Methods. A total of 656 HTA appraisals from 6 European countries were collected for NASs that received regulatory approval between 2012 and 2020. Multivariable logistic (positive and positive with restrictions vs. negative HTA recommendation as dependent variable) and linear regression (rollout time as dependent variable) models examined associations with regulatory orphan designation, expedited process, product type (biotechnological vs chemical), and jurisdiction (France, England, Germany, Poland, Scotland and Sweden). Rollout time was defined as months elapsed from regulatory submission to HTA recommendation (mean± standard deviation).
Results. Multivariable logistic regression analysis identified disparities in HTA recommendations between countries. Every month increase in rollout time conferred a 3 percent reduction in the odds of a positive recommendation ($p<0.001$). Review and product type did not show associations with HTA recommendation. Interestingly, orphan products showed a 99% increase in the odds of obtaining a positive HTA recommendation compared to non-orphan (p-value=0.003). We found 244 appraisals (37%) assessing an orphan product, of which 202 (83%) received a positive HTA recommendation.
Multivariable linear regression analysis indicated that orphan products presented a 4.4-month rise in rollout time when compared to non-orphan products ($p<0.001$). The mean rollout time in months for orphan products were 25±12 in France, 30±15 in England, 21±9.1 in Germany, 37±16 in Poland, 25±12 in Scotland and 27±14 in Sweden.
Conclusions. Orphan designated products showed greater odds of receiving a positive HTA recommendation compared with non-orphan. A more detailed review of orphan products could result in their longer rollout time compared with non-orphan counterparts. Considerable differences were found between HTA recommendations and rollout times between jurisdictions.

PD48 Does Unmet Need Influence The Scottish Medicines Consortium Health Technology Assessment Decisions For Rare Disease Conditions?

Karen Mark (karen.mark@clarivate.com) and
Vishnu Priya Wahal

Introduction. The Scottish Medicines Consortium (SMC) defines end-of-life medicines as drugs used for treating conditions that usually lead to death within three years using currently available treatments. Orphan medicines are drugs used for the treatment of very rare conditions and ultra-orphan drugs are used for the treatment of extremely rare conditions. The objective of this study is to determine the influence of unmet need on SMC health technology assessment (HTA) decisions for rare disease conditions.
Methods. The reimbursement data between 2004 and 2021 from SMC for rare disease conditions were included. These data were categorized based on the presence of an unmet need, that is, drugs considered under the orphan or ultra-orphan process, or those that fulfilled SMC end-of-life criteria. A chi square test was conducted to determine an association between the presence of an unmet need and the HTA decision. HTAs without a decision were excluded.
Results. A total of 91 HTAs were included in the analysis of which, 57.1 percent (n = 52) were recommended, and 42.9 percent (n = 39) were not recommended. Out of the recommended reviews, 32.7 percent (n = 17) addressed an unmet need and 67.3 percent (n = 35) did not. Recommended drugs had positive clinical evidence and high cost-effectiveness or the submission of a patient access scheme while negative decisions were associated with lower or uncertain cost-effectiveness. The chi square test result showed no association between the presence of an unmet need and the HTA decision ($p = 0.315$).
Conclusions. Unmet need does not influence the SMC HTA decisions for rare disease conditions. Economic elements were the driving factors in the decision-making process.

PD49 Burden And Cost Of Anterior Cruciate Ligament Reconstruction And Reimbursement Of Its Treatment In Indonesia: An Observational Study

Romy Deviandri (r.deviandri@rug.nl),
Hugo C van der Veen, Andri MT Lubis,
Inge van den Akker-Scheek and Maarten Postma

Introduction. The number of anterior cruciate ligament reconstruction (ACLR) procedures is increasing. However, the likelihood of not allocating sufficient funds for the ACLR procedure in a developing country, especially in Indonesia, is high. This study aimed to analyze costs for ACLR procedures in Indonesia's resource-limited context, determine the burden of ACLR, and propose national prices for ACLR reimbursement.
Methods. A retrospective observational study was conducted on the cost of ACLR from a payer perspective using inpatient billing records from 1 January 2019 to 31 December 2019 from four hospitals. The national burden of ACLR was calculated and national prices for reimbursement were developed.
Results. Of the 80 ACLRs performed, 53 (66%) were isolated ACLRs and 27 (34%) were combined with meniscus treatment. The mean hospital costs incurred per ACLR procedure were USD 2,853 (IDR 40.4 million), which was mainly attributed to the orthopedic implants (USD 1,387; IDR 19.6 million). The costs of ACLR with combined meniscus treatment were estimated to be 35 percent higher than for isolated ACLR. The national burden of ACLR revealed a total budget of USD 367.4 million (IDR 5.2 trillion) per 100,000 patients for ACLR with additional meniscus treatment and USD 271.3 million (IDR 3.8 trillion) per 100,000 patients for the isolated ACLR procedure.

https://doi.org/10.1017/S0266462322003087 Published online by Cambridge University Press

Conclusions. ACLR procedures in Indonesia are underbudgeted. Adjustment of reimbursement prices for ACLR procedures is needed to facilitate access for the Indonesian people. This study showed varying cost estimates for ACLR in Indonesia, necessitating a new reimbursement system that takes various clinical and patient factors into account.

