Developing Optimal Orphan Drug Legislation in Canada

Event

Orphan drug legislation creates incentives to encourage the development of treatments for rare diseases. The United States (US), Japan, and the European Union (EU) have all enacted orphan drug legislation over the last 30 years. In 2013 the Canadian government announced the imminent release of orphan drug policy following publication of a preliminary draft framework in December 2012 ('Canadian Framework')[1]. The new framework, however, is yet to be approved. For Canada, this is a critical time to review the challenges and successes experienced by other jurisdictions regarding their orphan drug legislation, such that these lessons can be incorporated into optimized local policy.

Significance

Rare or 'orphan diseases' affect very small percentages of the population making them less attractive targets for pharmaceutical R&D. Orphan drug legislation addresses this need by delivering millions of dollars in economic incentives to the industry every year to reduce development costs for orphan disease treatments. Despite this substantial public investment, there are concerns that current legislation is open to abuse and fails to address some barriers. Identifying and addressing these issues during the development of Canada's orphan drug policy framework will ensure Canadians benefit from the new legislation. PACEOMICS Guidance Document on Canada's Orphan Drug Legislation presents a comparative analysis of existing orphan drug policy, distilling key issues for the consideration of policy-makers[2].

Analysis

US, Japanese and EU legislation, and the Canadian Framework all employ population prevalence of rare diseases as a baseline criteria to identify targets for economic incentives. However, these definitions are challenged by evolving molecular understandings of disease, and have been strategically used by pharmaceutical companies to acquire many similar indications for a single drug. Further, personalized medicine legitimizes finer patient group stratification based on genetic differences in drug response, thus contributing to ever greater numbers of rare disease classifications[3]. Some orphan drugs end up making substantial profits. However, unlike some jurisdictions, the Canadian Framework does not allow for review of drug income, which may invoke a repayment of subsidies if profits exceed specified thresholds. Clinical trials for orphan disease present a number of challenges, including low patient numbers, relatively short trial durations, and a tendency to over-represent benefits. European and US orphan drug regulators have developed strategies from which Canada can learn. Orphan drugs are often extremely costly. In Canada, patient access will be determined by reimbursement decisions at the provincial level, and these may differ across the country. However, the current Canadian Framework does not address this issue of unequal access. Likewise, intellectual property mechanisms are common to all jurisdictions, nevertheless their potential to adversely affect patient access should also be considered by policy-makers. Finally, biosimilars are becoming part of the therapeutic landscape for orphan disease, but their approval displays regulatory heterogeneity across jurisdictions. In particular, Canada's stance on biosimilars differs from other jurisdictions and may act to limit availability in this country.

Conclusion

Canada is now presented with a unique opportunity to leverage the experience of other jurisdictions as it develops its orphan drug policy. The current draft of the Canadian Framework does not address several issues critical to equity, justice and the economic sustainability of an orphan drug strategy in Canada. Explicit consideration and engagement of these issues should contribute to the success of this program – ensuring access to treatments that will improve the lives of Canadians affected by orphan diseases.

1. Office of Legislative and Regulatory Modernization. (2012). Initial Draft Discussion Document for A Canadian Orphan Drug Regulatory Framework. http://www.orpha.net/national/data/CA-EN/www/uploads/Initial-Draft-Discussion-Document-for-A-Canadian-Orphan-Drug--Regulatory-Framework.doc
2. Luth W, Ali-Khan S, Bubela T (2015). PACEOMICS Guidance Document: Canada's Orphan Drug Framework: Lessons from the US, Europe and Japan. http://paceomics.org/wp-content/uploads/2015/10/Canadas-Orphan-Drug-Framework.pdf
3. Paulden M, Stafinski T, McCabe C (2015). Value-based reimbursement decisions for orphan drugs: a scoping review and decision framework. Pharmacoeconomics. 33(3):255-69.