Are patients and the public getting the information they need to make informed treatment decisions?

Pharmaceutical Policy Symposium on 'Improving the use of evidence in decision-making'
Helsinki, 23rd September 2025

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Outline of presentation

A. The importance of high quality medicines information for patients and the public

B. Study 1: Evaluating current regulated information sources for patients and the public

C. Study 2: Designing and testing better medicines information for patients

D. Policy developments and implications

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Acknowledgements and disclosures

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The importance of high-quality medicines information for the public

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Arguments for provision of high-quality written information about new drugs


ETHICAL


LEGAL


QUALITY OF CARE

Relevant. Accurate and transparent. Clear and understandable

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Patient and consumers value[a]…

"perception of risk is always proportional to the expected benefit of the medicine"[b]

High-quality written information from trustworthy and rel… …rces

Written information about drug *benefits* as well

"Informed choice requires transparency. That entails transparency of what is known, as well as what is not known."[c]

Information about the *quality* of the evidence

Information about important clinical *uncertainties* and *knowledge gaps*

To supplement (not replace) verbal information from clinicians

[a]Coulter et al., 1999; Coulter et al, 2006; Grime et al., 2007; Raynor et al., 2007; Autorité de Santé, 2008; Hamrosi et al., 2013; van Dijk et al., 2014;; Schneider et al. 2021; Treadgold et al., 2022; [b] EMA 'Information on the Benefit-Risk of Medicines: Patients' and Consumers' and Healthcare Professionals' Expectations', 2009; [c] European Patients Forum, 2017.

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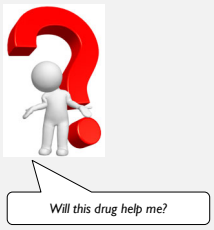
High quality information essential when balance of benefits and harms is uncertain

Cancer patients often need to balance drug toxicity against uncertain or marginal drug benefits

72% of cancer drugs authorised in the EU 2017-19 came onto the market without evidence of improved survival or quality of life[a] (the surrogate endpoint problem)

When survival benefits are demonstrated, tend to be marginal – median of 2-3 months [b]



[a]Davis et al, BMJ 2023;380:bmj-2022-073711; [b] Fojo T. et al., JAMA Otolaryngology-Head & Neck Surgery, 2014;140(12):1225-1236

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High quality information an essential part of high quality cancer care

Relevant, accurate and trustworthy information also important to counter prior misconceptions

Evidence suggests that cancer patients often overestimate drug benefits and misunderstand the goal of treatment[a]

Evidence that people tend to assume new treatments are underpinned by high quality evidence[b]

Medicines regulatory bodies, like the European Medicines Agency (EMA), are well placed to provide this information

[a]Weeks et al., NEJM 2012; 367(17):1616-1625; [b]Schwartz & Woloshin, Arch Int Med 2011;171-8, Sullivan et al. Druf Saf 2020;29:40

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Study 1: Evaluating regulated medicine information sources in the EU

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Regulated sources of information in the EU

European Public Assessment Reports (EPARs)

- Publicly available scientific assessment reports for all medicines granted a marketing authorisation

Summary of Product Characteristics

- Aimed at healthcare professionals
- Explains how to prescribe and use a medicine.

Patient Information Leaflets

- 'Package leaflets' the most widely available source of printed information on medicines
- Information on drug risks and safe use

Medicine Overviews

- Lay summaries of the Public Assessment report on drug risks & benefits
- Available on EMA website

Statutory product information

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Statutory patient leaflets

Current EU legislation does not require inclusion of information on drug benefits in regulated patient leaflets

Relevant legislation does allow for inclusion of other information which is useful for the patient, consistent with the SmPC and non-promotional (Article 62 of Council Directive 2001/83/EC)

EPAR summaries for the public ('Medicines Overviews') written by the EMA medical writers and include a section on the benefits shown in clinical studies.

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Study 1: aim and design

- Content analysis of information sources for 32 cancer indications authorized by the European Medicines Agency (EMA) 2017-19
- Evaluated frequency with which information about drug benefits, and benefit uncertainties, communicated to patients, consumers and clinicians

thebmj Davis et al. *The British Medical Journal*, 2023;380:1-13

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Compared information contained in the SmPCs, PLs and MOs with EPARs for 32 cancer indications (116 documents)

European Public Assessment Reports (EPARs)	Summary of Product Characteristics	Patient Information Leaflets	Medicine Overviews
• Publicly available scientific assessment reports	• Aimed at healthcare professionals	• 'Package leaflets' for consumers and patients	• Public summaries of the EPARs, available on the EMA website

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Problem 1: Patient leaflet prioritises the *least* important types of benefit information for patients

- No patient leaflets contained information about the nature/magnitude benefits shown in clinical studies
- 97% of patient leaflets contained information about how the drug works (mechanism of action)

- Poll of around 30 patients, consumers, clinicians and topic experts
- Most important category of information related to question "What difference did the drug make for patients in clinical studies?" (92% deemed this 'essential')
- Second most important category: "What is *not* known about the drug's benefits" (79% deemed this 'essential')
- *Least* important category of information was "How does the drug work?" (12% deemed this 'essential', 62% deemed this 'not important')

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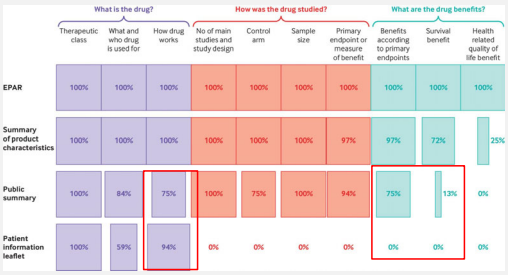
Communication of information about a drug, how it was studied, and evidence of benefit.



