

Summary Report on:

Access to Prescription Drugs: What Every Patient Should Know

Workshop Report

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Access to Prescription Drugs: What Every Patient Should Know

Executive Summary

Purpose

This two-day workshop was designed to provide patients and other lay persons with a basic understanding of the drug research and development process, the regulatory review of risks and benefits, and health technology assessment, including cost-effectiveness analysis as applied to drug access. There were 28 patient representatives.



Cost-Effectiveness and Values-Based Decision-Making

Day 1: Regulatory Approval of Drugs

Participants began by brainstorming the “positive” and “negative” aspects of the Canadian drug system. Elisabeth Fowler (World Health Advocacy) provided an overview of the drug development process, including a discussion of incremental vs. breakthrough innovation, steps from discovery to submission, likelihood of success (1 in 5,000 to 1 in 10,000), and the costs estimated at \$1.5 billion over 15 years.

Durhane Wong-Rieger PhD (Consumer Advocare Network) introduced the basic concepts of “evidence-based” medicine, with an emphasis on how data about safety and efficacy are generated and interpreted. Clinical trials are designed to provide evidence of efficacy, while safety is “no evidence of significant harm.”

Agnes Klein, MD (Health Canada) provided an overview of the drug development process and walked through a submission review. She discussed the types of data submitted and the final approval as based on a favorable “benefit-to-risk” assessment.

Dr. Klein also provided the presentation on Health Canada's new "life-cycle" to drug regulation that recognizes the continued accumulation of knowledge about benefits and risks of drugs, especially in specific populations.

Day 2: Access to Drugs through Public Drug Plans

Participants brainstormed the criteria for assessing whether "new drugs" should be available through public drug plans and whether they should be funded. Their ideas included: evidence of benefits, quality of life impact, compassion, long-term cost savings, percentage of population affected, and improvement over existing drugs.

Durhane Wong-Rieger, PhD (Consumer Advocare Network) gave an overview of health technology assessment, the process for assessing drugs for appropriate use and cost-effectiveness. In theory, HTA processes should also take into consideration principles, values, and ethics as well as benefits, risks and costs. New drugs are generally compared with old drugs in terms of "improved outcomes" relative to "additional cost." The concept of the "quality-adjusted life year" was introduced. Three basic CEA methods were presented: cost-comparison, cost-effectiveness, and cost-utility analyses.

Brian O'Rourke, Vice President, CADTH provided a presentation on CADTH, the Common Drug Review and CADTH's plans for patient input into the process. He identified challenges as including: drugs for rare disorders; subsequent entry biologics; drugs with high cost-effectiveness ratios; definition of "first in class" drugs; use of surrogate end points; and drugs with limited evidence. CADTH is still struggling with the role of the public members (two on CEDAC).

Dr. Mona Sabharwal provided an overview of how the Ontario Public Drug Program makes decisions about drug funding. Funding decisions are made by the Executive Director. Recommendations are made by the Committee to Evaluate Drugs, which is comprised predominantly of physicians, but also has pharmacists, health economists and two patient members.

Participants were divided into small groups to simulate a "drug funding allocation" process. They were given a budget and six drugs and asked to decide which to fund, based on data provided about the diseases, the patient populations, the added benefit of the new drugs, and the costs. They reported on both their group process and the allocation decisions. Groups tended to fund drugs for diseases with no alternative treatments, for children, that significantly improved quality of life, and treated life-threatening conditions.

Participant Evaluation

Participants were very positive about the content and format of the two-day training session. They reported greater knowledge about the drug review processes, the methods used, and, as a result, greater confidence in the decisions made by governments. They also felt that they were better equipped to provide input to drug review agencies and to participate in the review processes.

