EP.128

Incorporating Treatment Advances and a Multidisciplinary Approach with Duchenne Muscular Dystrophy: A study of online education impact on clinical care

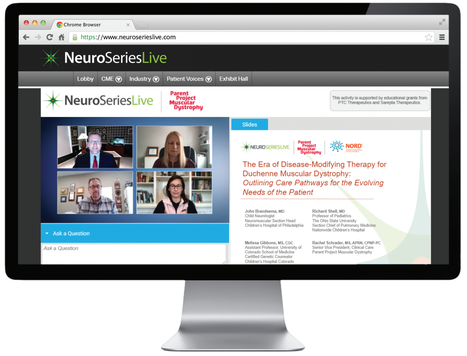
Carole Drexel, PhD[1], John Brandsema, MD[2], Elizabeth del Nido, BA[1]

1-PlatformQ Health, LLC, Needham, MA; 2-Children's Hospital of Philadelphia

INTRODUCTION

Early diagnosis and intervention are critical to preserving quality of life for patients with Duchenne Muscular Dystrophy (DMD). Updated guidelines and recent therapeutic advances have left clinicians with challenges including: incorporating new mutation-specific treatments, utilizing multidisciplinary approaches to care, and transitioning patients to adult care.

To address these gaps, a 60-minute CME activity was broadcast live-online in October, 2020, and remains on-demand through October, 2021, in partnership with Parent Project Muscular Dystrophy (PPMD) and National Organization for Rare Disorders (NORD). The faculty represents a multidisciplinary care team, with a neurologist, pulmonologist, genetic counselor, and a patient advocate. To incorporate the patient voice, content included several recorded stories from families from families with a DMD diagnosis.



Educational Program Details

Title

The Era of Disease-Modifying Therapy for Duchenne Muscular Dystrophy: Outlining Care Pathways for the Evolving Needs of the Patient

Format

- 1-hour video CME activity was produced and broadcast live-online on NeuroSeriesLive and made available on-demand for 12 months following the live session
- The activity featured downloadable educational slides, panel discussions, patient/family stories, live polling, and live Q&A
- Learners encouraged to submit questions before and during the live program
- Recorded patient videos were released via Facebook for a patient/family audience

Accredited Provider

- Postgraduate Institute of Medicine

Faculty

- John Brandsema, MD, The Children's Hospital of Philadelphia
- Richard Shell, MD, The Ohio State University, Nationwide Children's Hospital
- Melissa Gibbons, MS, CGC, University of Colorado School of Medicine, Children's Hospital Colorado
- Rachel Schrader, MS, APRN, CPNP-PC, Parent Project Muscular Dystrophy

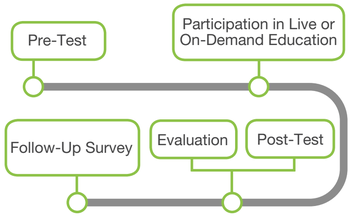
Learning Objectives

- Develop an enhanced index of suspicion for DMD in patients who present with signs and symptoms
- Recognize mutations associated with DMD and how they relate to treatment decisions
- Integrate key components of multidisciplinary care for patients with DMD, from initiation of targeted therapy to long-term care
- Assess the therapeutic potential of available and emerging treatment options for the management of DMD

METHODS

Educational Design & Measures

A 1hr CME activity launched live-online in October, 2020, to remain on demand through October, 2021. CME questions were administered at 3 points in time prior to and following the education (figure 1). 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.



INTERIM RESULTS (AS OF 7/7/21)

Learner Demographics

535 total learners

67% target audience*

55% MD/DO and NP/PA Learners

Learner Engagement

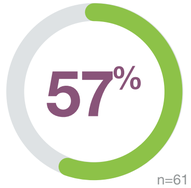
179 CE credits awarded

272 polling responses

8,882 patient/family video views

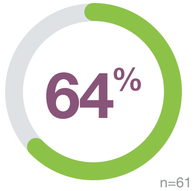
*Target Audience: Pediatrics, Neurology, Primary Care, Internal Medicine, Endocrinology, Gastroenterology, Pediatric Cardiologists, Physiatrists, Pulmonology, Nurses, NP/PA, Pharmacists, Dieticians

Positive Impact on Patient Outcomes and Clinical Practice



n=61

learners reported the activity positively impacted patient experience/outcomes



n=61

learners reported the activity positively impacted clinical practice

75 write-in impact examples were shared

> Being mindful of the complexity of Duchenne patients and the multiple resources that must be allocated and coordinated.

> I have a family member who was diagnosed with DMD so I can help the family with their son's care and treatment.

> We have developed a new protocol for possible new patients. Likewise have we been reviewing patients charts to locate patients who should be ruled out as previously were missed.

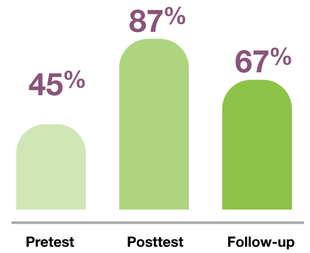
> Better utilization of diagnostic/ genetic testing tools to confirm the diagnosis of DMD.

INTERIM RESULTS (CONT.)

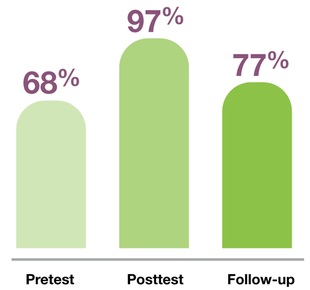
Changes in Knowledge/Competence

1 case-based competence question and 4 knowledge questions were posed pre and post education. All 5 questions showed statistically significant improvements post-education.

42% and 22% increase over pre-test in competence concerning recognition of DMD signs and symptoms



29% and 9% increase over pre-test in knowledge regarding dystrophin mutations common to DMD



n=326 pre; 125 post; 61, 2 mos. follow-up

Average effect size pre to post: d=1.34 & pre to 2 mos.: d=.323

CONCLUSIONS

Outcomes results and participant questions suggest the value of future education in the following areas:

Management of DMD

- Safety, efficacy, tolerability and indications for use of eteplirsen
- Exon-skipping therapies for managing DMD
- Process for referring patients for antisense therapy
- Strategies to improve cardiac outcomes
- Differentiating steroid therapies – assessing qualitative differences
- Impact of COV-19 on disease management of DMD
- Investigational agents - pipeline for the future
- Is there a role for combination therapies? Gene therapy plus exon-skipping?

DMD - the disease

- Signs and symptoms raising index of suspicion for DMD
- Principles of multidisciplinary care - engaging cardiology, endocrinology and PT
- Dystrophin mutations associated with DMD
- Essentials of accurate diagnosis
- Electrolyte abnormalities associated with DMD

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