observed that the orphan designation system in Japan has achieved certain results for increasing the accessibility of necessary drugs to patients suffering with rare diseases. However, several drugs are still not available in Japan, partly because of the difference in definitions of orphan disease among the 3 regions. To increase the accessibility to orphan drugs, further policy interventions should be considered.

**CONCLUSIONS:** Policy makers should consider a comprehensive approach, including a specific funding mechanism, incentives for drug development, and efficient regulatory processes to overcome the challenges associated with orphan drug accessibility in Japan.

**OBJECTIVES:** To assess the feasibility of orphan designation in Japan and propose policy recommendations to improve access to orphan drugs.

**METHODS:** A systematic review of existing literature, including guidelines, policies, and case studies, was conducted to identify best practices and success stories. A qualitative analysis was performed to gather expert opinions from stakeholders in the pharmaceutical industry and health authorities.

**RESULTS:** The analysis revealed that Japan has made significant progress in recognizing and approving orphan drugs, but challenges remain, such as limited funding, long approval timelines, and inadequate incentives for industry participation. The review identified several successful initiatives in countries like the United States and Europe that could serve as models for Japan.

**CONCLUSIONS:** To enhance orphan drug accessibility in Japan, policy interventions should focus on increasing funding, streamlining regulatory processes, and promoting partnerships between industry, academia, and healthcare providers. This comprehensive approach is likely to improve patient outcomes and contribute to the global effort in rare disease research.

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**EARLY ACCESS: ANALYSIS OF THE FRENCH ATU SYSTEM**

**OBJECTIVES:** To evaluate the early access policy in France and assess its effectiveness in accelerating the approval process for orphan drugs.

**METHODS:** A retrospective analysis of orphan drug approvals in France from 2000 to 2015 was conducted. The data included information on the number of orphan drugs approved, the time to approval, and the clinical trial requirements.

**RESULTS:** The analysis showed a marked increase in the number of orphan drugs approved in France, with a decreasing time to approval over the years. However, the high number of clinical trials required for approval remains a significant challenge.

**CONCLUSIONS:** The French ATU system has been successful in accelerating orphan drug approvals, but there is a need for further simplification of clinical trial requirements to enhance patient access.

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**ECONOMIC EVALUATION OF TELEHEALTH/TELEMEDICINE AND COST-EFFECTIVENESS ARGUMENT AMONG KEY DECISION-MAKERS**

**OBJECTIVES:** To explore the economic evaluation of telehealth/telemedicine among key decision-makers in healthcare.

**METHODS:** A qualitative study was conducted with healthcare professionals and policymakers to understand their perspectives on the economic impact of telehealth/telemedicine.

**RESULTS:** The study revealed that cost-effectiveness is a crucial consideration, but there is a lack of robust economic evaluation studies. Decision-makers highlighted the need for more comprehensive economic analyses to support evidence-based decision-making.

**CONCLUSIONS:** To improve policy decision-making, there is a need for increased investment in research to provide evidence on the cost-effectiveness of telehealth/telemedicine programmes.