Patient involvement in access decisions

NEIL BERTELSEN

CHAIR, HTAi PATIENT & CITIZEN INVOLVEMENT IN HTA

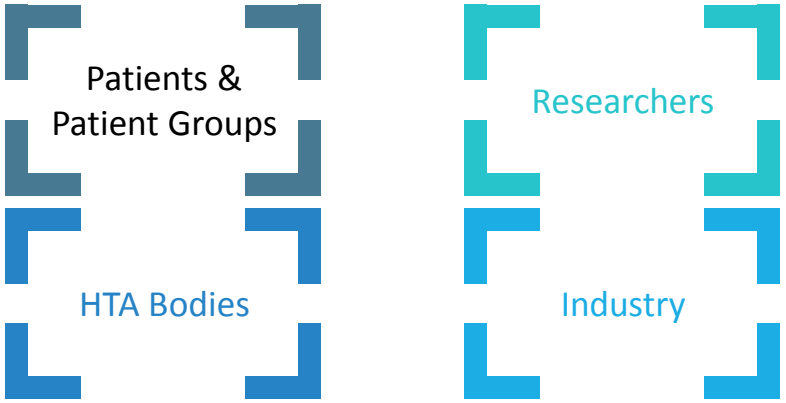
The patient and citizen involvement in HTA interest group

'Patients' perspectives in HTA: a route to robust evidence and fair deliberation'
Int. J. Tech Assess Health Care, 2010, 334-340

www.HTAi.org

"To implement a patient-based HTA, the focus must turn to the patient's issues and incorporate each patient's unique perspective and preferences. Processes must change to increase patient participation in all levels of HTA and aim to promote empowered patients who can make informed decisions."

Bridges et al. IJ of Technology Assessment in Healthcare 23:1 2007 30-35



- Working internationally across both developed and developing HTA systems
- Bringing multiple disciplines together to address the opportunities possible by better patient involvement
- Identifying and filling the gaps in research and resources in partnership with the patient members

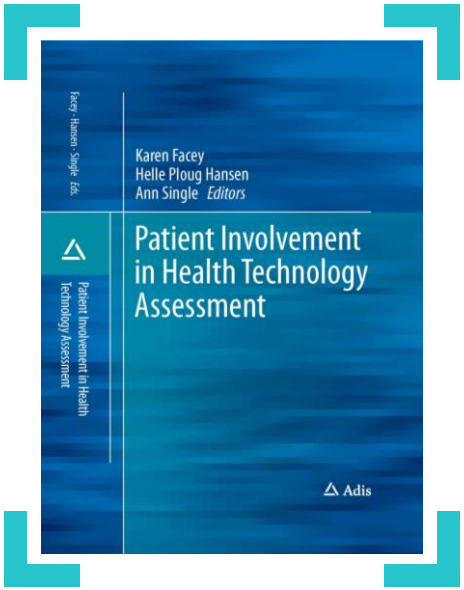
The book:
Patient involvement in HTA

From Dr. Brian O'Rourke – CADTH / INAHTA

"If you're not involving patients, you're not doing HTA!…

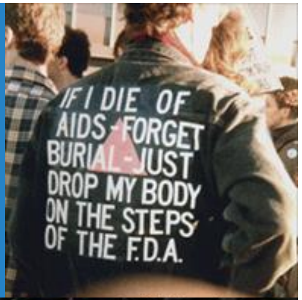
Don't think you've done your part by buying this book. Read it. Learn from it. Let it spark discussions about what you, your colleagues, your organisation, and your networks can do to improve patient involvement in HTA.

There are important messages for all stakeholders and I will do my part to support and indeed implement many of the proposals identified in the book – within my agency and more broadly across INAHTA member agencies."



We have been here before…

Access to HIV related medicines in the 1980s



- Patients waiting too long and dying before access is granted
- Complex decision making that excluded the patients and the advocates
- Technical criteria and systems used to defend the status quo

News & Comment

Quick Release of AIDS Drugs

In response to lobbying by patients, U.S. health officials agree to distribute experimental AIDS drugs before testing for effectiveness is complete; "parallel track" to be ready by the fall

THE HIGH COMMAND in the war against AIDS filed into Representative Henry Waxman's (D–CA) health subcommittee hearing room on 20 July to make a public concession.

You can't run a war without troops, they acknowledged, and their own troops—the AIDS victims who serve as volunteers in drug testing clinics—are in revolt over the tight federal rules that limit who gets new AIDS drugs and when. The patients want faster access to drugs as they come off the laboratory bench, before they go through time-consuming "Phase II clinical trials" that test their effectiveness.

