

Treatment Utilization of Trofinetide across Age Categories of ≤20 years versus >20 years among Individuals with Rett Syndrome: Descriptive Analysis of Real-World Data in the United States

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BACKGROUND

- Rett syndrome (RTT) is a rare and progressive neurodevelopment disorder which is estimated to be 1 out of every 10,000-15,000 births worldwide [1].
- Trofinetide (TROF) was approved by the US FDA in March 2023 as the first and only treatment for RTT in adults and pediatric patients 2 years of age and older [2].
- TROF clinical trial program included only individuals aged 2-20 years with a RTT diagnosis. Therefore, it is important to understand real-world TROF utilization patterns among those >20 years of age.

OBJECTIVES

- This real-world study examined the demographic, clinical characteristics, and treatment persistency among individuals ≤20 years and >20 years of age who initiated TROF.

METHODS

Study Design and Data Source

- Retrospective claims analysis of individuals diagnosed with RTT between 01/01/2021 to 12/31/2023 (study period) was conducted using linked data from IQVIA's Anonymized Patient Level Database and TROF prescription (RX) data from a specialty pharmacy database.

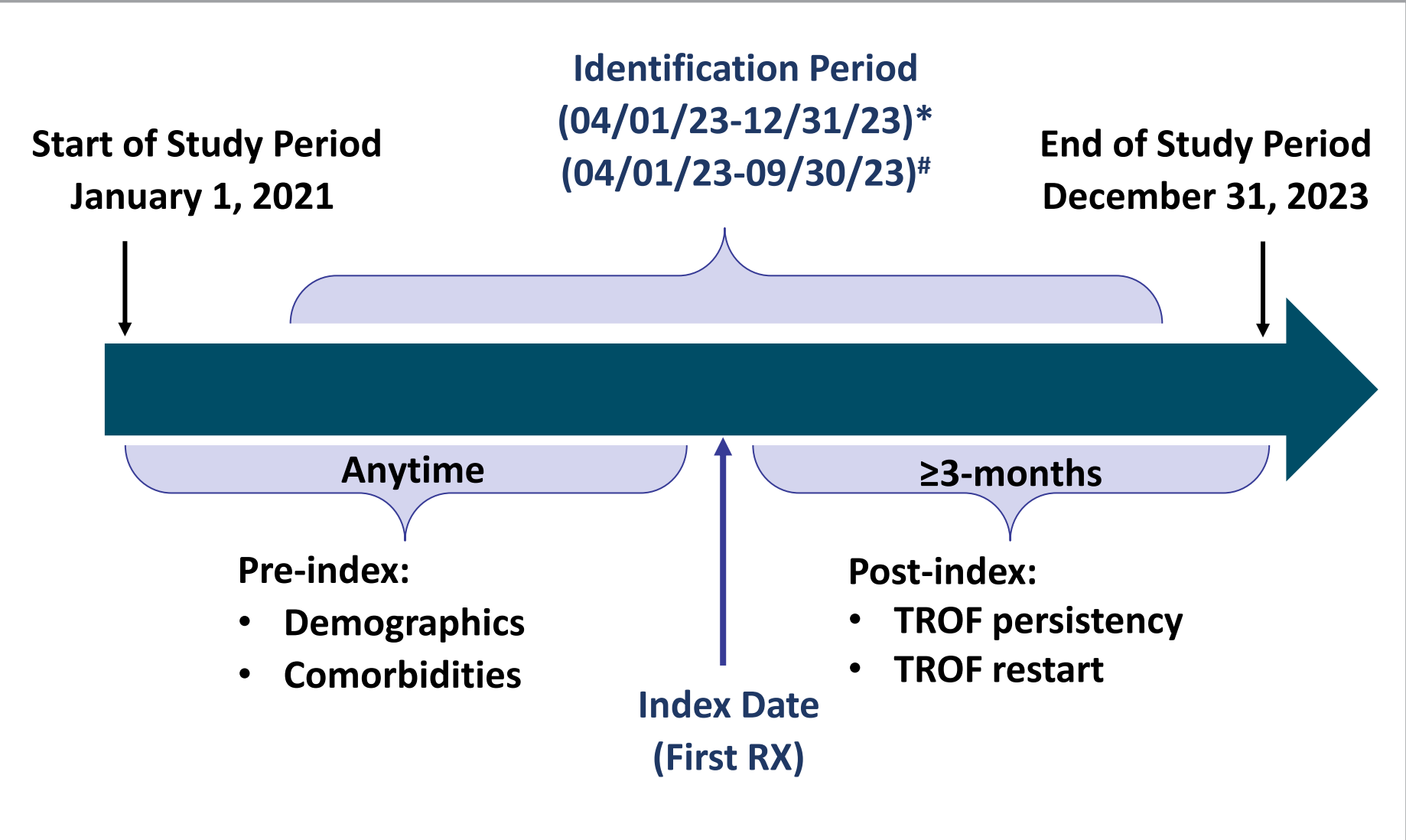
Study Population

- The study population comprised of individuals diagnosed with RTT (ICD-10 code F84.2) who received ≥1 RX of TROF between 04/01/2023 and 09/30/2023 (TROF Cohort). The first RX fill date of TROF was defined as the index date.
- Pre-index and post-index period (Figure 1).
 - Pre-index period: defined as anytime before index date.
 - Post-index period: defined as ≥3-months after index date.

Study Groups and Persistent/Non-persistent Sub-groups

- The TROF cohort was stratified into two age groups:
 - ≤20-years of age group, and
 - >20-years of age group.
- Both age groups were further categorized into persistent and non-persistent subgroups if they met the following eligibility criteria.
 - Have ≥2 RX fills, and
 - Have ≥3-months of post-index follow-up.

Figure 1: Study Schema



Pre-index Demographics and Clinical Characteristics

- Demographics:** age, gender at index
- Comorbidity Characteristics:** comorbidities assessed during pre-index

Post-index Outcomes

- Treatment Persistent/Non-persistent**
 - Treatment Persistent Group:** identified as the proportion of individuals on continuous TROF treatment with an allowable treatment gap of ≤60 days.

