ZTALMY

Procedural steps taken and scientific information after the authorisation

Application number	Scope	Opinion/ Notification [1] issued on	Commission Decision Issued [2] / amended on	Product Information affected [3]	Summary
II/0006	Update of section 5.1 of the SmPC in order to update open-label data based on the final report from study 1042-CDD-3001 OLE listed as a category 3 study in the RMP. This was the open-label portion of the pivotal study 1042-CDD-3001; a double-blind, randomized, placebo-controlled trial of adjunctive	27/06/2024		SmPC	For more information, please refer to the Summary of Product Characteristics.

[1] Notifications are issued for type I variations and Article 61(3) notifications (unless part of a group including a type II variation or extension application or a worksharing application). Opinions are issued for all other procedures.
[2] A Commission decision (CD) is issued for procedures that affect the terms of the marketing authorisation (e.g. summary of product characteristics, annex II, labelling, package leaflet). The CD is issued within two months of the opinion for variations falling under the scope of Article 23.1a(a) of Regulation (EU) No. 712/2012, or within one year for other procedures.
[3] SmPC (Summary of Product Characteristics), Annex II, Labelling, PL (Package Leaflet).

	ganaxolone treatment in children and young adults with cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder (CDD) followed by long-term open-label treatment. The RMP version 1.4 has also been submitted. C.I.4 - Change(s) in the SPC, Labelling or PL due to new quality, preclinical, clinical or pharmacovigilance data				
II/0002	Submission of the final report from study 1042-HME-1001 listed as post-authorisation measure (PAM) recommendation. This is an interventional Phase 1 Single Dose, Open-Label Crossover Comparative Bioavailability Study of Two Oral Formulations of Ganaxolone. The primary objective of this study was to evaluate and compare the pharmacokinetics of a new ganaxolone formulation (hot-melt extrusion [HME]) with ganaxolone oral suspension after a single oral dose administration under fed conditions. C.I.13 - Other variations not specifically covered elsewhere in this Annex which involve the submission of studies to the competent authority	16/05/2024	n/a		
PSUSA/93/202309	Periodic Safety Update EU Single assessment - ganaxolone	11/04/2024	n/a		PRAC Recommendation - maintenance
IB/0007	C.I.z - Changes (Safety/Efficacy) of Human and Veterinary Medicinal Products - Other variation	05/03/2024	n/a		

IB/0001	C.I.11.z - Introduction of, or change(s) to, the obligations and conditions of a marketing authorisation, including the RMP - Other variation	24/10/2023	n/a		