Panel Summary

Accessing Precision Therapies for Children and Youth in Canada

25 June 2021

McMaster University | HEALTH FORUM

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McMaster Health Forum

The McMaster Health Forum's goal is to generate action on the pressing health-system issues of our time, based on the best available research evidence and systematically elicited citizen values and stakeholder insights. We aim to strengthen health systems – locally, nationally, and internationally – and get the right programs, services and drugs to the people who need them.

The Hospital for Sick Children Research Institute, Child Health Evaluative Sciences

The Hospital for Sick Children (SickKids) Research Institute, affiliated with the University of Toronto, is Canada's largest, hospital-based child-health research institute dedicated to improving the health of children. Its research programs range from basic discovery research to clinical care and health policy, supported by state-of-the-art expertise, technologies and facilities. Child Health Evaluative Sciences (CHES) is the Research Institute's largest research program. Its multidisciplinary researchers seek to conduct cutting-edge research and translate their findings into clinical practices, systems and policies to improve the health and well-being of children and their families in Canada and around the world.

About citizen panels

A citizen panel is an innovative way to seek public input on high-priority issues. Each panel brings together 14-16 citizens from all walks of life. Panel members share their ideas and experiences on an issue, and learn from research evidence and from the views of others. A citizen panel can be used to elicit the values that citizens feel should inform future decisions about an issue, as well as to reveal new understandings about an issue and spark insights about how it should be addressed.

About this summary

On the 23rd and 30th of April, the 7th of May, and the 25th of June 2021, the McMaster Health Forum convened citizen panels on accessing precision therapies for children and youth in Canada. This summary highlights the views and experiences of panel participants about:

- the underlying problem;
- three possible elements of an approach to addressing the problem; and
- potential barriers and facilitators to implement these elements.

The citizen panel did not aim for consensus. However, the summary describes areas of common ground and differences of opinions among participants and (where possible) identifies the values underlying different positions.

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Summary of the panels

Four citizen panels were convened virtually between April and June 2021 to elicit public views on accessing precision therapies for children and youth in Canada. Two panels were composed of a representative sample of Canadians. A third was composed of youths aged 16-21, an under-represented group in policy-oriented deliberations. The fourth was composed entirely of caregivers of children with chronic and complex illnesses. Participants first discussed the problem of accessing precision therapies for children and youth. They identified predominant challenges related to: 1) scarcity of child-specific evidence for precision therapies; 2) inadequate awareness of regulatory and funding processes; 3) inequities in access; and 4) limitations in current health technology assessment (HTA) frameworks do not specifically account for unique dimensions of child health and illness.

In discussing potential solutions, participants considered three elements: 1) reform of federal drug regulations and policies; 2) development of an HTA framework tailored to child health; and 3) improvement of generation, collection and management of information about precision therapies for children and youth. In discussing the first element, participants felt that Health Canada should take legislative steps similar to those taken by the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) to incentivize or compel pharmaceutical companies to generate and submit more clinical trial data for pediatric precision therapies. A larger role for the federal government in financing access to pediatric precision therapy policies was also strongly suggested.

Most of the deliberations focused on element 2. There was consensus among participants in each of the panels that existing HTA frameworks should and could be revised to incorporate intrinsic, commonly held societal values attached to children and childhood, to better reflect child and family perspectives on and experiences of illness. Participants articulated views captured by four broad themes: 1) childhood distinction (related to 'fair innings'; future potential; vulnerability; and financial and non-financial impacts on families); 2) voices of citizens and of children and youth (with concurrence for inputs of the public, but dissonance for involvement of general citizens versus those with lived experience and for evolving capacities of children) ; 3) "the one versus the many" (individual versus societal benefits); and 4) health system context (restructuring of federal/provincial jurisdictions over health; funding arrangements).

With regard to element 3, discussion was centered on the generation of information. Participants suggested expanding federal funding or clinical studies and strengthening communications between stakeholders with various forms of evidence. A collaborative approach, at international, inter-provincial, and family levels, was at the core of participant discussion.

