# Ελληνική Εταιρεία Φαρμακευτικής Ιατρικής

# 5η Ημερίδα Φαρμακοεπαγρύπνησης

# Ψηφιακή προσυνεδρία

## Ενημέρωση ωφελειών έναντι κινδύνων για τους χρήστες των φαρμάκων: Πώς μπορούν οι ρυθμιστικές αρχές να ανταποκριθούν καλύτερα στις ανάγκες πληροφόρησης των ασθενών και των επαγγελματιών υγείας;

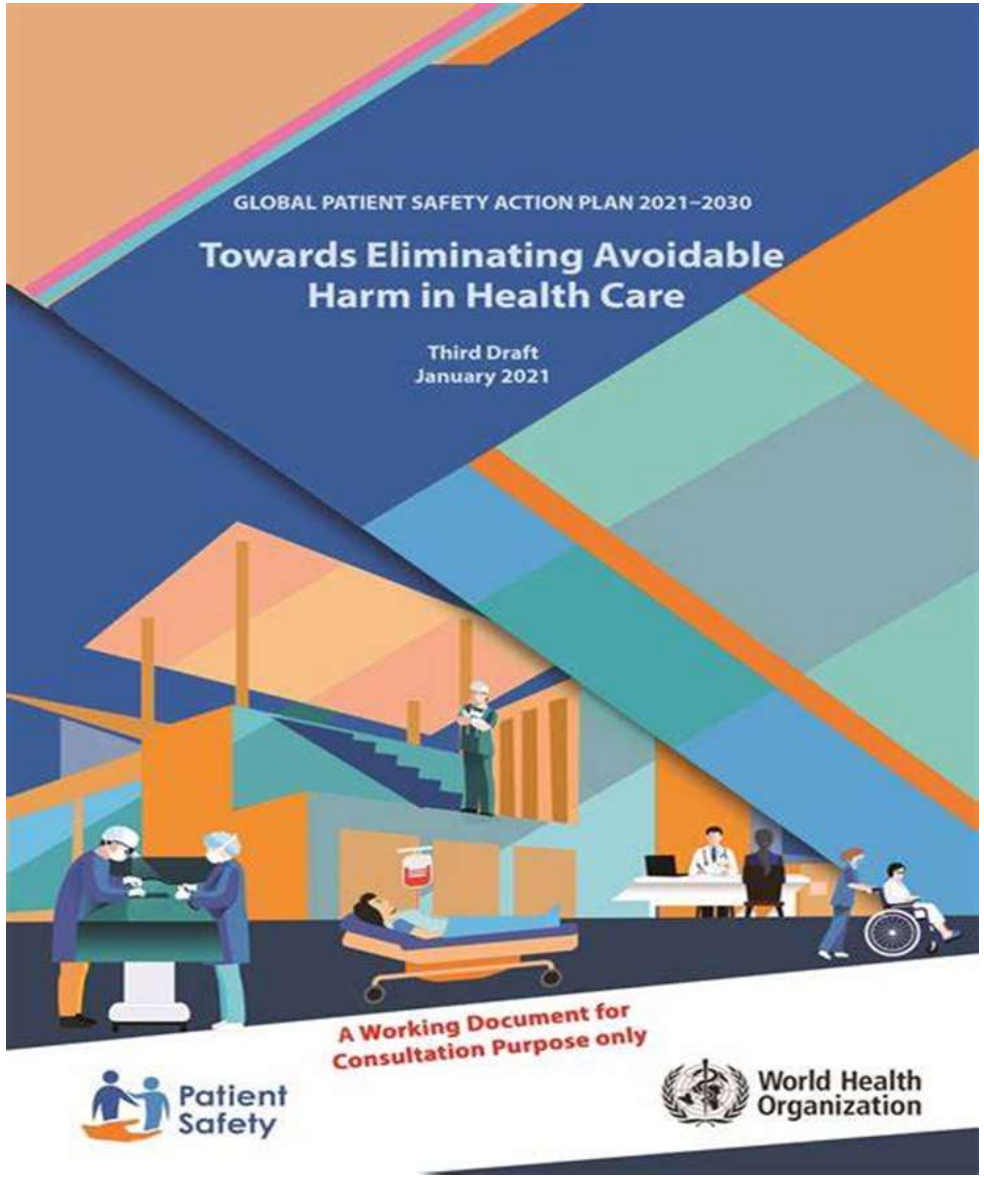
Βαρβάρα Μπαρούτσου

25-5-2023

# Patient Safety

# PATIENT SAFETY

2021-2030



# Ευαισθητοποίηση ,Ενεργοποίηση, Ενδυνάμωση, Συνηγορία

## World Patient Safety Day 17 September 2023

**Objectives of World Patient Safety Day 2023**

1. **Raise** global awareness of the need for active engagement of patients and their families and caregivers in all settings and at all levels of health care to improve patient safety.
2. **Engage** policy-makers, health care leaders, health and care workers, patients' organizations, civil society and other stakeholders in efforts to engage patients and families in the policies and practices for safe health care.
3. **Empower** patients and families to be actively involved in their own health care and in the improvement of safety of health care.
4. **Advocate** urgent action on patient and family engagement, aligned with the Global Patient Safety Action Plan 2021–2030, to be taken by all partners.

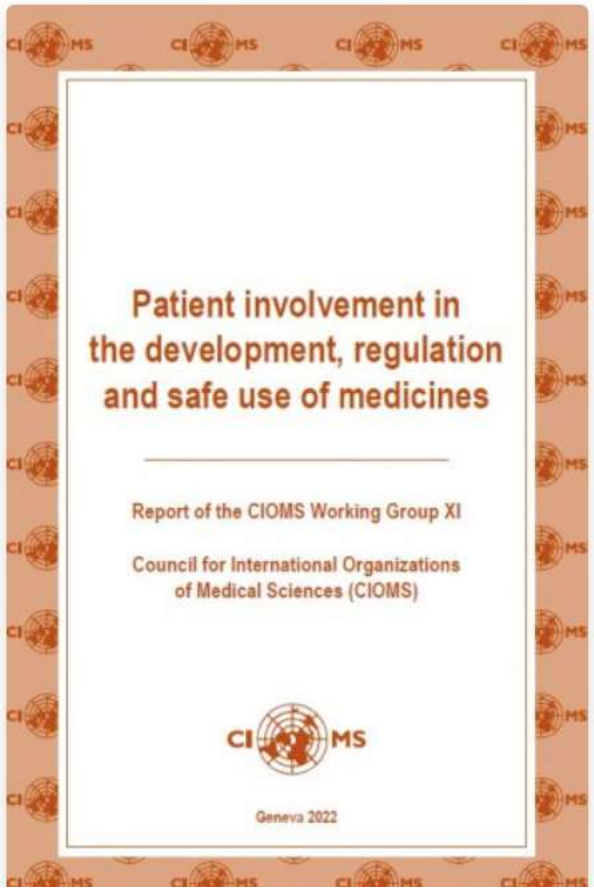
**Working together to make health care safer**

The global campaign for World Patient Safety Day 2023 will propose a wide range of activities for all stakeholders on and around 17 September, including national campaigns, policy forums, advocacy and technical events, capacity-building initiatives and, as in previous years, lighting up iconic monuments, landmarks and public places in the colour orange (the signature mark of the campaign).

