Kanuma

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/0044	C.I.4 - Change(s) in the SPC, Labelling or PL due to new quality, preclinical, clinical or pharmacovigilance data	22/06/2023		SmPC and PL	
IAIN/0045/G	This was an application for a group of variations.	16/05/2023		Annex II and	

[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).

	A.4 - Administrative change - Change in the name and/or address of a manufacturer or an ASMF holder or supplier of the AS, starting material, reagent or intermediate used in the manufacture of the AS or manufacturer of a novel excipient A.5.b - Administrative change - Change in the name and/or address of a manufacturer/importer of the finished product, including quality control sites (excluding manufacturer for batch release) A.5.a - Administrative change - Change in the name and/or address of a manufacturer/importer responsible for batch release A.4 - Administrative change - Change in the name and/or address of a manufacturer or an ASMF holder or supplier of the AS, starting material, reagent or intermediate used in the manufacture of the AS or manufacturer of a novel excipient			PL	
IAIN/0043	B.V.a.1.d - PMF - Inclusion of a new, updated or amended PMF in the marketing authorisation dossier of a medicinal product. (PMF 2nd step procedure) - Inclusion of an updated/amended PMF when changes do not affect the properties of the FP	05/09/2022	n/a		
IB/0041	B.II.g.5.c - Implementation of changes foreseen in an approved change management protocol - For a biological/immunological medicinal product	12/08/2022	n/a		
IB/0040	B.I.a.1.f - Change in the manufacturer of AS or of a starting material/reagent/intermediate for AS - Changes to quality control testing arrangements for	15/07/2022	n/a		

	the AS -replacement or addition of a site where batch control/testing takes place				
IB/0039	C.I.11.z - Introduction of, or change(s) to, the obligations and conditions of a marketing authorisation, including the RMP - Other variation	20/06/2022	26/05/2023	Annex II	Annex II has been updated to include the revised timelines of LAL-d registry from annual to every 2 years.
II/0036/G	This was an application for a group of variations. B.I.a.1.f - Change in the manufacturer of AS or of a starting material/reagent/intermediate for AS - Changes to quality control testing arrangements for the AS -replacement or addition of a site where batch control/testing takes place B.II.g.2 - Introduction of a post approval change management protocol related to the finished product B.I.a.1.e - Change in the manufacturer of AS or of a starting material/reagent/intermediate for AS - The change relates to a biological AS or a starting material [-] used in the manufacture of a biological/immunological product	19/05/2022	26/05/2023	Annex II	
PSUSA/10422 /202108	Periodic Safety Update EU Single assessment - sebelipase alfa	07/04/2022	n/a		PRAC Recommendation - maintenance
IB/0037	B.I.a.1.f - Change in the manufacturer of AS or of a starting material/reagent/intermediate for AS - Changes to quality control testing arrangements for the AS -replacement or addition of a site where batch control/testing takes place	08/03/2022	n/a		

IB/0035	B.I.a.2.a - Changes in the manufacturing process of the AS - Minor change in the manufacturing process of the AS	18/01/2022	n/a		
II/0032	Update of section 4.2 of the SmPC in order to introduce a new posology regimen (higher starting dose of 3mg/kg once weekly) based on cumulative data from clinical studies and real-world clinical practice for patients with Rapidly Progressive LAL deficiency presenting within the first six months of life. Consequently the dosing information for patients with Rapidly Progressive LAL deficiency and paediatric and adult patients with LAL deficiency is modified. In addition editorial update is made in sections 4.8, 5.1, 5.2 and 6.6 following the new posology regimen. The Package Leaflet is updated accordingly. C.I.4 - Change(s) in the SPC, Labelling or PL due to new quality, preclinical, clinical or pharmacovigilance data	11/11/2021	13/12/2021	SmPC and PL	After reviewing the submitted data, the CHMP recommended an updated starting dose in infants (< 6 months of age) presenting with rapidly progressive LAL deficiency of either 1 mg/kg or 3 mg/kg once weekly, depending on the clinical status of the patient.
IB/0033/G	This was an application for a group of variations. B.I.b.2.a - Change in test procedure for AS or starting material/reagent/intermediate - Minor changes to an approved test procedure B.V.a.1.d - PMF - Inclusion of a new, updated or amended PMF in the marketing authorisation dossier of a medicinal product. (PMF 2nd step procedure) - Inclusion of an updated/amended PMF when changes	04/11/2021	n/a		

