

Preparing for a New Era in SMA Care with Disease-Modifying Therapies: The Role of Tethered, Aligned, Patient/Caregiver, and Clinician Education

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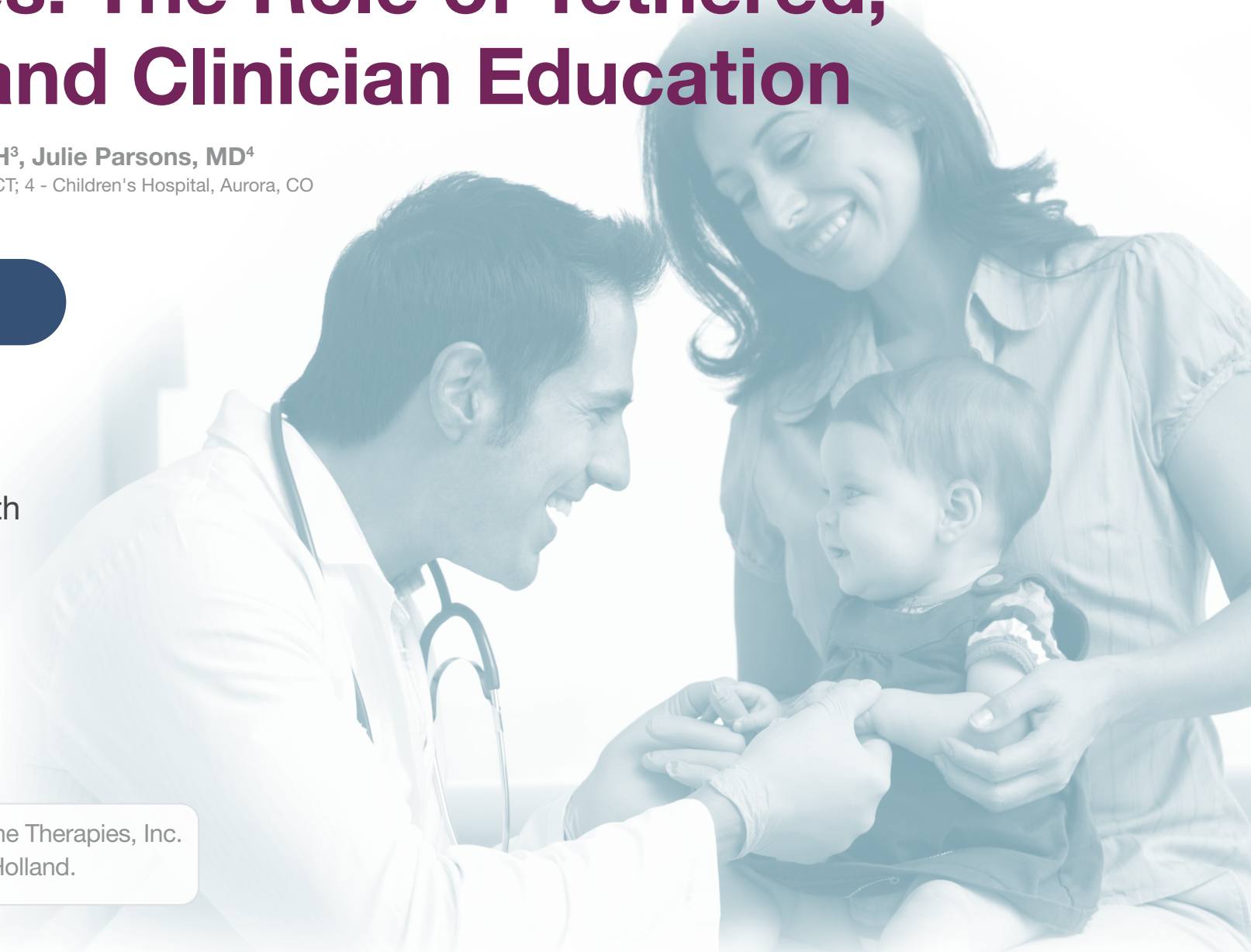
1 - PlatformQ Health, Needham, MA; 2 - CureSMA, Elk Grove Village, IL; 3 - National Organization for Rare Disorder, Danbury, CT; 4 - Children's Hospital, Aurora, CO

INTRODUCTION

With several available disease-modifying therapies (DMTs), the natural history of SMA is changing. Treatment expectations of healthcare providers (HCPs) and patients/caregivers (P/Cs) are evolving, along with the ongoing need for multidisciplinary care. The goal of this study is to assess the influence of aligned HCP and P/C education on knowledge and competence gaps and self-reporting behaviors related to the evolving needs of patients with SMA, including the obstetric and gynecologic needs of women with SMA.

