



# Social values and public policy on cancer drug funding for children in Canada

Avram Denburg, MD MSc PhD FRCPC  
ARCC Conference  
May 28, 2019

# Front Matter



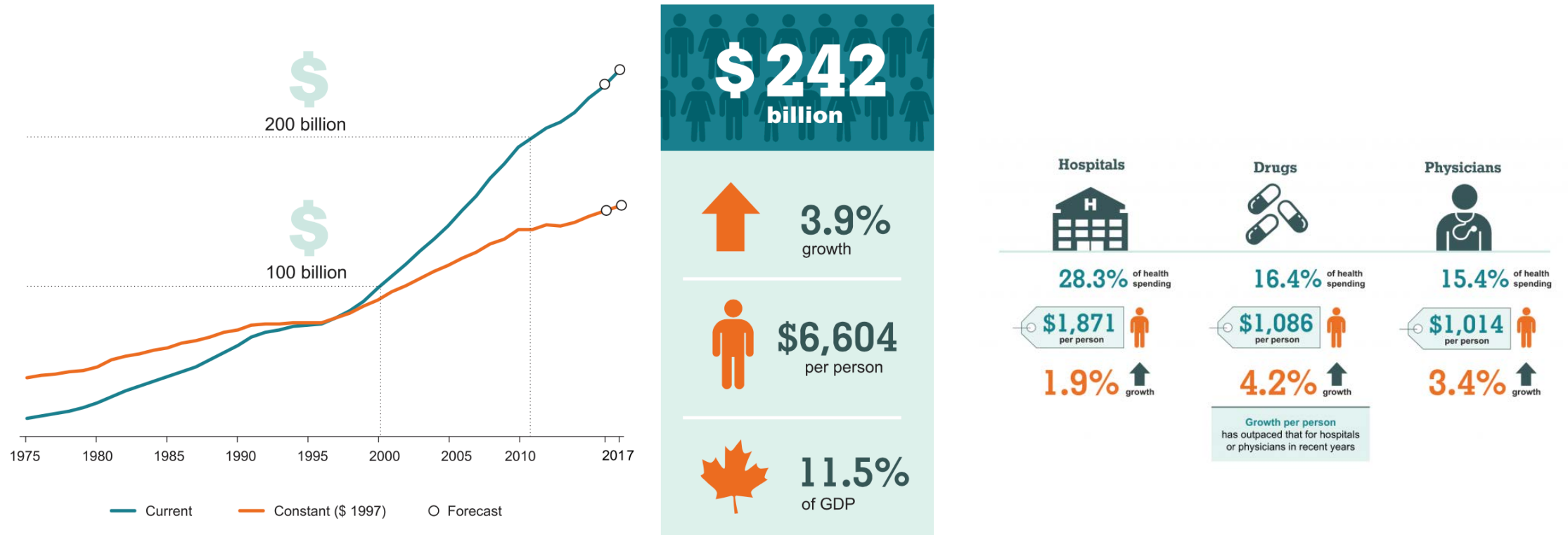
## Acknowledgments and Disclosures

- Research funding: CIHR, CCHCSP, CHEPA, Trudeau Foundation
- Co-investigators: Julia Abelson, Mita Giacomini, Wendy Ungar
- Conflicts of interests: None





# Resource Scarcity: Costs Outpacing Growth



Source: National Health Expenditure Database, Canadian Institute for Health Information.

