

Supporting Earlier Recognition and Safer Management of Rett Syndrome Through Case-based Education

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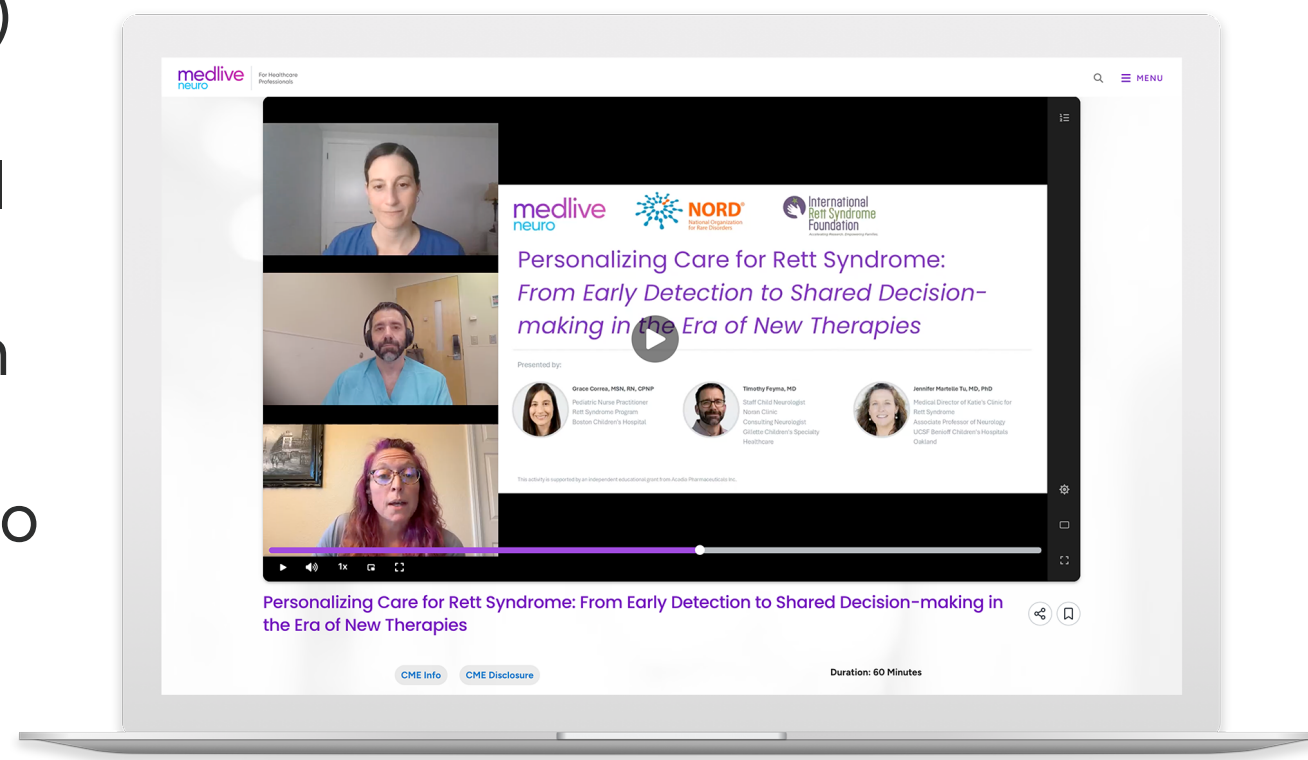


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INTRODUCTION

Rett syndrome (RTT) is a complex neurodevelopmental disorder that can be difficult to identify in the early stages. Management has also become more challenging with the availability of trofinetide, which requires informed treatment selection, caregiver counseling, adverse-event (AE) mitigation, and coordinated multidisciplinary care. Gaps in clinician knowledge and confidence can contribute to delayed diagnoses, fragmented care, and greater burdens on families. A case-based CME initiative was designed to improve clinician readiness to recognize and manage RTT. The education was intended for pediatric neurologists, neurologists, gastroenterologists, advanced practice providers, speech therapists, physical therapists, occupational therapists, and pediatricians.



METHODOLOGY

Educational Program and Evaluation Details

Intervention

A multidisciplinary faculty developed a curriculum featuring a foundational CME module and a three-part, case-based multidisciplinary CME track, supported by targeted LinkedIn micro-learning. Content addressed the early identification of RTT, evidence on trofinetide and emerging therapies, the management of AEs through neurology-gastroenterology collaborations, and the integration of speech, physical, and occupational therapies.