HCP education: Supported by educational grants from Biogen, MRC Holland, and Novartis Gene Therapies, Inc.

P/C education: Support for this patient education program was provided by Biogen and MRC Holland.



METHODOLOGY: EDUCATIONAL PROGRAM AND EVALUATION DETAILS



Partners

Advocacy groups: NORD and CURE SMA
Education: PlatformQ Heath



Interventions

Tethered education comprised of one 60-min CME activity for HCPs and four 15-min modules for P/Cs, covering similar themes relative to the changing landscape of SMA management



Data collected

Changes in knowledge, competence, and reported behavior, engagement, and identification of continuing gaps



Measurements

Questions asked before, immediately after, and 2 months post activity. Chi Square tests for statistical analysis.

Title

Collaborative Care in SMA: Evolving Roles for Neurologists, Ob-Gyns, PCPs, and Counselors

Learning Objectives

- Discuss how DMTs are expected to change the course of SMA disease and result in new phenotypes across the spectrum of patients
- Explain how new SMA phenotypes will lead to expansion of the roles of PCPs and Ob-Gyns in the management of SMA, from genetic carrier identification to early diagnosis and adult-care transitions
- Describe issues that may arise in patients with SMA who transition from pediatric to adult care and develop strategies on how those issues can effectively be addressed, with a focus on reproductive care

Faculty



Julie A. Parsons, MD

Haberfeld Family Endowed Chair in Pediatric Neuromuscular Disorders
Professor of Clinical Pediatrics and Neurology
University of Colorado School of Medicine
Children's Hospital Colorado



Lizbeth McCarthy, MD

Director of Genetics and Ultrasound
Denver Health



Melissa Gibbons, MS, CGC

Certified Genetic Counselor, Assistant Professor
University of Colorado School of Medicine
Children's Hospital Colorado

All activities featured downloadable slides, panel discussions, live polling, pre-program and live Q&A. Video vignettes of P/Cs were inserted within the content of each activity to bring in the patient experience.



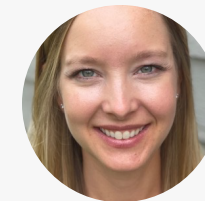
Amber Bosselman – Patient Interview Speaker

Amber is a young woman who was diagnosed with SMA as an infant. She discussed her desire to become pregnant, genetic testing/screening that would need to be done prior to pregnancy, and how her SMA symptoms may change with pregnancy.



Annie Heathcote – Patient Interview Speaker

Annie is a 22-year-old with SMA Type II. She joined the panel of the patient/caregiver session to discuss her treatment, hobbies, importance of discussing intimate relationships with SMA patients, and her future.



Julie Menk – Caregiver Interview Speaker

Julie has a 2-year-old son who was diagnosed with SMA through newborn screening at birth, so they were able to start treatment before his symptoms started. She discussed how her child's future will be brighter because of early detection and new treatment options.

