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Chair
Ontario Citizens' Council

March 27, 2010

Helen Stevenson
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Dear Helen Stevenson:

I am writing on behalf of the Ontario Citizens' Council to submit our recommendations to you on the question we were asked to consider at our meeting in January 2010: ***"Under what situations and /or conditions should the Ontario Government (i.e. taxpayers) pay for Drugs for Rare Diseases"***.

The members of the Citizens' Council feel privileged to have been selected for the important task of providing advice on drugs for rare diseases to the Ontario Public Drug Programs, one of the programs administered by the Ontario Ministry of Health and Long-Term Care.

This Ontario-oriented analysis took place in the shadow of the ever-present U.S. health care debate, which provided a sobering reminder of just how heated health care discussions can become. Fortunately, the atmosphere in which the Ontario Citizens' Council deliberations took place was respectful and productive. Our deliberations led to the recommendations contained within the body of this report.

The speakers from the medical and health care communities stressed that the deliberations of the Council were crucial, given the number of rare diseases in their sights, the challenges in making fair and transparent decisions for funding drugs for their treatment, and the pressures and motivations for pharmaceutical companies to develop and market drugs for rare diseases.

The impact of adding to the lists of available medications is, of course, a benefit to patients, at the same time as it creates financial strains upon the health care system. So clear was this reality to the Citizens' Council that the group felt compelled to recommend that the

Ministry develop a transparent plan for removing from the formulary drugs that are found to no longer be effective. We realize that this recommendation is outside the question posed to the Council. However, this may leave financial room to add new medications as needed.

The Council accepted that drugs for rare diseases cannot meet the usual criteria for effectiveness and efficacy. We endorse the proposed model for funding drugs for rare diseases which takes into account the inherent difficulty of approving drugs for low-incident diseases. However, there still remains a problem for which there is no easy solution – assessing a patient’s potential quality of life as well as his or her potential longevity, with or without the drug. Finally, we feel that a sympathetic and compassionate assessment must be made of the effects of the disease and its treatment, not only upon the patient, but also upon the immediate family.

The Council felt that the Ministry’s record with respect to developing, acquiring and publicly distributing drugs for rare diseases was well thought out. At the same time, the Council, at the Ministry’s request, responded to a series of critical questions to be considered in making decisions about funding drugs for rare diseases. These questions are answered in the section that closes this Report.

Unless the Ministry of Health and Long-Term Care provides drugs to every person with a rare disease, the Ministry and its officers will always be in the uncomfortable position of explaining to a sufferer and his or her loved ones that a “bureaucratic government department” has disallowed treatment. It will not be sufficient to remind a patient that the Ministry’s “budget suitcase” can hold only so much without forcing the Government to cut other services. The reasons proffered may be perfectly logical, bureaucratically defensible and even scientifically sound, but they will not likely be easily accepted on the level where most people in this situation find themselves – the level of their emotions.

To provide some help to health care professionals in dealing with this highly charged situation, the Council urges the Ministry to concentrate on raising awareness of its policies and procedures for approving drugs and removing old ones. These explanations should be aimed not only at those suffering from rare diseases, but also to the public at large.

We thank you for the opportunity of taking part in this interesting and important discussion.

Respectfully,

Gerri Gershon, Chair
The Ontario Citizens’ Council

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A REPORT OF THE ONTARIO CITIZENS' COUNCIL

CONSIDERATIONS FOR FUNDING DRUGS FOR RARE DISEASES

Submitted to:

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March 31, 2010

Submitted by:

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RELEVANT WEBSITES

For information about the Citizens' Council, including its Terms of Reference and its members, [click here](#) or see

www.health.gov.on.ca/en/public/programs/drugs/councils/citizens_council.aspx

For information about how drugs are approved in Ontario, [click here](#) or see

www.health.gov.on.ca/english/providers/program/drugs/how_drugs_approv/how_drugs_approv.html

For information about rare disorders, [click here](#) or see the website of the Canadian Organization for Rare Disorders (CORD) www.raredisorders.ca/links.html

For a glossary of terms related to Ontario Public Drug Programs, [click here](#) or see

www.health.gov.on.ca/english/providers/program/drugs/dr_glossary/opdp_glossary.pdf

For information about the Ontario Drug Benefit Program, [click here](#) or see

www.health.gov.on.ca/en/public/programs/drugs/funded_drug/

For information about the Ministry's Exceptional Access Program, [click here](#) or see

www.health.gov.on.ca/english/providers/program/drugs/odbf/odbf_except_access.html

For information about the Ministry's Compassionate Review Policy, [click here](#) or see

http://www.health.gov.on.ca/english/providers/program/drugs/pdf/compassionate_review_policy.pdf

For information about the Committee to Evaluate Drugs, [click here](#) or see

www.health.gov.on.ca/english/providers/program/drugs/how_drugs_approv/funding_ced.html

For presentations to the Citizens' Council about rare diseases, [click here](#) or see:

http://www.health.gov.on.ca/en/public/programs/drugs/councils/citizens_council.aspx

EXECUTIVE SUMMARY

The Ontario Citizens' Council is composed of twenty-five Ontarians from all walks of life appointed by the Minister of Health and Long-Term Care to provide their views on the values that reflect the needs, culture and attitudes of Ontario citizens about government drug policy. The Council reports to the Executive Officer of Ontario's Public Drug Programs and to the Minister.

