VP71 Barriers To Access Biologic Products: A Rapid Review

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Introduction. The elevated costs with biologic products threaten the sustainability of health services, and, therefore, the access to these medicines in the perspectives of user, health professional, health manager and system. The entry of biosimilar products in the market could be an option to subsidize the search for solutions to those problems.

Methods. We conducted a rapid review using the databases Medline (via PubMed), EMBASE, Cochrane Library and CRD. The eligibility criteria were HTAs, systematic reviews and cross-sectional studies.

Results. Literature search retrieved 640 registries and, after duplicate removal, screening of titles and abstracts and full text reading, nine cross-sectional studies were selected. From a user's point of view, the following barriers were identified: lack of knowledge about the medicine, distance between the place of living and the health service (especially in the rural area), long waiting periods for service, passivity in regard to treatment. From a health professional's point of view the barriers were: acceptability of the expert in regard to treatment, interchangeability and substitution, the perception of lack of data showing efficacy and safety. Finally, from the payer's (or health manager) point of view, the barriers were: high cost of medicine, problems with reimbursement and bureaucracy. We did not retrieve any barriers from the health system's perspective from the selected studies.

Conclusions. The entry of biosimilar medicines in the market can induce competition and, therefore, reduce prices of biologic treatments. It is necessary to search for potential solutions to the access barriers identified in this rapid review.

VP72 Impact Of Comparator Choice On Oncology Drugs' Market Access

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Introduction. In France, drug assessment is performed by the Transparency Committee (TC) of the French National Authority for Health (HAS). It's based on two criteria: the clinical benefit (CB) for reimbursement recommendation and the clinical added value (CAV) serving the pricing decision. The CAV is rated on a 5-point scale, from I (major) to V (no CAV). A critical step in the CAV assessment is the identification of the clinically relevant comparators (CRC) serving the TC to recognize the appropriateness of the comparators chosen in the randomized controlled trials (RCT). The objective of this study is to investigate the comparator choice consequences on TC appraisals and pricing.

Methods. A retrospective, descriptive study included all oncology indications assessed by the TC between 2015 and 2017. Based on a pre-specified grid, items on the comparators were extracted from final TC's appraisals.

Results. Among the 135 indications included, the assessed drugs had no CRC in 20% of cases. A RCT was submitted for 89 indications (66%) whose 67 (76%) were conducted versus a CRC. A CRC was identified by the TC for 70% of the 46 indications without RCT. An important/moderate CAV (II-III) was granted when there was a RCT versus a CRC in 70% of cases, versus 50% and 43% for minor (IV) and no CAV respectively. The public price was reduced by 13.5% in average compared to the claimed price without impact of the CAV level (n = 18).

Conclusions. In oncology, comparative data assessed by the TC met its expectations (RCT versus CRC) in a majority of cases. When there is no RCT or a comparison versus a non-relevant comparator the CAV appraisal is decreased. Surprisingly this study hasn't shown any impact of this decrease on the public price. A wider analysis in different medical areas would need to be performed to better investigate these results.

VP74 Orphan Black Box: Explanatory Principles

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Introduction. Orphan legislations over the past thirty years have successfully increased the number of drugs receiving marketing authorization for rare diseases. However, for a therapy to be accessible to most patients, it requires not only marketing authorization, but market access via public reimbursement. In many major markets, the pricing and reimbursement of new therapies is based on an assessment by a national Health Technology Assessment (HTA) body, for which economic value is typically a key consideration. This research evaluates the outcome of HTAs of orphan drugs in Europe.

Methods. HTA decision data (to 31/08/2017) was extracted from Gemeinsame Bundesausschuss (G-BA), Haute Autorité de Santé (HAS), National Institute for Health and Care Excellence (NICE), Pharmaceutical Benefits Advisory Committee (PBAC), and Scottish Medicines Consortium (SMC) websites. EC-approval data was extracted from the European Medicines Agency (to 31/08/2017).

Results. Only a small minority of drugs for orphan diseases received full recommendations for their licensed indication(s) by NICE (3/35, 9%), SMC (8/66, 12%) and PBAC (1/44, 2%). 37% (26/70) of drugs assessed received positive HTA outcome by HAS (ASMR I-III). In Germany, all approved orphan drugs (100/100) received automatic additional benefit post regulatory approval by G-BA.

Conclusions. There have been significant challenges for manufacturers in converting regulatory approval of orphan drugs into commercial success and optimised market access. Attaining positive HTA appraisals for these drugs, which have been approved under expedited regulatory pathways on a less than fully mature

https://doi.org/10.1017/S0266462319003271 Published online by Cambridge University Press