	do not affect the properties of the FP B.V.a.1.d - PMF - Inclusion of a new, updated or amended PMF in the marketing authorisation dossier of a medicinal product. (PMF 2nd step procedure) - Inclusion of an updated/amended PMF when changes do not affect the properties of the FP				
PSUSA/10422 /202008	Periodic Safety Update EU Single assessment - sebelipase alfa	09/04/2021	n/a		PRAC Recommendation - maintenance
IAIN/0031/G	This was an application for a group of variations. B.V.a.1.d - PMF - Inclusion of a new, updated or amended PMF in the marketing authorisation dossier of a medicinal product. (PMF 2nd step procedure) - Inclusion of an updated/amended PMF when changes do not affect the properties of the FP B.V.a.1.d - PMF - Inclusion of a new, updated or amended PMF in the marketing authorisation dossier of a medicinal product. (PMF 2nd step procedure) - Inclusion of an updated/amended PMF when changes do not affect the properties of the FP B.V.a.1.d - PMF - Inclusion of a new, updated or amended PMF in the marketing authorisation dossier of a medicinal product. (PMF 2nd step procedure) - Inclusion of an updated/amended PMF when changes do not affect the properties of the FP B.V.a.1.d - PMF - Inclusion of a new, updated or amended PMF in the marketing authorisation dossier of a medicinal product. (PMF 2nd step procedure) - Inclusion of an updated/amended PMF when changes	09/02/2021	n/a		

	do not affect the properties of the FP B.V.a.1.d - PMF - Inclusion of a new, updated or amended PMF in the marketing authorisation dossier of a medicinal product. (PMF 2nd step procedure) - Inclusion of an updated/amended PMF when changes do not affect the properties of the FP B.V.a.1.d - PMF - Inclusion of a new, updated or amended PMF in the marketing authorisation dossier of a medicinal product. (PMF 2nd step procedure) - Inclusion of an updated/amended PMF when changes do not affect the properties of the FP B.V.a.1.d - PMF - Inclusion of a new, updated or amended PMF in the marketing authorisation dossier of a medicinal product. (PMF 2nd step procedure) - Inclusion of an updated/amended PMF when changes do not affect the properties of the FP				
IAIN/0030	B.V.a.1.d - PMF - Inclusion of a new, updated or amended PMF in the marketing authorisation dossier of a medicinal product. (PMF 2nd step procedure) - Inclusion of an updated/amended PMF when changes do not affect the properties of the FP	09/02/2021	n/a		
IA/0029	A.4 - Administrative change - Change in the name and/or address of a manufacturer or an ASMF holder or supplier of the AS, starting material, reagent or intermediate used in the manufacture of the AS or manufacturer of a novel excipient	27/01/2021	n/a		
II/0026/G	This was an application for a group of variations.	15/10/2020	20/11/2020	SmPC, Annex II and PL	Please refer to Scientific Discussion Kanuma-H-C-004004-II-0026/G

	- Update of sections 4.2, 4.4, 4.8, 5.1, 5.2. 6.6 of the Summary of Product Characteristics (SmPC) in order to update the clinical information based on the pooled safety and efficacy analysis of already submitted studies (Studies LAL-CL04, LAL-CL03, LAL-CL06, LAL-CL08 and LAL-CL02) and updated population pharmacokinetics analyses in children and adults. The Package Leaflet has been amended accordingly. The RMP version 4.1 has also been submitted. Annex II is also updated to remove the specific obligation related to the provision of study LAL-CL08. - Submission of the final report from study LAL-EA01 An open-label study with sebelipase alfa (1 mg/kg every other week for up to 78 weeks or until drug commercialization) in United States (US) patients who did not otherwise qualify for an active sebelipase alfa trial (expanded access protocol) C.I.4 - Change(s) in the SPC, Labelling or PL due to new quality, preclinical, clinical or pharmacovigilance data C.I.13 - Other variations not specifically covered elsewhere in this Annex which involve the submission of studies to the competent authority				
IB/0027	B.I.b.2.z - Change in test procedure for AS or starting material/reagent/intermediate - Other variation	26/05/2020	n/a		
R/0025	Renewal of the marketing authorisation.	27/02/2020	23/04/2020	SmPC, Annex	

				II, Labelling and PL	
II/0023	B.II.b.1.c - Replacement or addition of a manufacturing site for the FP - Site where any manufacturing operation(s) take place, except batch release/control, and secondary packaging, for biol/immunol medicinal products or pharmaceutical forms manufactured by complex manufacturing processes	19/03/2020	n/a		
PSUSA/10422 /201908	Periodic Safety Update EU Single assessment - sebelipase alfa	12/03/2020	n/a		PRAC Recommendation - maintenance
IB/0022	B.I.b.1.z - Change in the specification parameters and/or limits of an AS, starting material/intermediate/reagent - Other variation	13/09/2019	n/a		
IAIN/0021	A.1 - Administrative change - Change in the name and/or address of the MAH	26/07/2019	05/12/2019	SmPC, Labelling and PL	
II/0019	C.I.13 - Other variations not specifically covered elsewhere in this Annex which involve the submission of studies to the competent authority	25/07/2019	n/a		
IB/0020	B.I.d.1.z - Stability of AS - Change in the re-test period/storage period or storage conditions - Other variation	17/05/2019	n/a		
PSUSA/10422 /201808	Periodic Safety Update EU Single assessment - sebelipase alfa	14/03/2019	n/a		PRAC Recommendation - maintenance

