

Impact of HCP and Caregiver Education on Multidisciplinary Care in Spinal Muscular Atrophy

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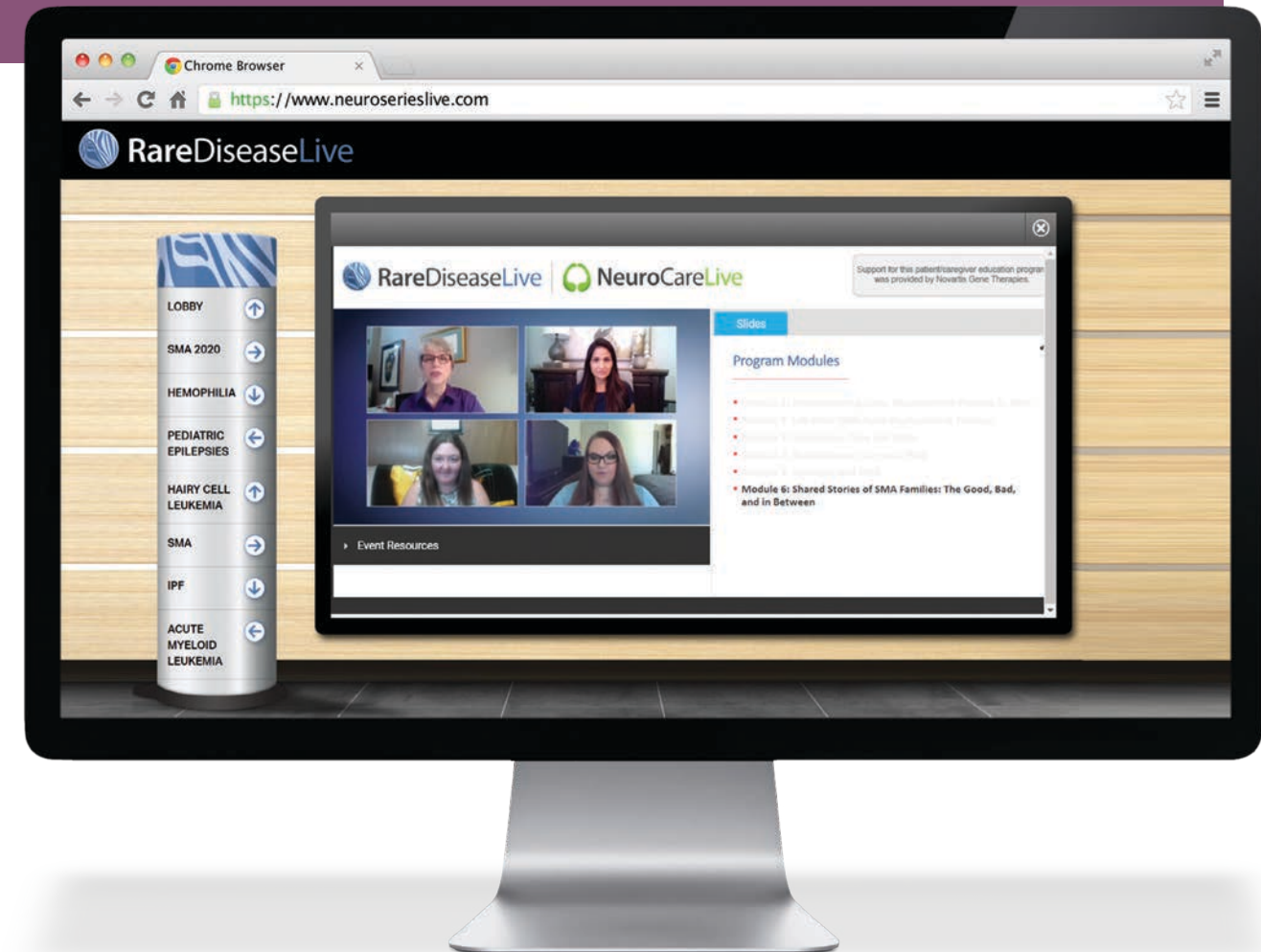
1 - PlatformQ Health, Needham, MA; 2 - CureSMA; 3 - NORD



INTRODUCTION

A spinal muscular atrophy (SMA) diagnosis is devastating to patients and families due to the impacts of progressive muscle weakness and impaired mobility, making it a leading cause of mortality in infants and young children. Early intervention and multidisciplinary care are urgently needed to provide the best possible treatment outcomes for those with SMA. Recent advances in the understanding of the molecular basis and disease pathogenesis have spurred significant treatment innovations in disease-modifying and gene therapies. These advances have challenged clinicians and families/caregivers alike to stay abreast of managing these novel treatment options and their impacts on patients, and to coordinate increasingly complex treatment plans across multidisciplinary specialties.