At its first substantive meeting in January 2010, the Council was mandated to provide advice on the values that should influence decisions on the funding of drugs for rare diseases.

Through presentations by experts and by patients with rare diseases and through facilitated discussions, council members identified several significant societal values and offered specific recommendations on each. They also reviewed a proposed model for the evaluation of drugs for rare diseases in the light of these values and principles. Members agreed that using this proposed model to evaluate drugs in the particular context of rare diseases would result in funding mechanisms that respect the recommended values.

The Executive Officer had asked Council members to provide advice on particular aspects of funding drugs for rare diseases. The advice was developed on the basis of the recommended values and principles.

The Council made the following recommendations:

1. Drugs for rare diseases should have their own set of funding criteria.
2. There must be different standards for the approval of drugs for rare diseases.
3. The common good of the majority of the population must take into consideration the needs of the minority of citizens suffering from rare diseases.
4. The competing needs for health care dollars mean that there should be an attitude fostering prudent fiscal management for drugs for rare diseases.
5. Decisions regarding funding for rare diseases need to be transparent, as does the rationale for them.
6. The medical community must document the progression of the disease when a drug is administered to sufferers of rare diseases. Sharing national and international research is essential, particularly for rare diseases.
7. 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.

8. Beneficiaries of drugs for rare diseases, their families and their caregivers and their health care professionals share the responsibility to use the drugs properly, to monitor, and to report on its effectiveness.
9. Quality of life and potential lifespan must underpin all consideration of funding for drugs for rare diseases.
10. The Ministry should raise awareness of its policies and procedures for approving and adding new drugs to the Formulary and removing old ones; these explanations should not only be focused on those suffering from rare diseases, but also directed to the public at large.
11. A definition of rare diseases must be established by experts and publicized. A national standard would be desirable.
12. The treatment must be monitored throughout to assess continued effectiveness and to add to medical knowledge. Patients must follow the prescribed regimen.
13. The patient, his or her caregivers and health care professionals share the responsibility to make data collection possible. The patient must agree – in a contractual arrangement - to participate in monitoring studies.
14. Funding for an individual patient can start if the patient has been diagnosed, is likely to benefit, and agrees to abide by conditions set by the Ministry experts even though the process for adding an approved critically-needed drug to the Formulary may not be completed.
15. Funding for an individual patient should be stopped if the drug is no longer effective or if, even with appropriate support, the patient does not comply with the contractual conditions of monitoring and of following the prescribed regimen.
16. The Ministry should develop a transparent plan for removing drugs from the Formulary that are found to be no longer effective

The Ontario Citizens' Council is the first of its kind in Canada and this experience may be helpful to other jurisdictions. This report therefore includes a description of the process used to assist members in arriving at productive conclusions.

1. THE QUESTION

In 2007 the Ministry of Health and Long-Term Care recognized that a new approach was needed for the funding of drugs for rare diseases. The Executive Officer convened a working group to recommend a new model for the review of drugs for rare diseases, a model that would ensure an impartial, systematic decision process. At the recommendation of the working group, the Executive Officer asked the Ontario Citizens' Council to report on the conditions under which taxpayers should fund drugs for rare diseases, with an emphasis on societal values.

The first substantive session of the Ontario Citizens' Council focused on this issue of "Drugs for Rare Diseases" and took place during the weekend of January 29 to 31, 2010. The Citizens' Council was asked to consider the following question:

Under what situations and /or conditions should the Ontario Government (i.e. taxpayers) pay for Drugs for Rare Diseases?

See Appendix 1 for information about the Citizens' Council.

The discussion was framed around the following considerations:

- Providing advice about the conditions under which funding should take place (as well as the values that should underpin the funding decisions).
- Recommending whether collecting data or monitoring a patient's condition while taking such drugs should be mandatory.
- Providing advice on the use of criteria for starting and/or stopping the public funding for use of a drug.
- Patient responsibility regarding routine physician follow-up towards the goal of safety and effectiveness monitoring, and long-term data collection for treatment.
- Providing societal values that might become part of the drugs for rare diseases framework.