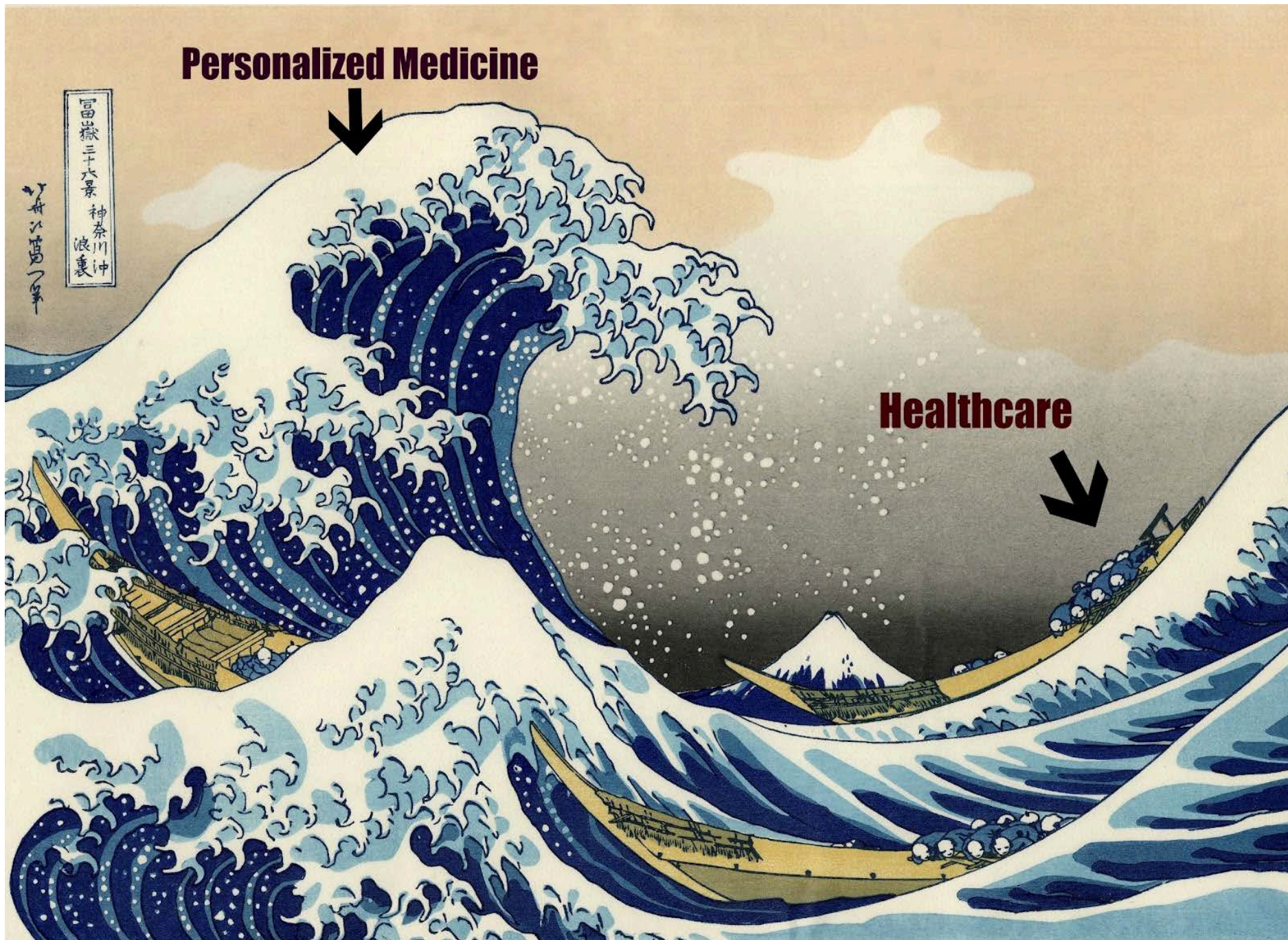
**Personalized Medicine**



富嶽三十六景 神奈川沖  
波裏

以舟の笛の聲

**Healthcare**



# *Mediating Public Policy*



# Public Drug Policy for Children in Canada

## The Context

- Patchwork national coverage
- Unique dimensions of child health relevant to drug policymaking
- Lack of child-specific policy: Regulator → HTA → Payer
- Opportunity to lead

