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Utilization of Real-World Evidence (RWE) in pharmacotherapy optimization in selected therapy areas

Streszczenie w języku angielskim / Summary of the dissertation in English

Real-World evidence (RWE) is a dynamically developing field in public health science, as it offers previously unprecedented richness in describing the health status of populations and their struggle with various diseases. Despite the rise in the use of RWE worldwide, its full potential is yet to be realized. RWE continues to be formally used in a relatively narrow way, and its popularity is often limited to registration of pharmaceuticals or reimbursement decisions. At the same time, the key element of the actual impact on patients' outcomes and behaviors is at the moment rarely measured, analyzed and utilized in decision making in healthcare. Hence, further use of RWE in optimizing pharmacotherapy offers a more holistic and practical understanding of treatment outcomes, costs, and real-world implications, providing valuable insights for evidence-based decision-making and policy development in healthcare systems.

This doctoral thesis presents the selected aspects of RWE utilization in the context of the societal implications of pharmacotherapy in Poland, emphasizing its potential as a crucial tool for health policy planners. The dissertation highlights the multifaceted impact of pharmacotherapy beyond healthcare, extending to areas such as patient wellbeing, worker productivity, and economic sustainability. It emphasizes the importance of utilizing available data sources to effectively plan, monitor, and assess the outcomes of pharmacologic interventions to optimize resource allocation for the purpose of achieving maximum available societal benefits.

The thesis consists of four thematically linked scientific articles published in peer reviewed journals. In Article 1, the focus is on the management of rare diseases, examining its impact on patients, caregivers, healthcare facilities, and the public financing system. Through the case study of spinal muscular atrophy (SMA), the article demonstrates the feasibility of implementing a system that improves patient outcomes while maintaining costs within a manageable budget. This was feasible also by the introduction of initiatives like the newborn screening program, which emphasizes a proactive approach to healthcare. Article 2 explores the link between pharmaceutical consumption and worker productivity, particularly in the context of the COVID-19 pandemic. It highlights a discrepancy where despite significant changes in worker behavior due to the pandemic, pharmaceutical consumption remained unaffected. This underscores an issue in mental health policy,

suggesting a need for further investigation and policy action to address underlying issues. Article 3 discusses the potential benefits of enforced generic substitution as a policy tool to positively impact patients and the public payer. Similarly, Article 4 demonstrates the superiority of combination therapy in terms of cost and patient adherence compared to alternative approaches.

The thesis concludes with practical implications, advocating for the use of pharmacotherapy to achieve various societal benefits such as reduced costs for patients and public payers, improved healthcare outcomes, enhanced treatment adherence, and increased worker productivity. Key recommendations include stronger utilization of RWE in shaping health policy and optimizing pharmacotherapy, widening of access to care and diagnosis, promoting optimal therapy selection and adherence, increasing awareness of the benefits of pharmacotherapy, and investing in initiatives such as newborn screening for rare diseases.

Overall, this work underscores the importance of optimal pharmacotherapy in shaping health policy and offers insights into how data can be leveraged to address societal challenges and improve healthcare outcomes in Poland.