PP574 Cost-Effectiveness Of Newborn Screening Of Primary Immunodeficiency Diseases: A Systematic Review And Economic Evaluation

Junting Yang, Fan Zhang, Zhao Liu, Ning Yue and Yuehua Liu (nhdrc_hta@126.com)

Introduction. Primary immunodeficiency diseases (PIDs) are a heterogeneous group of over 200 disorders with defects in the function and/or development of the immune system. Although early screening is imperative for improving therapeutic efficiency and preventing disease-associated morbidity, its widespread use has been limited, owing to the low incidence of PIDs. It is particularly important to evaluate the cost-effectiveness of PIDs screening for newborns. The aim of this study was to provide an overview of the existing cost-effectiveness evidence on newborn screening of PIDs and to provide reference for decision-makers in China and other developing countries.

Methods. We conducted a systematic review using three electronic databases (PubMed, CNKI, and CSPD) of cost and cost-effectiveness studies of PIDs screening published during 2000–2019. Two reviewers independently searched databases and screened titles, abstracts and full texts; a third reviewer resolved disputes when necessary. The initial search returned 124 references, of which 10 full articles were included in the review. Five of the studies conducted analyses using model-based techniques.

Results. Severe combined immunodeficiency (SCID) was the predominantly studied condition (80%). Most studies (70%) examined the T-cell receptor excision circle (TREC) assay. A healthcare system's perspective was commonly used (50%) for cost calculations, and most studies (50%) were US-based. The majority (67%) of the studies found the TREC assay an effective screening tool for SCID, but the incremental cost-effectiveness ratio (ICER) varied across screening test specificity and disease incidence.

Conclusions. Evidence from the published literature demonstrated that newborn screening for PIDs generally appeared to be cost-effective, and most importantly, it is lifesaving and allows children with PIDs an opportunity to live a healthier life. However, the type of PIDs included in this study were limited and most studies were done in developed countries whose health systems are different from low-/middle-income countries (LMIC). Further research is required to identify the cost-effectiveness of PIDs screening both in developed and developing countries.

PP581 Catastrophic Costs Of Multidrug-Resistant Tuberculosis: Estimation Based On The Cost Of Treatment In China

Lijun Shen, Shangshang Gu, Fan Zhang (zhangfan514c@gmail.com), Zhao Liu and Yuehua Liu

Introduction. China bears a considerably high burden of multidrug-resistant tuberculosis (MDR-TB). Second-line anti-TB drugs are urgently needed yet domestic MDR-TB drugs are expensive and lack policy support. Patients' living conditions are closely related to the drug affordability. The national TB prevention programs should play a critical role. The purpose of this study is to measure the cost of treating MDR-TB patients under different treatment schemes and price sources. The results of this study are expected to inform the relevant drug protection policies and provide inputs for further cost-effectiveness analyses.

Methods. Based on the treatment plan of China's Multidrug-Resistant Pulmonary Tuberculosis Clinical Path (2012 edition) and the World Health Organization (WHO) Drug-Resistant Tuberculosis Treatment Guide (2018 edition), the treatment costs of MDR-TB were measured under different scenarios. Catastrophic health expenditure was then calculated if the treatment cost exceeds 40 percent of the household's non-subsistence income. National, rural and disposable income per capita in 2018, were used to represent Chinese patients' affordability.

Results. Under varied treatment schemes and market price sources in China, the total costs for MDR-TB patients range from 19,401 to 126,703 CNY [2,853 to 18,633 USD] per person. Under current prices, all treatment schemes recommended by the WHO will incur catastrophic costs for Chinese MDR-TB patients. Significant differences were found between rural and urban areas as 52.8 percent of the treatment listed in the 2012 China Guideline would lead to catastrophic cost for rural patients but not urban ones.

Conclusions. Our study concludes that the domestic drugs are more expensive than the international purchase price and the treatment of MDR-TB imposes substantial economic burden on patients, especially in the rural areas. The results of the study also indicate that it is urgent for the state to emphasize government responsibility and initiate centralized procurement for price negotiations to reduce the market price of MDR-TB drugs. The urban-rural gap should also be addressed in the design of future policies to ensure the drug affordability for all patients in need.

PP585 A New Hope For Breast Cancer Survivors: Early Assessment Of A Breast Cancer Vaccine

Norrina Jamaluddin (norrina83@gmail.com), Izzuna Mudla Mohamed Ghazali and Junainah Sabirin

Introduction. Breast cancer is the most frequent cancer among women globally, impacting 2.1 million women each year, causing the greatest number of cancer-related deaths among women. In Malaysia, the new cases of breast cancer comprised of 32.7 percent of all new cancer cases in women as reported by The International Agency for Research on Cancer (IARC). The recurrence rate was about 16.4 percent post-mastectomy. This early assessment is to evaluate the effectiveness and safety of a breast cancer vaccine.

Methods. A systematic review was conducted. Searches were done through PubMed, Medline and ClinicalTrial.gov. The articles were selected based on inclusion and exclusion criteria and appraised

https://doi.org/10.1017/S0266462320002044 Published online by Cambridge University Press

using Critical Appraisal Skills Programme (CASP) checklist. More than twenty cancer vaccines under development were identified.