Participants pointed to implementation barriers caused by complexities and a lack of transparency in the processes of drug approval for marketing and regulation and for evaluating drugs for funding recommendations. Concerning facilitators, participants noted the expanding role of the federal government in creating policies for rare diseases and for child health. The inclusion of the public's voice, whether with or without lived experience, and including youth voices, was considered important to increasing access to precision therapies for children and youth.

About the panels

Four citizen panels were convened virtually between April and June 2021 to elicit public views on accessing precision therapies for children and youth in Canada. Two panels were composed of diverse and broadly representative samples of the Canadian public, a third panel was made up of youth aged 16 to 21, and the fourth comprised caregivers of children with complex and/or chronic illness (Box 2). The third and fourth panels were deliberately composed to account for youth perspectives being underrepresented as inputs to policymaking, and for caregiver perspectives to allow comparing their views and opinions with those of Canadians without any lived experience of the topic at hand. Panel participants were given orientation materials in advance in the form of a plain-language citizen brief, which highlighted what is known about the problem of accessing precision therapies for children and youth and its causes, options for addressing it and implementation considerations. They also drew upon recommended online educational resources about precisions therapies. At the beginning of the deliberations, participants viewed a video with a personal testimonial about the personal and familial impacts of caring for a child in need of precision therapy and the experience of trying to access it.

Box 1: Key features of the citizen panel

The citizen panel about improving access to precision therapies for children and youth had the following 11 features:

- it addressed a high-priority issue in Canada;
- it provided an opportunity to discuss different features of the problem;
- it provided an opportunity to discuss three options for addressing the problem;
- it provided an opportunity to discuss key implementation considerations (e.g., barriers);
- it provided an opportunity to talk about who might do what differently;
- it was informed by a pre-circulated, plain-language brief;
- it involved a facilitator to assist with the discussions;
- it brought together citizens affected by the problem or by future decisions related to the problem;
- it aimed for fair representation among the diversity of citizens involved in or affected by the problem;
- it aimed for open and frank discussions that will preserve the anonymity of participants; and
- it aimed to find both common ground and differences of opinions.

"She has a rare mutation even within the realm of cystic fibrosis, so precision medicine is important for us…We're still fighting for access for a drug that my daughter should be able to take that was Health Canada-approved back in 2012." (Caregiver)

Discussing the problem:

What are the most important challenges to improving access to precision therapies for children and youth in Canada?

In discussing the problem, participants articulated four challenges that were seen to significantly impact access to precision therapies for children and youth in Canada:

- scarcity of child-specific evidence for precision therapies;
- inadequate awareness of regulatory and funding processes;
- inequities in access; and
- limitations in current health technology assessment (HTA) frameworks do not specifically account for unique dimensions of child health and illness.

We review each of these challenges in detail below.

Scarcity of child-specific evidence for precision therapies

Most participants were unaware that the implication of a scarcity in child-specific evidence for precision therapies (specifically, from limited pediatric clinical trials) is few submissions of new pediatric therapies for Health Canada approval. Participants discussed the uncertainty about the effectiveness of novel therapies for children and youth. In addition, they emphasized the importance of the challenges that it creates for informing decision-making about public funding of the few precision therapies submitted and approved for marketing in children. For example, one caregiver asserted, "…absolutely we have a limited population in this country and it's a definite concern that we can't get enough children enrolled in these trials to get results that are robust enough for a viable answer."

Participants also linked their perceptions of large profits in the pharmaceutical industry to the need for corporate responsibility to redistribute earnings for more research targeted at children. One participant noted that "… there's lots of big companies making multimillions, hundreds of millions, billions of dollars surely can afford and I think would give some of their money to some of these important things if they had any clue that this is an issue." (General public)

Box 2: Profile of panel participants

The citizen panel aimed for fair representation among the diversity of citizens, including those most likely to be affected by the problem. We provide below a brief profile of panel participants:

- **How many participants?**
  45
- **Where were they from?**
  British Columbia, Alberta, Saskatchewan, Manitoba, Ontario, Quebec, New Brunswick, Nova Scotia, Prince Edward Island
- **How old were they?**
  Aged 16-21 (14), 22-34 (7), 35-49 (14), 50-64 (9); 65 and older (1)
- **Were they men, or women?**
  Men (13); Women (31); Prefer not to disclose (1)
- **What was the educational level of participants?**
  2% completed elementary school, 20% completed high school, 18% completed community college, 11% completed technical school and 49% completed a bachelor's degree/post-graduate training or professional degree
- **What was the work status of participants?** 7% self-employed, 38% working full-time, 7% working part-time, 9% unemployed, 7% retired, 27% students, and 7% homemakers
- **What was the income level of participants?**
  13% earned less than $20,000, 18% between $20,000 and $40,000, 11% between $40,000 and $60,000, 18% between $60,000 and $80,000, 22% more than $80,000, and 18% prefer not to answer
- **How were they recruited?** Selected based on explicit criteria from the AskingCanadians™ panel; social media advertisement; referral from clinical and patient networks

Some participants pointed out that the market disincentives for conducting clinical trials in Canada may not be easily overcome with a 'carrot-and-stick' approach by Health Canada given that such incentives are more effective in larger markets such as in the U.S. and Europe. One participant shared that, "…companies want to make more money, so they're going to go for the treatment that works for more people." (General public)

Participants also reflected on and debated about patient-level challenges to broader clinical trial enrolment among children. This included personal and sociocultural reticence to subject children to experimental therapies. One panellist questioned whether parents would want to put their child through the demands of a clinical trial, particularly with inherent uncertainty about therapeutic benefits. Conversely, a caregiver on the same panel emphasized that participating in a clinical trial was an important way to access drugs not yet approved for marketing in Canada and often represented an opportunity for hope in circumstances otherwise lacking in it.

Inadequate awareness of regulatory and funding processes

Most panellists were only partially aware of the sequencing of events and relationships between marketing approval, funding recommendation, coordinated pricing negotiation, and coverage decisions for a new drug to be included in provincial drug formularies. In particular, many were unclear about the two separate but interrelated processes and criteria for regulatory and HTA evaluation by Health Canada and the Canadian Agency for Drugs and Technologies in Health (CADTH), respectively. Some caregivers with prior knowledge of these institutions and processes found the system inadequately transparent. One caregiver said, that "I think all of this – health technology assessments, the CADTH involvement, Health Canada's review of all these processes and drugs, et cetera – is shrouded in a lot of mystery." Additionally, distinguishing the function and respective roles of HTA bodies at the provincial level, including the unique purview of INESSS in Quebec, as separate from CADTH's role at the national level, was a challenge for participants to fully understand. All participants agreed that greater transparency and enhanced public understanding of the regulatory and funding processes that govern pharmaceutical policy in Canada is fundamental to optimizing access to precision therapies for children and youth.

Inequities in access

Participants extensively discussed geographic and socioeconomic inequities in access to precision therapies for children and youth. They consistently and strongly argued that the principle of accessible healthcare, as embodied in the Canada Health Act, is a pillar of Canada's health systems and a core value of Canadians. They also emphasized the importance of equal access to care regardless of geography (both intra- and inter-provincial) or ability to pay. As one participant expressed, "Canadians value that we're equal across the country, …it shouldn't be because of your postal code that…your child gets access to a drug." (Caregiver) Another argued, "If there is treatment available in Canada and approved by Health Canada, you should be able to access it very easily – and that's not the case." (General Public).

The personal and familial financial burdens often associated with access to precision therapies were highlighted by participants as a key source of inequity. Many examples of financial burdens were identified by participants, including travel-related costs, the significant costs associated with purchase and administration of precision therapies, and indirect costs stemming from lost economic productivity for families. One caregiver made several appeals for provincial government support to travel to access CAR-T cell therapy, noting that "…parents shouldn't have to petition their local government to be able to access [treatments] from Alberta to Toronto." In addition, the unequal ability for caregivers to take time away from work to provide intensive care for their children was highlighted as a common but frequently overlooked source of inequity. Multiple caregivers felt they needed to quit their jobs, either temporarily or permanently, to care for their children.