RESULTS: LEARNER DEMOGRAPHICS AND ENGAGEMENT

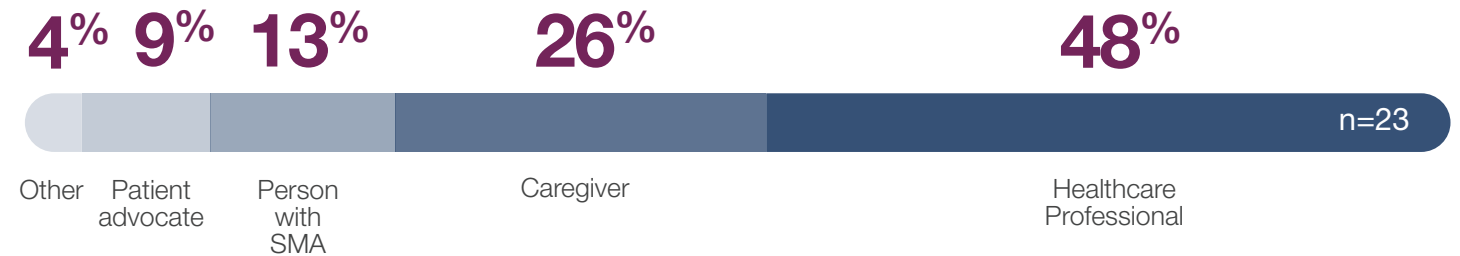
HCP Demographics and Engagement



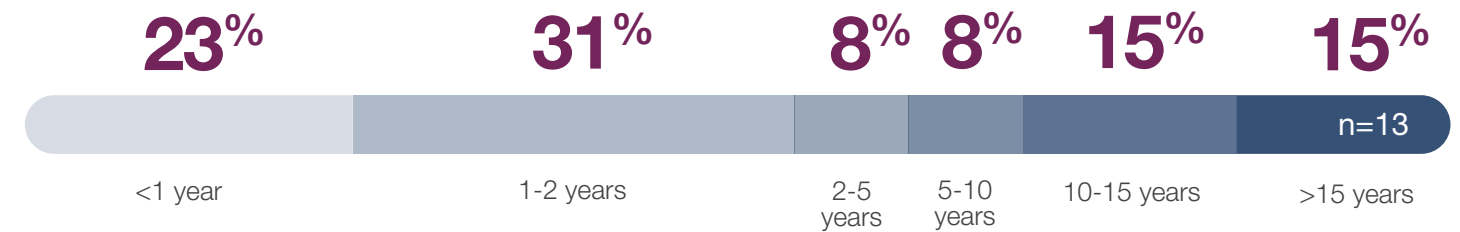
Patient/Caregiver Demographics and Engagement



Participant Type



Time Since Diagnosis

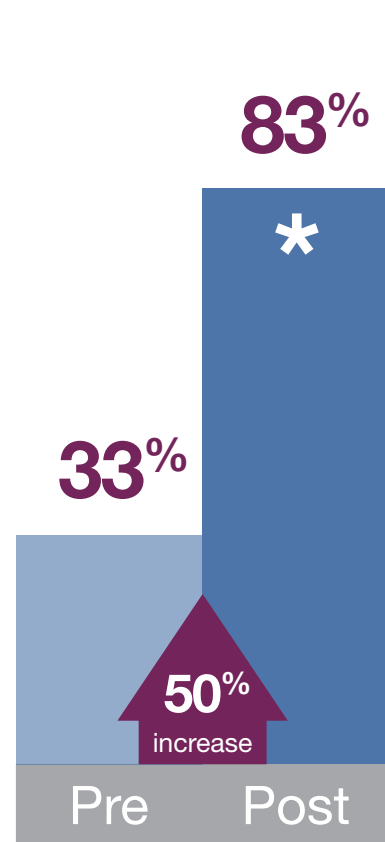


Learner – An HCP who starts the educational content; term is designated only for individuals who have progressed beyond the CME front matter and pre-test, and have started to consume the educational content

*HCP Target Audience: pediatric neurologists, neurologists, pediatricians, neuromuscular specialists, pulmonologists, pediatric gastroenterologists, primary care, Ob-Gyns, physical therapists/occupational therapists, nutritionists, and genetic counselors

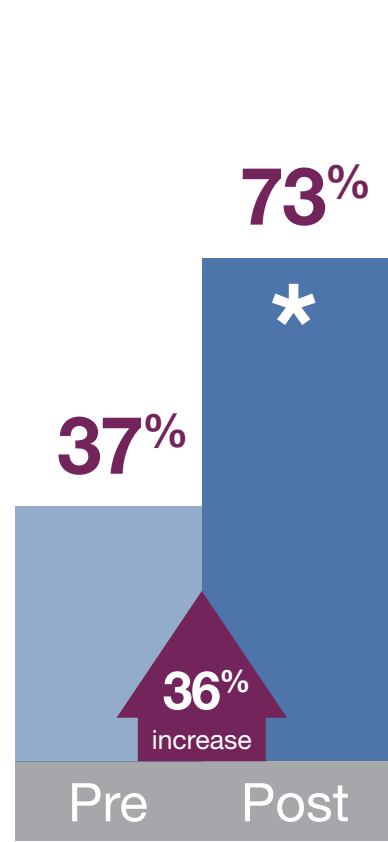
RESULTS: HCP CHANGES IN KNOWLEDGE/COMPETENCE

Which is an FDA-approved therapy for SMA that can be orally administered?



