Preparing for a Change in Care Delivery: The Impact on the Health Care Team of Live-Online Education on Gene-Based Therapies

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The FDA has approved a therapy that

employs antisense oligonucleotide to

induce inclusion of a normally excluded

exon and produces a functional

protein. For which condition was that

therapy approved?

(Answer: SMA)

n = 668 pretest, n = 297 posttest

P<0.05

Hematopoietic stem-cell gene therapy is

being applied to treat which conditions?

(Answer: Neurometabolic storage

disorders)

76%

40%





The Evolving Roles of Clinicians

24%



Attitudes of HCPs Regarding Gene-Based Therapy and

What would you like to learn about gene therapy?

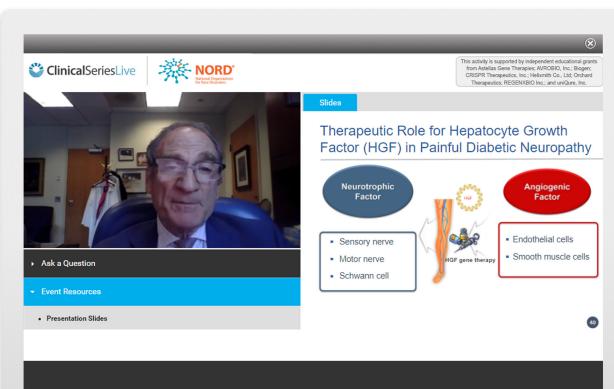
(select up to 2)



INTRODUCTION

Gene therapy has the potential to revolutionize treatment for a broad range of medical conditions, including neurodegenerative and neuromuscular diseases, ophthalmologic disorders, hematologic conditions, and cancers. While several gene therapies have received regulatory approval, the landscape is evolving rapidly with hundreds of others in various stages of investigation. However, patients who may qualify for gene therapy often have limited access to these breakthrough treatments, in part due to multiple knowledge and competence gaps among the clinicians (HCPs) who care for them. We seek to evaluate the impact of live-online education on HCPs' understanding of the principles of

gene-based therapies, awareness of therapies that are available and in development, and recognition of FDA regulatory oversight of gene-based therapy development.



METHODOLOGY

Educational Program and Evaluation Details



Partners

Advocacy groups: NORD, ASGCT Education: PlatformQ Heath, PIM



Interventions

2-day education comprised of four 60-min CME activities for HCPs covering gene-therapy technologies, vectors, and FDA regulations, as well as the need for multidisciplinary care after therapy; included five 10-min "deep-dives" into diseases with available or late-stage investigational gene therapies



Data collected

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



Measurements

Questions asked pre-, immediate post-, and 2 months post-activity. Chi Square tests used for statistical analysis

ACTIVITY DETAILS

The Time Is Now: Understanding Gene Therapy, Applications, and Implications

Learning Objectives:

- Explain the potential of gene-based treatment approaches to change the disease course for patients with genetic conditions
- Discuss different types of current and emerging gene-based therapies
- Summarize the key steps of development programs for gene therapies, identifying how and why some programs may differ from
- Explain the role of the FDA in providing regulatory oversight during the development process
- Cite evidence for the use of gene-based therapies that are approved or pending approval for the management of medical disorders
- Counsel patients about clinical-trial opportunities
- Discuss the role of genetic counselors in educating patients and caregivers on the decision-making processes for access to gene therapy, financial support, and post-treatment care
- Identify specialists and specialty care centers that patients may be referred to for further evaluation

Faculty:

Kevin Flanigan, MD Director, Center for Gene Therapy Robert F. & Edgar T. Wolfe Foundation Endowed Chair in

Stephan A. Grupp, MD, PhD Section Chief. Cellular Therapy and Transplant Director, Cancer Immunotherapy Program

Director, Division of Pediatric Hematology, Oncology, and Stem Cell Transplant

John Kessler, MD Davee Professor of Stem Biology Davee Department of Neurology

Julie A. Parsons, MD Haberfeld Family Endowed Chair in Pediatric Neuromuscular Disorders Children's Hospital Colorado



Perry B. Shieh, MD, PhD, FAAN Professor of Neurology and Pediatrics David Geffen School of Medicine



Kimberly Goodspeed, MD Assistant Professor of Pediatrics, Neurology Division University of Texas Southwestern Medical Center Dallas



John F. Tisdale, MD Senior Investigator Cellular and Molecular Therapeutics Branch Intramural Research Program

Session components included downloadable resources, live polling, and audience real-time Q&A.

