Canadian Organization for Rare Disorders

Drawing the Blueprint for Canada's Rare Drug Program 2022 Winter 2021

Webinar 9

Everything You Need to Respond to Discussion Guide on National Strategy for High-Cost Drugs for Rare Diseases

February 18, 2021

Countdown to Canada's Rare Drug Strategy 2022: Webinar Series

WEBINAR 7

Jan 29

@ 11AM (EST)

How Other Countries Provide Access to Rare Disease Drugs: What Canada can Learn … or Not

WEBINAR 8

Feb 12

@ 12PM (EST)

Laying a Factual Foundation for Pan-Canadian Rare Drug Program

WEBINAR 9

Feb 18

@ 6 PM (EST)

Everything You Need to Respond to Discussion Guide on National Strategy for High-Cost Drugs for Rare Diseases

WEBINAR 10

Feb 26

@ 12PM (EST)

Rare Disease Day: Celebrating Achievements that Support A Pan-Canadian Rare Drug Program

CORD Canadian Organization for Rare Disorders

Rare Disease Day Conference

From Draft to Action Plan

March 9 - 10, 2021

RARE DISEASE DAY®

Consultation Dates: January – September 2021

- Jan – Feb 2021: CORD ”Drawing the Blueprint” Webinar Series
- Jan – Mar 2021: Health Canada Public Consultations
- March 9 – 10, 2021: Rare Disease Day Conference ”From Draft to Action Plan”
- April 2021: Consolidated Design
- Jun – Jul 2021 Public Consultations
- Aug 2021: Collaborative Document

Jan 1, 2022: Ready to Launch 

CORD Canadian Organization for Rare Disorders

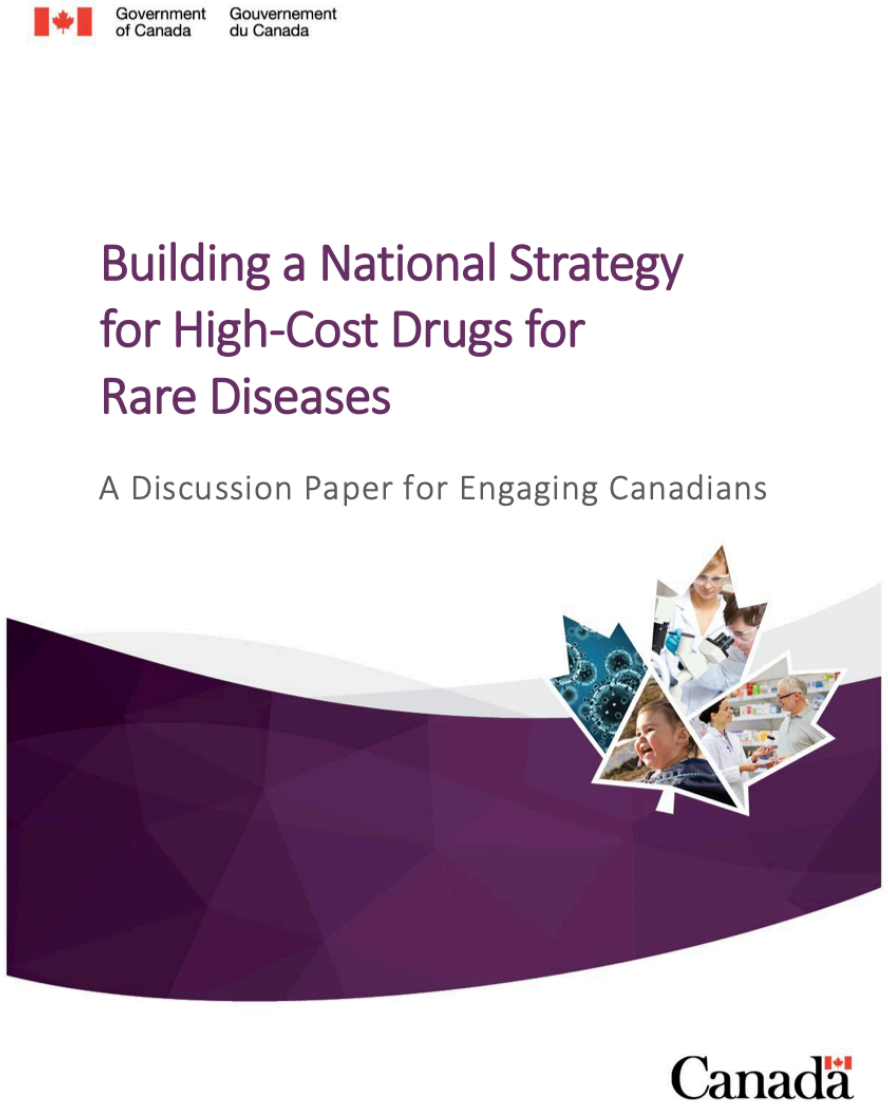
National Strategy for High-Cost Drugs for Rare Diseases Online Engagement