2. PREPARING FOR DELIBERATIONS

To prepare the Citizens' Council for its deliberations, presentations by medical professionals, economists, a pharmaceutical firm spokesperson and patients suffering from rare diseases were delivered on relevant topics, including:

- Drugs for Rare Diseases: Why Advice is Needed
- Defining Rare Diseases
- Living with Rare Diseases

- Developing Drugs for Rare Diseases
- Funding Drugs for Rare Diseases: Why it is such a challenge

See Appendix 2 for the session agenda, including a list of presenters. For presentations to the Citizens' Council, [click here](#) or see

http://www.health.gov.on.ca/en/public/programs/drugs/councils/citizens_council.aspx

For a glossary of relevant terms, [click here](#) or see

www.health.gov.on.ca/english/providers/program/drugs/dr_glossary/opdp_glossary.pdf

Council members particularly noted the following:

- A. The limited evidence available for drugs for rare diseases. Because there are so few cases of each rare disease, these drugs do not meet the traditional benchmarks of the Committee to Evaluate Drugs, including stringent clinical studies and trials.
- B. The concept of Quality Adjusted Life Years (QALY). This standard concept is used in assessing effectiveness of drugs and comparing health outcomes; it assigns each year of life a weight from 1 (perfect health) to 0 (judged the equivalent of death) dependent on the individual's health-related quality of life during that year. A total score of years multiplied by weight can then be compared across different interventions.
- C. The compelling image of a suitcase with limited capacity as a metaphor for the finite budget for drug funding - how to decide carefully what to pack and what to leave out. The Council was tasked with identifying the values that would determine what goes into the suitcase.

Questions and discussions followed the presentations, aimed at responding to the request from the Ministry to identify the values of significant importance in making funding decisions.

Several members of the public attended this session as observers.

3. CONSIDERATION OF DRUGS FOR RARE DISEASES

3.1 What is a Rare Disease?

The term "rare disease" applies to diseases that have a very low prevalence in the general population. In Canada, there is no national standard for defining a rare disease, though some countries have adopted clear definitions. Often these definitions were prompted by incentives of market exclusivity for the manufacture of drugs for rare diseases. In smaller countries, including Australia and Canada, drugs used to treat rare diseases are frequently manufactured in other countries. In Ontario, a working definition of 1 case per 100,000 is currently used.

For information on rare disorders, [click here](#) or see the website of the Canadian Organization for Rare Disorders (CORD) www.raredisorders.ca/links.html.

Some examples of Rare Diseases are:

- Tay-Sachs disease
- Hurler disease
- DiGeorge syndrome
- Hunter syndrome
- Leprosy (in Canada)

3.2 How are Rare Diseases Treated?

- Primary treatment often involves enzyme replacement therapy. In some cases this involves the development of a highly specific formula.
- Therapies are cumbersome because they often have to be administered by injection and usually require frequent trips to a hospital or clinic.
- They are expensive both in the cost of the drug itself and also in personal non-medical financial strain on affected individuals and their family members.
- There are too few patients scattered throughout the world to conduct standard clinical trials.
- It is difficult to determine the effectiveness of a new treatment because progression is slow and noticeable improvements can take a long time to become obvious.

3.3 Living with a Rare Disease

Sessions introduced Council members to three individuals, each of whom suffers from a rare disease or had a close family member with a rare disease - Neonatal-Onset Multisystem Inflammatory Disease (NOMID), Pompe Disease and Paroxysmal Nocturnal Hemoglobinuria (PNH). All are currently involved with publicly-funded drug programs. They spoke of their original diagnosis, personal experiences and their willingness to be involved with the data collection process in order to make life better for others who might have the same disease in the future. Each expressed their thanks for the treatments that are available and believe that treatment is increasing not only their quality of life but also their chances at longevity. See Appendix 3 for names of presenters in this session.

3.4 Funding Drugs for Rare Diseases

Ontario legislation dictates that drug funding decisions be based on the best clinical and economic evidence available, and that the decisions ensure that taxpayers' dollars are spent wisely. In order to be funded a drug must be listed on the Formulary. For information about the Ontario Drug Benefit Program, [click here](#) or see www.health.gov.on.ca/en/public/programs/drugs/funded_drug/

Drugs that are not listed in the Ontario Formulary are also considered for coverage through the Ministry's Exceptional Access Program on a case-by-case basis. [Click here](http://www.health.gov.on.ca/english/providers/program/drugs/odbf/odbf_except_access.html) or see www.health.gov.on.ca/english/providers/program/drugs/odbf/odbf_except_access.html. The criteria of the Compassionate Review Policy are also relevant. [Click here](http://www.health.gov.on.ca/english/providers/program/drugs/pdf/compassionate_review_policy.pdf) or see http://www.health.gov.on.ca/english/providers/program/drugs/pdf/compassionate_review_policy.pdf

