



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Pyrukynd

Procedural steps taken and scientific information after the authorisation*

*Due to the Agency`s update of its procedure management systems, an additional document, reflecting the historical lifecycle may be available in the 'Assessment history' section. For the complete product lifecycle procedures, you may need to also refer to **EPAR - Procedural steps taken and scientific information after authorisation (archive)**.

Application number	Scope	Opinion/ Notification ¹ issued on	Commission Decision Issued ² / amended on	Product Information affected ³	Summary
X/0010/G	This was an application for a group of	16/10/2025	21/05/2026	SmPC,	

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).



	<p>variations.</p> <p>Extension application to introduce a new strength (100 mg film-coated tablet) associated with a new orphan indication for the "treatment of adult patients with non-transfusion-dependent and transfusion-dependent alpha- or beta-thalassaemia". The extension application is grouped with a type II variation (C.I.4) to update of sections 4.2 and 5.2 of the SmPC in order to update pharmacokinetic information based on final results from study AG348-C-024 listed as a category 3 study in the RMP; this is a Phase 1, Open-label, Single-dose, Pharmacokinetic Study of Mitapivat in Subjects with Moderate Hepatic Impairment Compared to Matched Healthy Control Subjects with Normal Hepatic Function. The RMP (version 1.3) is updated in accordance.</p> <p>Annex I_2.(c) Change or addition of a new strength/potency C.I.4 - Change(s) in the SPC, Labelling or PL due to new quality, preclinical, clinical or pharmacovigilance data</p>			<p>Labelling and PL</p>	
<p>Variation type IB / EMA/VR/0000344092</p>	<p>Outcome: C.9 Introduction of, or change(s) to, the obligations and conditions of a marketing authorisation, including the risk management plan - C.9.b) Implementation</p>	<p>20/05/2026</p>			

	<p>of changes which require additional minor assessment (e.g. change to the due date of obligations and conditions of a marketing authorisation and required pharmacovigilance activities in the risk management plan, including changes to the due date of study milestones, and template updates) - Accepted</p> <p>C.9.b (Type IB) – To change the due date for category 3 study AG348-C-017 in the RMP for Pyrukynd from Q2 2026 to Q4 2026.</p>				
Variation type II / EMA/VR/0000315433	<p>Outcome: C.I HUMAN AND VETERINARY MEDICINAL PRODUCTS - C.I.13 Submission of additional clinical and non-clinical studies, including BE-studies. - Accepted</p> <p>Submission of the final report from study AG348-C-011 listed as a category 3 study in the RMP. This is a Phase 3, Multicenter, Open-label, Long-term, Extension Study of Mitapivat in Adults with PK Deficiency Previously Treated in Studies AG348-C-006 or AG348-C-007. The RMP version 2.1 has also been submitted.</p>	12/02/2026			
Variation type IB / EMA/VR/0000280226	<p>Outcome: This was an application for a group of variations.</p>	04/09/2025			

	<p>B.II.d.2 Change in test procedure for the finished product - B.II.d.2.d Other changes to a test procedure (including replacement or addition) - Accepted</p> <p>B.II.d.2 Change in test procedure for the finished product - B.II.d.2.d Other changes to a test procedure (including replacement or addition) - Accepted</p> <p>B.II.d.1 Change in the specification parameters and/or limits of the finished product - B.II.d.1.g Addition or replacement (excluding biological or immunological product) of a specification parameter with its corresponding test method as a result of a safety or quality issue - Accepted</p> <p>B.II.f.1 Change in the shelf-life or storage conditions of the finished product - B.II.f.1.e Change to an approved stability protocol - Accepted</p>				
PSUR / EMA/PSUR/0000274443	EURD: PSUSA/00011025/202502 Active substance: mitapivat Outcome: Maintenance	02/10/2025			Maintenance