Government of Canada / Gouvernement du Canada

1. Complete the online **questionnaire**
2. Send a written submission via **email** or **mail**
3. Participate in a virtual **public town hall**

Public Town Hall #1	February 9, 2021	3:00 – 4:30pm	Bilingual
Public Town Hall #2	February 25, 2021*	7:00 – 8:30pm	Bilingual
Public Town Hall #3	March 3, 2021*	1:00 – 2:30pm	French
Public Town Hall #4	March 12, 2021	10:00 – 11:30am	Bilingual
Public Town Hall #5	March 23, 2021	3:00 – 4:30pm	Bilingual

To find out more about this consultation on the National Strategy for High-Cost Drugs for Rare Diseases, please click on the following link: https://canada.ca/en/health-canada/programs/consultation-national-strategy-high-cost-drugs-rare-diseases-online-engagement.html



Webinar 9: Everything You Need to Respond to Discussion Guide on National Strategy for High-Cost Drugs for Rare Diseases

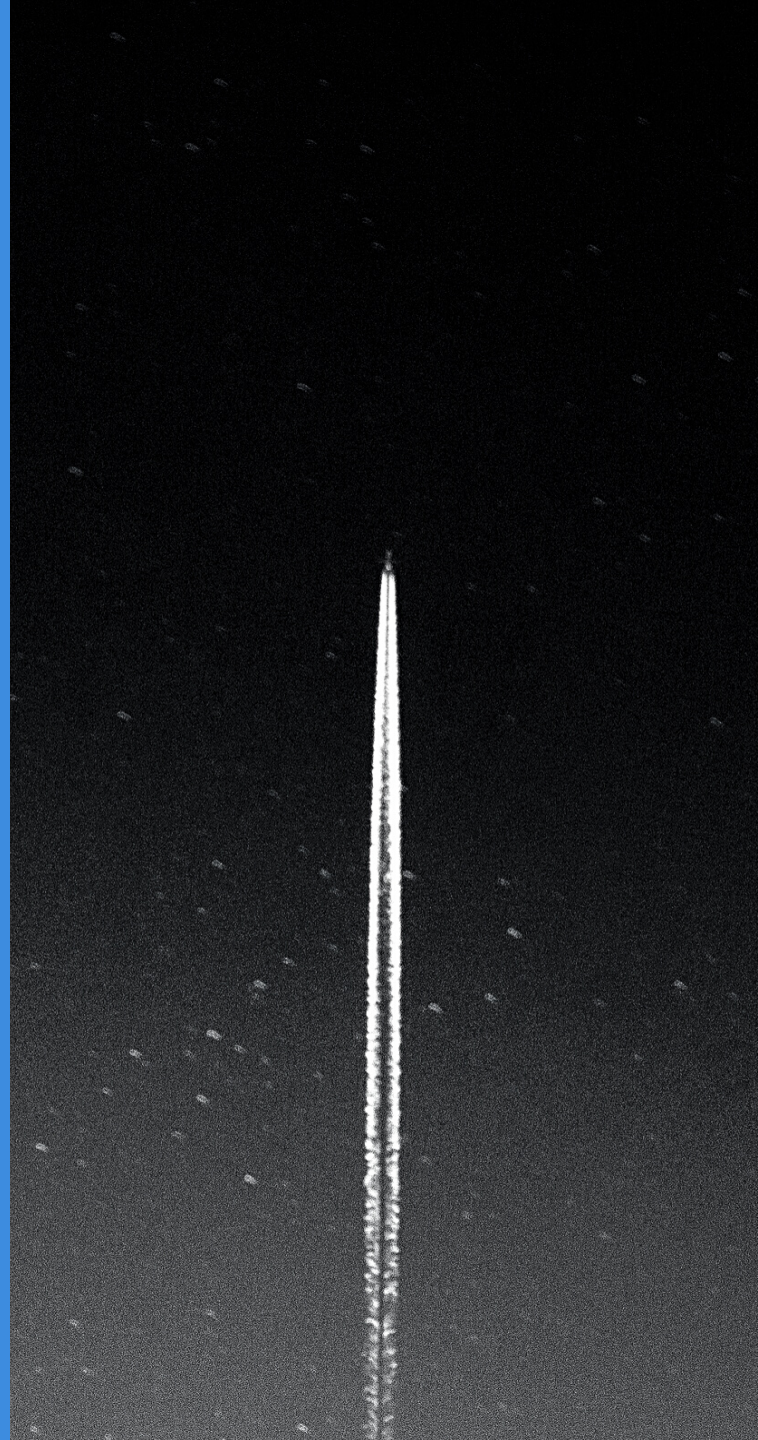
Webinar 9: Discussion Guide

Agenda

Putting Patient Needs at the CORE of Rare Drug Strategy

- Our Groundings: Rare Disease/Drug Ecosystem
- Our North Star: Leave No One Behind
- Our Basics: What Patients Can’t Live Without
- Our Responses: What (More) Needs to be Said

Moderators: Durhane Wong-Rieger, CORD
Sandra Anderson, Innomar
Maureen Smith



Key Stats on Rare Diseases

1 in 12

Canadians has a rare disease

That's MORE than 2.8 MILLION!

80% Genetic BUT 50% No Family History

APPROXIMATELY **7,000** rare diseases are known to exist today[1]