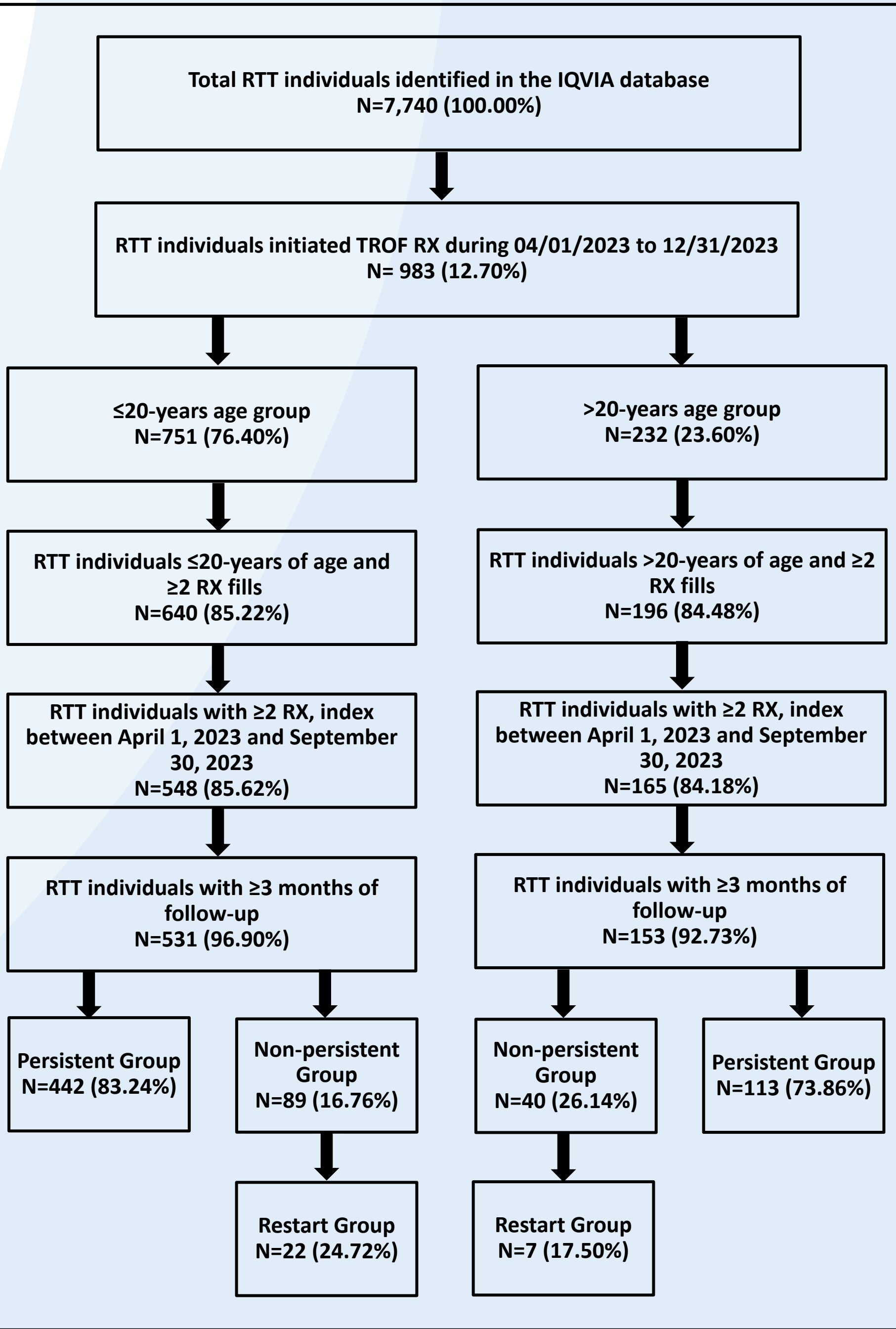
METHODS

- Non-Persistent Group:** identified as the proportion of individuals with no RX refill within the allowable treatment gap of ≤60 days
 - Median Time (in days) on TROF Treatment
 - defined as the time from first RX fill to last RX fill plus days of supply for both persistent and non-persistent sub-groups.
- TROF Restart Group:** identified as the proportion of individuals from the non-persistence group who re-initiated TROF after a gap of >60 days.
 - Median Time (in days) to Restart
 - defined as time from non-persistence (i.e., the end of days' supply for last RX fill date) to first restart RX.

Statistical Methods

- Continuous variables: expressed as means and standard deviations (SD), median and interquartile range (IQR).
- Categorical variables: expressed as counts (n) and percentages (%).
- Time on treatment: expressed as median (IQR) in days among both persistent and non-persistent groups.
- Time to restart: expressed as median (IQR) in days.

Figure 2: Study Population Selection



Abbreviations: TROF, Trofinetide; RX, Prescription; RTT, Rett syndrome

RESULTS

Demographic and Clinical Characteristics

- Of the 983 RTT individuals initiating TROF, 76.40% (n=751) were ≤20-years and 23.60% (n=232) were >20-years of age [Figure 2].
- Mean age in the ≤20-year age group was 10.54 years, while mean age in the >20-year age group was 32.42 years. Both age groups were predominantly female (96.94% & 93.97%, respectively) [Table 1].
- Pre-index clinical comorbidities were higher among ≤20-year age group compared to >20-year age group.

