A REPORT OF THE ONTARIO CITIZENS’ COUNCIL

DRUGS FOR RARE DISEASES

Submitted to:

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EXECUTIVE SUMMARY

The Ontario Citizens’ Council met in November 2017 to revisit the particularly difficult topic of funding drugs for rare diseases, which was the subject of the Council’s first meeting held in January 2010.

Today, a rare disorder is defined as “a seriously debilitating or life-threatening condition that affects no more than 5 in 10,000 people.”¹ Health Canada’s draft definition uses similar numbers - “one that affects fewer than 1 in 2,000 persons in Canada.” Alberta’s public drug plan uses the definition of “a genetic lysosomal storage disorder that occurs at a frequency of less than 1 per 5,000 Canadians.” Ontario Public Drug Programs’ previous definition was an incidence rate of “fewer than 1 in 150,000 live births or new diagnoses per year”, and when the Council discussed this topic in 2010 Ontario’s definition was “1 case in 100,000”. The change in definition alone demonstrates the complex landscape and the inherent complications.

The Canadian Organization for Rare Disorders (CORD) states that there are around 7,000 rare diseases affecting as many as one in twelve Canadians, and consequently affecting their families, the healthcare and social systems, the workplace, the economy, and our collective social welfare. The Canadian Institute for Health Information (CIHI) reports that Canadians with drug costs of $10,000 or more represented 2% of beneficiaries but accounted for one-third of public drug spending in 2016.² Considering the potential impacts to our publicly funded health care, Council members felt it would be important to establish and use a consistent definition to help quantify the scope of the challenge and enable comparisons over time and across geographic jurisdictions.

The Citizens’ Council provides advice based on a set of values that have been compiled in a framework to ensure a consistent reference point in all of their deliberations. However, as values can overlap and conflict, they don’t always move in the same direction, resulting in differences in how values are applied to a particular issue. For example, should health benefits be maximized for the largest number of people or should the focus first be on the most vulnerable among us? Additionally, changes to the membership of the Council also contribute to the evolution of the Values Framework. In fact, this meeting saw several changes in membership as a number of long-standing members left the Council as their terms expired, and three new members joined.

Cost effectiveness was a value of real concern for the Council. Drugs for rare diseases are extremely expensive in part due to the small user population/market, but also due to the uniqueness of these drugs which have no competitive offerings. This generated much discussion about the need to cover costs on the grounds of compassion, counterbalanced by the concern that, in the future, as more personalized drugs emerge, it would result in increasingly higher costs for the limited public purse.

Council questioned whether drug cost is driven by the true cost of research, development and manufacturing, or is more aligned with what people are willing to pay and the profit expectations of a drug company’s shareholders. It was impossible not to be influenced by the recent example

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¹ Now is the Time: A Strategy for Rare Diseases is a Strategy for all Canadians, CORD Canadian Organization for Rare Disorders, May 2015, p.5
of the drug whose price skyrocketed overnight by 5,000% simply because a new company bought the rights to distribute it. Council members would welcome an opportunity to examine the issue of pharmaceutical pricing in a future session if that would be useful to the Ontario Public Drug Programs (OPDP).

Another component of cost that was of real concern was whether other parts of the drug programs that already meet the cost-effectiveness requirement and serve greater numbers of people, especially vulnerable populations, could be put in jeopardy by funding drugs for rare diseases. This was not deemed acceptable, nor was it acceptable to negatively impact funding for preventative medicines such as flu vaccine, or curative drugs which, though expensive, ultimately result in savings to the overall health budget if they eliminate the cost of long-term treatment.

Evidence-based decision making is an important value to Council, so members grappled with the notion that, in the case of drugs for rare diseases, patient recruitment for clinical trials is not possible on a scale sufficient to produce statistically significant data, making it impossible for expert committees to evaluate efficacy, safety or cost-effectiveness for these drugs based strictly on evidence.

The concept of “rarity” sparked an interesting discussion as to whether or not this is an important point of differentiation, or does this simply create a silo within the system? Beyond the evidence standard for drug approval, members felt that drugs for rare diseases should be treated, to the extent possible, in the same manner as other drugs. Simply put, if a drug is too expensive, it is too expensive for any disease, not just for a rare disease.

These are just some of the issues that provided fodder for very interesting conversation over the course of the meeting and this report presents the details of the process, the discussions and the outcomes of deliberations. In the 2010 Council meeting on drugs for rare diseases, members developed 16 values-based recommendations. In this session, members compared their findings with those of 2010, and discovered that those recommendations were largely reinforced. A chart of the 2010 findings and 2017 comparisons appears at the end of Section 4 “Results of Deliberation”, and the conclusion provides a summary of current recommendations and rationale.

We trust that this report will provide assistance to OPDP when considering policy development around this very sensitive issue of funding drugs for rare diseases.

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1.0 INTRODUCTION AND THE QUESTIONS

The *Patients First: Action Plan for Health Care* has strengthened the ministry’s commitment to increasing access to drugs while maintaining costs at an affordable level. A sustainable drug program is critical to the success of the ministry’s broader priorities for health system transformation.

The Ontario Public Drug Programs (OPDP), and public drug programs across Canada, continue to face challenges with regards to drugs for rare diseases. In its 11th meeting, held November 17-19, 2017 in Toronto, the Ontario Citizens’ Council was asked to deliberate on the topic of public funding of drugs for rare diseases. This topic was addressed by the Council in its first meeting, in January 2010 (see [2010 Report](#) and [Executive Officer Response](#)). The Executive Officer of the Ontario Public Drug Programs believes it is important to revisit this topic now.