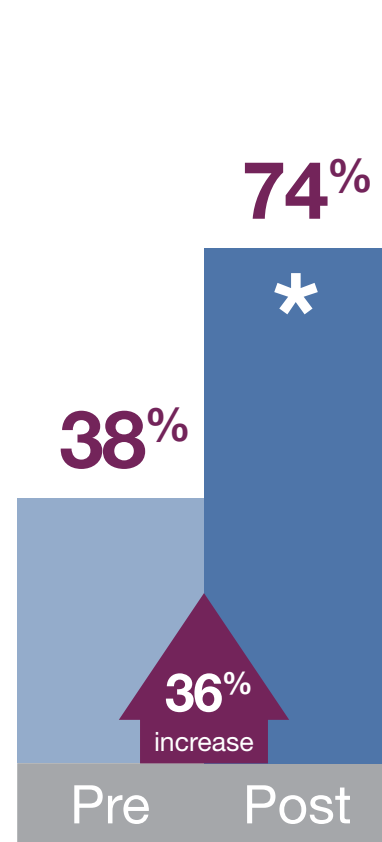
n = 759 pretest, n = 521 posttest;
2-month follow-up survey showed a sustained increase of 26% over pretest
*p<.05

Your SMA patient is pregnant. As her Ob-Gyn or her treating clinician, which statement is true regarding pregnancy and delivery?



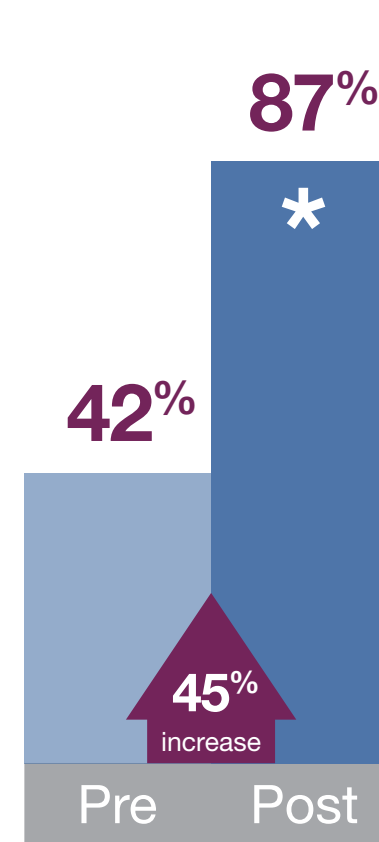
n = 759 pretest, n = 521 posttest;
2-month follow-up survey showed a sustained increase of 22% over pretest
*p<.05

Universal SMA carrier screening is recommended by ACOG to identify:



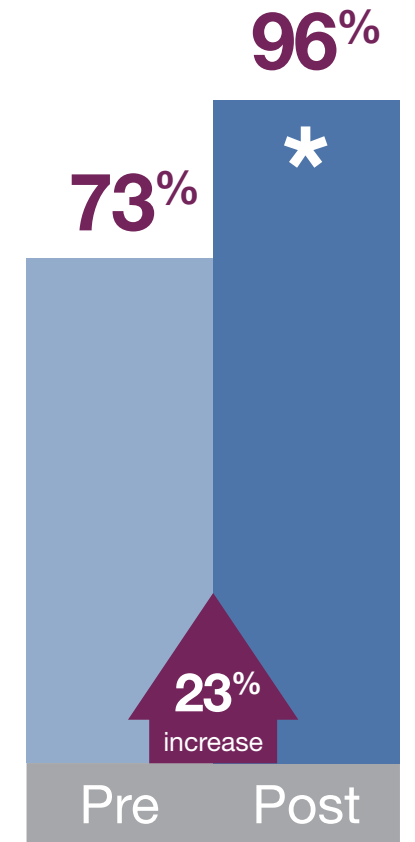
n = 759 pretest, n = 521 posttest
*p<.05

Which describes the mechanism of action of nusinersen?



n = 759 pretest, n = 521 posttest;
2-month follow-up survey showed a sustained increase of 8% over pretest
*p<.05

Which is correct regarding the currently available (DMTs)?



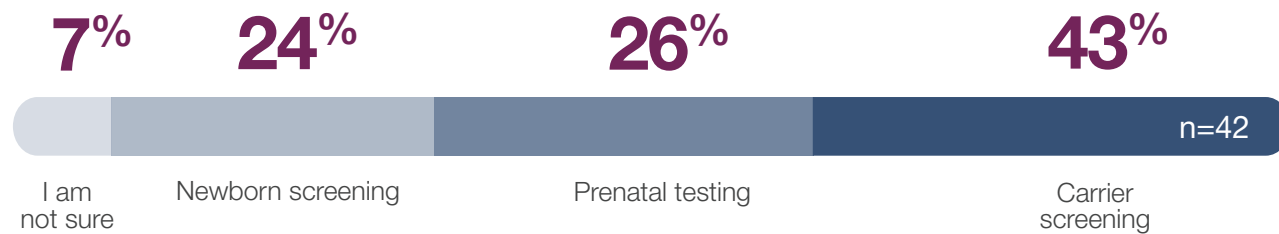
n = 759 pretest, n = 521 posttest;
2-month follow-up survey showed a sustained increase of 9% over pretest
*p<.05

RESULTS: GAPS IN COMMUNICATION BETWEEN HCP AND P/C IDENTIFIED BY TETHERED DESIGN

P/Cs report that HCPs do not discuss pregnancy, fertility, and sexual issues with SMA women. Responses from HCPs to polling, knowledge and competence-based questions may indicate the lack of communication is a result of low knowledge on these topics.