The nation's top health officials say they are ready to yield on this point and that they will relax the rules in a significant way this fall. The plan is to make drugs more widely available before Phase II tests, a major shift in policy that could have long-term consequences in the research and pharmaceutical communities. If exceptions are made for AIDS, the same arguments would hold for cancer and other currently incurable diseases afflicting millions of people.

Some researchers and clinicians have been calling for just such a policy shift for years. Others are skeptical, fearing that this will make clinical data harder to interpret and slow down the ultimate licensing decisions.

Several scientists who run AIDS studies worry that if experimental drugs are distributed early to people outside the clinic, this could make it impossible to control the patients' medication. This, they argue, will make it difficult, if not impossible, to determine whether a new drug is in fact responsi-

an advocacy group in San Francisco, says, "I argue that it is the FDA restrictions that are polluting the clinical trials," because patients desperate to get access to drugs lie about their medical history. With more lenient rules that would not happen.

The specific concession the activists want, and will get this fall, according to U.S. health officials, is a new structure for distributing experimental drugs called the "parallel track." It means essentially that an AIDS patient will be able to receive the latest drugs that appear safe, whether or not they have been licensed for sale, without having to be part of a clinical trial. Normally, a drug that is not approved for sale by the Food and Drug Administration (FDA) cannot be obtained except by people in an FDA-sanctioned test. In the past, FDA has allowed case-by-case "compassionate" or "emer-

second supply window, providing experimental drugs routinely to "persons for whom there are no satisfactory alternative drugs or therapies available . . . and who for some reason are not eligible for or not able to participate in a clinical trial." So declared James O. Mason, the assistant secretary for health of the Department of Health and Human Services, the top health official at Waxman's hearing. Mason was flanked by Anthony Fauci, chief of the National Institute for Allergies and Infectious Diseases, Samuel Broder, director of the National Cancer Institute, and Frank Young, commissioner of FDA, all of whom spoke in favor of the reform.

However, before the new plan can go forward, Mason said, critical details must be worked out. He has asked Young to assemble an advisory committee quickly and submit a report no later than 21 August. Among other duties, the committee will define procedures for identifying toxic effects among patients not in clinical trials, review liability issues and develop standards for informed consent. Young hasn't decided who will sit on the new committee as yet, but he may use an existing FDA advisory group that reviews antiviral medicines.

The FDA has been broadly criticized by the activists for its slowness in making new drugs available. (See comments of Vincent DeVita, former Na-

Changing the rules. *AIDS activists Jim Eigo (right) and Martin Delaney argue that drugs in clinical trials should be widely available.*

© Susan Muniak

Anthony Fauci: *"We can be humanitarian and do good science."*

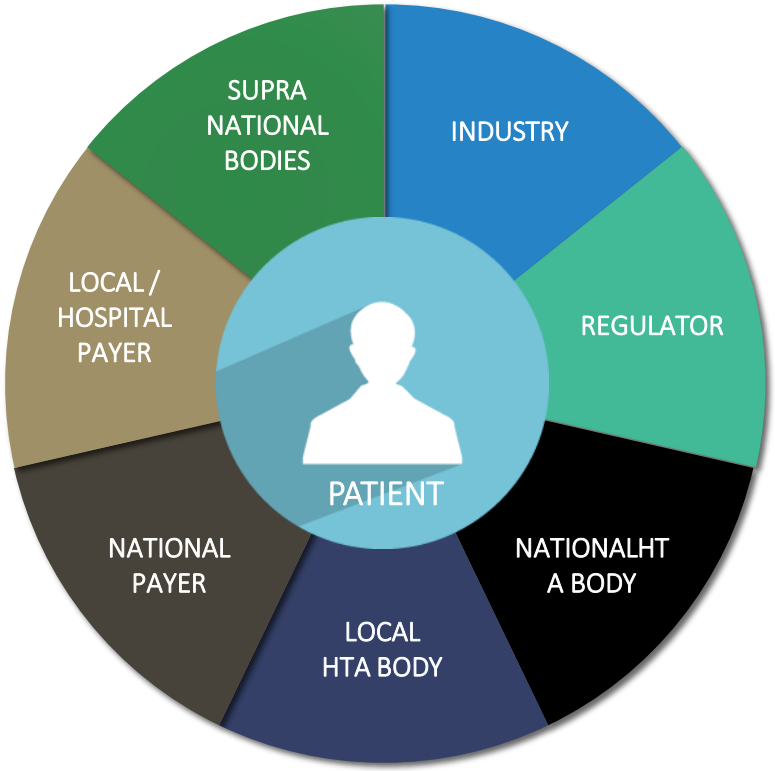
Science, 28 July 1989, Vol 245 Issue 4916 pp. 345

… so why are we here again?

