



Optimal Therapeutics in the Cauldron of Uncertainty

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When shall we three meet again?

- government decision-makers
- academic researchers
- manufacturers of pharmaceutical and biological products

In thunder, lightning, or in rain?



We know with certainty only when we know little.
With knowledge doubt increases.

Goethe

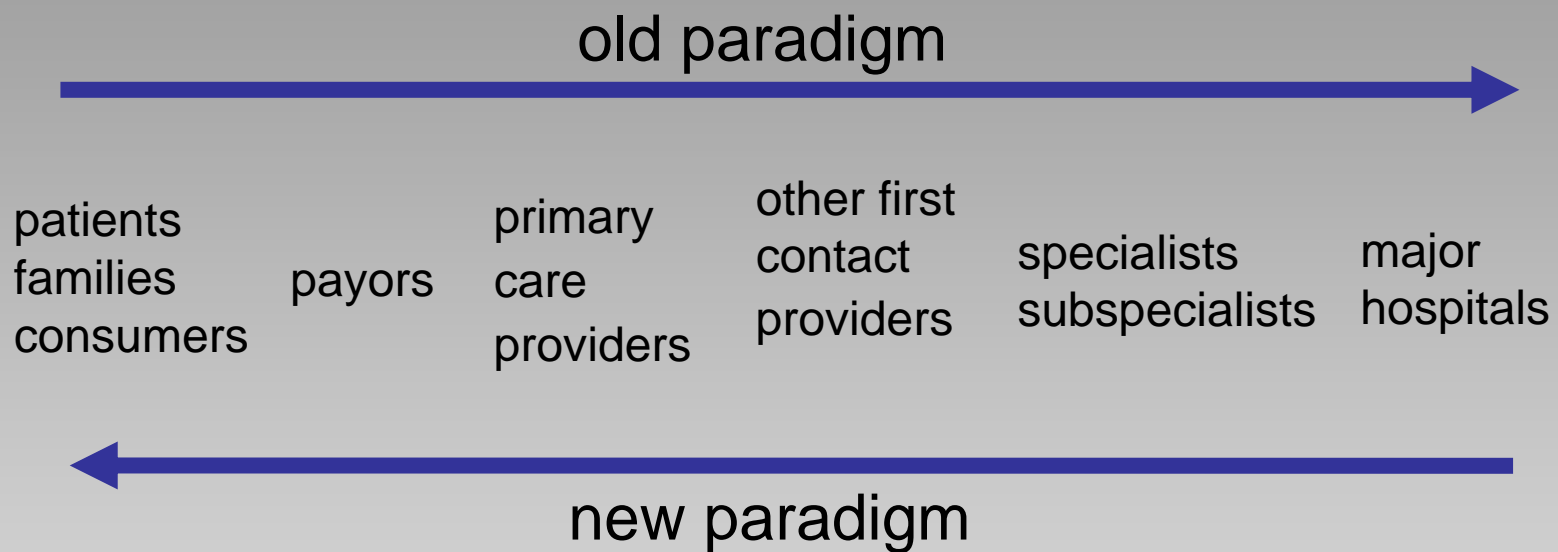
The subject who is truly loyal to the Chief Magistrate
will neither advise nor submit to arbitrary measures.

Junius

It is within our collective responsibility
to challenge conventional wisdom.



The shifting power spectrum in health decision-making



Diverse viewpoints on outcomes of value

- patients/parents
- scientists
- caregivers
- advocacy organizations
- manufacturers
- health/hospital administrators
- legal analysts
- regulators
- EBM reviewers
- payors, including government decision-makers



Applied health research & evaluation for decision makers

- Does it work in real life?
- For whom?
- Is it safe?
- Compared to what?
- At what cost?

public health
decision makers

physicians

patients

Reimburse drug?
Recommend treatments?
Healthcare direction

« The best for my
population »

Treat my patients?

« The best for my
patients »

What to choose?