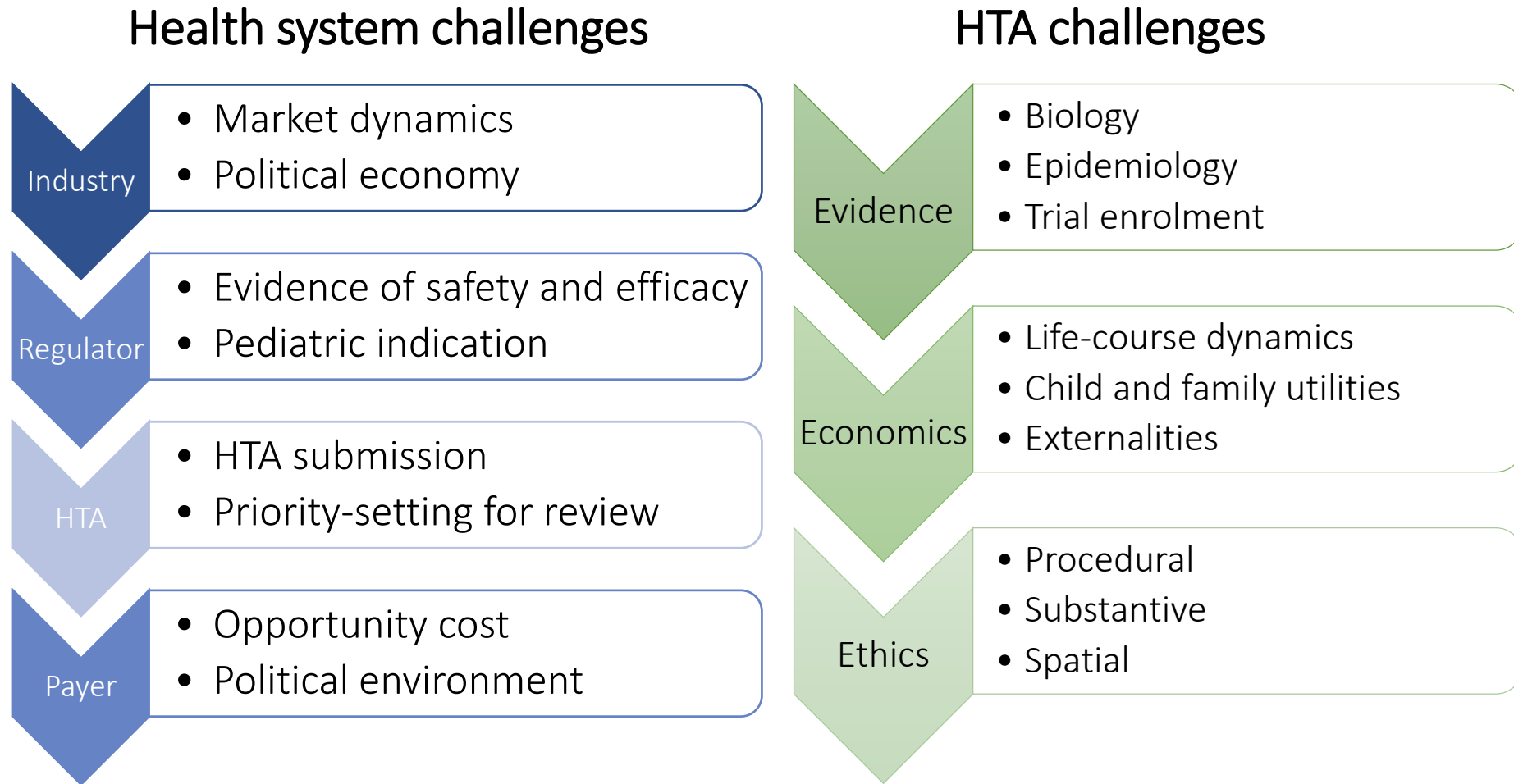
### KEY POINTS

- Making decisions about public funding for drugs for children is uniquely challenging.
- Health technology assessment frameworks need better ways to incorporate the unique evidentiary, economic and ethical dimensions of child health.
- Canada needs a child-focused national policy framework for drug funding that reflects partnerships between industry, provincial governments and health-system stakeholders to entrench reliable and equitable processes for evaluating child health technologies.

**Table 1: Provincial and territorial drug programs that provide benefits to children\***

Program type	NL	PE	NS	NB	QC	ON	MB	SK	AB	BC	YK	NWT	NU
Universal program for all residents without private insurance (deductibles are not income-indexed)				√	√	√†			√				√‡
Income-indexed drug plan							√			√			
Income-indexed catastrophic drug plan for persons with very high costs relative to income or transitional plan for persons leaving social assistance	√	√	√	√		√		√	√§				
Social assistance/welfare	√	√	√	√	√	√	√	√	√	√			
Special family/child program for low-income families	√	√						√	√			√	
Specific program for:													
Cystic fibrosis	√	√		√		√				√			
Diabetes		√		√									
Human growth hormone	√	√		√									
Children with severe disabilities			√	√		√				√		√	
Umbrella program for chronic disease						√		√	√		√	√	√

# Pediatric Drug Access in Canada



Lack of coherent, consistent and equitable drug policy

# Research Aims

1. Review and critically analyze the literature on the moral dimensions of child health and social policy (**Study 1**)
2. Probe the complexities of HTA and drug policy for children, with specific emphasis on the Canadian context (**Study 2**)
3. Generate empirical evidence on societal preferences related to the allocation of public funds for child health interventions (**Study 3**)



*'The problem is small enough, the problem is big enough'*: Social values and public policy on drug funding decisions for children in Canada

