PP97 How Health Technology Agencies Estimate Target Population Size For Medical Devices: The Example Of Spinal Cord Stimulation

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Introduction: Spinal cord stimulation (SCS) is an effective and safe option for patients with refractory neuropathic pain, with positive health technology assessment (HTA) recommendations across Western Europe, yet SCS uptake remains low. Estimating target patient populations within HTAs may impact upon medical device uptake, pricing and access. However, there is a dearth of information on how this is typically conducted. This study aimed to compare the approaches of Western Europe HTA agencies for estimating the target population for SCS.
Methods: A survey was conducted among members of the Health Economics and Reimbursement function from Western Europe to collect country-specific information on how HTA agencies assess the target population of medical devices (MD). The estimations of the target population for SCS were extracted from HTA publications and compared.
Results: Eight Health Economics and Reimbursement (HER) Analysts from France, Germany, the United Kingdom (UK), Belgium, Spain, Italy, Sweden and Norway completed the survey. HTA Agencies in France, UK and Belgium routinely ask for epidemiological data in the manufacturer submission, whereas in Germany, Sweden and Norway the request is dependent on the type of HTA submission. All HTA agencies, except NICE (UK), perform an independent estimation of the target population. HTA agencies in Germany and UK typically use epidemiological data from industry. In all countries, the estimation of the target population may indirectly impact the price of the MD, especially when budget impact analysis indicates a potential high use of healthcare resources. Only France, Belgium and UK have published HTA recommendations about SCS, however the estimated target population, nor the number of patients with refractory neuropathic pain, is not always included. Only the French and UK HTA agencies publish an approximation of the target population for SCS using the number of patients implanted every year.
Conclusions: This study showed there is a lack of harmonization between Western European HTA agencies' guidelines on the inclusion and estimation of target patient populations. The new EU HTA Regulation should help to address this situation.

PP98 Phelan-McDermid syndrome: Methodology For Creating A Patient Adapted Version Of A Clinical Practice Guideline

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Introduction: Phelan-McDermid syndrome (PMS) is a chromosomal disorder caused by the loss of the end of chromosome 22, that is manifested as a neurodevelopmental disorder. Providing an adapted version of a guideline was seen as essential, as currently, there are no such booklets based on an international guideline for PMS patients, families and caregivers. The European Reference Networks (ERN) Guidelines programme results from a call for proposals funded (DG SANTE/2018/B3/030) for the development of Clinical Practice Guidelines (CPG) and Clinical Decision Tools in the area of rare diseases. Based on this European consortium, the purpose of this study is to describe how two Spanish HTA agencies, OSTEBA-BIOEF (Basque Office for Health Technology Assessment) and AETSA (Andalusian Health Quality Assessment Department) methodologically support the ERN-ITHACA (Rare malformation syndromes and rare intellectual and neurodevelopmental disorders) in the development of a comprehensive patient booklet based on a CPG to be used as an adjunct in the management of PMS syndrome that will be published in 2023.
Methods: A preliminary booklet was created by HTA agencies using the new European guideline for PMS and a Dutch guideline. The booklet structure is an adaptation based on a European Commission template with the guidance of the methodological Handbook#11.
Results: Through a comprehensive adaptation, following the PMS guideline and the Handbook #11, a booklet is developed for the PMS patients. Composed by 28 pages in DIN A5 format were introduction, diagnosis, treatment, pregnancy, do's, supportive care, social networks (including a QR code) and a glossary are included. The selection of a symbol, colors at a chromatic level, a typography and graphical elements as illustrations were created as a corporate identity. Clinical experts and patient representatives that have participated in the creation of the guideline will assess and validate the booklet.
Conclusions: Collaboration between agencies, clinicians and patients is critical to obtain evidence-based products adapted to the needs of patients and people involved in their care.

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