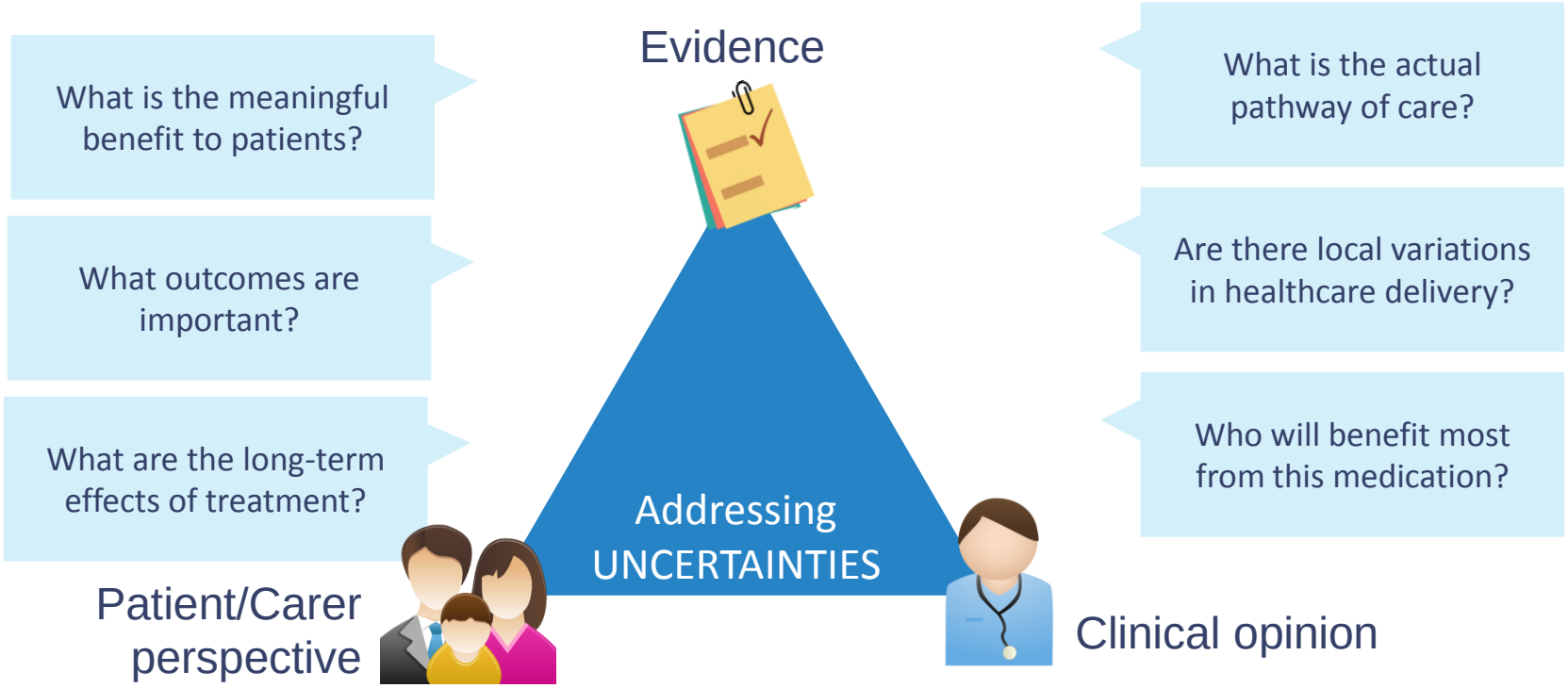
"AZT is the only drug approved for use against AIDS. It's now known that AZT also postpones the onset of AIDS in people infected with the virus. ***Yet there's a massive obstacle to wider use of this life-saving drug - its extraordinary cost. At $8,000 a year for users, AZT is said to be the most expensive prescription drug in history."***

New York Times, Opinion, August 28 1989

- Healthcare access has evolved to meet the cost challenges of modern healthcare
- This has led to fragmentation of decision making across multiple bodies and disciplines
- Patients are often left confused by divergent decisions within countries and across borders