RESULTS

Table 1: Demographics Characteristics among ≤20-years Age Group vs. >20-years Age Group

Characteristic	TROF cohort N=983	
	≤20-years age group (n=751, 76.40%)	>20-years age group (n=232, 23.60%)
Age		
Mean (SD)	10.54 (5.40)	32.42 (13.19)
Median (IQR)	10 (9)	28 (12)
Gender, n (%)		
Male	23 (3.06%)	14 (6.03%)
Female	728 (96.94%)	218 (93.97%)
Pediatric, n (%)		
2-4	128 (17.04%)	0 (0.00%)
5-10	257 (34.22%)	0 (0.00%)
11-17	260 (34.62%)	0 (0.00%)
Adult, n (%)		
18-20	106 (14.11%)	0 (0.00%)
21-29	0 (0.00%)	133 (57.33%)
30-39	0 (0.00%)	55 (23.71%)
40-49	0 (0.00%)	21 (9.05%)
≥ 50	0 (0.00%)	23 (9.91%)

Abbreviations: IQR, Interquartile range; SD, Standard deviation; TROF, Trofinetide

Demographic and Clinical Characteristics

- Comorbidities such as epilepsy (56.46% vs. 64.66%), gastrostomy (33.29% vs. 37.93%) and respiratory failure (19.44% vs. 21.55%) were lower among ≤20-years of age compared to >20-years of age [Figure 3].

