

Given the inherent uncertainty in evaluation of precision pediatric drugs, a child-tailored value assessment framework such as *The Comprehensive Assessment of Technologies for Child Health (CATCH)* may bolster child-relevant funding adjudication by health technology assessment organizations.

HTA168

Widening the Value Lens for Child Health Technologies: Development of a Child-Tailored HTA Value Assessment Framework using Multi-Criteria Decision Analysis Incorporating Patient, Public and Expert Perspectives

Cindy L. Gauvreau, PhD¹; Leighton Schreyer, BSc²; Paul Gibson, MD^{3,4}; Alicia Koo, PharmD⁵; Wendy J. Ungar, MSc PhD^{1,6}; Dean Regier, PhD⁷; Kelvin Chan, MD PhD⁸; Robin Hayeems, PhD^{1,6}; Jennifer Gibson, PhD⁹; Antonia Palmer, MSc¹⁰; Stuart Peacock, DPhil¹¹; Avram Denburg, MD PhD^{1,6}

1. Child Health Evaluative Sciences, The Hospital for Sick Children Research Institute, Toronto, ON, Canada 2. Temerty Faculty of Medicine, University of Toronto, Toronto, ON, Canada 3. McMaster Children's Hospital, Hamilton, ON, Canada 4. Pediatric Oncology Group of Ontario, Toronto, ON, Canada 5. Department of Pharmacy, The Hospital for Sick Children, Toronto, ON, Canada 6. Institute of Health Policy, Management and Evaluation, University of Toronto, Toronto, ON, Canada 7. Cancer Control Research, British Columbia Cancer, Vancouver, BC, Canada 8. Odette Cancer Centre, Sunnybrook Hospital, Toronto, ON, Canada 9. Joint Centre for Bioethics, University of Toronto, Toronto, ON, Canada 10. Ac2orn: Advocacy for Canadian Childhood Oncology Research Network, Toronto, ON, Canada 11. Faculty of Health Sciences, Simon Fraser University, Vancouver, BC, Canada.

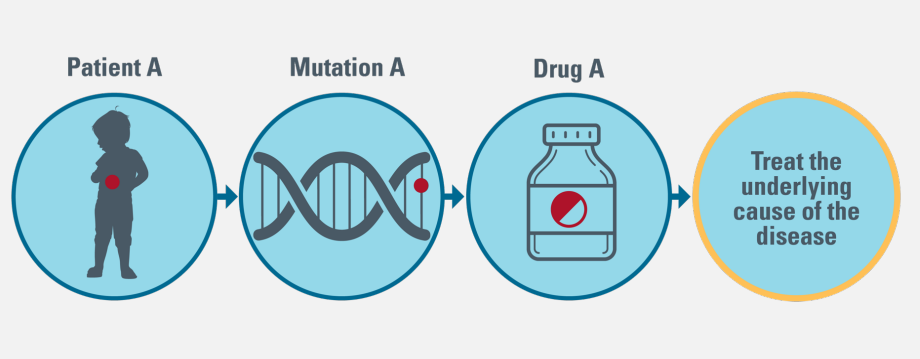
BACKGROUND

Health technology assessments (HTAs) to determine precision oncology drug funding do not systematically account for the circumstances and needs of children and youth.

In an era of exceptionally-priced drugs, “value” and health system sustainability are also being challenged.

Evidence-based deliberations in HTA processes are increasingly recognized as important approaches in holistic evaluations.

To inform and complement traditional HTA, we aimed to develop and test a child-tailored value assessment framework based on a multi-criteria decision analysis (MCDA) model derived from deliberative engagement.



Adapted from: <https://www.danafarberbostonchildrens.org/innovative-approaches/precision-medicine/what-is-precision-medicine.aspx>