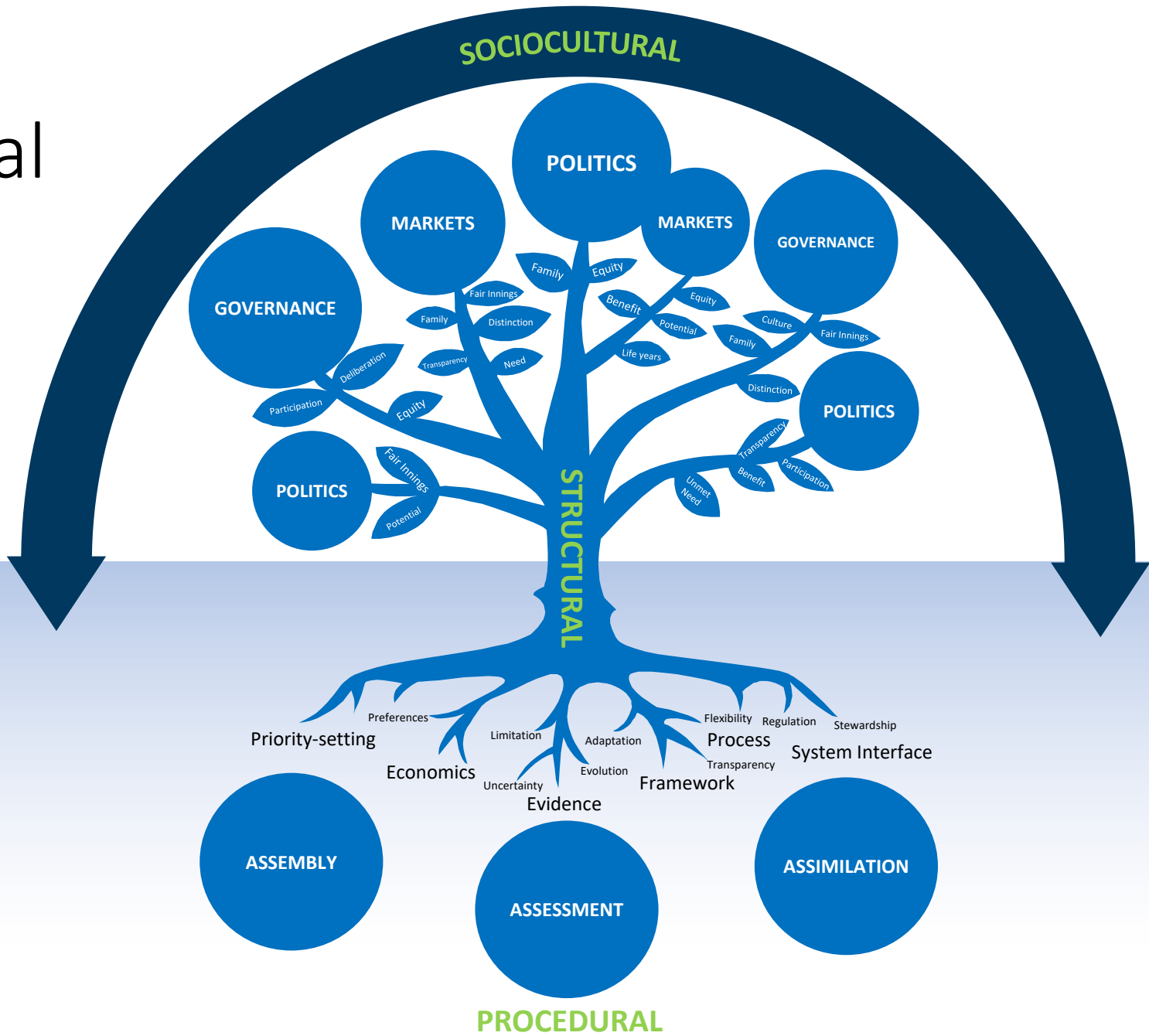
Denburg AE, Giacomini M, Ungar WJ, Abelson J

# Research Objective and Methods

- **Objective:** To explore the determinants of public policy decisions on pediatric drug funding in Canada
- **Methodology:** Grounded theory<sup>13</sup>
- **Data sources:** In-depth, semi-structured interviews with stratified purposive sample ( $n=22$ ) of stakeholders involved with or affected by HTA for child health interventions
- **Study population:** Parents of children with cancer, health professionals, national HTA professionals, Ontario policymakers
- **Analysis:** Inductive coding (open to axial), constant comparison, theoretical sampling to saturation
  - Theoretical frameworks:
    - 'Technology-as-policy'<sup>14-15</sup>
    - Sociopolitics of health technologies<sup>16</sup>

# Results: Conceptual Overview

SOCIETY  
TECHNOLOGY



PROCEDURAL

# Assembly: Priority-setting principles

*“If your view is you have to look first at the most common cancers, one hundred percent of the time children are going to be back of the queue.”* (Provincial policymaker)