To assist the Executive Officer, the Committee to Evaluate Drugs (CED) reviews the drugs submitted by manufacturers for public funding. This committee of expert advisors reviews the scientific and clinical evidence submitted by the manufacturer as well as the impact on health services as compared to existing treatments. It evaluates whether the drug will provide good clinical value and good use of limited health resources if it is funded. The Committee to Evaluate Drugs makes recommendations to the Executive Officer who makes the funding decision within the parameters of the legislation. For information about the Committee to Evaluate Drugs, [click here](http://www.health.gov.on.ca/english/providers/program/drugs/how_drugs_approv/funding_ced.html) or see www.health.gov.on.ca/english/providers/program/drugs/how_drugs_approv/funding_ced.html.

In many cases, drugs for rare diseases cannot meet the benchmarks established for inclusion in the Formulary. By definition, few people have the disease and as a result there is little long-term information available to evaluators. There are not enough patients to conduct formal clinical trials that could provide strong evidence of efficacy using statistical methods. The cost of these drugs is usually very high due to the small market from which to recover the high costs of development. It is difficult to demonstrate that the use of these drugs will be cost-effective. As a result, many drugs for rare diseases cannot be approved under the current funding model. **A specific model is needed for funding drugs for rare diseases.**

4. HOW THE COUNCIL DID ITS WORK

Following the information sessions, the Citizens' Council turned its attention to the question it was asked to consider:

Under what situations and /or conditions should the Ontario Government (i.e. taxpayers) pay for Drugs for Rare Diseases?

Following the presentations, the Council broke into two groups. These were closed deliberations with only Citizens' Council members and facilitators present. The goal was to have thoughtful conversations about the topic and to begin to identify common ground as well as areas of difference on matters that could be reported back to the Ministry. It should be noted that while Council members freely aired their views, all members did not agree with every opinion shared. However, the point was to highlight commonly agreed understandings and also to note places where members held different views.

Under the guidance of facilitators the Council engaged in Deliberative Dialogue. This is a structured method of discussion which presents a number of approaches as tools for dialogue. The approaches stress different priorities and principles and are developed to

promote thoughtful dialogue on a current and challenging issue. The approaches are not mutually exclusive and are not policy options, nor do they offer “right” or “wrong” answers. In deliberative dialogue, each person is able to explore the issue through sharing his or her views and listening and learning with others. See Appendix 4 for a brief overview of Deliberative Dialogue.

4.1 Four Ways to Think About Funding Drugs for Rare Diseases

In the Deliberative Dialogue, the Council considered four approaches to the matter of funding drugs for rare diseases:

- Benefit the most people
- Fulfill society’s responsibility to care for the most vulnerable
- Focus on advancing medical knowledge under safe conditions
- Use resources where they can be most effective

Each approach was examined in turn. These were intended to be the foundation for generating discussion about the pros and cons of each approach, what Council members liked or appreciated as well as what concerned them. The goal was to arrive at the values and principles ordinary people, like the Ontario Citizens’ Council members, would want governments to take into account as they make decisions about funding drugs for rare diseases.

While no one approach totally captured the values that most members held dear, taken together the approaches supported Council members in identifying the conditions that they want decision-makers to consider when funding drugs for rare diseases. For example, although there was a general agreement that drug funding should benefit the largest number of people, there was some tension as to the role compassion should play in the formation of drug policy that may address only a small number of people. While resources of the government should be spent where they would be most effective, society should also fulfill its responsibility to care for the vulnerable.

4.2 Highlights from Each Approach

Approach 1: Benefit The Most People

By their very nature, drugs for rare diseases do not serve large groups of people. During the deliberative dialogues, members reached consensus that compassion was an acceptable societal reason for finding a way to fund drugs for rare diseases, even though such funding benefitted a small number of people compared to other potential uses for the funding. However, people also felt that compassion needed to be tempered with the reality that the drug budget was limited with many other worthy needs to fund and that drug effectiveness should still be a crucial consideration. There still needs to be some level of evidence that the drugs will be useful in managing the condition they are intended to address.

Approach 2: Fulfill Society's Responsibility To Care For The Most Vulnerable

It was agreed that equality is not the same as equity. By contrast, fairness and equity to all patients, including those with a rare disease, were very important. There was much discussion around the idea that society can't be responsible for *every* need. Some people felt that financial limits had to be set on how much should be spent to fund drugs for rare diseases. However, while recognizing that these drugs are very expensive, others countered that setting a 'cap' was unfair to patients with rare diseases, especially since the total number of patients at present is relatively small. As more knowledge is gained, the number of patients in that category will likely increase.