We sought to meet these educational gaps through online education, and subsequently assessed the impact of the targeted continuing medical education (CME) and caregiver-patient education through survey-based evaluation.



CME: This activity is supported by an educational grant from AveXis. Caregiver education: Support for this patient/caregiver education program was provided by Novartis Gene Therapies.

METHODS: EDUCATIONAL PROGRAM AND EVALUATION DETAILS

Title

Spinal Muscular Atrophy: A New Era for Both Patients and Clinicians

Format

- 70-minute video CME activity produced and broadcast live-online on NeuroSeriesLive.com, and immediately on-demand 12 mos.
- 145-minute video caregiver activity produced and broadcast on NeuroCareLive.com and RareDiseaseLive.com
- Downloadable slides, panel discussions, live polling, pre-program and live Q&A.

Accredited Provider

Postgraduate Institute for Medicine

Caregiver Program Partners

- CureSMA
- National Organization for Rare Disorders

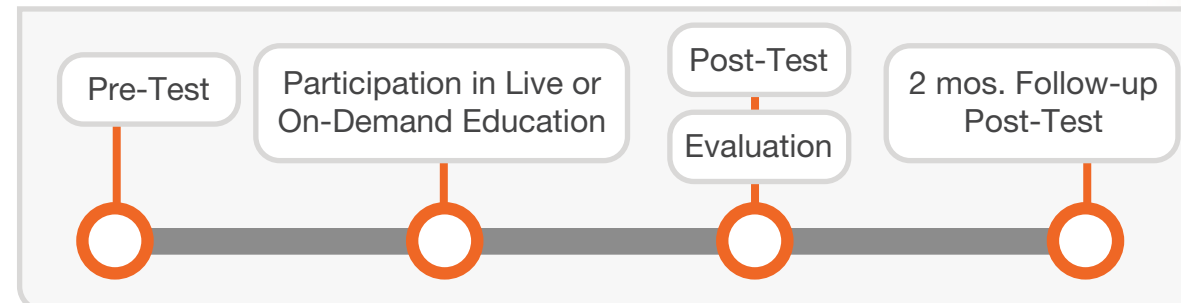
HCP Learning Objectives

- Describe the evolution of SMA phenotypes that may be associated with gene-replacement therapy in patients with SMA
- Identify best practices for multidisciplinary post-treatment management of patients with SMA who have received gene-replacement therapy

Caregiver Learning Objectives

- Discuss the benefits, administration, and treatment outcomes of gene-replacement therapy for SMA
- Describe the beneficial effects of treatment with gene replacement therapy on patients with SMA
- Identify strategies and/or methods to maintain respiratory health in patients with SMA
- Identify strategies and/or methods to maintain motor function and mobility in patients with SMA
- Recognize the therapeutic importance of maintaining optimal nutrition in patients with SMA
- Communicate with patients and families about the impact SMA has on quality of life, the challenges, and the best practices