# Περίγραμμα

- Ασφάλεια των Ασθενών και χρηστών Επαγγελματιών Υγείας
- Ρυθμιστικές Αρχές & δράσεις
- Ισοζύγιο ωφελειών έναντι κινδύνων
  Πλαίσιο
- Προοπτικές

# Patient involvement in the development, regulation and safe use of medicines




## Working Group XI – Patient Involvement

Participants at the fourth meeting of the Working Group, Basel, 16-17 October 2019

# Ασθενοκεντρική Προσέγγιση Οφέλους έναντι Κινδύνου
# Patient Preferences Benefit –Risk Assessment

frontiers in Medicine

Front Med (Lausanne). 2020; 7: 543046.
PMCID: PMC7649266
Published online 2020 Oct 26. doi: 10.3389/fmed.2020.543046
PMID: 33195294

Use of Patient Preference Information in Benefit–Risk Assessment, Health Technology Assessment, and Pricing and Reimbursement Decisions: A Systematic Literature Review of Attempts and Initiatives

Lylia Chachoua,[1,*] Monique Dabbous,[1] Clément François,[1,2] Claude Dussart,[3] Samuel Aballéa,[1,2] and Mondher Toumi[1,2]



Front Med (Lausanne)

https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7649266/pdf/fmed-07-543046.pdf

**Conclusion:** Despite the initiatives undertaken, the pace **of progress remains slow.** The use of PPs remains **poorly implemented,** and evidence of proper use of these data in decision making is lacking. Guidelines and recommendations formalizing the purpose of collecting PPs, **what methodology** should be adopted and how, and **who should be responsible for generating these data throughout the decision-making processes are needed** to **improve and empower integration of PPs in BRA and HTA**

# Ασθενοκεντρική Προσέγγιση Οφέλους έναντι Κινδύνου Benefit –Risk Assessment of Medicines Systemic Metanalysis


J Med Libr Assoc. 2022 Apr 1; 110(2): 185–204.
Published online 2022 Apr 1. doi: 10.5195/jmla.2022.1306
PMCID: PMC9014953
PMID: 35440905

Patient-based benefit-risk assessment of medicines: development, refinement, and validation of a content search strategy to retrieve relevant studies

Hiba El Masri,[1] Treasure M. McGuire,[2] Christine Dalais,[3] Mieke van Driel,[4] Helen Benham,[5] and Samantha A. Hollingworth[6]





https://www.ncbi.nlm.nih.gov/pmc/articles/PMC9014953/pdf/jmla-110-2-185.pdf

This is the first study, to develop a content search strategy that contains generic keywords and MeSH terms and subheadings that retrieve published data on patient-based BRA of medicines with high sensitivity, specificity, and accuracy (RA 36papers +Lung Cancer15 papers). This search strategy for identifying patient-based BRA of medicines can be used with confidence by not only information specialists but also clinicians or regulators

# Ασθενοκεντρική Προσέγγιση Οφέλους έναντι Κινδύνου Patients Outcomes , Processes, Costs

Received: 24 May 2022 Accepted: 25 August 2022 Published: 20 September 2022

Patient Preference and Adherence Dovepress
open access to scientific and medical research
Open Access Full Text Article REVIEW

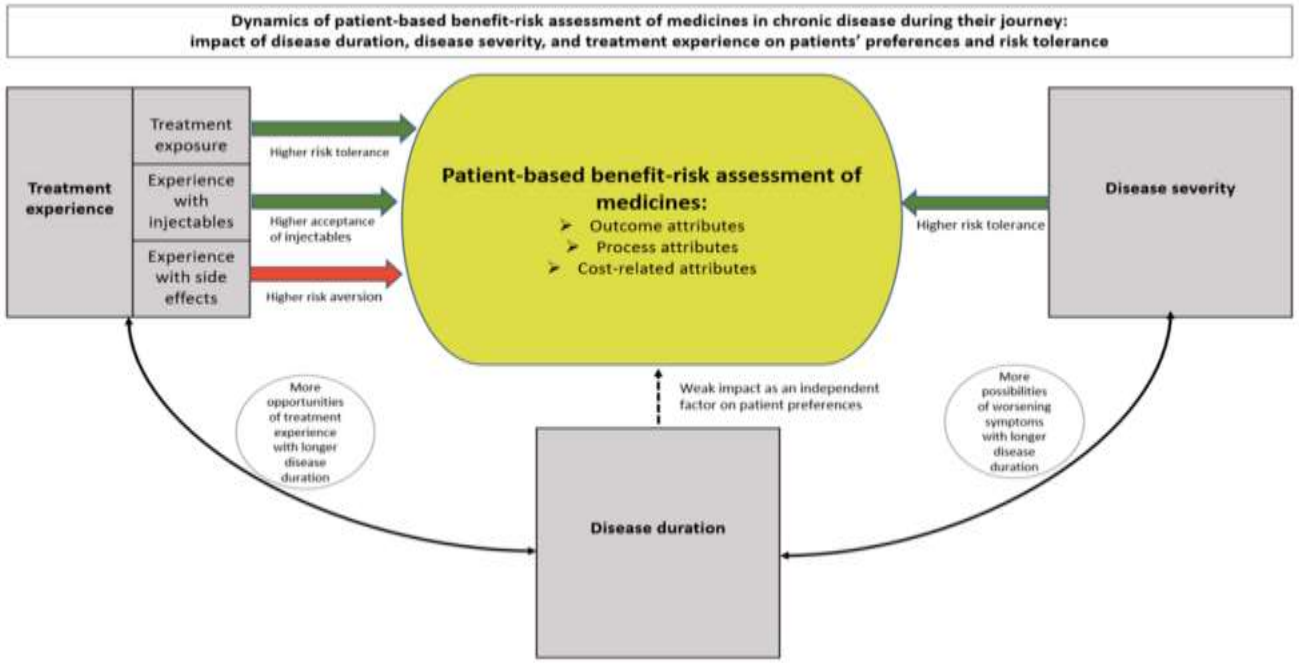
## Dynamics of Patient-Based Benefit-Risk Assessment of Medicines in Chronic Diseases: A Systematic Review

Hiba EL Masri[1], Treasure M McGuire[1–3], Mieke L van Driel[4], Helen Benham[5,6], Samantha A Hollingworth[1]

[1]School of Pharmacy, The University of Queensland, Brisbane, Queensland, Australia; [2]Faculty of Health Sciences and Medicine, Bond University, Robina, Queensland, Australia; [3]Mater Pharmacy, Mater Health, Brisbane, Queensland, Australia; [4]Primary Care Clinical Unit, Faculty of Medicine, The University of Queensland, Brisbane, Australia; [5]Faculty of Medicine, The University of Queensland, Brisbane, Queensland, Australia; [6]Department of Rheumatology, Princess Alexandra Hospital, Brisbane, Queensland, Australia

Correspondence: Hiba EL Masri, School of Pharmacy, The University of Queensland, 20 Cornwall St, Woolloongabba, Brisbane, Queensland, 4102, Australia, Tel +61 478512234, Email h.elmasri@uqconnect.edu.au

https://www.dovepress.com/getfile.php?fileID=84076




**Conclusion:** Factors important for patients' BRA of their medicines during a chronic disease journey vary more with their clinical situation and previous treatment experience. Due to the importance of these factors and potential impact on their decision-making and clinical outcomes, there is a need for more studies to assess the dynamics of patients' BRA in every disease

# Ασθενοκεντρική Ανάπτυξη Φαρμάκων : Να ακούσουμε τους Ασθενείς


# FDA :ασθενοκεντική συμβουλευτική διαβούλευση επί του ισοζυγίου οφέλους έναντι κινδύνων- REM


Figure 1: A Framework for Benefit-Risk Counseling to Patients About Drugs with a REMS


**A Framework for Benefit-Risk Counseling to Patients About Drugs with a REMS: Guiding Principles**

**Principle 1:** Counseling is a necessary activity. It is essential for counseling discussions to take place between a HCP and patient about potential benefits and risks when a drug that has a potential serious risk requiring a REMS is a treatment option and patient understanding or actions are needed to ensure safe use.

