A Report of the Ontario Citizens’ Council

QALYs and Drug Funding Decisions in Ontario

Submitted to:

Diane McArthur
Executive Officer and Assistant Deputy Minister
Ontario Public Drug Programs
Ontario Ministry of Health and Long-Term Care
Hepburn Block
80 Grosvenor Street, 11th floor
Toronto, Ontario M7A 1E9

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Submitted by:

Gerri Gershon
Chair
Ontario Citizens’ Council
26 Apollo Dr
North York, ON M3B 2G8
Executive Summary

The Ontario Citizens’ Council is mandated to provide advice to the Executive Officer of the Ontario Public Drugs Program on the values that reflect the needs and concerns of Ontario’s citizens about government drug policy.

At its June 2013 meeting, the Council was asked to provide advice on the values that should guide their government in making drug funding decisions. In particular the Council was asked to assess the ethical and values-oriented issues associated with the use of QALYs as a cost effectiveness measure. The June 2013 meeting also welcomed and oriented several new members to the Council and the work of OPDP.

Through presentations by experts and through facilitated discussions, Council members examined the use of QALYs as well as other possible considerations in making drug funding decisions such as therapeutic gap. The Council’s Values Framework was revisited and the Council offered specific recommendations on how those values applied to the question of QALYs and other drug funding criteria.

Recommendation 1: The Use of QALYs

QALYs should continue to feature prominently in making decisions for drugs to be put on the formulary. However they should not be the only consideration or even the primary consideration.

Recommendation 2: Other Considerations

- Clinical evidence and safety considerations should be paramount criteria in decision-making, with safety being looked at over the long-term as drugs are used in real life.
- Therapeutic gap (or need) is an essential consideration.
- Disease severity is too subjective a measure to be useful.

Recommendation 3: Decision-making Process

A clear, transparent decision-making process should be established. It should be premised on procedural fairness, possibly using the A4R as an approach.

Recommendation 4: Other

Both prevention of disease and individual responsibility for health need to be recognized and considered as important aspects of ensuring a sustainable drug program. To this end, the Council would urge the government to consider prevention as a critical ingredient in achieving a public drug program that can be financially responsible and sustainable into the future.

In conclusion, deliberations of the Council were rich and substantive, with new Council members fully integrated into the work of the Council. We anticipate that our recommendations will assist the Ministry as it continues to use QALYs as an important element of its drug funding decisions.
1.0 Introduction and the Question

The Ontario Citizens' Council is comprised of a diverse group of Ontarians appointed by the Minister of Health and Long-Term Care (See Appendix 1 for the list of Council Members). The mandate of the Council is to offer societal values reflecting the needs, culture, and attitudes of the Ontario citizenry about topics or issues related to government drug policies. The Council reports to the Executive Officer of the Ontario Public Drug Programs and to the Minister of Health.

In this, the fifth session of the Council, Diane McArthur, the Executive Officer and Assistant Deputy Minister for the Ontario Public Drug Programs, posed the following question for the Council's consideration:

To what extent should the Ontario Public Drug Program consider QALYs in drug funding decisions?

The intent of this dialogue was to better understand the values and principles that Ontarians believe should guide their government in making drug funding decisions. In particular the Council was asked to assess the ethical and values-oriented issues associated with the use of QALYs as a cost effectiveness measure. They were also asked to consider other factors such the severity of a disease, high cost of a drug and situations where advances in survival without further disease progression may be the only noted improvement offered by a new treatment.

A Quality Adjusted Life Year – or QALY – is a measure developed as a way of relating the patient benefits of a drug treatment to its cost. QALYs are considered to be a measurement of how effective a particular drug treatment is. Drug funding reviews may compare the QALYs (effectiveness) of a treatment against the cost of the treatment to decide whether a product should be funded publically by the province.

QALYs take into account both the quantity and quality of life generated by a drug or treatment by placing a value on both mortality (length of life) and morbidity (quality of life). In simple terms, a QALY represents life years weighted by a patient's quality of life. It is expressed in terms of a utility score between 0 (= death) and 1 (= perfect health). For any condition and drug, a QALY is determined at least in part by patient assessments of their quality of life. This is often done as a part of a clinical trial.

In Canada, provincial drug administrators are beginning to question the initial promise of the usefulness of QALYs, as a means to allow simple and defensible comparisons across drugs and diseases, in difficult drug funding decisions. Indeed Ontario's Committee to Evaluate Drugs (CED) uses a multi-factorial decision making process where QALYs play only one part in a drug funding decision.