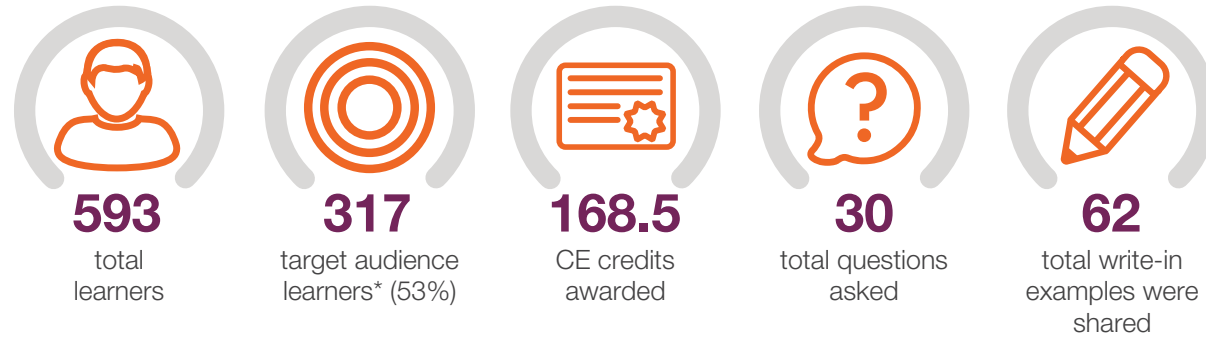
Educational Impact Measurement



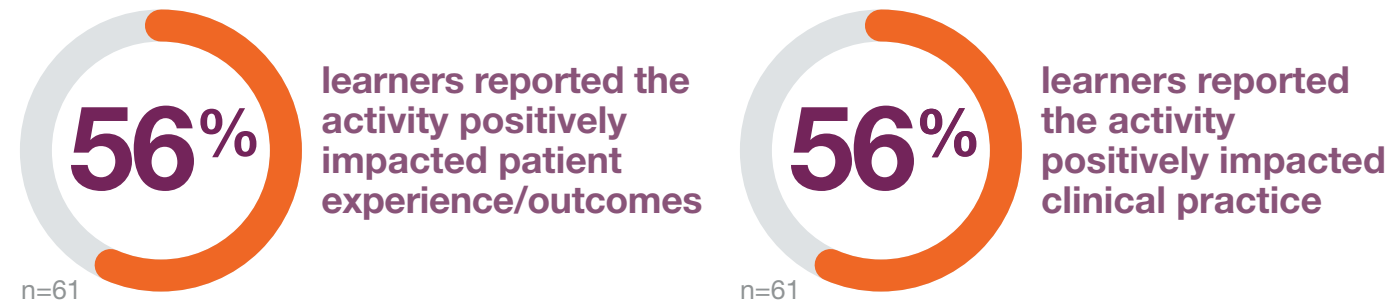
RESULTS

HCP Results

Engagement



Positive Impact on Patient Outcomes and Clinical Practice

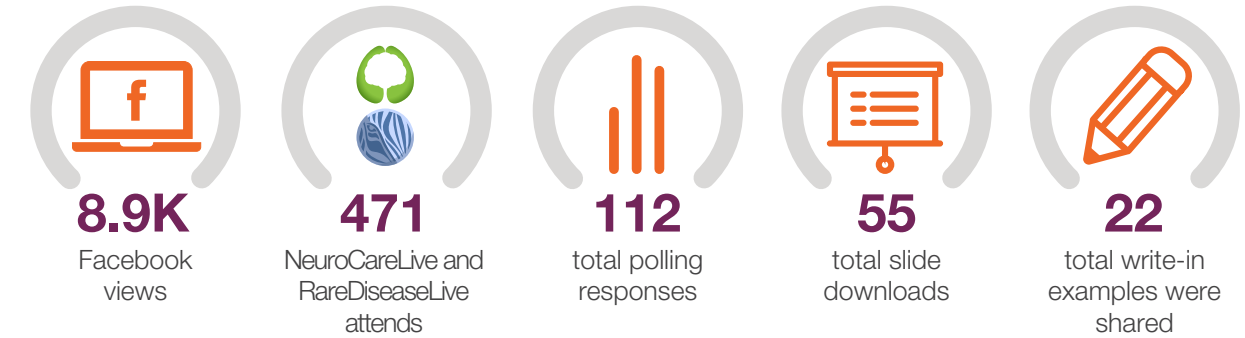


Earlier gene therapy for patients with SMA leads to better survival and functioning.

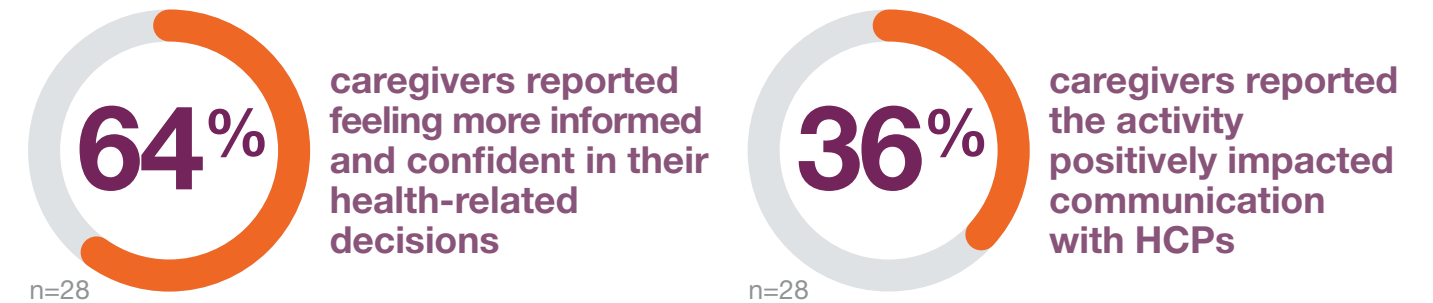
Better assess the subtle evolution of SMA phenotypes as a consequence of gene replacement therapy.