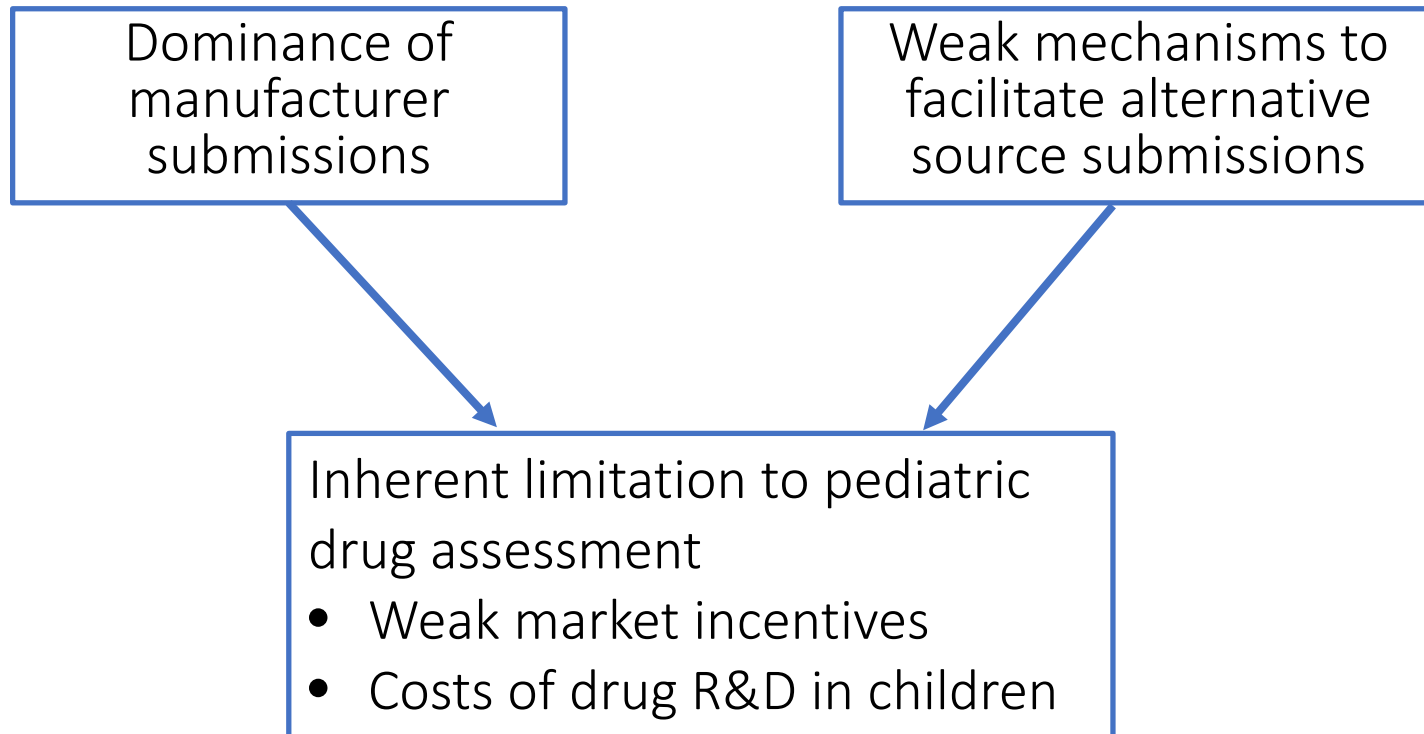
- **Prioritization criteria**
  - Limited palette of values for technology priority-setting and assembly, haphazard application
  - Potential for intrinsic bias against paediatric drugs
  - Need to consider the upstream impact of priority-setting principles on the selection and downstream assessment of child health technologies

**Table 3. Prevalence of Criteria across HTA Agencies**

Category	Agencies using criteria under the category (%)
Alternatives	1 (9)
Budget impact	6 (55)
Clinical impact	11 (100)
Controversial nature of proposed technology	2 (18)
Disease burden	7 (64)
Economic impact	10 (91)
Ethical, legal, or psychosocial implications	2 (18)
Evidence	5 (45)
Expected level of interest	5 (45)
Timeliness of review	4 (36)
Variation in rates of use	3 (27)

# Assembly: Process limitations

*“The one policy thing that really bothers me in drugs is, it relies almost universally on the manufacturer putting something forward. So, the biggest issue I find in HTA – and...it's particularly relevant to paediatrics – is a lot of things never actually come forward for HTA.” (Provincial policymaker)*



## **Proposed solutions:**

- Investment to support provider submissions
- Pediatric indication requirements on HTA submissions
- ‘Pediatric HTA watchdog organization’

# Assessment: Evidence

*"When you have children who are being put into very specific baskets of their genomic and genetic markers...this problem of the uncertainty of the evidence is going to really hit an apex. It's going to mean looking at clinical trials and looking at evidence...very differently. We're going to have to see a major shift." (Parent)*

*"Small numbers, small bodies, long outcomes: there has to be a different perspective." (Health professional)*

## **Limitation**

- Tension with epistemic hierarchy of EBM
  - Overlaps, but not coincident with, rare disease issues
- Salient factors:
  - Population health dynamics, complexity of paediatric research, market incentives, life-course impacts