The Ontario Citizens’ Council is composed of Ontarians from diverse backgrounds, appointed by the Lieutenant Governor in Council to serve as an advisory body to the Ministry of Health and Long-Term Care’s Ontario Public Drug Programs. They provide their views on the values that reflect the needs, culture, attitudes and expectations of all Ontario citizens about government drug policy and the health care system. Throughout meeting discussions, Council members are reminded that their advice must consider the good of the people of Ontario as a whole. The Council reports to the Executive Officer and Assistant Deputy Minister of the Ontario Public Drug Programs. The mandate of the Council is to provide values-based perspectives in response to specific topical questions. To this end, Council members have identified a number of values on which to base their advice and recommendations (see Appendix 3 - Values Framework).

This meeting represented an opportunity for the Council to discuss core values and considerations that could guide funding decisions relating to access to, and the sustainability of, the public drug programs for generations to come. Council members were asked to provide values-based input on the following key questions:

- *Given the increasing number of products, expectation for access, uncertainty of value and rising costs of drugs for rare diseases, what values should the Ontario government prioritize when public funding decisions are being made for drugs for rare diseases?*
- *Should there be a minimum evidence standard to assess drugs for rare diseases? How should the evidence base be balanced with other values?*
- *How have the views of the Council evolved since it first considered this topic in 2010?*

2.0 PREPARING FOR DELIBERATION

As is customary for Council meetings, preparing for deliberation involved a wide range of background reading. A binder of information was delivered to members in advance of the meeting and contained an overview of several presentations that would be given, the meeting agenda, and supporting documentation for the dialogue process. A key document was the Backgrounder: *Funding Drugs for Rare Diseases*, which explained what rare diseases are, the drug
review process, the current status and challenges in funding for rare disease drugs, as well as providing a brief overview of some of the strategies being used in other parts of the world to provide a regulatory framework for these drugs.

Other documents included:

- *Now is the Time: A Strategy for Rare Diseases is a Strategy for all Canadians.* Prepared by CORD (Canadian Organization for Rare Disorders) (May, 2017)

Preparation continued Friday evening with updates on the OPDP and then a presentation by Suzanne McGurn, Assistant Deputy Minister and Executive Officer, OPDP, to set the context for why a discussion on public funding of rare disease drugs was needed now. Sang Mi Lee, Senior Pharmacist with the Pan-Canadian Pharmaceutical Alliance provided background on what rare diseases are, examples of rare diseases, and challenges with treatment. Angie Wong, Director of the OPDP Policy & Strategy Branch, completed the evening’s presentations with an overview of the current drug approval process.

Saturday morning was dedicated to presentations, which provided valuable background information. Council members were given an opportunity to ask questions of the individual presenters, and at the end, the floor was opened to further questioning and discussion with all presenters. The list of presentations, given by experts in their respective fields, is provided below. They focused on the personal, pharmaceutical, ethical and economic challenges of funding drugs for rare diseases.

### TABLE 1: LIST OF SATURDAY PRESENTATIONS

<table>
<thead>
<tr>
<th>TITLE</th>
<th>PRESENTER(S)</th>
<th>PRESENTER TITLE</th>
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<tbody>
<tr>
<td>a. Living with Rare Disease – A Patient Perspective</td>
<td>Maureen Smith</td>
<td>Patient Member, Ontario’s Committee to Evaluate Drugs</td>
</tr>
<tr>
<td>b. Championing Rare Diseases – A Manufacturer’s Perspective</td>
<td>Lindsay Williams, Lena Fontaine and Ahmed El-Zoeiby</td>
<td>Head of Public Affairs &amp; Patient Advocacy; Government Affairs; Medical Advisor; All from Shire Canada</td>
</tr>
<tr>
<td>c. Challenges of Funding Drugs for Rare Diseases - A Bioethicist’s Perspective</td>
<td>Sally Bean</td>
<td>Director, Health Ethics Alliance &amp; Policy Advisor, Sunnybrook Health Sciences Centre</td>
</tr>
<tr>
<td>d. Funding Challenges – A Health Economist’s Perspective</td>
<td>Don Husereau</td>
<td>Adjunct Professor, Department of Epidemiology and Community Medicine, University of Ottawa</td>
</tr>
</tbody>
</table>
A Personal Perspective:

In “Living with a Rare Disease (My Journey)” Maureen Smith, a patient member of Ontario’s Committee to Evaluate Drugs, provided a personal account of her experience of living with a rare disease—from the challenges experienced in childhood through to those in adulthood in getting drugs for rare diseases funded. She emphasized that little to no research is being conducted on adults with rare disease since the focus is more on pediatric conditions.

A Manufacturer Perspective:

Lindsay Williams, Head of Public Affairs and Patient Advocacy and Ahmed El-Zoeiby, Medical Advisor, Shire Canada gave a presentation entitled “Championing Rare Diseases”. Also present was Lena Fontanne who works with government affairs in Ontario. Shire is a biotechnology company working in eight therapeutic areas, five of which are in rare diseases. The presentation touched on new scientific discoveries such as gene mapping and personalized medicines. They noted that over 50% of rare diseases begin in childhood, discussed the difficulties in running drug trials, particularly since less than thirty patients in Canada have some of these rare diseases, and stressed the importance of collaboration between industry, government and academia. Significant delay often occurs between disease onset and diagnosis, with many overlaps, which can result in being diagnosed as a multitude of other diseases—more than 25% of patients are misdiagnosed. Council was told about patient support programs sponsored by pharmaceutical companies, which help patients bridge the gap between Health Canada’s approval of the drug and subsequent government funding, which often takes two or more years. The topic of Health Technology Assessment (HTA) of Rare Disease Medicines was also discussed, and the need for a commitment to generate real world evidence, which means that patients are part of a study to collect data on their outcomes as they use a drug in their day-to-day lives.