« The best for me »

revised from Jean-Paul Collet, 2008

The illusion of
rational decision-making



Seeking an improved decision basis: a deliberative process that considers context

- evidence-based vs evidence-informed
- reduced reliance on RCTs
- examine observational studies that go beyond RCTs
- consider composite outcomes
- acceptance of colloquial evidence (Lomas et al)
- use of EPOC approach (Cochrane Effective Practice and Organization of Care Group)

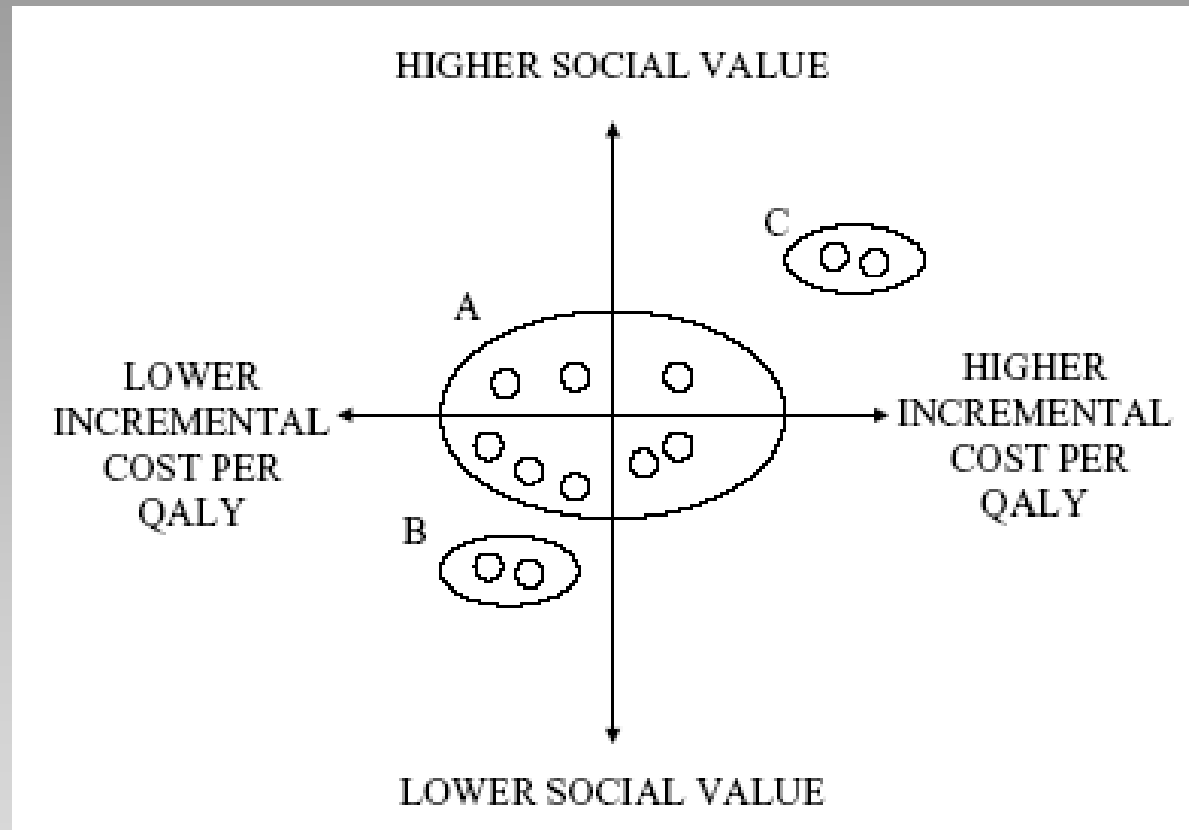


What is a rare disease?

