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Health technology assessment: oncology drugs with orphan designation as an example

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Background
Since (limited) health care resources have to be invested efficiently, HTA/ health technology assessment is applied ever more often in many health care systems for 'rational decision-making'. Around 40% to 50% of all drugs with orphan designation are high-priced oncology drugs. Within EUnetHTA (an EU-supported project) a work package deals with the pre-coverage exchange of early assessments among European countries and the collaboration on projects for further generation of evidence.

Methods
Description of methodology of the early assessment of oncologic drugs and possible mechanisms of exchange of pre-coverage knowledge within Europe.

Results
A temporary coverage/funding of drugs with orphan status often requires additional collection of data on safety, effectiveness, cost-effectiveness, and the appropriate use of the drug. Many of the oncology drugs show little (or marginal effectiveness) at time of approval and reimbursement agencies demand further data before deciding whether to cover the new drug. Pragmatic clinical trials, patient access schemes and standard data requirements on patient relevant outcomes in registries across Europe are some of the approaches to generate further evidence and to fill the gap between knowledge on efficacy at time of approval and demanded knowledge on effectiveness for coverage decisions. EUnetHTA provides the necessary structures for coordinated efforts.

Conclusion
Exchanging information on and developing tools to facilitate evidence generation and collaboration on the assessment of new costly technologies, many of them drugs with orphan designation and a reduction of duplication of assessments is the intention of EUnetHTA.

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