

Tackling Care Deserts in Rett Syndrome: A Multidisciplinary, Multi-phase, Geotargeted Initiative to Bridge the Gap Between Centers of Excellence and Community-based Clinicians

Authors: Vandana Gupta¹; Carole Drexel¹; Alissa Stone¹; Katie Kowalski²; Melissa Kennedy³; David Lieberman⁴; Henry Hasson⁵
 Affiliation: ¹PlatformQ Health Education, Needham, MA; ²National Organization for Rare Disorders (NORD), Washington DC; ³International Rett Syndrome Foundation (IRSF), Cincinnati, OH; ⁴Boston Children's Hospital, Boston, MA; ⁵Sheepshead Bay, NY

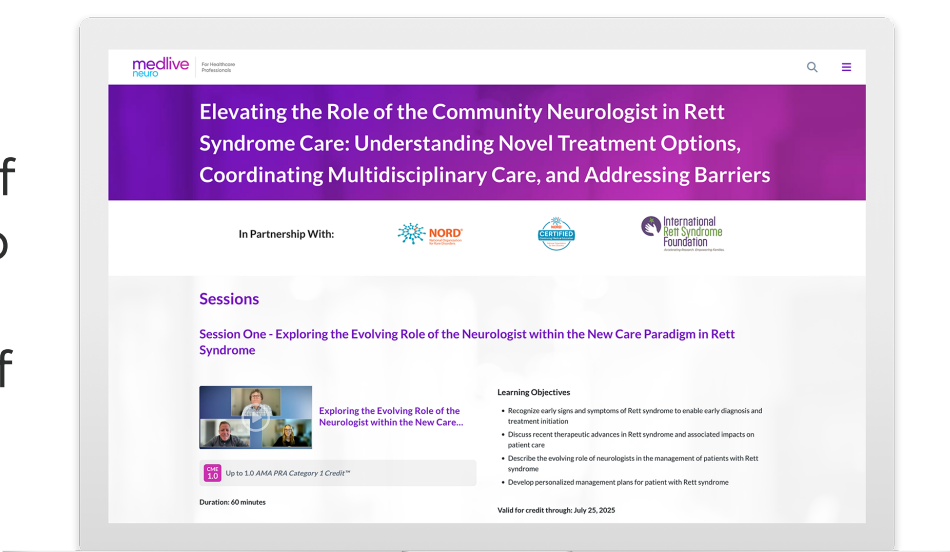


This activity is supported by an independent educational grant from Acadia Pharmaceuticals Inc.



INTRODUCTION

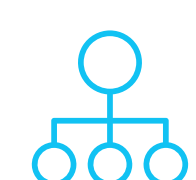
Purpose: To enhance community-based clinicians' ability to diagnose Rett syndrome (RTT) in a timely manner, enhance awareness of newly approved therapies, provide strategies to effectively leverage team-based approaches, and foster greater collaboration with Centers of Excellence (CoEs) through continuing medical education.



Background: The first treatment for RTT was approved in March 2023, with other investigational agents on the horizon. However, the number of patients and families requiring RTT care exceeds the current capacity of RTT CoEs. Many community-based clinicians, including neurologists, would benefit from education that prepares them for this change to the standard of care.

METHODOLOGY

Educational Program and Evaluation Details



Interventions

PQHE partnered with NORD and IRSF on this three-phase initiative for the multidisciplinary, community-based RTT care team. The education was nationally broadcast. Geotargeted marketing efforts facilitated participation from health care professionals (HCPs) in four "care deserts" across the United States, as identified by IRSF. Care deserts are areas where RTT care is sparse and not easily accessible for patients. Phase 1 was an educational activity designed to set the foundation of RTT recognition, diagnosis, and treatment. Phase 2 was composed of four discussions between community-based HCPs and COE experts in each identified "care desert." Phase 3 consisted of a multidisciplinary panel discussion of lessons learned from the previous phases and best practices for enhancing RTT care in community settings.



Partners

Advocacy groups: International Rett Syndrome Foundation (IRSF) and National Organization for Rare Disorders (NORD)
Education: PlatformQ Health Education (PQHE) and Global Education Group



Data Collected

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



Measurements

Questions asked before and immediately after participating in an activity and qualitative interviews. Chi Square tests were used for statistical analysis.