Approach 3: Focus On Advancing Medical Knowledge Under Safe Conditions

Given the high cost of drugs for rare diseases, Council members agreed that there should be a contractual arrangement between the doctor/medical team and the patient. During treatment, information about the rare disease would be culled from the patient, to benefit others with the disease, the medical profession and society as a whole. This gathering of information would be an important way to advance medical knowledge. This was seen as particularly crucial since so much is still to be learned about rare diseases. Naturally, the patient's co-operation would be necessary to collect this information. Lack of co-operation in any part of the process would be considered reason for the government to stop paying for the drug for that patient. One member emphasized the importance for the medical team to ensure that vulnerable patients have adequate assistance from their families, if possible, or from community services, to ensure the patient's likelihood of success in fulfilling his or her side of the contract.

Approach 4: Use Resources Where They Can Be Most Effective

Members felt that quality of life and prevention of early death due to the rare disease, were key elements to be considered in a decision about whether or not to fund a drug.

There was consensus that medical professionals should take these points into account when making decisions about a drug's effectiveness.

5. COUNCIL VIEWS ON RELEVANT SOCIETAL VALUES AND RELATED RECOMMENDATIONS

On the final morning, participants concentrated on comparing the values and conclusions of the small group deliberations of the previous day. They worked to draw out and deepen areas of agreement across the two groups.

During the discussion, there was agreement on the need for compassion, fairness and equity as well as fiscal responsibility and accountability to tax payers. Council members recognize that there are complex tensions between individual rights and societal rights. The government must strive to strike the appropriate balance. Individuals have a right to

receive assistance from the government regardless of their disease; at the same time, public funds are limited and should benefit all citizens.

The Citizens' Council agreed on the following values and principles that should inform decisions about public funding of drugs for rare diseases. The Council recommended an approach based on these values and principles and endorsed recommendations associated with each one:

5.1 Compassion

Society must care for its vulnerable members. There must be mechanisms to allow funding of their treatment. The Ministry already has a compassionate review policy regarding exceptional access.

RECOMMENDATION 1: *Drugs for rare diseases should have their own set of funding criteria.*

5.2 Equity and Fairness

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.

RECOMMENDATION 2: *There must be different standards for the approval of drugs for rare diseases.*

5.3 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 exercise prudent management of available resources for the benefit of all.

RECOMMENDATION 3: *The common good of the majority of the population must take into consideration the needs of the minority of citizens suffering from rare diseases.*

5.4 Fiscal Responsibility

Decisions must be made responsibly. The legislation mandates that dollars be spent wisely.

RECOMMENDATION 4: *The competing needs for health care dollars mean that there should be an attitude fostering prudent fiscal management for drugs for rare diseases.*

5.5 Accountability to Taxpayers

The public drug program uses public funds and taxpayers are entitled to know how these funds are used.

RECOMMENDATION 5: *Decisions regarding funding for rare diseases need to be transparent, as does the rationale for them.*

5.6 Advance Medical Knowledge

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.

RECOMMENDATION 6: *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.*

5.7 Evidence-Based Decisions

Decisions on drugs for rare diseases must rely on acceptable evidence for that category of drug.

RECOMMENDATION 7: *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.*

5.8 Shared Responsibility

All parties need to collaborate to ensure that funds are used responsibly.

RECOMMENDATION 8: *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.*

5.9 Importance of Quality of Life and Longevity

This is an important way to look at the effectiveness of the drug when deciding if it warrants funding. Quality Adjusted Life Years is a widely used concept for the discussion of the effectiveness of drugs.

One member did not agree to include longevity when considering funding.

RECOMMENDATION 9: *Quality of life and how long a person lives must underpin all consideration of funding of drugs for rare diseases.*

5.10 Other Areas of Concern

In discussions, members explored several areas that could also be considered when developing decisions on the funding of drugs for rare diseases.

CAPPING EXPENSES: There were a variety of opinions on the need to “cap” expenses for drugs for rare diseases. Some believe that all those who need drugs for rare diseases should receive them. Others caution that scarce resources must be carefully managed and controlled so that patients with “common” diseases are not deprived.

INCREASING AVAILABLE FUNDS: This led the group to suggest various ways of increasing the budget available for such drugs, such as tapping external sources, taxing certain lifestyles, systematic review, delisting of drugs when necessary and bulk buying on a national or even international level.

COST SHARING: It was also suggested that the Ministry explore cost sharing among stake holders, including the patient, sponsors, the drug company and the government.

CRITERIA IN THE EVENT OF LIMITED SUPPLY: In a situation where there is a limited supply of a drug, Council members suggested that criteria would be needed to decide who among qualified recipients would actually receive it.

It was concluded that, while these questions were beyond the mandate of Council, they should be mentioned for consideration by the Ministry.

