Optimizing Patient Involvement in Payer Health Care Decisions to Access New Therapies

ISPOR 18th Annual European Congress
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Speakers

Don Husereau, MSc, BSc, Senior Associate, Institute of Health Economics; Senior Scientist, University for Health Sciences, Medical Informatics and Technology; Adjunct Professor, University of Ottawa, Ottawa, ON, Canada

Nicola Bedlington, Director, European Patients' Forum, Brussels, Belgium

Michael Barry, MD, PhD, Clinical Director, National Centre for Pharmacoeconomics, Dublin, Ireland

Veronica Foote, Head of Patient Relations & External Communications, Novartis Oncology Region Europe, Surrey, UK
Case Studies in Health Technology Assessment

Don Husereau, MSc, BSc,
Senior Associate, Institute of Health Economics; Senior Scientist, University for Health Sciences, Medical Informatics and Technology; Adjunct Professor, University of Ottawa, Ottawa, ON, Canada
ISPOR Tools for Patients

Collaboration with Global Organizations

ISPOR Partners with the European Patients’ Academy (EUPATI)
ISPOR leads Task Force 4 of Work Package 4 (curriculum development) to develop educational modules for patients on health technology assessment and the economics of health care. ISPOR strives to provide patients with relevant knowledge and links to help them informed decisions regarding their health.

Patient Involvement in Health Technology Assessment in Europe: A study of the European Patient Forum

ISPOR Patient Centered Initiatives

Patient Organizations Worldwide
An ever-expanding list of patient organizations from different regions of the world.

ISPOR Patient Representatives Roundtable
Patient Representatives Roundtable provides an opportunity for patient representatives to learn and discuss how they can participate in the assessment of a new health technology (drug, medical device, diagnostics) and increase their involvement in health policy decision making.

Case Report of Patient Involvement in HTA

ISPOR Patient Centered Special Interest Group
The goal is to determine how best to involve patients and their representatives, in all stages of the decision making, to improve health care delivery and outcomes.

Videos on Patient Topics

ISPOR YouTube channel

ISPOR Educational Videos
PCORI’s Programs to Assure the Patient is the Center of Effectiveness Research
Joe W. Selby, MD, MPH, PCORI, Washington, DC, USA

What Does “Patient-Centered Outcomes” Mean?
Donald L. Patrick, PhD, MSPH
University of Washington, Seattle, WA, USA

FDA’s Patient-Focused Drug Development Initiative
Theresa Mullin, PhD
U.S. FDA, Silver Spring, MD, USA

See more videos

Other Videos
eMEET video on The Role of Patient Advocacy
Case Template

- Developed by the ISPOR European Patient Representative Roundtable

- Available in .ppt, .rtf

- 1 page – based on other initiatives in regulatory involvement
Learnings, to date

- Patients tend to be consulted, rather than active collaborators
- Most activity focused on presentations to HTA expert committees

**Benefits:** Perception that views are heard and that patients can influence reimbursement recommendations or pricing decisions

**Challenges:** Time, lack of adequate training, resources, differences across jurisdictions, not always included in all aspects of collaboration
Learnings, to date

- There is a consensus that interaction with HTA organizations has been positive. However:
  - Patients require better understanding and training
  - HTA organizations need to involve patients earlier
  - HTA organizations need to be more transparent
  - In one case, a patient group felt like HTA placed too much emphasis on costs rather than individual patient benefit
ISPOR PATIENTS’ ROUNDTABLE
EPF COLLABORATION IN THE HTA/PAYER ENVIRONMENT

Nicola Bedlington
EPF Secretary General

13 October 2015
PPRI conference

“ A STRONG PATIENTS’ VOICE TO DRIVE BETTER HEALTH IN EUROPE ”
About the European Patients’ Forum

- Independent, non-governmental umbrella organisation set up in 2003

- **OUR VISION:** All patients in the EU have equitable access to high quality, patient-centred health and social care

- **OUR ROLE:** United patients’ voice in EU health and social policy

- **OUR MEMBERS:** disease-specific EU & national coalitions – 64 member organisations

“A STRONG PATIENTS’ VOICE TO DRIVE BETTER HEALTH IN EUROPE”
Access and equity – a major concern

Healthcare provision must be based on the fundamental values of equity and solidarity.

Innovative treatments that add real value should be accessible to all patients, not only those who can pay.

A holistic approach is needed that embraces not only therapeutic innovation, but also systems and societal innovation.

Huge disparities across the EU and within countries in access to even basic healthcare, let alone innovative treatments.