Phase 1: Exploring the Evolving Role of the Neurologist within the New Care Paradigm in Rett Syndrome (launched February 27, 2024)

Learning Objectives:

- Recognize early signs and symptoms of RTT to enable early diagnosis and treatment initiation
- Discuss recent therapeutic advances in RTT and associated impacts on patient care
- Describe the evolving role of neurologists in the management of patients with RTT
- Develop personalized management plans for patients with RTT

Phase 2: Teaming Up to Treat Rett Syndrome: The Importance of Fostering a Multidisciplinary Approach to Patient Care

Four regionally targeted activities:

- Northeast (launched April 25, 2024)
- Southeast (launched May 3, 2024)
- Pacific Northwest (launched May 15, 2024)
- Southwest (launched July 11, 2024)

Learning Objectives:

- Summarize the components of the multidisciplinary approach for RTT
- Discuss the need for enhanced neurologist-gastroenterologist collaborations for optimal adverse-event monitoring and management
- Apply relevant strategies to facilitate team-based care in RTT in the community settings
- Recognize where new treatments fit in the multidisciplinary care of RTT

Phase 3: Sharing Real-World Experiences with New Rett Syndrome Therapies (launched July 24, 2024)

Learning Objectives:

- Recognize early signs and symptoms of RTT to enable early diagnosis and treatment initiation
- Discuss recent therapeutic advances in RTT and associated impacts on patient care
- Describe the evolving role of neurologists in the management of patients with RTT
- Develop personalized management plans for patients with RTT

Faculty

Amitha Ananth, MD
 Assistant Professor
 Department of Pediatrics
 University of Alabama Birmingham

Dallas Armstrong, MD
 Medical Director,
 Rett and Related Disorders Clinic
 Children's Health
 Assistant Professor,
 Child Neurology Division
 University of Texas Southwestern
 Medical School

Theresa Bartolotta, PhD, CCC-SLP
 Professor of Speech-Language Pathology
 Thomas Jefferson University
 Parent of a Child with Rett Syndrome

Henry Hasson, MD
 Child Neurologist

Abigail Jordan, PT, DPT
 Physical Therapist
 Tender Ones Therapy Services

Karen Keough, MD
 Medical Director
 Pediatrix Medical Group

Lauren Lazar, MD
 Assistant Professor of Pediatrics
 Division of Pediatric Gastroenterology,
 Hepatology, and Nutrition
 University of Texas Southwestern
 Medical School

David N. Lieberman, MD, PhD
 Child Neurologist
 Boston Children's Hospital

Kathleen J. Motil, MD, PhD
 Associate Professor in Neurology
 Director, Katie's Clinic for Rett
 Syndrome
 UCSF Benioff Children's
 Hospital-Oakland

Vivien Nguyen, MD
 Associate Professor in Neurology
 Department of Pediatric
 Gastroenterology,
 Hepatology, and Nutrition
 UCSF Benioff Children's
 Hospital-Oakland

Jennifer Martelle Tu, MD, PhD
 Associate Professor in Neurology
 Director, Katie's Clinic for Rett
 Syndrome
 UCSF Benioff Children's
 Hospital-Oakland

Taylor Woodfin, MD
 Attending Physician
 Pediatric Gastroenterology
 Associates

Kimberly L. Limbo, MD
 Child Neurologist
 Huntsville Hospital Pediatric
 Neurology

Carmen Luna, MPH
 Clinical Network Manager
 International Rett Syndrome
 Foundation

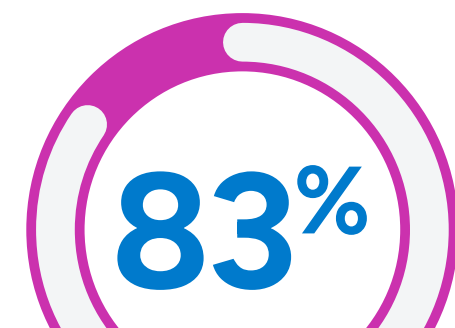
Kathleen J. Motil, MD, PhD
 Associate Professor in Neurology
 Director, Katie's Clinic for Rett
 Syndrome
 UCSF Benioff Children's
 Hospital-Oakland

RESULTS

Learner Demographics (as of August 2024)