The Council's task was to weigh in on what members consider important about QALYs and the extent to which they should be considered in the overall process of decision making. Members also examined other factors that might be important such as disease severity.
Members of the Citizens’ Council were asked to consider criteria that the Council to Evaluate Drugs and the Executive Officer use when making a decision to fund a particular drug and reflect on questions such as:

- What values are inherent in a drug funding decision? How can citizen values inform this process?
- What kind of situations could present a problem or issue for drug funding decision-makers?
- How do the different criteria or metrics that are used in funding decisions, such as QALYs, reflect the values of the Ontario Citizens’ Council?

It should be noted that the Council’s discussions took place concurrent with a meeting of the Committee to Evaluate Drugs (CED, the Ministry’s expert drug review advisory group), with the goal of providing both professional clinicians’ and ordinary citizens’ input into using QALYs in drug funding decisions.
2.0 Preparing for Deliberation

2.1 Advance Reading Materials

Council members were provided with a range of background reading. One abstract described Ontario’s Drug Review Process, defined a QALY and how it is calculated to give an overview of a patient’s state of health, along with how this information is used in drug funding decisions and concerns with the use of QALYs.

A journal article “QALYs: The Basics” provided a much more intensive examination of the values associated with health states, improvements and outcomes, implementation of QALYs. It also dealt with issues of equity and fairness and how they are not being quantitatively incorporated into the conventional QALY approach. The overall position of the authors, however, was favourable to the use of the QALY metric as a way “to guide health-care resource allocation decisions”.

From another perspective, a media release highlighted a report from the European Consortium in Healthcare Outcomes and Cost-Benefit Project. They commented that the QALY formula, as currently used by the UK’s National Institute for Health and Clinical Evidence (NICE), should be “abandoned” and that QALY “is not a valid yardstick for determining the extent to which the method can weigh the cost of a drug against extending or improving lives.”

2.2 Presentations for Council Members

To augment and expand on the reading material, Council members heard expert presentations on the following topics:

- *QALYs and Their Role in Economic Evaluation and Decision Making* by Dr. Doug Coyle, Professor Department of Epidemiology and Community Medicine, University of Ottawa
- *Resource Allocation, Health Technology Assessment and QALYs* by Dr. Christopher McCabe, Research Chair, Faculty of Medicine and Dentistry, University of Alberta
- *A Cocktail of Health Policy Decision Making* by Dr. Allan Grill, MD, Chair of Ontario MOHLTC CED
- *Ethics and Values in Drug Funding Decisions* by Dr. Jennifer Gibson, Director of the Joint Centre for Bioethics, University of Toronto

Following presentations, Council members had the opportunity to question each expert individually as well as in panel format.

(See Appendix 2 for the session agenda which includes the names of the presenters. See Appendix 3 for biographies of presenters)
QALYs and Their Role in Economic Evaluation and Decision Making

Doug Coyle provided an overview of how an economic analysis is applied to drug funding decisions, with reference to the data collection required; the nature of an economic evaluation, and application of QALYs to reflect overall the “multifactor nature of outcomes.”

In summary, he indicated that an economic evaluation using QALYs “allows the consideration of multifactor outcomes and costs associated with alternate plans of action”. Given how complex drug funding decisions can be, a method is required which “synthesizes the knowledge we have gathered on treatment effects and costs”.

In discussions with presenters, Council members seemed to agree that QALYs don’t encompass the whole nature of the disease and there is a need to be sensitive to other values. They also recognized that, while not a perfect instrument, QALY is currently the best metric we have. This was discussed in considerably more detail later in the day.

Resource Allocation, HTA and QALYs

Chris McCabe introduced the Council to the Health Technology Assessment (HTA) process used in drug funding decisions. This weighs clinical effectiveness against cost effectiveness, balanced by the social values of the population in order to arrive at a decision in line with public policy objectives.

This was followed by an examination of quantitative instruments used to measure health conditions, or more simply, how the seriousness of a disease is measured. In particular the Euro QoL or EQ-5D questionnaire designed to measure health states was described. This is a model in which patients describe their health-related quality of life along five dimensions: mobility, self-care, usual activities, pain/discomfort, and anxiety/depression.

Finally we were asked to think about procedural justice: “if we cannot get outcomes that everyone will agree to, can we at least get processes that everyone agrees are fair?” We were then introduced to A4R – Accountability for Reasonableness - as the most widely used form of procedural justice.

A4R identifies four criteria which should characterize decisions:

- Reasons which are relevant
- Transparency and public accessibility
- Opportunity to revisit and revise if new evidence or argument is provided
- Empowerment and equalization of power differences, and to ensure that these four are met

To ensure that these criteria are met, A4R includes an agreement of “enforcement through a commitment by leadership”.

In discussions with presenters, Council members expressed concern about how to measure the seriousness of a disease. They also wanted to be sure that patients were empowered and actively involved in this process. There were questions raised about whether emphasis on
quality of life did not adequately consider situations where patients might assess their level of
pain or disability as worse than death.

