Interim Access in Scotland: Successful Innovation or a Cautionary Tale?

Katherine Leong and Richard Macaulay | Consulting, Precision AQ

For further information, contact Richard.Macaulay@precisionaq.com or visit us on https://www.precisionaq.com

INTRODUCTION

- The Scottish Government's Review of access to new medicines (2016) recommended that the Scottish Medicines Consortium (SMC) should have the option to accept a medicine for use subject to future reassessment
- This process, known as interim acceptance, was introduced in 2018 and extended in 2021
- In Oct 2018, the SMC also introduced the ultra-orphan pathway, allowing medicines targeting ultra-rare diseases to be reimbursed for up to three years while further evidence is generated for re-assessment
- This research evaluates medicines with interim acceptance decisions and medicines under the ultra-orphan pathway

METHODS

- Interim acceptance decisions and medicines approved via the ultra-orphan pathway were identified from the SMC website (04-Sep-2024)

RESULTS

- 11 products have received an interim acceptance decision (3 in 2020, 2 in 2021, 3 in 2022, 2 in 2023, 1 in 2024)
  - None has received a re-assessment outcome, although the data collection deadline has passed for 2 products
- 11 products have been accepted via the ultra-orphan pathway (1 in 2019, 3 in 2020, 2 in 2021, 3 in 2022, 2 in 2023)
  - Only 2/11 (18%) have been re-evaluated (with a positive recommendation), although the three-year evidence generation deadline has passed for 5 other products with no re-assessment outcome available yet

Table 1: SMC-approved medicines with an interim acceptance decision or via the ultra-orphan pathway

	Product	Indication	SMC decision	Data collection deadline	Reassessed?
Interim acceptance	Lorviqua	ALK+ NSCLC	Mar 2020	Not specified	No
	Holoclar	Limbal stem cell deficiency	Sep 2020	Dec 2020	No (deadline passed)
	Ondexxya	Life-threatening or uncontrolled bleeding	Sep 2020	Jun 2023	No (deadline passed)
	Tecartus	MCL	Aug 2021	Sep 2025	No
	Retsevmo	RET TC and RET+ MTC	Sep 2021	Feb 2025	No
	Enhertu	HER2+ breast cancer	Jan 2022	Not specified	No
	Lumykras	KRAS G12C NSCLC	Mar 2022	Jun 2023	No
	Jemperli	dMMR/MSI-H EC	Mar 2022	2024 (month not specified)	No
	Gavreto	RET+ NSCLC	Mar 2023	Dec 2026	No
	Retsevmo	RET+ NSCLC	Nov 2023	Not specified	No
	Hemgenix	Haemophilia B	Aug 2024	Not specified	No
Ultra-orphan	Spinraza	Types 2 and 3 SMA	Jul 2019	Jul 2022	No (deadline passed)
	Luxturna	Retinal dystrophy	Feb 2020	Feb 2023	Yes (accepted in Jul 2024)
	Brineura	CLN2 and TPP1 deficiency	Oct 2020	Oct 2023	No (deadline passed)
	Waylivra	FCS	Nov 2020	Nov 2023	No (deadline passed)
	Scenesse	Photoxicity in EPP	Feb 2021	Feb 2024	No (deadline passed)
	Translarna	DMD	Apr 2021	Apr 2024	No (deadline passed)
	Libmeldy	MLD	Apr 2022	Apr 2025	No
	Bylvay	PFIC	Jul 2022	Jul 2025	No
	Lamzede	Alpha-mannosidosis	Sep 2022	Sep 2025	No
	Crysvita	X-linked hypophosphataemia	Feb 2023	Feb 2026	Yes (accepted in Jan 2024)
	Rezurock	cGvHD	Jul 2023	Jul 2026	No

CONCLUSIONS

- The interim acceptance decision and ultra-orphan pathway have provided access to medicines whilst evidence generation activities are ongoing, with two examples of successful re-assessment
- However, re-assessment delay for several products suggest potential challenges with the process and/or quality of data collection
- It would be important to track future outcomes to examine whether these routes offer access to effective products, or risk exposing patients to costly therapies with no proven clinical benefits

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