Access to Prescription Drugs: What Every Patient Should Know

Introduction

Purpose

The purpose of this workshop was to address the need for patients and patient advocates to participate effectively in drug review processes. There are many benefits to having knowledgeable patients engaged in all drug review phases, from clinical trial design to regulatory review to funding decisions and optimal use guidelines to on-going monitoring. At an individual level, informed patients choose and use drugs more appropriately; at a societal level they help assure regulatory and funding decisions optimize health outcomes for all, and at a policy level, patient input contributes to the development of review processes that reflect patient and societal values. One barrier to patient participation is the increasing complexity of drug development and the concomitant review processes for safety, efficacy, quality, and cost-effectiveness. As agencies have introduced pathways for patient engagement (often in response to patient advocacy), patients are challenged to acquire the knowledge, technological expertise, and group skills to participate meaningfully. To those ends, this workshop was designed to provide basic understanding of the drug research and development process, the regulatory review of risks and benefits and health technology assessment, including cost-effectiveness analysis as applied to drug access.



Role of Regulator: Balance of Benefits and Risks

Participants

The 28 participants were recruited from disease-specific organizations and patient support groups and included patients, caregivers, staff, and board members. No previous knowledge or experience with drug development or review was required.

Format

The format was designed to maximize applied learning. Brainstorming and problem-solving techniques employing scenarios and case studies were combined with didactic presentations and examples. Participants worked in plenary sessions as well as small groups. They received introductory level presentations on four key topics: drug research and development process, evidence-based medicine, health technology assessment (cost-effectiveness analysis) and models of patient participation in HTA. In addition, representatives from Health Canada (regulatory agency), Common Drug Review (health technology assessment and cost-effectiveness analysis) and Ontario Public Drug Program (funding body) provided insights into their respective drug review processes.

Day 1 Summary: Research, Development and Review for Safety and Efficacy

Patient Perspectives on System for “Access to Drugs” in Canada

To help ground the discussions for the two days, participants began by brainstorming the “positive” and “negative” aspects of the Canadian drug system based on their personal experience or opinion. The following lists were generated.

Positive aspects of the current Canadian drug system

- New drugs are becoming available
- Through effective advocacy, access can be attained
- Drugs for more indications are becoming available
- Drugs are considered safe if approved by Health Canada
- There is increased awareness of the issues related to drug access from both patients and governments' perspectives
- Processes exist to help with access if you cannot afford drugs

Negative aspects of the current Canadian drug system

- Process is too slow, complex & bureaucratic
- No orphan drug plan in Canada
- Little understanding on the part of MPs on how the system works
- Unequal access across provinces
- No options for off-label use
- Step-wise process (need to fail one drug before gaining access to another) needs to be changed
- Process not respectful to patients
- Not enough consideration given to quality of life
- Efforts often duplicated; process is inefficient
- Patients have to ‘expose’ themselves in their advocacy (to gain access to the drugs they need)

Overview of drug development process

Elisabeth Fowler (World Health Advocacy) provided an overview of the drug development process, focusing on the following points.

Incremental vs. breakthrough innovation

- Breakthrough innovation is characterized by first-in-class, or new therapies; first to treat effectively a particular illness or address a particular indication. Examples include: Penicillin (first antibiotic); Insulin (first animal insulins); Thorazine (first anti-psychotic); first birth control pill
- Incremental innovation is “bit-by-bit”, cumulative improvement. Examples include: modern-day antibiotics (4 days, one pill/day), newer insulin therapies, modern birth control pills, etc.

Steps from discovery to regulatory submission

- Steps: Discovery, preclinical testing, Phase 1, Phase II, Phase III clinical trials, Regulatory approval, Post-market testing
- Length of time: Up to 12 years to get to regulator, with 2 to 6 years for each step

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Likelihood of success and costs through the process

- 5, 000 to 10,000 molecules are screened to yield 250 molecules that make it to preclinical testing and only 5 that progress to clinical trials and 1 that will get approval (success is 1 in 5,000 to 1 in 10,000)
- Costs (including failures) are estimated at \$1.5 billion over 15 years
- Most drugs that reach market never recoup R&D costs (patent life of 20 years begins with registration of molecule, so that effective patent life for recovery of costs averages 7 – 10 years.)

Evidence-Based Medicine

Durhane Wong-Rieger PhD (Consumer Advocare Network) introduced the basic concepts of “evidence-based” medicine, with an emphasis on how data about safety and efficacy are generated and interpreted. The following points were discussed.

