

POLICY AND ECONOMICS CONFERENCE
CANCER
& **PERSONALIZED MEDICINE**

Πέμπτη 17 Οκτωβρίου 2019, Αθήνα

Διοργάνωση HealthDaily
conferences

A POLICY DEBATE ON THE VALUE & COST OF TREATMENT

Newsline | communications

HTA & Reimbursement Issues of Personalized Therapies : The patient perspective

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Partner PharmEcons Easy Access

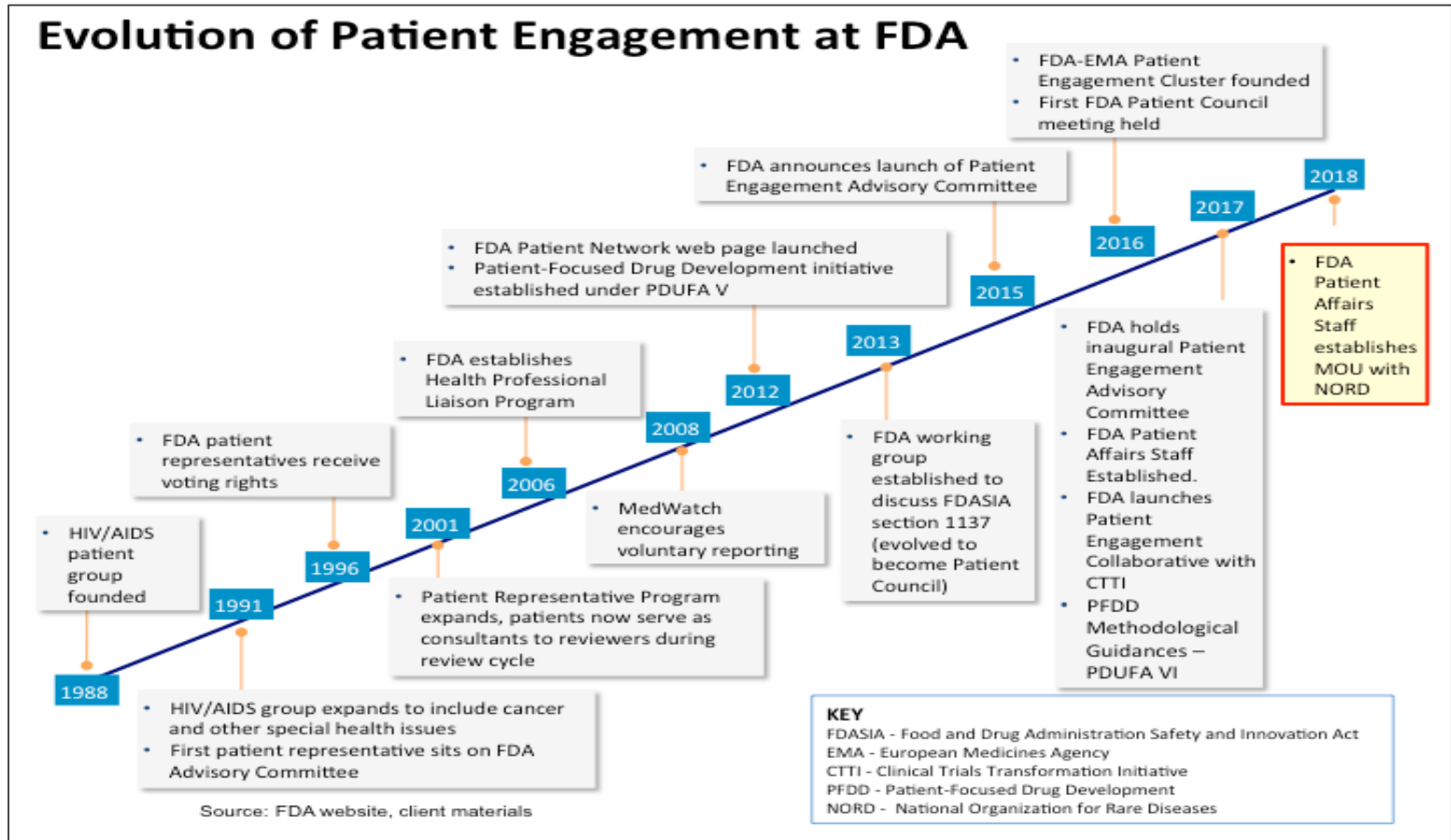
17th October 2019



Breast Cancer Patients' participation in Decision Making Process: The Experience of Discrete Choice Experiment from Europe

