NICE VS SMC: Reimbursement Differences for Non-Oncology Orphan Drugs

HTA138

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INTRODUCTION

In the United Kingdom, the health technology assessment (HTA) bodies, namely the National Institute for Health and Care Excellence (NICE) and Scotland's Scottish Medicines Consortium (SMC), both evaluate clinical and cost-effectiveness in their decisions. However, each organization utilizes distinct criteria, methodologies, and decision-making processes for assessing orphan medicines. Both agencies have unique approaches to evaluating medicines used to treat orphan diseases (≤5 in 10,000).

NICE appraises such medicines through the Highly Specialized Technology (HST) route, while SMC follows the Ultra Orphan Pathway (UO). However, some orphan drugs may also undergo the regular appraisal process. Variations in the processes of NICE and SMC may result in differences in patient access to medicines for treating orphan diseases.

OBJECTIVES

To investigate whether the gap between positive recommendations from NICE vs SMC for non-oncology orphan drugs is widening over time.

METHODOLOGY

All HTA assessments by NICE and SMC from January 2021 to December 2023 are sourced from the NAVLIN HTA Database. Each intervention's orphan designation status, granted by the European Medicines Agency (EMA) with marketing authorization from 2018 to 2023, is verified. Relevant details, including drug name, indication, and assessment decision, are organized to highlight key differences.

RESULTS

Out of the 27 non-oncology orphan drug appraisals examined, NICE and SMC assessed 10 and 7 drugs, respectively, through their HST and UO pathways.

Both organizations gave positive recommendations to 9 drugs, which included Amvuttra, Bylvay, Livtencity, Namuscla, Ngenla, Evrysdi, Libmeldy, Lamzede, and Crysvita. Neither NICE nor SMC received submissions for Cystadrops, Isturisa, or Ebvallo.

In total, **NICE issued more recommendations** with 16, compared to 12 by SMC. However, SMC experienced a higher rate of non-submissions from companies (7) compared to NICE (5) and had one non-recommendation, whereas NICE recorded none.

Comparison of Drug Assessments by NICE and SMC

Drug	NICE (Pathway)	SME (Pathway)
AMVUTTRA	Recommended	Recommended
ASPAVELI	Recommended	Restricted
BYLVAY	Recommended **(HST)**	Recommended **(UO)**
CRYSVITA	Recommended **(HST)**	Recommended **(UO)**
CYSTADROPS	No Submission	No Submission
EBVALLO	No Submission	No Submission
EPIDYOLEX	Restricted	Recommended
EVRYSDI	Recommended	Recommended
FILSUVEZ	Recommended **(HST)**	Restricted **(UO)**
FINTEPLA	Restricted	Restricted
GIVLAARI	Restricted **(HST)**	No Submission
HEPCLUDEX	Restricted	Restricted
IDEFIRIX	Restricted	Recommended
IMCIVREE	Recommended **(HST)**	No Submission
ISTURISA	No Submission	No Submission
JORVEZA	Recommended	Restricted
KINPEYGO	Restricted	No Submission
LAMZEDE	Recommended **(HST)**	Recommended **(UO)**
LIBMELDY	Recommended **(HST)**	Recommended **(UO)**
LIVTENCITY	Recommended	Recommended
MYALEPTA	Recommended **(HST)**	Restricted **(UO)**
NAMUSCLA	Recommended	Recommended
NGENLA	Recommended	Recommended
OXLUMO	Recommended **(HST)**	No Submission
VYVGART	No Submission	Not Recommended
XENPOZYME	No Submission	Recommended **(UO)**
ZOLGENSMA	Recommended **(HST)**	Restricted **(UO)**

RECOMMENDED NOT RECOMMENDED RESTRICTED NO SUBMISSION

HST: HIGHLY SPECIALIZED TECHNOLOGY **UO:** ULTRA-ORPHAN PATHWAY

CONCLUSION

The findings suggest that NICE consistently shows greater flexibility in assessing non-oncology orphan drug treatments, issuing more positive access recommendations compared to SMC. In contrast, SMC adopts a stricter approach, with a higher number of rejections and non-submissions. This trend suggests that the gap in positive recommendations between the two bodies is not narrowing; rather, NICE's more inclusive approach contrasts with SMC's stricter stance, potentially widening the access disparity over time. Notably, there are no instances where SMC provides access that NICE does not, underscoring NICE's comparatively inclusive stance in these evaluations.

REFERENCES

- **NAVLIN Data**: https://data.navlin.com
- **NICE**: https://www.nice.org.uk
- **Scottish Medicines Consortium**: https://scottishmedicines.org.uk

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