

ADVISORY COMMITTEE BRIEFING MATERIALS: AVAILABLE FOR PUBLIC RELEASE

**PERIPHERAL AND CENTRAL NERVOUS SYSTEM DRUGS
ADVISORY COMMITTEE**

25 April 2016

ERRATA FOR ADVISORY COMMITTEE BRIEFING DOCUMENT

ETEPLIRSEN

NDA 206488



Sarepta Therapeutics, Inc.
215 First Street
Cambridge, MA 02142 USA

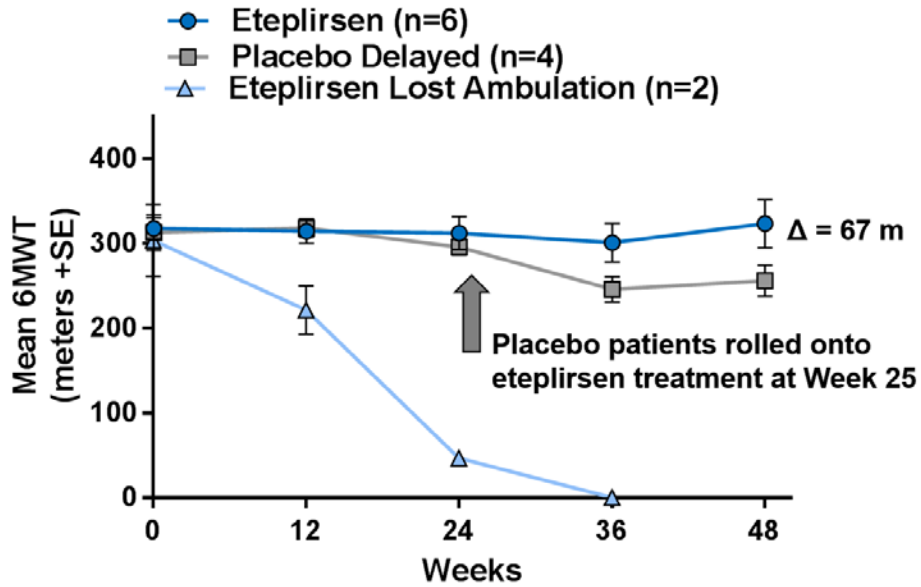
This errata provides minor corrections to one figure and one table within the briefing document to support the upcoming Advisory Committee meeting scheduled 25 April 2016. This errata is intended to provide transparency of these minor corrections and does not affect the conclusions within the briefing document.

AVAILABLE FOR PUBLIC DISCLOSURE WITHOUT REDACTION

PAGE 53, FIGURE 15

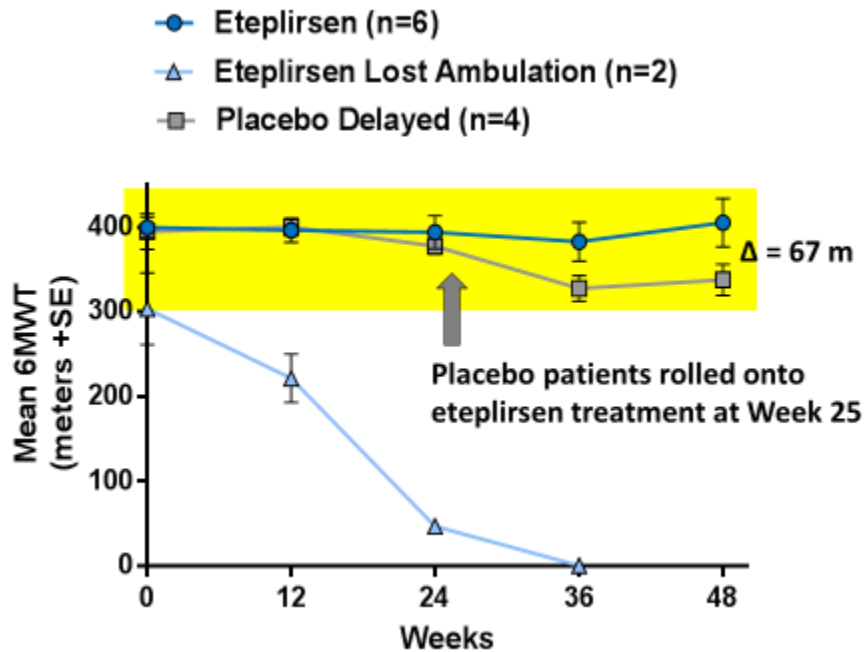
Was:

Figure 15: Study 201/202: Exploratory Analysis of 6MWT for Eteplirsen vs. Placebo (excluding 2 eteplirsen-treated boys with ambulatory decline prior to significant dystrophin production)



Is amended to:

Figure 15: Study 201/202: Exploratory Analysis of 6MWT for Eteplirsen vs. Placebo (excluding 2 eteplirsen-treated boys with ambulatory decline prior to significant dystrophin production)



Rationale: The “eteplirsen” and “placebo delayed” curves are moved to the correct positions on the graph.

PAGE 151, APPENDIX 7. ETEPLIRSEN PATIENTS EXTERNAL CONTROL WITH RESULTS OF 6MWT AT YEAR 4

Was:

External Control Subjects (N = 13) with Results of 6MWT at Year 4

Patient Number	Baseline 6MWT	Year 3 6MWT	Year 4 6MWT	Age at Year 4	Age at LOA
ECM2**	380	195	*Missing	12.6	13.1‡
ECM5	295	35	0	13.3	13.3
ECM6	380	0	0	12.0	11
ECM4	329	218	0	12.8	12.8
ECM10	388	0	0	12.1	11.1
ECM9	388	0	0	14.1	12.1
ECM7	325	0	0	11.3	10.3
ECM8	458	362	300 [†]	14.2	15‡
ECM1	200	0	0	15.5	13.5
ECM3	373	273	0	13.0	13
ECG1	327	0	0	15.5	13.8
ECG2	451	240	0	15.2	15.5
ECG3*	355	Missing	* Missing	12.3	12.8‡

Note: External Control patients ECM2 and ECG3 were missing 6MWT data at Year 4. Of note, these patients were subsequently reported to have loss of ambulation with “0” meters on the 6MWT at ~4.5 years.

[†]External Control Patient ECM8 walked 300m on 6MWT at Year 4, and was subsequently reported to have loss of ambulation at 4.8 years.

[‡]LOA after year 4

Is amended to:

External Control Subjects (N = 13) with Results of 6MWT at Year 4

Patient Number	Baseline 6MWT	Year 3 6MWT	Year 4 6MWT	Age at Year 4	Age at LOA
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[†]External Control Patient ECM8 walked 300m on 6MWT at Year 4, and was subsequently reported to have loss of ambulation at 4.8 years.

[‡]LOA after year 4

Rationale: Age at Year 4 for external control patients ECG1, ECG2 and ECG3 is corrected, and age at loss of ambulation for ECG3 is corrected.