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Management of rare diseases: An integrated approach to break down barriers and facilitate patient access to healthcare

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Background: In Europe, a rare disease is a disease that affects less than 1 in 2000. Although this seems to affect a fraction of the population, collectively it imposes a colossal. Rare diseases are disabling, dramatically reducing the quality of life, hampering routine activities and autonomous survival. Rare diseases management is intricate and varies across geographies. Understanding the complexities is important to resolve issues and improve care. **Methodology:** Secondary research of published and grey literature. **Findings:** Some of the challenges encountered in rare diseases management are delayed or misdiagnosis, lack of awareness on diseases/management, lack of quality healthcare at proximity, struggles to continued care, treatment access, and social consequences. In recent times, stakeholders such as healthcare providers, patient organizations, pharmaceutical companies, regulatory bodies and policy makers have taken many initiatives to resolve these challenges. National policies/plans are devised exclusively to manage rare diseases. Compassionate use programmes have encouraged research and provided accelerated entry of orphan drugs into the market. Organizations like EURORDIS have given voice to more than 300 rare disease organizations in over 30 countries. While these stakeholders, individually have endeavored to improve healthcare and social care, the complex challenges demands novel and integrative approach to effectively manage the rare diseases scenario (Figure). Additionally, leveraging on technology to create unified platforms and eHealth/mHealth programs can aid quick and better reach. **Recommendation:** Literature review has established some challenges and approaches to overcome them. However, in order to develop and implement an integrative approach in practice, the current drivers and barriers to effective management must be clearly explored. This demands a sequential explanatory research (quantitative study followed by qualitative) to explain quantitative results on current practices by exploring certain outcomes (especially unexpected ones) in depth. Eventually, an integrated approach can be developed to successfully address concerns and strengthen the management.

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