A Cocktail of Health Policy Decision Making
Allan Grill provided the Council with a detailed presentation including: a brief statistical
overview of drug expenditures both nationally and provincially; the range of participants in
Health Technology Assessment and drug funding procedures in Ontario; the process through
which drugs are approved for listing on the formulary; and, funding of the public drug program.

Specific drug therapies were used as examples of the many factors considered by the CED in
deciding whether a specific drug therapy would be included on the formulary. These included
Victrelis (boceprevir) and Incivek (telaprevir) used for infections.

In summary, drug funding policy requires “in-depth analysis of several factors including
efficacy, safety, cost and cost-effectiveness.” Publicly funded drug plans need to use an
evidence-based approach, be transparent to health care providers with their deliberations and,
above all, be consistent and accountable regarding their recommendations.

Ethics and values in drug funding decisions
Jennifer Gibson provided a brief definition of ethics and identified the positive features of
QALYs in terms of the values of equality, efficiency, fairness, and simplicity, underscored by the
value that “benefits gained by the treatments...should be greater than the benefits sacrificed by
those who were denied treatment“. The challenges of QALYs rest in their ambiguities (for
example, who defines quality?)

The Ontario Citizens’ Council had previously created a Values Framework to guide their
deliberations as well as to provide decision makers with a set of societal values that the Council
would like taken into account as decisions are being made. These include: evidence-based,
equity, compassion, public good, quality of life and efficiency.

See Appendix 4 for the Ontario Citizens’ Council Values Framework.

Since QALY is only one input among many, ethical treatment requires the consideration of
other relevant values in addition to cost and clinical effectiveness.

In the discussion with panellists following all the presentations, Council members raised
questions about how drugs are removed from the formulary, advantages of a pan-Canadian
approach to drug access and funding, and the differences between resource allocation
decisions and individual treatment decisions.
3.0 HOW THE COUNCIL DID ITS WORK

In preparation for the discussion, Council members completed the Euro QoL or EQ-5D questionnaire. (See Appendix 5). One of the presenters tabulated the results which indicated that the Council in general was representative of the usual patterns found in the general public. The questionnaire itself offered a tangible illustration of one of the steps used in developing a QALY and raised a number of questions for members, including whether patients with a certain disease would rate the subjective questions differently than a healthy person might. For example, would death necessarily be the worst possible scenario, given the pain, suffering and/or loss of independence that a patient might experience?

Following the presentations, Council members began their own exploration of QALYs and other considerations. As with other sessions of the Council, the conversation was a dialogue with facilitated deliberation on the pros, cons and tradeoffs involved in considering “To what extent should the Ontario Public Drug Program (OPDP) consider QALYs in drug funding decisions?”

The small group process began with a sharing of personal experiences that linked to the problem at hand. Then members explored QALYs based on the following questions:

- What have we learnt about QALYs?
- What questions do we have?
- What are the advantages?
- What are the limitations?
- What are our concerns?

They then examined other considerations, including the idea of disease severity and what it means to members. Finally each group came to common ground in terms of the place of QALYs in today’s drug funding decisions, any tradeoffs this entails, and the values that guide the determination of this common ground.

The facilitators compared the results of the two groups and pulled together points of common ground and areas of possible divergence. Through an intensive dialogue process, these were refined by the Council.

The Council shared its discussions with the Executive Officer of the Ontario Public Drug Programs, Diane McArthur, and she responded to outstanding Council comments and questions, after which the common ground was once more refined.

The Council’s "Values Framework" was reviewed (see Appendix 4) and the Council members worked to connect the discussion of QALYs with this framework. The objective was to see whether the discussions elaborated the existing values or principles, or whether they provided other values for possible inclusion in the framework. Council members brought several anecdotal stories to the discussion to help explore the values. Examples included situations where a child was the patient and the impact on his/her family or caregiver had to be
considered; patients who were not taking personal responsibility for their health and disease prevention; and, circumstances when the QALY was cost effective, but in funding a particular drug the negative impact on society was too great to be an effective option. Oxycontin was the example discussed.

The session ended with a further clarification of the key points of advice that needed to be incorporated into this report.
4.0 Results of deliberation

The following questions were posed to the Council to think about while looking at the role of QALYs in decision making:

- To what extent should the OPDP consider QALYs in drug funding decisions? Why?
- To what extent should other criteria be considered? Why?
- How do these different considerations reflect the values of the Council?