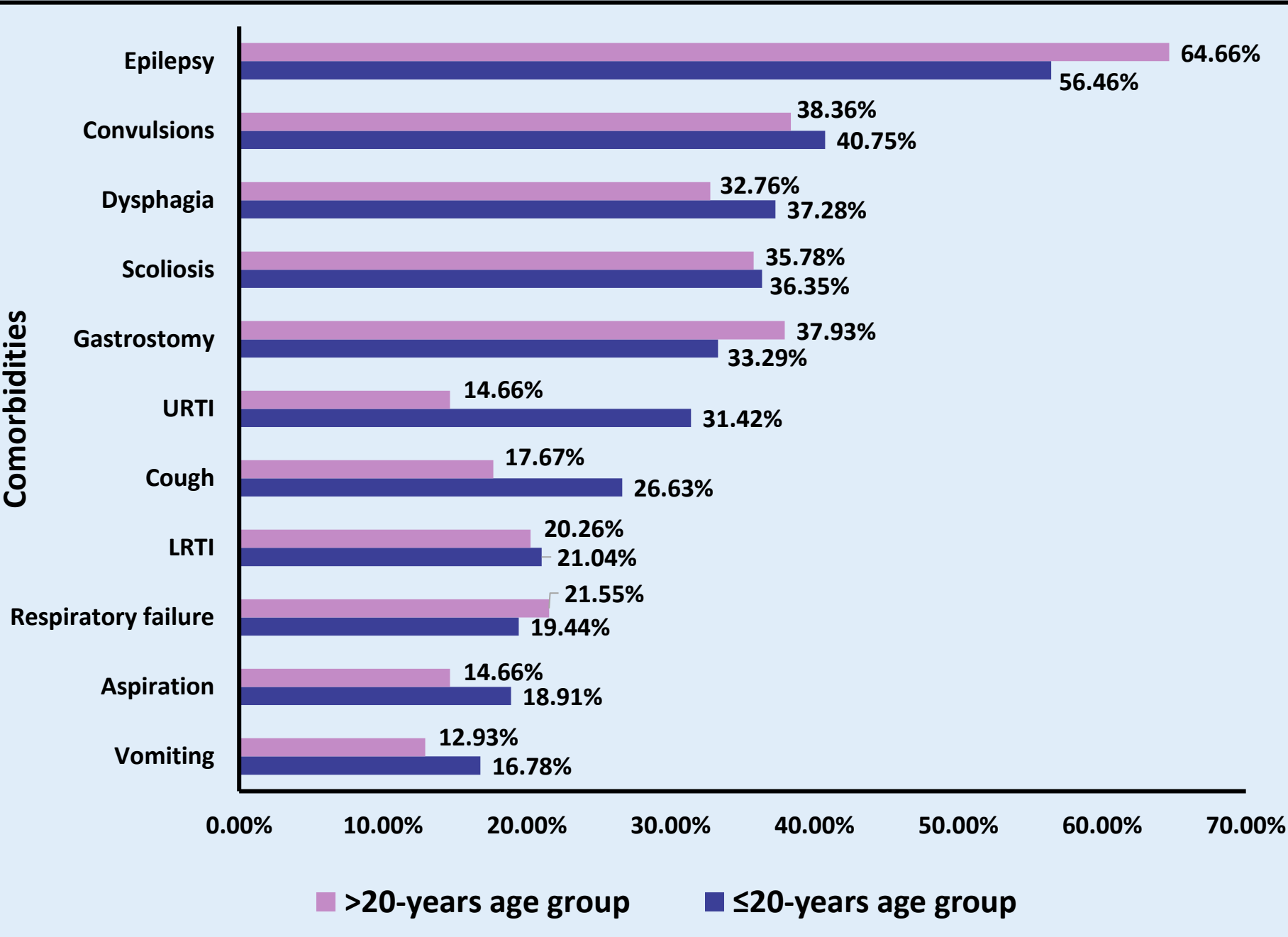
Post-Index Outcomes

- Of the 684 (69.58%) in the persistent sub-group of individuals on TROF; 77.63% (n=531) were ≤20 years and 22.37% (n=153) were >20 years of age

Treatment Persistency and Restart (≤20-years Age Group):

- Of the 531 persistent individuals in the ≤20-years age group, approximately 83.24% (n=442) of individuals were persistent and 16.76% (n=89) were non-persistent.
 - The median (IQR) time on treatment among persistent and non-persistent groups were 174 (81) and 63 (29) days, respectively [Figures 4a, 4b].
- Among non-persistent individuals (n=89), 24.72% restarted TROF within a median (IQR) days of 80 (33) [Figures 5a, 5b].

Figure 3: Rates of Comorbidities among ≤20-years Age Group vs. >20-years Age Group