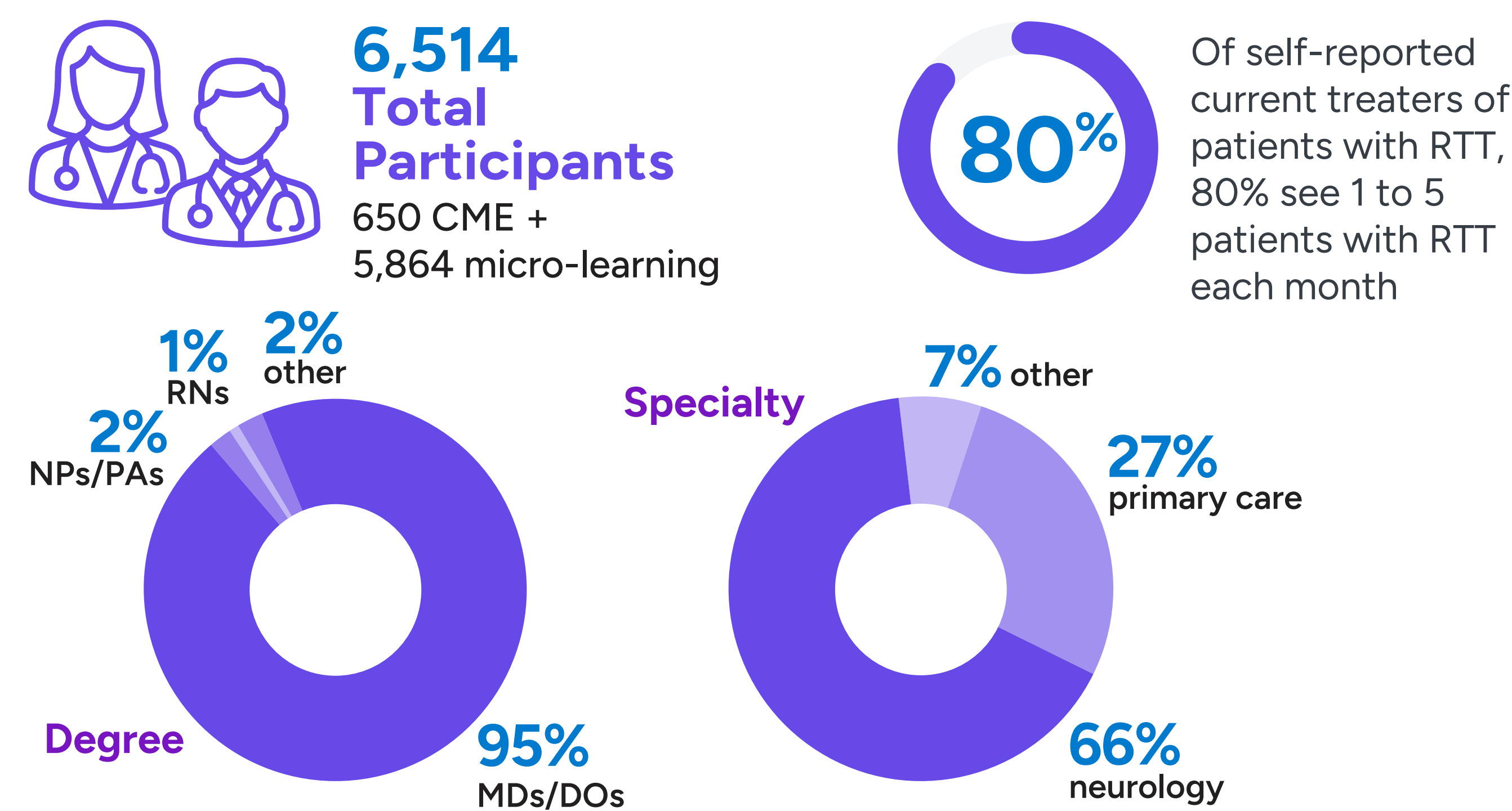
Data Collected

A survey-based approach measured the impact of each CME activity as aligned with its stated learning objectives via pre- and post-activity knowledge/competence questions developed in accordance with National Board of Medical Examiner guidelines,¹ as well as a post-activity evaluation (inclusive of intended practice changes: open-ended). Statistical tests of significance were applied to comparisons of individual questions (pre- vs post-activity).