Less medical literacy and health system knowledge, and consequently less ability to advocate for their children, were also acknowledged by participants as sources of inequity, especially for parents whose first language is not English or French. Participants noted that the complexities and "mystery" surrounding the processes of clinical trials, regulatory approval, and assessments for funding decisions contributed to these inequities. A caregiver asserted that "I just don't feel like patients should have to be fighting and trying to figure out this monolith of a system and learning like CADTH, PCPA, and all these different systems and having to navigate them and get a politician interested in you in order for your child to get life-saving drugs."

Overall, inequities founded on geographic, linguistic, educational, and socioeconomic disparities were seen as a principal yet remediable barrier to fair and sustained access to precision therapies for children and youth in Canada.

Limitations in current HTA frameworks do not specifically account for unique dimensions of child health and illness

Critical examination of the values underlying core criteria for HTA assessment of new precision therapies for public funding occupied a central focus of the panels. At the heart of participant deliberations on values was a foundational and widely held acknowledgement that child and family perspectives are inadequately incorporated into current HTA frameworks and processes.

Effectiveness

Among established criteria that inform value assessments of health technologies in Canada, participants agreed that therapeutic effectiveness should continue to occupy a dominant position. However, several participants felt that current approaches to measuring effectiveness to adjudicate the worth of precision therapies for children are often inadequate or incomplete. In particular, some suggested that evaluation processes are poorly calibrated to the evolving disease dynamics and treatment paradigms in an era of precision medicine. One caregiver pointed to the impact that relatively small improvements in disease-specific outcomes can have on daily functioning and overall quality of life. This participant noted that HTA review resulted in "a negative recommendation because it only improved lung function by 3 to 5%. So that's what it said on paper, but I've been trying to explain to them that maybe 3 to 5% on paper doesn't look like much, but to me that's my child going to a full year of school…not having to miss you know, 50 something days of school that he had missed previously." Others noted the need for greater focus on alternative measures of value – notably, family impacts – in the face of less certain evidence of effectiveness: "…if there's not enough trials to show how effective or not effective a specific therapy is, I think then there is more…priority given to…impacts on family and…having it be their choice, whether they are willing to take the risk to spend that much money or have the government spend that much money regardless if it is effective or not." (Youth)

Safety

Similarly, while many participants thought that therapeutic safety should remain a paramount value of HTA, a number noted greater need to situate safety considerations in the context of other values – such as disease severity, hope, and risk tolerance – informed by patient and family preferences. For example, some highlighted that an emphasis on the adverse effects of a given therapy might not adequately account for the degrees of risk that families might be willing to take in the absence of other options to treat rare and often severe or debilitating diseases. They recognized, however, that while potentially improving disease-specific or survival outcomes, the adverse effects of a drug could compromise the quality of life of a child. One participant thought nevertheless, "….that doesn't mean…we shouldn't invest in it just because we get an extended longevity of a poor quality of life. I think it would matter more on the impact to the individual and their family at that point. …at that point it becomes kind of like a trade-off that each individual would have to make." (General public)

Cost

While participants thought that the financial cost and affordability of precision therapies is important, they also focused on other costs from both health system and family points of view. Many emphasized that the opportunity cost of adopting any high-cost drug meant forgoing or replacing other drugs or activities in the health system. A number of caregivers emphasized that potential averted costs for families are not wholly considered, such as when time off work to provide intensive caregiving is avoided. One caregiver shared, "I have two degrees and I haven't worked since…[the two children's] diagnosis because it's a lot of work. So that's lost income for our family and it's also lost tax dollars…." Some participants also thought that non-economic costs like lost schooling or degraded quality of life need to be included in assessments. "I think all those costs, they separate them all out, but they all need to be looked at when you're looking at HTA for children." (Caregiver) Cost-effectiveness was only implicitly considered by participants as they weighed costs versus benefits. However, the concept of a cost-effectiveness threshold was clearly intolerable to one caregiver, who expressed, that "…this idea that you can put a price on my child's life, like my daughter is only worth… $100,000 a year…I think any parent that you know just hurts you inside."

"…the more I read…the content and the [citizen] brief it just became really apparent that this needs some attention."

Discussing the elements:

How can we address the problem?