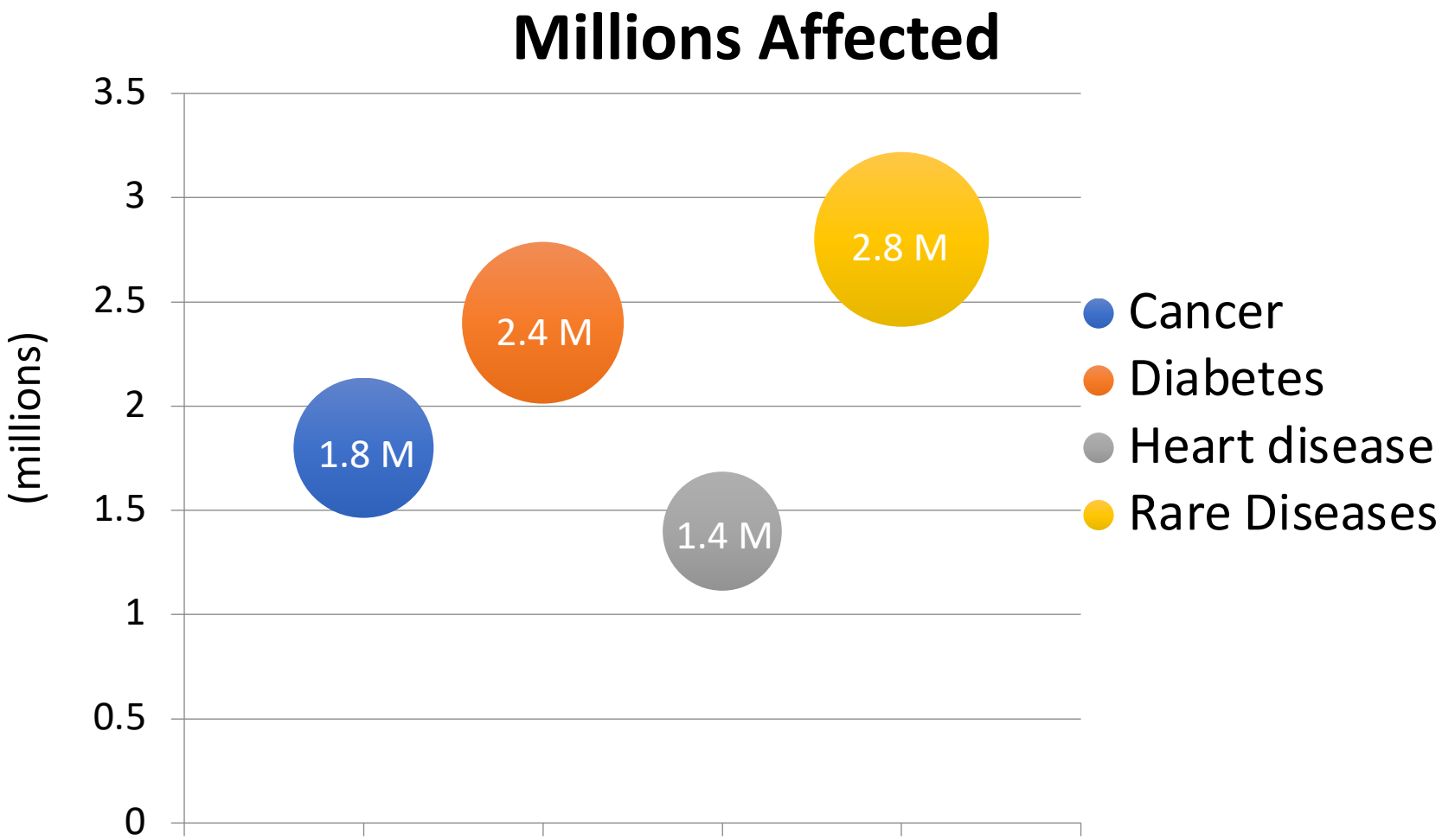
30%

of children with a rare disease will not reach their 5th birthday

2/3

of Canadians with Rare Diseases are Children

ONLY **5%** of rare diseases have an approved treatment[1]



Canadians with Common vs. Rare Diseases

Now is the Time for a Rare Disease Strategy

- Rare disease is a Major Public Health Issue
- RD Patients rarely access effective therapies
- Health systems waste resources, achieve limited benefits
- Rare disease strategies work in other countries  
- Canadian strategies work in other areas: Mental health, cancer, diabetes, cardiovascular disease
- Leverage & coordinate expertise and resources across disciplines and sectors and internationally