Courtney Davis et al. BMJ 2023;380:bmj-2022-073711

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Problem 2: Patient leaflets and public summaries contain potentially misleading information

- "[Drug X] inhibits a type of enzyme called tyrosine kinase and triggers the death of cancer cells in patients with alterations in genes for ALK"
- Without information about the drug benefits demonstrated in clinical studies, information about the drug's effects in the body may be misleading

- Communication of findings based on surrogate endpoints likely to be misleading
- "Patients taking [Drug X] lived for longer without their disease worsening"
- Study involving nearly 900 US adults, showed that without an explicit disclosure people were more likely to interpret this as meaning people lived longer.[2]

Sullivan et al., The Oncologist 2023; 5;28(7):e542-e553

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Problem 3: Patient leaflets and public summaries do not communicate important evidence uncertainties or gaps

- EU regulators frequently raised concerns about the quality of evidence
- Deficiencies undermined the reliability or interpretability of study findings on drug benefits or created uncertainty with respect to the clinical relevance of those findings

- These uncertainties were rarely communicated to patients, the public or clinicians

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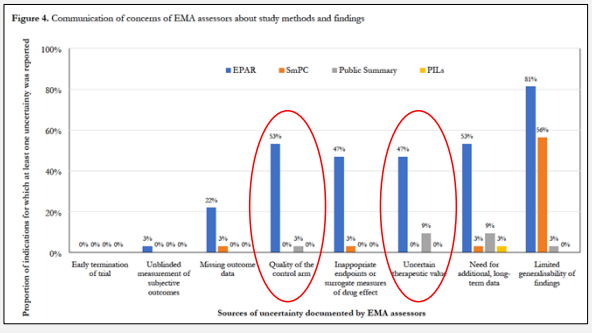

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Key findings

Courtney Davis et al. BMJ 2023;380:bmj-2022-073711

PLs provide no information about drug benefits or how the drug was tested

MOs are an improvement – they communicate accurate information about studies/ findings BUT rarely explain their clinical significance (eg. surrogate endpoints)

Neither PLs nor MOs communicate important uncertainties or provide explicit information about the quality of the evidence base

Neither PLs nor MOs communicate absence of evidence with respect to patient-important outcomes (survival, quality of life) – 72% of drugs approved without evidence of a survival or quality of life benefit

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Study 2: Designing and testing better medicines information

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Recent efforts to improve PL in the EU

- June 2023 EMA working group established to improve the SmPC and PL, and consider inclusion of a Key Information section (KIS) with benefit information in the patient leaflet
- Study 2: Online randomized controlled trial involving representative sample of 2,000 UK adults to evaluate the effect of including a key information section (KIS) in EU patient leaflets on individuals' expectations and attitudes towards new medicines
- April 2025 – EMA launched a public survey seeking feedback on inclusion of a KIS in the patient leaflet – no specific content or format proposed

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Communicating drug benefit information – things *not* to do

Do not equate drug benefit information with statements about the drug's mechanism of action, or generic descriptions about what the drug is expected to do ('this medicine reduces pain associated with arthritis')

Patients want qualitative and quantitative information on the *actual* benefits they might expect based on evidence from clinical studies

Do not report clinical study results without also explaining their clinical significance for patients (how might this affect the way a patient feels, functions, survives)

Patients want "meaningful explanations" of study endpoints

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Communicating drug benefit information – things *not* to do

Do not include descriptions of surrogate endpoints that have not been properly user tested

Patients misunderstand commonly used terminology to describe some surrogate endpoints[a]

Do not include information about drug efficacy based on surrogate endpoints without an explicit statement about the relationship between the surrogate endpoint and the outcomes of interest (how a patient feels, functions, survives)

Without explicit disclosure ('We do not know whether drug x helps patients live longer') people overestimate drug benefits and misunderstand the goal of treatment[b]

[a] Sullivan et al. The Oncologist 2020;25:1060-1066; Sullivan et al. The Oncologist 2023;28(7):e542-e553

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Drug facts boxes - things to do!

