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EDITORIAL

Therapeutic innovations: the future of health economics and outcomes research – increasing role of the Asia-Pacific

The pace of global therapeutic innovation in some branches of clinical medicine, such as infectious diseases, and treatment of some noncommunicable diseases, such as mental disorders and dementia, has been surprisingly stagnating over the past few decades[1]. Yet, at the same time research and development investment has expanded rapidly in oncology, autoimmune, and some rare diseases, harvesting an accelerated pace of pharmaceutical innovation. This has been witnessed by the rise in targeted oncology and diabetes drugs such as (PD-1/PD-L1) inhibitors, tyrosine kinase inhibitors, monoclonal antibodies, and ever more biologics and biosimilars[2]. Some of these innovations are deemed truly essential since they may extend survival prognosis of life threatening diseases such as metastatic cancer from several months up to a few years in some cases[3]. Yet, many of these exceptionally useful medical interventions come at a cost per QALY which is by far exceeding the willingness-to-pay threshold of even the wealthiest OECD countries[4]. This is creating additional pressures on the national health and pharmaceutical expenditures given the existing resource constraints driven by population aging and blossoming of NCDs in most of these societies. Budget impact assessment-based annual and biannual price cuts of blockbuster medicines have become a matter of standard procedure across the leading Asian economies, China and Japan inclusive[5]. Build-up of health technology assessment capacities and implementation of cost-effectiveness based resource allocation is recognized as the unmet need across most rapidly growing emerging economies[6]. Evolving of global biopharmaceutical landscape has been further accelerated with the advent of E-Health principles, 4.0 industrial revolution and in particular medical care robotics. Last, but not least, ongoing rivalry between the US and China in a massive scale investment in Artificial Intelligence,[7] followed by significantly lower AI spending by the European Commission and Russian Federation, are adding to the complexity of contemporary momentum in health care[8].

Keeping in mind all of these diverse factors we decided to run this Special Issue and invite submissions that might explore them and fill some knowledge gaps in the seminal HEOR literature. The first article in this series worked on polatuzumab vedotin-bendamustin-rituximab (PBR) and tafasitamab-lenalidomide (Tafa-L) indicated for relapsed/refractory diffuse large B-cell lymphoma (R/R DLBCL) in autologous stem cell transplant (ASCT) ineligible patients. This was a rare case of an industry-independent pharmacoeconomic evaluation from a US payer perspective. It has proven that sustained Tafa-L treatment demonstrated better survival outcomes than 6-cycle PBR, though at greater cost[9].

The second contribution dealt with economic evaluation using dynamic transition modeling of ebola virus vaccination in lower- and-middle-income countries. It has shown that EVD vaccination in the hypothetical population was found to be cost-effective from the payer perspective. A peculiarity of resource constrained settings, such as some Sub-Saharan African countries, is that various outbreaks may be occurring simultaneously. Thus, such comparative transnational cost-effectiveness assessments might be even more sensitive[10].

The following research by Abraham et al.[11] focused on exploration of highly aggressive small-cell lung cancer using the datasets from a pivotal clinical trial and a US payer perspective. It is widely known that adverse effects such as neutropenia caused by myelotoxicity of chemotherapy significantly decrease survival chances and the ability of the patient to withstand necessary treatment cycles to the end of their prescribed duration. These events are frequently associated with opportunistic infections with rather weak hospital pathogens leading to rare but life threatening conditions like fungal sepsis or low pathogenic bacterial infections poorly responsive to even gold reserve antibiotics. This study has proven that administration of trilacyclib prior to chemotherapy was attributed to lower frequency of these adverse events, higher life quality during essential palliative care in many cases, and even to be significantly cost beneficial.

The next piece is a valuable methodological article which conducted a thorough literature search based on the extracted data of cost-effectiveness pancreatic oncology studies taking place in diverse national jurisdictions while adopting a 2013–2021 time horizon. OECD methodology to convert these findings into one single currency, 2021 USD, relied on a so-called mPPP approach. Surprisingly, even after careful econometric adjustments increasing transnational comparability of findings to almost the highest achievable degree, substantial heterogeneity of findings remains, even within the same national market[12].

Chen et al.[13] explored a quite rare pediatric disease of spinal muscular atrophy (SMA) while relying on retrospective registry analysis from Alberta, Canada. They revealed that direct average costs of care in the first year after diagnosis was approximately $29,774 ($38,407). Hospital admissions, attending physician visits, and primary outpatient care accounted for the majority share of these costs. Although this is a rather traditional cost-of-illness analysis, it brings valuable insights, filling some knowledge gaps given its rare indication and scarcity in seminal literature.

The huge global burden of morbidity of rare genetic disorders accounts for almost 350 million cases with exceptional variability and difficulties to capture the early childhood

diagnosis window. The advanced technique of Next Generation Sequencing being the gold standard in this arena allows room for opportunity, but health economics evidence in this area remains rare. Alam et al.[14] have attempted to prepare a methodologically sound systematic review of the current literature for economic evaluations of NGS in pediatric indications. They largely succeeded in this uneasy task. Conclusive remarks point out that validity of outcomes obtained remains of questionable methodological reliability due to large differences in the unit measurement of cost and efficacy outcomes in singular studies. Yet this challenge may be tackled in the future via guidelines development for health economic assessments in pediatric rare disease populations .

The Asia–Pacific region has been observed by Jakovljevic et al.[6] as being the second leading biotech market globally, outpaced only by North America. Asian pharmaceutical expenditure has been growing substantially faster than real GDP growth for well over a decade. Unmet needs of rising middle class citizens are only partially covered by supply provided by domestic born enterprises and the state-owned hospital sector. Japanese Takeda, Astellas, Daiichi Sankyo, Otsuka and Chinese Sinopharm, Guangzhou Pharmaceuticals Corporation, SPH, and Yunnan Baiyao today belong to the top 25 global pharmaceutical multinationals based on 2020 – 2021 revenues. Even more Asia-Pacific companies contribute to cutting-edge innovation to the global industry landscape.

Last but not least, a large group of authors, primarily based in low- and middle-income countries of the Global South, have provided an insight into a piece of its abundant economic history[15]. Post-Cold War decades have witnessed accelerated real GDP growth across many LMICs and emerging countries of the Global South[16]. Health financing mechanisms and the political economy of health spending continues to evolve rapidly in these vast regions. The insufficient domestic medical device and pharmaceutical industry are complemented by supply of medical goods and services provided by multinational industry[17]. Most global biotech giants are developing their long-term investment and market access strategies with pillars in East Asia and ASEAN countries[18]. Fiscal gaps faced by the local governments need feasible solutions to secure financial sustainability. This is particularly challenging given the accelerated population aging and blossoming of expensive noncommunicable diseases[19]. Cost-effectiveness based resource allocation and health technology assessment (HTA) capacities build-up remain among short listed policy solutions yet are not easy to achieve, even in high-income OECD nations such as Japan and South Korea[6].

This diverse set of contributions has described some of the hot spots of contemporary health economics and outcomes research, ranging from methodological challenges to regional contributions[20]. We sincerely hope that the authors and editors managed to give an intriguing insight into some of the most challenging issues of contemporary development in interdisciplinary HEOR sciences.

Transparency

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