Results. The most advanced breast cancer vaccine is Nelipepimut-S (NPS). In a Phase 2b clinical trial, improvement disease-free survival (DFS) in the NPS group was 89.8 percent. The DFS rate in the NPS group was 92.6 percent for triple negative patients. Median DFS in the NPS group [hazard ratio (HR): 0.26 (95% confidence interval, Cl: 0.08–0.81)] showed a significant difference. A projection study showed the cost-effectiveness will be 90 percent success if the cost less than USD1,000 per patient. No safety issues were reported.

Conclusions. Early assessment showed potential benefit in patient with triple-negative breast cancer (TNBC). However, further research is required to ensure its efficacy, safety, and cost-effectiveness.

PP612 Use of Rapid Review Methods In Health Technology Assessment For Central Procurement Decisions: Experience In Ukraine

Oresta Piniazhko, Kachveci Rabia, Dumenko Tetyana, Mariya Leleka (lelekamariya@gmail.com), Alona Masheiko and Valeriia Serediuk

Introduction. The Health Technology Assessment (HTA) Department of the "State Expert Center of the Ministry of Health of Ukraine" was established in 2019 for conducting assessments and development of recommendations for informing decisions of the Ukrainian Ministry of Health regarding financing of the health technologies. Since 2015, procurement of medicines by the state budget in Ukraine was carried out through international specialized organizations. The central procurement program covers forty-one programs for different disease areas. The list of medicines and medical devices purchased were based of procurement agreements with specialized organizations approved by Cabinet of Ministers Decree of Ukraine № 255 of 13 March 2019.

Methods. Descriptive analysis of methodological processes around rapid review methods in HTA used for the procedure of central procurements of medicines. In the process of conducting rapid assessment we use a modern tool based on the HTA Core Model® for Rapid Relative Effectiveness Assessments version (V3.0). HTA Guidance in Ukraine was developed in 2017.

Results. The list of government programs, for which rapid review methods in HTAhave been conducted, included following diseases: adult and pediatric oncology, juvenile rheumatoid arthritis, hemophilia, and orphan diseases. Reports include an overview of comparative efficacy, effectiveness and safety, as well as analysis of reports from HTA agencies and thresholds, budget impact for informing decisions for development of the central procurement programs in 2019. The HTA Department conducted more than forty reviews in less than 6 months.

Conclusions. Use of rapid review methods in HTA in Ukraine for informing decisions for the central procurement programs of medicines is the perspective for rational resource allocation and spending. This increases and improves patients' access to effective, safe and cost-effective medicines.

PP613 The Cost Analysis Of Subcutaneous And Intravenous Dosage Forms For Systemic Juvenile Idiopathic Arthritis Treatment In Ukraine

Alona Masheiko (amash014@gmail.com), Oresta Piniazhko, Iryna Romanenko, Mariya Leleka and Valeriia Serediuk

Introduction. The Health Technology Assessment (HTA) Department of the "State Expert Center of the Ministry of Health (MoH) of Ukraine" was established in 2019 for conducting assessments and development of recommendations for informing decisions for the Ministry of Health of Ukraine (MoH) regarding the financing of health technologies based on HTA. The study aimed to conduct cost analysis and compare the treatment costs of two available dosage forms of tocilizumab for subcutaneous and intravenous administration for systemic juvenile idiopathic arthritis in Ukraine. Currently there is a central procurement program financed by the state budget with pharmaceuticals provided to patients with juvenile idiopathic arthritis approved by the Cabinet of Ministers Decree of Ukraine №255 dated 13.03.2019.

Methods. The cost analysis was carried out for tocilizumab over a 1-year horizon per one patient. The analysis included drug manufacturers' prices from the registry of the MoH dated 19.09.2019. The annual number of tocilizumab vials for intravenous infusion for each weight category of patients is approved by the order MoH №334 from 14 February 2019. The direct medical costs were included in the analysis omitting cost of administration.

Results. The direct medical costs of treatment with intravenous tocilizumab per one patient for one budgetary year ranged from between USD7,563.83 and USD30,255.30 depending on patient's weight that was in the range of 10–80 kg. The direct medical costs of treatment per one patient for one year with subcutaneous tocilizumab was USD7,782.40 for patients < 30 kg and USD15,564.80 for patients ≥ 30 kg.

Conclusions. The introduction of subcutaneous tocilizumab can potentially lead to cost savings on average USD 4,041.97 (34.2%) for patients < 30 kg and USD 5,245.82 (25.2%) for patients ≥ 30 kg per one patient for 1-year treatment compared to the intravenous route. Intravenous tocilizumab has an economic advantage over the subcutaneous route solely for the pediatric population of certain weight categories. The cost of intravenous tocilizumab was USD 218.58 lower (2.9%) for patients ≤ 13 kg and USD 2,320.25 lower (17.5%) for patients with weight 31–35 kg, compared to the cost of subcutaneous tocilizumab.

https://doi.org/10.1017/S0266462320002044 Published online by Cambridge University Press