

**Errata – FDA Briefing Document for NDA 200896 (ataluren)**  
**Peripheral and Central Nervous System Drugs Advisory Committee Meeting**  
**September 28, 2017**

In Table 9 on Page 19 ('Memorandum to the Committee' Page 15) of FDA briefing package: The units of ataluren concentrations should be "ug/mL" instead of "ng/mL" Table from FDA briefing package showing the error is provided below.

**Table 9: Summary of Baseline Clinical Endpoints by Treatment and/or Concentration Groups in Study 007**

	Baseline Time(s) to Descend 4 Stairs		Baseline Time(s) to Climb 4 Stairs	Baseline Time(s) Taken to Walk/Run 10m	Baseline Time(s) to Rise from Supine	6 Minute walk Distance at Baseline (m)	Age at Baseline
	N	Mean	Mean	Mean	Mean	Mean	Mean
High Dose (20/20/40 mg/kg) High Concentration => <u>19.3 ng/mL</u>	30	8.53	10.25	9.66	15.63	332.91	9.23
High Dose (20/20/40 mg/kg) Low Concentration < <u>19.3 ng/mL</u>	29	4.97	5.05	5.94	9.04	391.09	7.53
Low Dose 10,10,20 mg/kg	57	6.08	6.94	7.45	10.80	355.59	8.77
Placebo	56	5.53	6.02	6.81	11.36	361.52	8.32
All	172	6.14	6.90	7.37	11.53	359.59	8.49

*Source: FDA OCP Review*

In Table 1 on Page 86 ('Summary of Office of Clinical Pharmacology Findings' Page 13) of briefing package: The units of ataluren concentrations should be "ug/mL" instead of "ng/mL". Table from FDA briefing package showing the error is provided below.

**Table 1. Summary of baseline clinical endpoints by treatment and/or concentration groups in Study 007**

	Baseline Time(s) to Descend 4 Stairs		Baseline Time(s) to Climb 4 Stairs	Baseline Time(s) Taken to Walk/Run 10m	Baseline Time(s) to Rise from Supine	6 Minute walk Distanc at Baseline (m)	Age at Baseline
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Source : Reviewer's Analysis