Launch of Canada's Rare Disease Strategy


THE GLOBE AND MAIL

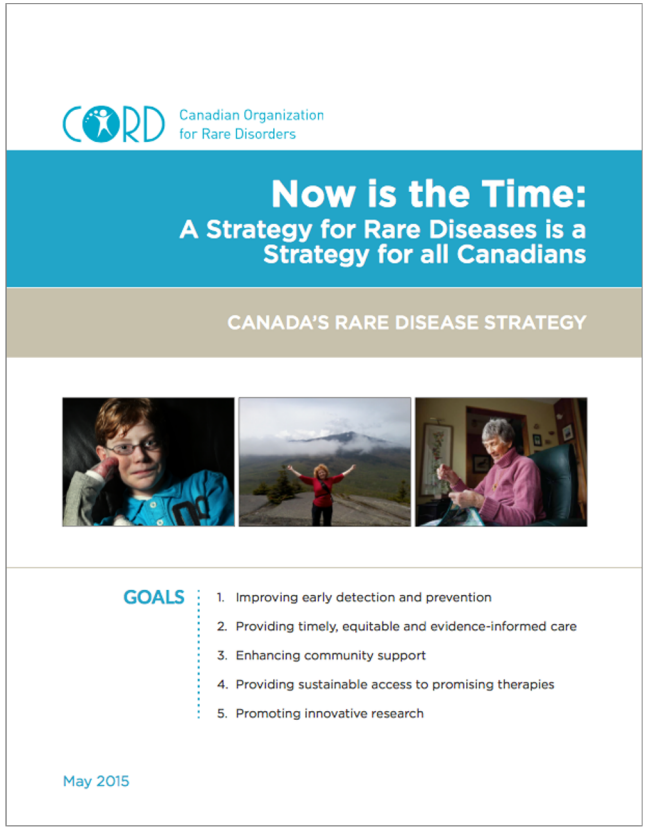
Group releases strategy to help Canadians with rare diseases get care

May 25, 2015

- Extensive consultations with rare disease stakeholders since 2012
- Strategy Launched on Parliament Hill in May 2015
  - Now is the Time
- Shared across Canada:
  - Political parties, policy makers
  - Healthcare providers & admin
  - Researchers, patients, public

5 Key Goals of Canada's Rare Disease Strategy

1. Improving early detection and prevention
2. Providing timely, equitable and evidence-informed care
3. Enhancing community support
4. Providing sustainable access to promising therapies
5. Promoting innovative research