In addition to discussing the challenges, participants were invited to reflect on the following three elements of a potentially comprehensive approach to improve access to precision therapies for children and youth in Canada:

1) reform federal drug regulations to address key challenges in access to precision therapies for children and youth;
2) develop a health technology assessment framework that is tailored to child health; and
3) improve generation, collection and management of information about precision therapies for children and youth.

The three elements could be pursued together or in sequence. A description of these elements, along with a summary of the research evidence about them, was provided to participants in the citizen brief that was circulated before each panel.

Most of the deliberations focused on the second element (develop a health technology assessment framework that is tailored to child health), as it was viewed by many as the one that could be most directly informed by their values and preferences.

Element 1 – Reform federal drug regulations and policies to address key challenges in access to precision therapies for children and youth

Box 3: Key Messages About Element 1

What are the views of participants regarding this element?

- Child-specific regulatory incentives and penalties are reasonable and necessary
- Stakeholders should engage industry innovatively
- Create a federal government model of delivery specific to precision therapies
- Integrate family voice in regulatory process

There was a consensus among participants that the federal government should take legislative steps similar to those taken by the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) to incentivize or compel pharmaceutical companies to generate and submit more clinical trial data for pediatric precision therapies to the federal regulatory body, Health Canada. Health Canada's continuing collaboration with other stringent regulatory authorities internationally, including the U.S. FDA and EMA, was also highly encouraged.

One of the themes that emerged in this discussion was innovative ways to engage with the pharmaceutical industry to increase its investment into clinical trials. One participant suggested that they "…would not be against compelling drug companies in Canada to say if you want to produce and distribute medication for let's say cold and flu or something else then you have to invest about…let's say 2% or 5% of your resources as a company into doing research for children and youth in Canada who do have rare diseases….". (Caregiver) Another participant highlighted the potential role for innovative financing mechanisms to create greater incentives for rare-disease drug development, noting that "…the Cystic Fibrosis Foundation in the United States, they invested instead of into traditional research, they invested into the drug company that eventually developed these drugs that we're talking about, …the modulators, and they get a royalty on that drug."

One participant advocated for the inclusion of patients and families from the earliest stages of drug development research. The shared that "…it even trickles down to how these drug companies are designing their trials because at the end of the day they are the ones submitting the evidence and if they're not capturing everything that is valuable to assessing

these drugs then there is a problem right there from the get go. So yeah, maybe [having families] helping design something to help capture more evidence."

Another key theme from the deliberations about this element was the view that the federal government should take a larger role in financing access to precision therapies for children and youth. Participants suggested shifting from a provincially-centered model for funding precision paediatric therapies to a federal model to ensure more equal access to novel agents for rare diseases throughout the country. Others suggested dedicated federal funding could help offset differential costs borne by patients in the process of gaining access to novel therapies. Examples of such costs that were identified included travel to specialist centres in-province or to other jurisdictions for clinical trials or treatment, or out-of-pocket costs attached to purchasing off-formulary agents off-trial. In the words of one participant, "…if we're not able as a society…to set up something federally that will cover all provinces, if we can instead perhaps expand our funding or insurance for folks who have to go internationally, who have to go transborder or even if we have to go to another province to seek treatment…".

Element 2 – Develop a health technology assessment framework that is tailored to child health

This element was the central focus of panel deliberations, which comprised querying, weighing and debating inclusion or exclusion of values important to a child-tailored HTA framework, including for precision therapies for children and youth. Participants were asked to reflect on a pre-defined set of twelve values outlined in the citizen brief. The set comprised: effectiveness, safety, disease severity, unmet need, future potential, costs, impacts on families, rarity, equity, vulnerability, child and youth views, and citizens' values and preferences. These values reflected both established HTA criteria and additional values of potential relevance to child health and illness that were derived from prior research focused on social and ethical values important to evaluating health

Box 4: Key messages about Element 2

Participants identified four central themes that distinguish technology appraisal for child and youth precision therapies:

- Childhood distinction
- Voices of citizens and children and youth
- "One versus the many"
- Health systems context

technologies for children. In an accompanying online exercise before and after deliberations in each panel, participants were asked to rank the values in order of importance for guiding HTA for pediatric precision therapies. The values and their rankings guided prompts for discussion during deliberations to generate informed opinions.