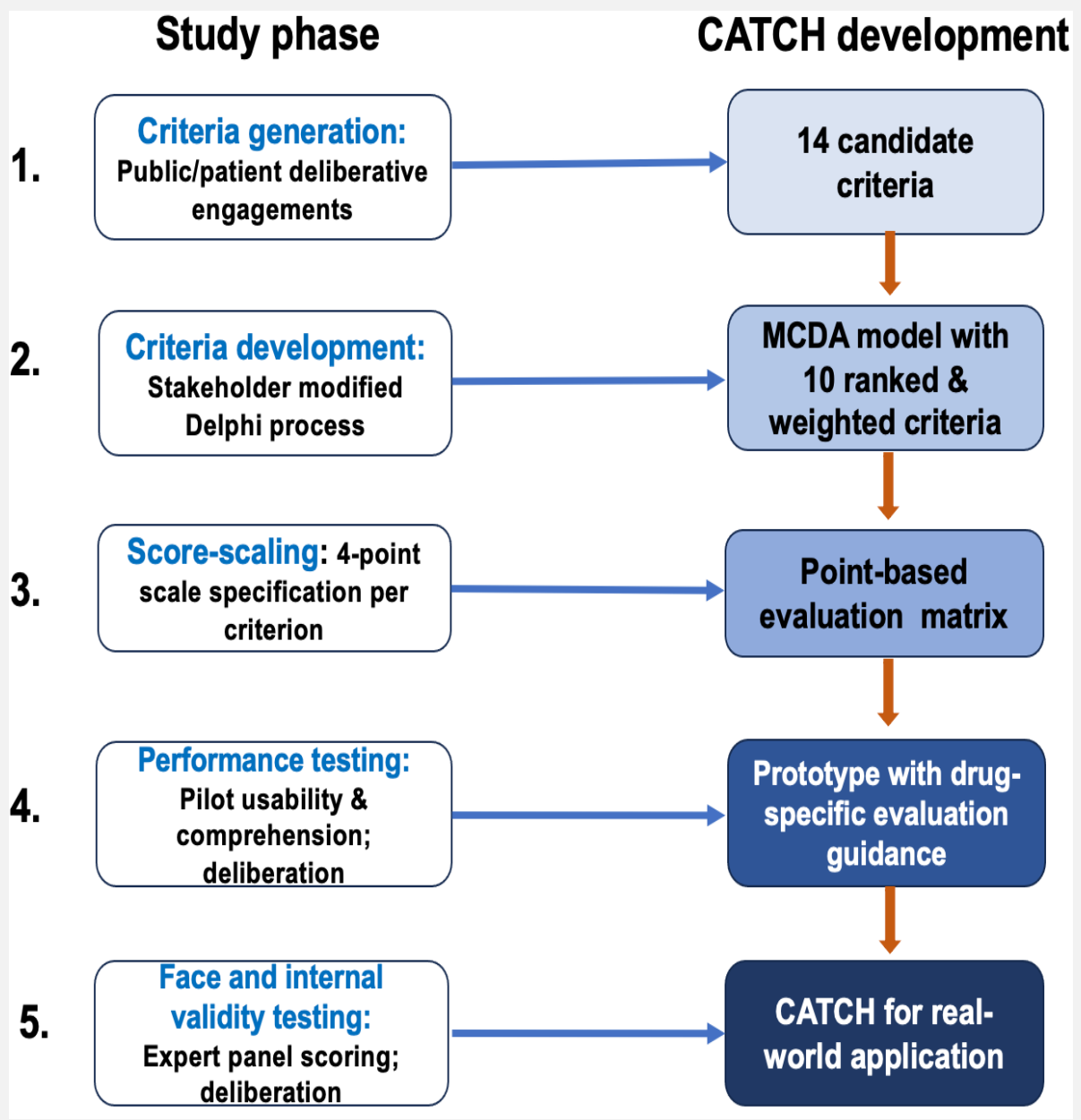
METHODS

We constructed an additive, rank-based MCDA model using qualitative (deliberation, survey opinions, consensus-building) and quantitative (ranking, Likert-scoring) inputs from deliberate engagements and a modified Delphi process.

Calculated criteria weights were based on proportional representation of importance and other inputs, and validated against the Simple Multi-Attribute Rating Technique Exploiting Ranks (SMARTER) technique.

To complete the *Comprehensive Assessment of Technologies for Child Health (CATCH) Framework*, we developed a 4-point scoring rubric. We mapped aggregate scores to funding priority levels and related funding recommendations to each level.

We pilot-tested and validated CATCH with clinicians and pediatric oncologists and performed sensitivity analysis.



RESULTS

Phase 1: 4 panels of citizens (n=45) with one purposive group of youths/young adults (16-22 yrs.) yielded 14 candidate criteria.

Phase 2: The modified Delphi process (two surveys and a deliberation) with HTA stakeholders (n=23) yielded 10 refined and reduced criteria and corresponding rank-based weights.

Phase 3: Scoring rubric with criteria-pertinent score scale. Total score out of 300.*

Phase 4: Overall, there was agreement between three reviewers. Four drugs were scored as “high priority” or “priority” for funding by all reviewers (dinutuximab, larotrectinib, blinatumomab, dabrafenib).