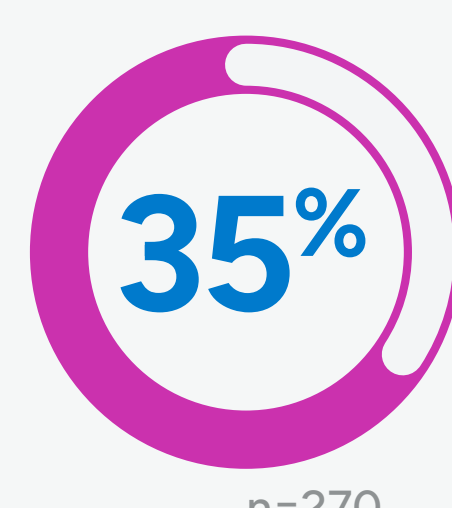
2,300
Total learners



83%
of total learners were MDs/DOs/NPs/PAs



65%
see 5 or fewer patients with RTT each month

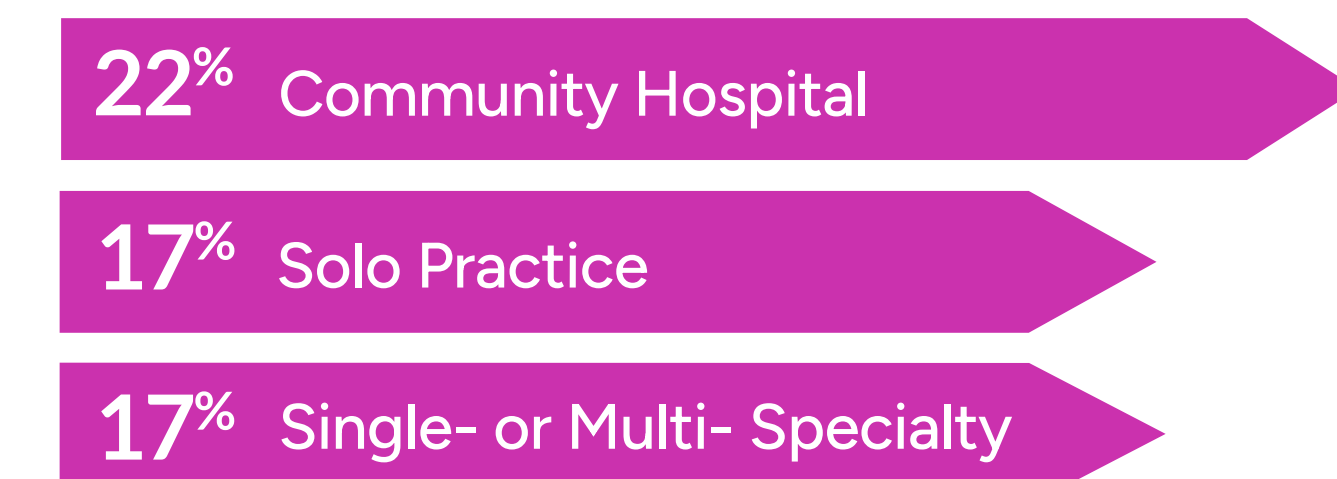


35%
see more than 5 patients with RTT each month

Specialty:



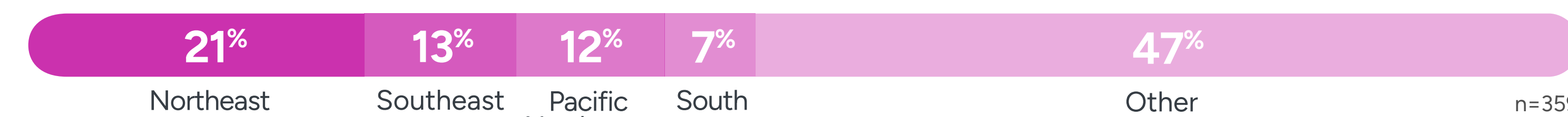
Practice Type: Community-based



Practice Type: Academic-based

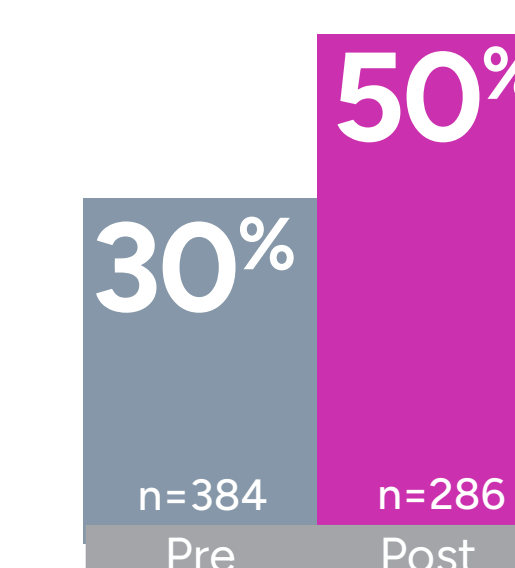


Region of the United States:

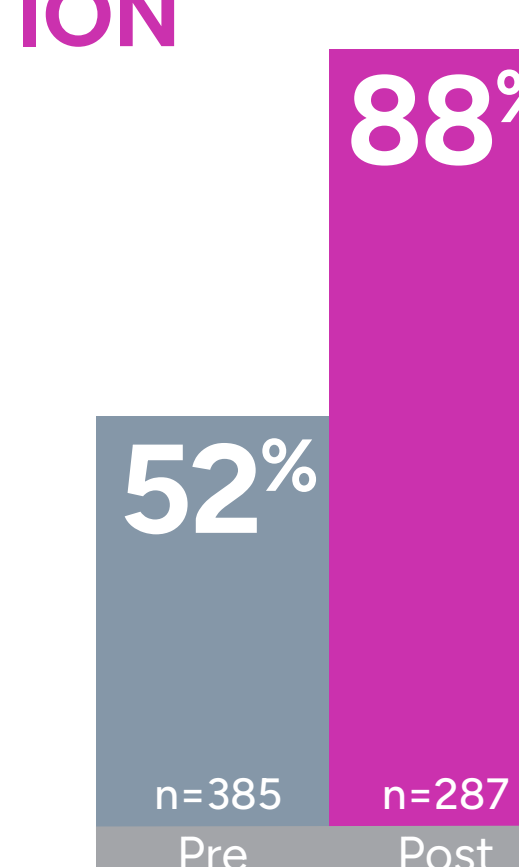


PHASE 1: IMPACT OF EDUCATION

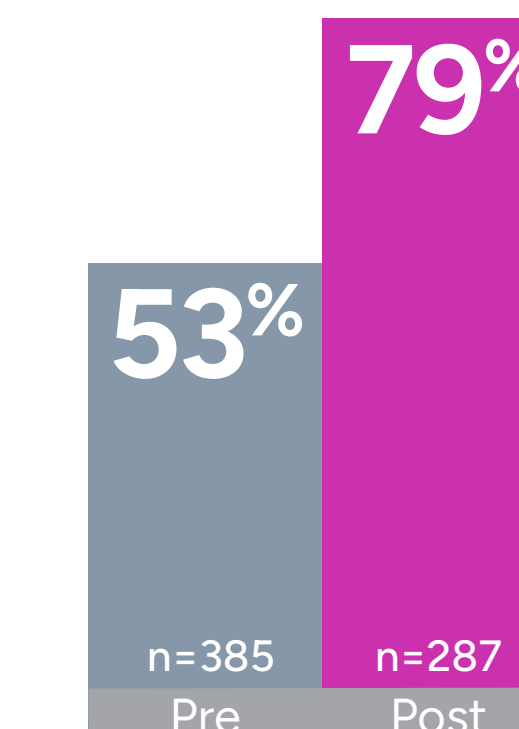
Part 1 Overall Score (all learners)



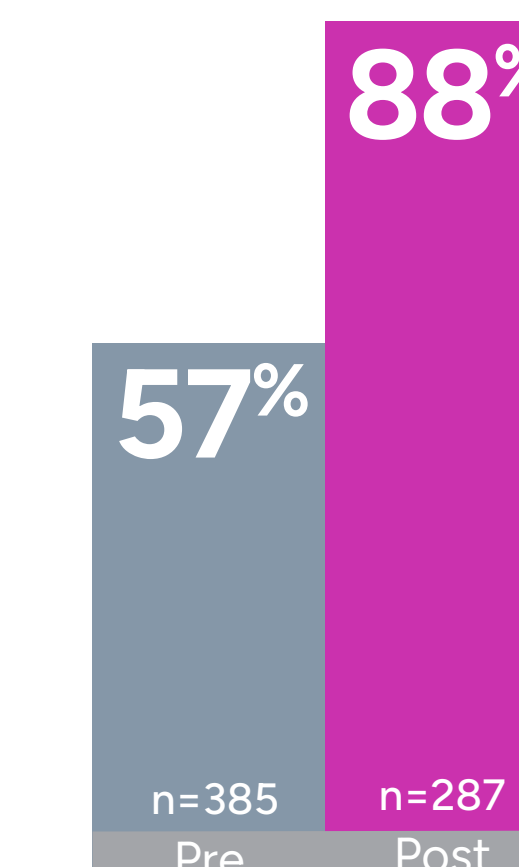
Percent correct
 Average score (%) on 2 items linked to learning objectives



