Managing the "Expense" in Expensive Drugs for Rare Diseases (EDRD)

PANEL DISCUSSION

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Outline

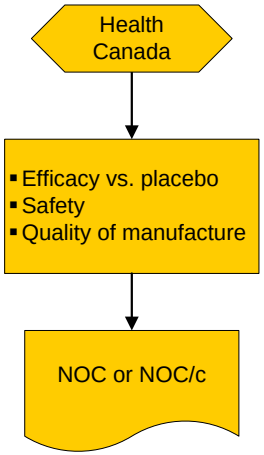
Drug plan management of the "Expense" in EDRDs involves various approaches, processes, and activities, both nationally (with health partners and other drug plans) and by individual drug plans.

1. Drug Review Processes
2. Pan-Canadian Pharmaceutical Alliance (pCPA)
3. P/T EDRD Working Group
4. Management of the "Expense" & Opportunity Cost
5. Summary

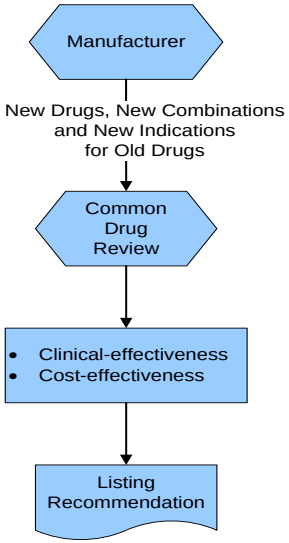
Drug Review Processes - Patented Drugs

Health Canada | CADTH Evidence Driven. | pCPA pan-Canadian Pharmaceutical Alliance | Patented Medicine Prices Review Board

I. Health Canada



II. CADTH – HTA



III. pan-Canadian Pharmaceutical Alliance

IV. F/P/T governments

Public Drug Plans

V. PMPRB

Price Regulator

Consumer protection against excessive pricing

Evidence-informed process
Aim to select best drugs for best value

Source: BC Ministry of Health

Health Technology Assessment

CADTH Evidence Driven.

- **Provide Reimbursement Recommendations to Public Drug Plans**
  - Common Drug Review (CDR) & Pan-Canadian Oncology Drug Review (pCODR)

- **CDR/pCODR "Standard" Reviews:**
  - Systematic review of available clinical evidence
  - Review of pharmacoeconomic evidence
  - Includes input from clinicians and patient groups
  - **Expert Panel Recommendations Options:**
    - Reimburse
    - Reimburse with criteria and conditions
    - Do Not Reimburse (DNR)

- **Drugs for Rare Diseases**
  - Part of standard review process (i.e., not separate process)
  - Enhanced input opportunities for patients & clinicians
  - DRD recommendations:
    - Supportive recommendations often narrow due to limited evidence; (narrower than market authorization indication)
    - Supportive recommendations often include condition of significant price reduction in order to be cost-effective
    - DNR (and limiting recommendations) → stakeholder criticism against HTA process

Pan-Canadian Pharmaceutical Alliance

- **Established in August 2010** by Canada's 13 provincial and territorial (P/T) Premiers' Council of the Federation (Health Care Innovation Working Group)

- Conducts national negotiations for patented & generic drugs to achieve greater national value & coverage consistency for public drug plans

- **Generics:** ~ 70 drugs with lower price (10% to 18% of brand) April 1, 2018

- **Patented / Biosimilar Drugs** (as of March 31, 2019)
  - 44 active negotiations
  - 282 negotiations completed (247 agreements; 35 no agreement)
  - 63 no negotiations

Source: http://www.canadaspremiers.ca/pan-canadian-pharmaceutical-alliance/

pCPA Brief to HESA on DRD

pCPA submitted Brief to House of Commons' Standing Committee on Barriers to Access Treatment & Drugs for Canadians Affected by Rare Diseases (Dec/2018)

- Public drug plans: make drug funding decision that are evidence-informed, cost-effective, and affordable.
- Experiencing significant challenges - Expectations and needs of the public drug plans from the pharmaceutical industry are not being met.
- 3 key challenges related to: (1) Evidence Limitations; (2) High Drug Pricing and (3) Gaps in national alignment and coordination of processes.
- *pCPA negotiations:*
  - *Conducted under very challenging circumstances*
  - *pCPA has completed many negotiations but not able to adequately address pricing concerns*

pCPA's Key Recommendations to Federal Government:

1. Provide national funding for DRD
2. Implement PMPRB's proposed modernization
3. Continue to work with P/T public drug plans and the pCPA to better align, collaborate, and coordinate our efforts.