PD50 Value Of Healthcare Journey For Patients With Rare Diseases In The Brazilian Public Healthcare System: Methods And Preliminary Results

Ludmila Gargano (ludgargano@gmail.com), Bianca Caroline Salvador, Luiz Carlos Santana-da-Silva, Gabriel Ogata, Marcelo Eidi Nita, Têmis Maria Félix and JAV-Raras Study Group

Introduction. Implementing value-based healthcare (VBHC) services requires the ability to assess and integrate evolution of patient-centered outcomes (PCO), clinical/epidemiological data and resource consumption. Aligned with Porter's framework of value, the "Value of Healthcare Journey for Patients With Rare Diseases Project" (JAV-RARAS) was implemented to evaluate the value of the healthcare journey of patients with rare diseases (RD) in the Brazilian Public Healthcare System (SUS). The goal of this project is to identify the value (clinical outcomes and costs) associated with the management of RD in the SUS.
Methods. Patients diagnosed with eight pre-defined RD under active clinical follow-up in SUS medical centers were invited. Retrospective data on diagnosis and assistance carried out after diagnosis were collected from medical records. Prospective follow-up of one year will be collected through validated clinical questionnaires on three visits, with an average interval of 6 months. Clinical outcomes include exams and disease-specific features, quality of life, productivity loss, treatment adherence and satisfaction. Total cost of patient's journey in each medical center will be assessed through Time-Driven Activity-Based Costing method. All data collection is being carried out through the Think Patient Value (TPValue®), a computerized management support system, based on Porter's paradigm of value.
Results. Thirty-five medical centers currently assisting patients with RD were enrolled, representing all macro-regions in Brazil: Midwest (n=4), North (n=5), Northeast (n=10), South (n=5) and Southeast (n=11). Recruitment started in March, and until November 2021, 28 centers had reported retrospective data of at least one RD. So far, JAV-RARAS recruited patients with osteogenesis imperfecta (number of patients=106), phenylketonuria (n=52), familial amyloidotic polyneuropathy (n=32), classical homocystinuria (n=25), Prader–Willi syndrome (n=30), acromegaly (n=33), hereditary angioedema associated with C1 esterase-inhibitor deficiency (n=42) and mucopolysaccharidosis type-II (n=17).
Conclusions. Future results of JAV-Raras real-world evidence study will bring PCO and costs according to Porter's Value, bringing insights for decision-makers in SUS. This study was funded by National Council for Scientific and Technological Development – CNPq and Ministry of Health of Brazil –MoH.

PD51 Patient Perspective: A Preliminary Analysis Of The First Year Of The Patient Involvement in CONITEC Meetings

Clarice Portugal (clarice.portugal@saude.gov.br), Andrea Brigida de Souza, Adriana Prates, Andrija Almeida, Bruna Cabral de Pina Viana, Luiza Losco and Vania Canuto

Introduction. The Patient Perspective is an initiative to include the testimonials of patients or their representatives at the time of the initial recommendation of health technology assessment (HTA) processes developed by the National Committee for Health Technology Incorporation in the Brazilian Public Health System (CONITEC). It allows these actors to be involved prior to the public consultation. This action is articulated with a framework of strategies to encourage patient and public involvement (PPI) in HTA in Brazilian Public Health System (SUS). This paper aims to analyze the first year of its implementation.
Methods. This is a descriptive study, based on document analysis (minutes of meetings, technical and society reports, videos of CONITEC meetings, tables for internal control, etc.) and systematic observation of the activities related to the initiative. Field notes were taken and their contents were coded, following content analysis criteria.
Results. From December 2020 to November 2021, 1,052 subscribers were registered in 75 public calls, which resulted in the participation of 42 people. The testimonials presented information about the illness experience, its impact on quality of life, use of technologies, and difficulties of accessing and adherence to treatment. The patient perspective has been useful to give visibility to specific dimensions of experiences regarding not only the health conditions but SUS as well. Furthermore, some testimonials produced tensions and repercussions about HTA processes, such as the consideration of aspects or technologies not covered in the clinical studies and thus the HTA, as well as the need of reviewing some results of the technical report.
Conclusions. The patient perspective has had the support of the target audience. Additionally, it has been successful in including various types of participants and different aspects of the experience with the health condition and/or the technologies under evaluation. In general, it has been possible to observe the diversification of modes of PPI and institutional learning towards the improvement of HTA in SUS.

https://doi.org/10.1017/S0266462322003087 Published online by Cambridge University Press