- Medicine is both science and art, employing both facts and expert opinion
- Scientific evidence in drug development and review focuses on safety and efficacy
 - Evidence of safety is usually “no evidence of harm”; tolerance of harm, or risk (potential harm); Is usually very low, so only drugs with very low risk of harm proceed to clinical trials (with humans)
 - Evidence of benefits (efficacy) usually requires demonstrating to a very high level that positive outcomes (in humans) are most likely due to the drug and not some other factors, including chance or “placebo effect”
- Evidence obtained in laboratory and animal (preclinical) trials and human (clinical) trials
 - Laboratory, preclinical: is there reason to believe drug would work?
 - Is it reasonable to believe it is safe (based on animal data)?
- Phase I: Is drug safe and potentially efficacious
 - Does it cause harm in healthy volunteers
 - What is effect of drug on body, and what are side effects?

- Phase II: Clinical trials to establish drug works in body as predicted
 - Usually small number of volunteers with condition
 - Does drug have the predicted effect on the body?
 - What is the right dosage (usually minimum dosage to achieve the desired effect)?
- Phase III: Clinical trials to demonstrate outcomes “caused” by drug
 - Usually volunteers with condition
 - Usually randomized to receive drug or no treatment (or standard treatment), also called placebo
 - Usually patients and researchers are unaware of who is received drug and who is receiving no/standard treatment (blinding)
- Accepted scientific evidence of outcomes is based on probability that clinical trials (research) findings were due to drug and not due to chance or some other factor
 - If those who received the drug had a net observed benefit greater than those who received the placebo, then the treatment may be efficacious
 - If the characteristics of those receiving the drug were similar to the characteristics of those receiving the placebo, this increases the chances that the observed differences were due to the drug and not underlying differences in the two groups
 - The greater the actual difference in outcomes, the smaller the research sample size to demonstrate the new drug better than “nothing” or standard treatment

Example of Regulatory Approval Process

Agnes Klein, MD (Health Canada) provided an overview of the drug development process from the perspective of the regulator and walked through a submission review using Fabrazyme as an example. She proposed that the primary objective of all elements of the process (including regulatory review, ethics review and risks disclosure) was the protection of clinical trial subjects. The submission from the manufacturer must contain all of the studies that were carried out to demonstrate the quality, safety and effectiveness of the drug. Using Fabrazyme as an example, she discussed the data from preclinical (laboratory and animal) studies showing that the drug worked as expected in the organs of the body and had no significant toxicity in animals. She also summarized the evidence of safety and efficacy from human clinical trials. Data on efficacy consisted primarily of surrogate (substitute) endpoints demonstrating impact on organs but not clinical endpoints (improved patient functioning). Safety was demonstrated by the lack of evidence of severe adverse reactions or deaths; however, there were no data on long-term effects. Dr. Klein highlighted the limitations and the need for on-going studies, including post-market extension studies to a larger patient population. She concluded that Fabrazyme was approved on basis of a favorable “benefit-to-risk” assessment, even though all outcomes were “substitute” measures and the long-term clinical benefit and safety were still “unclear.”

Life-Cycle Approach to Regulation of Drugs

Dr. Klein also provided the presentation on Health Canada’s new “life-cycle” (also known as progressive licensing) approach to drug regulation. She characterized Health Canada’s current approach as focusing on the pre-market information with limited capacity for regulating the post-market phase. She identified the drivers for change as including the lack of “real-world” safety and effectiveness data, limitations of passive surveillance, need for increased transparency, pressure from better informed patient groups, and the role of the regulator changing from “gatekeeper” to information provider.

The proposed life-cycle approach is part of a modern regulatory framework that will support access to new therapies, the continuous monitoring, assessment, and communication of benefits and risks, and the optimal use of

drugs. The life-cycle approach recognizes the continued accumulation of knowledge about benefits and risks of drugs, especially in specific populations (children, pregnant women, elderly), drug-drug interactions, and new indications. In summary, it acts on the need for evaluating a drug through its life cycle.

