



## Strimvelis

### 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
T/0014	Transfer of Marketing Authorisation	27/07/2018	23/08/2018	SmPC, Labelling and PL	
IA/0013/G	This was an application for a group of variations.  B.I.b.1.d - Change in the specification parameters and/or limits of an AS, starting material/intermediate/reagent - Deletion of a	15/06/2018	n/a		

<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).



	<p>non-significant specification parameter (e.g. deletion of an obsolete parameter)</p> <p>B.I.b.2.a - Change in test procedure for AS or starting material/reagent/intermediate - Minor changes to an approved test procedure</p> <p>B.I.d.1.c - Stability of AS - Change in the re-test period/storage period or storage conditions - Change to an approved stability protocol</p>				
PSUSA/10505 /201711	Periodic Safety Update EU Single assessment - autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human ADA cDNA sequence	14/06/2018	n/a		PRAC Recommendation - maintenance
IB/0012	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	22/05/2018	n/a		
IB/0010	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	01/02/2018	n/a		
PSUSA/10505 /201705	Periodic Safety Update EU Single assessment - autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human ADA cDNA sequence	11/01/2018	n/a		PRAC Recommendation - maintenance
IB/0009	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	04/12/2017	n/a		

IB/0007	B.I.b.2.a - Change in test procedure for AS or starting material/reagent/intermediate - Minor changes to an approved test procedure	02/08/2017	n/a		
II/0006	B.I.b.1.z - Change in the specification parameters and/or limits of an AS, starting material/intermediate/reagent - Other variation	20/07/2017	30/07/2018	SmPC, Annex II and PL	To introduce changes in section 4.3 and 4.4 of the product information following changes in the analytical control of bone marrow samples of patients to allow prospective patients who have previously tested positive for hepatitis C can be treated with Strimvelis, provided they demonstrate absence of ongoing infection using a nucleic acid test with a limit of quantification of $\leq 15$ international units/ml.
PSUSA/10505 /201611	Periodic Safety Update EU Single assessment - autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human ADA cDNA sequence	09/06/2017	n/a		PRAC Recommendation - maintenance
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	28/03/2017	n/a		
IB/0003/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.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	30/01/2017	n/a		

	<p>B.1.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</p> <p>B.1.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</p> <p>B.1.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</p> <p>B.1.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</p> <p>B.1.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</p>				
II/0001/G	<p>This was an application for a group of variations.</p> <p>B.1.b.1 z) To change the acceptance criteria for the transduction efficiency test for the drug substance from "Transduction detected" to "≥ 29%".</p> <p>B.1.b.2 d) To replace the method for the measurement of transduction efficiency of the drug substance with an improved transduction efficiency method (the qPCR method).</p> <p>B.1.b.1.z - Change in the specification parameters</p>	13/10/2016	n/a		

	and/or limits of an AS, starting material/intermediate/reagent - Other variation 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				
IB/0002	B.I.b.2.a - Change in test procedure for AS or starting material/reagent/intermediate - Minor changes to an approved test procedure	06/09/2016	n/a		