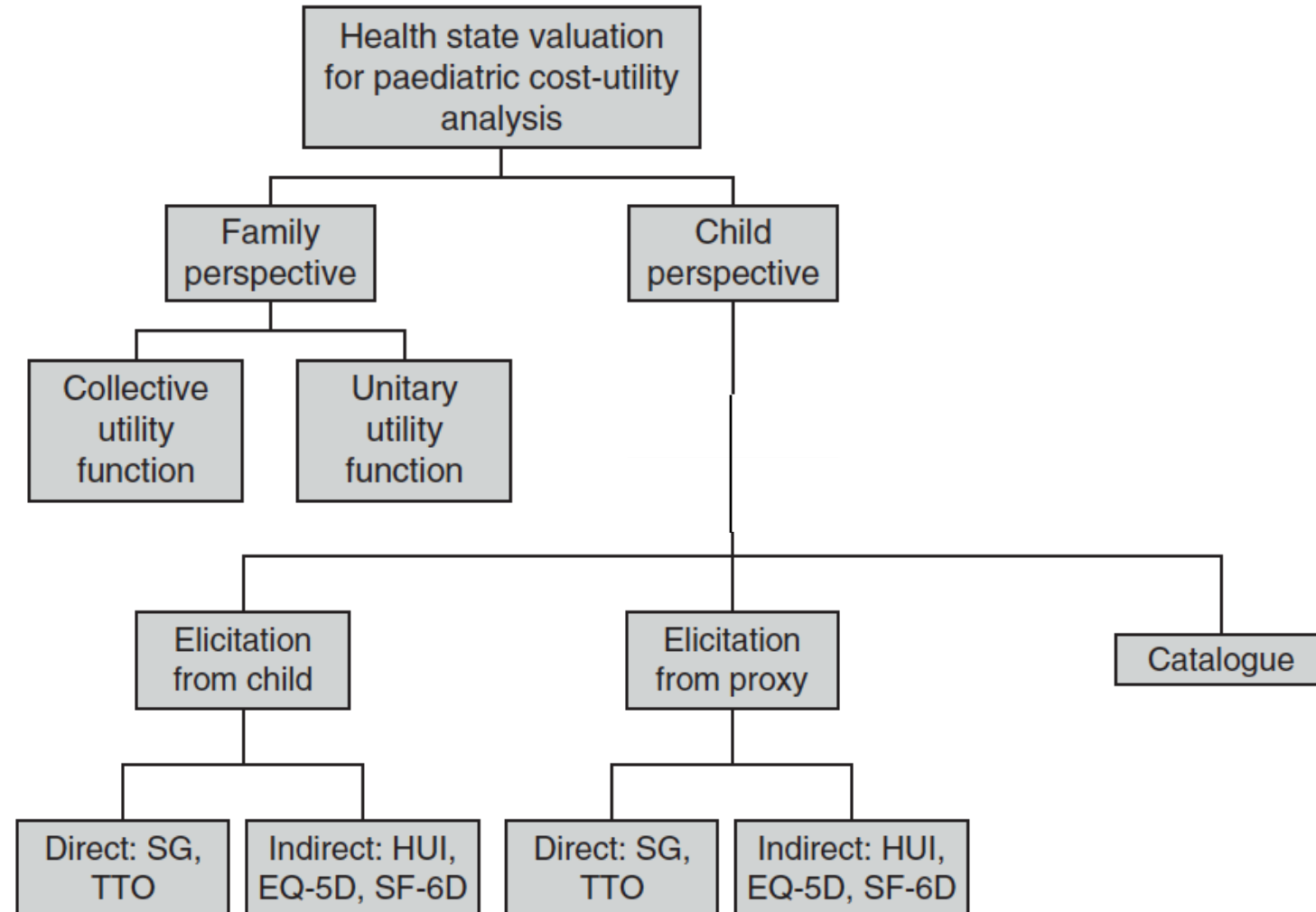
## **Evolution/Adaptation**

- Respect for diverse forms of evidence, importance of clinical and social context
- Need for innovative ways to produce, incorporate, and assess paediatric evidence
- Examples: basket trials, n-of-1, adaptive pathways

# Assessment: Economics

*“An unhealthy child is generally an unhealthy mother and, not uncommonly, an unhealthy father and siblings as well. So, the notion of unit of analysis, I think, is very germane to childhood.”* (Health professional)

- Life-course modelling
  - Time horizon and uncertainty
  - Late effects and health state transitions
- Preference elicitation
  - Child utilities
  - Family utility
- QALY weighting/aggregation
  - Fair innings
  - Sensitive periods
  - Individual vs population



# Assimilation

*"You want guidelines that are going to be able to give people appropriate constructs to make decisions, but within those guidelines, you want to make sure they are not so hard and fast. Because the world – especially of paediatric cancer – is changing, and if you're creating guidelines which are too rigid that could be a real danger." (Parent)*

## **Framework and Process**

- Emphasis on means above ends
  - Challenge and opportunity of integrating diverse forms of evidence
  - e.g. Deliberative public engagement
- Value of distinct framework for child-focused HTA
  - Sample considerations: Developmental phases, future potential, family context, alternative voices