The deliberations yielded four key themes that distinguish technology appraisal for child and youth precision therapies: 1) childhood distinction; 2) voices of citizens and children and youth; 3) "the one versus the many" – individual versus societal benefits; and 4) health-system context.

Childhood distinction

Participants focused on several inter-linked ideas that set children apart from adult or geriatric populations and which ultimately justified a child-tailored HTA framework, including 'fair innings', future potential, vulnerability, and financial and non-financial impacts on families.

Barring one dissenting view for equal treatment across all ages, the consensus among panellists was that children have not yet experienced the significant life events that come later in life and should be given a chance to do so. As one participated shared, "as much as …you love your grandmother, they've already lived up to their potential…rather than someone who is 10 years old…". In addition, they argued that developmental harms imposed by disease in early years – including physical, psychosocial and cognitive harms – create long-term repercussions for health and well-being that justify prioritized allocation of scarce healthcare resources to children and youth. Participants related this directly to the future potential of children, in both sociocultural and economic terms, a key distinguishing feature from adults. The notion of the potential of children and youth to contribute positively to society was frequently expressed. For example, one participant asked the group to "…think of all the value added to that person, their family and potentially society as a result of letting that child live a long full life." (General public) Participants also discussed how future potential coalesced with the idea of hope, both for the child and for advances in precision medicine. It was viewed that adding an initial handful of years to a child's life could open up more therapeutic opportunities, insofar as further advances in precision medicine may have taken place.

Participants articulated a multi-faceted conception of vulnerability that distinguishes children from adults, and which they felt warrants consideration in HTA evaluation. To this point, one participant noted: "I think society…seems to view children as being more

fragile…kind of with how like children first in a lot of rescue stuff, I don't know (sic) in general with society we kind of try to prioritize children." (Youth) However, participants were uncertain about how vulnerability should be defined or measured and, thus, weighed against other values. The lack of child voices, or dependence on proxy voices, in most policy decisions concerning children contributed to a conception of vulnerability among participants and was connected to a broader theme about greater inclusion of the voices of citizens of all ages, which is discussed below.

Participants also uniformly and forcefully emphasized that sick children are distinct from adults in their near exclusive reliance on parents for providing care, enabling treatment, and sustaining advocacy. Many felt that family impacts of both disease and therapy should be better incorporated into HTA for paediatric precision therapies. One stated, that "…one thing I think that's…kind of unequivocal is the impact on family". Another participant asked, "[a rare disease] doesn't affect a lot of people, but what about all the people around that person?" Family stress was also often brought up by participants, as one sharing that "monetary concerns definitely add an undue stress to those who cannot afford to add more stress to their life". A caregiver related that, "…before my daughter was on the modulator, there was two years where she had a really rough time, sometimes we were at clinic every week and you see five or six different practitioners every time."

Voices of citizens and children and youth

Participants concurred that the voices of all citizens were needed in an HTA framework, based on shared values as Canadians in a universal healthcare system and at the most basic level, as taxpayers. One participant affirmed that, "I'm a firm believer that…getting input from society on these important issues is critical." Despite this belief about general engagement in HTA processes, however, some participants who were not caregivers felt that they themselves were not adequately qualified to provide their own voice. One participant who expressed this perspective shared that "…if you don't have children or you don't know anybody who has this disease, rare or otherwise, then the value of, or the perceived value of, investing in treatment for that at the expense…[of] something else…might be hard to get your head around. …I think as a [member of the] general population, if you don't understand the severity, the suffering, …the non-financial costs to family of dealing with these diseases and the outcomes and the treatment, it's hard to, you know, prioritize these…when you're weighing other possible considerations." (General public)

There was general agreement that voices of those who had lived experience would be especially valuable for inclusion, but those of children who had lived experience was debated. A number of participants thought children's evolving capacity made it problematic to interpret and incorporate their voices in medical decisions and health policymaking. While some participants pointed to children's lack of cognitive grasp of their condition or of long-term implications of illness or treatment, others countered that even at young ages some children were aware, informed and could valuably contribute to the HTA process for paediatric drugs. For example, one participant shared, "So I don't think children… depending on the age too, they don't have that good of understanding as to what should and shouldn't be done. And that's why parents have to step in and make a decision for the kids". (General public) In contrast, a caregiver argued that they thought "…children and youth understand a lot more than necessarily given credit for. In my own case, my son understood very well what drugs' names were and what they did and how they affected him and he was under the age of 10 years old."