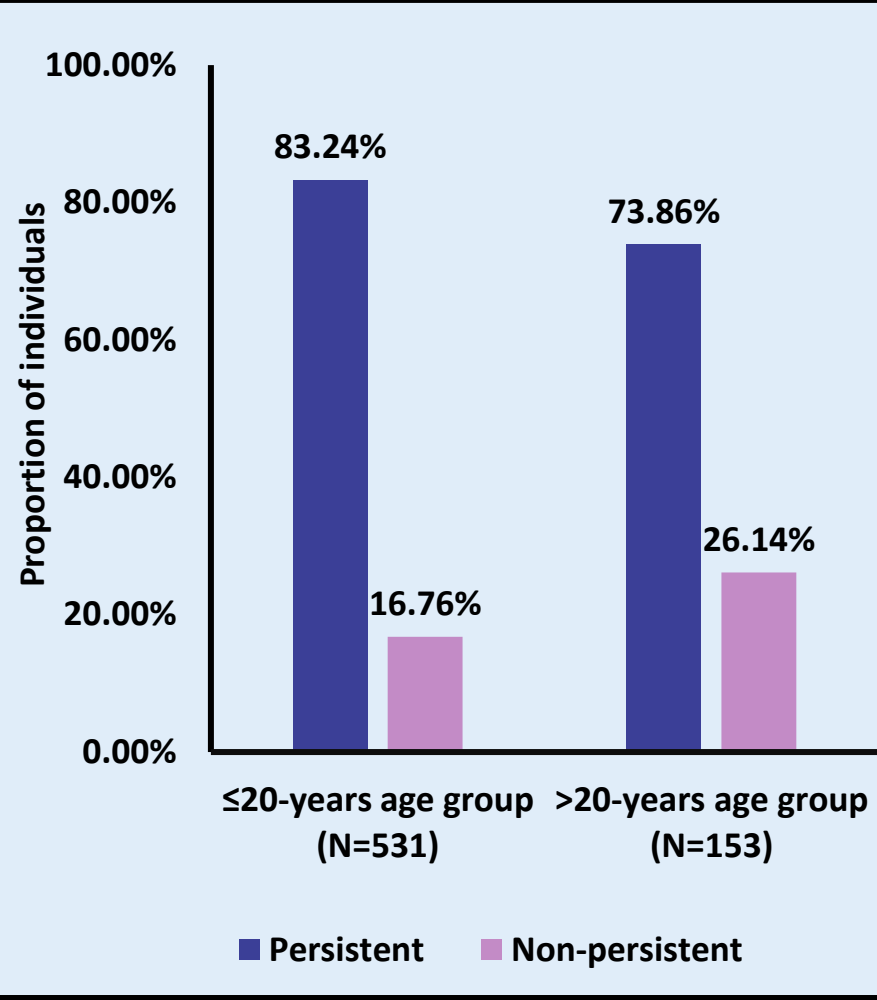


Abbreviations: LRTI, Lower respiratory tract infection; URTI, Upper respiratory tract infection

Treatment persistency and restart (>20-years Age Group):

- Of the 153 non-persistent individuals in the >20-years age group, approximately 73.85% (n=113) were persistent and 26.14% (n=40) were non-persistent.
 - Median (IQR) time on treatment among persistent, and non-persistent were 159 (76) and 69 (40) days, respectively [Figures 4a, 4b].
- Among non-persistent (n=40), 17.50% restarted TROF within a median (IQR) days of 83 (18) [Figures 5a, 5b].

