with DMD

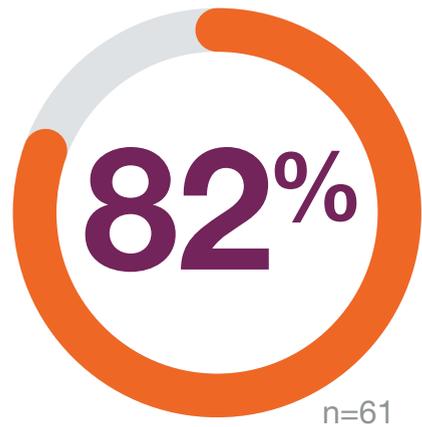
Educational Impact Measurement

- A 2hr CME activity launched live-online in 2019, and remained on-demand through 2020.
- CME questions were administered at 3 points in time prior to and following the education.
- Responses from CME, live polling, and pre/post testing were analyzed to determine engagement, lessons learned, and continuing gaps. McNemar testing compared matched pair responses (pre/post & pre/2 mos.) with Cohen's d for effect size.



RESULTS

Positive Impact on Patient Outcomes

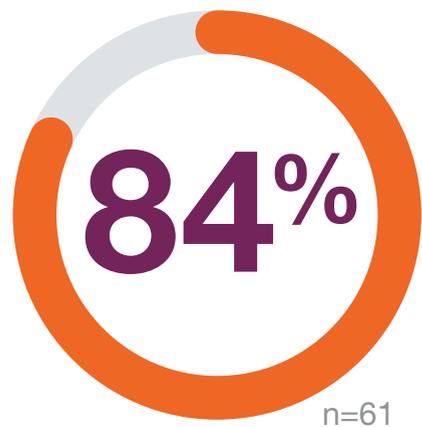


learners reported the activity positively impacted patient experience/outcomes

Helped us diagnose patient early.

My patient feels more confident.

Parents of patients have more hope.



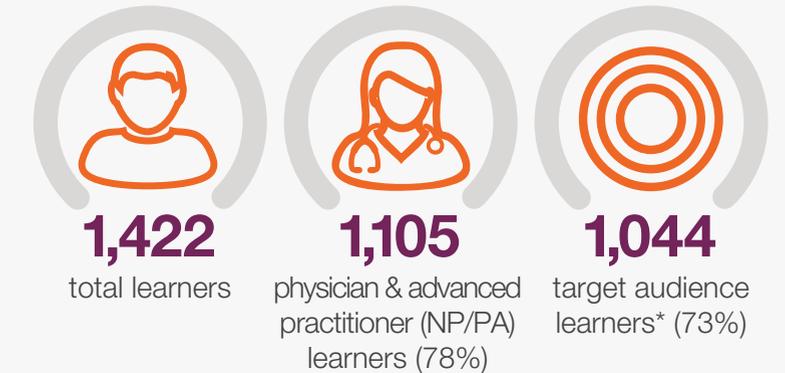
learners reported the activity positively impacted clinical practice

Early recognition of the disease and proceeded with molecular genetic testing after the preliminary tests.

I'm more confident in distinguishing new cases and advising existing cases.

Better plan on how to take care of Duchenne patients. Think more about the different treatment options.

Learner Demographics



*Target Audience: Pediatrics, Neurology, Neuromuscular Specialists, Endocrinology, Gastroenterology, Pediatric Cardiologists, Psychiatrists, Pulmonology, Pediatric Neurologists, PCPs, NPs, PAs, Pharmacists, Nurses, and Dietitians