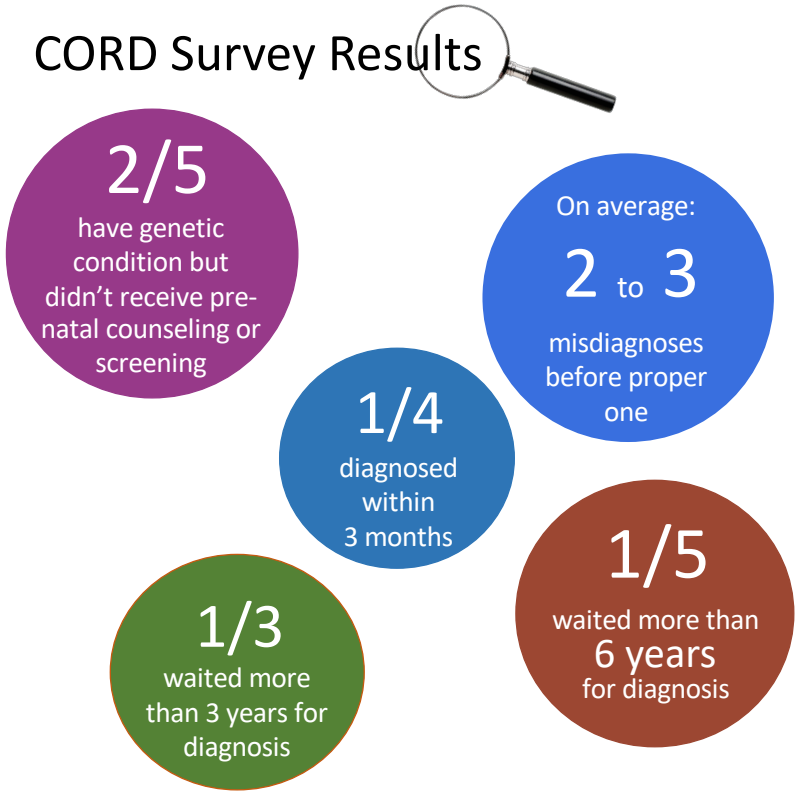


3 Guiding Principles

Fundamental Consensus Principles for Effective, Cost-Effective, and Sustainable RD Programs

I. Equity of Access
II. Patient-Centered
III. Collaboration and Coordination

GOAL 1
Improving Early Detection and Prevention



- Newborn screening in all provinces
- Next-generation diagnostic testing; state-of-the art international labs
- Standards for pre-conception, pre-natal genetic screening and counseling
- Consistent, comprehensive, up-to-date genetic testing guidelines and tests
- Genetic testing linked to RD registries, expert centers, healthcare services

GOAL 1
Improving Early Detection and Prevention

Priority actions:

a) Adopt national state-of-the art newborn screening

b) Implement early detection and preventive services across Canada

IAN'S LONG ROAD TO DIAGNOSIS

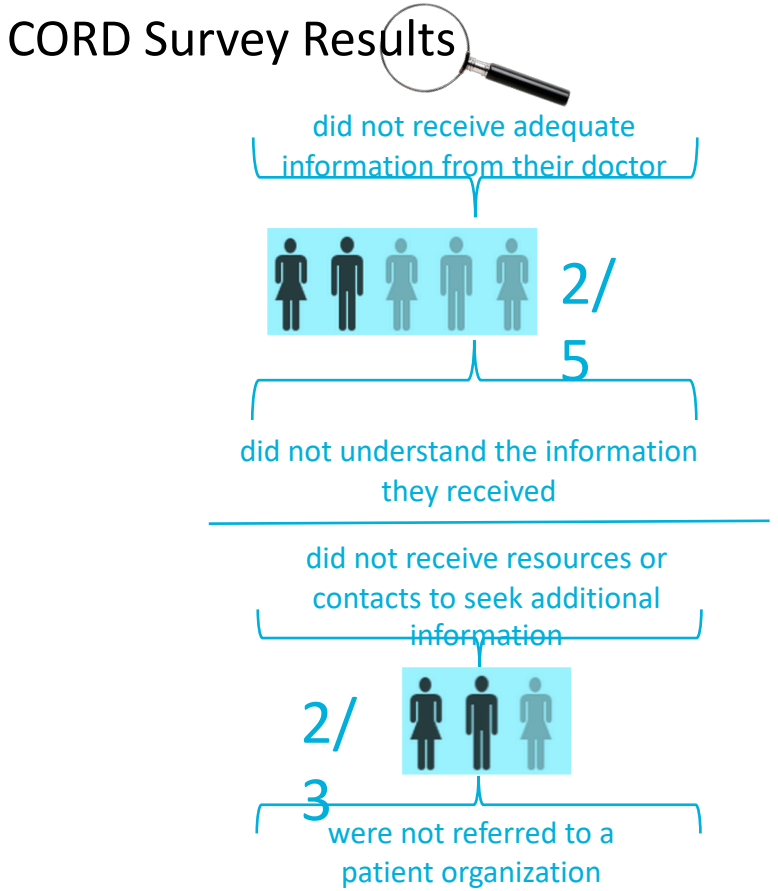
"At 33, I finally got a diagnosis at about the same time as my 2-year-old daughter who was diagnosed with the same genetic condition. But we started her on therapy right away so hopefully she'll never suffer the way I did."

