Upstaza

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
S/0025	2nd annual re-assessment	27/02/2025	23/04/2025	SmPC and PL	The CHMP, having reviewed the evidence of compliance with the specific obligations and the impact of the data submitted by the MAH on the benefit/risk profile of the medicinal product, concluded that marketing authorisation of Upstaza should be maintained.

[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).

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PSUSA/11004 /202406	Periodic Safety Update EU Single assessment - eladocagene exuparvovec	16/01/2025	n/a		PRAC Recommendation - maintenance
II/0023/G	This was an application for a group of variations. B.I.a.1.k - Change in the manufacturer of AS or of a starting material/reagent/intermediate for AS - New storage site of MCB and/or WCB B.I.a.1.c - Change in the manufacturer of AS or of a starting material/reagent/intermediate for AS - The proposed manufacturer uses a substantially different route of synthesis or manufacturing conditions B.I.a.2.c - Changes in the manufacturing process of the AS - The change refers to a [-] substance in the manufacture of a biological/immunological substance which may have a significant impact on the medicinal product and is not related to a protocol	12/12/2024	n/a		
II/0022/G	This was an application for a group of variations. B.II.b.2.b - Change to importer, batch release arrangements and quality control testing of the FP - Replacement/addition of a site where batch control/testing takes place for a biol/immunol product and any of the test methods at the site is a biol/immunol method B.II.d.1.z - Change in the specification parameters and/or limits of the finished product - Other variation	25/07/2024	n/a		Not applicable
PSUSA/11004 /202312	Periodic Safety Update EU Single assessment - eladocagene exuparvovec	11/07/2024	n/a		PRAC Recommendation - maintenance

II/0021	B.I.b.z - Change in control of the AS - Other variation	30/05/2024	n/a		
II/0014/G	This was an application for a group of variations. Update of sections 4.2, 4.4, 4.8, 5.1 and 5.2 of the SmPC in order to update information on safety and efficacy, based on final results from studies NTUH-AADC-010 and NTUH-AADC-011. NTUH-AADC-010 is an open-label, single arm, externally controlled trial to evaluate safety, efficacy, pharmacodynamics and immunogenicity of AGIL-AADC in children from 18 months to less than 18 years of age with severe AADC deficiency, while NTUH-AADC-011 is an open-label, single arm, externally controlled trial to evaluate efficacy and safety of AGIL-AADC in children from 18 months to less than 6 years of age with severe AADC deficiency. In addition, sections 4.5, 4.9 and 6.6 of the SmPC are updated in order to provide better clarification and guidance for the medical practice. The Package Leaflet is updated accordingly. The MAH also took the opportunity to update the due date of the final report of study AADC-1602 in the Annex II to 2032, considering the 10-year follow up of the last patient in study AADC-011, and to introduce minor editorial changes to the PI. 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	25/01/2024	11/03/2024	SmPC, Annex II and PL	Update of sections 4.2, 4.4, 4.8, 5.1 and 5.2 of the SmPC in order to update information on safety and efficacy, based on final results from studies NTUH-AADC-010 and NTUH-AADC-011. Sections 4.5, 4.9 and 6.6 of the SmPC are updated in order to provide better clarification and guidance for the medical practice.

	new quality, preclinical, clinical or pharmacovigilance data				
S/0017	1st annual re-assessment	22/02/2024	n/a		The CHMP, having reviewed the evidence of compliance with the specific obligations and the impact of the data submitted by the MAH on the benefit/risk profile of the medicinal product, concluded that marketing authorisation of Upstaza should be maintained.
II/0013	C.I.11.b - Introduction of, or change(s) to, the obligations and conditions of a marketing authorisation, including the RMP - Implementation of change(s) which require to be further substantiated by new additional data to be submitted by the MAH where significant assessment is required	22/02/2024	12/02/2025	Annex II	
PSUSA/11004 /202306	Periodic Safety Update EU Single assessment - eladocagene exuparvovec	11/01/2024	n/a		PRAC Recommendation - maintenance
IB/0019	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	13/11/2023	n/a		
IB/0018	B.I.b.2.a - Change in test procedure for AS or starting material/reagent/intermediate - Minor changes to an approved test procedure	24/10/2023	n/a		
PSUSA/11004 /202212	Periodic Safety Update EU Single assessment - eladocagene exuparvovec	06/07/2023	n/a		PRAC Recommendation - maintenance

IB/0012	B.II.f.1.b.5 - Stability of FP - Extension of the shelf life of the finished product - Biological/immunological medicinal product in accordance with an approved stability protocol	08/06/2023	04/10/2023	SmPC	
IB/0010	B.I.b.1.z - Change in the specification parameters and/or limits of an AS, starting material/intermediate/reagent - Other variation	24/05/2023	n/a		
IB/0011	B.I.d.1.a.4 - Stability of AS - Change in the re-test period/storage period - Extension or introduction of a re-test period/storage period supported by real time data	08/05/2023	n/a		
IB/0009	C.I.11.z - Introduction of, or change(s) to, the obligations and conditions of a marketing authorisation, including the RMP - Other variation	04/04/2023	04/10/2023	Annex II	
IB/0007/G	This was an application for a group of variations. B.II.f.1.b.5 - Stability of FP - Extension of the shelf life of the finished product - Biological/immunological medicinal product in accordance with an approved stability protocol A.6 - Administrative change - Change in ATC Code/ATC Vet Code	21/03/2023	04/10/2023	SmPC	
II/0005/G	This was an application for a group of variations. B.I.b.1.f - Change in the specification parameters and/or limits of an AS, starting material/intermediate/reagent - Change outside the	23/02/2023	n/a		

	approved specifications limits range for the AS B.II.d.1.e - Change in the specification parameters and/or limits of the finished product - Change outside the approved specifications limits range				
II/0004/G	This was an application for a group of variations. B.II.d.2.c - Change in test procedure for the finished product - Substantial change to or replacement of a biol/immunol/immunochemical test method or a method using a biol. reagent or replacement of a biol. reference preparation not covered by an approved protocol B.I.b.2.d - Change in test procedure for AS or starting material/reagent/intermediate - Substantial change to or replacement of a biological/immunological/immunochemical test method or a method using a biological reagent for a biological AS	23/02/2023	n/a		
IB/0003	B.I.b.1.c - Change in the specification parameters and/or limits of an AS, starting material/intermediate/reagent - Addition of a new specification parameter to the specification with its corresponding test method	09/12/2022	n/a		
IB/0001/G	This was an application for a group of variations. B.II.f.1.b.5 - Stability of FP - Extension of the shelf life of the finished product - Biological/immunological medicinal product in accordance with an approved stability protocol	29/09/2022	04/10/2023	SmPC	

	B.II.f.1.e - Stability of FP - Change to an approved stability protocol				
N/0002	Minor change in labelling or package leaflet not connected with the SPC (Art. 61.3 Notification)	19/09/2022	04/10/2023	PL	