IB/0017	C.I.11.z - Introduction of, or change(s) to, the obligations and conditions of a marketing authorisation, including the RMP - Other variation	17/12/2018	05/12/2019	Annex II	
PSUSA/10422 /201802	Periodic Safety Update EU Single assessment - sebelipase alfa	06/09/2018	n/a		PRAC Recommendation - maintenance
IAIN/0016	B.II.b.1.a - Replacement or addition of a manufacturing site for the FP - Secondary packaging site	14/06/2018	n/a		
PSUSA/10422 /201708	Periodic Safety Update EU Single assessment - sebelipase alfa	08/03/2018	n/a		PRAC Recommendation - maintenance
IB/0014/G	This was an application for a group of variations. A.7 - Administrative change - Deletion of manufacturing sites B.I.a.2.z - Changes in the manufacturing process of the AS - Other variation	13/12/2017	n/a		
IAIN/0012	B.II.b.2.c.1 - Change to importer, batch release arrangements and quality control testing of the FP - Replacement or addition of a manufacturer responsible for importation and/or batch release - Not including batch control/testing	12/10/2017	20/09/2018	Annex II and PL	
PSUSA/10422 /201702	Periodic Safety Update EU Single assessment - sebelipase alfa	28/09/2017	n/a		PRAC Recommendation - maintenance

IB/0011	C.I.11.z - Introduction of, or change(s) to, the obligations and conditions of a marketing authorisation, including the RMP - Other variation	11/07/2017	n/a		
IB/0009/G	This was an application for a group of variations. B.I.b.2.e - Change in test procedure for AS or starting material/reagent/intermediate - Other changes to a test procedure (including replacement or addition) for the AS or a starting material/intermediate B.I.b.2.z - Change in test procedure for AS or starting material/reagent/intermediate - Other variation	28/03/2017	n/a		
PSUSA/10422 /201608	Periodic Safety Update EU Single assessment - sebelipase alfa	09/03/2017	n/a		PRAC Recommendation - maintenance
II/0006/G	This was an application for a group of variations. B.II.b.1.c - Replacement or addition of a manufacturing site for the FP - Site where any manufacturing operation(s) take place, except batch release/control, and secondary packaging, for biol/immunol medicinal products or pharmaceutical forms manufactured by complex manufacturing processes B.II.d.2.d - Change in test procedure for the finished product - Other changes to a test procedure (including replacement or addition) B.II.e.6.b - Change in any part of the (primary)	23/02/2017	n/a		

	packaging material not in contact with the finished product formulation - Change that does not affect the product information B.II.b.2.a - Change to importer, batch release arrangements and quality control testing of the FP - Replacement/addition of a site where batch control/testing takes place				
IB/0008	B.II.e.4.c - Change in shape or dimensions of the container or closure (immediate packaging) - Sterile medicinal products	03/02/2017	n/a		
PSUSA/10422 /201602	Periodic Safety Update EU Single assessment - sebelipase alfa	29/09/2016	n/a		PRAC Recommendation - maintenance
IB/0004/G	This was an application for a group of variations. B.I.a.1.a - Change in the manufacturer of AS or of a starting material/reagent/intermediate for AS - The proposed manufacturer is part of the same pharmaceutical group as the currently approved manufacturer B.I.a.4.a - Change to in-process tests or limits applied during the manufacture of the AS - Tightening of in-process limits	06/07/2016	n/a		
IA/0003/G	This was an application for a group of variations. A.4 - Administrative change - Change in the name and/or address of a manufacturer or an ASMF holder or supplier of the AS, starting material, reagent or	12/11/2015	n/a		

	intermediate used in the manufacture of the AS or manufacturer of a novel excipient A.4 - Administrative change - Change in the name and/or address of a manufacturer or an ASMF holder or supplier of the AS, starting material, reagent or intermediate used in the manufacture of the AS or manufacturer of a novel excipient A.4 - Administrative change - Change in the name and/or address of a manufacturer or an ASMF holder or supplier of the AS, starting material, reagent or intermediate used in the manufacture of the AS or manufacturer of a novel excipient A.4 - Administrative change - Change in the name and/or address of a manufacturer or an ASMF holder or supplier of the AS, starting material, reagent or intermediate used in the manufacture of the AS or manufacturer of a novel excipient				
T/0001	Transfer of Marketing Authorisation	14/09/2015	05/10/2015	SmPC, Labelling and PL	