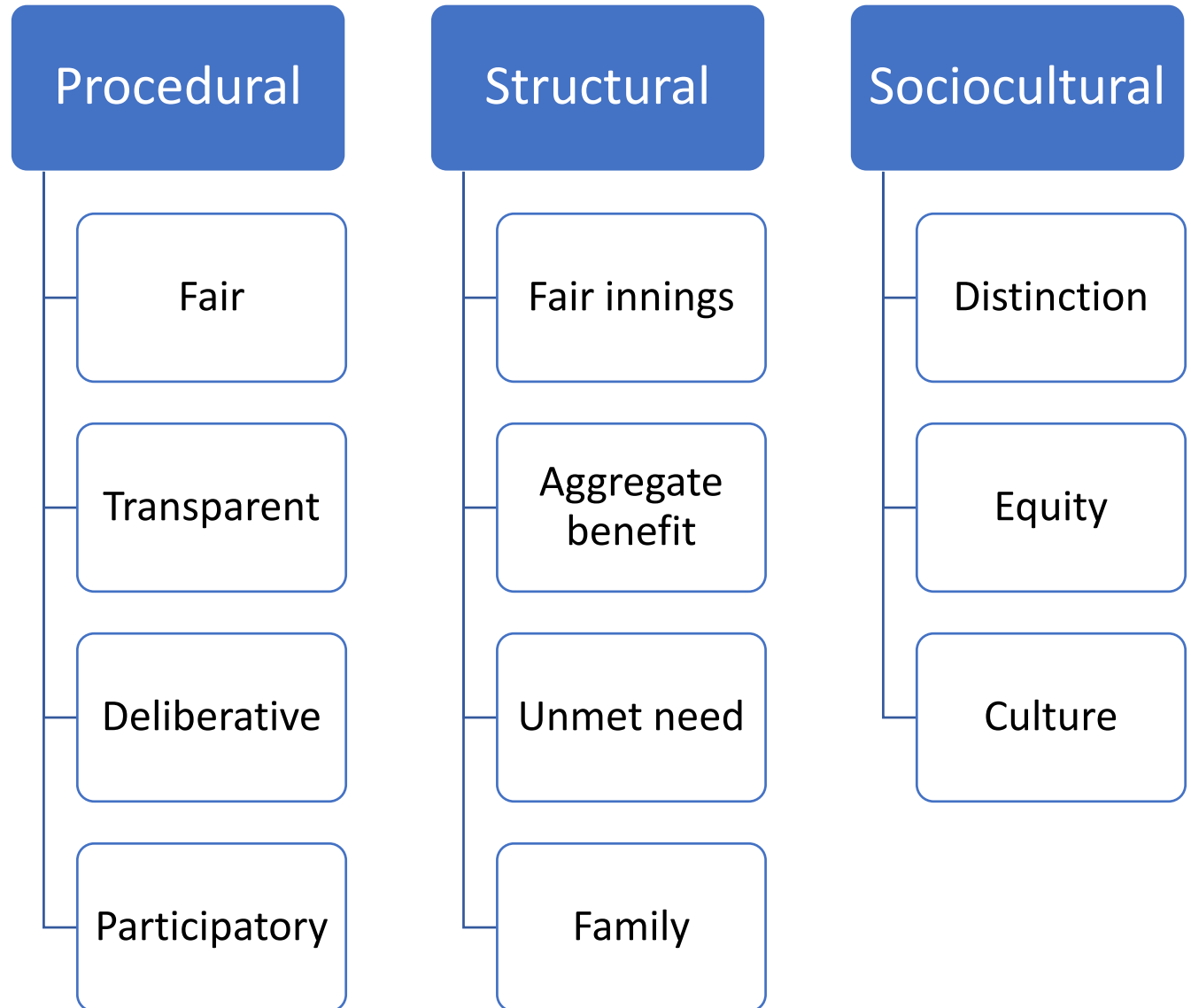
## **System Interface**

- Enhanced institutional collaboration and process integration
  - e.g. Adaptive pathways
- Need to take a system-level view
  - Opportunity costs
  - Societal preferences
- Balance between normative structure and flexibility

# Values Typology

*"Societies are judged by how they treat the elderly, the infirm, and the children. When the infirm are also the children, I think there is a double ethical responsibility by society."* (Parent)

*"Before we get some people to move from 80 to 90 we should get everyone to 20 first in terms of fairness. Before everyone has two houses everyone should have one place to live."* (Health professional)



# Qualitative Analysis: Key Messages

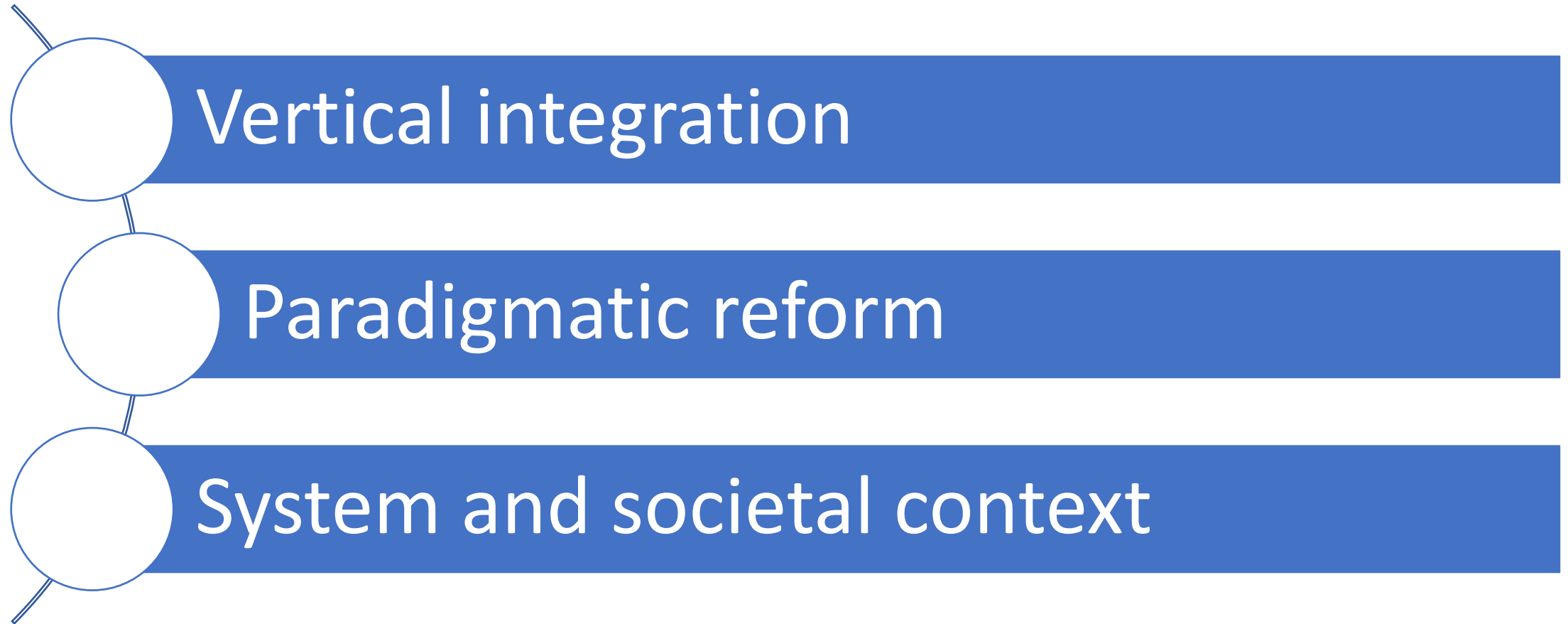
## 1. Differences of context and substance