HCP

Which is a recommendation from ACOG regarding SMA testing?



Which is correct regarding fertility and pregnancy for women with SMA?

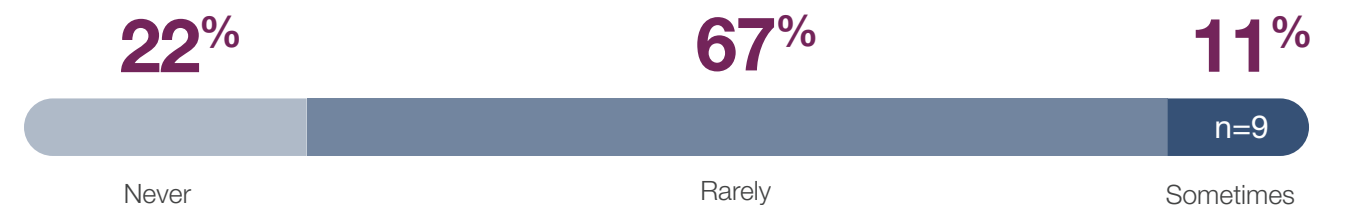


Patient Caregiver

How would you rate the information you received at the time of diagnosis about genetic testing?



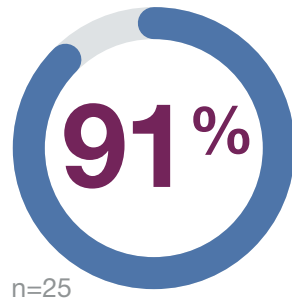
How often do healthcare providers discuss living with SMA and sexual health and intimacy?



CONCLUSIONS

HCP

Positive Impact on Patient Outcomes and Clinical Practice



learners reported the activity positively impacted clinical practice, patient experience/outcomes



29

total write-in examples were shared

I am able to determine which treatments will be appropriate for which patients.

I consider the diagnosis of SMA more frequently.

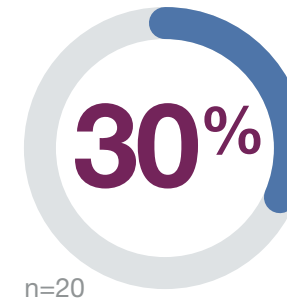
Begun to think more about the importance of establishing transitional care coordination as children age out of pediatric clinical care.

Better utilization of FDA-approved medication for SMA.



Patient/Caregiver

Positive Impact on Health-Related Behavior and Communication

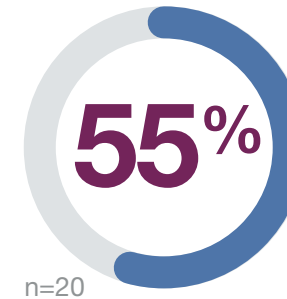


patients/caregivers reported the activity positively impacted communication with HCPs



18

total write-in examples were shared



patients/caregivers reported the activity positively impacted their control of healthcare decisions

I feel more comfortable and confident encouraging my 11-year old and my 8-year old to speak on what they are thinking, what their concerns are, and to advocate for their opinions as well, making sure the care team knows what they are thinking and feeling.

We feel more knowledgeable about deciding which treatment to pick for our newborn and how to care for him after.

Feel more empowered to speak up and ask questions or express concerns.

The options are always changing, and just being aware of all of them is an important step in making the decisions for which treatment path is best for me.



CONCLUSIONS

Data support the positive impacts of tethered live-online HCP and P/C education to prepare both entities for the evolving care needs of children and young adults with SMA who receive DMTs. However, findings also highlight the need for additional education for both groups regarding the role of prenatal screening, perinatal care, sexual health, and pregnancy for women with SMA and to enhance the quality of communication between both entities.



CME
Presentation



Patient/Caregiver
Presentation

For questions or any requests for additional information, please reach out to Carole Drexel, PhD at cdrexel@platformq.com.