Patient Engagement at FDA

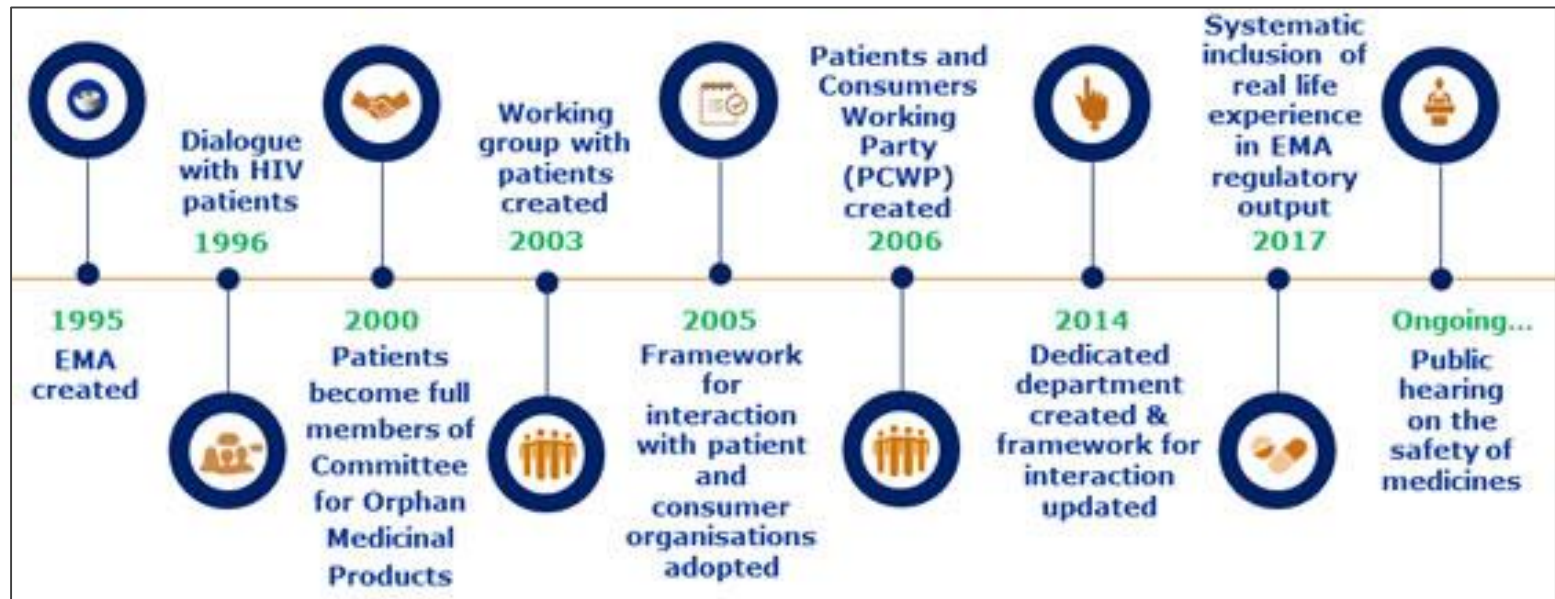
Patient Representatives involvement in decision making since 1996



Patients' involvement at European Medicines Agency (EMA)

Key milestones of EMA interaction with patients

- Only since 2017 the systematic inclusion of real life experience (patients' involvement) was introduced at EMA



Sources:

- <https://www.ema.europa.eu/en/partners-networks/patients-consumers>
- <https://www.fda.gov/forpatients/patientengagement/default.htm>

Patient Engagement in Greece

ΕΦΗΜΕΡΙΔΑ ΤΗΣ ΚΥΒΕΡΝΗΣΕΩΣ ΤΗΣ ΕΛΛΗΝΙΚΗΣ ΔΗΜΟΚΡΑΤΙΑΣ

17 Ιανουαρίου 2018

ΤΕΥΧΟΣ ΠΡΩΤΟ

Αρ. Φύλλου 5

ΝΟΜΟΣ ΥΠ' ΑΡΙΘΜ. 4512

Ρυθμίσεις για την εφαρμογή των Διαρθρωτικών Μεταρρυθμίσεων του Προγράμματος Οικονομικής Προσαρμογής και άλλες διατάξεις.

ουργία και τήρηση ψηφιακών γεωχωρικών δεδομένων στο πλαίσιο των αρμοδιοτήτων του.

3. Η ανώνυμη εταιρεία με την επωνυμία «ΕΘΝΙΚΟ ΚΤΗΜΑΤΟΛΟΓΙΟ ΚΑΙ ΧΑΡΤΟΓΡΑΦΗΣΗ Ανώνυμη Εταιρεία», η οποία συστάθηκε με την υπ' αριθμ. 81706/6085/1995

Άρθρο 250:

Παρ. 7.: Η Επιτροπή Αξιολόγησης μπορεί να καλεί εκπροσώπους συλλόγων ασθενών και επιστημονικών σωματείων ή εταιρειών ιατρικών ειδικοτήτων για να εκφράσουν τις απόψεις τους.