- Important differences in the production, evaluation, and use of child health technologies
- Differences span the technical aspects of HTA and the surrounding sociopolitical milieu
- These distinctions reveal shortcomings of current HTA paradigms for evaluation of child health technologies

## 2. Distinct typology of social values

- Range of social values elicited from varied stakeholder groups with relevance by conceptual domain of drug policy process
- Novel typology of values applicable to child drug policy analysis in varied health system contexts

# Policy Implications: Paediatric Drug Coverage



# Future Directions

1. Normative analyses of child health and social policies
  - Use of CIS conceptual framework to analyze policy and media documents in specific policy domains
2. Comparative health system analyses of pediatric drug policy environments
  - Cross-provincial, international, temporal
3. Economic evaluation of novel child health technologies
  - Modelling life-course benefits and costs
  - Elicitation of child and family health state utilities
4. Public preferences for allocation involving children
  - Stated preference/discrete choice: Children of varied ages, health conditions, diagnostic and therapeutic technologies
  - Deliberative public engagement on child funding priorities

# Questions?



“Don’t—they’ll just spend it on drugs.”

# References

1. Denburg AE, Ungar WJ, Greenberg M. Public drug policy for children in Canada. *CMAJ* 2017; July 31; 189: E990-4. DOI: 10.1503/cmaj.170380.
2. Moss P, Petrie P. *From Children's Services to Children's Spaces: Public Policy, Children and Childhood*. London: RoutledgeFalmer, 2002.
3. Dixon-Woods M, Cavers D, Agarwal S, et al. Conducting a critical interpretive synthesis of the literature on access to healthcare by vulnerable groups. *BMC Medical Research Methodology* 2006; 6: 35-48.
4. Wyness M. *Childhood and Society: An Introduction to the Sociology of Childhood*. New York: Palgrave MacMillan, 2006.
5. Mayall B. Towards a sociology of child health. *Sociology of Health & Illness* 1998; 20(3): 269-288.
6. Schneider A, Ingram H. The social construction of target populations. *American Political Science Review* 1993; 87(2): 334-347.
7. Mayall B. Towards a sociology of child health. *Sociology of Health & Illness* 1998; 20(3): 269-288.
8. Kahn A. From child-saving to child development. In: Kamerman SB, Phipps S, Ben-Arieh A, eds. *From Child Welfare to Child Well-Being: An International Perspective on Knowledge in the Service of Policy-Making*. New York: Springer, 2009: 3-7.
9. Ross LR, Saal HM, et al. Technical report: Ethical and policy issues in genetic testing and screening of children. *Genetics in Medicine* 2013; 15(3): 234-245.
10. Hardart GE, Chung WK. Genetic testing of children for diseases that have onset in adulthood: the limits of family interests. *Pediatrics* 2014; 134(S2): S104-110.
11. Zawati MH, Parry D, Knoppers BM. The best interests of the child and the return of results in genetic research: international comparative perspectives. *BMC Medical Ethics* 15: 1-13.
12. Clayton EW, McCullough LB, Biesecker LG, et al. Addressing the ethical challenges in genetic testing and sequencing of children. *American Journal of Bioethics* 2014; 14(3): 3-9.
13. Ungar WJ. Challenges in health state valuation in paediatric economic evaluation. *Pharmacoeconomics* 2011; 29(8): 641-652.
14. Kahneman D. A perspective on judgment and choice – Mapping bounded rationality. *American Psychologist* 2003; 58: 697–720.
15. Stanovich KE, West RF. Individual differences in reasoning: Implications for the rationality debate? *Behavioral and Brain Sciences* 2000; 23: 645–665.