A Bioethicist’s Perspective:

A presentation by Sally Bean, Director of the Health Ethics Alliance & Policy Advisor, Sunnybrook Health Sciences Centre focused on the ethical challenges of funding drugs for rare diseases. She discussed research, institution, and system ethics with emphasis on health law and policy. The overview of ethical theories focused on resource allocation issues and the ethical justification involved in decision making, including the concept of macro allocation decisions, which occur at the policy level – deciding how healthcare dollars should be spent, and the need to provide rationale or ethical justification, as well as consequence-based and duty-based ethics. An analysis of the Health Act was also provided during her presentation. The phrase “medically necessary” was examined to illustrate what services could be offered with public funds and how health inequity prevents many from getting treatment. The presentation concluded with a summary that “a range of lenses exists” when considering the complex resource allocation decisions pertaining to funding drugs for rare diseases.
A Health Economist’s Perspective:

The last presentation, via Skype, was made by Don Husereau. Don is a Senior Associate at the Institute of Health Economics in Edmonton Alberta and Adjunct Professor, Department of Epidemiology and Community Medicine at the University of Ottawa. He is also a Senior Scientist for the Institute for Public Health, Medical Decision Making and Health Technology Assessment UMIT, and serves on the review committee for Pan-Canadian Oncology to evaluate drugs.

Don’s presentation addressed the high price for drugs for rare diseases and whether they are justified; the premise that cost is what we are willing to give up to get something; and the opportunity exchange in any transaction (e.g. better health vs better education vs. better infrastructure). He suggested that if we are willing to pay a premium for drugs for rare diseases, the implication is that we are willing to give up health goals for other people. We could tax people more, but that’s also unfair to some and will cause suffering for people just getting by. He highlighted the need for clearly stated healthcare goals (e.g. health benefit, saving lives, increasing function, etc.) and the need for a standard definition of “rarity”. He suggested that funding for rare diseases can be individually beneficial yet societally harmful from an economics perspective that focuses on a cost/benefit analysis. The presentation proceeded to examine balancing health benefits (economic factors) with ethical factors such as equity and compassion and re-introduced the idea of Quality-Adjusted Life Years (QALYs). The presentation illustrated that the manufacturers of drugs for rare diseases have obligations to shareholders to maximize returns and that this factor often drives cost of a drug. He also posed the question of how we decide when the term “rare” comes into play since there is no standard definition of a rare disease, and questioned whether there should be a “price premium for rarity”. He drew from the results of Canadian surveys and stated, “The truth is, again and again, people don’t believe rarity, in and of itself, deserves a higher price”.

3.0 HOW THE COUNCIL DID ITS WORK

With full disclosure, the deliberative portions of the Council’s work started Saturday afternoon through to Sunday morning in the presence of Maureen Smith who had earlier presented to the Council her personal journey of living with a rare disease. This may have influenced members’ comfort level in speaking candidly.

In their discussions Council members considered the information and input provided by speakers, as well as the background materials provided to them by the Ontario Public Drug Programs (OPDP). The process began with a short opportunity-cost activity that asked members to judiciously spend a finite sum of money on a range of drug program activities as a way to determine, in context of other drug program expenditures, whether drugs for rare diseases would emerge as a priority.

The Council broke into two groups and with the guidance of facilitators, considered the dilemma that funding drugs for rare disease creates an opportunity-cost somewhere else in the system,
which may mean that other programs for patients with more common diseases may be denied services.4

Each Council Member received 5 dots worth $20 each to allocate to as many of the following options as they wished:5

- Increase cancer drug spending
- Provide diabetes test strips
- Cover essential drugs for all Ontarians
- Provide free flu shots
- Reduce deductible required for patients accessing drugs through the Trillium Drug Program
- Increase spending on drugs for rare diseases
- Maintain current drug coverage for seniors
- Expand funding for Hepatitis C curative drugs

Following that exercise, the facilitators tallied the results and led their respective groups in a discussion on the rationale behind their selections; why dots were put on some programs and not others, as well as which values informed those choices. The groups then separately discussed how these values apply to the question of public funding for drugs for rare diseases and worked towards common ground on the most important. Next, the groups focused on the question of whether there should be a minimum evidence standard to assess drugs for rare diseases, and what the advantages, disadvantages and trade-offs might be.

On Sunday, Council reconvened in its entirety and reviewed the discussions held by the two groups the previous afternoon to identify the tensions or areas of divergence amongst Council members, as well as common ground to solidify key messages in response to the three questions that had been posed by OPDP, and the values represented.

Council then compared their findings and advice developed over the course of this meeting with those of the meeting held in 2010, with the intent of identifying whether any views had evolved or changed or if new insights had emerged.

On Sunday afternoon the Council shared its conclusions with the Director of the OPDP Policy and Strategy Branch, who responded to residual comments and questions, after which the Council’s findings and advice were further nuanced and finalized.

4 Also see: Daniel A. Ollendorf, PhD, Rick Chapman PhD, Steven D. Pearson, MD, MSc., Institute for Clinical and Economic Review, Assessing the Effectiveness and Value of Drugs for Rare Conditions A Technical Brief for the ICER Orphan Drug Assessment & Pricing Summit May 2017, p 6
5 These options were chosen for the purposes of the exercise only. They are not in any way trade-offs being considered by the OPDP.
4.0 RESULTS OF DELIBERATION

On the Key Questions:

A. Given the increasing number of products, expectation for access, uncertainty of value and rising costs of drugs for rare diseases, what values should the Ontario government prioritize when decisions are being made about whether to pay for drugs for rare diseases?

Two values statements rose to the top as the Council members deliberated on the issue of public funding for drugs for rare diseases:

- Benefit the greatest number of people for the greatest good. – This was based on the notion of the public good, which includes, for the Council, the prudent use of available resources for the health benefit of the majority of Ontarians.6

- Be responsive to the most vulnerable. – Members felt, on compassionate grounds, that it was important for the drug programs to consider funding drugs for rare diseases. This also related to values of fairness and equity which, as expressed by the Council in previous reports, included the concept that to the greatest extent possible within budget, all citizens should be able to access the drugs they need.7

Members recognized that there was tension between these two value statements. There were, for example, competing views on who the most vulnerable are in society and the role the drug program should play in supporting them. In line with previous discussions of the Council, members put more emphasis on programs that assist those on reduced income who may not have any drug coverage and can’t afford even basic drugs. Maintaining existing drug coverage for seniors (another group that includes some vulnerable citizens) was also a high priority. Therefore, while members recognized the compassionate need to help fund drugs for rare diseases, they were clear that it should not be at the cost of existing programs that were also helping vulnerable populations.