Council members expressed concern about the time lag that can occur between the end of a “trial” period and the actual availability of the drug on the Formulary, wanting to ensure that patients who are benefiting are not “cut off”. However, officials assured the members that this was not an issue, particularly in the case of rare diseases.

Council members felt strongly that it is important to share information with the public about rare diseases and how Ontario makes decisions about drugs to treat them, and made the following recommendation:

RECOMMENDATION 10: *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.*

Council members recognized that the definition of a rare disease is interpreted differently by various groups and individuals, and made the following recommendation:

RECOMMENDATION 11: *A definition of rare diseases must be established by experts and publicized. A national standard would be desirable.*

6. PROPOSED MODEL FOR FUNDING DRUGS FOR RARE DISEASES

Over the last 2 years, the Working Group on Drugs for Rare Diseases has been developing a new approach to drugs for rare diseases.

Their model is based on the “best *achievable* evidence” to predict the progression of a disease and the potential benefit or lack of benefit of a drug treatment in terms of slowing the disease progression in specific groups of patients.

The model uses a working definition of a rare disease as 1 case in a population of 100,000. It uses an evidence-based approach to review the natural history of the disease. The model includes an evaluation of the total budget impact and cost, not in terms of cost-effectiveness (since drugs for rare diseases will never be cost-effective), but in terms of benefit in quality of life and longevity. Affordability is a factor, however, since total budget impact will be affected by factors such as incidence/prevalence of the disease, changes in screening tests leading to an increased discovery of cases, dose and dosing schedule, particularly if weight-based.

Finally, the model provides for the identification of additional follow-up data, depending on the specific drug and disease. The model uses available medical evidence as well as patient (and care-giver) input. To date two diseases have been “put through” the model and one disease is in the process.

Some elements of the model, particularly affordability and need for additional follow-up data, lead to decisions that must also be framed by the values of society. The Ontario Citizens’ Council was mandated to provide advice on these societal values.

7. COUNCIL RESPONSES TO THE MODEL AND EXECUTIVE OFFICER’S QUESTIONS

Following presentation of the model, Council members reviewed their deliberations to determine how well their values and recommendations fit with the model. Members agreed that the model is in harmony with the values identified by the Council.

The model acknowledges that available evidence cannot be as solid as that provided by formal clinical trials and is built to reflect the best achievable evidence. When applying this model in funding decisions, the Executive Officer must consider individual cases and decide for each:

- Under what conditions it is appropriate to fund the drug for a certain patient;
- Whether the patient should be expected to accept systematic monitoring and reporting;
- What the conditions are for starting the funding of a treatment;
- Whether – and under what circumstances - it is acceptable to stop funding of a drug for an individual patient.

In summary the following reflect the answers to the questions posed, based on the values of Council.

7.1 Conditions under Which Funding Should Take Place

Keeping in mind the values of compassion, equity, accountability, fiscal responsibility, shared responsibility and quality of life, Council members made the following recommendation:

RECOMMENDATION 12: *The treatment must be monitored throughout, to assess continued effectiveness and to add to medical knowledge. The patient must follow the prescribed regimen.*

7.2 Mandatory Data Collection and Monitoring

Keeping in mind the values of balancing the requirements of the common good, evidence-based medical knowledge and shared responsibility, Council members made the following recommendation:

RECOMMENDATION 13: *The patient, his or her caregivers and health professionals share the responsibility of making the data collection possible. The patient must agree – in a contractual arrangement - to participate in monitoring studies.*

7.3 Criteria for Starting To Fund the Drug for a Patient

Keeping in mind the values of compassion, fairness and the need to balance the common good with the need of particular individuals, Council members made the following recommendation:

RECOMMENDATION 14: *Funding for an individual patient can start if the patient has been diagnosed, is likely to benefit and agrees to abide by conditions set by the Ministry experts, even though the process for adding an approved critically needed drug to the Formulary may not be completed.*

The issue of choosing from among qualified patients if there is a limited supply of a drug was suggested as requiring further discussion.

7.4 Criteria for Stopping the Funding For a Patient

Keeping in mind the values of fiscal responsibility, equity, accountability, shared responsibility and quality of life:

RECOMMENDATION 15: *Funding for an individual patient should be stopped if the drug is no longer effective or if, with appropriate support, the patient does not*

comply with the contractual conditions of monitoring and of following the prescribed regimen.

Council members also felt that it was important to develop a plan for removing drugs from the Formulary.