4.1 Use of QALYs

Given the available options, Council members felt that QALYs needed to continue to be used as a significant part of the decision-making process, for the following reasons:

- They are consistent, easy to understand, and level the playing field across different medications
- The process of developing QALYs is transparent and based on patient input
- The results are objective, comparable, evidence-based and quantifiable. This makes them defensible and provides a benchmark
- They are universal, apolitical and can be used (and compared) across Canada (and around the world.)
- They assess value for money where value is based on both length of life and quality of life
- They appear to be fair, unbiased, just, non-discriminatory
- They are familiar – the system is already set-up to produce and use them.
- They are better than no measure; QALYs are the best we have right now (which should not preclude improvement)

However, Council members also identified a number of limitations in the use of QALYs which need to be compensated for in the decision-making process used by OPDP. These include:

- In being so objective, QALYs lack compassion in terms of how a disease impacts an individual
- There seems to be uncertainty as to how well QALYs accommodate subpopulations who may be affected by a disease or drug differently than the general public. It provides averages and therefore exemptions or uniqueness are not considered
- The implications of non-medical effects in assessing cost effectiveness are not included (for example, costs to access care in remote locations)
- Overall QALYs seem too simplistic for real world comparison. For example, a QALY is usually developed as part of clinical trial, which is a time-limited assessment and not
necessarily representative of what might happen with long-term use. Additional data would need to be collected with a drug’s use outside of a clinical trial.

- How might QALYs be adjusted for those who have multiple conditions?
- There is ambiguity in the QALY definition and the questions asked are not specific enough. Given the need for patients to complete the questionnaire, what are the limitations for those who can’t do this for themselves (such as, for example, children or those with severe mental health issues)

Overall, while members saw strength in the fair, consistent, comparable, evidence-based approach of QALYs, there were concerns about issues of equity for those who did not fit into the mainstream (e.g., Access to services depending on where you live could influence how you may perceive your quality of life), and about a lack of compassion in the decision-making process if it were to be based solely on QALYs.

4.2 Other Criteria and Considerations

When deciding whether or not a drug should be funded, there are other factors that must come into play. Of these, clinical evidence, therapeutic gap and safety were common ground priorities for Council members.

- The quality of the clinical evidence is key and must be reviewed over time. The quality will determine how “evidence-based” the decision actually is. Evidence could interact with disease prevalence, as one might assume there would be better data for common diseases in contrast to rare diseases
- Is there a therapeutic gap? In other words are there already other drugs on the market that address the need? Are alternative treatments available? What is the capacity to benefit from the new drug? Are there no drugs or treatments available for a particular condition or set of conditions?
- Has safety been considered? There is a need for follow-up to determine long-term side effects
- What is the budget impact? Overall budget limitations still need to be considered. Has there been consideration of removing drugs from the formulary?

The innovativeness of a drug/treatment had some appeal, but there were concerns raised regarding who defines something as innovative and to whose benefit

The idea of considering the type of disease was not seen as appropriate. For example, Council members did not feel that greater consideration should be given to drugs for diseases that are high on the public radar, such as cancer.

Overall other considerations offered by presenters did not have much resonance for Council members, in so far as there was time to discuss each. These included age, socio-economic status; idea of “fair innings”; rule of rescue; end-of-life.
Finally the idea of procedural fairness as represented by the Accountability for Reasonableness (A4R) framework (noted under Presentations section above) resonated strongly with the Council and connected strongly to their key points of common ground and their Values Framework. The component of “empowerment” raised a number of questions for members that they flagged for further consideration by OPDP.

4.3 Disease Severity

Council members were asked to consider disease severity as a possible criterion in drug funding decisions. The first step required getting a clearer understanding of what disease severity means and to whom, as no working definitions were provided.

For members, descriptors of disease severity included:

- Caring responsibility increases (along with disease severity)
- Acute vs. chronic vs. contagious (attacking healthy people, e.g. SARS)
- Lower quality of life
- Lower life expectancy
- Progression of disease – treatment could divert/hold off later severity
- Think longer term - severity over time
- Incurable; level of pain; debilitation
- Ability of patient to benefit, (e.g. cure vs. delay death)

While some thought that impact on caregivers and other family members were significant factors, others did not. In the end there was common ground that disease severity was too subjective to be a useful criterion. It was also suggested that in the areas where it could be useful, it overlapped with QALYs (quality of life assessment) and yet did not have the advantages of QALYs noted above.

4.4 Outstanding Issues

Council members had a number of outstanding questions for the Executive Officer that arose from the conversation of QALYs. In a Q&A, the following emerged:

Question: Who should be responsible for ongoing drug evaluation over time?

It depends on the drug, but the industry, prescribing doctors and government all have a role to play. For example, doctors will see Adverse Drug Reactions (ADRs) and are required to report those. Government can ensure information is disseminated.
Question: How can drugs be removed from the formulary?

Unless there are clear safety issues, or even if there are, it can be quite difficult to remove drugs from the formulary\(^1\). The drug may be effective for some people. Others may be willing to run the risk of ADR. Risk and pain tolerance are both key in people making individual choices about drugs and it is difficult for government to weigh in at this level.