Additionally, while the basis of submission remains safety, efficacy and quality data, there is opportunity to use evidence from other countries as well as patient input. Some of the benefits of the life-cycle approach will be more informed decision-making, earlier identification of risks, and better engagement of healthcare professionals and patients in decision-making. The needs of specific populations, including those with rare diseases, will be better served. The life-cycle approach needs to be supported by new regulations with added regulatory powers to require information, tests, risk disclosure, and post-market actions.

Day 2: Access to Drugs through Public Drug Plans

Patient Perspectives on Drug Funding

To set the stage for the second day, participants were asked to brainstorm the criteria they would use to assess whether “new drugs” should be accessible through public drug plans. The responses were as follows.

- Evidence of benefits
- Values – Will it improve quality of life?
- Elements of fairness
- Long-term cost savings
- Compassion: fund products for children
- Personal empathy or bias
- Whether the therapy is life saving or life extending
- Preventive therapy
- Who is most deserving?
- Evidence of long -term benefits
- Availability of advocacy groups (for consultation or to fight for the product)

Participants were asked to brainstorm the criteria they would use to decide whether “new drugs” should be funded in public drug plans. The responses were as follows.

- Overall cost to the system: will this therapy help avoid costs in other areas? What about societal costs? Will it allow an individual to go back to work?
- Does it prolong life?
- Long-term benefits to the patient
- Long-term benefits to society
- Does it help increase compliance?
- What % of the population is affected?
- Is it life saving, or is it a maintenance drug? What is the impact of the drug?
- What is the drug’s place in therapy?
- Are there alternatives?
- Is it a significant improvement over other drugs currently available?

Access to Drugs: Health Technology Assessment

Durhane Wong-Rieger, PhD gave an overview of health technology assessment, the process for assessing drugs for appropriate use and cost-effectiveness. The main reasons for using health technology assessment are to promote the appropriate use of drugs (right drug for right person at right time) and to allocate limited drug resources to achieve the greatest societal benefit. In theory, HTA processes should also take into consideration principles, values, and ethics as well as benefits, risks and costs. But these are difficult to apply for many reasons: lack of agreement, inability to measure or quantify, and lack of process for deliberation. For example, fairness may be defined as equal share, greatest good, market exchange, greatest need, most merit, equal chance or first come. Similarly, allocations may differ depending on the prioritization of values. Is it preferable to allocate to achieve equal outcomes or to give based on need? Should we favour the young, the old, or those who can contribute most to society? There are potentially serious consequences to individuals and to society if the “wrong” funding decisions are made, including patients not getting access to needed drugs, less beneficial use of scarce resources, and stifling of innovation (new drug development).

In contrast to HTA, cost-effectiveness analysis focuses on effectiveness and cost of therapies to identify those that provide the most societal benefit for the money. New drugs are generally compared with old drugs in terms of “improved outcomes” relative to “additional cost.” Whether using HTA or CEA, health outcomes are often measured in terms of improved health status (fewer symptoms, slower disease progression, cure, number of additional years of life, or improved quality of life). Across diseases, a common outcome measure used is “additional years of life” with a “discount” for “less than perfect” quality of life, often called a “quality-adjusted life-year” or QALY.

Three basic CEA methods were presented. Cost comparison is used when two (or more) drugs have the same outcomes. The rational choice is the cheaper or cheapest option.

When two drugs have different outcomes and different costs, we conduct a cost-effectiveness analysis. How much does it cost to give a patient an added “quality year of life (QALY)?” The plan chooses the drug that costs less per QALY.

Cost –utility analysis is used when the drug plan is trying to decide whether to fund a new drug or to do fund something else. The cost to provide one “additional year of quality life” is calculated and this “\$/QALY” is compared to a benchmark “\$/QALY.” If the \$/QALY is less than the benchmark, it is funded. Otherwise, it is denied as too expensive. Cost-utility analysis allows the drug plan to decide among drugs for different conditions, using a common denominator. It assumes that a “year of life” is equivalent regardless of who is gaining the year of life or the underlying need.