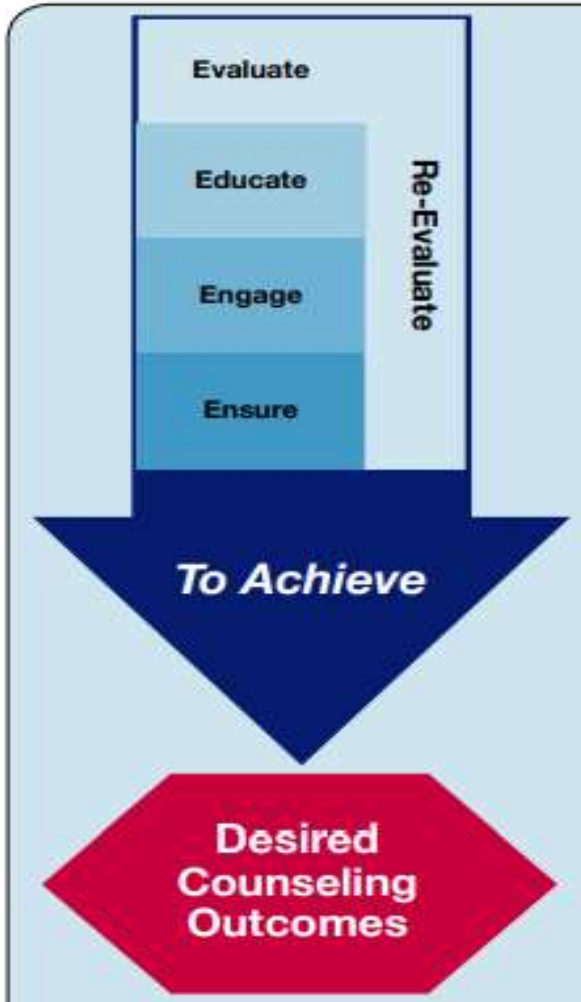
**Principle 2:** Counseling should be ongoing. Effective counseling is a dynamic process that starts with the initial assessment of the patient and should be reinforced at subsequent patient-HCP interactions.

**Principle 3:** Counseling should support collaborative informed decision-making. It should support making informed treatment decisions by a patient and HCP and follow a process of active patient engagement and participation. It should be based on potential benefits and risks, as well as a mutual understanding of patient and HCP obligations under the REMS for appropriate and safe use.

**Principle 4:** Counseling should be individualized. The counseling discussion about the potential benefits relative to risks of different treatments should be individualized to the patient's profile, considering their needs and preferences.

**Principle 5:** Counseling should reflect evidence-based and/or best practices and tools. Counseling about drugs with REMS should incorporate techniques, decision support tools, and other resources for effective risk communication and health care counseling.

# FDA :ασθενοκεντική συμβουλευτική διαβούλευση επί του ισοζυγίου οφέλους έναντι κινδύνων- 4Es




## The 4 E's for a Framework for Benefit-Risk Counseling to Patients About Drugs with a REMS

**Evaluate** the patient's health profile, potential treatment options, and counseling needs. Continuously re-evaluate against desired outcomes.

**Educate** the patient on his or her health condition and treatment options – their benefits, risks, and steps needed to minimize risks (safe use conditions).

**Engage** and support the patient in the treatment decision-making process to the extent that is possible and to the extent that he or she desires.

Help to **Ensure** that the patient understands and is able to adhere to the REMS requirements. If a drug with a REMS is prescribed, re-evaluate at follow-up the patient's experience with treatment and REMS requirements.

**Desired outcomes for the PATIENT:**

- Able to contribute to the treatment decision-making process
- Informed about treatment needs and treatment options
- Confident in ability to adhere to the REMS requirements
- Feels that decisions are right for him or her
- Understands the need to reassess his or her treatment experience over time

**Desired outcomes for the HEALTH CARE PROVIDER:**

- Able to elicit information needed to effectively assess options and counsel
- Confident that the patient understands treatment options and can adhere to the REMS requirements
- Feels that decisions are appropriate for the patient
- Able to make informed decisions about continuing or adjusting treatment

# Εκπαιδευτική προσέγγιση του FDA με τους ασθενείς

**Techniques to consider when educating patients:**

- Ask patients how they prefer to receive information (e.g., using numbers, words or pictures), and how much they wish to participate in decision making
- Use the best available scientific evidence
- Provide information to patients at a literacy level appropriate to supporting their understanding of their condition and their various therapeutic options
- Provide examples of written information (handouts, pamphlets, articles), relevant media programs (videos, podcast), and/or online sources of information
- Use plain language (not technical jargon)
- Convey simple, quantitative evidence of the probability of benefits and harms (e.g., round numbers and denominators, minimize computations, provide comparisons, timeframes, and uncertainties)
- Use tested visual depictions to support the communication of quantitative risk information to improve risk perception and understanding
- When feasible, tested visual aids should depict the underlying event risks separately from the risks related to treatment
- Pictographs, icon array, bar charts and/or graphs can be used depending on the differing types of messages being conveyed and graphical literacy
- Focus discussions on a limited number of key benefit and risk messages
- Attempt to translate the bottom line qualitative meaning or gist of risk information
- Link recommended treatment options to patient treatment goals
- Use the teach back method, asking each patient to teach the HCP about what they have heard using their own words, to confirm their understanding

# EUROPEAN MEDICINES AGENCY

B/R

Assessment

Activities

# EMA Benefit  Risk Assessment Activities

## Learning from a tragedy

Decades after the tragedy, thalidomide and analogues were evaluated by the regulators as potential treatment for different pathologies.

Pregnancy prevention programme had to be agreed between industry and regulators and put in place prior to marketing

But did we consult those who were most concerned?

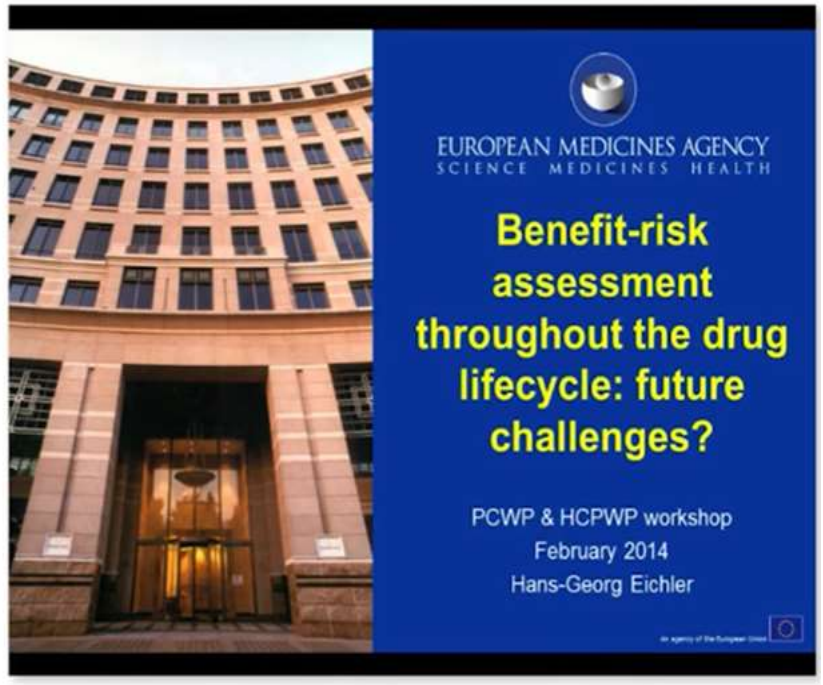
In Europe, EMA invited thalidomide victims and myeloma patients in July 2006 to discuss the Risk management plan, package leaflet and labelling during the evaluation of the marketing authorisation application of lenalidomide, a thalidomide analogue.

# EMA Benefit  Risk Assessment Activities

European Medicines Agency

London, 23 June 2009
Doc ref.: EMEA/40926/2009

**Information on benefit-risk of medicines:**
**patients', consumers' and healthcare professionals' expectations**


https://www.youtube.com/watch?v=VASgg2445rs&ab_channel=EuropeanMedicinesAgency

# EMA Benefit  Risk Assessment Activities

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

26 September 2014
EMA/578072/2014
Press Office

**Press release**

## Patients to discuss benefit-risk evaluation of medicines with the Committee for Medicinal Products for Human Use

EMA launches pilot project to integrate patients' unique and critical views into CHMP discussions

https://www.ema.europa.eu/en/documents/other/pilot-phase-involve-patients-benefit/risk-discussions-chmp-meetings_en.pdf

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

23 October 2014
EMA/372554/2014 – rev. 1
Stakeholders & Communication Division

## Pilot phase to involve patients in benefit/risk discussions at CHMP meetings

https://www.ema.europa.eu/en/news/patients-discuss-benefit-risk-evaluation-medicines-committee-medicinal-products-human-use

# EMA Benefit  Risk Assessment Activities

Benefit-risk methodology

Last updated: 18/06/2018

**Benefit-risk** methodology ... balancing the desired effects or **'benefits'** of a medicine against its ... its undesired effects or **'risks'.** The Agency can recommend ...