Question: Are there products that are helping certain diseases that would be better treated in palliative ways?

Government is looking at the drug system and a rational decision making process. There are many drugs that exceed the cost of home care and offer little in terms of increased length of life. Many oncology drugs are thousands of dollars a day and are not cost effective. If the drug is a major advancement or innovation however, it could still be worthy of funding.

Palliative care is comparatively inexpensive. However, in its current form it relies heavily on volunteers. The end-of-life decision making process is one that we as a society have not grappled with effectively. Quebec is currently looking into this issue, having introduced end-of-life legislation.

Question: Does the Committee to Evaluate Drugs (CED) look at the potential for abuse of the drug in society?

One example is Oxycontin. It was initially perceived to be less addictive than comparable medications, and this was the primary reason it was introduced, despite prior hesitancy around opioids.

The CED does a good job of looking into issues such as this from a public safety perspective, and takes these kinds of issues into consideration. But we often run into a scenario where we don’t know how a drug is going to work in the broader population. There are a variety of risks that exist for products as they are made widely available and some of these risks are hard to predict.

Question: Could OPDP work with pharma to draw attention to disease areas lacking attention?

We currently have limited contact with manufacturers in regard to some key areas such as chronic disease, vaccines and prevention of disease, and mental health (e.g. Alzheimers and dementia products).

Often the discovery process by manufacturers is organic. Society also determines where investments are made based on market principles.

\(^1\) In Spring 2011, the Council submitted a report to the Ontario Public Drug Program called “Managing the Formulary” which provided advice on the values that should influence the management of the Formulary, including the delisting of drugs.
5.0 Council Views on Relevant Societal Values and Related Recommendations

The Council was asked to specifically identify the values that were important in reaching their conclusions about the use of QALYs in decision-making. To do this, the Council reviewed its existing and evergreen Values Framework. It recognizes that the values are not mutually exclusive, nor do they operate in a vacuum. They must be applied in a manner that respects the real-life experience of patients, always keeping the public good in mind.

There were several values that came to the forefront in the dialogue on QALYs. Embedded in these are additional values that are italicized. These were:

Fairness
The objective, consistent nature of QALYs was seen as being fair. This idea of fairness is most closely related to the notion of procedural fairness – that the process is fair even though the outcome may not be agreeable to all. It also relates to the value of public good in that QALYs provide for the objective and prudent use of resources available for the OPDP.

Equity
Previous work of the Council, as expressed in the Values Framework, has seen equity as being somewhat different from the notions of equal or identical. In essence it is the difference between everyone getting the same and everyone getting what they need to get the same result. In the context of QALYs, the Council recognized a tension between fairness as discussed above and equity where the reality of some people’s living situations or subpopulations’ disease prevalence may need to be considered in some different ways than the general QALY determination in order for there to be equity in drug provision.

When looking at this “trade-off” between fairness and equity, members noted that it is perhaps another expression of a stated principle in the Values Framework, which reads: "Balance the common good with the needs of particular individuals”

Compassion
Concern was expressed that QALYs did not provide any room for the exercise of compassion in drug-funding decisions.

Evidence-Based
Members liked that QALYs were evidence-based and thus resulted in objective, consistent, comparable, universal and quantifiable measures.

Council members noted that there was a tension between these two values that related to the second principle put forward in the Values Framework: “Balance evidence-based decisions and compassion”
Two other core values contained in the Values Framework were very prominent in the discussions on QALYs. Council members noted that, in many ways, the reconciliation of these two values was at the heart of the QALY measurement. These two values are:

- Cost-effective – which is contained in the Council’s understanding of efficiency in the Values Framework
- Quality of life – which in the case of QALYs also contained the notion of the quantity of life

Other values noted in the Values Framework that resonated for members in this discussion were accountability, transparency and sustainability.

Clarity was a new value that emerged in the discussion on QALYs. In this application, members interpreted clarity as meaning simple and easy to understand.
6.0 Council Responses to the Executive Officer’s Question

After reviewing all of the supporting material and presentations and getting answers to many questions, Council agreed that QALYs are a useful tool in helping to determine how drug funding decisions are made. However, Council felt that it was also important to address areas of concern which QALYs do not take into consideration. The following recommendations were developed from the common ground:

**Recommendation 1: The Use of QALYs**

QALYs should continue to feature prominently in making decisions for drugs to be put on the formulary. However they should not be the only consideration or even the primary consideration.

**Recommendation 2: Other Considerations**

- Clinical evidence and safety considerations should be paramount criteria in decision-making, with safety being looked at over the long-term
- Therapeutic gap (or need) is an essential consideration – this refers for example, to situations where currently no drugs are available or there has been no significant advance in treatment for a considerable period of time
- Disease severity is too subjective a measure to be useful

**Recommendation 3: Decision-Making Process**

A clear, transparent decision-making process should be established. It should be premised on procedural fairness, possibly using the A4R as an approach.

