

EXECUTIVE SUMMARY

AT A GLANCE

What is a rare disease?

The World Health Organization classifies as rare any disease/condition affecting 1 in 2,000 people or less.

In Quebec, there are approximately 690,000 people living with a rare disease on a daily basis.

Many rare diseases are chronic, progressive and fatal. Nearly 75% of these diseases affect children.

CURRENT SITUATION IN QUEBEC

Until May 2022, Quebec did not yet have a specific policy or action plan for rare diseases.

Over the past several years, families, patient advocacy groups and the life sciences community have acted to educate the public and the government to take action.

BACKGROUND

In 2019, Montréal InVivo established a specialized rare disease project committee to propose a strategic plan for rare diseases.

Unveiled in April 2022, the objective of this plan is to inform and inspire the Quebec government to act quickly and seize the opportunity to be a pioneer in Canada in the field of rare diseases.

Inspired by international best practices, this document complements the Quebec policy on rare diseases and is in line with the Quebec Life Sciences Strategy (SQSV) and the Quebec Research and Innovation Strategy (SQRI).

Composition of the Committee

Researchers

Clinicians

Patient groups

Life sciences industry

GUIDING PRINCIPLES CENTRAL TO THE REFLECTION

Patient-centered approach and economic development in Quebec.

PLAN OBJECTIVES

- Allow Quebec patients suffering from a rare disease to have rapid and optimal access to therapeutic and diagnostic innovations.
- Improve the competitiveness of Quebec's innovation ecosystem to attract investment and develop our skills by drawing on international best practices.



ISSUES

01

Sub-optimal and complex patient care

02

The level of funding limits the competitiveness of research and development activities

03

Significantly less competitive business environment

04

Concerns about the ability to pay for new value-added innovations



RECOMMENDATIONS



Speed up diagnosis
in order to limit diagnostic delays



Improve care
for patient health



Aggregate health data
to optimize care pathways and research opportunities



Support the development of innovations
in Quebec



Leverage clinical trials
to improve patient care and create economic wealth



Improve access to approved treatments
so that Quebec patients can benefit quickly from innovations



Create a public-private fund
dedicated to rare diseases

NEXT STEPS

The creation of a **consultation table** of stakeholders will be essential to develop a global vision, mobilize the ecosystem, strengthen collaboration between all stakeholders and ensure quick and optimal deployment of the action plan stemming from the Quebec policy on rare diseases and other initiatives of the rare diseases community.

The Quebec government, following the unveiling of its National Policy on Rare Diseases, will also have to develop and implement an **action plan** to ensure the implementation of concrete measures.

The Government of Canada has committed to move forward with a national strategy on drugs for rare diseases and plans to invest up to \$1 billion to provide Canadians with access to treatments for rare diseases, starting in 2022-2023. Quebec must therefore also **seize this opportunity** without delay.