Reviewers felt applying CATCH to a wide variety of drugs allowed them to test its generalizability, but suggested more extensive supporting evidence summaries.

Phase 5: 10 experts in pediatric oncology validated CATCH for brentuximab vedotin (high-risk Hodgkin’s lymphoma) and blinatumomab (relapsed B-cell ALL).

Panelists thought CATCH captured important health and non-health attributes that would impact decision-making for children and youth in a meaningful manner.

In sensitivity analysis, there were no changes in funding priority levels when weights were varied for child-specific criteria.

Final ten criteria and weights

Effectiveness	19	Equity	10
Child-Specific Health-Related Quality-of-Life	18	Family Impacts	9
Disease Severity	17	Childhood Development	3
Unmet Need	11	Rarity	2
Therapeutic Safety	10	Fair Innings	1

SCORE

CRITERION	DEFINITION	WEIGHT	0 (No improvement)	1 (Minimal improvement)	2 (Moderate improvement)	3 (Significant improvement)
Therapeutic Safety	The absence of acute and/or long-term safety concerns or adverse effects (AEs) of a treatment, compared to existing treatments or standards of care.	10	Additive therapies			
			Increased burden of Grade 3-4 treatment-related AEs compared to existing standards of care	Increased burden of Grade 1-2 treatment-related AEs compared to existing standards of care	No increased burden of Grade 3-4 treatment-related AEs compared to existing standards of care	No increased burden of treatment-related AEs compared to existing standards of care
			Substitutive therapies			
			Increased burden of treatment-related AEs compared to existing standards of care	No increased burden of treatment-related AEs compared to existing standards of care	Reduction in Grade 1-2 AEs compared to existing standards of care, no increase in Grade 3-4 AEs	Reduction in Grade 3-4 treatment-related AEs compared to existing standards of care

* Total score (value function) = $\sum_{i=1}^n X_i Y_i$ Where X_i =individual criterion weight, Y_i =criterion-specific score, n=number of criteria.

Total Score, Funding Priority Category

DRUG	Reviewer 1	Reviewer 2	Reviewer 3	Median
Dinutuximab	158 Priority	238 High priority	199 Priority	199
Larotrectinib	213 High priority	175 Priority	172 Priority	175
Blinatumomab	210 High priority	217 High priority	225 High priority	217
Nelarabine	103 Potential value	203 High priority	128 Potential value	128
Brentuximab	125 Potential value	201 High priority	150 Potential value	150
Dabrafenib	150 Potential value	212 High priority	160 Priority	160
Pembrolizumab	119 Potential value	185 Priority	103 Potential value	119
Crizotinib	170 Priority	224 High priority	119 Potential value	170
Gemtuzumab ozogamicin	148 Potential value	133 Potential value	88 Low priority	133

	Base Scenario			Scenario 1			Scenario 2			Scenario 3		
CATCH Criterion	Wt	BV	Blina	Wt	BV	Blina	Wt	BV	Blina	Wt	BV	Blina
Effectiveness	19	3	3	19	3	3	25	3	3	10	3	3
Child-specific Health-related QoL	18	1	2	18	1	2	24	1	2	10	1	2
Disease Severity	17	1	3	17	1	3	23	1	3	10	1	3
Unmet Need	11	1	1	11	1	1	15	1	1	10	1	1
Therapeutic Safety	10	1	3	10	1	3	13	1	3	10	1	3
Equity	10	0	0	5	0	0	0	0	0	10	0	0
Family Impacts	9	1	2	5	1	2	0	1	2	10	1	2
Life-course Development	3	2	2	5	2	2	0	2	2	10	2	2
Rarity	2	3	3	5	3	3	0	3	3	10	3	3
Fair Share of Life	1	2	3	5	2	3	0	2	3	10	2	3
Total Weight/Score	100	136	218	100	153	235	100	151	247	100	150	220

Wt = Weight; BV = Brentuximab vedotin; Blina = Blinatumomab

Note: Reweighting of criteria in Scenarios 1 and 2 based on relative proportion of weight in original weighting.

We sincerely thank:



Contacts: avram.denburg@sickkids.ca
cindy.gauvreau@sickkids.ca

CONCLUSIONS

The CATCH framework is a societally responsive, transparent, and coherent value assessment framework, child-tailored to inform evaluation of child health technologies.

CATCH includes novel child-specific value assessment criteria founded on societal preferences, which reflect the importance of family impacts and long-term flourishing of children beyond immediate health gains obtained through treatment.

CATCH could improve the relevance of HTA decision-making for children in Canada and comparable health systems.