Learner Engagement

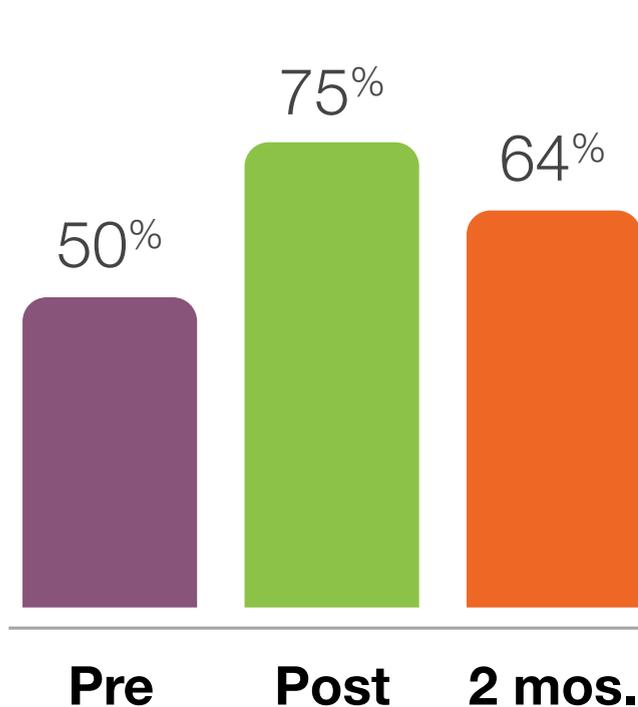


RESULTS

Changes in Knowledge/Competence

25% & 14% increase over pre-test

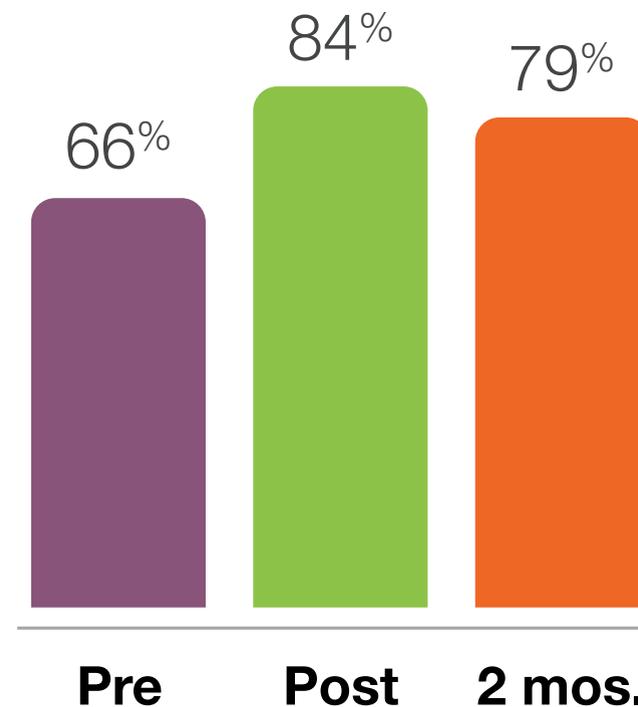
in competence concerning DMD characteristics and identification



n = 202 pre; 124 post; 61, 2-mos. follow-up

18% & 13% increase over pre-test

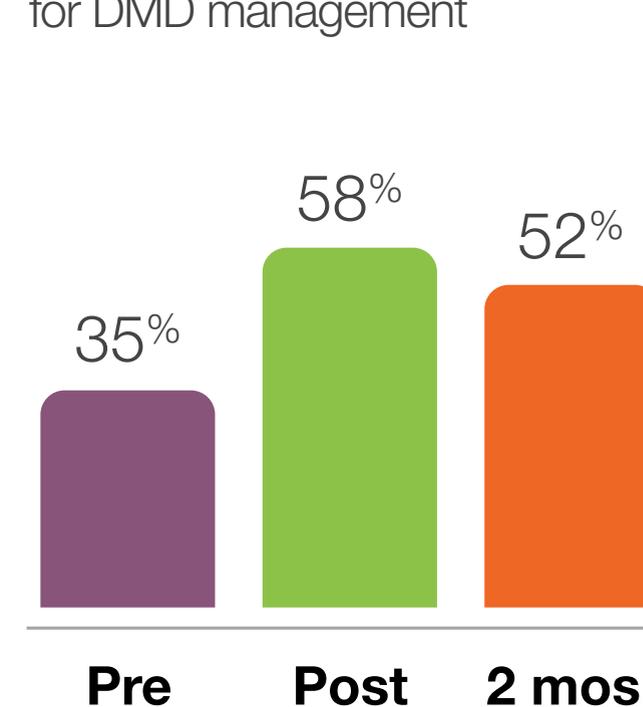
in knowledge regarding dystrophin mutations associated with DMD



n = 202 pre; 124 post; 61, 2-mos. follow-up

23% & 17% increase over pre-test

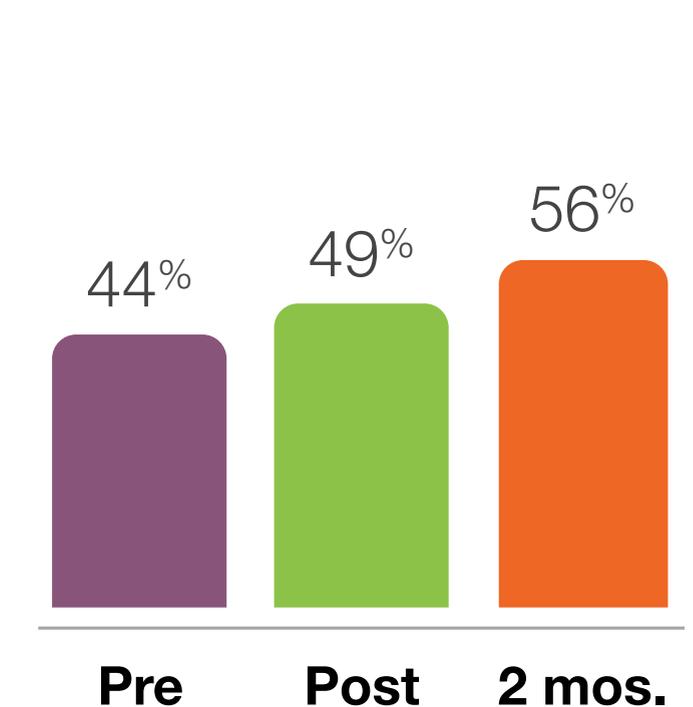
in knowledge regarding cardiovascular therapies for DMD management



n = 129 pre; 85 post; 61, 2-mos. follow-up

5% & 12% increase over pre-test

in knowledge regarding indications of DMD interventions



n = 202 pre; 124 post; 61, 2-mos. follow-up

CONCLUSIONS & FUTURE EDUCATIONAL TARGETING

Quantitative testing and surveying supported the positive impact of CME focused on improving knowledge and competency concerning new guidelines, novel therapeutics, and multidisciplinary care integration for DMD. Ongoing education is advised on:

Treatment of DMD:

- Gene therapy combined to exon skipping therapy
- The place of corticosteroids in the evolving treatment landscape
- Role of eteplirsen, and other exon skipping therapies in the management of DMD
- Role of ACE-inhibitors and ARBs in improving cardiac function

DMD - the disease:

- Recognizing / diagnosing of DMD
- How to set-up interdisciplinary care for patients with DMD
- Genetic counseling implications
- Strategies in transitioning from pediatric to adult care