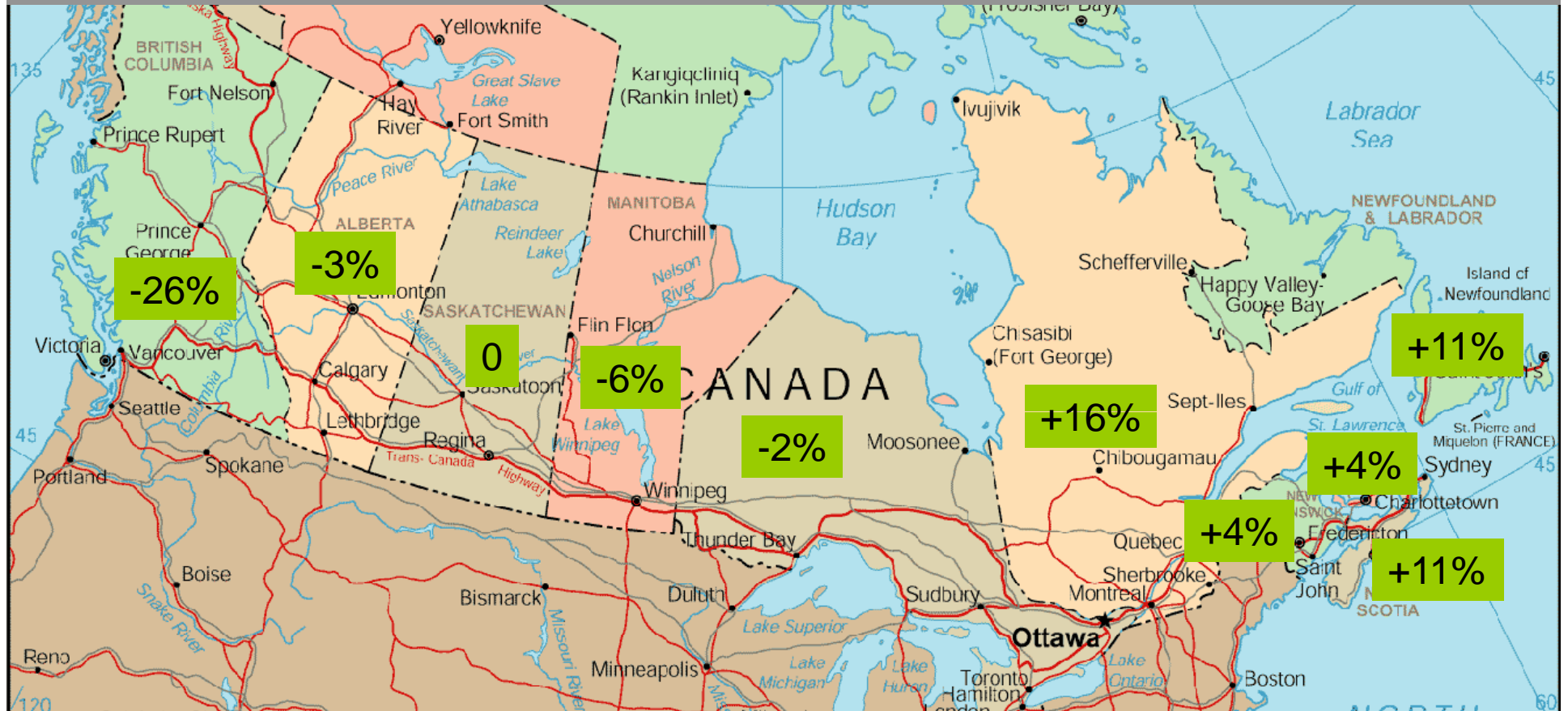
- consensus: 1 in 2,000
- USA: 1 in 1,500
- Australia: 1 in 10,000
- orphan diseases
- orphan drugs
- orphan patients



The relationship between social value and incremental cost per quality-adjusted life-year (QALY)



