PP52 Pharmaceutical Innovativeness Index – FDA-Approved Prostate Cancer Medications (2011 To 2021): A Case Study

Ariane André,
Ludmila Gargano (ludgargano@gmail.com),
Isabela Freitas, Francisco de Assis Acurcio,
Juliana Alvares-Teodoro and Augusto Guerra Jr

Introduction: Prostate cancer (PC) constitutes 13.5% of all cancer cases globally. Treatment is individualized and depends on various factors. In recent years, there has been an increase in the approval of drugs for the disease by the U.S. Food and Drug Administration (FDA). The study aimed to assess the innovativeness of FDA-approved drugs for PC treatment from 2011 to 2021.
Methods: A search was conducted in the FDA database to identify medications approved from 2011 to 2021 and their supporting studies for approval. The assessment of the value of innovations was performed through the Pharmaceutical Innovativeness Index (PII), a methodology that quantitatively evaluates innovations in four domains: Therapeutic Need and Added Therapeutic Value of the new medication (graded on five levels from important to absent), and Study Design and Methodological Quality (graded on three levels) of the pivotal studies used as a data source for evaluation. Medications are assessed for a specific clinical indication and compared to available therapeutic alternatives.
Results: Seven medications were identified for the treatment of PC, targeting different stages of the disease. The drugs were evaluated with a score ranging from 0 to 100, measuring the degree of innovativeness across the four assessed domains. Five (70%) medications scored above 50.0. The majority of the medications addressed a significant Therapeutic Need (n=4; 56%). The Added Therapeutic Value was assessed based on the survival gain compared to the comparator, with five medications considered poor (70%) and two moderate (30%). Regarding the quality of evidence, most studies showed a low risk of bias and a partially adequate design.
Conclusions: In recent years, there has been an increase in the development of drugs for prostate cancer. However, the Added Therapeutic Value shows a small to moderate increase in survival compared to existing treatments. Many studies used a placebo instead of comparing new medications with available therapies. The PII indicates that, despite advancements, new technologies are needed to improve patient survival.

PP53 Potential Cost-Effectiveness And Innovation Headroom Of A More Accurate Companion Diagnostic For PD-L1 In Non-Small-Cell Lung Cancer

Toluwase Akinsoji, Nick Dragojlovic, Cécile Darviot,
Michel Meunier and Larry D. Lynd (larry.lynd@ubc.ca)

Introduction: While targeted therapies have substantially improved survival rates for non-small-cell lung cancer (NSCLC), it remains the leading cause of cancer mortality in the US. Companion diagnostics (CDx) measuring programmed death-ligand 1 (PD-L1) expression help inform NSCLC treatment but have limited accuracy. We assessed the potential value of a more accurate PD-L1 CDx for multiple stakeholders.
Methods: We developed decision tree models to assess the potential cost-effectiveness of a hypothetical new CDx for atezolizumab, a PD-L1 inhibitor used as a first-line therapy for metastatic NSCLC and as an adjuvant therapy for stage II–IIIa NSCLC patients. The sensitivity and specificity of current PD-L1 assays as well as cost and health payoffs were estimated based on data extracted from the scientific literature. We calculated incremental cost-effectiveness ratios (ICERs) for both indications, conducted headroom and threshold analyses, and used model outputs to estimate the size of the US serviceable addressable market (SAM) for a new PD-L1 CDx in the adjuvant indication.
Results: Approximately five percent of metastatic and seven percent of stage II–IIIa NSCLC patients currently tested for PD-L1 expression receive false negative results. An equivalently priced 100 percent accurate PD-L1 CDx would add an average of 0.04 quality-adjusted life years (QALYs) and cost USD6,069 more per metastatic NSCLC patient (ICER: USD144,512/QALY gained; 95% confidence interval [CI]: USD74,178, USD206,937). It would add 0.08 QALYs and cost USD3,682 more for stage II–IIIa NSCLC patients (ICER: USD49,031/QALY gained; 95% CI: USD47,104, USD50,064). The maximum value-based price in stage II–IIIa NSCLC (assuming a USD100,000/QALY willingness-to-pay threshold) would be ˜USD4,000/patient. At a USD500 price per unit, the SAM would be ˜USD6.5M/year.
Conclusions: Because existing PD-L1 assays for NSCLC are optimized for specificity, the cost-effectiveness of a more accurate CDx reflects that of the immunotherapy drug it is paired with. Stage II–IIIa NSCLC has much greater innovation headroom for a new PD-L1 CDx than metastatic NSCLC. Early cost-effectiveness modeling can identify CDx use cases with higher value headroom and help inform market sizing.

https://doi.org/10.1017/S026646232400223X