Confidence in counseling caregivers and patients about newly approved and emerging therapies

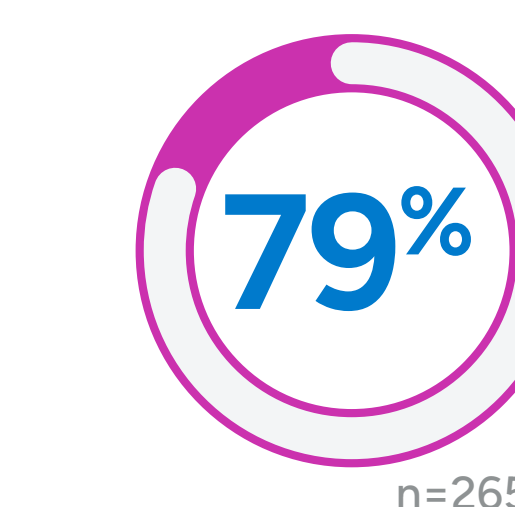


Confidence in developing treatment plans that incorporate newly approved treatments



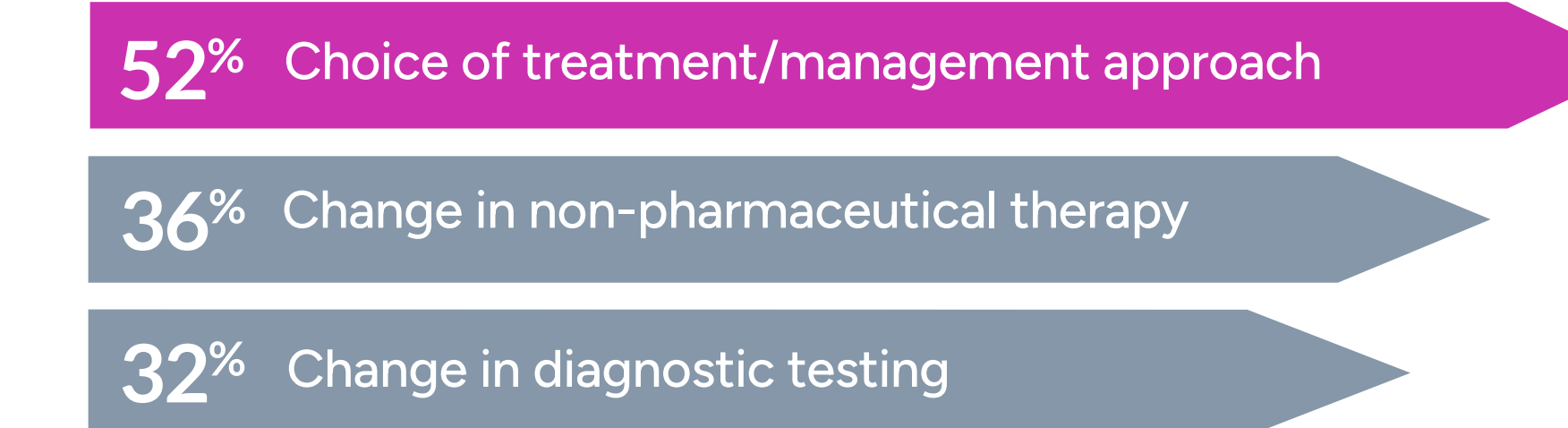
Agreement that trofinetide can be administered to children aged 2 years and older with an RTT diagnosis

After participating in the education:



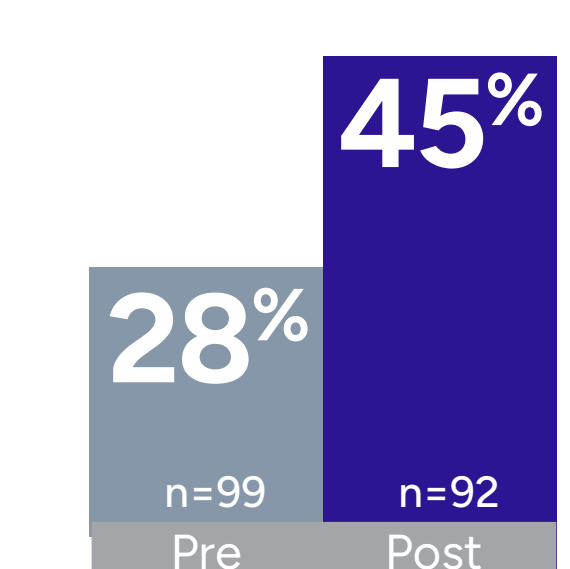
intend to implement a change in their practice