Keeping in mind the values of fiscal responsibility, accountability, fairness, balancing the needs of the common good with the needs of particular groups, and in order to make financial room for new and more effective drugs, Council members made the following recommendation:

RECOMMENDATION 16: *The Ministry should develop a transparent periodic and systematic review for removing from the Formulary drugs that are no longer found to be effective.*

8. CONCLUSION

The Ontario Citizens' Council accepted the challenge of responding to the question ***“Under what situations and/or conditions should the Ontario Government (i.e. taxpayers) pay for Drugs for Rare Diseases?”*** The Council determined the values that should be considered when the Ministry decides what goes into funding drugs for rare diseases and developed the foregoing sixteen relevant recommendations.

The discussion of the Council was rich and substantive. It is anticipated that the recommendations can act as a basis to assist the Ministry in making value-laden decisions about funding drugs for rare diseases.

APPENDIX 1

ONTARIO CITIZENS' COUNCIL

1. FORMATION

The Ontario Citizens' Council came into being with the passage of the "***Transparent Drug System for Patients Act***" which received Royal Assent on June 20, 2006. The act includes legislative, regulatory and policy changes aimed at strengthening accountability, promoting appropriate use of medications, improving patient access to drugs, ensuring better value for money and investing in innovative health system research.

These commitments included the formation of the Ontario Citizens' Council:

"The Minister shall establish a Citizens' Council whose duty shall be to ensure the involvement of patients in the development of pharmaceutical and health policy."

The formation of the Council is intended to demonstrate the government's commitment to meaningfully engage ordinary citizens in discussions about specific policy questions related to the province's public drug programs. It is also intended to build legitimacy, fairness and transparency into the "priority setting" process and ultimately into decisions about which drugs should be funded with taxpayers' money.

The Citizens' Council is the first of its kind in Canada and is modelled after the Citizens' Council of the National Institute for Clinical Excellence (NICE) in the UK.

2. SELECTION PROCEDURE

In June 2008 advertisements appeared in most of Ontario's major newspapers and on the website of the Ministry of Health and Long Term Care inviting Ontarians from all walks of life to participate in the Citizens' Council.

All interested citizens could apply. A short list of 200 was identified from the more than 800 applications. Following a rigorous screening process, twenty-five citizens plus a chair were chosen and appointed by Minister's letter to become founding members of the Ontario Citizens' Council.

3. CITIZENS' COUNCIL MANDATE

The Council's role is to provide advice to the Executive Officer of the Ontario Public Drug Programs. This advice will be considered, along with other input, in decisions taken by the Executive Officer and the Minister. Members are expected to express their views as members of the public and not represent the view of any specific interest, group or organization. The Council is not a decision-making body and it does not have to reach agreement as a group on issues.

Its advice is to be provided in a report that reflects the values, culture and attitudes of ordinary Ontario citizens. The report is to be delivered to the Executive Officer within sixty days of a meeting. The Executive Officer, in turn, is required to provide feedback to the Council outlining how

the advice will be applied by the government in developing drug funding policies and programs to ensure a sustainable and effective drug system for all Ontarians.

At its inaugural meeting in October 2009, the Citizens' Council's members were introduced to such topics as:

- The Councils' Mandate
- Ontario Public Drug Programs
- Access to Drug Funding in Ontario
- Approval process for funding drugs in Ontario
- Deliberative Dialogue

This session provided Council members with a common background on the Ontario Public Drug Programs, its operations and its current decision making processes, and helped orient them/prepare them for their advisory role on substantive policy matters.

APPENDIX 2

AGENDA DRUGS FOR RARE DISEASES

Intercontinental Toronto Yorkville Hotel
220 Bloor Street, West, Toronto, Ontario M5S 1T8

January 29th, 30th and 31st, 2010

TIME	ACTIVITY	PRESENTER
FRIDAY, JANUARY 29TH		
5:30 pm	Check-in Registration and Light Supper	
6:30 pm	Welcome / Possible Options for Councils Final Report	Chair, Gerri Gershon
OPEN SESSION		
7:00 pm	Drugs for Rare Diseases: Why Advice is Needed	Helen Stevenson
7:15 pm	What are rare diseases? The purpose of this session is to provide information about rare diseases, including: <ul style="list-style-type: none"> ▪ What are rare diseases ▪ What makes them rare ▪ Examples of rare diseases ▪ How much we know (and don't know) about them and what this means for treatment ▪ How they compare to "awful common diseases" 	Joe Clark, MD
8:00 pm	Questions and Answers	All
8:30 pm	Research Initiative	Janet Parsons, PhD
9:00 pm	Adjournment	
END OF OPEN SESSION FOR FRIDAY		
Please note that members may stay longer if they have additional questions about the weekend proceedings.		