In making this recommendation, the Council recognizes that further consideration will need to be given to how the empowerment principle is realized. For example, are patient group submissions sufficient? How does the government ensure equity for patients who do not have an organized group to represent them?

**Recommendation 4: Other**

Both prevention of disease and individual responsibility for health need to be recognized and considered as important aspects of ensuring a sustainable drug program.

Council members discussed the study shown by one presenter that documented an impressive gain in QALYs from physical exercise. Council would urge the government to consider prevention as a critical ingredient in achieving a public drug program that can be financially responsible and sustainable into the future. This would include establishing enabling environments that allow individuals to take more responsibility for their health, (e.g. walking or bike trails).
7.0 Conclusion

The Ontario Citizens’ Council responded thoughtfully and reflectively to the question posed for this session; “To what extent should the Ontario Public Drug Program consider QALYs in drug funding decisions?” and developed four broad recommendations.

Again, deliberations of the Council were rich and substantive, with new Council members fully integrated into the work of the Council. We anticipate that our recommendations will assist the Ministry as it continues to use QALYs as an important element of its drug funding decisions.
APPENDIX 1

MEMBERS OF THE ONTARIO CITIZENS’ COUNCIL

Benita Baker
Nigel Berrisford
Shelley Blidner
Jeff Bondett
Remy Boulbul
Beverly Browne
Lorna Bruce
Prem Dhir
Gerry Gershon (Chair Citizens’ Council)
Prem Lachhman
Sherry Marshall
Debbie Marson
Isabel Metcalfe
Dorothy Modritsch
Robert Moore
Nazih Nasrallah
Josephine Quercia
Mark Roberts
Abe Schwartz
Gary Spergel
Theresa Tasse
Marilyn Wood
# APPENDIX 2

## AGENDA

**QALYS AND DRUG FUNDING DECISIONS**

**CITIZENS’ COUNCIL**

Fairmount Royal York  
Toronto, ON  
June 14-15, 2013

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<td>Updates since Last Council Meeting</td>
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<td>Break</td>
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<td>Reflections on the Questionnaire</td>
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<td>8:00 am</td>
<td>Breakfast</td>
</tr>
<tr>
<td>8:30 am</td>
<td>Morning Check-in and Questions / Answers</td>
</tr>
<tr>
<td>8:45 am</td>
<td>Where Are We? Overview</td>
</tr>
<tr>
<td></td>
<td>Refining Common Ground</td>
</tr>
<tr>
<td>10:30 am</td>
<td>Break</td>
</tr>
<tr>
<td>10:50 am</td>
<td>Round Table: Field Testing Our Advice</td>
</tr>
<tr>
<td>11:45 am</td>
<td>Lunch</td>
</tr>
<tr>
<td>12:45 pm</td>
<td>Clarifying Council Advice</td>
</tr>
<tr>
<td>1:30 pm</td>
<td>Revisiting the Values Framework</td>
</tr>
<tr>
<td>2:15 pm</td>
<td>Preparing the Citizens Council report</td>
</tr>
<tr>
<td>2:45 pm</td>
<td>Wrap-up</td>
</tr>
<tr>
<td>3:00 pm</td>
<td>Adjournment</td>
</tr>
</tbody>
</table>
APPENDIX 3

BIOGRAPHIES OF PRESENTERS

**Doug Coyle** is a health economist and Professor in the Department of Epidemiology and Community Medicine at the University of Ottawa. Doug obtained his PhD from Brunel University in the UK. His research centres on the methods of conducting economic evaluation of health care interventions. He has published over 100 peer reviewed papers and has taught graduate programs in health economics for over fifteen years. He is a past member of the Scientific Advisory Panel of the Canadian Agency for Drugs and Technology in Health and currently mentors health economists in this agency. He has advised Health Canada, the Public Health Agency of Canada and various provincial ministries of health with respect to the cost effectiveness of health care interventions. He is a member of the Ontario Ministry of Health and Long Term Care's Committee to Evaluate Drugs.

**Jennifer Gibson** is the Interim Director for the Joint Centre for Bioethics at the University of Toronto, where she is also Associate Professor in the Institute of Health Policy, Management, and Evaluation. In 2012, she was the inaugural recipient of the Ross Upshur Award for Excellence in Course Direction.

**Dr. Allan Grill** is an Assistant Professor in the Department of Family and Community Medicine at the University of Toronto, and enjoys an active community-based clinical practice as part of the Markham Family Health Team. Dr. Grill also teaches medical students and family medicine residents at Sunnybrook Health Sciences Centre in Toronto where he is a part-time physician in the Division of Long-Term Care.