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Workshop on benefit-risk of medicines used during pregnancy and breastfeeding

Virtual meeting, 22/09/2020, Last updated: 12/11/2020

Workshop on **benefit-risk** of medicines used during ... Documents Agenda - Workshop on **benefit-risk** of medicines used during ... Generic Report - Workshop on **benefit-risk** of medicines used during ...

https://www.ema.europa.eu/en/about-us/what-we-do/regulatory-science-research/benefit-risk-methodology

https://www.ema.europa.eu/en/events/workshop-benefit-risk-medicines-used-during-pregnancy-breastfeeding

# MINIMIZING THE RISK: PATIENT CONTRIBUTION

## What do we expect from patient engagement in Risk Management

- Input into identification of key areas for additional risk minimisation, choice of appropriate risk minimisation tools and acceptability
- Input into the design of risk minimisation materials needed during CTs
- Input into authorisation decisions regarding risk minimisation measures and monitoring their effectiveness
- Input (including plain language) into risk communication material, "routine" information on medicines, and additional risk minimisation material-testing and developing materials

Minimizing the risk of medicines:
How patient can contribute?

Open meeting on Patient involvement in development and safe use of medicines
CIOMS group XI




Presented by Isabelle Moulon on 30 April 2019
Senior EMA adviser on stakeholders' engagement

# EMA Benefit  Risk Assessment Activities

Patient involvement in the design, implementation and evaluation of additional risk minimization measures: Outline

Framework

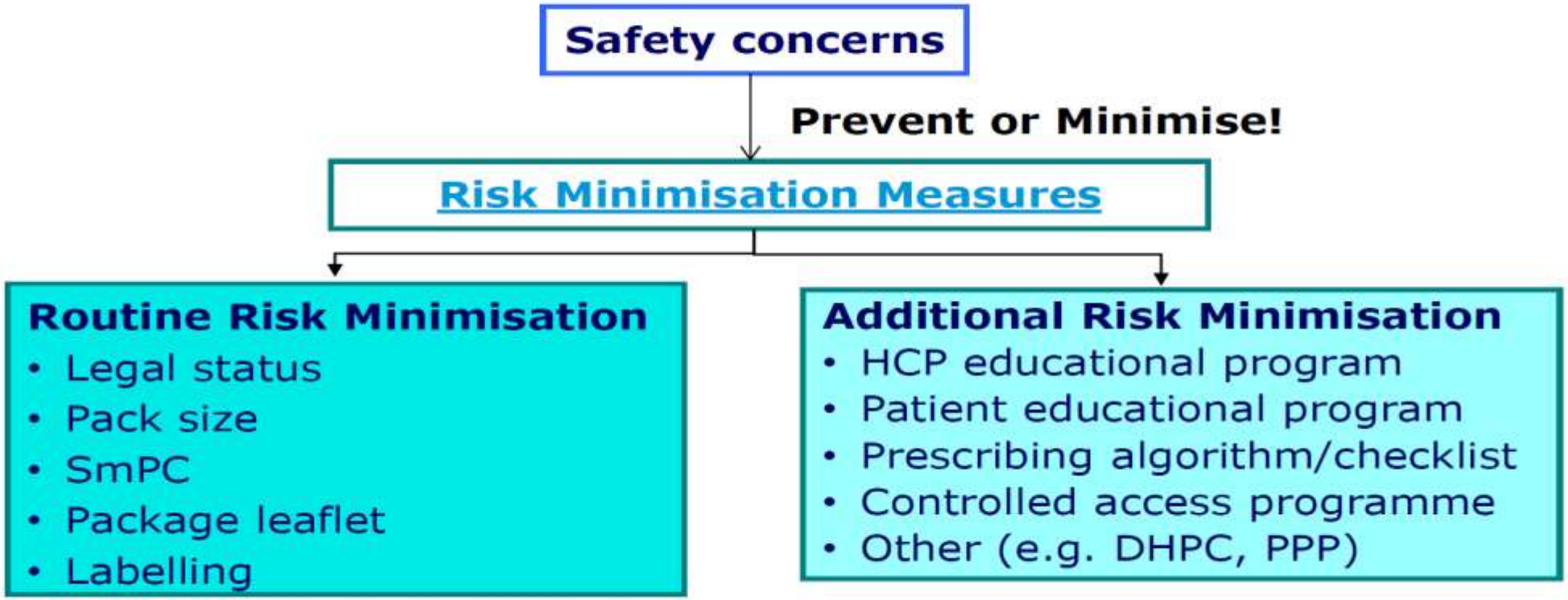
- supporting access to individual patients' real-life experiences of living with a condition, its management and the current use of medicines.
- promoting the generation, collection and use of evidence-based patient experience data for benefit-risk decision-making;
- enhancing patients and consumers understanding of medicines regulation and their role in the process;
- contributing to efficient and targeted communication to patients and consumers to support their role in the safe and rational use of medicines and to foster trust in the EU Medicines Regulatory Network.


https://www.ema.europa.eu/en/documents/other/engagement-framework-european-medicines-agency-patients-consumers-their-organisations_en.pdf

# EMA Benefit Risk Assessment Activities

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

## Risk Minimisation Measures



# EMA Benefit  Risk Assessment Activities

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

## Review of safety communications

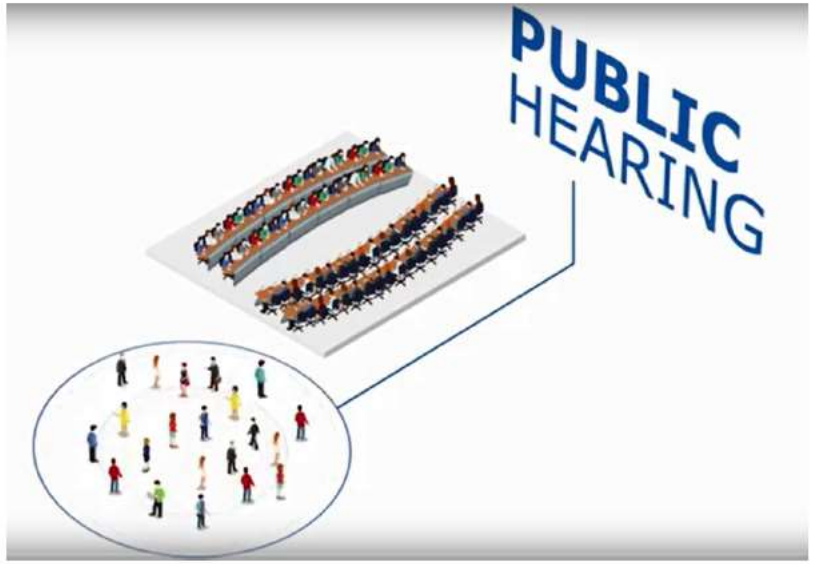

Safety referrals – PRAC recommendation- CHMP /CMDh

## Reporting side effects

# EMA Public Hearing

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

## Attendance to public hearing




2017: Valproate containing medicines

2018: Quinolones and fluoroquinolones

# Benefit  Risk Assessment and stakeholders involved

# FDA Guidance & Initiatives

2009

**Guidance for Industry**

**Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims**

*"Without adequate documentation of patient input, a PRO instrument's content validity is likely to be questioned."*