Patient Input in Total Product Lifecycle

Discovery & Ideation	<ul style="list-style-type: none">• Patient Preferences<ul style="list-style-type: none">○ Usability & Needs• Patient Owned Data<ul style="list-style-type: none">○ Comparisons, identified problems and unmet needs
Preclinical	<ul style="list-style-type: none">• Patient Informed Clinical Trial Design• Patient Reported Outcomes• Patient assembled Cohorts
Clinical	<ul style="list-style-type: none">• Regulatory Decision<ul style="list-style-type: none">○ Benefit-Risk Determination○ Labeling
Product Launch	<ul style="list-style-type: none">• Shared Clinical Decision Making
Post Market Monitoring	<ul style="list-style-type: none">• Patient Reporting Outcomes• Health Technology Assessment Participation• Benefit-Risk Determination• Patient Directed Communications

Sources:

[1] <https://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM446680.pdf>

Impact of patient involvement

The view of Regulatory and HTA authorities

“Patient involvement has the highest impact on ‘understanding of the impact of technologies in a real-life context’ (e.g. barriers to complying with current therapy, side-effects, the patient’s ability to pay, etc.), ‘understanding of the quality of life aspects’, ‘accuracy in measuring needs and preferences of patients’, and ‘quality of assessment and comprehensive information’.^[5]”

European Patients Forum (EPF)

Sources:

[5] <http://www.eu-patient.eu/globalassets/projects/hta/hta-epf-final-report2013.pdf>

Impact of patient involvement

“Patient advocate success stories demonstrate reduced medical costs and improved outcomes for patients”



“**Accessing treatment:** Severe OCD 32 years old. She contacted OCD Action because after years of fighting for the specialist support she needs, she had reached a dead end. Finally, her local care team agreed to refer her to a center of excellence.”

ocdaction

Reduction in Out-Patient Prescription Drug Costs
Reduce cost barriers to consumers accessing prescription drugs by **1) requiring insurers to cover medically necessary prescription drugs**, including those for which there is no therapeutic equivalent; **2) requiring formularies to be based on clinical guidelines and peer-reviewed scientific evidence** first and not only on cost and, **3) capping out of pocket drug costs at no more than \$275** for a 30 day prescription

