

22730

Routes for access to off-label treatments: A comparative analysis of 19 European countries

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Background

The EU Health Technology Assessment Regulation (EU HTAR) and the European Medicines Agency (EMA) enable European collaboration on the evaluation of new indications; off-label use remains outside its scope. Off-label use is common in oncology; in The Netherlands, 35% of reimbursed oncology and haemato-oncology indications is off-label (331/900). It is unclear how such indications are regulated and reimbursed across countries.

Methods

Through the Pharmaceutical Pricing and Reimbursement Network (PPRI) for governmental agencies, we used a questionnaire to collect information from 19 European countries regarding legal requirements and reimbursement pathways for off-label use.

Results

All countries allow off-label prescribing. 53% (10/19) require or advise informed consent for non-established use. Only 21% (4/19) require that no on-label alternative exists. For reimbursement, 79% (15/19) of countries have a dedicated route, 53% (10/19) have an off-label reimbursement list, and 53% (10/19) may require prior authorisation. In 26% (5/19), decisions on off-label reimbursement are made regionally. All countries reported that off-label access is sufficiently ensured. Table: 22730

Summary of results

	Legal prescription requirements		Reimbursement requirements			
	Informed consent required or advised (%)	No on-label alternative: As last resort only (%)	Dedicated route for off-label requests (%)	Off-label list of approved indications available (%)	Prior authorisation necessary for (some) requests (%)	Regional decision making (%)
Yes	10 (53)	4 (21)	15 (79)	10 (53)	6 (32)	5 (26)
No	9 (47)	15 (79)	4 (21)	9 (47)	10 (53)	14 (74)
Unknown	0 (0)	0 (0)	0 (0)	0 (0)	3 (16)	0 (0)

Conclusions

Although legal requirements vary, all countries allow off-label prescriptions under certain circumstances. For reimbursement, there are major differences between countries. Unfortunately, it is untransparent which indications are reimbursed in which countries, hindering sharing assessments and reasons for allowing or rejecting reimbursement. Especially for expensive drugs, many countries require individual permission before reimbursement. This process may be untransparent, time-consuming, and depend on the oncologist's initiative and funder's approval. Particularly for rare cancers off-label use may be the only treatment option. To provide access to valuable off-label treatment options, policy changes are recommended, preferably harmonised at the European level.

Legal entity responsible for the study

The authors.

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Disclosure

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