Hemgenix

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
IB/0024/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	16/12/2024	n/a		

[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).

	batch control/testing takes place 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.II.d.2.d - Change in test procedure for the finished product - Other changes to a test procedure (including replacement or addition)				
II/0018	Update of sections 4.4, 4.8 and 5.1 of the SmPC in order to reflect a modified 9-point anti-AAV5 Neutralising Antibody (NAb) assay. In addition, the MAH took the opportunity to introduce minor editorial changes to the PI and update the list of local representatives in the Package Leaflet and bring the PI in line with the QRD version 10.4. C.I.4 - Change(s) in the SPC, Labelling or PL due to new quality, preclinical, clinical or pharmacovigilance data	12/12/2024		SmPC and PL	SmPC new text: Preexisting neutralising anti-AAV5 antibodies above a titre of 1:898, based on the neutralising anti-AAV5 antibody assay with extended measuring range (equivalent to 1:678 titre based on the previous clinical study assay), may impede transgene expression at desired therapeutic levels and thus reduce the efficacy of Hemgenix therapy. For more information, please refer to the Summary of Product Characteristics.
IA/0025	B.I.b.2.a - Change in test procedure for AS or starting material/reagent/intermediate - Minor changes to an approved test procedure	11/12/2024	n/a		
R/0020	Renewal of the marketing authorisation.	17/10/2024	04/12/2024		The CHMP, having reviewed the available information on the status of the fulfilment of Specific Obligations and having confirmed the positive benefit risk balance, is of the opinion that the quality, safety and efficacy of this medicinal product continue to be adequately and sufficiently demonstrated and therefore recommends the

					renewal of the conditional MA for Hemgenix, subject to the Specific Obligations and Conditions as laid down in Annex II to the opinion.
IA/0023/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 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.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)	03/12/2024		Annex II and PL	
IB/0022	B.I.a.2.a - Changes in the manufacturing process of the AS - Minor change in the manufacturing process of the AS	05/11/2024	n/a		
II/0016/G	This was an application for a group of variations.	19/09/2024	n/a		

	B.II.b.2.c.3 - Change to importer, batch release arrangements and quality control testing of the FP - Including batch control/testing for a biol/immunol product and any of the test methods is a biol/immunol/immunochemical method B.II.b.2.c.3 - Change to importer, batch release arrangements and quality control testing of the FP - Including batch control/testing for a biol/immunol product and any of the test methods is a biol/immunol/immunochemical method B.II.b.2.z - Change to importer, batch release arrangements and quality control testing of the FP - Other variation B.II.b.2.z - Change to importer, batch release arrangements and quality control testing of the FP - Other variation				
II/0015	Submission of the final report from study AMT-061-01/CSL222_2001 listed as a Specific Obligation in the Annex II of the Product Information. This is a Phase IIb, open-label, single-dose, single-arm, multi-center trial to confirm the factor IX activity level of the serotype 5 adeno-associated viral vector containing the Padua variant of a codon-optimized human factor IX gene (AAV5-hFIXco-Padua, AMT-061) administered to adult subjects with severe or moderately severe hemophilia B. The Annex II is updated to reflect the fulfilment of this commitment. C.I.11.b - Introduction of, or change(s) to, the obligations and conditions of a marketing authorisation, including the RMP - Implementation of	19/09/2024	04/12/2024	Annex II	Not applicable

	change(s) which require to be further substantiated by new additional data to be submitted by the MAH where significant assessment is required				
II/0014/G	This was an application for a group of variations. 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 B.I.a.1.j - Change in the manufacturer of AS or of a starting material/reagent/intermediate for AS - Replacement or addition of a site where batch control/testing takes place and any of the test method at the site is a biol/immunol method	19/09/2024	n/a		
IB/0021	B.I.b.2.a - Change in test procedure for AS or starting material/reagent/intermediate - Minor changes to an approved test procedure	17/09/2024	n/a		
IB/0017/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.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.k - Change in the manufacturer of AS or of a starting material/reagent/intermediate for AS - New storage site of MCB and/or WCB	05/08/2024	n/a		

	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.k - Change in the manufacturer of AS or of a starting material/reagent/intermediate for AS - New storage site of MCB and/or WCB 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				
PSUSA/11037 /202311	Periodic Safety Update EU Single assessment - etranacogene dezaparvovec	13/06/2024	n/a		PRAC Recommendation - maintenance
IB/0012	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	21/02/2024	n/a		
IA/0013	A.6 - Administrative change - Change in ATC Code/ATC Vet Code	13/02/2024	04/12/2024	SmPC and PL	
PSUSA/11037 /202305	Periodic Safety Update EU Single assessment - etranacogene dezaparvovec	11/01/2024	n/a		PRAC Recommendation - maintenance

II/0009/G	This was an application for a group of variations. B.II.b.2.c.3 - Change to importer, batch release arrangements and quality control testing of the FP - Including batch control/testing for a biol/immunol product and any of the test methods is a biol/immunol/immunochemical method B.II.b.2.c.3 - Change to importer, batch release arrangements and quality control testing of the FP - Including batch control/testing for a biol/immunol product and any of the test methods is a biol/immunol/immunochemical method B.II.b.2.c.3 - Change to importer, batch release arrangements and quality control testing of the FP - Including batch control/testing for a biol/immunol product and any of the test methods is a biol/immunol/immunochemical method B.II.b.2.z - Change to importer, batch release arrangements and quality control testing of the FP - Other variation	14/12/2023	n/a		
R/0007	Renewal of the marketing authorisation.	12/10/2023	07/12/2023		The CAT and CHMP, having reviewed the available information on the status of the fulfilment of Specific Obligations and having confirmed the positive benefit risk balance, is of the opinion that the quality, safety and efficacy of this medicinal product continue to be adequately and sufficiently demonstrated and therefore recommends the renewal of the conditional MA for Hemgenix, subject to the Specific Obligations and Conditions as laid down in Annex II to the opinion.
IB/0010	B.I.a.2.a - Changes in the manufacturing process of	23/11/2023	n/a		

	the AS - Minor change in the manufacturing process of the AS				
IB/0006/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.II.d.2.a - Change in test procedure for the finished product - Minor changes to an approved test procedure	07/08/2023	n/a		
IB/0005	B.I.b.2.a - Change in test procedure for AS or starting material/reagent/intermediate - Minor changes to an approved test procedure	17/07/2023	n/a		
N/0003	Minor change in labelling or package leaflet not connected with the SPC (Art. 61.3 Notification)	19/06/2023	07/12/2023	PL	
IB/0001/G	This was an application for a group of variations. B.I.a.2.a - Changes in the manufacturing process of the AS - Minor change in the manufacturing process of the AS B.I.a.2.a - Changes in the manufacturing process of the AS - Minor change in the manufacturing process of the AS B.II.b.3.a - Change in the manufacturing process of the finished or intermediate product - Minor change in the manufacturing process	05/04/2023	n/a		

N/0002	Minor change in labelling or package leaflet not connected with the SPC (Art. 61.3 Notification)	23/03/2023	07/12/2023	PL	