Benchmarking study on public policies for rare diseases

Montreal InVivo is working on a new initiative aiming to benchmark the performance of Quebec and Canada with regard to economic and health policies for rare diseases, in comparison with other Canadian provinces and international jurisdictions.

For this reason, Montreal InVivo mandated "Synergyx Consulting" to carry out a comparative study of policy policies for rare diseases in order to :

- Understand best practices and strategies implemented in the countries and regions that support health and economic development policies for rare diseases, which enhance their competitiveness.
- Make recommendations to the government of Quebec and Canada for health policies and economic measures that may be implemented to attract more private investments and improve competitiveness.
- Advocate for the development of a comprehensive rare disease strategy in Quebec

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Context

Canada lags far behind when it comes to rare diseases, both in terms of legislation, health and economic policies aimed at encouraging research and access to treatment for those affected.

Most industrialized countries have laws, plans or strategies for rare diseases: The United States adopted the Orphan Drug Act in 1983, other countries have followed or are inspired by the American model, in particular Japan (1993), Australia (1998) and the European Union (2000). France is already in its third National Rare Disease Plan (2018-2022). Germany is currently working on its second National Action Plan for People with Rare Diseases. In fact, 26 of the 28 member countries of the European Union have their own national rare disease plan.

The Canadian government initially rejected the idea of an orphan drug policy in 1997. Then in 2012, Health Canada announced its intention to develop a policy framework. Almost ten years later, Canadians with rare diseases continue to wait for a comprehensive plan to be implemented. In January 2021, Health Canada announced a consultation with rare disease patient associations as part of the Online Mobilization on a National Strategy for Expensive Drugs for the Treatment of Rare Diseases.

In Quebec, the provincial government set up the Quebec Working Group on Rare Diseases in July 2018. The Working Group presented the results of its work in a report published in July 2020 with general recommendations at the strategic level concerning the organization of care and services for the support of people affected by rare diseases.

Although the actions initiated by the Canadian and Quebec governments represent the first steps in the right direction, the fact remains that there are still many measures to be taken before arriving at a national plan or a strategy addressing the complex needs required by people with rare diseases.

Health policies and economic policies

Business incentives have been the cornerstone of policies to stimulate research and development of so-called orphan drugs for the treatment of rare diseases. These incentives have had the desired effect of stimulating research into treatments for rare diseases.

Laws, regulations and policies in Europe and the United States have been developed with the aim of encouraging the research and development of drugs for the treatment of rare diseases. Essentially, five broad categories of incentives were created: tax credits, priority drug reviews by regulatory agencies, research grants, scientific support in clinical research protocol development, and market exclusivity.
These financial and structural benefits in support of research and development of treatments for rare diseases have had a significant impact on the expansion of the pharmaceutical industry and the commercialization of drugs for rare diseases.

Access to treatments

The current Canadian policy on drug development and market access is inappropriate for orphan drugs. As for the Quebec drug reimbursement policy, it does not, in its current state, address the issue of accessibility to drugs for rare diseases.

Although there is no national drug coverage program, considered expensive for rare diseases, most Canadians have some protection against the cost of these drugs through provincial, territorial, federal, or private insurance plans.

In short, Canada does not currently have a national drug insurance plan for rare diseases like France and Germany, but rather a motley mix of public and private drug plans. As a result, this results in inequities in drug coverage for rare diseases across provinces.

Need to develop a national and integrated plan

Canada should be inspired by the development model of the national rare disease plans of France and Germany. The central framework of the national plan must be developed with the priority objectives of improving the diagnosis and screening of rare diseases, offering better coordination of care and support adapted to patients. In addition, the plan should put in place measures for more efficient and equitable access to treatment, as well as to foster the emergence of innovation and research for rare diseases.

It would be essential for Canada to put in place a legislative and regulatory framework, specifically developed for rare diseases. Through this process, Canada should also consult with patients and families with rare diseases, specialist clinicians, researchers in the field and representatives of the pharmaceutical industry. Canadians need a framework for orphan drugs to ensure timely access to safe and effective treatments.