As the current Chair of the Committee to Evaluate Drugs (CED), an expert advisory committee to the Ontario Ministry of Health & Long-Term Care on drug policy issues, as well as being a member of the pan-Canadian Oncology Drug Review Expert Review Committee (pERC), Dr. Grill has experience in the critical appraisal of drug therapies as well as evaluating funding decisions for public formularies.

**Diane McArthur** is Assistant Deputy Minister and Executive Officer of Ontario Public Drug Programs, appointed in June 2010. Ms. McArthur has been the Assistant Deputy Minister responsible for seniors’ issues within the Government of Ontario, and Executive Coordinator of Health and Social Policy in the Cabinet Office of the Government of Ontario.

Ms. McArthur has held progressively more senior positions in several ministries since joining the Government of Ontario as a Management Intern in 1989. She has extensive experience in human resources, health policy and planning for health provider training, data and health information planning and analysis, health care provider negotiations, rural health policy, labour relations and service delivery restructuring.

Ms. McArthur has a Bachelor of Public Relations degree from Mount Saint Vincent University in Halifax and a Master of Business Administration degree from the University of Ottawa.

**Christopher McCabe** holds the Endowed Research Chair in the School of Community based Medicine, at the University of Alberta. He is a health economist with over 20 years experience of working with health care funders, clinicians and researchers on the efficient allocation of
limited health care resources. He has previously held Chairs in Health Economics the Universities of Leeds, Warwick and Sheffield.
APPENDIX 4

Ontario Citizens Council
Preliminary Values Framework,
Developed From Council Meeting of June 17-19, 2011

EXECUTIVE SUMMARY
The Ontario Citizen’s Council is composed of 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 Drugs Program (OPDP).

To assist in this mandate, the Council has begun to develop 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 the Council’s advice.

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 should be done. They often set standards or 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 started this process and 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 what we care about as Ontarians to the OPDP. 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 preliminary 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 program in a manner that is fiscally responsible and accountable to taxpayers and contributes to the fostering of a sustainable health system for the health 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)

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 the real-life experience of both 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):

Science-Oriented
- Evidence-based decision-making
- Advancing medical knowledge
- Shared responsibility

Economic-Oriented
- Fiscal responsibility
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), the following six values seemed both high priorities and demanding of greater clarity. While the work to understand and clearly define these values in relationship to OPDP has only started, the following descriptions 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 particular 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 as well as 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.

**Compassion**

- While this 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 patients’ perspective needs to be considered and balanced along with medical expertise. 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.

PRINCIPLES
As we have 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.
CONCLUSION

The values framework will be an important contribution to the Council’s future 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, if 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 Drugs Program.
## APPENDIX 1 (Of the Values Framework)

### VALUES HIGHLIGHTED FROM PREVIOUS COUNCIL DISCUSSIONS

<table>
<thead>
<tr>
<th>SESSION: DRUGS FOR RARE DISEASES</th>
<th>SESSION: MANAGING THE FORMULARY</th>
</tr>
</thead>
<tbody>
<tr>
<td>Compassion</td>
<td>Compassion and the Importance of Quality of Life:</td>
</tr>
<tr>
<td>Society must care for its vulnerable members. There must be mechanisms to allow funding of their treatment.</td>
<td>Consideration needs to be given to any undue discomfort and inconvenience that a delisting decision may result in for individual patients.</td>
</tr>
<tr>
<td>Equity and Fairness</td>
<td>Equity and Fairness</td>
</tr>
<tr>
<td>Equity and fairness do not imply equality. Resources should benefit a wide majority of the population at the same time as there must be provision for those with rare diseases.</td>
<td>Reaffirmed from previous session. ²</td>
</tr>
<tr>
<td>Balance the Common Good with the Needs of Particular Individuals</td>
<td>Balance the Common Good with the Needs of Particular Individuals</td>
</tr>
<tr>
<td>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.</td>
<td>Reaffirmed from previous session.</td>
</tr>
<tr>
<td>Fiscal Responsibility</td>
<td>Fiscal Responsibility</td>
</tr>
<tr>
<td>Decisions must be made responsibly. The legislation mandates that dollars be spent wisely.</td>
<td>Reaffirmed from previous session.</td>
</tr>
<tr>
<td>Accountability to Taxpayers</td>
<td>Accountability to Taxpayers</td>
</tr>
<tr>
<td>The public drug program uses public funds and taxpayers are entitled to know how these funds are used.</td>
<td>Reaffirmed from previous session.</td>
</tr>
<tr>
<td>Advance Medical Knowledge</td>
<td>Advance Medical Knowledge</td>
</tr>
<tr>
<td>All observations on the use of a drug become part of the bank of knowledge that can support continuous improvement of treatments for all citizens requiring that drug.</td>
<td>All observations on the use of a drug become part of the bank of knowledge that can support continuous improvement of treatments and alert the medical community to potential harmful effects of a drug that might only emerge as the drug is used in the real world.</td>
</tr>
</tbody>
</table>