M Drummond, 2007

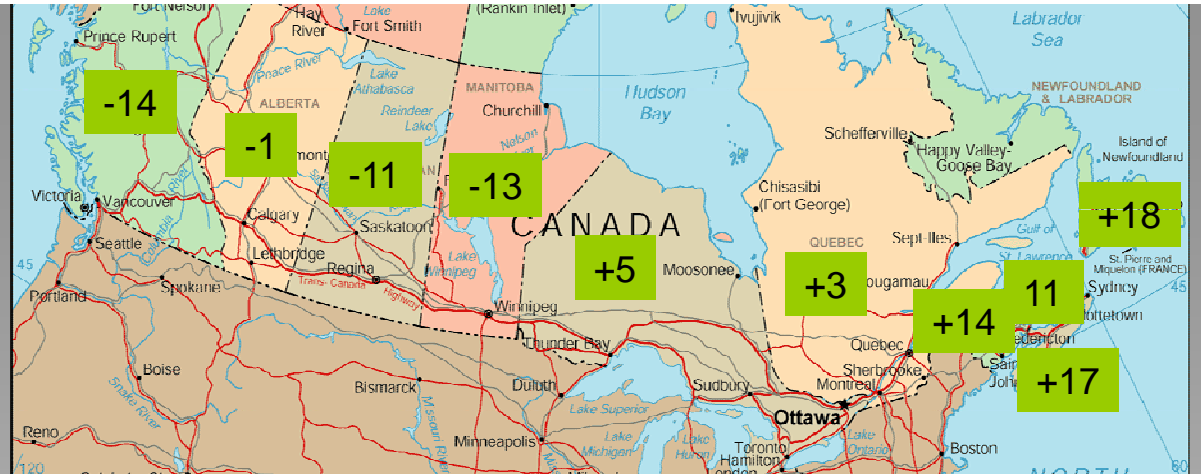


Age standardized variation from the national average per capita spending by province on antihypertensives, 2007

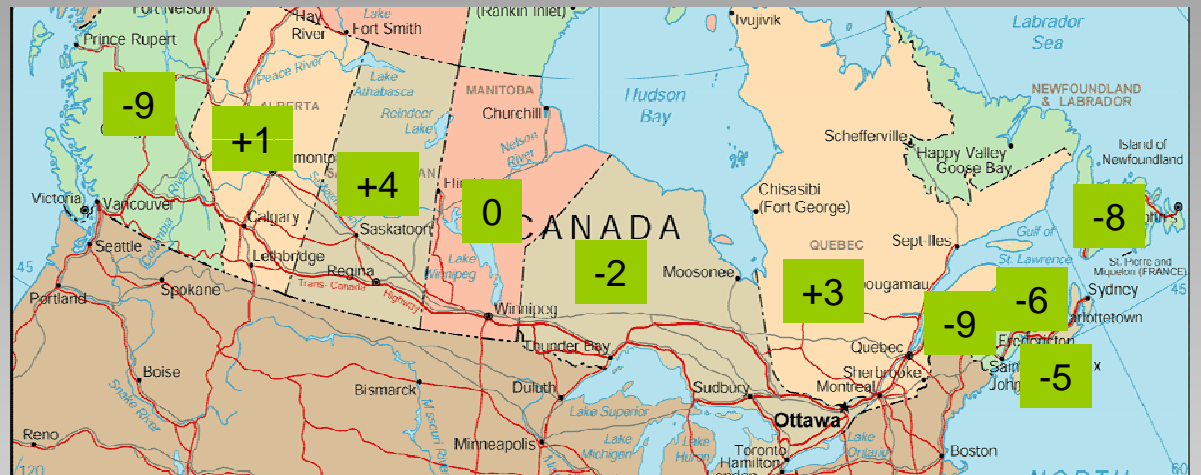
From: Morgan S, Raymond C, Mooney D, Martin D.
The Canadian Rx Atlas. December 2008

Sources of age-standardized variation from the national average per capita spending by province, 2007