- Ian, 34-year-old man with Muckle-Wells

GOAL 2
Timely, Equitable and Evidence-Informed Care

Priorities

- RD training for GPs, pediatricians, other HCPs
- Clinical practice guidelines
- Disease registries
- Comprehensive care & support
- Centres of Excellence and virtual networks
- Linkage to social care, education, disability and work supports



TRACY'S JOURNEY TO OBTAIN APPROPRIATE CARE

"Surgery and radiation did not successfully remove the tumour causing my rare disorder. Treating the tumour resulted in other rare conditions for which medications are generally not covered. After a long battle, coverage was attained for one medication, but a new drug that could treat the tumour is not yet available in Canada. So the fight by our patient community continues."
- Tracy, 44-year-old mom with Cushing's Disease

GOAL 3
Enhancing Community Support

- **RD patient community key in patient & family support**
- **Priorities:**
  - Adequate funding
  - Accessible information on Canadian resources to HCPs, patients and public
  - Well-resourced / utilized Canadian Orphanet database

- **The RD community:**
- Supports patients & families
- Connects patients to resources and one another
- Communicates RD information to policy-makers, decision-makers, the media & the public
- Ensures patient voices are informed, empowered and heard

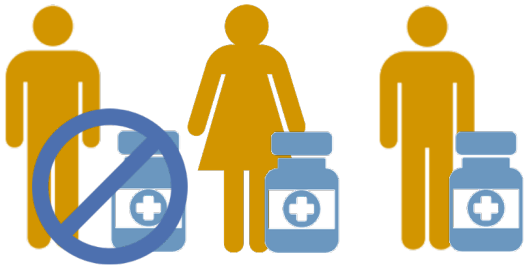
THE IMPORTANCE OF COMMUNITY SUPPORT FOR CASSANDRA

“When you grow up thinking monthly blood transfusions and nightly 10-hour drug infusions are “normal”, almost anything else is possible. A rare disease isn’t limiting if you have treatment but most important friends and family support.“
- Cassandra, 16-year-old girl with Thalassemia major

GOAL 4
Sustainable Access to Promising Therapies

- Challenges for drug access: small patient populations; lack causes, natural history, and long-term benefits of therapy; high individual cost
- Priorities:
  - Canadian Orphan Drug Regulatory Framework
  - HTA process for common disease drugs inappropriately used for orphan drugs
  - Consistent pan-Canadian access
  - Immediate access through risk sharing/managed access programs

CORD Survey Results



1 in 3

couldn't access appropriate drug treatments

MICHAEL'S FIGHT TO ACCESS TREATMENT

"I have an ultra-rare blood disease that leads to kidney failure. The only effective therapy is a drug approved by Health Canada but denied by the provincial public drug plans because of cost. This disease destroyed my own kidneys as well a transplanted kidney donated by my wife. The doctors won't allow another transplant unless I'm on this drug, but my province refuses to approve this drug for transplants. So I must remain on dialysis."
- Michael, 50-year old with aHUS

GOAL 5
Promoting Innovative Research

- Leverage pre-clinical research strengths
- Priorities:
  - Collaborative research programs (SPOR PARTNERS)
  - Patient registries to enable Canadians in clinical trials
  - Patient-reported outcome measures and input on acceptable harm / benefit trade-offs
  - Studies on disease etiology and natural history of disease
  - Small clinical trial designs, adaptive designs
  - Applied research; pilot projects toward best practices

GOAL 5
Promoting Innovative Research

Priority actions:

a) Provide **increased and dedicated funding** for RD research and Centres of Excellence on RDs

b) Establish **new Canadian Partnership for RDs** to coordinate national research agenda and Centres of Excellence

