

A New Era in the Management of Rett Syndrome: Are Neurologists Ready for a Changing Management Landscape?

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INTRODUCTION

The development of new therapies for Rett syndrome (RTT) makes it imperative that HCPs diagnose affected children early so they can benefit from multidisciplinary care and breakthrough treatments. The objective of this initiative was to assess the influence of two years of continuing medical education (CME) on clinicians' (HCPs') ability to recognize key signs of RTT, confirm its diagnosis, and evaluate the place of emerging treatments in the standard of care.





Partners

Advocacy groups: National Organization for Rare Disorders (NORD), International Rett Syndrome Foundation

Education: PlatformQ Health Education, Global Education Group



Interventions

The first 60-minute CME activity was launched live-online on 4/1/21 and expired on 4/1/2022 (year 1). The second activity launched on 8/18/22 and will remain on-demand until 8/18/23 (year 2).



Data Collection

Test questions were administered pre, immediately post, and 2 months post activity.



Measurements

Responses from polling and surveys were analyzed for engagement, lessons learned, and continuing gaps. Chi-square compared responses (pre/post and pre/2 mos, $P < 0.05$).

Session #1: Rett Syndrome: Early Recognition and Emerging Agents to Reduce Disease Burden

Educational topics:

1. Early signs and symptoms of Rett syndrome
2. Therapeutic potential of agents currently being investigated as treatments
3. The need for coordinated, comprehensive care

Faculty (For both sessions)



Cary Fu, MD

Child Neurologist and Epileptologist
Medical Director, Vanderbilt Rett Syndrome Clinic
Vanderbilt University Medical Center
Department of Pediatrics



David N. Lieberman, MD, PhD

Child Neurologist
Director, Comprehensive Rett Syndrome Clinic
Department of Neurology
Boston Children's Hospital



Paige Nues

Director of Family Empowerment
International Rett Syndrome Foundation (IRSF)

Session #2: Rett Syndrome: Hope for Patients and Caregivers

Educational topics:

1. Early signs and symptoms of Rett syndrome
2. Place of genetic testing in diagnosis
3. Symptomatic, multidisciplinary management
4. Emerging agents



RESULTS

Learner Demographics



865

Total learners



70%

Clinician*
(MD/DO/NP/PA)



27%

Treaters who see an avg of **12**
Rett syndrome patients/week**

Specialty*

15% Neurology

20% PCP (FM/GM/IM)

10% Pediatrics

3% Psychiatry

1% Genetics

Learner Engagement



949

Average
minutes viewed
(activity length 60 minutes)



108

Slide
downloads



581

Matched
pre/post responses



51

Follow-up
survey responses

Source: *registration (n = 865) and **evaluation data (n = 428)

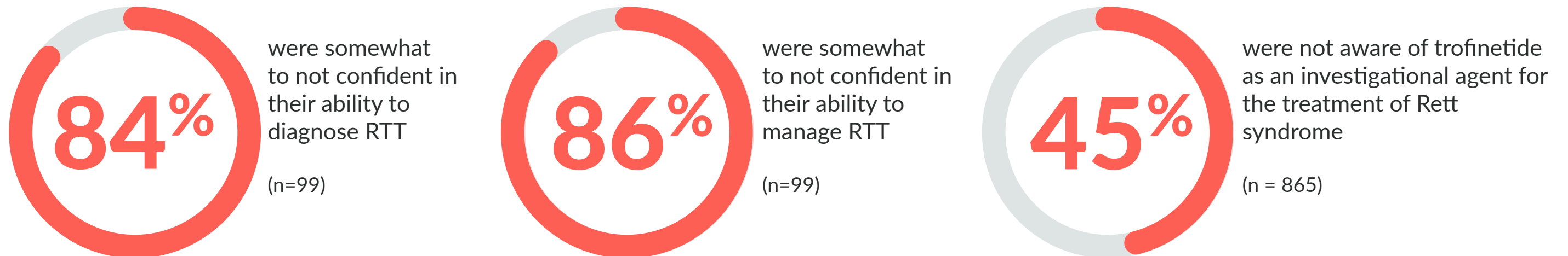
RESULTS

Changes in Knowledge/Competence

| Topic Areas | Symptoms of RTT (n) | Multidisciplinary care (n) | Emerging agents (n) |
|---------------|---------------------|----------------------------|---------------------|
| Pre activity | 56% (865) | 54% (868) | 47% (865) |
| Post activity | 90%*(581) | 81%*(579) | 81%*(581) |

* P<0.05, pre vs post. Baseline performance levels from activity 1 and 2 did not differ significantly.

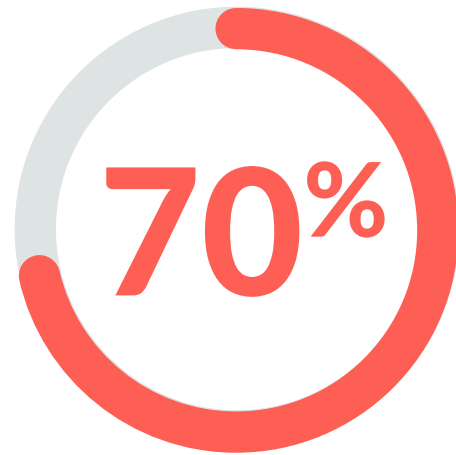
Provider Insights Entering the Activity



Positive Impact on Clinical Practice and Patient Experience



reported a positive impact on their clinical practice



are more comfortable (post-activity) in their ability to discuss investigational therapies with their patients

(n=353)



believed that participation in this activity had a positive impact on their patients



19

respondents identified a practice change, primarily related to *improved recognition of Rett syndrome* (but not otherwise specified)



18

respondents identified a positive patient outcome, primarily related to *improved management and QoL* (but not otherwise specified)

"I made a new diagnosis of Rett syndrome in a new patient that was referred to me."

CONCLUSION

Data support the positive impact of live-online CME to prepare HCPs to care for patients with RTT. However, baseline knowledge and competence were similar in years 1 and 2, indicating that there is a need for additional education to induce lasting changes in the care of RTT.