2018-2021

**Patient-Focused Drug Development: Collecting Comprehensive and Representative Input**

**Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders**

***DRAFT GUIDANCE***

2: Methods to Identify What is Important to Patients
3: Selecting, Developing or Modifying Fit-for-Purpose COAs
4: (Title Forthcoming) COA-related

2018

**Developing and Submitting Proposed Draft Guidance Relating to Patient Experience Data**

Guidance for Industry and Other Stakeholders

***DRAFT GUIDANCE***

Parent Project Muscular Dystrophy

ALS ASSOCIATION

https://www.fda.gov/media/77832/download

# Αλληλεπιδράσεις ΕΜΑ με Ενώσεις Ασθενών και καταναλωτών 2022

**European Medicines Agency**

- Framework for interaction with patients and consumers =
- Scientific Committee, Scientific Advice and Scientific Advisory Groups;
- Review product information and communication material

**Interest and early action in numerous countries**

# Δράσεις Ενώσεων Ασθενών

- Advocacy
- Capacity building
- Fair-market value, conflict of interest, and legal templates

# Πρωτοβουλίες –Συνεργασίες Ενώσεων Ασθενών

prefer.
PATIENT PREFERENCES

PARADIGM
Patients Active in Research and Dialogues for an Improved Generation of Medicines

CLINICAL TRIALS TRANSFORMATION INITIATIVE

- Identify when, and how patients' treatment preferences can be used in medical product development
- Develop processes and tools for three key decision-making points: research priority setting, design of clinical trials and early dialogue
- To develop and drive adoption of practices that will increase the quality and efficiency of clinical trials

# Ενώσεις Ασθενών και Health Technology Assessment

International Society for Pharmacoeconomics and Outcomes Research

HTAi

International Society for Quality of Life Research

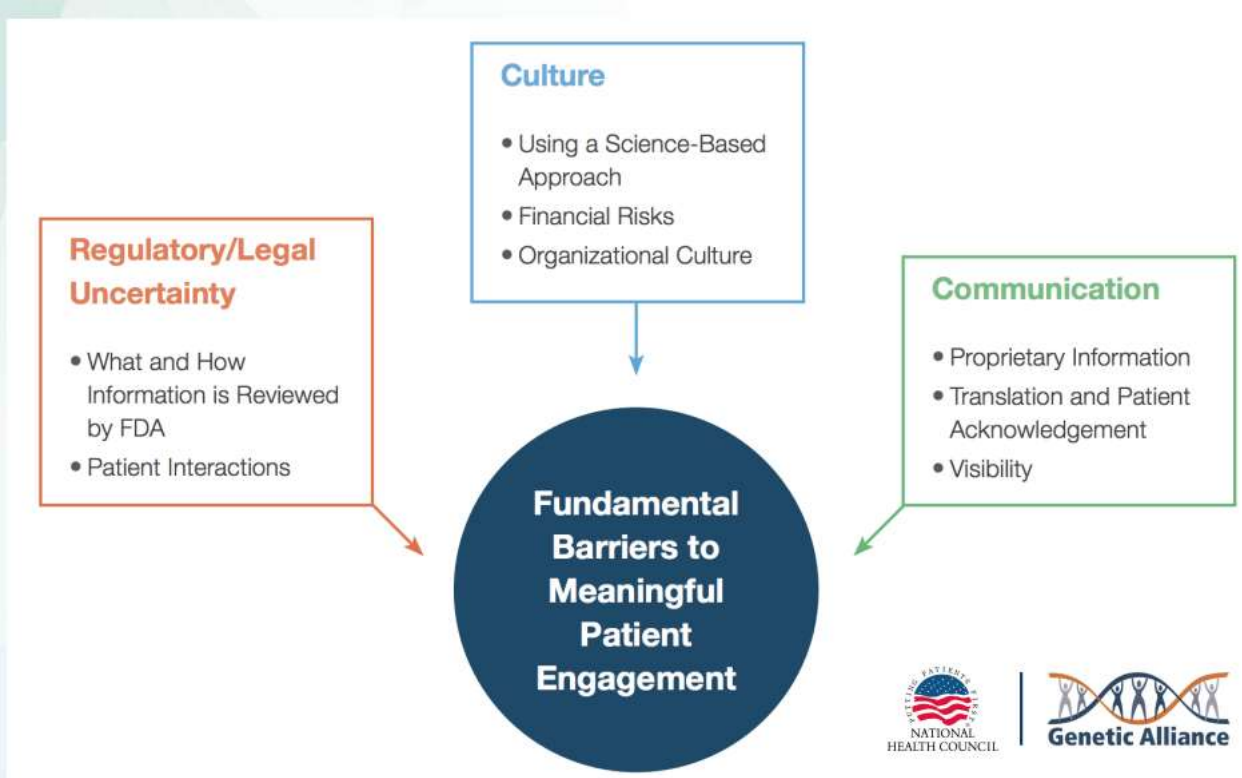
DIA

Clinical societies

Patients Included

# Ενώσεις ασθενών και Οργανισμοί Health Technology Assessment

# Building a Framework for Meaningful Patient Engagement FDA




National Health Council and Genetic Alliance. Dialogue / Advancing Meaningful Patient Engagement in Research, Development, and Review of Drugs.
http://www.nationalhealthcouncil.org/sites/default/files/PatientEngagement-WhitePaper.pdf

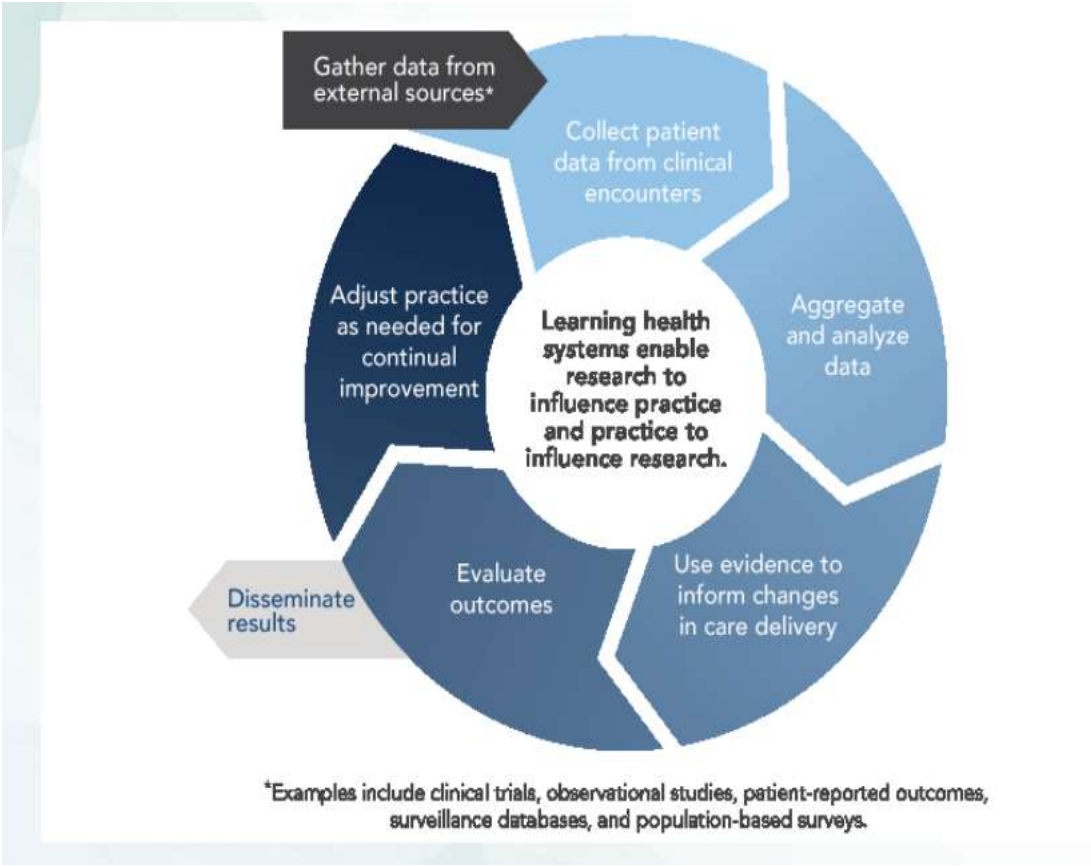
# With whom Regulatory Agencies engage on Research & B/R