As a starting premise, the following principle enunciated in the Values Framework resonated:

**Balance the common good with the needs of particular individuals**

The government has a mandate to serve all citizens, including those with special needs, but it must provide prudent management of available resources for the benefit of all.

Both the cost and the opportunity-cost of funding drugs for rare diseases were of real concern for Council members. From the initial exercise designed to kick-start the discussion of values,

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6 See the Values Framework in Appendix 3
7 See for example, the May 2017 report on Prescriber Responsibility
8 See the Council report: “Informing Development of a New Drug Program in Ontario” for more on this concept
where people selected a number of program options for increased spending, it was clear that funding drugs for rare disease was among the least popular of the options.

Other aspects of the public good that resonated for Council members more than funding drugs for rare diseases were curative drugs such as those for Hepatitis C. The reasoning behind this was that paying for curative drugs would result in savings to the overall health budget, as patients would not need to be treated for that disease long-term. Similarly there were strong arguments for preventative measures such as the flu vaccine. (For more on prevention, see below.) The value of efficiency captured in the Values Framework is relevant to these views:

**Efficiency** includes the notion of maximizing the results achieved with a minimum of wasted effort or time. It encompasses how well the system works in a cost effective manner, ensuring that taxpayers’ money is used well.

Cost-effectiveness is one concept that helps to express these views. An economic estimate often used in drug funding decisions to assess the cost-effectiveness of a drug is “Quality Adjusted Life Years” (or QALYs). This is a measure of the disease burden experienced by a patient, and considers an individual’s quality of life and the length of life following a particular treatment. The use of QALYs in the case of funding for rare diseases resonated for members. From the information provided to the Council, both by speakers and in the background readings, members understood that rare diseases are not cured by the currently available drugs, and that life expectancy is often quite low, even with drugs. In cases where the drugs do help prolong life, patients could still require ongoing drug coverage at a very high cost for the rest of their lives which, in the case of early diagnosis, could be 60+ years. In addition, the use of other areas of the health care system is increased, as ongoing care requires additional costs to manage the disease, e.g. regular visits to specialists or hospitals, psycho-social, and physical therapy, respite care etc. One speaker explained that funding drugs for a rare disease to provide one QALY for one patient at a cost of $700,000 meant foregoing six QALYs for other patients who require less expensive drugs. This was not considered by Council members to be a prudent trade-off.

While members were struck by the reality that children are disproportionately diagnosed with rare diseases, they cautioned against funding based solely on compassion and “rule of rescue” - 'the powerful human proclivity to rescue a single identified endangered life, regardless of cost, at the expense of any nameless faces who will therefore be denied health care'. However, all other things being equal, some members felt that the concept of fairness as the number of “innings” one gets in life should be a consideration. This could mean, for example, that a young child would take precedence over a 50 year-old if they require drugs that have similar cost-effectiveness and overall cost.

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9 See the Citizens’ Council report on QALYs and Drug Funding Decisions in Ontario for more on QALYs
Another aspect of cost efficiency that was a real stumbling block was the high cost of drugs for rare diseases. Within a limited budget, there was deep concern over setting up a program that in the future, as more personalized drugs emerge, might result in higher and higher costs for the public purse.

The Council heard a presentation from one pharmaceutical company that supplies drugs for rare diseases, and while members appreciated the patient support programs they provide, they were nevertheless skeptical of the high cost of these drugs. They tended to agree with the health economist who argued that costs are driven by what people are willing to pay and profit expectations of shareholders, rather than the true cost of research and manufacture. One member referred to the example of a drug called Daraprim,\textsuperscript{11} used by some AIDS and transplant patients, whose price skyrocketed overnight from $13.50 to $750 a pill, an increase of 5,000% simply because a new company bought the distribution rights. This example also highlights how difficult it is for governments to fight for better pricing and the burden put on patients who then need to apply for programs that can provide the drug to them based on income and need.

Concern was also raised that the situation of high costs may just increase as companies produce more biologics, which members understand are more difficult to produce generically (which would normally provide a price decrease).

While recognizing that it may be an uphill battle, members strongly urge the Ontario Public Drug Programs (OPDP) to:

\begin{verbatim}
Work with other provinces and territories to utilize procurement policies and collective procurement to reduce drug costs as possible.
\end{verbatim}

They were encouraged that the Patented Medicines Prices Review Board (PPRB) is currently reviewing pricing policies for Canada. Members would welcome an opportunity to look at the issue of pharmaceutical drug pricing in a future session if that is useful to the OPDP.

**Prevention and Awareness-Building:**

A consistent value for the Council, prevention, was seen as having application to the question of funding drugs for rare diseases because it is important both to prevent suffering (e.g. delay or prevent disease onset) and to achieve a public drug program that can be financially responsible and sustainable into the future.

In terms of rare diseases, there were several relevant aspects:

- The use of genetic testing, counselling and prenatal screening if there is a reason to feel there is higher likelihood of a rare disease for one's offspring. It is important this is not

meant to be testing and screening for all – instead its value is in those cases where a rare disease might be genetic in a particular family line. This at least would provide more information to prospective parents and allow them to make informed decisions.

- Building awareness among family doctors and other healthcare providers so that early diagnosis and treatment can occur. This could help prevent morbidity. If drug funding is not possible and/or drugs are not available for the condition, ensuring other levels of care are provided to reduce and/or manage symptoms and treatment options would be important.