1. **Drug Facts Boxes** address patients' information needs by presenting *qualitative* and *quantitative* information about drug benefits, harms and uncertainties in a summary form
2. Shown in large randomized trials in nationally representative populations to improve understanding and decision-making[a]
3. Endorsed by independent, expert advisory committees in the US, UK and Canada[b]

[a] Schwartz et al. *Annals of internal medicine*, 2009;151:516-527; Woloshin & Schwartz *Annals of internal medicine*, 2011;155:87-96; Schwartz & Woloshin *Proceedings of the National Academy of Sciences*. 110;14069-74
[b] FDA's Risk Communication Advisory Committee, 2009; Council of Canadian Academies, 2015; UK Academy of Medical Sciences, 2017

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The Drug Facts Box

Developed by Lisa Schwartz and Steven Woloshin

- ✓ Communicates goal of treatment
- ✓ Concise, easy to read summary of benefits & risks
- ✓ Qualitative & quantitative information on benefits & risks
- ✓ Benefits & risks presented together

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Lunesta Study Findings

788 healthy adults with insomnia for at least 1 month – sleeping less than 6.5 hours per night and/or taking more than 30 minutes to fall asleep – were given LUNESTA or a sugar pill nightly for 6 months. Here's what happened:

What difference did LUNESTA make?	People given a sugar pill	People given LUNESTA (3 mg each night)
Did Lunesta help?		
LUNESTA users fell asleep faster (15 minutes faster due to drug)	45 minutes to fall asleep	30 minutes to fall asleep
LUNESTA users slept longer (37 minutes longer due to drug)	5 hours 45 minutes	6 hours 22 minutes
Did Lunesta have side effects?		
Life threatening side effects:		
No difference between LUNESTA and a sugar pill	None observed	None observed
Symptom side effects:		
More had unpleasant taste in their mouth (additional 20% due to drug)	6%	26%
More had dizziness (additional 7% due to drug)	3%	10%
More had drowsiness (additional 6% due to drug)	3%	9%
More had dry mouth (additional 5% due to drug)	2%	7%
More had nausea (additional 5% due to drug)	6%	11%

Source: https://dartmed.dartmouth.edu/spring08/html/disc_drugs_we.php

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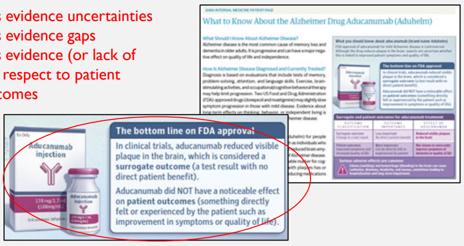
- ✓ Communicates evidence uncertainties
- ✓ Communicates evidence gaps
- ✓ Communicates evidence (or lack of evidence) with respect to patient important outcomes


What to Know About the Alzheimer Drug Aducanumab (Aduhelm)
The bottom line on FDA approval
In clinical trials, aducanumab reduced visible plaque in the brain, which is considered a surrogate outcome (a test result with no direct patient benefit).
Aducanumab did NOT have a noticeable effect on patient outcomes (something directly felt or experienced by the patient such as improvement in symptoms or quality of life).

Communicating uncertainties and important clinical evidence gaps

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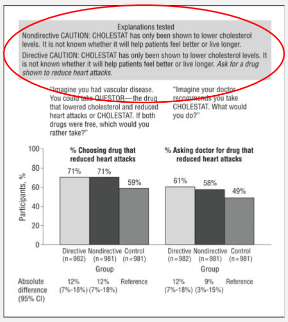
- ✓ Experimental studies show that inclusion of explicit disclosures improved patient understanding and decision-making.
- ✓ Non-directive explanations worked as well as directive ones.[a]
- ✓ Content and format should be properly user-tested


Explanations tested
Nondirective CAUTION: CHOLESTAT has only been shown to lower cholesterol levels. It is not known whether it will help patients feel better or live longer.
Directive CAUTION: CHOLESTAT has only been shown to lower cholesterol levels. It is not known whether it will help patients feel better or live longer. Ask for a drug shown to reduce heart attacks.
% Choosing drug that reduced heart attacks
% Asking doctor for drug that reduced heart attacks
Participants, %
71%
71%
59%
61%
58%
49%
Directive
Nondirective
Control
Group
Absolute difference (95% CI)
Reference

[a] Schwartz, L. and Woloshin S. *Arch. Intern. Med* 2011;171(16):1463-68

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Preferences for speed of access versus certainty of the survival benefit of new cancer drugs: a discrete choice experiment

Findings Between July 7 and July 20, 2023, 998 eligible respondents completed the survey. 870 respondents (461 [53%] male, 406 [47%] female, and three [<1%] other) were included in the final analysis. Respondents showed strong preferences for high certainty of survival benefit (coefficient 2·61, 95% CI 2·23 to 2·99), and strong preferences against a 1-year delay in FDA approval time (coefficient –1·04, 95% CI –1·31 to –0·77). Given very low certainty a drug would provide survival benefit (no evidence linking a surrogate endpoint to overall survival), respondents were willing to wait up to 21·68 months (95% CI 17·61 to 25·74) for high certainty (strong evidence) of survival benefit. A drug's effect on a surrogate endpoint had no significant impact on drug choices (coefficient 0·02, 95% CI –0·21 to 0·25).

www.thelancet.com/oncology Published online November 18, 2024 https://doi.org/10.1016/S1470-2045(24)00596-5

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