Figure 4a: Persistency among ≤20-years age group vs. >20-years age group



Abbreviations: TROF, Trofinetide

Figure 4b: Time on treatment among ≤20-years age group vs. >20-years age group

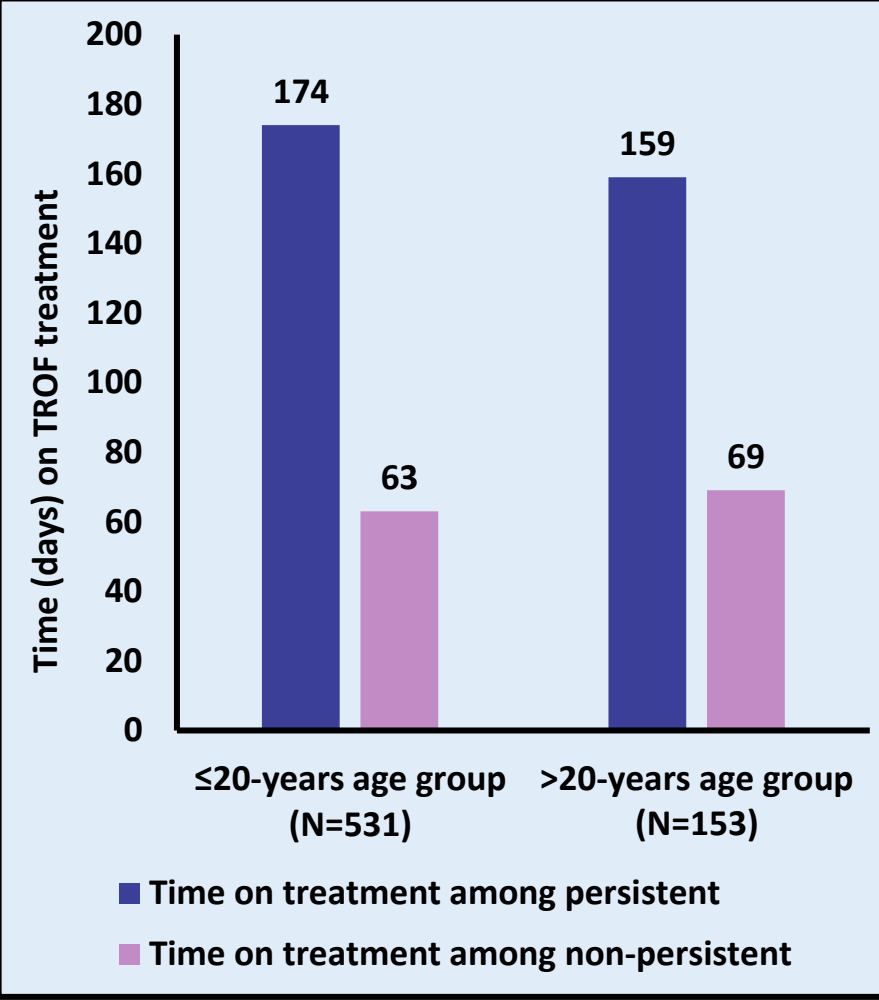


Figure 5a: Restart among ≤20-years age group vs. >20-years age group

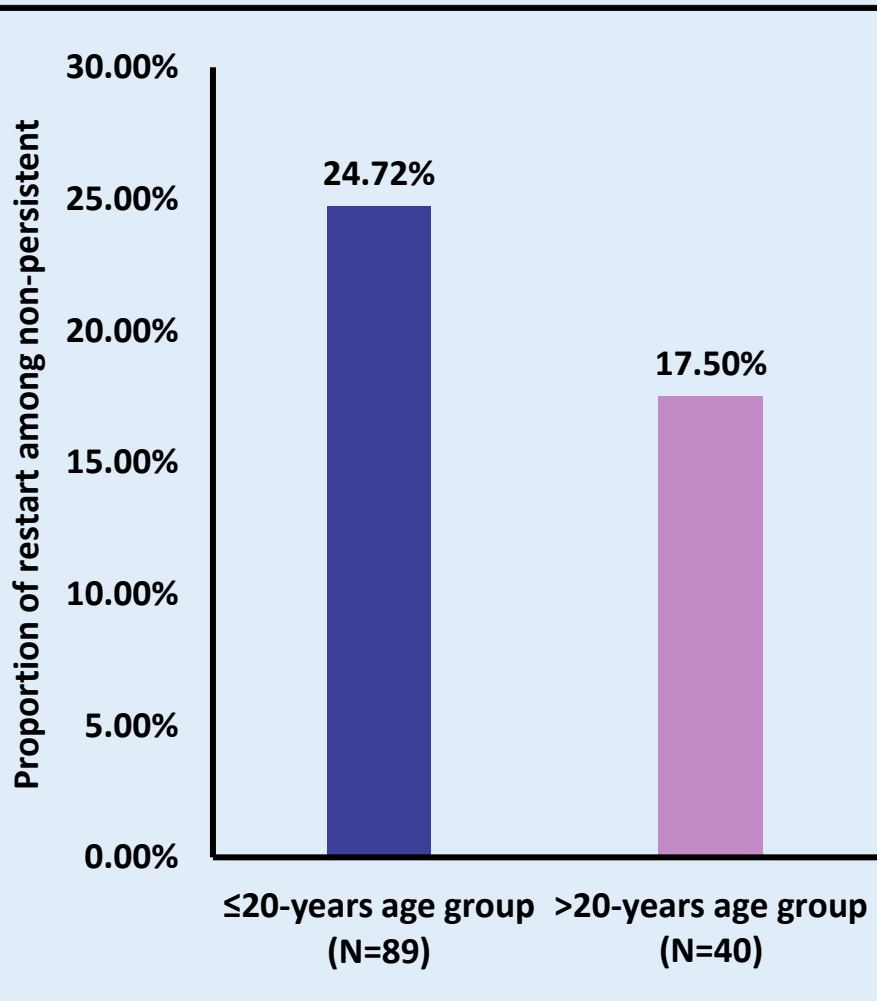
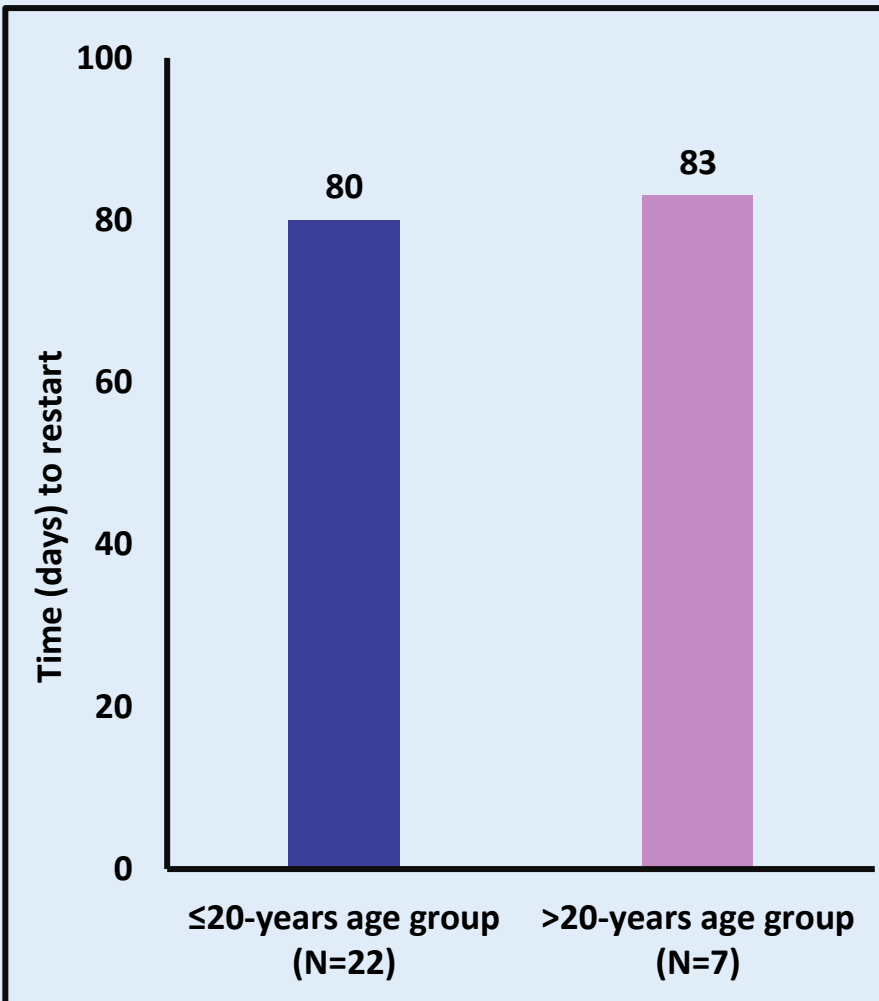