- Access to information for patients and families. The Quebec hotline was provided as an example by one of the speakers. People can phone in and be helped with navigating the system in order to get a diagnosis. Another example given was telemedicine which can reduce the cost and inconvenience factor for those in regions where specialists may not be available. Members felt it was important that people have equal access to the possibility of early diagnosis and not be disadvantaged because of location.

- Members also raised the need to support cutting-edge advances in healthcare, for example, genetic replacement therapy that delivers a functional gene to replace a faulty, disease-causing one. While still expensive, the premise is that patients might only require a one-time dose of gene therapy for a long-term benefit or cure versus taking pills or receiving injections for a prolonged period of time.

B. Should there be a minimum evidence standard to assess drugs for rare diseases? How should the evidence base be balanced with other values?

The concept of a minimum evidence standard proved to be problematic for Council members, as it was unclear what this minimum might be. Members understood that a decision to publicly fund a medication involves reviews by multiple expert committees that consider the evidence from clinical trials and compare the cost of the drug to current therapies. The purpose is to ensure the drug works for the intended purpose, is safe and cost-effective, and that the public plan can afford it. Based on their final reviews of the available evidence, the expert committees provide a funding recommendation to participating jurisdictions.

Drugs for rare diseases, however, typically do not have large clinical trials that produce statistically significant, meaningful results due to issues with patient recruitment, and it is challenging for the expert committees to review the efficacy and safety of the drug based on the available evidence. In addition, drugs for rare diseases are very expensive due to the small user population coupled with the uniqueness of the products with no competitive offering.

Given this, members felt that trying to hold drugs for rare disease up to the same standards seemed to be both uncompassionate and impractical, and reached common ground that a different standard must be accepted. However, they also stressed that collecting real-life data to advance the overall evidence base and increase medical knowledge would be an important corollary, including accessing available international data as well as the experience of patients.

living with the particular rare disease. This common ground aligned with the second principle from the values framework:

**Balance evidence-based decisions and compassion**

When making effective drugs accessible for compassionate reasons, and when normal evidence standards cannot be met, programs should encourage the collection of real-life data to advance the overall evidence base and medical knowledge.

However there was an important caveat in that a different evidence standard was acceptable as long as funding the drug for a rare disease did not put in jeopardy other parts of the drug programs that serve greater numbers of people (especially other vulnerable populations) and meet the cost-effectiveness requirement. Members felt that it was important not to take away from what is already working well to serve the public good in the drug programs, in order to fund drugs for rare diseases.

Throughout this deliberation members noted that the discussion was made more difficult given the lack of a standard definition for a rare disease. They would recommend that such a definition be developed and used by OPDP.

**Shared Responsibility:**

Members agreed that there should be a shared responsibility to collect ongoing, real-life evidence to advance the overall evidence base for any drug for rare diseases. This would include pharmaceutical companies, patients/families and health care practitioners.

Members felt it was important that supports be put in place for patients to comply with the treatment regime, especially when their specific living situation may make it difficult, e.g. living in poverty or in a remote location. However, there was divergence around whether it was reasonable to require patients to enter into a contractual relationship to provide and release their medical data for evidence-gathering purposes. This was an idea supported in the 2010 deliberation of the Council on Drugs for Rare Diseases.

Some who supported this idea argued that if people wanted to receive public funding for drugs for rare diseases and were benefitting from an acceptance of a lower evidence standard for their high cost drug, they should expect to partner in building a better evidence base or forgo public funding. There was recognition that many patients would want to assist in this way in order to make things easier for those who might come after them. Members also noted that clinical trials would demand this information.

Others argued that it was an unfair expectation that patients share their medical information, especially if the consequence of not sharing would mean not receiving the drug and potentially suffering or earlier death. They argued this was not ethical as it coerces the patient to agree to release their medical data beyond the circle of care. In addition, it could be an unfair burden on a patient if collecting the necessary data would require additional hospital visits or testing.
Concept of Rarity:

Members were intrigued by a question posed by one of the speakers as to whether rarity in and of itself is an important consideration in drug funding. Once the difference in evidence standards is managed, can other considerations be normalized? One example provided was from Sweden where a €1 million cap is in place on any instance of public drug funding, whether or not it is for a rare disease. And, as more personalized medicine evolves, new drugs are being developed that, while treating a non-rare disease like cardiovascular disease, work for only a very small portion of the population because of the way the drugs have been designed. These drugs are usually much more expensive as well.

Members felt there was benefit in treating drugs for rare diseases in the same manner as other drugs as much as possible (once the difference in evidence standards is taken into account). If a drug is too expensive (and not cost-effective), it is perhaps too expensive for any disease, not just for a rare disease. The rarity is not a special consideration and de-siloing rare disease could be advantageous for the patient as well as the system. Instead the usual decision-making factors such as level of severity, degree of unmet need, cost and quality of life and longevity gain (QALYs) should be applied.

C. How have the views of the Council evolved since it first considered this topic in 2010?

There were only two members of the Council present for this session that participated in the first discussion on drugs for rare diseases in 2010. Given that, it was interesting to compare the views of the current Council members with those expressed in the previous meeting. In large part the views from 2010 were reinforced. Two principles that form part of the current Values Framework came from the 2010 discussion and were reaffirmed in the session by Council members. The chart below provides the 2010 recommendations, with the third column noting the 2017 comparison.