At Risk for Being Left Behind

- Spinal Muscular Atrophy: Removing roadblocks to diagnosis, treatment & “cure”
- Inherited retinal disease: Ultimate miracle: restoring sight; preventing blindness
- Hypophosphatasia/temia: Strong bones let kids play and adults work!
- Cystic fibrosis: Why funding the 5% drug but not the 90% drug?
- Rare blood disorders: Reducing transfusions saves and improves lives
- Lysosomal storage disorders: Will we fund the gene therapy that replaces the replacement therapy?
- Prader Willi Syndrome: 20-year ordeal to cheap drug approved for PWS everywhere except Canada
- High-certainty and low-cost drugs: Let’s give it to almost everyone!
- High-uncertainty and high-cost drugs: Let’s give to almost no one.

Help Stephanie and Tiffany Fight SMA

TEAM FUNDRAISER

April Chan and 2 others are organizing this fundraiser on behalf of Kimsaung Sov.

Health Canada Rationale for National Strategy for High-Cost Drugs for Rare Diseases

- Most RDs rely on medicines for treatment but limited #'s
- High (individual) cost unaffordable without drug plan
- National strategy leads to better access: consolidating efforts leads to timely decisions, better planning, and easing strain on public and private plans
- Like pCPA, common decisions on pre-determined principles
  - Strengthen negotiating power
  - Simplify process to funding
  - Post-marketing monitoring to determine meeting expectations
  - Stimulate innovative R&D to more breakthrough products
  - Improve consistency of access
  - Improve knowledge, collection of RW data, information sharing
  - Support early-stage research and development and manufacture in Canada
  - Need for new approach for providing and paying for high-cost drugs for rare diseases

Misinformation in rationale and underlying premises

- Misinformation #1: Treating rare diseases is too expensive; will break the drug and healthcare budget
  - Total about 2.5%; take out oncology; take out expansions to common diseases
  - Projected maximum: 6%
- Misinformation #2: RD drugs is fastest growing pharmaceutical sector (and will take over pharma budget)
  - Around 6,000 rare diseases with no treatment
  - Most of these are very small number of people affected

Misinformation in rationale and underlying premises

- Misinformation #3: Canada is at par with EU and USA in access to clinical trials and innovative therapies
  - Canada currently lags access to drugs for rare diseases by years with some drugs never getting to Canada
  - Canada currently lags access to clinical trials
  - Disparities with EU and USA will be further exacerbated by PMPRB restrictive pricing policies

Issue 1: Improve patient access to high-cost drugs

- Options for improving consistency
  - Single decision-making framework on HC drugs: principle approach on which drugs to cover and which patients to cover
  - Transparent coordinating body among public and private payers to which drugs, agreed-on conditions, rationale, timelines
  - Patient and clinician engagement: increase awareness
  - Coordinated support for research
- Discussion questions
  - How can access be made consistent
  - Which options improve access and consistency

Issue 2: Decisions informed by best evidence available

- Evidence often limited
  - RCTs not possible
  - Ethics of waiting for enough evidence to be gathered
  - May need to be based on limited evidence and uncertain health benefits and risks
- Tailored regulatory processes
  - Accelerated
  - NOC with conditions
  - Changing regulations to expand terms and conditions for approval with further evidence
  - Pressure to access when no other therapies and evidence of benefits minimal
- Options
  - Innovative approval and coverage with payment tied to long-term studies on effectiveness and safety; agreement on clear, objective indicators
  - National expert panel: study data to make informed recommendations and monitor how drugs are used
  - National data system: capture comprehensive data on rare diseases
  - Independent national and international networks
- Questions
  - How to make decisions when evidence liited
  - Which option or combination best

Issue 3: Spending on HC drugs for RD does not pressure on sustainability of Canadian health care system

- Premise
  - RD drugs fastest growing by annual 32% and account for 10% of pharma sales putting strain on budgets
  - pCPA: negotiate fair prices but prices are high, few competitors, diseases severe, patients want quick access
  - Lack or treatment Investment options so investing in research and development; need reasonable ROI without affecting patient access or unsustainably high spending
- Options
  - Sharing costs and pooling risk: negotiate together
  - Up-front investment to reduce risk' negotiated agreements to limit prices
  - Pay for performance: funding tied to how well drugs work
  - Supports for Canadian innovations from drug discovery, R&D, through manufacturing, trials, approval and sale
  - International collaboration
- Questions
  - Which options

Canadian Organization
for Rare Disorders