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Accessing Drugs for Rare Diseases in Canada: The Patient Perspective

November 3-4, 2009

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Overview

- State of Orphan Drug Legislation in Canada
- Provincial Programs (ON, AB)
- Example: Fabry Disease
  - Program: Explanation
  - Issues for patients with this program
- General Comments
- CORD's "Chance for Life" Strategy

State of Orphan Drug Legislation in Canada

- Short Answer: There isn't one.
- Unlike the US, the UK, Japan, Australia and the EU, Canada does not have an orphan drug policy
- Canada does not have a definition of "rare disease" or "rare condition"

Policies Related to Orphan Drugs

- Health Canada concluded in **1997** that there was no need for a specific orphan drug policy. (Citing as reasons: there never has been one, there has not been significant pressure from industry or special interest groups to develop a policy).
- The National Pharmaceutical Strategy (**2004**) did include a provision for “expensive drugs for rare disorders” (Status: ???)

Don Bell, Private Members' Motion

- May 17, 2007, The House of Commons passed Private Members Motion (M-426) on Rare Diseases.
- MP Don Bell gained support of all parties and the Motion was passed unanimously (with the exception of the Bloc Quebecois)
- Government was to respond within 1 year.
- The Government was to work towards addressing rare diseases in Canada, establishing an orphan drug policy.
- Unfortunately, election was called, therefore Motion died.

Standing Committee on Health

- Review of the Common Drug Review in 2007
- Recommendation:
  - The Federal Government work with its provincial and territorial CDR counterparts to urge CADTH to establish a specifically designed approach for the review of drugs for rare disorders and for first-in-class drugs
  - The Government Response: “work with provincial and territorial counterparts to urge CADTH to establish a specifically designed approach for the review of drugs for rare disorders and for first in class drugs.
- To date, CADTH has not changed its review of drugs for rare disorders

CDR process not suited to orphan disorders

- CDR applies standard cost-effectiveness review even to orphan indications
  - Typically, drugs for orphan indications are new treatments based on surrogate markers with no long-term studies
  - Typically, drugs for orphan indications will be more costly
  - No specific criteria for treatments that are life-saving or have no other drugs available
  - Cost-effectiveness ($ for Quality-adjusted life year gained) approved if less than $50,000
- Off-label (use not approved by Health Canada) will not be assessed by CDR (no clinical data)

Ontario Orphan Drug Policy

- Have proposed an evaluation model:
  - 1: Based on the Ontario definition, is the disease "rare"? (Ontario definition: 1 in 100,000 – 1 in 150,000)
  - 2: Review natural history of disease
  - 3: Assess the potential effectiveness of the treatment, using the best available evidence
  - 4: Evaluate total budget impact
  - 5: Identify additional follow-up data required.
  - And consider "social values" based on input of Ontario's Citizen Council

Alberta Rare Disease Drug Program

- Effective April 1, 2009
- Alberta definition “a rare disease is a genetic disorder that occurs in fewer than 1:50,000 Canadians (or fewer than 50 Albertans)
- Diseases under consideration:
  - Gaucher’s Disease
  - Fabry Disease
  - MPS - I
  - Hunter Disease
  - Pompe Disease

Alberta Rare Disease Drug Program

- Eligibility: Albertans (5 years of residence) with rare diseases who have government sponsored drug coverage & whose physician has applied for coverage. (these individuals will be considered)
- A panel of specialists will consider each patient case individually.
- The government is funding the program for compassionate & ethical reasons (cost of drug beyond reach for most Albertans).

Private Insurers

- Whether an orphan drug is paid for by private insurance depends upon insurer and type of insurance received.
- Patients ability to pay may also rely on ability to pay co-payments and/or deductibles (20% of $50,000 leaves the patient paying $10,000/ year)
- Private insurers are increasingly following provinces decisions in whether to insure drugs

Example: Fabry Disease

- Very rare genetic condition that affects 200-300 families in Canada
- Lack of enzyme leading to accumulation of waste materials causing severe pain, liver and heart damage and early death
- Enzyme replacement therapy (recombinant technology) approved by Health Canada in January 2004
- Rejected by Common Drug Review as “not cost-effective” ($250,000 per patient per year)
- Therapy is available in more than 40 countries worldwide
- After much advocacy, agreement by F/P/T Health Ministers to fund under research protocol in 2005
- Agreement finally signed in October 2006 “Fabry’s Disease Initiative”

Canadian Fabry Disease Initiative Study

- CFDI being conducted by a consortium of clinical researchers at 5 universities across Canada.
- Object of the study is to gain additional information regarding the use of enzyme replacement therapies to treat patient with Fabry Disease
- CFDI funded for 3 years through a joint partnership of health Canada, participating provinces & the private sector
- CIHR (funding agency) is responsible for administering the federal funding of the CFDI study.

Canadian Fabry Disease Initiative Study

- Established an Independent Scientific Oversight Committee to monitor, evaluate & communicate the results of the research
- Progress reports every year. 2006-2007 report completed, none others (2007/2008 online as of October 2009)
- Criteria for admission to study exist
- Patients are not given a choice of which treatment they will receive. (Randomized)

CFDI – Patients' perspective

- Study ended in September 2009 – what happens now?
- Enrolled patients will continue to get access to treatment (commitment from provinces)
- As a Fabry patient in Canada, you can only get access to therapy through the CFDI. What happens to newly diagnosed patients now?
- Why does Canada not follow international guidelines for access to this therapy?
- Positive: first time in Canada that the federal government has contributed towards funding a drug.

CFDI – Research Results

- Key informant interviews (patients, researchers, industry, government)
- CFDI is not a well designed approach for access or reimbursement
- Too many restrictions for patient access
- Patients unable to chose treatment options – or to change treatment options
- Patients must consent to being part of a research trial before gaining access to the treatment
- Lack of lead sponsor – unable to implement changes or improvements to study design
- Trial protocol for 10 years, funding for 3 years
- Bottom line: a reimbursement problem, solved with a research project.

Comments

- Not a good time to be living with a rare disease in Canada, although there is hope for the future (PLF)
- Costs are prohibitive: for the first time in Canada's history of 'universal healthcare' we are hearing of people going bankrupt to pay for their health needs
- We need to implement a *Canadian* solution

“Chance for Life” Strategy

- Applies to drugs that meet the international definition of “orphan drug” (1:2,000)
- Drugs must be approved by Health Canada
- A national, multi-stakeholder Advisory committee to consider each drug and make recommendations:
  - Develop drug access protocol based on drug profile, patient impact, patient preferences.
  - Develop monitoring and evaluation procedures including patient registries, individual patient protocols and data collection
  - Will recommend the optimal funding mechanism
  - Funding mechanisms may include a national fund established for rare disorders and/or funding by healthcare jurisdiction

Thank You

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