



## Xenpozyme

Procedural steps taken and scientific information after the authorisation

Application number	Scope	Opinion/ Notification <sup>1</sup> issued on	Commission Decision Issued <sup>2</sup> / amended on	Product Information affected <sup>3</sup>	Summary
II/0012/G	This was an application for a group of variations.  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	08/05/2025		SmPC and PL	

<sup>1</sup> 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.

<sup>2</sup> 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.

<sup>3</sup> SmPC (Summary of Product Characteristics), Annex II, Labelling, PL (Package Leaflet).



	new quality, preclinical, clinical or pharmacovigilance data				
II/0013/G	<p>This was an application for a group of variations.</p> <p>Update of section 4.2 of the SmPC in order to update the 'Missed Doses' section to facilitate the appropriate clinical management of patients based on pre-existing data from the clinical trials.</p> <p>Update of section 4.2 of the SmPC in order to include a clarification of the infusion rate during the home infusion based on pre-existing data from the clinical trials</p> <p>C.I.4 - Change(s) in the SPC, Labelling or PL due to new quality, preclinical, clinical or pharmacovigilance data</p> <p>C.I.4 - Change(s) in the SPC, Labelling or PL due to new quality, preclinical, clinical or pharmacovigilance data</p>	13/02/2025		SmPC	<p>Section 4.2 of the SmPC has been updated to reflect that patients should re-initiate the dose escalation regimen starting at first escalation dose, i.e. 0.1 mg/kg (adult patients) and 0.03 mg/kg (paediatric patients) in case 3 or more consecutive infusions are missed during escalation phase.</p> <p>Section 4.2 of the SmPC has also been updated to clarify that the infusion rate and dose used in the home settings should remain the same as used in the supervised clinical settings, as prescribed by the prescribing/treating physician, and should not be changed without supervision of the prescribing/treating physician.</p> <p>For more information, please refer to the Summary of Product Characteristics.</p>
PSUSA/11003 /202403	Periodic Safety Update EU Single assessment - olipudase alfa	31/10/2024	n/a		PRAC Recommendation - maintenance
N/0011	Minor change in labelling or package leaflet not connected with the SPC (Art. 61.3 Notification)	30/07/2024		PL	
II/0009	B.II.d.1.e - Change in the specification parameters and/or limits of the finished product - Change outside the approved specifications limits range	30/05/2024	n/a		

PSUSA/11003 /202309	Periodic Safety Update EU Single assessment - olipudase alfa	11/04/2024	n/a		PRAC Recommendation - maintenance
PSUSA/11003 /202303	Periodic Safety Update EU Single assessment - olipudase alfa	09/11/2023	11/01/2024	SmPC	Refer to Scientific conclusions and grounds recommending the variation to terms of the Marketing Authorisation(s)' for PSUSA/11003/202303.
IB/0007	C.I.11.z - Introduction of, or change(s) to, the obligations and conditions of a marketing authorisation, including the RMP - Other variation	18/12/2023	n/a		
IB/0006	B.II.b.5.f - Change to in-process tests or limits applied during the manufacture of the finished product - Addition or replacement of an in-process test as a result of a safety or quality issue	21/11/2023	n/a		
IAIN/0004	A.1 - Administrative change - Change in the name and/or address of the MAH	21/04/2023	11/01/2024	SmPC, Labelling and PL	
PSUSA/11003 /202209	Periodic Safety Update EU Single assessment - olipudase alfa	14/04/2023	n/a		PRAC Recommendation - maintenance
II/0002/G	This was an application for a group of variations.  B.II.d.2.a - Change in test procedure for the finished product - Minor changes to an approved test procedure B.II.f.1.d - Stability of FP - Change in storage conditions of the finished product or the diluted/reconstituted product B.II.f.1.b.5 - Stability of FP - Extension of the shelf life of the finished product - Biological/immunological	23/03/2023	11/01/2024	SmPC, Labelling and PL	

	<p>medicinal product in accordance with an approved stability protocol</p> <p>B.II.b.4.z - Change in the batch size (including batch size ranges) of the finished product - Other variation</p> <p>B.II.b.3.c - Change in the manufacturing process of the finished or intermediate product - The product is a biological/immunological medicinal product and the change requires an assessment of comparability</p> <p>B.II.e.1.b.2 - Change in immediate packaging of the finished product - Change in type/addition of a new container - Sterile medicinal products and biological/immunological medicinal products</p> <p>B.II.e.5.c - Change in pack size of the finished product - Change in the fill weight/fill volume of sterile multidose (or single-dose, partial use) parenteral medicinal products, including biological/immunological medicinal products</p>				
II/0001/G	<p>This was an application for a group of variations.</p> <p>Grouped application comprising two type II variations as follows:</p> <ul style="list-style-type: none"> <li>- To update sections 4.6 of the SmPC in order to include a recommendation to use effective contraception during treatment and for 14 days after the last dose if Xenpozyme is discontinued based on embryo-foetal study in mice (study TER0694). In addition, the MAH proposes an update of section 5.3 of the SmPC based on a re-calculation of exposure margins for the embryo-foetal study. MAH also proposes to align the SmPC with the updated CCDS.</li> <li>- To update sections 4.6 and 5.3 of the SmPC in</li> </ul>	16/03/2023	11/01/2024	SmPC and PL	

	<p>order to include data in lactating mice based on final results from study MSSM-1120 - Evaluation of Olipudase alfa Transfer Into Milk of Lactating Mice. The Package Leaflet is updated accordingly. The RMP version 2.2 has also been submitted.</p> <p>C.I.4 - Change(s) in the SPC, Labelling or PL due to new quality, preclinical, clinical or pharmacovigilance data</p> <p>C.I.4 - Change(s) in the SPC, Labelling or PL due to new quality, preclinical, clinical or pharmacovigilance data</p>				
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