Patient

HCPs

Patient Organisations

Pharma Industry



# Οι ασθενείς ως εταίροι στην διαμόρφωση των θεραπευτικών κατευθυντηρίων οδηγιών
# Patient-Public involvement in guidelines

APPRAISAL OF GUIDELINES FOR RESEARCH & EVALUATION II

AGREE II

INSTRUMENT

**STAKEHOLDER INVOLVEMENT**

**5. The views and preferences of the target population (patients, public, etc.) have been sought.**

How to Rate:

**Item content includes the following *CRITERIA:***

- statement of type of strategy used to capture patients'/public's' views and preferences (e.g., participation in the guideline development group, literature review of values and preferences)
- methods by which preferences and views were sought (e.g., evidence from literature, surveys, focus groups)
- outcomes/information gathered on patient/public information
- description of how the information gathered was used to inform the guideline development process and/or formation of the recommendations

Annals of Internal Medicine | CLINICAL GUIDELINE

Guidelines International Network: Toward International Standards for Clinical Practice Guidelines

*A guideline development panel should include diverse and relevant stakeholders, such as health professionals, methodologists, experts on a topic, and patients or other health care consumers.*


3.2 Patient and public involvement should be facilitated by including (at least at the time of clinical question formulation and draft CPG review) a current or former patient, and a patient advocate or patient/consumer organization representative in the GDG.

3.3 Strategies to increase effective participation of patient and consumer representatives, including training in appraisal of evidence, should be adopted by GDGs.

äzq

Corinna Schaefer, Geneva 30.04.2019

G-I-N Public

Guidelines International Network

# Ελληνική Εταιρεία Φαρμακευτικής Ιατρικής

GIN Guidelines International Network

## GIN Public Toolkit:

patient and public involvement in guidelines.

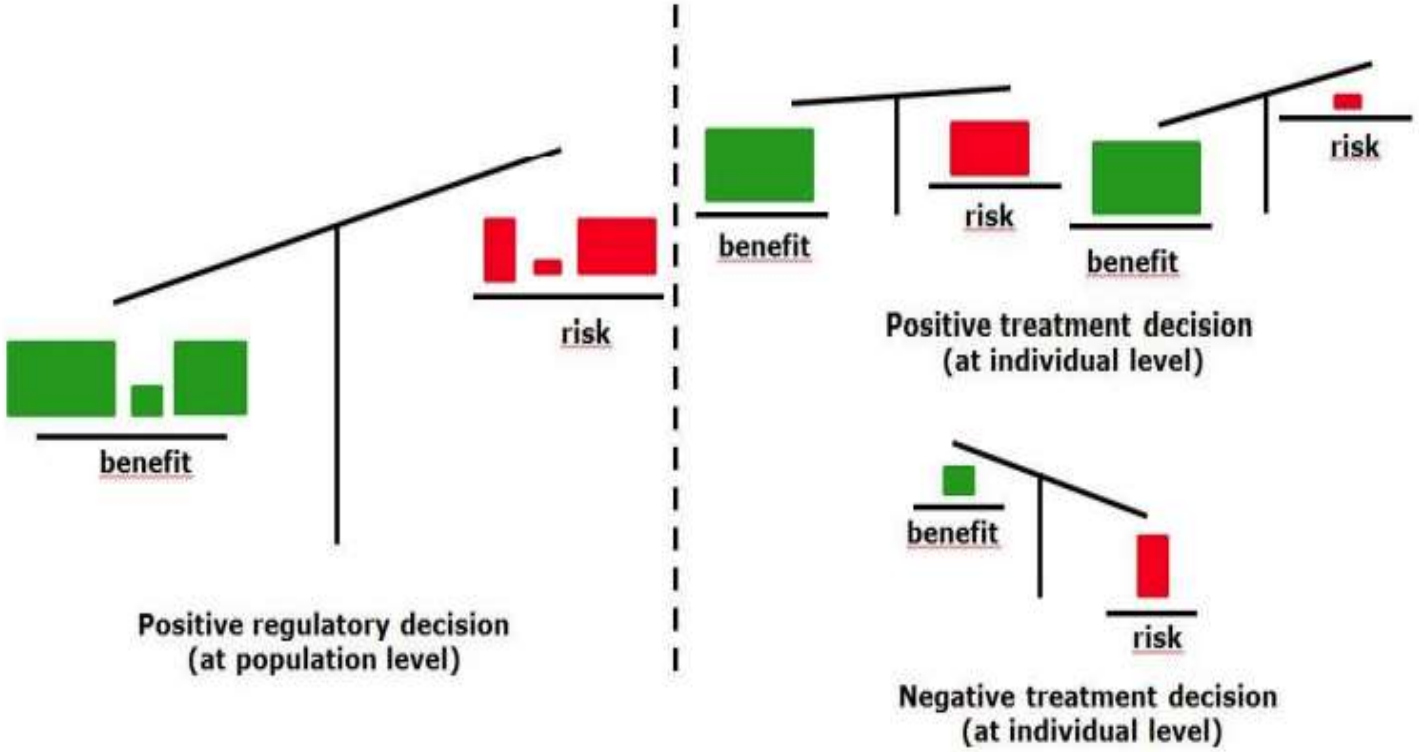
https://g-i-n.net/toolkit


**Three involvement strategies: consultation, participation and communication**

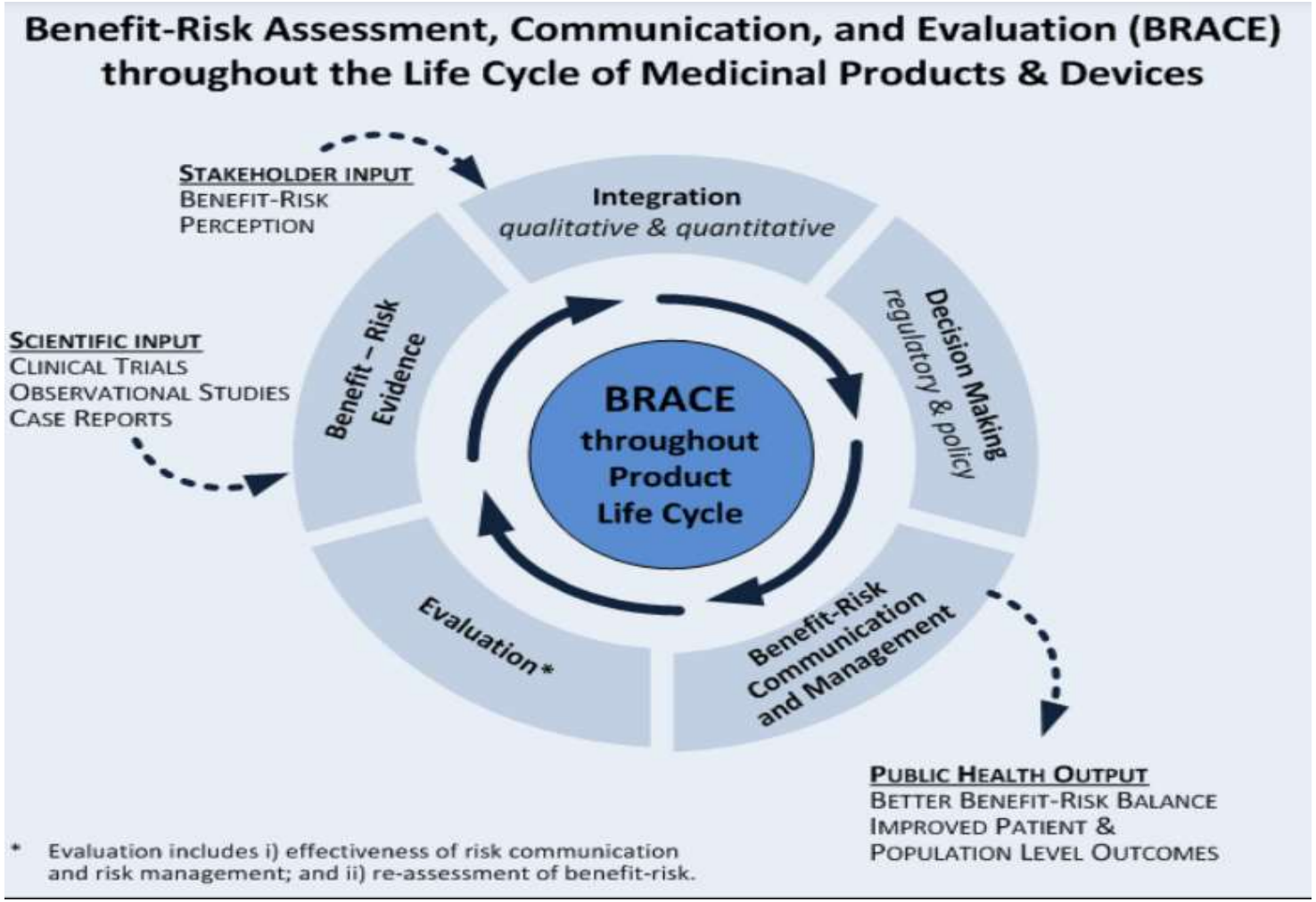
Guideline organisations use a number of different methods to involve patients and the public.[2,3] It is helpful to distinguish three general involvement strategies, based on the flow of information between your organisation and the public:[4]