SATURDAY, JANUARY 30TH		
8:00 am Breakfast		
8:30 am	Morning check-in and review of agenda Further Q&A based on evening's information	Chair, Gerri Gershon
OPEN SESSION		
9:00 am	Living with Rare Diseases: A Panel Discussion The purpose of this session is to address such questions as: <ul style="list-style-type: none"> ▪ What are the challenges that people with rare disorders face? ▪ What does it do to people? ▪ In what way are they sick? ▪ What is their life like? ▪ How does it make them feel? ▪ What are their symptoms? ▪ Do they die? ▪ What is the nature of treatment? ▪ What are the challenges of treatment? 	Michael Geraghty, MD Audrey Segal Jocelyn Gardner Ian Macpherson
9:30 am	Q & As	All
9:45 am	Developing Drugs for Rare Diseases Manufacturer's perspective	
10:10 am Break		
10:30 am	Funding Drugs for Rare Diseases: Why it is such a challenge The purpose of this session is to address such questions as: <ol style="list-style-type: none"> 1) <ul style="list-style-type: none"> ▪ How recommendations are currently made by the Committee to Evaluate Drugs (CED) perspective ▪ Why this doesn't work for drugs for rare diseases 2) <ul style="list-style-type: none"> ▪ Opportunity cost ▪ Health system cost 	Helen Stevenson, Gerald Evans, MD Jeff Hoch, PhD
11:30 am	Q & A's	All
END OF OPEN SESSION FOR THE WEEKEND		
11:45 pm Lunch		

12:30 pm	Introduction to deliberative dialogue about drugs for rare diseases	Miriam Wyman and Jacquie Dale
	Connecting to the issue: Personal Experiences	
12:45 pm	Deliberative dialogue: Four approaches Break included when needed	
4:00 pm	Identifying Common Ground and Areas of Divergence	
4:30 pm	How are we doing?	Chair, Gerri Gershon
4:45 pm	Wrap-up	Chair, Gerri Gershon
5:00 pm	Adjourn	Chair, Gerri Gershon

SUNDAY, JANUARY 31ST		
8:00 am Breakfast		
8:30 am	Morning check-in and review of agenda	Chair, Gerri Gershon
8:45 am	Where We're At: Sharing Our Common Ground	Miriam Wyman and Jacquie Dale
9:00 am	Testing Our Common Ground	Miriam Wyman and Jacquie Dale
10:15 am Break		
10:30 am	A new model for funding drugs for rare diseases Q & As	Gerald Evans, MD
11:15 pm	The new model and our common ground: How good is the fit?	Miriam Wyman and Jacquie Dale
12:00 pm Lunch		
12:45 pm	Clarifying Council advice for Helen <ul style="list-style-type: none"> ▪ Advice on the model ▪ Advice on the issue 	Chair, Gerri Gershon
1:45 pm	Preparing the Council report: How will we do this?	
2:30 pm	Wrap-up and evaluation	
3:00 pm	Adjournment	

APPENDIX 3

Living with a Rare Disease: Presenters

Audrey Segal presented on her personal experiences with Paroxysmal nocturnal hemoglobinuria (PNH)

Jocelyn Gardner presented on her 6-year-old grandson's (Mikey) experience with Neonatal-Onset Multisystem Inflammatory Disease (NOMID).

Ian Macpherson presented on his personal experience with Pompe Disease

APPENDIX 4

DIALOGUE GUIDELINES

GROUND RULES FOR DIALOGUE	
<p>The following ground rules can help us engage in good dialogue.</p> <ol style="list-style-type: none"> 1. The purpose of dialogue is to understand and to learn from one another (you cannot “win” a dialogue). 2. All dialogue participants speak for themselves, not as a representative of others’ interests. 3. In a dialogue everyone is treated as an equal: leave status and stereotypes at the door. 4. Be open and listen to others, especially when you disagree. Suspend judgement. 5. Identify and test assumptions (even your own). 6. Listen carefully and respectfully to the views of others: acknowledge that you have heard the other, especially when you disagree. 7. Look for common ground. 8. Express disagreement with ideas, not with personalities or motives (disagree without being disagreeable). 9. Respect all points of view. 	
DEBATE VS. DIALOGUE	
DEBATE	DIALOGUE
<ul style="list-style-type: none"> ▪ Assumes there is one right answer (and you have it) ▪ Attempts to prove the other side wrong ▪ Objective is to win ▪ Listening to find flaws ▪ Defends personal assumptions ▪ Criticizes others’ point of view ▪ Defends one’s views against others ▪ Searches for weaknesses and flaws in the others’ positions ▪ Seeks an outcome that agrees with your position 	<ul style="list-style-type: none"> ▪ Assumes that others have pieces of the answer ▪ Attempts to find common understanding ▪ Objective is to find common ground ▪ Listening to understand ▪ Explores and tests personal assumptions ▪ Examines all points of view ▪ Admits that others’ thinking can improve one’s own ▪ Searches for strengths and value in the others’ positions ▪ Seeks an outcome that creates new common ground