EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Patient's perception of risks and benefits of medicines

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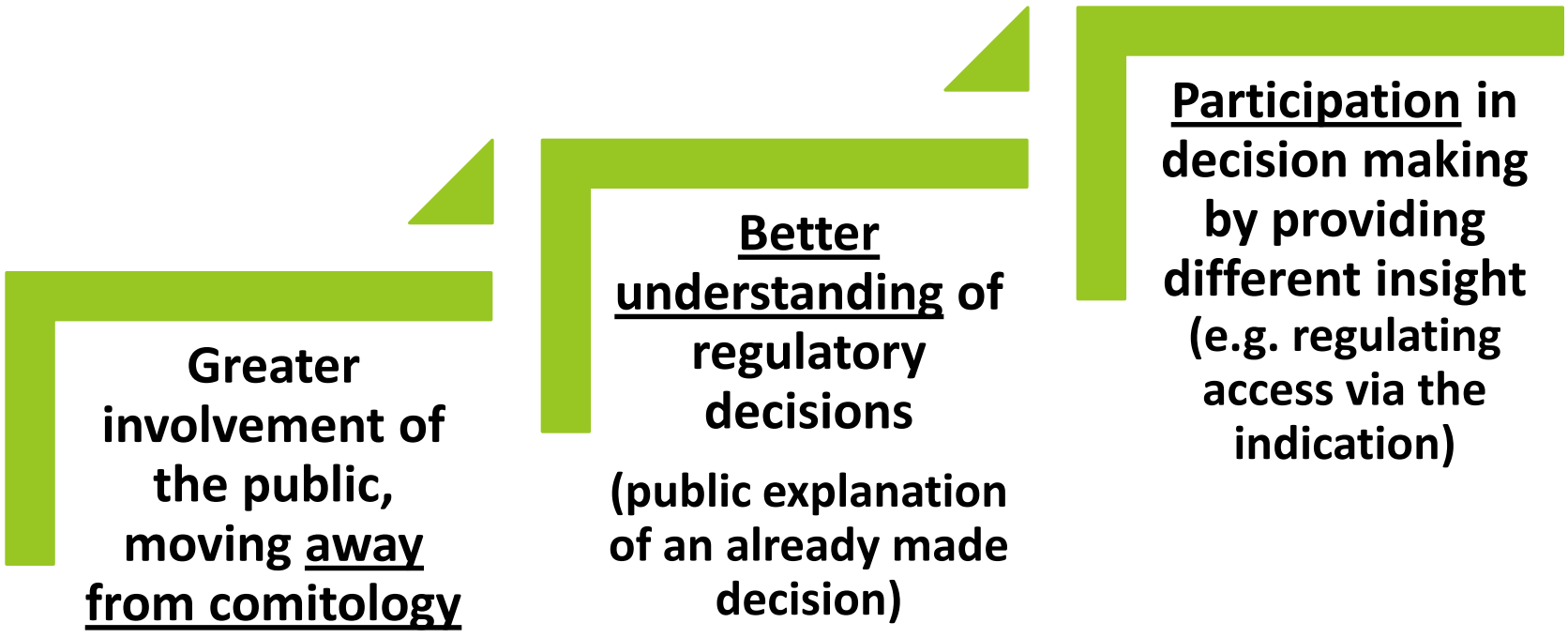
Guido Rasi

An agency of the European Union

“Our expectations”



Modified from François Houÿez

Agenda

- Bringing patients into the system
  - Public hearings
  - Patient representatives on committees

- Bringing patients' values and preferences into the system
  - How to systematically obtain values and preferences
  - Would it change the outcome of the decision?

Public hearings:
What the legislation says

- "Where the urgency of the matter permits, the PRAC may hold public hearings, ... The hearings shall be held in accordance with the modalities specified by the Agency and shall be announced by means of the European medicines web-portal. The announcement shall specify the modalities of participation.
- The Agency shall, in consultation with the parties concerned, draw up Rules of Procedure on the organisation and conduct of public hearings..."

Article 107j of Directive 2001/83/EU as amended

Public hearings: Current debate

- Purpose of public hearings: transparency or engagement?
- Timing of public hearings: for which procedure and when during the process?
- Participants: who should attend?
- Conduct of public hearings:
  - Language and location
  - Participation: in person or online?
  - Ground rules for participants
  - Time and resources
  - Online real-time streaming

Patient representatives on committees

- Standing EMA working party with consumers and patients
- ‘Permanent’ patient representatives on some EMA committees and Advisory groups, but not CHMP
- Patients effectively excluded from key decisions on licensing.
- Direct involvement of patients with the disease under discussion extremely rare (e.g. thalidomide for MM)

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Weight, Utility, Clinical significance

"If stroke or systemic embolism and major hemorrhage were considered equally undesirable…."

"Most people would agree, however, that the irreversible effects of strokes and systemic emboli have greater clinical significance than non-fatal bleeding"

"Any benefit-risk assessment in which strokes and systemic emboli are given more weight than non-fatal bleeding…"

Beasley BN, Unger EF, Temple R. NEJM 2011; 364(19): 1788-90

Benefit-Risk assessment
Art or science?

Ingredients of regulatory decisions:

- Data (incidences)
- Uncertainty
- Values (utilities/disutilities)

Decisions driven by:

probability of event x “value/utility” of event

→ **“expected utilities”**

Whose values should count?

How to bring patients' preferences /values into BR decisions?

- 'Patient' is not necessarily the same as 'patient representative'
- Patients with the specific disease condition know which outcomes and symptoms matter most to them.
- Patients enrolled in regulatory drug trials are (ideally) the target group for treatment once a drug is licensed, yet we do not usually explore their values and preferences in a systematic way.
- In terms of listening to the patients' voice, trial patients are an underutilized resource.

Can we quantify patients' value judgements?

One Thousand Health-Related Quality-of-Life Estimates

TAMMY O. TENGS, ScD, AND AMY WALLACE, MA

OBJECTIVE. Analysts performing cost-effectiveness analyses often do not have the resources to gather original quality-of-life (QOL) weights. Furthermore, variability in QOL for the same health state hampers the comparability of cost-effectiveness analyses. For these reasons, opinion leaders such as the Panel on Cost-Effectiveness in Health and Medicine have called for a national repository of QOL weights. Some authors have responded to the call by performing large primary studies of QOL. We take a different approach, amassing existing data with the hope that it will be combined responsibly in meta-analytic fashion. Toward the goal of developing a national repository of QOL weights to aid cost-effectiveness analysts, 1,000 health-related QOL estimates were gathered from publicly available source documents.

METHODS. To identify documents, we searched databases and reviewed the bibliographies of articles, books, and government reports. From each document, we extracted information on the health state, QOL weight, assessment method, respondents, and upper and lower bounds of the QOL scale. Detailed guidelines were followed to ensure consistency in data extraction.

RESULTS. We identified 154 documents yielding 1,000 original QOL weights. There was considerable variation in the weights assessed by different authors for the same health state. Methods also varied: 51% of authors used direct elicitation (standard gamble, time tradeoff, or rating scale), 32% estimated QOL based on their own expertise or that of others, and 17% used health status instruments.

CONCLUSIONS. This comprehensive review of QOL data should lead to more consistent use of QOL weights and thus more comparable cost-effectiveness analyses.

KEY WORDS: Quality of life; cost-effectiveness; utility assessment; quality-adjusted life-year. (Med Care 2000;38:583–637)

Medical Care
Vol 38, 6 p583-637

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