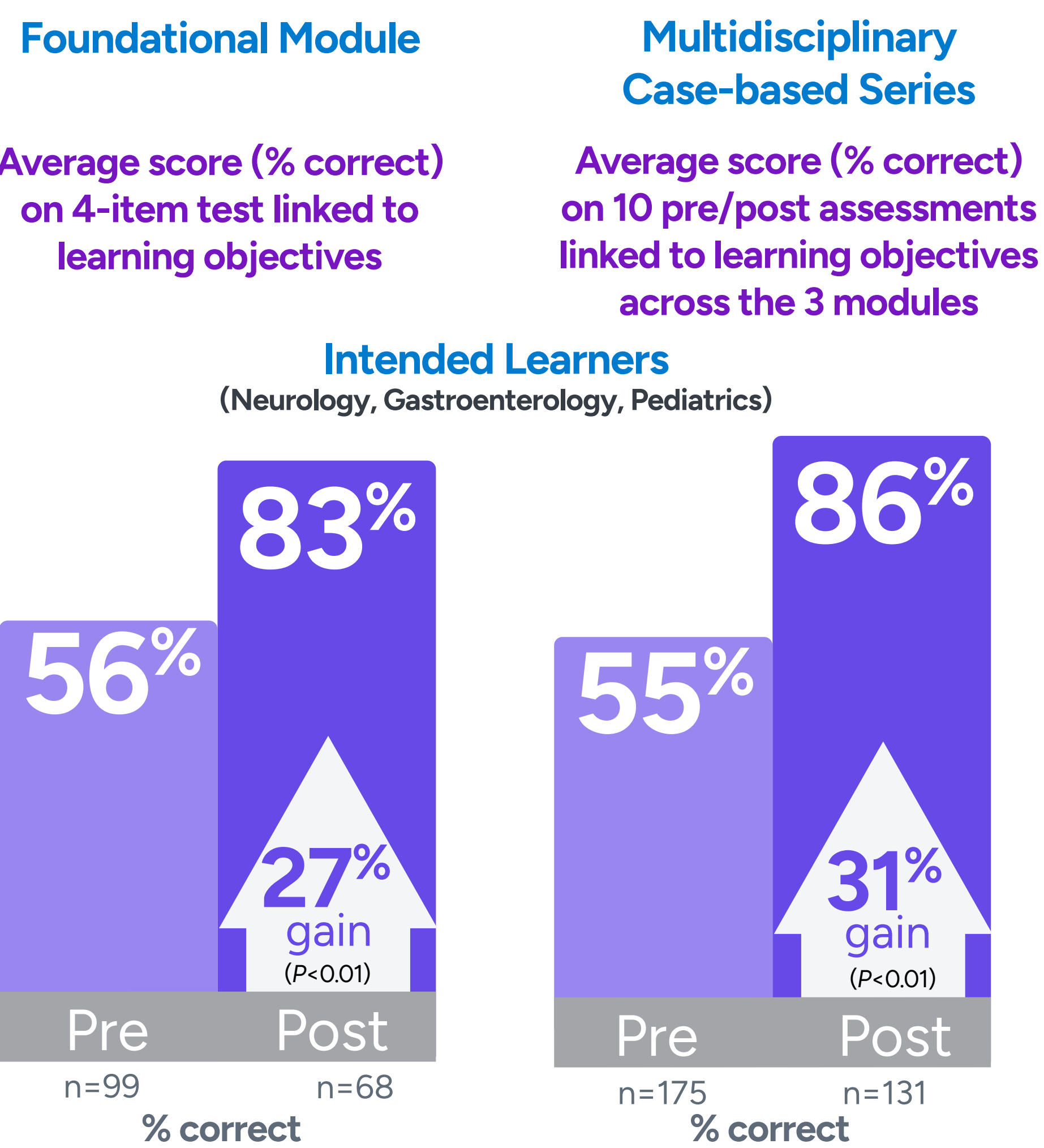
Reference:
 1. NBME Item-Writing Guide. Constructing written test questions for the health sciences. Nov 2020. www.nbme.org/item-writing-guide.

RESULTS

Foundational Module



Among intended learners, average scores improved from 56% to 83% for the foundational module and from 55% to 86% for the case series.