Role of CADTH and CDR

Brian O’Rourke, Vice President, CADTH provided a presentation on CADTH, the Common Drug Review and CADTH’s plans for patient input into the process. Highlights of Dr. O’Rourke’s presentation are below:

- CADTH’s 20-year anniversary is this year. The organization works at arm’s length from the government, but all funding comes from provincial and federal governments.
- Challenges for Canadian Expert Drug Advisory Committee (CEDAC): drugs for rare disorders; subsequent entry biologics; drug costs (what price to use?); drugs with high cost-effectiveness ratios; definition of “first in class” drugs; use of surrogate end points; drugs with limited evidence; use of appropriate comparators versus indirect comparisons; regulatory versus reimbursement assessments.
- CADTH is still struggling with the role of the public members (two on CEDAC). Their roles are evolving and changing.
- At the moment, CEDAC members do not have a specific ‘term’. (Two years is the minimum, but there is no maximum).
- CEDAC is contemplating a new possible recommendation: “Do not list at submitted price.” This recommendation has been given for one drug and according to Dr. O’Rourke both the province and the manufacturer were happy with this recommendation.
- CADTH is attempting to determine how best to include patient input into CDR Reviews. Key principles (draft) are: meaningful input to inform CEDAC, template-based submission, adherence to CDR timelines, and advocacy groups only.

Ontario Public Drug Plan

Dr. Mona Sabharwal, senior policy advisor in the Ontario Provincial Drug Plan of the MOHLTC provided an overview on how provincial drug plans make decisions about drug funding. Highlights of her presentation are included below:

- Drug system in Canada is a complex landscape with a lot of players.
- Why do you need a Committee to Evaluate drugs when you have the CDR? Because some drugs do not go through CDR, there are some instances where manufacturers don't make CDR submissions, we want to constantly re-evaluate what we are funding – the formulary should not be a static entity as our knowledge changes over time.
- Funding decisions are now made by the Executive Director (who is also the ADM of Health) and this has allowed Ontario to be very flexible.
- CED membership: predominance of the membership are physicians, there are pharmacists, health economists, pharmacists, patient members.
- The 2 patient members have to carry a lot of weight on their shoulders. They are working on how to bring forward a broader experience that the patient representatives may not personally have (i.e. different conditions).
- Ontario is one of the few jurisdictions that is able to negotiate pricing & utilization.
- Moving Forward: new initiatives: (1) Rapid Review process; (2) Compassionate review policy; (3) Product Listing agreements; (4) Drugs for Rare Disorders; (5) Citizen's Council.

Making Values-Based Allocation Decisions

Participants were divided into small groups, given a list of drugs, the indications for usage, the cost, and number of patients eligible for treatment. They were also given a drug budget that was not sufficient to cover all drugs for all potential patients. They were asked to come to a group decision as to which drugs they would fund, with the provision that they must fund a drug for the entire eligible population, or not at all. They were also asked to report on the group discussion and the reasons for their final allocation decisions. An additional complicating factor was that each group member was assigned a role that reflected someone personally affected by one of the drugs on the list. Interestingly, groups came to similar but not identical decisions. They reported that decisions were based on the following principles and values.

- Where no alternatives existed, treatments were funded;
- Children were more likely to receive treatment;
- Products that significantly improved quality of life were funded;
- If a good alternative existed, the treatment was not funded;
- If the benefits were uncertain, the product was not likely to be funded;
- Personal empathy (reflections on personal situations) was not a significant aspect of the discussions or the final allocations. Most participants fairly quickly abandoned their personal role bias to consider the best interest of the society.
- Whether the product was life saving and/or life improving were the differentiating factors in making a decision.

Participant Evaluation

Participants were asked about their knowledge and attitudes regarding access to drugs prior to and following the two-day workshop. Because the number of participants was small, there was no attempt to perform any statistical testing. Nevertheless, where the differences between pre- and post-scores are large, it is feasible to conclude that the results are meaningful. Also included in each table are comparable data from patients surveyed on-line, patients attending a previous workshop, and non-patients at a drug access conference.

Overall, the evaluation suggests participants experienced important benefits from the training.

Table 1 shows that participants reported considerable more knowledge about the process for regulatory approval following the workshop, with only 15% indicating “much or very much” knowledge before and 72% afterwards. Similar results can be seen in terms of their self-reported knowledge of Health Canada’s role in reviewing drugs, the role of the Common Drug Review in making recommendations about reimbursement, the purpose of health technology assessment, and how HTAs are conducted. Moreover, 61% of participants after the workshop reported they were comfortable interpreting an HTA report or participating in treatment based on an HTA assessment, while less than 15% were comfortable doing so prior to training.

