

A Systematic Review on Orphan Drugs Pricing, and Prices Challenges

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Abstract : Background: Orphan drug development is limited by very high costs attributed to the research and development and small size market. How health policymakers address this challenge to consider both supply and demand sides need to be explored for directing the policies and plans in the right way. The price is an important signal for pharmaceutical companies' profitability and the patients' accessibility as well. Objective: This study aims to find out the orphan drugs' price-setting patterns and approaches in health systems through a systematic review of the available evidence. Methods: The Preferred Reporting Items for Systematic Reviews and Meta-Analysis (PRISMA) approach was used. MedLine, Embase, and Web of Sciences were searched via appropriate search strategies. Through Medical Subject Headings (MeSH), the appropriate terms for pricing were 'cost and cost analysis', and it was 'orphan drug production', and 'orphan drug', for orphan drugs. The critical appraisal was performed by the Joanna-Briggs tool. A Cochrane data extraction form was used to obtain the data about the studies' characteristics, results, and conclusions. Results: Totally, 1,197 records were found. It included 640 hits from Embase, 327 from Web of Sciences, and 230 MedLine. After removing the duplicates, 1,056 studies remained. Of them, 924 studies were removed in the primary screening phase. Of them, 26 studies were included for data extraction. The majority of the studies (>75%) are from developed countries, among them, approximately 80% of the studies are from European countries. Approximately 85% of evidence has been produced in the recent decade. Conclusions: There is a huge variation of price-setting among countries, and this is related to the specific pharmacological market structure and the thresholds that governments want to intervene in the process of pricing. On the other hand, there is some evidence on the availability of spaces to reduce the very high costs of orphan drugs development through an early agreement between pharmacological firms and governments. Further studies need to focus on how the governments could incentivize the companies to agree on providing the drugs at lower prices.

Keywords : orphan drugs, orphan drug production, pricing, costs, cost analysis

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