<table>
<thead>
<tr>
<th>Values and Principles</th>
<th>2010 Recommendations</th>
<th>Comparison Based on 2017 Deliberation</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. Compassion</td>
<td>Drugs for rare diseases should have their own set of funding criteria</td>
<td>Yes, compassion was important and was considered in agreeing that a different standard of evidence was acceptable. However this Council also felt rarity shouldn't be a distinguishing factor in funding determination. Other areas such as prevention were also seen as part of a compassionate and practical response.</td>
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<tr>
<td>2. Equity and Fairness</td>
<td>There must be different standards for the approval of drugs for rare diseases</td>
<td>Yes, agreed that a different evidence standard was acceptable.</td>
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<tr>
<td>3. Balance the Common Good with the Needs of Particular Individuals</td>
<td>The common good of the majority of the population must take into consideration the needs of the minority of citizens suffering from rare diseases</td>
<td>Yes, the Council reaffirmed this principle but was perhaps stronger in leaning towards the public good and not wanting current programs jeopardized by funding drugs for rare diseases—especially those that serve other vulnerable populations.</td>
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<td>4. Fiscal Responsibility</td>
<td>The competing needs for health care dollars mean that there should be an attitude fostering prudent fiscal management for drugs for rare diseases</td>
<td>Yes, the importance of system sustainability was recognized. However, the need to include procurement policy in reducing drug costs was also seen as part of fiscal responsibility.</td>
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<td>5. Accountability to Taxpayers</td>
<td>Decisions regarding funding for rare diseases need to be transparent, as does the rationale for them.</td>
<td>Not explicitly explored but transparency is an ongoing value of importance, as well as continuous, rigorous collection of data.</td>
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<td>6. Advance Medical Knowledge</td>
<td>The medical community must document the progression of the disease when a drug is administered to sufferers of rare diseases. Sharing in national and international research is essential, particularly for rare diseases.</td>
<td>Yes, members also suggested building the rare disease knowledge of health care professionals and developing clinical practice guidelines and rare disease registries and databases to track disease progression.</td>
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<td>7. Evidence-Based Decisions</td>
<td>It is the responsibility of experts to evaluate the evidence for the effectiveness of drugs for rare diseases. This evaluation must not be determined by politics or economics.</td>
<td>The issue of a drug approval being based on politics was not discussed. However, members felt that the process for approval should be similar to what occurs for other drugs with the exception of a different evidence standard.</td>
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<td>8. Shared Responsibility</td>
<td>Beneficiaries of drugs for rare diseases, their families, caregivers and health care professionals, share the responsibility to use the drug properly, to monitor and to report on its effectiveness.</td>
<td>Members felt there was a shared responsibility but there was divergence as to whether that should result in a contractual relationship with the patient/family or the ability to withhold a drug if evidence-gathering protocols were not followed.</td>
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<td>9. Quality of Life and Longevity</td>
<td>Quality of life and how long a person lives must underpin all consideration of funding of drugs for rare diseases.</td>
<td>Members saw quality of life and longevity gain as important factors but felt that these should be considered as part of the cost-effectiveness determination for funding as with other drugs.</td>
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<tr>
<td>10. Other Areas of Concern</td>
<td>The Ministry should raise awareness of its policies and procedures for approving and adding new drugs to the Formulary and removing those no longer found to be effective; these explanations should be aimed not only at those suffering from rare diseases, but also directed to the public at large.</td>
<td>Members continue to have concerns about formulary management. They also raised the issue of pharmaceutical drug pricing, particularly in terms of cost of a drug versus its price. They urge OPDP to work with other provinces and territories to utilize procurement policies and collective procurement to reduce drug costs.</td>
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<tr>
<td>11. Other Areas of Concern</td>
<td>A definition of rare diseases must be established by experts and publicized. A national standard would be desirable.</td>
<td>Yes, a national definition for rare disease is still highly recommended.</td>
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### 5.0 VALUES FRAMEWORK

The Values Framework was developed at the Council’s first meeting in 2010, and is re-visited at each meeting. The topic of funding drugs for rare diseases was also discussed at that first meeting. The chart in Section 4 above captures the comparison between the 2010 and 2017 deliberations. The values and principles from the Framework that were pertinent in the 2010 deliberation still resonate with today’s members and no changes were required to the Values Framework based on the 2017 discussion.

### 6.0 CONCLUSIONS AND RECOMMENDATIONS:

The following is a summary of Council’s observations and recommendations from this meeting, expressed first in terms of key values.

1. **Benefit the greatest number of people for the greatest good:**
   The notion of public good includes the prudent use of available resources for the health benefit of the greatest number of people in Ontario. Both the cost and the opportunity-cost of funding drugs for rare diseases were of real concern and Council would welcome an opportunity to discuss the issue of pharmaceutical pricing in a future session if that would be useful to the Ontario Public Drug Programs (OPDP).

2. **Be responsive to the most vulnerable:**
   It must be acknowledged that there are competing views of who the most vulnerable in society are, and what role the drug program should play in supporting them. Members
continue to put more emphasis on programs that assist those on reduced incomes who may not have any drug coverage and can’t afford even basic drugs. They were also clear that funding drugs for rare diseases should not be at the expense of existing programs that also help vulnerable populations, and members cautioned against taking away what is already working well.

3. **Balance the common good with the needs of particular individuals:**
The government is mandated to serve all citizens, including those with special needs, but providing prudent management of available resources also benefits the common good.

4. **Efficiency and fiscal responsibility:**
This speaks to ensuring that taxpayers’ money is used well and that the system works in a cost effective manner. Council strongly recommends that the OPDP continue to work with other provinces and territories to realize collective procurement savings.

5. **Compassion:**
Council felt that while it is important for the drug programs to consider funding drugs for rare diseases on compassionate grounds, compassion cannot extend indefinitely, and must not be used as a substitute for strong clinical evidence and economic measures such as cost-effectiveness. Members discussed the need to resist the powerful inclination to rescue a single identified endangered life, regardless of cost, at the expense of nameless others who will therefore be denied health care - a concept referred to as the rule of rescue.\(^{13}\)

6. **Informed public and healthcare professionals:**
Low awareness about rare diseases can result in delayed diagnoses, limited treatment options and difficulties accessing quality care. These challenges could, at least in part, be addressed through increased public awareness and greater access to information for patients; building awareness among family doctors and other healthcare providers to enable early diagnosis and treatment; setting up a hotline to assist with navigating the system; and encouraging the use of telemedicine to reduce cost and inconvenience of travel and to ensure equal access for those disadvantaged by location.

