Drug safety and effectiveness: real-world challenges and opportunities for health care policy, practice and research - provincial perspective

2009 CAHSPR Conference
Calgary, Alberta
May 12-13, 2009

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Goals of decisions

- Society's therapy needs
- No need → no development or approval = no pressure to pay
- Funding aligned with needs
- Real, tangible advantages
- Measured improvement in health outcomes, not hypothesized likelihoods (don't work = don't pay)

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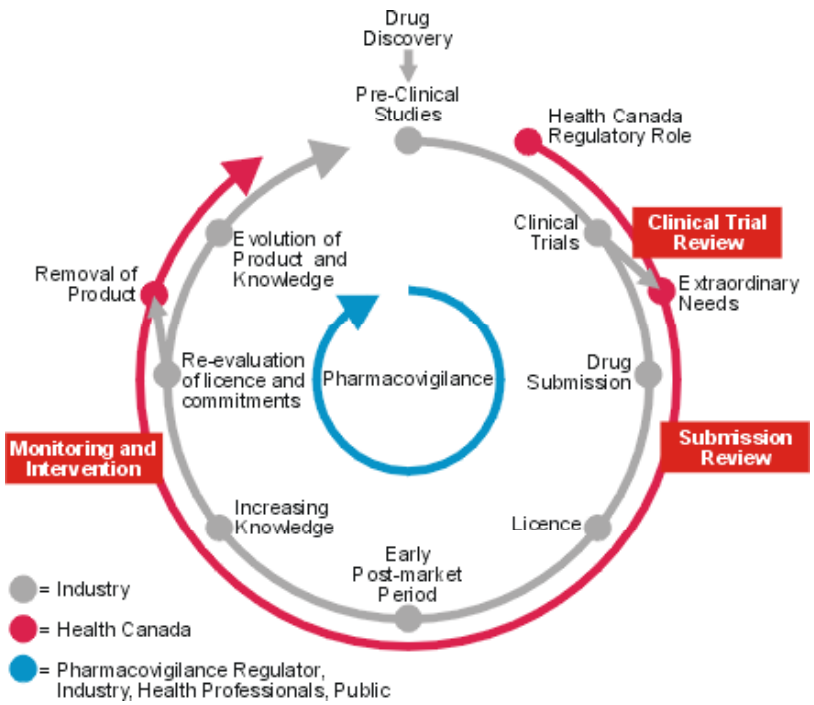
Life-cycle of a drug



Adapted from Health Canada http://www.hc-sc.gc.ca/dhp-mps/homologation-licensing/model/life-cycle-vie-eng.php