This dissent between general public and youth participants and caregivers over the capacity of the child to speak was among the largest demonstrated during deliberations. The dissent was also evident in the pre- and post-deliberation ranking of HTA values. General public and youth participants ranked the value of children and youth views of low importance for HTA evaluation, and one participant in referring to the ranking expressed, "honestly, I feel like the child and youth views can probably be taken off [the list of values] completely…" (Youth) However, caregivers not only ranked it highly in the pre-deliberation poll, third after effectiveness and safety, but even raised it to second spot after the deliberation.

One versus the many – individual versus societal benefits

Opposing perspectives from humanitarian and utilitarian perspectives coloured discussion on how values should be adapted or added within the HTA framework. Humanitarian views led some to argue that neither cost nor medical need should be considered. In the extreme, participants who held this value argued that precision therapies should be funded for anyone who was sick, regardless of severity of disease, everywhere in Canada. One participant declared, "…funding should not have been…a deciding factor whether the child gets to live or not. That just makes me so mad." (General public) Another added, "…because a lot of these diseases are life or death…and I don't think in these discussions it's worth debating whether spending money on someone's life is worth it or not. …I believe that life is what is worth it more than anything else." (Youth) A few participants believed that age should not be considered for making decisions. For example, one stated that "even for the…elder person, eighty years old for example, it's for her own benefit as

well. She wants to see her grandchild. It's a joy for her life, for her spirit. I don't think we should give more weight to some, a group to another…we cannot just choose who to ignore. Every human life is important." (General public)

On the other hand, participants sympathetic to utilitarian views emphasized the importance of providing the greatest medical benefits for the most children, and to be mindful of opportunity costs. A participant expressed that "I know we…can't put a price on a life, but I think we should try to maybe focus on the therapies that will have a resolution for many people rather than one person." (General public) Another participant shared an approach to make the most of limited resources, saying "…treating someone who has more potential for ability and basically function…it would be worth…putting more resources into that individual rather than someone who does not have a good outlook based on what has been seen so far in treatment." (Youth) Rarity of disease prompted one participant to say, "this is a very, very small percentage of children who are affected by things that need precision therapy. …obviously kids should get help if it is available, but thinking of…that opportunity cost…I feel like…there's some things that are more prevalent than things that are in need of precision therapy and that should be considered…" (Youth)

Health system context

HTA evaluation takes into account the contexts in which a new drug will be introduced to ensure and optimize implementation feasibility, sustainability and fairness. Participants referred repeatedly to the need to re-structure jurisdictional responsibilities for healthcare in Canada to resolve unequal access between provinces, despite the constitutional challenge of doing so and despite Quebec's extraordinary status in managing its own affairs. One participant stated plainly that they "…think what we really need to do in Canada… is…start looking at a constitutional amendment to put more medical in the federal rather than provincial realm." (General public) Specific to the high cost of precision therapies for families, federal government-led funding mechanisms held appeal for many participants. Participants also pointed to streamlining the way precision therapies are accessed if a single entity were to coordinate all the bodies involved, including HTA bodies. Federal government assumption of more legislative and funding responsibilities for precision therapy also emerged in discussions to reforming regulations for marketing approval, all with a view to a more integrated process of regulation and HTA evaluation.

A participant also encouraged inter-provincial collaboration, suggesting "…provinces should work together to fund clinical trials for everyone in the country and not just those inside their jurisdiction." (General public) To address geographical access concerns,

participants thought that centres of excellence for pediatric precision therapies could be more numerous and more spread out across Canada. Complementarily, travel support could be consistently provided for children needing access to clinical trials or for approved and funded treatment.