As shown in Table 2, more participants reported trust in Health Canada decisions after the workshop than before (100% agreed “strongly or very strongly afterwards versus 72% before). Similarly, following the workshop, 94% agreed “strongly or very strongly” that they trusted the Common Drug Review when it made a funding recommendation, as compared to only 70% before the workshop. Finally, prior to the workshop, only 53% said they trusted the provincial drug plan’s decisions about drug availability whereas afterwards 71% agreed they trusted the provincial drug plan decisions.

Table 3 compares “accuracy of knowledge” about the drug review process. Prior to the workshop, about 70% knew that Health Canada approval meant that benefits outweighed risks, whereas 92% reported this accurately afterwards. After the training, more participants were able to recognize that a large sample provided a better chance of proving a drug was effective (67% afterwards compared to 39% before). More participants were also able to report accurately that the absolute cost of a drug was not necessarily a reason to deny listing (56% post-workshop and 37% pre-workshop). They were more likely to recognize that a positive CDR recommendation did not mean a new drug should be used instead of an older drug (80% post- compared to 42% pre-workshop). Paradoxically, they were less likely to agree that a CDR recommendation meant that the new drug provided more “value for money” than the older one (39% provided correct response post-workshop and 65% responded correctly pre-workshop).

Table 4 compares participant preferences for values and principles used in drug allocation decisions. Overall, there was very little change post-workshop, which is not surprising and confirms the premise that values and principles are deep-seated and pervasive and not swayed by new information or knowledge. Participants were not asked to rank or prioritize the values or principles but it is clear from the ratings that almost all felt that drugs should be funded, even if costly, if they had fewer side effects, improved quality of life, or treated a life-threatening conditions. In terms of fair access, most participants seemed to be oriented towards equity (give to those most in need) rather than equality (give everyone same regardless of need), preferring to fund drugs to bring everyone to a basic level of well-being and giving preference to those with life-threatening disorders or to those who are the worst off. Moreover, they did not support giving preference to the young rather than the old and rejected a utilitarian principle to give preference to those who could contribute most to society. Finally, they also rejected the option to fund drugs that would provide a small benefit to many instead of more costly drugs that could provide a large benefit to a few.

Table 5 summarizes the participants' evaluation about the benefit and quality of the workshop. Overall, they responded very positively to all of the presentations and activities. All presentations were rated as "excellent" or "good" by 75% to 100% of participants. All three brainstorming and small group exercises were rated as "good" or "excellent" by nearly 90% of participants. They were especially positive in their response to the presentations from the representatives of the agencies (CADTH, Ontario Public Drug Program, and Health Canada). They rated as mostly excellent or good the sessions on drug development, evidence-based medicine, HTA/CEA, and patient involvement. In response to an open-ended question, participants indicated the training would help them to advocate more effectively for appropriate drugs for their disease or condition. Some responded that they would be able to share the knowledge with others in their patient group. Others indicated the knowledge helped them to appreciate the complexity of the drug review process while others expressed greater confidence in understanding (and challenging) reports from Health Canada, the Common Drug Review, and the provincial drug plan.

Table 1
Knowledge of Drug Review Process
Participants Pre- and Post-Workshop

% Respondents Agree "Much" or "Very Much"	Patients Pre-Workshop (n=21)	Patients Post-Workshop (n=18)	Patients Online (n=57)	HCPs, Industry, Government (n=21)
Know process of testing drug for regulatory approval	15%	72%		
Know Health Canada role in reviewing drugs	10%	78%	16%	90%
Know Common Drug Review role in recommendation	13%	78%	5%	95%
Know purpose of Health Technology Assessment	5%	61%	7%	70%
Know how HTA's are conducted	0%	63%	20%	63%
Comfortable interpreting HTA report for self or another patient?	11%	67%	13%	63%
Comfortable providing personal experience into HTA process?	35%	61%	23%	56%
Comfortable participating in treatment based on HTA recommendation	15%	61%	13%	50%

