



U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research
Office of Translational Sciences
Office of Biostatistics

STATISTICAL REVIEW AND EVALUATION – ERRATA

CLINICAL STUDIES

NDA #: NDA 217026
Supplement #: N/A
Drug Name: Trofinetide (ACP-2566)
Indication(s): Treatment of Rett syndrome (in adults and pediatric patients 2 years of age and older)
Applicant: Acadia Pharmaceuticals, Inc.
Date(s): Submission date: 7/12/2022, PDUFA date: 3/12/2023
Review Priority: Priority
Biometrics Division: Division of Biometrics I
Statistical Reviewer: Andrew N. Potter, PhD
Concurring Reviewers: James Hung, PhD
Medical Division: Division of Neurology I
Clinical Team: Michael Dimyan, MD
Project Manager: Brenda Reggett, PharmD

Errata for the original Statistics Review of 02/04/2023.

This Review contains corrections to several typos in the original statistics review dated 02/04/2023.

In Section 3.2.2, the description of the stratification factors for Study 003 is missing age group (5-10 years old, 11-15 years old, and 16-20 years old).

Table 3 should have the final two rows deleted:

Table 1: Patient Disposition / Primary Analysis (ACP-2566-003)

Subject Disposition - Randomized Analysis Set

	Placebo N=94	Trofinetide N=93	Total N=187
Completion Status, n(%)			
Completed the Study	85 (90.4)	70 (75.3)	155 (82.9)
Early Termination	9 (9.6)	23 (24.7)	32 (17.1)
Reason for Early Termination, n(%)			
Adverse Event	2 (2.1)	16 (17.2)	18 (9.6)
Lack of Efficacy	0	1 (1.1)	1 (<1)
Non-Compliance with Study Drug	0	4 (4.3)	4 (2.1)
Protocol Deviation	1 (1.1)	0	1 (<1)
Subject Withdrew Consent	1 (1.1)	1 (1.1)	2 (1.1)
Other	5 (5.3)	1 (1.1)	6 (3.2)
Marked as Dropout but Had a Week 12 Visit, n(%)			
Completed the Study	93 (98.9)	86 (92.5)	179 (95.7)
Early Termination with Week 12 Visit	1 (1.1)	7 (7.5)	8 (4.3)

The Reviewer's Note above Table 4 should state eight patients instead of seven.

On page 10, the sentence beginning "In the trofinetide arm, there were only three subjects;" should read "In the **placebo** arm, there were only three subjects[.]"

On page 26, the "6.7 point greater improvement" should read "4.7 point greater improvement."

This is a representation of an electronic record that was signed electronically. Following this are manifestations of any and all electronic signatures for this electronic record.

/s/

ANDREW N POTTER
03/10/2023 11:21:16 AM

HSIEN MING J HUNG
03/10/2023 11:25:16 AM