

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

The FDA Briefing Document for the September 28, 2017, advisory committee meeting to discuss the ataluren application for the treatment of nonsense mutation Duchenne Muscular Dystrophy (nmDMD) requires a clarification. The FDA memorandum to the committee indicates that the applicant's 2011 NDA was based on post hoc changes to the analytical methods and populations from Study 007. As described in the memo, the post hoc analyses of Study 007 included (1) changes to the primary analysis method, (2) the use of what the applicant referred to as a "corrected ITT" population which omitted the baseline 6-minute walk distance (6MWD) values for 2 subjects, and (3) the dismissal of the fact that the high-dose performed numerically worse than the low-dose. In the memo, the post hoc analyses giving rise to the "ambulatory decline phase (ADP)" population are identified as the basis for the 2011 NDA. Although results from post hoc analyses were the basis of the 2011 NDA, the ADP population was not explicitly described at the time of the 2011 NDA submission. The post hoc ADP population was the basis for the enrichment of Study 020, as indicated.