#HTA321

The interim acceptance and ultra-orphan pathways in Scotland – an update

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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 introduced a similar route unique for medicines targeting ultra-rare diseases, deemed the 'ultra-orphan pathway', allowing these products to be reimbursed for up to three years while further evidence is generated for reassessment.

OBJECTIVE

- This research evaluates the impact of interim acceptance decisions and the ultra-orphan pathway on access to medicines in Scotland.
- The aim is to understand the frequency of reassessment within the given timeframe, and to ascertain whether these approaches are successful in driving access to medicines.

METHODS

- Interim acceptance decisions and medicines approved via the ultra-orphan pathway were identified from the SMC website (16-Oct-2025).
- This included key decisions regarding initial assessments, reassessments as well as non-submissions (where applicable).

RESULTS

- Since its inception, **12 products have received an interim acceptance decision.**
  - No products thus far have received a re-assessment outcome, despite the data collection deadline having passed for 7/12 products (58%); 1 product was globally discontinued.
- For the ultra-orphan pathway, **17 products have been accepted.**
  - 3/17 (18%) have been re-evaluated (all with positive recommendations) and 1 (6%) was not recommended for use due to non-submission.
  - The three-year evidence generation deadline has passed for 5 other products (29%), with no re-assessment outcome available yet.

Table 1: SMC-approved medicines with an interim acceptance decision

	Product	Indication	SMC decision	Data collection deadline	Reassessed?
Interim acceptance	**Lorviqua**	*ALK+ NSCLC*	Mar 2020	Not specified	Pending
	Holoclar	*Limbal stem cell deficiency*	Sep 2020	Dec 2020	No
	Ondexxya	*Life-threatening or uncontrolled bleeding*	Sep 2020	Jun 2023	No
	Tecartus	*MCL*	Aug 2021	Sep 2025	No
	Retsevmo	*RET TC and RET+ MTC*	Sep 2021	Feb 2025	No
	Enhertu	*3L+ HER2+ breast cancer*	Jan 2022	Not specified	Pending
	Lumykras	*KRAS+ G12C NSCLC*	Mar 2022	Jun 2023	No
	Jemperli	*dMMR/MSI-H EC*	Mar 2022	2024 (month not specified)	No
	Retsevmo	*RETi naïve RET+ NSCLC*	Nov 2023	Not specified	Pending
	Hemgenix	*Haemophilia B*	Aug 2024	Not specified	Pending
	Elrexfio	*4L relapsed/refractory multiple myeloma*	Sep 2024	Not specified	Pending
	Gavreto	*RET+ NSCLC*	Mar 2023	Dec 2026	No (discontinued aside from US/CN in 2024)

Table 2: SMC-approved medicines via the ultra-orphan pathway

	Product	Indication	SMC decision	Data collection deadline	Reassessed?
Ultra-orphan	**Spinraza**	*Type 2 & 3 SMA*	May 2018	Jul 2022	Yes
	Luxturna	*Retinal dystrophy*	Feb 2020	Feb 2023	Yes
	Brineura	*CLN2 & TPP1 deficiency*	Oct 2020	Oct 2023	No
	Waylivra	*FCS*	Nov 2020	Nov 2023	No (non-submission)
	Scenesse	*Phototoxicity in EPP*	Feb 2021	Feb 2024	No
	Translarna	*DMD*	Apr 2021	Feb 2025	No
	Bylvay	*PFIC*	Jul 2022	Jul 2025	No
	Libmeldy	*MLD*	Apr 2022	Oct 2025	No
	Crysvita	*X-linked hypophosphataemia*	Feb 2023	Mar 2026	Yes
	Lamzede	*Alpha-mannosidosis*	Sep 2022	Apr 2026	Pending
	Rezurock	*cGvHD*	Jul 2023	Aug 2026	Pending
	Myalepta	*Generalised or partial LD (both confirmed/acquired)*	Jun 2023	Sep 2026	Pending
	Xenpozyme	*CNS manifestations of ASMD with type A/B or B*	Sep 2023	Feb 2027	Pending
	Upstaza	*AADC deficiency with a severe phenotype*	Sep 2023	Apr 2027	Pending
	Filsuvez	*Partial thickness wounds of DEB & JEB*	Jul 2024	Oct 2027	Pending
	Nulibry	*MoCD Type A*	Jan 2025	Feb 2028	Pending
	Casgevy	*TDBT*	May 2025	Jun 2028	Pending

Key:	*Yes (accepted)*	*Pending (not passed deadline or unclear)*	*No (deadline passed)*	*No (non-submission/discontinued)*

CONCLUSIONS

- The interim acceptance decision and ultra-orphan pathways have provided access to medicines whilst evidence generation activities are ongoing.
- However, there is a trend of delayed reassessment, with only 3 successful examples in the ultra-orphan pathway (vs. 0 in interim), potentially indicating greater motivation for these higher unmet need indications, but broadly highlighting challenges with the approach (i.e., process data and/or quality of data collection).
- It will 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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