Caregiver Results

Engagement



Positive Impact on Decision-Making and Communication



It allowed me to speak with more confidence and advocate for occupational therapy in addition to the physical therapy that is already taking place.

My daughter, who has SMA1, knows why it's important to continue all her therapies even though she has received about a dozen injections already.

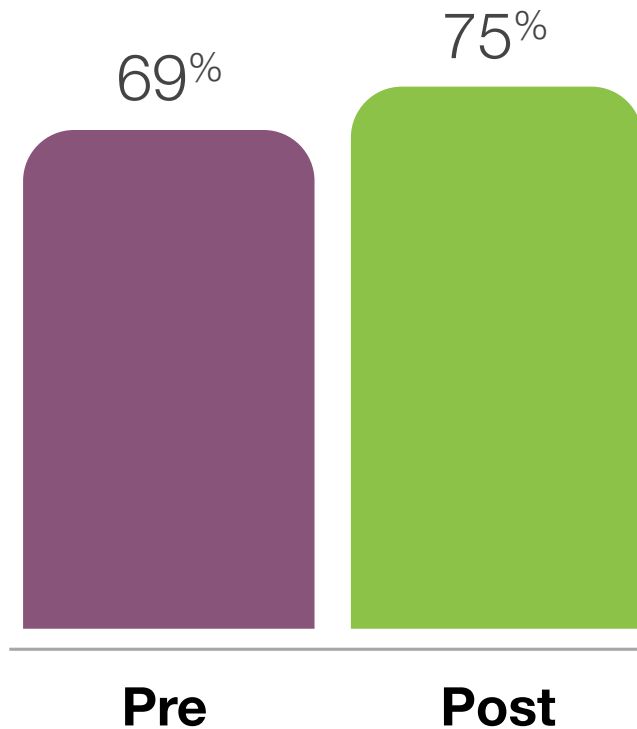
*Target Audience: Neurology/Pediatric Neurology, Pediatrics, Neuromuscular Specialists, Pulmonology, Pediatric Gastroenterologists, PT/OT, Nutrition, Genetics, NP/PA

RESULTS: CHANGES IN KNOWLEDGE/COMPETENCE

HCP Results

6% increase over pre-test

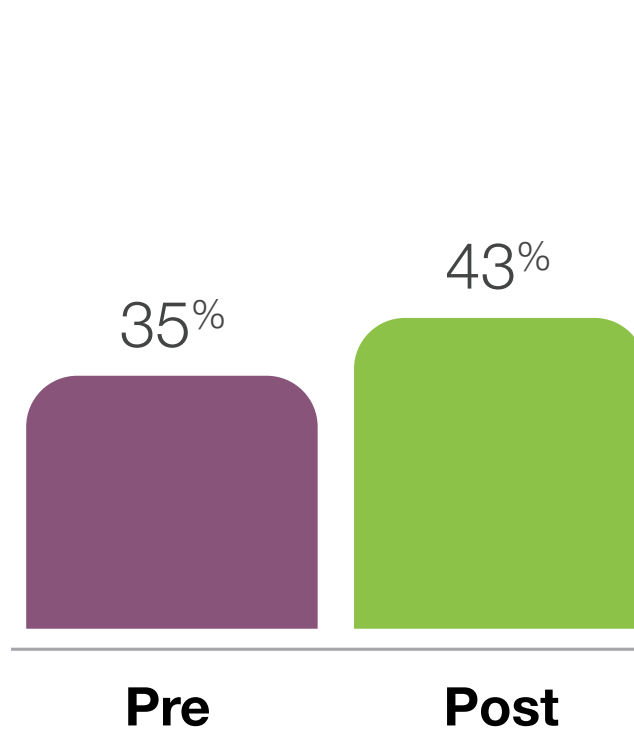
in knowledge regarding gene-replacement therapy for SMA



