CENTER FOR DRUG EVALUATION AND RESEARCH

APPLICATION NUMBER:

213801Orig1s000

STATISTICAL REVIEW(S)

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

CLINICAL STUDIES

NDA Serial Number:	213801
Drug Name:	Myrbetriq Granules (Mirabegron for extended release oral suspension)
Indication(s):	Treatment of neurogenic detrusor overactivity (NDO) in pediatric patients aged 3 years and older
Applicant:	Astellas Pharma Global Development, INC.
Date(s):	Receipt Date: 09/28/2020
	PDUFA Date: 03/28/2021
Review Priority:	Priority
Biometrics Division:	Division of Biometrics IV
Statistical Reviewer:	Jia Guo, Ph.D.
Concurring Reviewers:	Daphne Lin, Ph.D. Acting Team Leader, Deputy Director
Medical Division:	Division of Urology, Obstetrics and Gynecology (DUOG)
Clinical Team:	Elena Boley, M.D., Clinical Reviewer
	Mark Hirsch, M.D., Clinical Team Leader
Project Manager:	Nenita Crisostomo

Keywords: Change from Baseline

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1 EXECUTIVE SUMMARY

Myrbetriq® 25 mg and 50 mg tablets (Mirabegron) is currently approved under NDA 202611 for treatment of overactive bladder (OAB) with symptoms of urge urinary incontinence, urgency, and urinary frequency in adults. A Written Request (WR) for the use of mirabegron in treatment of neurogenic detrusor overactivity (NDO) in pediatric patients was issued under NDA 202611 on 18 March 2016.

In this submission, the Applicant submitted the safety and efficacy data from one study to fulfill the WR and seek approval of mirabegron for NDO in pediatric patients. This review is to evaluate from a statistical perspective if the submitted information supports this claim.

The study was a multinational, multi-center, open-label, single arm phase 3 study with a 12-week dose titration period followed by 40 weeks of treatment with fixed dose in subjects between 3 to 18 years old.

The primary efficacy endpoint was the change from baseline in maximum cystometric capacity (MCC) during treatment period at week 24 (measured in mL). It was summarized using descriptive statistics and the mean change from baseline estimate, together with 95% CI. The lower bound of the two-sided 95% CI was assessed to see if it excluded 0. Due to lack of a control group, the interpretation of the results is descriptive in nature. Secondary efficacy endpoints based on urodynamics and patient diary were also evaluated in a similar way as the primary efficacy endpoint.

In children (3-12 years old), the MCC was increased by 72.1 mL (SD: 87.1, 95% CI 45.3 to 98.9); in adolescents (12-18 years old), the MCC was increased by 113.2 mL (SD: 83.0, 95% CI 79.0 to 147.5);

The study demonstrated that there is clinical benefit of mirabegron in treatment of NDO in pediatric subjects.

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