



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*

	<b>Placebo N=94</b>	<b>Trofinetide N=93</b>	<b>Total N=187</b>
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."

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/s/  
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