Canadian Policy to Provide Drugs for Patients with Rare Disorders: Learning from the Best

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Canadian Organization for Rare Disorders
Finally…Drugs for Rare Disorders

• Decade before 1983: 34 new drugs for rare disorders
• In 2 decades since 1983 Orphan Drug Act, more than 270 drugs approved for rare conditions.
• European Union ODA, 2000: 22 new drugs
• For Patients and Clinicians: “Thank God, finally a ‘chance for life.’ Hopefully, next drug is for us.”
• For Drug Plan “Gatekeepers”: “Oh my gosh, how can we afford this, and how many MORE new therapies are there in the pipeline?”

Canadian Organization for Rare Disorders
New Orphan Drugs Treat Severe, Life-threatening conditions

- **Metabolic disorders (Gaucher > decade ago)**
  - Method for cloning missing enzyme led to effective therapies for related disorders (Fabry, MPS, Pompe)
  - Now a 2nd-line oral therapy for Gaucher

- **Rare blood pressure and blood disorders**
  - Pulmonary arterial hypertension: new therapy in 1995, now three important drugs
  - Hemophilia, thrombocytopenia: improved clotting with less risk

- **Pituitary, thyroid, and parathyroid-related,**
  - Last resort treatment for acromegaly, causing “giantism

- **Childhood disorders, e.g., rare leukemias**

- **One-third OD’s for rare or resistant Cancers**
CDR process Inherently Biased Against Orphan Drugs

- Process relies on large randomized clinical trials with diseases where the impact of treatment over a long period of time is pretty well known.

- Not true for most rare disorders, where the patient populations are very small and the natural history of the disease may not be known.

- Not surprisingly, CDR has rejected almost every drug for rare disorder.
Access to new therapies (Canada)?

- **NAGLAZYME** for MPS VI, not submitted to Health Canada but funded on compassion by Ontario
- **ELAPRASE** for MPS II, submitted to HC, interim funding on compassion by BC but not by Ontario
- **ZAVESCA** for Gaucher, 2nd-line oral, approved by Health Canada but rejected by CDR for insufficient evidence and cost (though better data and lower cost than the 1st-line CERAZYME when it was approved prior to CDR)
- **MYOZYME** for Pompé’s: approved by Health Canada but delayed CDR recommendation; concerns of limit to infants; not adults
Access to new therapies (Canada)?

- **NEXAVAR for advanced kidney cancer, approved by Health Canada but rejected by CDR.**
  - Strong interim data; FDA suggested, for ethical reasons, placebo group be given option to cross over
  - Loss of control group => sufficient long-term survival data
  - Likely to be approved by BCCA (despite CDR rejection)

- **EXJADE for transfusion-related iron overload:**
  - First oral chelator that replaces 12-hour infusion drug, approved by HC but recommended by CDR only for those “contraindicated” for infusion drug.
  - Potential lack of understanding of the significant barrier to compliance of infusion drug, leading to organ failures and deaths
Canada Only Developed Country Without Orphan Drug Policy

- Canadians with RD last to access new therapies
- Drug companies delay (or ignore) filing in Canada
- Since 2003, CDR has recommended against public funding for almost every drug for rare disorders.
- Two-tiered access: those with private drug plans get and those relying public drug plans do not.
- No drug for rare disorders has been approved by a Canadian public drug plan without strident patient advocacy, often resulting in “one-off” political decisions for individual patients.
All stakeholders agree that the current system isn’t working for rare disorders

Former Chair of CEDAC; public acknowledgment that CDR/CEDAC not the right process for rare disorders

Proposed F/P/T Program for Drugs for Rare Disorders is good first step; just don’t call it “Expensive”

We have an opportunity to learn from the best and to create a “unique” Canadian solution based on Canadian values
What are Canadian Healthcare Values re: Rare Disorders?

• *Healthcare is a fundamental right of all Canadians.*

• *Canadians with severe and life-threatening conditions deserve a “chance for life.”*

• *It is unconscionable to deny treatment to Canadians with severe and life-threatening rare disorders because it they costly.*
Dealing with Uncertainty: Perspective Matters

- **Drug Plan Gatekeepers:** How can we make sure that we don’t fund “expensive” drugs with uncertainties about safety, effectiveness, and cost-effectiveness, despite lack of options and willingness of patients to assume risks? How can we be sure that new demands do not exceed our resource limits?

- **Patients and Clinicians:** How can we recognize the uncertainties about long-term safety, effectiveness and cost-effectiveness of orphan drugs but still make them available to as many patients with severe and life-threatening rare disorders as soon as reasonably possible? How can we assure that resources are available to meet new opportunities for severe and life-threatening disorders?
Lessons for Other Countries

• UK supports (through NICE) need to pay more for rarity; model program for “ultra-orphans” that provides approved treatment with agreed-upon “outcomes” for continued access

• Dutch have dedicated drug fund for “orphan drugs” that are handled by separate committee process

• France: rare disorders among top 5 priorities with comprehensive program including compassionate access, centers of reference, and market incentives

• Italy: mandatory reimbursement for all orphan drugs following market authorization
Recommendations for Fair Access to Drugs for Rare Disorders

• Establish separate process for reviewing orphan drugs that is appropriate to small patient populations with severe and life-threatening conditions, where treatments on per patient basis will often be costly
• Establish a “chance for life” fund for rare disorders equal to 2% of total public drug budget, about $190 million annually to be managed jointly
• Include patients in priority setting and decision making on allocations of public expenditures for rare disorders.