7. **Prevention:**
Genetic testing, counseling and prenatal screening should be considered if there is likelihood of a rare disease being passed to offspring. This is not testing and screening for all, but for those whose rare disease might be genetic, and could provide information to prospective parents for informed decision making. Cutting-edge advances in health care should also be supported, such as genetic replacement therapy\(^{14}\) which replaces a faulty, disease-causing gene with a functional one. While still expensive, patients may only have to receive a one-time application of gene therapy for an ongoing benefit or cure, rather than long term drug therapy. Also within the topic of prevention, it was agreed that funding drugs for rare diseases must not jeopardize current funding for preventative medicines such as the flu vaccine, or curative drugs which ultimately result in savings to the overall health budget if they eliminate the cost of long-term treatment.

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\(^{13}\) Osborne, M., & Evans, T.V. (1994) Allocation of resources in intensive care: A transatlantic perspective The Lancet, 343. pp 779

8. **Transparency:**
   Throughout these deliberations it was noted that discussion was made difficult by the lack of a standard definition for rare disease. It is recommended that such a definition be developed and used by OPDP.

9. **Evidence-based decision making and minimum evidence standard:**
   Holding drugs for rare diseases to the same evidence standards as those applied to other drugs seemed both impractical and uncompassionate. A different evidence standard seems appropriate and just, but it is unclear what the different evidence standard should be.

10. **Fairness and equality:**
    Beyond the evidence standard for drug approval discussed above, members felt that drugs for rare diseases should be treated, to the extent possible, in the same manner as all others. If a drug is too expensive for the public purse (and is not cost-effective), it is too expensive for any disease, not just for a rare disease. In fact it was agreed that rarity should not be a special consideration and that “de-siloing” rare diseases could be advantageous for both the patient and the system.

11. **Sustainability:**
    The exceptionally high cost of drugs for rare diseases and the potential to consume a large proportion of Ontario’s drug budget triggered real concern that publicly funding such drugs could put the sustainability of our Ontario Public Drug Programs and/or other parts of the drug programs at risk.

12. **Shared responsibility:**
    When making drugs accessible for compassionate reasons, and when normal evidence standards cannot be met, members agreed that there should be a shared responsibility to provide and collect ongoing, real-life evidence to advance the overall evidence base for any drugs for rare diseases. This was seen as involving pharmaceutical companies, patients, families and health care practitioners and should include accessing available international data as well as the lived experience data of patients with rare diseases.
### APPENDIX 1

**MEMBERS OF THE ONTARIO CITIZENS’ COUNCIL**

<table>
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<th>Members</th>
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<td>Marilyn Wood (Chair)</td>
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1.0 EXECUTIVE SUMMARY

The Ontario Citizen's Council is composed of up to twenty-five Ontarians from all walks of life appointed by the Minister of Health and Long-Term Care. The mandate of the Council is to provide values-based perspectives on questions put to it by the Executive Officer of the Ontario Public Drug Programs (OPDP).

To assist in this mandate, the Council has developed a Values Framework to bring increased clarity to its values-based deliberations. It is hoped that the framework will also be useful for the OPDP to use in considering citizens’ values in their decision-making and be applicable to the whole OPDP including stewardship of the drug formulary.

The framework is a work-in-progress. It will evolve as the Council considers further issues and values and will be updated as needed. We hope over time that it will provide a way to assess/measure which values have been the most important in formulating the Council’s advice.

2.0 PREAMBLE

Each society upholds a set of values that define it and help guide decisions on how to share limited goods and services. Values help us decide what we should do. They influence standards and norms of behaviour, e.g. compassion, freedom of choice, equity. They represent what we most care about.

Values are often divided into three groups: personal (“my” values), social (“our” values) and ethical (universal values). As the Council represents the public voice on behalf of Ontarians, our focus is on the social and ethical values that should help guide OPDP and our own deliberations.

Working with values poses a number of challenges. The first is creating a shared understanding of what a value means and how it is being interpreted/used. As a Council we have captured our thinking to date in this document. It gives us language to explain our advice and recommendations and provides a shared vocabulary for communicating to the OPDP what we care about as Ontarians. It helps make our values more explicit.

The second challenge is that values can overlap and conflict. They don't always take us in the same direction as we think about an issue and what is important to consider in resolving it. For example, should we maximize health benefits for the largest number of people or should we help the most vulnerable? We have found that while we often share a common set of values, there can be real differences in how we apply those values in a particular context on a particular issue. The weighing of values is very context-specific and so while the framework contains important values and some sense of priority, it is conditional, based on context. The framework will help us be more explicit about our deliberations on competing values and how we have weighed them in determining our recommendations on a particular issue. It will also help us compare our
deliberations and ultimately draw out some principles that can be applied more broadly. This framework offers a couple of starting points for this.

**Relationship to the Ontario Drug Formulary**

As we developed the preliminary framework, we wrestled with whether we needed to consider the values that are embedded in the Ministry’s mandate regarding the Ontario Drug Benefit Formulary. At this stage, we determined that our own value deliberations could take as a given that the Ministry must manage the drug programs in a manner that is fiscally responsible and accountable to taxpayers and in a way that contributes to the fostering of a sustainable health system for the benefit of Ontarians. Thus the economic values of fiscal responsibility, accountability and sustainability are already mandated and will figure less into our own deliberations.

