Engaging publics on reimbursement decision-making for drugs for rare diseases

Elizabeth Wilcox
School of Population and Public Health
University of British Columbia
April 7, 2014
Overview

• Problem
• Objectives
• Plan
  – EDRD deliberative public engagement
  – Wider public dialogue
  – Knowledge-user workshop
• Potential
Problem

- Rare diseases
  - Fewer than 5 in 10,000 people
  - +7,000 different rare diseases

- Expensive drugs
  - +$100,000 per patient per year

- Limited budget
  - Clinical and cost effectiveness
  - Value-based decisions
Whose values?

- Experts
- Decision-makers
- Patients
- Publics
Public engagement

Goals

1. Developmental/ Educative
   - Impart information
2. Democratic/ Political
   - Increase accountability and legitimacy
3. Instrumental
   - Improve decision-making
Objectives

• Determine the societal values and perspectives associated with reimbursement decision-making for EDRD
• Explore the potential usefulness and impact of recommendations on reimbursement decision-making processes
Phases

• Preparation
  – Literature review
  – Consultation with experts and decision-makers

• Engagement
  – EDRD deliberative public engagement
  – Wider public dialogue
  – Knowledge-user workshop

• Dissemination
EDRD public engagement

• ~30 people from across British Columbia
  – Geographical distribution ~ health authorities
  – Demographic distribution ~ sex, age, ethnicity

• Two weekends (four days)

• To deliberate on reimbursement decision-making for expensive drugs for rare diseases
  – Share their perspectives and the REASONS behind them
EDRD public engagement

Weekend 1
- **Day 1**
  - Listening, learning and asking questions
- **Day 2**
  - Exploring issues and developing recommendations

Weekend 2
- **Day 3**
  - Exploring issues and developing recommendations
- **Day 4**
  - Revising and voting on final recommendations
Deliberative questions

- What criteria should be considered during drug review processes?
- Should there be a maximum price or threshold for EDRD?
- Who should be involved in drug reimbursement decision-making processes? How?
Frequency of factor selection by subjects (%)
Wider public dialogue

• Survey of ~2,000 people across BC
• Explain the problem and the process
• Describe the final recommendations
• Elicit feedback
Knowledge-user workshop

• Presentation of process and final recommendations to experts and decision-makers
• Discussion and survey on how these might impact decision-making processes
• Follow-up
Potential

• Share information regarding difficult drug reimbursement decisions
• Increase transparency, accountability, legitimacy of decision-making processes
• Lead to better-informed decision-making processes that account for societal values
Acknowledgements

• Supervisory Committee
  – Craig Mitton
  – Larry Lynd
  – Jennifer Gibson
  – Michael Burgess
• CIHR New Emerging Team for Rare Diseases
• Western Regional Training Centre for Health Services Research
• Pfizer Canada

Thank you!
wilcox.liz@gmail.com