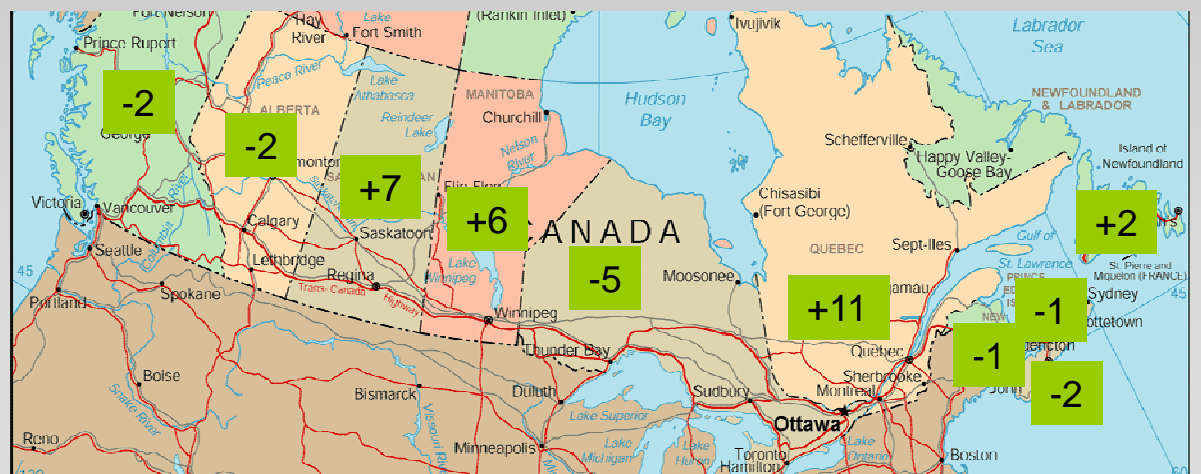
volume effects



therapeutic choice effects



price effects



Treatment of hypertension in British Columbia

- 13.2% of those over 12 years of age carry a current diagnosis of hypertension
- of these, 84% took treatment in preceding month
- a further 4.8% have been told they have 'high blood pressure; but deny current diagnosis or treatment
- up to 38% of British Columbian hypertensives may be undertreated
- Ontario prevalence figures are 16% higher*

* Canadian Community Health Survey 2005



Characteristics of hypertensive Canadians not receiving drug therapy

Hypertension diagnosis inconsistently made

Diagnosed hypertension with no therapy:

20-39 years	50%
40-59 years	17%
>60 years	5%

Factors associated with absence of therapy:

- male sex
- fewer health care professional consultations
- perceived excellent health status
- markers of lower cardiovascular risk
(daily smoking is an exception)

Campbell NR, So L, Amankwah E, Quan H, Maxwell C.
Canadian Hypertension Education Program Outcomes Research Task Force
2005 Canadian Community Health Survey. Can J Cardiol 2008;24:485-90.

Treatment of hypertension in Canada

- Is cost a deterrent to effective therapy?
- Why is it so difficult to improve adherence with effective therapy?
- Are lower costs in some provinces associated with better or worse outcomes?

Reduction in cardiovascular death and hospitalization is associated with an increase in antihypertensive prescriptions following patient education programs.

CCHS 2005



Daniels and Sabin 1997

There are four elements of legitimacy and fairness in public decision-making:

- stakeholder involvement
- publicity (transparency, dissemination)
- revision or appeal
- leadership, including accountability for reasonableness



Canadian initiatives

- CCOHTA → CADTH
- CAPIA
- NPDUIS
- CDR - CEDAC
- HC - MEDEFECT
- National Pharmaceutical Strategy
- DSEN
- Health Canada legislative renewal
“Life Cycle Approach”



American initiatives

- FDAMA
- PDUFA
- FDAAA – nation-wide system for ADR monitoring
- coverage with evidence development
- American Recovery and Reinvestment Act of 2009
\$1.1billion for comparative effectiveness research





Canadian Agency for
Drugs and Technologies
in Health

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Optimizing the Use of Drugs and Health Technologies

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There are no more great men, only great committees.