The SMC ultra-orphan framework: 3-year report card

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Introduction

- Health Technology assessment (HTA) in ultra-orphan indications can be very challenging
- Since April 2019, the Scottish Medicines Consortium (SMC) has a new framework for ultra-orphan medicines allowing them to be prescribed and reimbursed for three years while additional evidence is generated pending a final SMC assessment
- This research systematically evaluates all therapies included under this new pathway and compares outcomes to other HTA agencies

Methods

- Medicines included under the new ultra-orphan pathway were identified from https://www.scottishmedicines.org.uk/ (to 16-May-2022)
- These were compared with the respective HTA reports by NICE, GBA, HAS, and NCPE

Results

- Eight therapies have been made available under the new ultra-orphan SMC pathway an average of 30.2 months post-European Commission approval (range:18.3-90.7 months).
  - 5/8 represented their first assessment but 3/8 had previously been not recommended/restricted by SMC
- Compared to other HTA bodies:
  - NICE recommended 6/8 under the Highly Specialized Technologies pathway, all at earlier timepoints (mean 12.3 months), with one restricted (STA) and another HST ongoing
  - NCPE did not recommend any of the six it had assessed
  - G-BA deemed all eight offered additional benefit although for 4, a non-quantifiable benefit was proven due to orphan designation
  - HAS granted four an ASMR IV, three ASMR III and one ASMR II

Table 1: How therapies approved under the SMC ultra-orphan pathway have been assessed by other HTA bodies

Brand	Indication	SMC	NICE	NCPE	GBA	HAS
Spinraza	Type 2 & 3 SMA	Indication expansion Jul-19	Optimized 24-Jul-19	Not recommended 19-Dec-17	Major benefit# 20-May-21	ASMR III* 31-Jan-18
Luxturna	RPE65 IRD	Initial assessment 10-Feb-20	Recommended 09-Oct-19	Not recommended 18-Sep-20	Considerable benefit# 17-Oct-19	ASMR II 27-May-19
Crysvita	XLH	Initial assessment 10-Feb-20	Recommended 10-Oct-18	Not recommended 12-Mar-20	Non quantifiable (orphan)# 04-Oct-18	ASMR IV* 12-Mar-20
Brineura	TPP1	Initial assessment 12-Oct-20	Recommended 27-Nov-19	Not recommended 25-Mar-19	Non quantifiable (orphan)# 21-Dec-17	ASMR III 24-Oct-18
Waylivra	FCS	Initial assessment 9-Nov-20	Recommended 21-Oct-20	Not recommended 12-Mar-20	Non quantifiable (orphan) 20-Feb-20	ASMR IV* 12-Mar-20
Scenesse	EPP	Re-assessment 08-Feb-21	Assessment ongoing	Not assessed	Non quantifiable (orphan)# 15-Feb-16	ASMR IV 01-Oct-20
Translarna	DMD	Re-assessment 18-Feb-22	Recommended 20-Jul-16	Not recommended 26-Apr-16	Minor# 21-May-15	ASMR IV 18-Nov-19
Libmeldy	MLD	Initial assessment 11-Apr-22	Recommended 28-Mar-22	Ongoing	Major benefit# 04-Nov-21	ASMR III* 30-Apr-21

Time-limited resolution
* SMR important only in a sub-population

Table 2: Stratification of HTA outcomes and delay according to payer archetype and ultra-orphan pathways

		SMC	NICE	NCPE	GBA	HAS
Archetype		Cost-effectiveness	Cost-effectiveness	Cost-effectiveness	Clinical benefit	Clinical benefit
Ultra-orphan pathway?		✓	✓	✗	✓	✗
HTA outcomes	**Positive**	100%	75%	-	100%	38%
	Moderate	-	12%	-	-	62%
	Negative	-	-	75%	-	-
	Not assessed		12%	25%		
Time to reimbursement *(EMA approval to HTA, mean)*		30.2 months	13.1 months	N/A All negative	N/A Available at day 0	14.5 months

Conclusions

- Eight medicines have been made available under this new SMC ultra-orphan pathway
- These have almost all been approved more rapidly by a cost-effectiveness-driven HTA with a specialized very rare disease pathway (NICE) but all been rejected by another cost-effectiveness-driven HTA without such a specialized pathway (NCPE)
- Clinical-benefit driven HTA bodies (HAS and GBA) also issued generally favorable outcomes
- While the focus of most HTA bodies currently remains on evidence at submission, post-launch data collection is becoming an increasingly important access requirement in some markets, and this need for real-world evidence will likely further increase in the future

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