Moreover, patient groups for rare diseases, such as the Canadian Organization for Rare Disorders (CORD) and the Regroupement Québécois des maladies orphelines (RQMO), had each developed strategies on rare diseases, policies promoting innovative research, action plans and numerous proposals, mainly aimed at reducing screening and diagnosis times, improving patient care and optimizing access to treatment. The federal and provincial governments could adopt and implement the strategies developed by CORD and RQMO, since these strategic frameworks have been developed and validated by scientific and clinical experts in rare diseases.

Possible solutions

There are many sources of inspiration in the development and reimbursement of drugs for rare diseases. Most of the 30-member countries of the Organization for Economic Co-operation and Development (OECD) have policies, programs or laws on orphan drugs, in the form of designations, market exclusives, subsidies for the research, access to drugs before market authorization, tax credits, research grants and exemptions from assessment fees by regulatory agencies.

First, while respecting the sharing of health responsibilities between the provinces, the Government of Canada should create a federal bill including a national strategy for rare diseases. A detailed action plan with precise work schedules as well as amounts of money allocated to support actions and the implementation of the plan across the provinces. The efficiency of this approach has been demonstrated in France and Germany. In fact, in 2017 Ontario developed its strategic framework aligned with that proposed by CORD, including an action plan, a work schedule and subsidies from public funds.

Second, the federal government should amend the Food and Drug Regulations to add additional intellectual property protection for drugs for rare diseases. Since the time required to research and develop these drugs can be excessively long, granting additional patent protection time could be an important strategic incentive for drug companies. This measure would allow Canada to be more competitive in the international market and to be more attractive to foreign investment.

Another incentive for the federal government to consider is the possibility of granting commercial exclusivity to pharmaceutical companies that develop drugs for rare diseases. Studies in the United States and Europe show that this measure has been one of the best incentives for research and innovation in rare diseases, as it provides certainty for pharmaceutical companies to derive tangible business benefit. The period of commercial exclusivity is seven years in the United States, five years in Australia, ten years in Japan and the European Union (with an additional two years for a pediatric indication, for a total of twelve years of exclusivity in Europe).

Another interesting intervention would be to provide tax credits, grants or funding to encourage research and development of therapies for rare diseases. The experience in the United States and Europe shows that these financial incentives allow pharmaceutical companies to recover a certain return on their investment, otherwise not possible with the mere marketing and sale of drugs. In the United States, a 50% tax credit is applicable to costs associated with clinical research on orphan drugs. In Canada, the Scientific Research and Experimental Development (SR&ED) Program exists to obtain tax credits. However, this program is not specifically designed for research in rare diseases.

Then, there is a need for a concrete accessibility bills, such as drug reimbursement policies, would need to be adopted at the provincial level. A pan-Canadian initiative to harmonize provincial reimbursement policies would make access to treatment faster, more efficient and more equitable.

An often-overlooked initiative is public awareness and education of healthcare professionals about rare diseases. A very small portion of the Canadian population understands precisely what a rare disease is. It is therefore important to quickly remedy this issue to obtain a network of specialist clinical experts across Canada. Moreover, one of the recommendations of the Quebec Working Group in its July 2020 report is to develop a training plan for front-line professionals for the recognition of rare diseases and the rapid referral of patients to experts to make a diagnosis.

Finally, CORD and RQMO's proposals regarding the creation of a registry for collecting data on rare diseases would be relevant in the Canadian and Quebec context. This would make it possible to count the number of people with rare diseases, to monitor these people throughout their illness, to have access to a patient bank in order to facilitate recruitment in clinical trials, to accumulate data on the actual clinical situation, rare disease treatment costs and drug insurance coverage. This real-world data will aid in the development of evidence-based legislation.

Conclusion

In conclusion, what Canada needs is more than a strategy, but a national plan for rare diseases, articulated around major axes. The success of this approach has been well demonstrated in Europe, particularly in Germany, France, and the United Kingdom. A national plan must encompass several strategic axes, particularly support for research and development through innovation, sharing of data on rare diseases at the national international level, as well as appropriate support for patients in disease management.

The national plan for rare diseases must therefore be drawn up with the main goal of promoting the emergence of therapeutic innovations in Canada, reducing the delays in obtaining a diagnosis, rapid access to care and treatment, and better care management for people with rare diseases.