WANTED:
Canadian Solutions for Rare Diseases

RARE i
THE CANADIAN FORUM FOR RARE DISEASE INNOVATORS

Quick Facts

1 in 12 Canadians
have a rare disease[1]

More than 10,000
known rare diseases[2]

65% of rare disease treatments
make it to Canada[3]

2 years
is the average time for public reimbursement of rare disease drugs in Canada – over 2x longer than other developed countries[4]

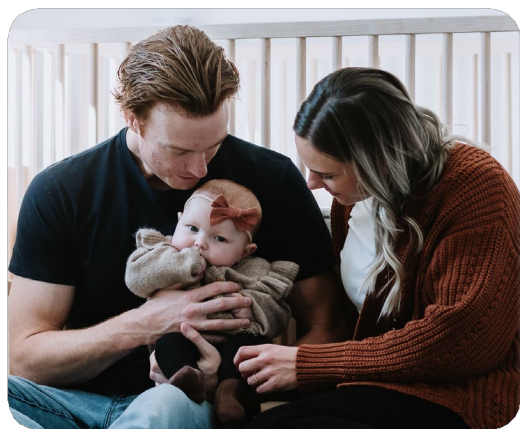
90% of rare disorders
have no treatment options[5]

2 in 3 people affected
by rare disorders are children[6]

Ryan's story – a patient's journey

Ryan was diagnosed with Gaucher disease when she was just three weeks old. Gaucher is a rare disease caused by an enzyme deficiency that results in the accumulation of harmful quantities of certain fats. Now one year old, Ryan experiences hearing and vision loss, seizures, and difficulty swallowing. Her parents, Mallory and Cameron, have stepped back from their chiropractic practice to get Ryan the care she needs. Her first year of life has been consumed by endless specialist appointments and noticeable losses of function and, while there are treatments approved for some Gaucher patients, the restrictions on access to them have caused Ryan's parents to worry that she could be taken off therapy at any moment. Ryan is now enrolled in a gene therapy clinical trial in the United States.

For more patient stories visit: www.canada4rare.ca



What are the challenges?

- Rare disease treatments typically require higher per-patient costs compared to medicines for common conditions in the context of high-risk research and development programs and small patient populations
- Canada's complex drug review system can deter or delay launches of new treatments in Canada. Patients in other developed jurisdictions, including the US and Europe get access earlier to new therapies
- Patients face many hurdles, including disparities in coverage between provinces and high out-of-pocket costs

What are the opportunities?

- Canada is one of the only developed countries without a national plan for rare diseases, but efforts are underway to implement one
- Improving access to treatments and clinical trials can save and significantly improve lives of patients
- Improving access to treatments can free up health system resources and ensure Canadians are healthier and contributing to the economy

What is happening

The Government of Canada promised $1 billion over two years starting in 2022–23 to launch Canada's Rare Disease Drug Strategy to improve access to these medicines. It is unclear how this funding will be used. Key provincial leadership on the file includes the launch of Quebec's first rare diseases strategy in June 2022.

Governments invested $525 million on non-cancer rare disease treatments in 2021, representing just 3.2% of total public drug spending.[7] Increased investment to expand access to rare disease medicines is manageable. Many access gaps and delays could be addressed through federal and provincial rare disease strategies.

Potential solutions

- **Invest in infrastructure:** Endorse, support and fund proposals for networks of rare disease centres of expertise in Canada
- **Separate track for rare:** Create programs for rare disease patients and their doctors to apply for funded access to medicines
- **Remove access hurdles:** Create customized health technology assessment review processes which acknowledge the distinctive nature of rare disease treatment development; avoid the requirement for such reviews for medicines with low budget impact

1 https://www.ourcommons.ca/Content/Committee/421/HESA/Reports/RP10349306/hesarp22/hesarp22-e.pdf
2 https://rare-x.org/case-studies/the-power-of-being-counted
3 An international comparative analysis of public reimbursement of orphan drugs in Canadian provinces compared to European countries: https://ojrd.biomedcentral.com/track/pdf/10.1186/s13023-022-02260-6.pdf
4 IMC's Explaining Public Reimbursement Delays for New Medicines for Canadian Patients (Jun. 30, 2020) http://innovativemedicines.ca/wp-content/uploads/2020/07/CADTH-TTL-8.5x11-EN-Final.pdf
5 https://rarediseases.org/new-report-finds-medical-treatments-for-rare-diseases-account-for-only-11-of-us-drug-spending-nearly-80-of-orphan-products-treat-rare-diseases-exclusively/
6 https://www.raredisorders.ca/content/uploads/CORD_Canada_RD_Strategy_22May15.pdf
7 Eversana Poster, Canadian Association for Population Therapeutics 2021 Annual Conference https://www.capt-actp.ca/wp-content/uploads/2021/10/35-Lech-CAPT_2021_Poster_27Sept2021_final.pdf