

CANADA'S "CHANCE FOR LIFE" STRATEGY for Canadians with Rare Disorders

Proposed by: Canadian Organization for Rare Disorders

Introduction

Canadians with rare disorders need timely access to innovative therapies. Many of these represent the only effective therapy for severe, life-threatening disorders. They are often approved with "early-stage" evidence to allow patients access while additional data are collected. While some therapies are expensive on an individual patient basis, reflecting the high cost of development and small patient populations, the total budget impact may be very low.

Chance for Life Strategy

The Canadian Organization for Rare Disorders proposes a "Chance for Life" Strategy to innovative therapies, which is directed to the following outcomes.

1. Patients who potentially benefit for therapy will get access.
 - Those who demonstrate greater benefit than harm will continue treatment.
 - Those who demonstrate no benefit or greater harm than benefit will discontinue.
2. The process for determining access considers societal values and patient impact, as well as clinical evidence and cost-effectiveness factors. The following factors are considered:
 - a) Values of beneficence (doing good) and non-maleficence (avoiding harm)
 - Individual patient need (not want) to attain or maintain life and quality of life
 - Public good (basic level of health for all)
 - b) Values of Canada Health Act: universal (available to all), comprehensive (core needs), portable (similar everywhere), medically necessary (sustain life), accessible (available when needed), sustainable (funded, affordable to patient)
 - c) Values of patient impact (outcomes experienced by patients), self-determination (informed consent and participation in decision making)
3. Process includes all stakeholders to assure "balance" of needs
 - Patients for timely access to safe, effective, affordable medicines
 - Payers to assure optimal value for healthcare investment (balancing individual and collective need)
 - Developers to receive adequate return on investment to justify current and future investment.

Overview of “Chance for Life” Strategy

The Chance for Life strategy applies to drugs that meet the international definition of “orphan drug”, including drugs for rare disorders that affect fewer than 1 in 2,000 persons.

Drugs must be approved by Health Canada for safety, efficacy, and positive benefits-to-risks profile. Approval within a progressive licensing framework may specify on-going patient monitoring, data collection and re-evaluation of market access.

Emergency access for drugs prior to Canadian market authorization may be permitted for patients with serious or life-threatening conditions who have no other effective therapies according to the process of Health Canada’s Special Access Programme.

A national (Federal/Provincial/Territorial) multi-stakeholder Orphan Drug/Rare Disorders Advisory Committee, including experts, clinicians, patients, and policy makers will consider each approved drug and make recommendations as follows.

1. The Committee will develop a drug access protocol based on drug profile (published data and clinical data in progress), disease profile (severity, alternative treatments), international treatment guidelines (published and internationally accepted standards of care), patient impact (anticipated clinical outcomes, side effects, contra-indications), and patient preferences (discretionary role of patients and families especially in cases of uncertainty). The Committee may also specify expected patient outcomes that can serve as benchmarks for deciding continuance of therapy in individual cases. These would be negotiated with physician and patient family prior to commencement of treatment.
2. The Committee will develop monitoring and evaluation procedures including patient registries (with international linkages), individual patient protocols (as defined above), and collection of data for health technology assessment (real-world effectiveness, safety, costs, cost-savings, and public health impact).
3. The Committee will recommend the optimal funding mechanism, based on the access protocol, and may include: listing as general benefit in the public drug plans; funding out of hospital drug budgets; risk-sharing agreements with manufacturers based on number of eligible patients, expected patient outcomes, and value to healthcare system; review of listing (health technology assessment) at appropriate time intervals to assess effectiveness and value.
4. Funding Mechanisms may include a national fund established for rare disorders and/or funding by the respective healthcare jurisdictions (provincial, territorial, federal, regional and/or institutional).

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