- **Consultation** strategies involve the collection of information *from* patients and the public. This can include methods such as surveys, focus groups, individual interviews, online consultation, the use of primary research on patients' needs and expectations, or the use of a systematic review of studies on patients' and the public's perspective.
- **Participation** involves the *exchange* of information between guideline developers and the public. This can be done through participation of patient and public representatives on guideline development groups and other methods.[5]
- **Communication** strategies involve the communication of information *to* patients and the public to support their individual health care decisions and choices. This can include the production of plain language versions of guidelines or the development of patient decision aids or education material.

# Benefit Risk assessment- population vs individual assessment

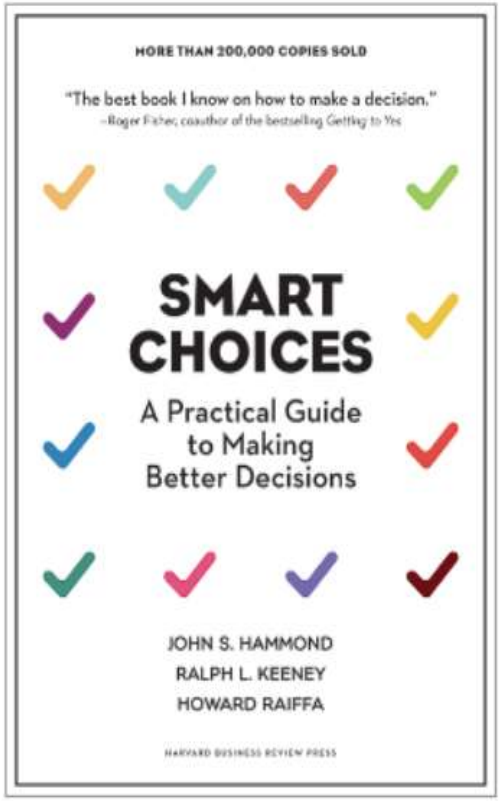


# Iterative process of Benefit Risk Assessment



# Benefit Risk Assessment and Decision Making

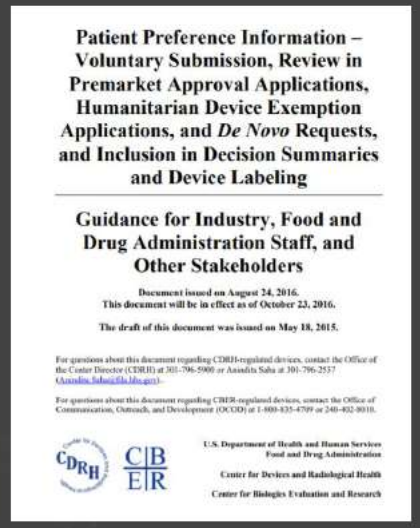
- **Pr**oblem: Is Benefit-Risk balance positive?
- **O**bjective: Goal of therapy? Attributes
- **A**lternatives
  - Approve; reject; (reframe, e.g., restrict indication)
- **C**onsequences of alternatives
  - Estimated based on data
- **T**rade-offs
  - Based on value judgments
- **U**ncertainties (and how to cope with them)
- **R**isk-attitude and **L**inked decisions



# Patient Preferences Information and Regulatory Bodies

## Incorporating PPI in Regulatory environment

FDA U.S. FOOD & DRUG ADMINISTRATION



**Patient Preference Information – Voluntary Submission, Review in Premarket Approval Applications, Humanitarian Device Exemption Applications, and *De Novo* Requests, and Inclusion in Decision Summaries and Device Labeling**

**Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders**

Document issued on August 24, 2016.
This document will be in effect as of October 23, 2016.

The draft of this document was issued on May 18, 2015.

For questions about this document regarding CDRH-regulated devices, contact the Office of the Center Director (CDRH) at 301-796-5900 or Anadita Saha at 301-796-2557 (Anadita.Saha@fda.hhs.gov).

For questions about this document regarding CBER-regulated devices, contact the Office of Communication, Outreach, and Development (OCOD) at 1-800-835-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Devices and Radiological Health
Center for Biologics Evaluation and Research

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

January 2021
EMA/97615/20212021
Stakeholders & Communication Division

**Pilot phase for CHMP early contact with patient / consumer organisations**

**Background and rationale**

Patients and their representatives are involved in many activities at EMA and the added value of including their perspectives within committee evaluations has been well demonstrated.

They are currently involved at various timepoints during the medicines' lifecycle, including CHMP evaluations. However, requests for patient input generally come at a later stage of the evaluation, often once major objections have been identified (e.g. expert meeting, oral explanation). Experience shows that late input may lead to missed opportunities to properly incorporate patient perspectives into the assessment process. Therefore in order to make current engagement practices more efficient and enhance timely participation, it is proposed to establish contact with relevant patient / consumer organisations at the start of new medicines assessment. This will enable patients to share aspects such as quality of life, treatment options and unmet medical needs so that the CHMP is well-aware of all aspects from the beginning. This is also expected to facilitate further interactions with patients as the procedure progresses.

This proposed action and process improvement is in line with both the CHMP work plan objective to: 'Incorporate additional and regular processes to capture and include patients' views and preferences in the benefit/risk evaluations', and EMA's Regulatory Science Strategy recommendations which highlight the need to enhance methods to systematically incorporate patient data in regulatory decision-making.