RESULTS

Learner Demographics



target audience learners*



monthly visits of patients who may be candidates for a gene-based therapy

Learner Engagement



total

learners

certificates awarded





*Target Audience: Primary care, internal medicine, pediatrics, pulmonology, cardiology, endocrinology, gastroenterology, hepatology, neurology, neuromuscular, infectious disease, obstetrics/gynecology, rheumatology, dermatology, hematology, oncology, neonatology, genetic counselors, social work, psychology, nurse practitioners, physician assistants, nurses, pharmacists

Positive Impact on Ability to Identify a Patient with a Condition for Which a Gene-based Therapy is Being Developed



Improved ability to counsel

patients regarding the

continuous advances in gene

therapy

I'm trying to get prepared

and find a genetic counselor

for my patients who can

afford treatment

learners reported the activity positively impacted their ability to identify patients who may benefit from gene therapy



Discussed with patients and

families ways to stay

educated regarding the

latest research

I provided new treatment

ideas for my patients and

directed them to appropriate

clinics

shared

Examples of diseases for which HCPs have discussed gene therapy as a treatment option:

- Spinal muscular atrophy (SMA) Duchenne muscular dystrophy
- Amyotrophic lateral sclerosis
- Hemophilia
- Seizure disorders
- Cystic fibrosis (CF)
- Retinitis pigmentosa

- Application to patients
- CRISPR use and regulation in
- FDA-approved therapies and those in clinical trials that may be
- Available centers, cost of treatment, success rate, and complications
- Gene therapy for congenital kidney diseases

Gene therapy is discussed

more frequently as a

treatment option

Changes in Knowledge/Competence: General Knowledge and Disease-/Technology-specific Knowledge

CRISPR (clustered regularly interspace palindromic repeats)/Cas9 is: (Answer: Gene-editing technology)

75%

42%

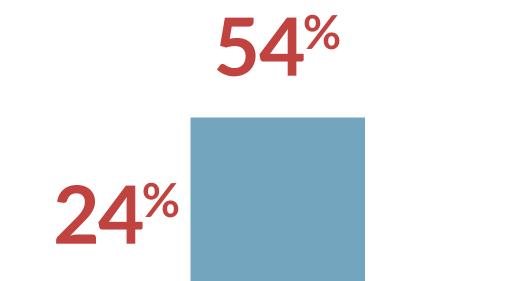
Additional education needed on:

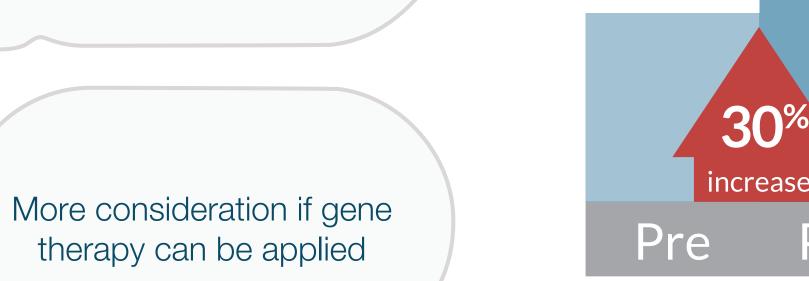
- Combination therapies
- Role in diabetes mellitus Clinical orientation
- Muscle disorders
- The ethics of gene therapy
- Long-term safety data and historical genetic studies to now
- approved in the future
- How to find more resources for patients going through therapy

technology that age-related macular degeneration, mucopolysaccharidoses (MPS I, II, III A/B and VI), Fabry disease, and hemophilia have in common? (Answer: AAV)

Pre Post

n = 668 pretest, n = 297 posttest





increase

n = 392 pretest, n = 208 posttest

P<0.05

Pre Post

33% increase 66%

Your patient who has diabetes asks

about clinical trial opportunities with

gene therapy for his neuropathic pain.

