Early Access Programs in Northwest European Countries

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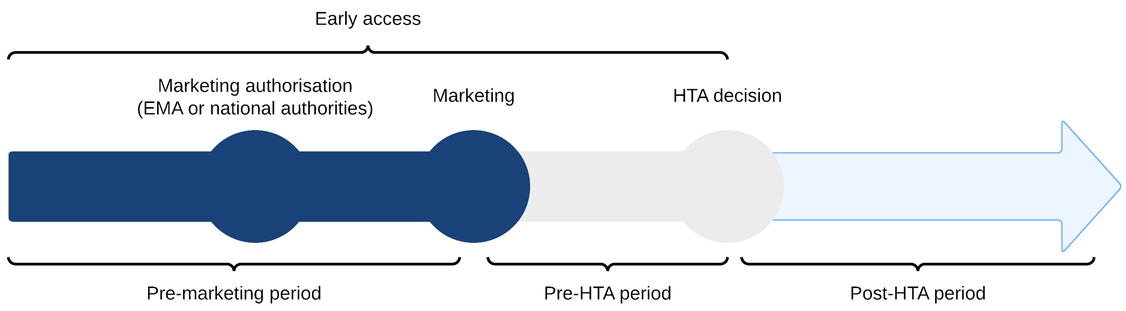
OBJECTIVES

Early access programs (EAPs) refer to patient access to medicines before marketing authorization, potentially extending to price and reimbursement approval, addressing the critical gap in timely access to innovative treatments for patients with unmet medical needs. These programs are especially important as they offer pathways for patients to receive potentially life-saving medications when no alternatives are available. Most European countries have implemented EAPs following the EU legislative/regulatory framework for early access of innovative products at the national level, including compassionate use (CU), named-patient basis access (NP), and expanded access (options arising from participation in clinical trials). However, international research indicates that there are more differences than similarities among national EAPs. This study aimed to compare the EAPs for innovative medicines and their utilization and implementation in Germany, France, the United Kingdom, Sweden, Denmark, and Norway.

Fig 1. Overview of events for early access concerning pharmaceuticals.



METHODS

A mixed-method analysis was employed, incorporating a systematic literature search, semi-structured interviews, and descriptive statistics. Interviews were conducted with national experts from the selected countries, and statistics on the number of EAPs were gathered from national authorities.

RESULTS

All the countries have national EAPs, however, the lack of standardization and common terminology poses challenges for comparison. Differences were observed in the utilization of programs, potentially depending on the funding sources. The main differences include the types of programs implemented, who pays for the pharmaceuticals, the existence of external funding opportunities, whether data collection for health technology assessment (HTA) purposes occurs, the degree of centralized/decentralization, and whether programs could continue post-marketing.

CONCLUSION

Significant differences exist between the countries' EAPs, which can create unequal access to new innovative medicine for patients across national borders.

Table 1. Summary of differences between early access to pharmaceuticals in selected countries.

		Denmark	Sweden	Norway	United Kingdom	Germany	France
Type of programmes	Pre-marketing authorisation period	General and single dispensing permits	License and compassionate use programme (CUP)	Godkjenningsfritak and CUP	Early access to medicines scheme (EAMS) and named patient programme (NPP)	CUP	Autorisations temporaires d'utilisation nominatives and autorisation temporaire d'utilisation de cohort
	Pre-HTA period	Individual assessment			Managed access agreement (MAA) (by fund financing) and NPP		
Data collection for HTA application		No	No	No	Yes (only applicable for EAMS and MAA)	No	Yes
Decentralised/ centralised management		Centralised (pre-marketing authorisation period) Decentralised (pre-HTA period)	Centralised	Centralised	Centralised	Centralised	Centralised
Continuation of patients in the pre-HTA period		No	Yes	Yes	Yes	Yes	Yes

FUNDING This study was funded by The Danish Association of the Pharmaceutical Industry. The sponsor contributed to the process, inputs, and contacts, but the academic authors made the final determination on which analyses to present.