n = 592 pre; 180 post; 61, 2-mos. follow-up

8% increase over pre-test

in knowledge regarding respiratory function in patients with SMA

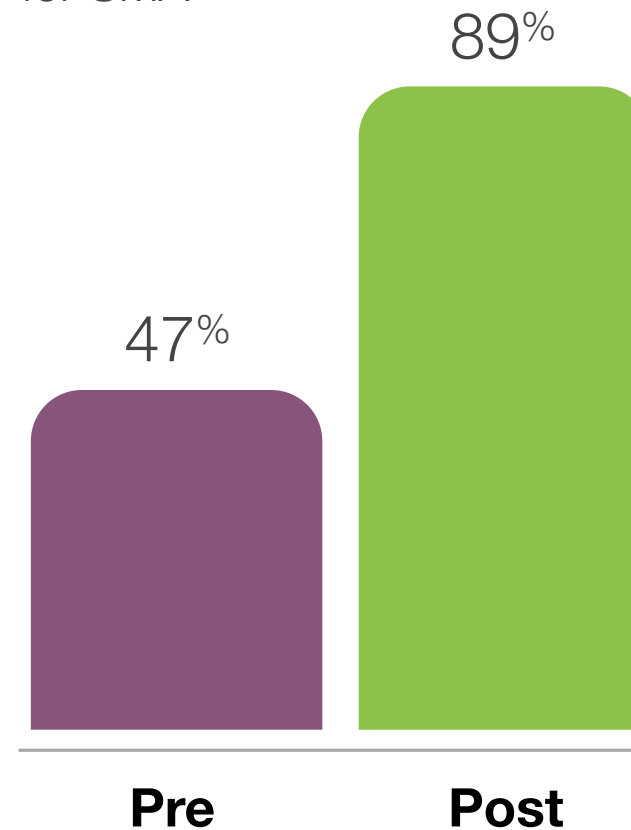


n = 592 pre; 180 post; 61, 2-mos. follow-up

Caregiver Results

42% increase over pre-test

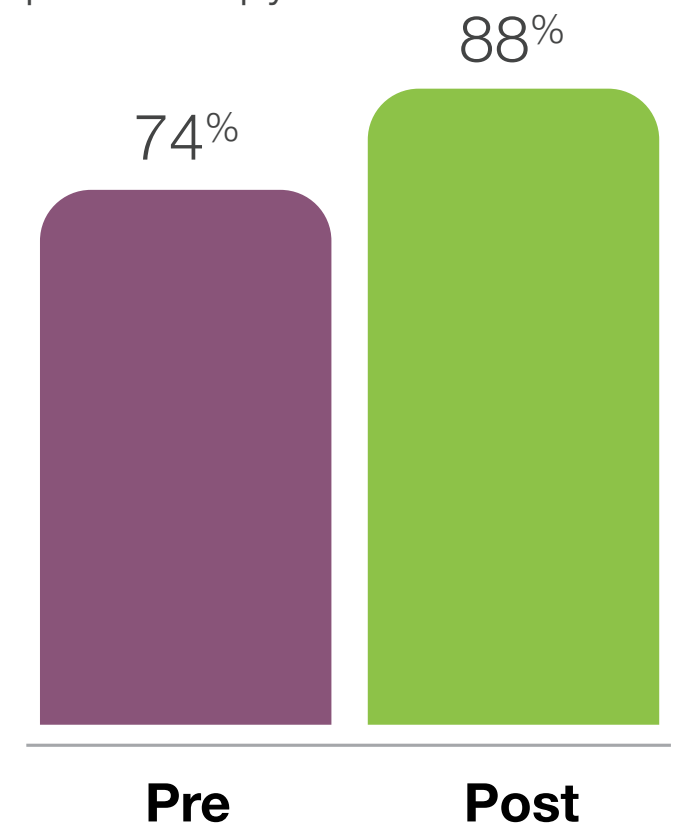
in knowledge regarding gene-replacement therapy for SMA



n = 118 pre; 24 post

14% increase over pre-test

in knowledge regarding ongoing care needed post-therapy



n = 47 pre; 8 post

CONCLUSIONS & FUTURE EDUCATIONAL TARGETING

HCP

Quantitative testing and surveying supported the positive impact of CME and caregiver education focused on SMA characteristics, gene replacement and disease modifying therapies, condition management, and multidisciplinary care delivery.

Ongoing HCP education is advised on:

Management of SMA:

- Factors in determining referral to orthopedic specialist
- Implications of PT after gene replacement
- Time course between diagnosis and therapeutic intervention
- Post-gene replacement precautions for patients/families/caregivers
- Current thinking on potential combination therapy - onasemnogene and nusinersen and therapy sequencing

SMA - the Disease:

- Impact of COVID-19 on outcomes in patients with SMA
- Strategies to keep families/caregivers engaged in disease management
- The role of the genetic counselor in supporting patients/families/caregivers
- Expectations of SMA phenotypes after gene replacement therapy
- Key principles of multidisciplinary care

Caregiver

Ongoing caregiver education is advised on:

Management of SMA:

- Post-gene replacement precautions for patients/families/caregivers
- Setting realistic expectations of gene replacement therapy
- Assessing current treatment options
- Strategies for effectively taking an active role in disease management

SMA - the Disease :

- The role of the genetic counselor in supporting patients/families/caregivers
- Communicating with members of the multidisciplinary care team
- Online resources for support