The Top 3 intended practice changes were:

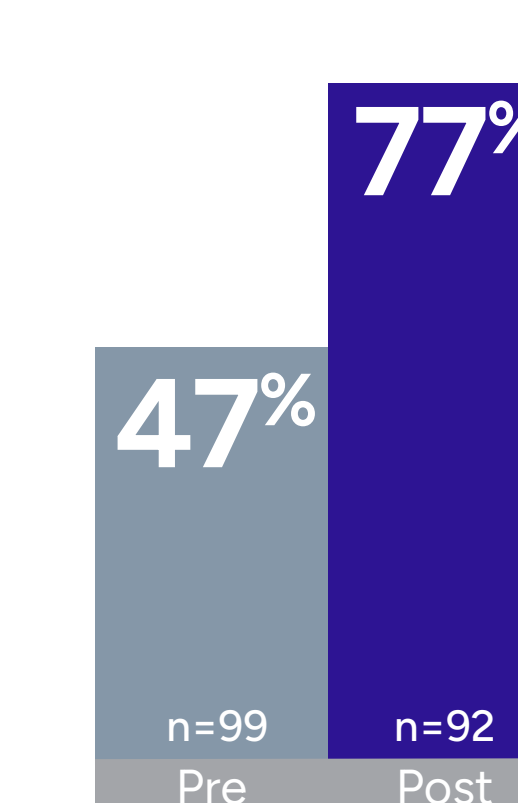


PHASE 2: IMPACT OF EDUCATION

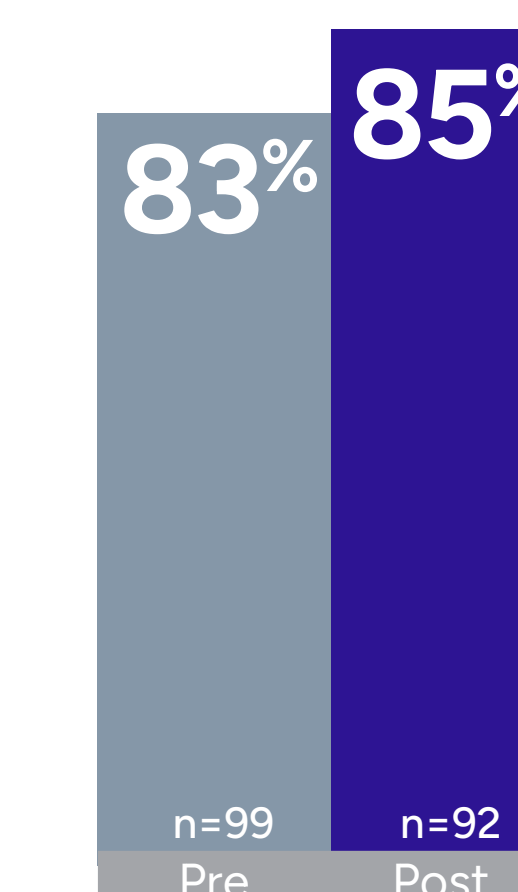
Part 2 Overall Score (all learners)



Percent correct
 Average score (%) on 3 items linked to learning objectives

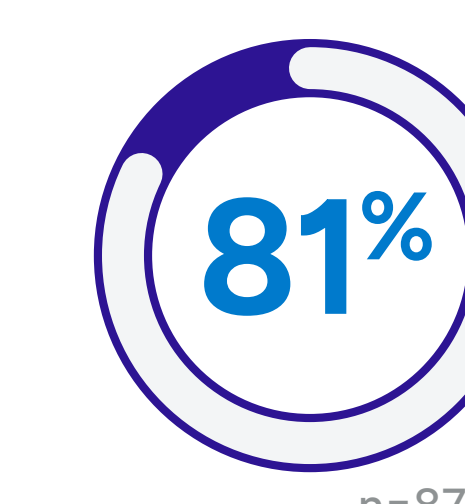


Familiarity with how the multidisciplinary needs of patients with RTT can be addressed