² Values noted as having been reaffirmed were not elaborated on further in the plenary session in terms of their direct application to formulary management.
### SESSION: DRUGS FOR RARE DISEASES

<table>
<thead>
<tr>
<th>Evidence-Based Decisions</th>
<th>Evidence-Based Decisions</th>
</tr>
</thead>
<tbody>
<tr>
<td>Decisions on drugs for rare diseases must rely on acceptable evidence for that category of drug.</td>
<td>Decisions must be made on a full range of evidence, both positive and negative. This includes evidence based on ongoing reporting of adverse effects.</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Shared Responsibility</th>
<th>Shared Responsibility/Collaboration</th>
</tr>
</thead>
<tbody>
<tr>
<td>All parties need to collaborate to ensure that funds are used responsibly.</td>
<td>All parties have a role to play in ensuring the ongoing viability and effectiveness of the Formulary system. In particular, this refers to building and providing up-to-date and balanced information related to the risks and benefits of drugs.</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Importance of Quality of Life and Longevity</th>
<th>See above value: compassion</th>
</tr>
</thead>
<tbody>
<tr>
<td>This is an important way to look at the effectiveness of the drug when deciding if it warrants funding.</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Public Safety</th>
<th>Informed Public</th>
<th>Transparency</th>
</tr>
</thead>
<tbody>
<tr>
<td>Decisions that err on the side of caution need to be taken effectively and in a timely manner, when there is emerging evidence that a drug already on the Formulary has harmful effects.</td>
<td>It is essential that members of the public, patients, and their medical professionals have the information they need to make informed decisions about taking a drug, staying on a drug or discontinuing its use.</td>
<td>The decision-making process for both listing and delisting drugs must be fully transparent. Full disclosure of research findings is essential. There must be a transparent plan for removing drugs from the Formulary if they are found to be harmful or ineffective.</td>
</tr>
</tbody>
</table>

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3 The Council defined parties broadly to include – the federal and provincial governments, drug companies, and medical professionals (including pharmacists).
**APPENDIX 5**

**EURO QoL OR EQ-5D QUESTIONNAIRE**

By placing a tick in one box in each group, please indicate which statement best describes your health today.

**MOBILITY**

<table>
<thead>
<tr>
<th>Statement</th>
<th>Option</th>
</tr>
</thead>
<tbody>
<tr>
<td>I have no problems in walking about</td>
<td>☐</td>
</tr>
<tr>
<td>I have some problems in walking about</td>
<td>☐</td>
</tr>
<tr>
<td>I am confined to bed</td>
<td>☐</td>
</tr>
</tbody>
</table>

**SELF-CARE**

<table>
<thead>
<tr>
<th>Statement</th>
<th>Option</th>
</tr>
</thead>
<tbody>
<tr>
<td>I have no problems with self-care</td>
<td>☐</td>
</tr>
<tr>
<td>I have some problems washing or dressing myself</td>
<td>☐</td>
</tr>
<tr>
<td>I am unable to wash or dress myself</td>
<td>☐</td>
</tr>
</tbody>
</table>

**USUAL ACTIVITIES**

<table>
<thead>
<tr>
<th>Statement</th>
<th>Option</th>
</tr>
</thead>
<tbody>
<tr>
<td>I have no problems with performing my usual activities</td>
<td>☐</td>
</tr>
<tr>
<td>I have some problems with performing my usual activities</td>
<td>☐</td>
</tr>
<tr>
<td>I am unable to perform my usual activities</td>
<td>☐</td>
</tr>
</tbody>
</table>

**PAIN/DISCOMFORT**

<table>
<thead>
<tr>
<th>Statement</th>
<th>Option</th>
</tr>
</thead>
<tbody>
<tr>
<td>I have no pain or discomfort</td>
<td>☐</td>
</tr>
<tr>
<td>I have moderate pain or discomfort</td>
<td>☐</td>
</tr>
<tr>
<td>I have extreme pain or discomfort</td>
<td>☐</td>
</tr>
</tbody>
</table>

**ANXIETY/DEPRESSION**

<table>
<thead>
<tr>
<th>Statement</th>
<th>Option</th>
</tr>
</thead>
<tbody>
<tr>
<td>I am not anxious or depressed</td>
<td>☐</td>
</tr>
<tr>
<td>I am moderately anxious or depressed</td>
<td>☐</td>
</tr>
<tr>
<td>I am extremely anxious or depressed</td>
<td>☐</td>
</tr>
</tbody>
</table>