

Health Economics and Healthcare Policy in Rare Disorders

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Description

Rare diseases are often more difficult to diagnose and treat than the more common diseases. These diseases are typically chronic, progressive and often life-threatening. Despite their rarity, the aggregate burden of rare disorders is substantial due to the high costs associated with their diagnosis, treatment and management. Health economics and healthcare policy play vital role in addressing the unique challenges posed by rare disorders, balancing the need for emerging with the practicalities of funding and resource allocation. Rare disorders encompass a wide range of diseases, each with distinct characteristics and medical needs. The economic burden of these disorders includes direct costs. According to studies, the annual per-patient cost of treating rare disorders can be exponentially higher compared to common diseases. Rare disorders often require extensive and expensive diagnostic testing, including genetic sequencing, to accurately identify the condition. Treatments for rare disorders are frequently cutting-edge and costly, involving gene therapies, biologics or orphan drugs that are priced at a premium due to limited patient populations. Many rare disorders necessitate lifelong treatment and management, which adds to the cumulative economic burden over time. Healthcare policies for rare disorders must strike a balance between incentivizing pharmaceutical and ensuring patient access to necessary treatments.

Quality in healthcare systems

Key aspects of healthcare policy in this context include policies like the U.S. orphan drug act and similar legislation in other countries provide incentives such as tax credits, grant funding and market exclusivity to encourage the development of treatments for rare disorders. Expedited regulatory pathways, such as the FDA's fast track, breakthrough therapy and priority review designations, aim to accelerate the approval of treatments for serious conditions with unmet medical needs. Determining fair pricing for orphan drugs is challenging due to their high costs and the need to ensure sustainability for healthcare systems. Policymakers must consider mechanisms such as value-based pricing, risk-sharing agreements, and tiered pricing models. Patient assistance programs, expanded access schemes, and insurance coverage mandates help improve access

to high-cost therapies for patients with rare disorders. While significant strides have been made in the realm of rare disorders, several challenges persist ensuring equitable access to treatments across different socioeconomic groups and geographic regions remains a critical issue. Patients in low-income countries often face significant barriers to accessing orphan drugs. The high cost of orphan drugs poses a sustainability challenge for public and private payers. Healthcare systems must find ways to integrate these treatments without compromising the overall quality and availability of care. Funding and supporting research for ultra-rare disorders is particularly challenging due to limited commercial incentives. Strong patient advocacy groups plays a vital role in raising awareness, influencing policy and driving research funding.

Patient advocacy

Their involvement is essential in healthcare policies that address the needs of rare disorder patients. The landscape of health economics and healthcare policy in rare disorders is evolving, driven by advances in medical science, technology and patient advocacy. Advances in genomics and personalized medicine hold promise for more targeted and effective treatments for rare disorders, potentially reducing costs and improving outcomes. Exploring alternative funding models, such as public-private partnerships, crowdfunding, and social impact bonds, to support research and patient access. Enhancing international collaboration to share data, resources, and best practices can improve the efficiency and effectiveness of rare disorder research and treatment. Greater harmonization of regulatory and reimbursement policies across countries can facilitate faster access to treatments and reduce disparities. Addressing the health economics and healthcare policy challenges of rare disorders requires a multifaceted approach that balances innovation, access, and sustainability. Policymakers, healthcare providers, researchers, and patient advocacy groups must collaborate to create a supportive environment that fosters the development of life-saving treatments while ensuring that all patients can benefit from medical advancements. As our understanding of rare disorders deepens and technology advances, there is hope for a future where these diseases are better managed, and patients can lead healthier, more fulfilling lives.