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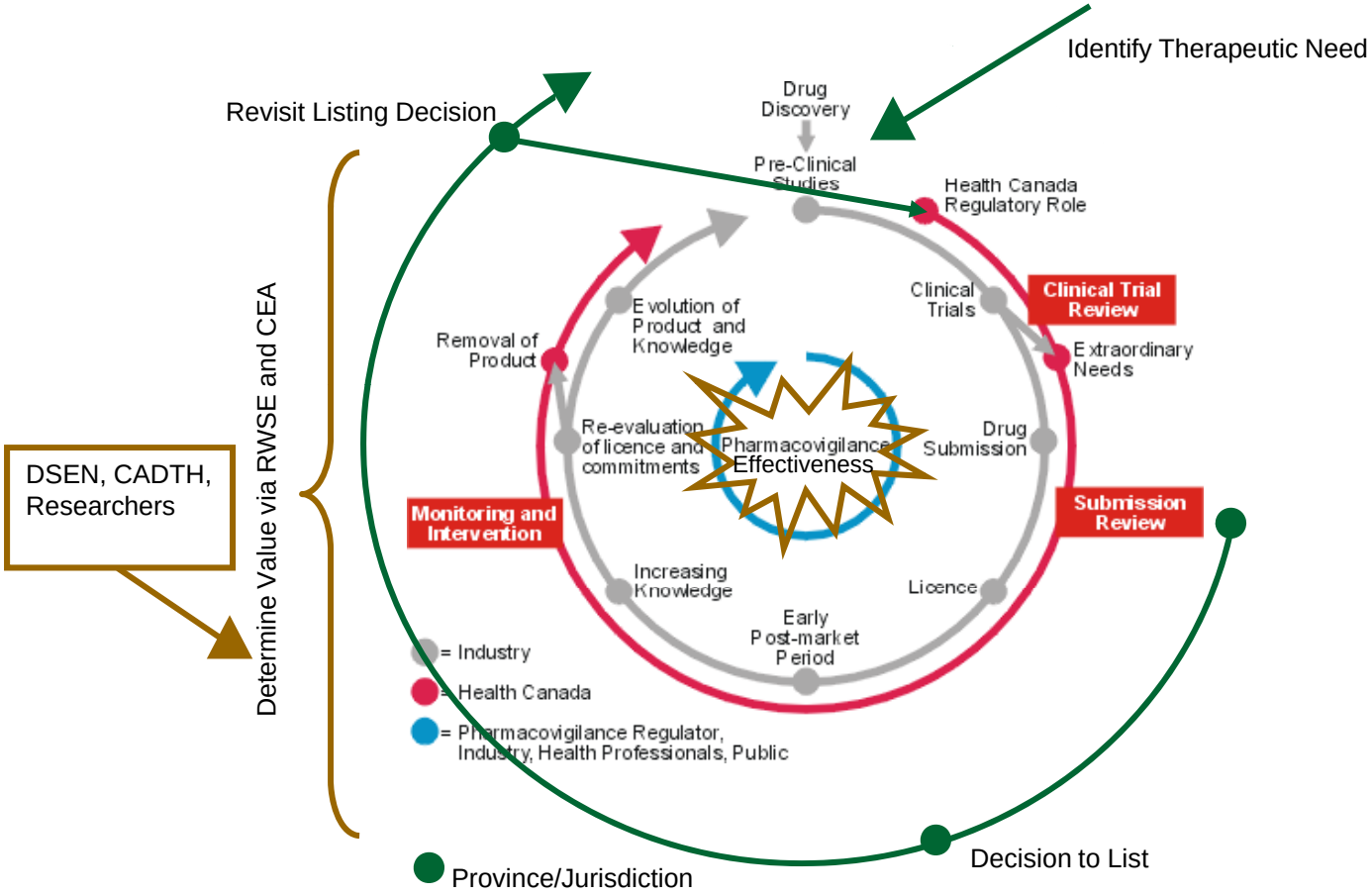
Life-cycle of a listing decision



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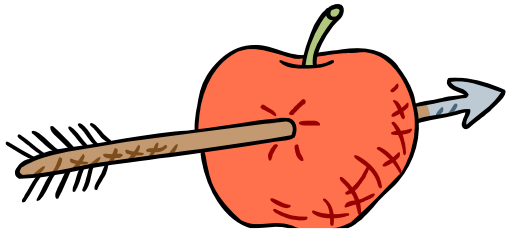
Decision points

To list

- RCT evidence
- safety and efficacy
- cost-efficacy
- investing in improved health outcomes

To continue or adjust listing

- pragmatic evidence
- RWSE
- cost-effectiveness
- achieving improved health outcomes



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Challenges to realizing the real world

RCT

- Methodology meets standard publication requirements
- Producers perform this research for regulatory purposes already

RWSE

- Newer methodology not as readily accepted for publication
- Data: availability, comparability, linkages, privacy issues
- Funding: who pays? who does the work?

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Coverage with evidence development

- When we think a drug will meet our needs, but the evidence isn't quite there
- Conditional coverage with data collection
  - enhance evidence base
  - determine targeted populations for optimal effect
- BC example = Alzheimer's Drug Therapy Initiative
- Role of the DSEN

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Real world safety and effectiveness

- Was the needed outcome provided?
- Simplest concept
  - determine outcome endpoints from efficacy trials
  - monitor same endpoints in real life
- Compares effectiveness to efficacy
- Compare cost-effectiveness to cost-efficacy
- Role of the DSEN - Justify/modify formulary decisions → transparency

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Prescription for optimal real world decisions

- Regulators, payers, and producers identify and agree on priority needs
- Coverage with evidence development and RWSE as health outcome measurement tools
- Infrastructure for RWSE and real world CEA
- real world evaluation in a rigorous yet timely way that can inform decisions
- Role of the DSEN

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