We also recognize that good stewardship of the drug formulary requires:

- The need for feasibility/practical application
- The need for a balance of values
- The need for responsiveness – the ability to act quickly based on new information
- The importance of context – each value must be applied in its context and applied with reason and clarity
- The need for regular review (in terms of how we operationalize or justify advice)

**3.0 KEY VALUES**

In the Council’s deliberation to date, several values have risen to the fore. The Council reaffirms the importance of all these values and recognizes that any of them may be deemed a top priority depending on the context and issue at hand. We also recognize that these values are not mutually exclusive, nor do they operate in a vacuum. They must be applied in a manner that respects both the real-life experience of patients and the public good. Striking a balance between competing values will be an ongoing challenge.

In trying to organize our own thinking about values, we categorized the key values as follows (in no particular order). It is important to note however that the values may move from one quadrant to another depending on the context and how they are being used.
## 4.0 PRIORITIZING AND CLARIFYING VALUES

Given the importance of context, it is extremely difficult to determine absolute priorities in terms of values. However, given the caveat that a number of the economic-oriented values are covered off in OPDP’s own mandate (as well as public safety), nine values seemed both high priorities and demanding of greater clarity. While the work to understand and clearly define all the values, particularly in relationship to OPDP, is ongoing. The following descriptions for nine of the values are offered as a starting point:

### Evidence-Based Decision-Making

This should include:

- Systematic expert review of the relevant published literature as well as grey literature (informal or unpublished evidence, including evidence gleaned from real life drug use).

- Full range of both positive and negative aspects including ongoing reporting of adverse events

And recognize that:

- The standard of acceptability for a specific drug may vary depending on particular situations, but still needs to be defensible and based on good and comprehensive data, derived from both clinical sources and real world experience.

### Equity

- The provision of equitable access to drugs and treatments for all citizens while protecting the vulnerable and being non-discriminatory.

- Equity does not necessarily mean identical – how equity is achieved may be different in different places or situations.
In application, drug formulary decisions should not further existing inequities in drug accessibility, and should mitigate health inequities when possible – e.g. those due to income, geography, or other factors.

**Fairness**

- There are different dimensions to the value of fairness. For example, procedural fairness – that the process is fair (e.g. objective, consistent) even though the outcome may not be agreeable to all.

- Fairness is also related to the public good and can relate to the fairness of the system in consideration of both the common good and the needs of individuals.

- Fairness and equity are often closely related, but can be in tension with one another depending on the issue. For example, procedural fairness could lead to inequity if the reality of some people’s living situations or subpopulations’ disease prevalence do not fit well into the regular decision-making process/criteria.

**Compassion**

- While compassion is an emotion of sympathy towards the plight of others, as a value it reflects concern for a society’s vulnerable members.

- However, given its strong emotional pull, the value of compassion needs to be weighed in with all factors and a judgment made based on thought and consideration that does not just look at any one factor.

- Over time a procedure could be put in place to integrate compassion in decisions made. This would increase the consistency and predictability of decisions and hence their defensibility.

**Public Good**

- Public includes all Ontarians
- Good includes the health of the population
- Requires prudent use of all the resources available, that include but are not limited to evidence based resources, for the health benefit of most people in Ontario

**Quality of Life**

- One’s quality of life and how that is valued is very subjective. Therefore, the patient’s perspective needs to be considered and balanced along with the medical expert’s. This needs to be taken into account in the decision making process.

- It is very hard to put a dollar value on quality of life and determine what weight to put on it when making drug funding decisions. The Council recognizes one way to do this in a more objective way is through Quality Adjusted Life Years (QALY) - the number of years of living to expect on a particular treatment and how well the patients are living during that period.
Efficiency

- This includes the notion of maximizing the results achieved with a minimum of wasted effort or time. It encompasses how well the system works in a cost effective manner, ensuring that taxpayers’ money is used well.

- It is important to consider efficiency as a means to an end – a valued way to achieve valued results. Making sure that these results align with our values must also be considered. Decisions should not be based solely on evidence of their relative costs and benefits.

- Having an efficient system usually requires the buy-in and involvement of all stakeholders (e.g. citizens province wide, patients, administrators of the program), which means being user-friendly and transparent.

Prevention

- This includes both the role of drugs and individual responsibility for health.

- Prevention is important both to prevent suffering (e.g. delay or prevent disease onset) and to achieve a public drug program that can be financially responsible and sustainable into the future.

Simplicity

- Simplicity espouses a system where users understand what is available, how the services apply to them and how they can access the services that best suit their requirements.

5.0 PRINCIPLES

As we noted earlier, we consider the application of values to be context-dependent. However, even given this, we have found that it is possible to begin to develop some principles of application. Key to this is the notion of balance – perhaps another value in its own right.

Two principles have emerged for us to-date:

**Balance the common good with the needs of particular individuals**

The government has a mandate to serve all citizens, including those with special needs, but it must provide prudent management of available resources for the benefit of all.

**Balance evidence-based decisions and compassion**

When making effective drugs accessible for compassionate reasons, and when normal evidence standards cannot be met, programs should encourage the collection of real-life data to advance the overall evidence base and medical knowledge.
6.0 CONCLUSION

The values framework will be an important contribution to the Council’s work. We expect to use this framework in future sessions as a guidepost for our recommendations and advice. We want to use the framework as a standing item at each meeting to consider whether new values have emerged during that meeting’s “deliberations”, and as a way to identify any particular values relevant to the topic at hand. Since the framework will be “evergreen,” (that is an iterative document, reviewed and revised over time), there will be ongoing opportunities to refine it and to develop principles which exemplify citizens’ values.

The framework is important from several perspectives:

- It assists the Citizens’ Council in providing common language for the Council’s deliberations and lending consistency to its recommendations.
- For MOHLTC, it can provide defensible decisions based on identifiable and consistent evidence and values-based reasons.
- For the public, it can provide a rationale for funding decisions that considers both evidence and values important to citizens.

Council members respect the scope, importance and challenge of building a values framework and are committed to continuing this rich dialogue as we deliberate on issues concerning the Ontario Drug Programs.