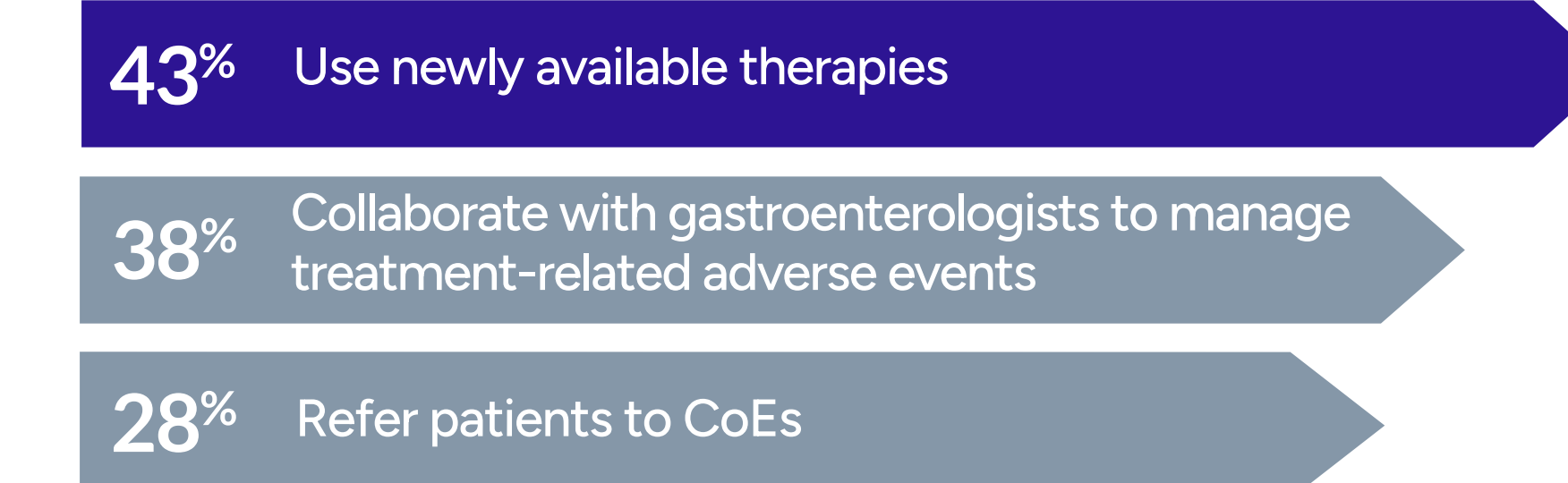
Likelihood of collaborating with a gastroenterologist to manage treatment-related adverse events in patients with RTT

After participating in the education:



intend to implement a change in their practice

The Top 3 intended practice changes were:

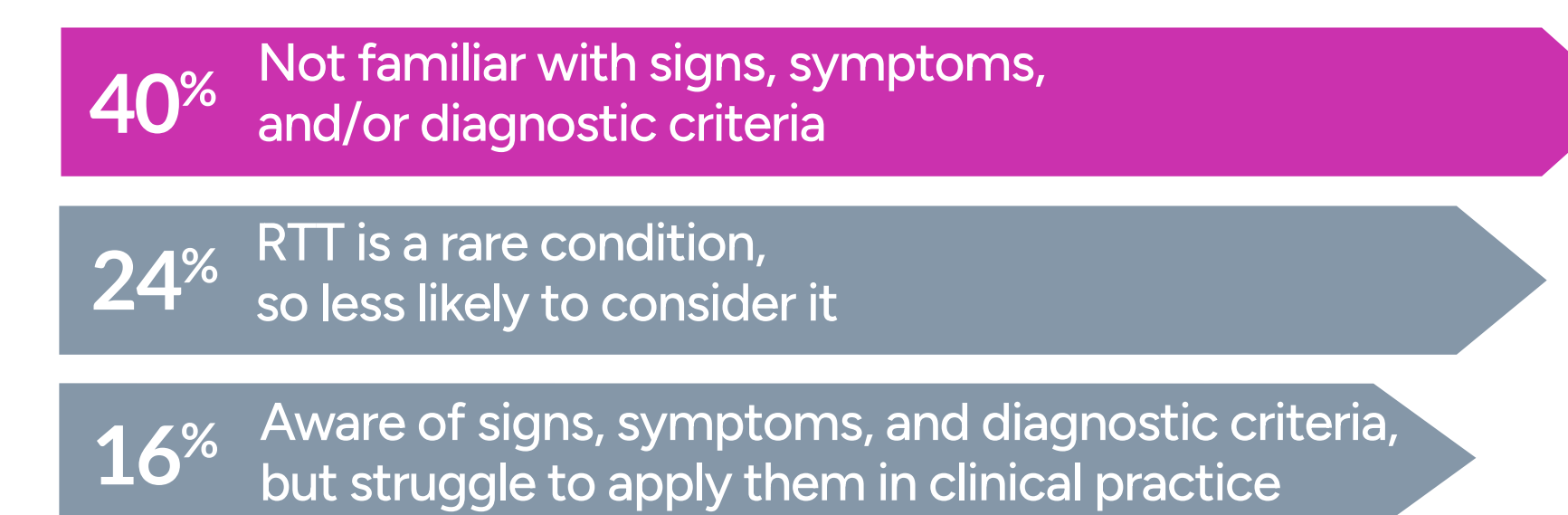


PHASE 3: IMPACT OF EDUCATION

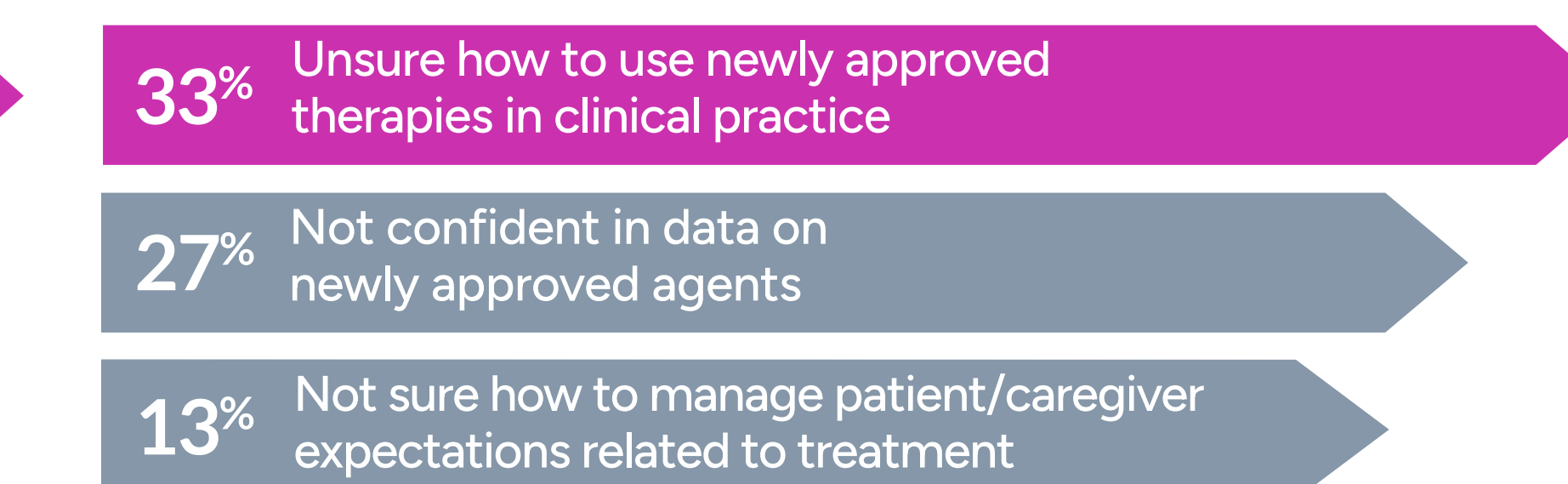
Data collection for Phase 3 is ongoing.

Barriers and Challenges in RTT Care

The Top 3 challenges when diagnosing RTT:



The Top 3 challenges when treating RTT:



CONCLUSION

To date, practice data before and after the education and intent-to-change data support the effectiveness of the educational initiative in addressing attitudinal and knowledge gaps related to RTT care among community-based neurologists in identified care deserts. Challenges in the diagnosis and treatment of RTT underscore a need for targeted continuing education among clinicians who care for people with RTT. Findings also highlight the importance of enhanced systemic change to facilitate the adoption of new therapies and ensure greater access to care for people with RTT.