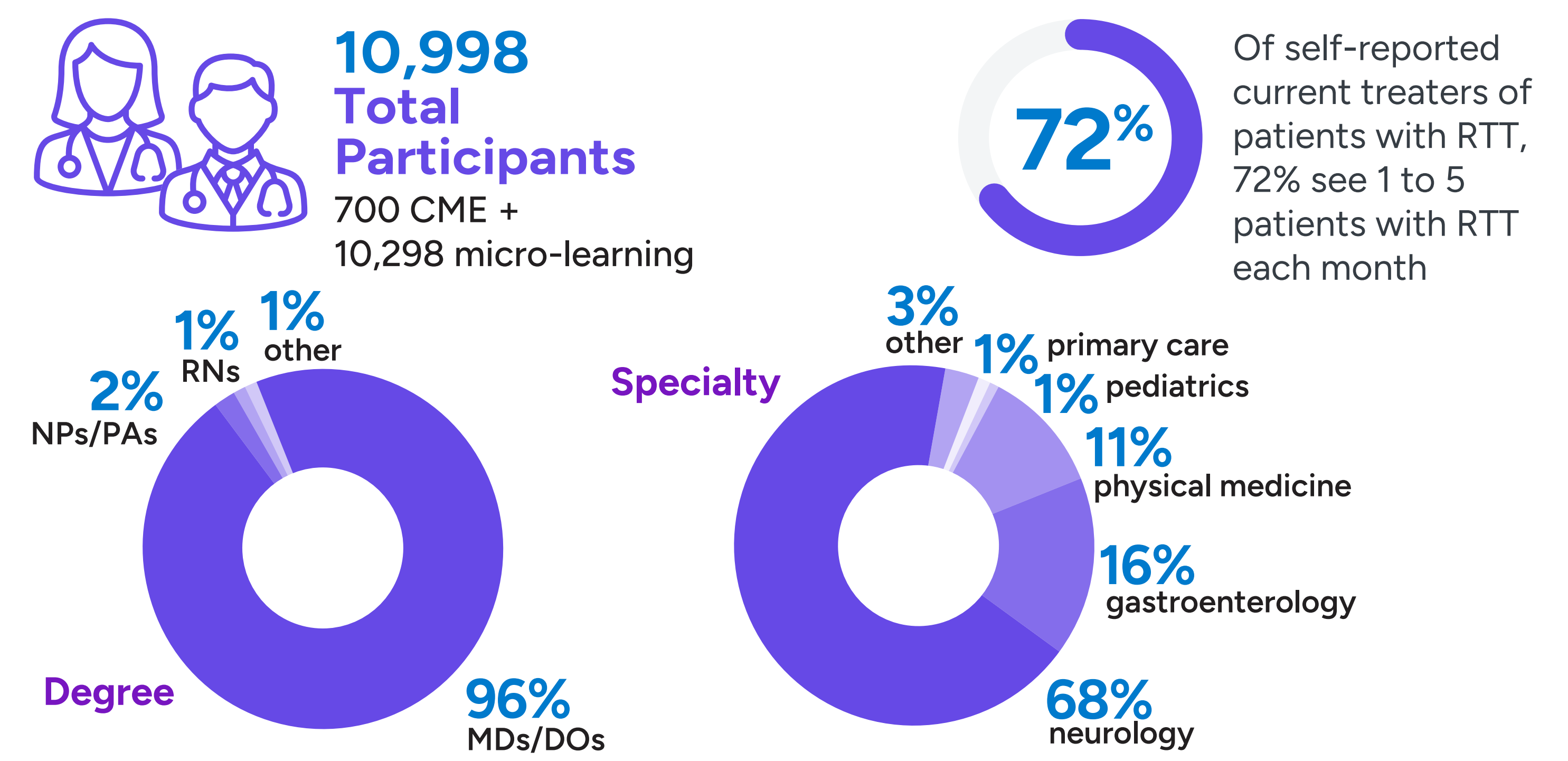


74% of learners in the foundational module and

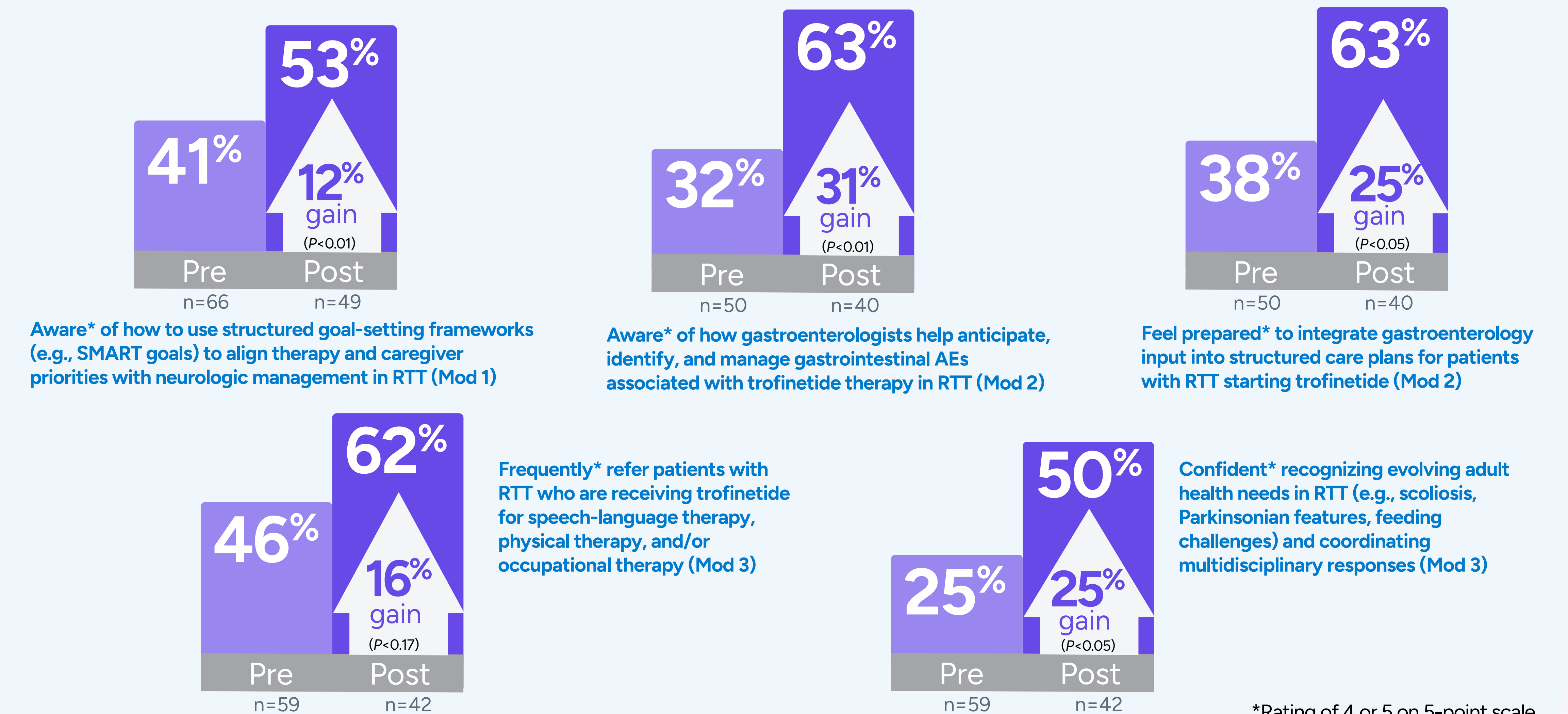
80% in the case-based CME series reported that the education would improve their clinical practice.

The top 3 intended practice changes among foundational module learners were:	Percentage
Increased collaboration with other members of the care team	53%
Choice of treatment approach	24%
Change in non-pharmaceutical therapy	12%

Three-part, Multidisciplinary, Case-based CME Series



Learners also reported greater confidence in earlier recognition of RTT, safer use of trofinetide, caregiver counseling, and multidisciplinary referrals/collaborations.



*Rating of 4 or 5 on 5-point scale

CONCLUSION

This initiative demonstrated meaningful practice-relevant impacts on RTT care, including supporting earlier diagnoses, safer treatment implementation, more coordinated care, and improved family-centered management.

Learners in the case series reported 41 (open-ended) intended practice changes, which fell into the following categories:

- Implement new treatment options
- Increased use of a multidisciplinary approach, including GI care
- Management approaches that incorporate speech therapy, physical therapy, and occupational therapy
- Better/earlier referrals