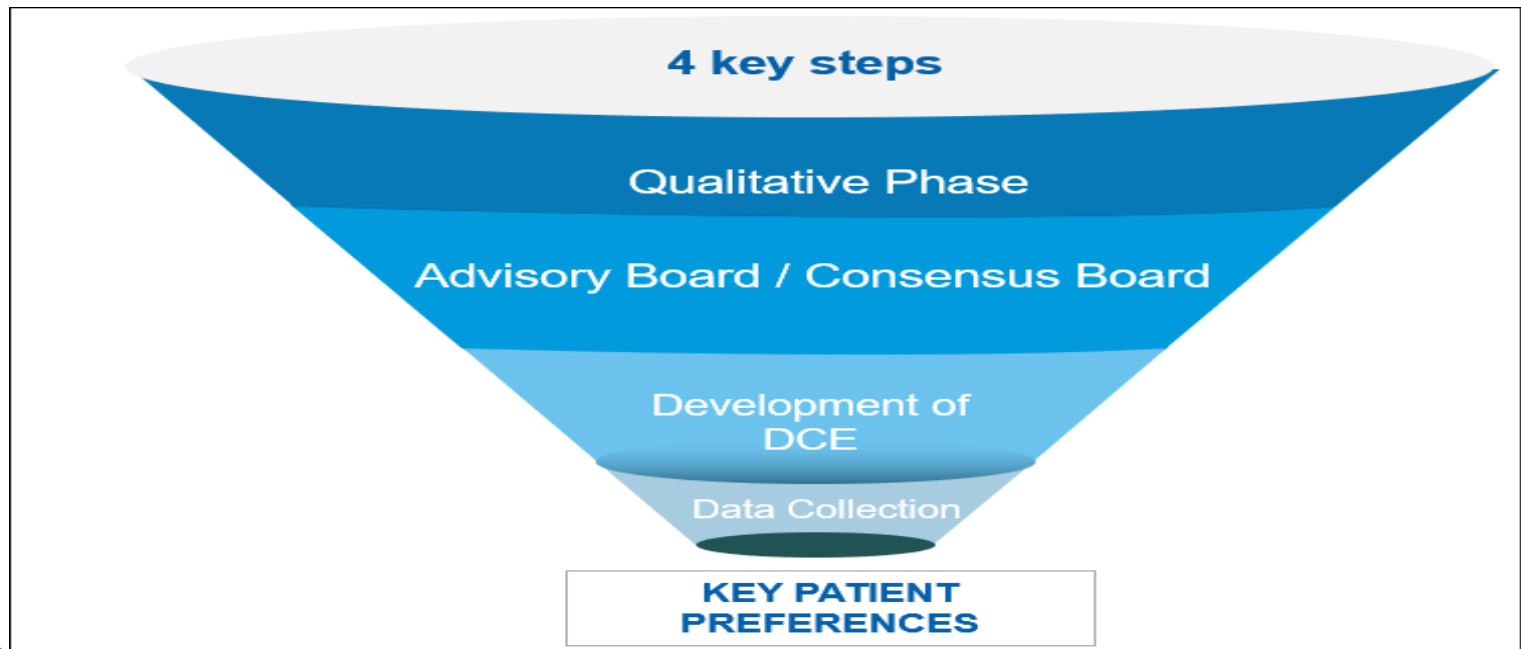


Sources:

- [HTTTPs://www.guardiannurses.com/](https://www.guardiannurses.com/)
- <https://www.ocdaction.org.uk/>
- <https://www.nationalmssociety.org/>

What are DCEs

- DCE: A **QUANTITATIVE** method that measures **individuals' (patients') preferences** and allows **examination of trade-offs** they make for different options of health care services and interventions
- Participants are presented with **alternative *hypothetical* scenarios** and **asked to indicate their most preferred option**, with each option involving several attributes



DCEs and understanding preferences

Understanding individual preferences...



“All my decisions are well thought out.”

Applications of DCE in health care research

- Elicit preferences for a wide range of health applications:
 - Treatments: cancer, asthma, diabetes
 - Testing and screening: colorectal cancer, genetic counselling, depression
 - Prevention: HIV
- Decisions on the design and provision of healthcare services
- Decisions on workforce recruitment
- Decisions on the design of RCTs

Breast Cancer Patients Preference Survey: A Discrete Choice Experiment



of its kind Discrete Choice Experiment conducted to understand Breast Cancer patients preferences in EU

4

Countries



371

Patients



11

External experts



1

Renowned
Health economist



Prof. Mandy Ryan
University of Aberdeen

3

Major
Conferences



1

Manuscript



1

Comprehensive
ORE tool kit



OBJECTIVE OF DCE: Quantified patients' treatment preferences & Opportunity to collaborate with PAGs and HC Systems towards better informed decision making

Why did we conduct the pivotal BC DCE ?



1 Address the increasing request for patient involvement in decision making

Understand and elicit quantified patient preferences

3 Compensate for the lack of RWE at the time of launch

Study Objectives

- 01** Understand BC patients' perspectives in the choice of treatment for their disease
- 02** Gain information on patients' willingness to accept trade-offs between treatment features
- 03** Highlight those treatment characteristics that are valued as most important from patients' perspective
- 04** Demonstrate the added value of the treatment based on its valued features

DCE - Breast Cancer Patients

Preferences in 4 European Countries

Objectives of survey: Which treatment characteristics and outcomes are the most important to patients with breast cancer

Attributes & Levels

- Progression Free Survival (in months)
- Toxicities – in our case expressed as Febrile Neutropenia
- Pain (None/mild), moderate, severe
- Functional wellbeing: Not impaired/mildly impaired, moderate impaired, severe impaired
- Willingness to pay

Web-based survey

Please view each choice set independently, there is no need to remember previous choices. There are no wrong or right answers.

Task 4 of 18

	Treatment A	Treatment B	No treatment
Progression-free survival (PFS):	15 months Two women in every 100 (2% chance of occurring)	10 months One woman in every 100 (1% chance of occurring)	5 months 0 women in every 100 (0% chance of occurring)
Febrile neutropenia:			
Pain:	Severe	None/Mild	Severe
Functional wellbeing:	Not impaired/Mildly impaired	Severely impaired	Severely impaired
The out-of-pocket expenses:	Euros 5,150	Euros 0	Euros 0
	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Results of DCE Breast Cancer Survey

ISPOR Europe 2019

2-6 November 2019 | Copenhagen, Denmark
#ISPOREurope

BREAKOUT SESSION 6
P9: STATED PREFERENCE & PATIENT SATISFACTION
RESEARCH

PODIUM PRESENTATION

Tuesday, November 5, 2019
14:15 - 15:15

Pros and cons of DCEs

Why not use DCEs

- Cannot fully depict the “real world”
- Limited transferability of the results?
- Cognitively demanding for the respondents?

Why use DCEs

- Give answer to policy relevant questions
- Easy to design, estimate and interpret
- Easy for the individuals to complete the task

The End





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