What can you tell them?

(Answer: Clinical trial with a plasmid)

22%

n = 392 pretest, n = 208 posttest

one-time administration for

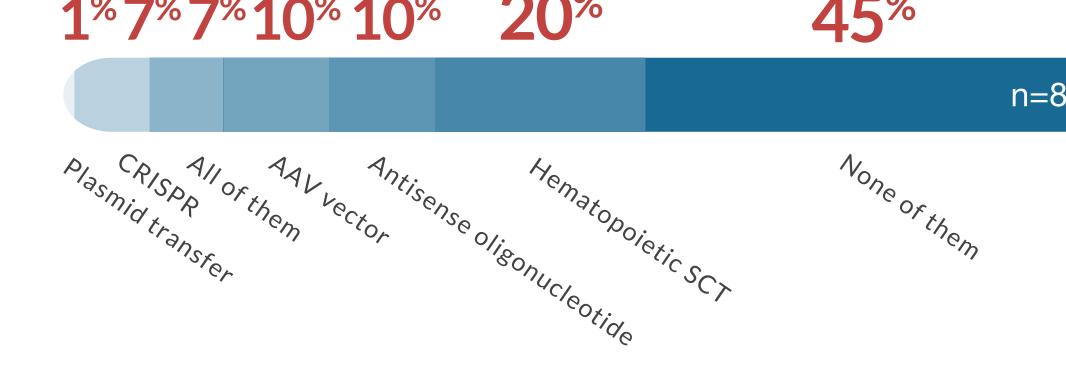
hemophilia B?

69%

(Answer: Etranacogene dezaparvovec)

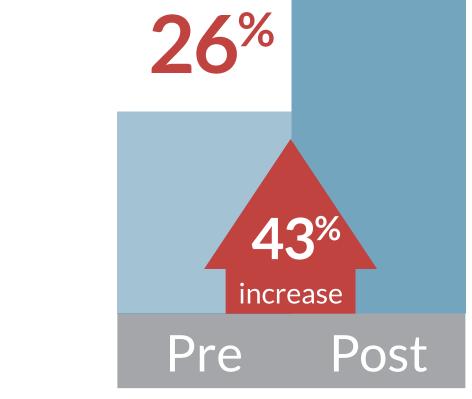
1%7%7%10%10% 20% following available or under-investigation agents requires a

10%



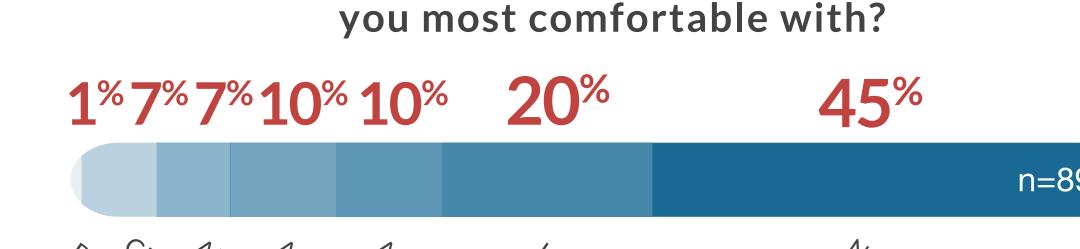
CONCLUSION

Following the educational initiative, clinicians demonstrated large improvements in their knowledge and competence in critical areas related to gene therapies, resulting in enhanced ability to discuss the topic with their patients. Our data also point to a continuing need for education, especially as medical experience with gene therapy grows.



n = 390 pretest, n = 193 posttest n = 392 pretest, n = 208 posttest

How confident are you explaining what gene therapy is and does to your patients? 66%



Which of the following gene therapy techniques are