



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Loargys

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/0002/G	This was an application for a group of variations. Grouped application comprising two type II variations as follows: C.I.4 – Update of sections 4.8 and 5.1 of the SmPC in order to update efficacy and safety information	05/09/2024		SmPC, Annex II and PL	The section 4.8 of the SmPC was updated, to reflect the final results of the two efficacy and safety studies. It was noted that a higher frequency of injection site reactions was reported, all categorised as non-serious and which did not lead to discontinuation of treatment. The text was amended in section 5.1, as to reflect the extension of the

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).



	based on final results from study CAEB1102-300A (SOB 003), listed as a specific obligation in Annex II. Study 300A was a Phase 3, randomized, double blind, placebo-controlled study of the efficacy and safety of pegzilarginase in adults, adolescents and children with arginase 1 deficiency (ARG1 D). C.I.4 – Update of section 4.8 of the SmPC in order to update efficacy and safety information based on final results from study CAEB1102-102A (SOB 004), listed as a specific obligation in Annex II. Study 102A was an open label extension study to evaluate the long-term safety, tolerability, and efficacy of pegzilarginase in adults, adolescents and children with arginase 1 deficiency (ARG1 D). The Package Leaflet and Annex II are updated accordingly. The RMP version 1.2 has also been submitted. In addition, the MAH took the opportunity to bring the PI in line with the latest QRD template version 10.4 and to introduce minor editorial changes. C.I.4 - Change(s) in the SPC, Labelling or PL due to new quality, preclinical, clinical or pharmacovigilance data C.I.4 - Change(s) in the SPC, Labelling or PL due to new quality, preclinical, clinical or pharmacovigilance data				long-term treatment with the product. For more information, please refer to the Summary of Product Characteristics.
IB/0004	B.I.c.2.b - Change in the specification parameters and/or limits of the immediate packaging of the AS - Addition of a new specification parameter to the specification with its corresponding test method	31/07/2024	n/a		

IB/0003	B.II.b.5.b - Change to in-process tests or limits applied during the manufacture of the finished product - Addition of a new test(s) and limits	12/07/2024	n/a		
N/0001	Minor change in labelling or package leaflet not connected with the SPC (Art. 61.3 Notification)	23/05/2024		PL	