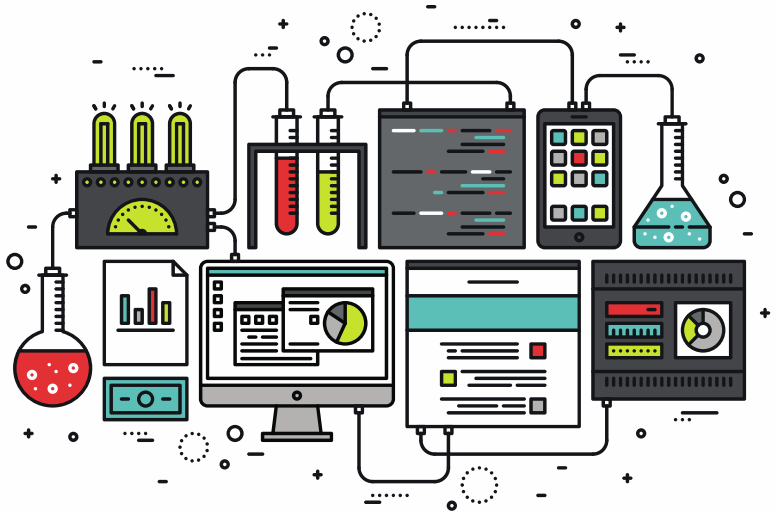
HTA Deliberation – why patient involvement is needed



No gold standard for patient involvement in HTA

- There has yet to emerge a ‘best’ way of integrating patient insights and experiences into HTA
- A wide continuum of approaches:
  - Informal discussions
  - Public consultations
  - Formal written submissions
  - Representation at committees
  - Workshops and scoping meetings

Faster regulatory decisions make this even more important



- Uncertainties in the evidence package are going to increase over the coming years
  - Accelerated approval processes from the regulators lead to reduced evidence packages at the time of launch
  - Adaptive pathways potentially further reduces that initial evidence package
  - Surrogate endpoints rather than hard outcomes used to speed up time to approval
  - Agreement needed on subsequent evidence generation plans and patient perspectives on those evidence plans

Barriers to patient and advocate involvement

1

Investment in patient involvement processes lacking – no clear priority given

2

Patient participation perceived as biased – “they want everything and work too close to industry”

3

Lack of understanding of the value that patient insights can bring to decision making

4

Established HTA have ‘set the bar too high’

Values for Patient Involvement in HTA

Relevance

Patients have knowledge, perspectives and experiences that are unique and contribute to essential evidence for HTA.

Fairness

Patients have the same rights to contribute to the HTA process as other stakeholders and have access to processes that enable effective engagement.

Equity

Patient involvement in HTA contributes to equity by seeking to understand the diverse needs of patients with a particular health issue, balanced against the requirements of a health system that seeks to distribute resources fairly among all users.

Legitimacy

Patient involvement facilitates those affected by the HTA recommendations/decision to participate in the HTA; contributing to the transparency, accountability and credibility of the decision-making process.

Capacity building

Patient involvement processes address barriers to involving patients in HTA and build capacity for patients and HTA organizations to work together.

Quality Standards for Patient Involvement in HTA

General HTA process

1. HTA organizations have a strategy that outlines the processes and responsibilities for those working in HTA and serving on HTA committees to effectively involve patients.
2. HTA organizations designate appropriate resources to ensure and support effective patient involvement in HTA.
3. HTA participants (including researchers, staff, HTA reviewers and committee members) receive training about appropriate involvement of patients and consideration of patients' perspectives throughout the HTA process.
4. Patients and patient organizations are given the opportunity to participate in training to empower them so that they can best contribute to HTA.
5. Patient involvement processes in HTA are regularly reflected on and reviewed, taking account of the experiences of all those involved, with the intent to continuously improve them.

For individual HTAs

6. Proactive communication strategies are used to effectively reach, inform and enable a wide range of patients to participate fully in each HTA.
7. Clear timelines are established for each HTA with advance notice of deadlines to ensure that appropriate input from a wide range of patients can be obtained.
8. For each HTA, HTA organizations identify a staff member whose role is to support patients to contribute effectively to HTA.
9. In each HTA, patients' perspectives and experiences are documented and the influence of patient contributions on conclusions and decisions is reported.
10. Feedback is given to patient organizations who have contributed to an HTA, to share what contributions were most helpful and provide suggestions to assist their future involvement.

Recommendations

For member states

Promote values and quality standards of patient and citizen involvement in HTA bodies

Share approaches, processes, and methods across the member states

Bring more transparency to the role of patients and advocates in the process

Invest in patient-based evidence from national health services

Involve patients and patient organisations in the development of new approaches