In acknowledging the limits of the public purse given the high cost of precision drugs, several participants suggested patient/family out-of-pocket payment models such as co-pay, or payment scaled to income. To mitigate inequities of access connected with out-of-pocket payments a participant suggested that "…if we have to go to another province to seek treatment…I'm wondering if there's an opportunity perhaps for a governing body to sort of oversee funding for things that they can't sponsor within their home province to perhaps treat patients elsewhere." (General public)

Element 3 – Improve generation, collection and management of information about precision therapies for children and youth

There was limited focused discussion on this potential approach to improving access of precision therapies for children and youth given the focus of most in-depth deliberation on element 2. Moreover, in post-deliberation evaluations of the panels, participants thought this element was the least important of the three approaches and had the least opportunity to be implemented. Nevertheless, participants expressed support for this approach and offered several suggestions for the generation of information.

Box 5: Key messages about element 3

What are the views of participants regarding this element?

- Active federal and provincial funding for clinical trials to generate more effectiveness data
- A global and collaborative approach to data generation and sharing is needed

Participants concurred that more clinical trials on pediatric precision therapies were needed, and some participants supported an expanded federal role. One participant indicated that "this is where…I believe the federal government should step in and maybe take some of the responsibility…of funding some studies that would benefit all the provinces equally." (General public)

A collaborative approach, at international, inter-provincial, and family levels, was at the core of discussion. For example, one participant shared that what they "…found was that patients and their families are very willing to come forward and say, okay, this is a new disease that we don't know a lot about. Let's share as much information as we can in order to continue the development of research and to continue some treatment options." (Caregiver) Furthermore, it was felt that families could be involved even more fundamentally in evidence generation with one participant noting that "it even trickles down to how these drug companies are designing their trials because at the end of the day they are the ones submitting the evidence and if they're not capturing everything that is valuable to assessing these drugs then there is a problem right there from the get go. So yeah, maybe [having families] helping design something to help capture more evidence." (Caregiver)

"Generate more awareness in the public space about this issue - I would not have considered or thought twice about it before the panel because it is not front of mind in my life…"

Discussing implementation considerations: What are the potential barriers and facilitators to implementing these elements?

While discussing the three elements of a potentially comprehensive approach for improving access to precision therapies for children and youth, participants identified potential barriers and facilitators for moving forward.

Participants pointed to barriers caused by complexities and a lack of transparency in the processes of drug approval for marketing and regulation and for evaluating drugs for funding recommendations. Participants felt that there is lack of general public awareness even regarding the existence of the respective organizations. From experience, a caregiver expressed, "I think that Canadians need…[to have] an idea [of] what goes into all that so that they can have more of a voice whether they're participating in something like this or lobbying their MPs and Health Canada…." How decisions are actually made to adopt drugs into public funding seemed particularly difficult to understand or get information about. Advocates and caregivers also expressed the need for help navigating access to precision therapies. One caregiver disclosed that they "…only just learned a few days ago that patient

groups have an opportunity even to contribute to the CADTH reviews. …I didn't even know that… there was an opportunity." Asked for at least one important action to address the problems of access, one participant exhorted the need to "generate more awareness in the public space about this issue. I would not have considered or thought twice about it before the panel because it is not front of mind in my life situation." (General public)

Concerning facilitators to implementation of the elements, participants noted the expanding role of the federal government in creating policies for rare diseases and for child health. A proposed national pharmacare program was also viewed as a key potential opportunity for funding precision therapies for children and youth.

The strength and energy of the public's voice was also viewed as an important facilitator. One caregiver advanced advocating on behalf of other caregivers who did not have the financial resources, time, or medical literacy to do so. This participant stated that "…although I feel like I have the capabilities to advocate for my kids and I believe they will get these modulators…I want to be able to make it easier for other people. …So I want to help make the system better for the people are going to come after us." To many participants, public input, particularly from those with lived experience, could advance access all along the continuum of research to marketing approval to HTA evaluation. From the youth panel, optimism was expressed for young adults as facilitators with one participant having noted that they "…think it's really important to engage a lot of university students…specifically in the health field because they are the ones that are…going to be…actually implementing maybe some of these therapies and different strategies, …so I think looking at …[the] new opinions they could bring or like anything they would have to say is also really important."

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Conflict of interest

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