WHAT MAKES A PHARMACEUTICAL PRICING & REIMBURSEMENT PROCESS PATIENT-CENTRIC? A COMPARATIVE ANALYSIS OF 11 SYSTEMS

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OBJECTIVES: To understand how 11 pricing & reimbursement (P&R) processes assess the value of innovative medicines to provide sustainable and timely patient access. BACKGROUND: In November 2014, the UK Minister for Life Sciences announced the Literature Medicines and Medical Technology Review (LM & MTR) to consult various stakeholders on how to improve access to medicines in England. METHODS: Eleven processes were analysed focusing on oncology: Australia, Belgium, Canada, England, France, Germany, Italy, Netherlands, New Zealand, Scotland and Sweden. The decision-making process was split into eight steps: regulatory approval, health technology assessment, appraisal, reimbursement decision, price negotiations, decision enforcement, routine access and later revisions. Data collection was based on the LM & MTR frameworks [Hutton et al. 2006] [Allen et al. 2013]. The analysis relies on a proposed definition of patient-centricity, assuming that value creation for patients should determine the reward of other stakeholders [ Porter 2010]. It was designed to enhance the rationality of the healthcare system (economic and financial sustainability) and the research-based industry (innovation reward). RESULTS: In patient-centric systems, the reimbursement decision tends to be solely based on the therapeutic value of the medicine. Cost considerations are generally addressed by price negotiations in a second stage. Other processes focus more on cost-effectiveness or budget impact (potentially with thresholds), which then gives the reimbursement decision alongside clinical effectiveness; pricing and reimbursement are decided jointly. The English and Scottish processes are the only ones that have no price negotiations with manufacturers. CONCLUSIONS: Patient-centric processes succeed in delivering value to major stakeholders by first deciding on the reimbursement status of a new medicine based on its value to patients. They then independently negotiate a price with manufacturers to maximize financial sustainability for the healthcare system and innovation reward for the manufacturer.

WHAT LIFECYCLE MANAGEMENT LESSONS CAN WE LEARN FROM PD-1 IMMUNO-ONCOLOGY THERAPIES?

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OBJECTIVES: To understand the early lifecycle management strategies of innovative oncology immunotherapies, specifically the PD-1 drugs pembrolizumab (Keytruda, Merck) and nivolumab (Opdivo, Bristol-Myers Squibb), for application in disease areas. METHODS: Targeted secondary research using combinations of key words (‘PD-1’, ‘Keytruda’, ‘Opdivo’, ‘FDA’, ‘Pembrolizumab’, ‘Nivolumab’, ‘Approval’, ‘Immuono-oncology’) identified source literature, which was abstracted and analyzed qualitatively. Key themes were discussed in a consensus meeting and implications of findings were theorized. RESULTS: Several lifecycle management strategies were identified from secondary research, including: indication expansion, patient segmentation using biomarkers, and combining with other drug treatments. Pembrolizumab and nivolumab both received accelerated approval from the FDA for advanced melanoma in late 2014. Nivolumab subsequently received approval for NSCLC in March 2015, while pembrolizumab received approval under FDA Priority Review for the same indication as of June 2015. Nivolumab is also under Priority Review in combination with ipilimumab (Yervoy, Bristol-Myers Squibb) for melanoma. Both PD-1 therapies are in numerous clinical trials, as pembrolizumab and nivolumab show improved response rates in patients with a specific genetic biomarker, which is predictive of response across a range of cancers. CONCLUSIONS: In highly competitive therapeutic areas, manufacturers of innovative products need to consider multiple strategies for on-going, maintaining, protecting and increasing product value. Demonstration of substantial improvements in clinical efficacy over the standard of care in one indication is not sufficient for ‘success’. Earlier access through the FDA’s Breakthrough Therapy designation and accelerated approval program is critical for first-to-market entrants. Expansion into both larger and more niche indications offers a complementary access strategy, while gaining a foothold in combination regimens provide opportunity for further product differentiation. Similar considerations apply to therapies that can treat several indications within a broader disease area.

REAL WORLD EVIDENCE IN ONCOLOGY – STATUS QUO IN GERMANY

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OBJECTIVES: Observational studies can be useful to fill in gaps of randomized studies or for maintaining, protecting and increasing product value. Demonstration of substantial improvements in clinical efficacy over the standard of care in one indication is not sufficient for ‘success’. Earlier access through the FDA’s Breakthrough Therapy designation and accelerated approval program is critical for first-to-market entrants. Expansion into both larger and more niche indications offers a complementary access strategy, while gaining a foothold in combination regimens provides opportunity for further product differentiation. Similar considerations apply to therapies that can treat several indications within a broader disease area.

A PRAGMATIC APPROACH TO DATA SOURCE SELECTION FOR USE IN REAL-WORLD EVIDENCE (RWE) GENERATION

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OBJECTIVES: Real-world evidence (RWE) can be gathered from various sources, including existing registries, claims data or medical records. The challenge is selecting the most appropriate data sources to answer specific research questions. As data sources collect different data elements, the best RWE collection approach for gathering the necessary data will differ per country. The objective was to test a systematic approach for selecting RWE sources per indication (RWE-sources).

Our approach consisted of two workstreams: assessment of existing RWE-sources and inventory of data elements collected in clinical practice. We selected oncology registries and the European Union as the target area. The first step of workstream one is a targeted literature search to identify RWE-sources. Database owners were surveyed on their data. A scoring algorithm was developed to prioritize RWE-sources on the number and type of relevant data elements. Finally, the most promising RWE-sources were selected, and specific data collection recommendations were made on their scope and collaboration possibilities. For workstream two, a small sample of practicing physicians were interviewed on what data is routinely collected in clinical practice. The results from the two workstreams were combined to analyse per country which collection approach is optimal for RWE generation for answering specific research questions. RESULTS: We identified 327 national or regional general cancer registries. Almost each country has databases collecting information on diagnosis and survival data, but databases collecting information on treatment and response to treatment are rare. Interviews revealed that medical records typically collect detailed information on diagnosis, treatment and response, although specific details vary per country. The study provides a methodology for identifying and assessing available RWE-sources. Patient-registries containing detailed data are good sources for RWE gathering, but in countries without cancer registries, data collection from clinical practice is still a feasible alternative for RWE collection.

FROM DIAGNOSIS TO TREATMENT AND SURVIVAL: THE EMOTIONAL JOURNEY OF PATIENTS WITH BREAST CANCER

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OBJECTIVES: Breast cancer has important economic, psychological and social impact for the patients, their families and the health system. Our aim was to record and analyze patients’ specific research questions. METHODS: A qualitative study was performed in December 2014, using a semi-structured interview guide. Participants were recruited through a patient organization. Women with a diagnosis of primary or secondary breast cancer, some of whom had completed treatment were eligible.