Table 2
Trust in Drug Review Agencies and Process
Participant Pre- and Post-Workshop

% Respondents Agree "Somewhat" or "Strongly"	Patients Pre-Workshop (n=21)	Patients Post-Workshop (n=18)	Patients Online (n=57)	HCPs, Industry, Government (n=21)
Trust Health Canada when it approves drug as safe and efficacious	72%	100%	84%	95%
Trust Common Drug Review when it recommends whether drug is cost-effective and should be funded	70%	94%	20%	25%
Trust Provincial Drug Program when it decides whether to make drug available	53%	71%	13%	25%
Trust physician when he/she recommends drug for me	90%	94%	73%	79%
Trust patient groups when they inform about drug options	87%	58%	71%	90%
Patient experiences should be included in drug review process	95%	100%	82%	90%
Patients should be members of committees to recommend drug listings on public plans	90%	100%	78%	100%
Meetings of drug review committees should be open to public	90%	73%	91%	85%
Patients/public should be able to provide testimony to drug review committees	100%	100%	89%	90%

Table 3
Accurate Information About Drug Review Processes
Participant Pre- and Post-Workshop

% Reporting "Right" Answer	Patients Pre-Workshop (n=21)	Patients Post-Workshop (n=18)	Patients Online (n=57)	HCPs, Industry, Government (n=21)
HC approval of new drug means its benefits outweigh risks (TRUE)	70%	92%	59%	95%
A large sample gives a better chance of proving a drug is effective (TRUE)	39%	67%		
A drug that costs more than the current will NOT be listed (FALSE)	37%	56%		
CDR recommendation of new drug means it should be used instead of older drug (False)	42%	80%	42%	84%
CDR recommendation of new drug means it provides more value than other drugs (True)	65%	39%	42%	69%

Table 4
Principles and Values for Drug Allocation Decisions
Participant Pre- and Post-Workshop

% Respondents Agree "Somewhat" or "Strongly"	Patients Pre-Workshop (n=21)	Patients Post-Workshop (n=18)	Patients Online (n=57)	HCPs, Industry, Government (n=21)
When drugs have same benefit, should only provide cheaper drug.			41%	15%
If new drug costs more but is more effective or has fewer side effects, should be funded	100%	100%	98%	100%
If new drug costs more but improves quality of life, should be funded	100%	100%	100%	100%
Drug for life-threatening disorder should be funded, even if costly	100%	100%	100%	100%
Budget allocation should assure everyone same basic level of well-being	95%	94%	94%	89%
Priority should be given to treatments for life-threatening disorders	90%	84%	83%	90%
Allocate more to those worst off, regardless of cost or benefit	71%	61%	70%	75%
Preference should be given to treatments for the young rather than the very old.	44%	45%	42%	42%
Preference should be given to those providing greatest societal benefit (e.g., working age)	19%	33%	21%	20%
Give preference to drugs with small benefits for many, not large benefits for few	33%	12%	37%	25%

Table 5
Workshop Evaluation

% Respondents Agree "Somewhat" or "Strongly"	Patients Pre-Workshop (n=21)	Patients Post-Workshop (n=18)	Patients Online (n=57)	HCPs, Industry, Government (n=21)
When drugs have same benefit, should only provide cheaper drug.			41%	15%
If new drug costs more but is more effective or has fewer side effects, should be funded	100%	100%	98%	100%
If new drug costs more but improves quality of life, should be funded	100%	100%	100%	100%
Drug for life-threatening disorder should be funded, even if costly	100%	100%	100%	100%
Budget allocation should assure everyone same basic level of well-being	95%	94%	94%	89%
Priority should be given to treatments for life-threatening disorders	90%	84%	83%	90%
Allocate more to those worst off, regardless of cost or benefit	71%	61%	70%	75%
Preference should be given to treatments for the young rather than the very old.	44%	45%	42%	42%
Preference should be given to those providing greatest societal benefit (e.g., working age)	19%	33%	21%	20%
Give preference to drugs with small benefits for many, not large benefits for few	33%	12%	37%	25%