**Legal basis**

Article 78 of Regulation (EC) Nº 726/2004 allows EMA scientific committees to establish contacts on an

**US Food and Drug Administration (FDA)** - Center for Devices and Radiological Health & Center for Biologics Evaluation and Research

- Guidance on how to collect patient preference
- Recommendation on incorporating data into a benefit-risk assessment framework
- Recommendation on including preferences information in labelling
- Voluntary submission of preference data
- **Discrete Choice Experiments, the most suitable methods for eliciting PPI**

**....this proposed action and process improvement is in line with both the CHMP work plan objective to: 'Incorporate additional and regular processes to capture and include patients' views and preferences in the benefit/risk evaluations', and EMA's Regulatory Science Strategy recommendations which highlight the need to enhance methods to systematically incorporate patient data in regulatory decision-making.**

# EMA&FDA umbrella of Patient Experiences

## Patient preferences ≠ PROs

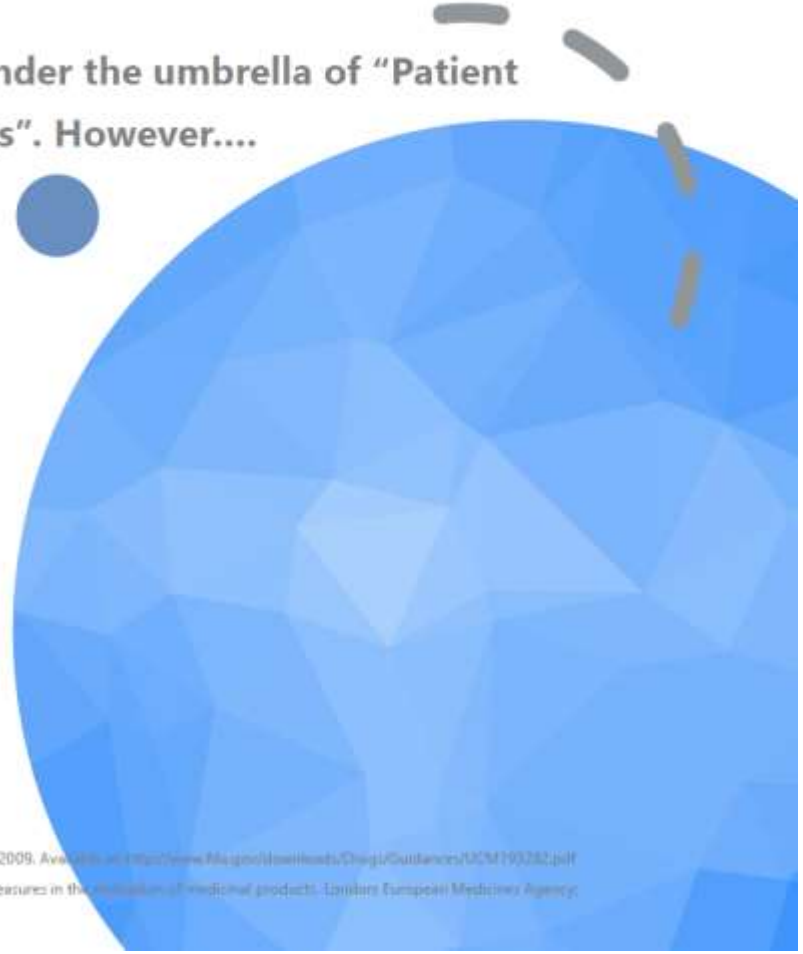
Both fall under the umbrella of "Patient experiences". However....

**Patient Reported Outcomes**

"any report of the status of a patient's health condition that comes directly from the patient, without interpretation of the patient's response by a clinician or anyone else" [1,2]

**Patient Preferences**

"measurements of the relative importance of treatment characteristics/benefits/risks of treatments regardless of whether the treatments currently exist.."



1. FDA Guidance for Industry. Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims. 2009. Available at: http://www.fda.gov/downloads/Drugs/Guidances/UCM193282.pdf

2. European Medicines Agency(EMA) Reflection paper on the regulatory guidance for the use of health related quality of life (HRQL) measures in the evaluation of medicinal products. London: European Medicines Agency; 2005

# B/R HISTORIC PERSPECTIVE

Therapeutic Advances in Drug Safety

Ther Adv Drug Saf. 2019; 10: 2042098619871180. PMCID: PMC6712756
Published online 2019 Aug 26. doi: 10.1177/2042098619871180 PMID: 31489173

## Benefit-risk evaluation: the past, present and future

Juhaeri Juhaeri

▸ Author information ▸ Article notes ▸ Copyright and License information Disclaimer

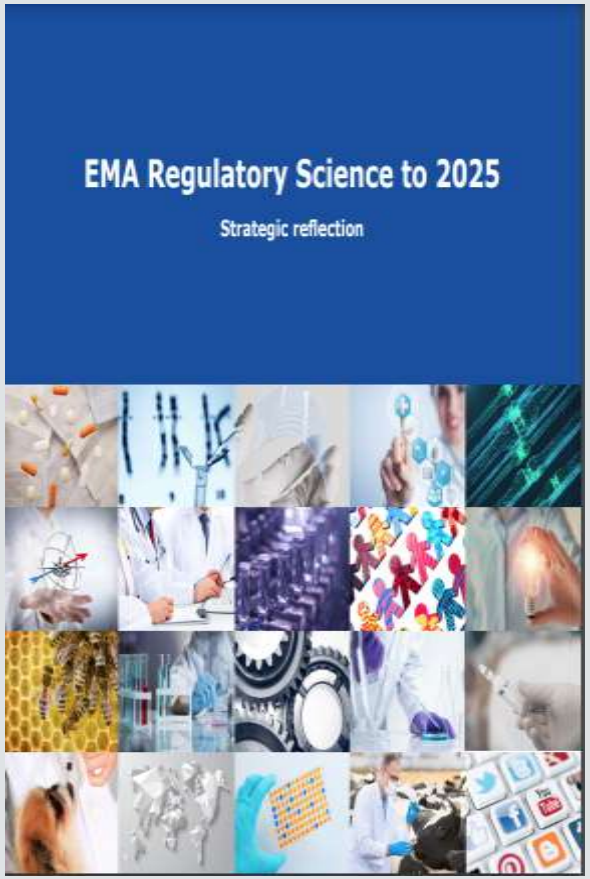
Abstract Go to: ▸

In the last two decades there has been a shift in the approach to evaluating the benefit-risk (BR) profiles of medicinal products from an unstructured, subjective, and inconsistent, to a more structured and objective, process. This article describes that shift from a historical perspective; the

Ther Adv Drug Saf

There has been great improvement in the BR field in the last 2 decades, from a subjective and inconsistent, to a more structured, transparent and consistent approach, with a number of quantitative methods to incorporate preference weight from various stakeholders. While this development is encouraging, there is still more work to be done.

Benefit-Risk Balance




https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/ema-regulatory-science-2025-strategic-reflection_en.pdf

## Goal 3: Advancing patient-centered access to medicines in partnership with healthcare system

- Bridge from evaluation to access through collaboration with payers: Collaborate with stakeholders to monitor the performance (safety and effectiveness) of products newly launched on the market (learning healthcare system), and link to the planning of evidence through risk management plans (RMPs).
- Reinforce **patient relevance in evidence generation: Coordinate the approach to patient reported outcomes (PROs)and Patient Preferences Studies (DCE);**
- Promote use of high-quality real-world data (RWD) in decision making: Review of the utility of using **electronic health records for detecting drug safety issues (including drug interactions)**
- Develop network competence and specialist collaborations to engage with big data
- Deliver improved product information in **electronic format (ePI)**
- Conduct research on optimising the impact of **risk communication in changing the behaviour of patients and healthcare professionals, including as part of risk management and pharmacovigilance**

# Συμπερασματικά

B/R

- Βελτίωση επικοινωνίας αξιολόγησης του B/R με τους Ασθενείς , Επαγγελματίες Υγείας ως κύριους εταίρους

B/R

- Διαφάνεια και Δάλογος με τους εταίρους για την αξιολόγηση B/R
- Εγκαιρη έναρξη του Διαλόγου για το B/R

B/R

- Η συνεχής αξιολόγηση του BR θα έχει μέγιστη απόδοση εάν πέραν των εγκριτικών αρχών και της Φ/Β συμμετέχει και συμβάλει η κοινότητα των ασθενών & των υγειονομικών

# ΣΑΣ ΕΥΧΑΡΙΣΤΩ ΠΟΛΥ ΓΙΑ ΤΗΝ ΠΡΟΣΟΧΗ ΣΑΣ

Ερωτήσεις

email : president @elefi.gr

**ΕΛ.Ε.Φ.Ι.**

ΕΛΛΗΝΙΚΗ ΕΤΑΙΡΕΙΑ
ΦΑΡΜΑΚΕΥΤΙΚΗΣ
ΙΑΤΡΙΚΗΣ

Μαιάνδρου 23, 115 28 Αθήνα | T 210 7211845 | E info@elefi.gr | W www.elefi.gr