Source: pCPA Briefing to HESA: http://www.ourcommons.ca/Content/Committee/421/HESA/Brief/BR10275853/br-external/PanCanadianPharmaceuticalAlliance-e.pdf

Report of the Standing Committee on Health

Committee Made 19 Recommendations (Feb 28/19):

10 - Health Canada's Market Authorization of DRD:

- Coordinate market authorization and reimbursement with P/T
- HC and CADTH to review drugs in tandem
- Ensure that drug manufacturers meet NOC obligations
- Special Access Program enhancements

5 - Drug Prices:

- Implement modernization changes with PMPRB
- Establishing requirements for determining price ceilings for DRD
- Add regulatory requirements in the *Patent Act* that require manufacturers to reveal R&D costs for DRD

3 - Reimbursement of Drugs for Rare Diseases:

- F/P/T Governments and manufacturers fund DRD while under review
- Cover DRD as part of a national pharmacare program

1 – Research: Fund CIHR for RWE of treatments for DRD

Source:https://www.ourcommons.ca/DocumentViewer/en/42-1/HESA/report-22

Provincial/ Territorial EDRD Working Group

- P/T EDRD WG Established in fall of 2014 by P/T Health Ministers
- **2015-2016:** Focus Areas: (1) evidence, (2) pricing, (3) access & (4) communications
- **Fall 2018:** The EDRD WG developed a proposal for supplemental processes for complex/specialized drugs that builds upon existing review processes with health partners.
  1. **Early identification of eligible drugs**
  2. **Concurrent submission review process**
  3. **HTA review** (e.g., start/stop criteria, identify RWE requirements)
  4. **pCPA negotiations & implementation** (e.g., consider Managed Access Agreements - MAA, central clinical panels)
  5. **RWE - Collection and reassessment**

- **Proposal Status:** stakeholder consultations complete; reviewing input; developing draft work plan

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Managing the "Expense" in EDRDs

Drug Budget

- Allocation as part of larger health & gov. budget
  - Growth or Reduction
- Ministry Service Plans

Fixed Budget Management:

- Expected to manage within allocated budget
- Many funding demands: Base growth, new demands (drugs, services, programs)
- Approaches:
  - Lower costs (e.g., price negotiations)
  - Find savings (e.g., generics, biosimilars etc.)
  - Request more funding (challenging when budget is set)

→ **Difficult Decisions:**

- **Opportunity Costs: fund one thing→ not able to fund something else**
- **Fund; Do not fund; Defer later**

Example EDRD "Expenses"

DRUG NAME	BRAND NAME	CONDITION	ANNUAL COST[a]
Asfotase alfa	Strensiq	Hypophosphatasia	**$2,200,000**
Nusinersen	Sprinraza	SMA	**$708,000 Yr1** **$354,000 Yr2+**
Eculizumab	Soliris	aHUS	**$701,000**
Alglucosidase	Myozyme	Late Onset Pompe	**$612,000**
Eculizumab	Soliris	PNH	**$526,000**
Ivacaftor	Kalydeco	Cystic Fibrosis	**$306,000**
Agalsidase beta	Fabrazyme	Fabry disease	**$291,000**

[a] Ingredient cost only (excludes markup and dispensing fees) and based on list price using usual maintenance dosing for an assumed 70kg adult (unless otherwise specified; figures rounded to the nearest $1,000).

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Significant Opportunity Costs with EDRDs

- British Columbia Example:
  - BC PharmaCare total budget = $1.23 BN (FY 17/18)
  - Any growth must cover base (existing drugs/pharmacy services) and new demands (e.g., new drugs, new indications, new policy changes etc.)

BC Ministry Coverage	Annual Expenditures	Number of beneficiaries
EDRD	Est. **$33 M** (FY 18/19) Doubled since 2016 30% growth in 2018	**~100**
PharmaCare Plan G (Mental Health)	**$32.7 M** (FY 16/17)	**38,000**
PharmaCare Plan B (Residential Care)	**$37.9 M** (FY 16/17)	**31,000**
PharmaCare Plan P (Palliative Care)	**$19.5 M** (FY 16/17)	**13,000**
Fair Pharmacare Plan Refresh (Universal Income-Based) (lowered income threshold Jan/19)	**Est. $35 M / Yr** (FY 19/20) ($105/3 yrs)	**helps 240,000 more families**

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Summary & What's New?

Drug plan management of the "Expense" in EDRDs:

- **Aim to make drug funding decisions that are evidence-informed, cost-effective, affordable and sustainable.**
- Key challenges with EDRDs: (1) Evidence Limitations; (2) High Drug Pricing and (3) Gaps in national alignment and coordination of processes.
- Various national and individual drug plan approaches, processes, and activities underway
- **Drug plans under fixed budget: Unsustainable → Difficult decisions with EDRD's with significant opportunity costs**

New Proposed Federal Budget (March 2019):

1. ***Canadian Drug Agency*** *(for drug evaluation & price negotiation)*
   $35M over 4 years, starting 2019/20, to set up CDA transition office
2. ***National formulary***
3. ***National strategy for high-cost drugs for rare diseases:***
   $1Bn over two years, starting in 2022/23, with up to $500M per year ongoing