Figure 5b: Time to restart among ≤20-years age group vs. >20-years age group



CONCLUSIONS

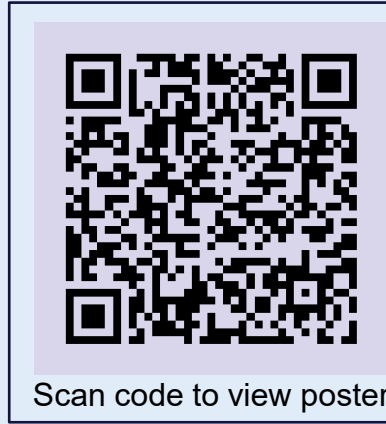
- In this real-world analysis, majority of individuals with RTT remained persistent on TROF therapy in both (≤20-years and >20-years) age groups. Median time on TROF among persistent and non-persistent sub-groups were also similar in both age groups suggesting a similar effectiveness across all ages.
- Approximately 1 in 4 of the RTT individuals who were non-persistent restarted TROF within 90 days in both age groups.
- The ≤20-years age group had lower rates of epilepsy, gastrostomy and respiratory failure compared to >20-years age group.
- Future analysis with larger sample sizes and longer follow-up is needed to better understand TROF long-term persistency patterns.

LIMITATIONS

- The study has the same limitations that are common to administrative claims database analyses such as under coding and miscoding.
- Shorter follow-up time in the study sample limited the ability to examine long-term persistency.

REFERENCES

- Rett Syndrome Fact Sheet. National Institutes of Health (NIH).
<https://www.nichd.nih.gov/health/topics/factsheets/re>
- A First for Rett: FDA Approves Trofinetide for Treatment of Rett Syndrome! International Rett Syndrome Foundation



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