“A STRONG PATIENTS’ VOICE TO DRIVE BETTER HEALTH IN EUROPE”
Political will and leadership

Autumn 2013
Lithuanian EU Presidency Conference on Sustainable Health Systems – Vilnius Declaration

April 2014
Commission Communication on effective, accessible and resilient Health Systems

June 2014
Council Conclusions on the Economic Crisis and Impact on Health

December 2014 Council Conclusions New medicines for the benefit of patients


A STRONG PATIENTS’ VOICE TO DRIVE BETTER HEALTH IN EUROPE
The Patient Access Partnership

Working towards a European Partnership for equity of access to quality healthcare

New momentum: EPF Election Campaign
A multi-stakeholder partnership with patients, healthcare professionals, healthcare industries, and health experts, and key decision makers

Wide Definition of Access – a holistic approach

Objectives
• Join forces to explore solutions to overcome inequities, based on individual and collective expertise
• EU political agenda- MEP Interest Group
• Commission’s Expert Panel Report on Access

“A STRONG PATIENTS’ VOICE TO DRIVE BETTER HEALTH IN EUROPE”
Promoting Innovation – Key dimensions

WHO Priority Medicines Report

- **Public Health Based R&D**
- **Public Private Partnerships** learning from IMI
- **Models for Stakeholder Involvement including patients and citizens**
- **Redesigning of the Regulatory System**
- **Real life data and learning from practice**
- **New pricing and reimbursement mechanisms for innovation**

“A STRONG PATIENTS’ VOICE TO DRIVE BETTER HEALTH IN EUROPE”
Innovation in pricing

- More transparency on pricing mechanisms and what elements go into the final price tag

- Exploration of meaningful alternatives to external reference pricing. The value of ERP as a policy tool for regulating prices warrants further examination — LSE Report 2012

- Study – enhanced cross country cooperation in the area of pharmaceutical product pricing – led by Austria
  - External Price Referencing
  - Differential Pricing (sensitivity to purchasing power of countries)
  - Parallel Trade
  - Generic Medicine Substitution
  - Joint Procurement

- Complex, political nature of most policy options – but recognition that the current models do not work
Innovation in pricing

• EPF+ EURORDIS appeal to national competent authorities-
  ‘table for price negotiations’ – scale up of early dialogue pilots
  involving payers

• STAMP Council Working Party (Safe and Timely Access to
  Medicines for Patients)

• Recent bilateral and trilateral discussions between
  countries – moving towards a ‘Coalition of the Willing’

• Major focus on the upcoming NL EU Presidency

• Collaboration, collaboration, collaboration

Alone we can do so little, together we can do so much

Helen Keller
Patients as part of the solution

**Patients as experts**: to identify unmet service and therapeutic needs and point out inefficiencies and waste in systems and processes

**Patient involvement in co-designing healthcare**

**Smart spending where needed!**

**Strong evidence base**
Patient-centred care models: cost-effective, better health outcomes, and patient satisfaction

**Patient empowerment, self management and self care**

► **Health literacy**, the right skills and competencies for all players and an enabling environment

“A STRONG PATIENTS’ VOICE TO DRIVE BETTER HEALTH IN EUROPE”
Patient-centred innovation

Innovation that is important for patients

- Priority-setting for research
- Better alignment of innovation with real needs
- Valuable innovation

Need to involve patient throughout the innovation chain

Co-design research/patient-centred clinical trials, pricing and reimbursement decisions...

- Better quality research results
- Strengthened trust and acceptance

“A STRONG PATIENTS’ VOICE TO DRIVE BETTER HEALTH IN EUROPE”
Main challenges in involving patients in HTA are:

- Lack of an agreed methodology
- Lack of resources/capacity
- Not knowing the best stages to involve patients in HTA
- Patient evidence not being credible enough

Need for

- **a framework and methodology** for patient involvement in HTA
- education/training for patient representatives *and* the HTA community
- Key role of **EUnetHTA, HTAi Patients and Citizens Group, ISPOR, EUPATI**
EUPATI: an innovative training model

An unprecedented collaboration: Patient organisations, health professionals, regulators, health tech experts, health NGOs, pharmaceutical industry

Ethical framework and ethics panel
- Funded by IMI (PPP between EC and EFPIA)
- Launched Feb 2012
- Runs for 5 years
- Consortium of 29 members – led by EPF

Will develop and disseminate objective, credible, correct and up-to-date information on medicines R&D in 7 European languages

Will build competencies & capacity among patients & public to get involved

Will facilitate patient involvement in R&D to support academia, industry, authorities, HTA and ethics committees
• A sustainable Multi-stakeholder Approach from Research to Treatment - A structured Patient Engagement Knowledge Platform for Improved Outcomes

Understanding how, and at which juncture, patients can be engaged and facilitating this in a coherent, ethical and effective way.

• This must include collaboration with the regulatory environment, payers and the HTA community
Patients are engaged and committed to be part of the change process that is needed: preparedness and clarity.

Fair, transparent, participatory systems = societal acceptance of difficult choices.

We need to strive for the balance between innovation and solidarity.

And together, create a trusted, enabling environment to move forward.
THANK YOU FOR YOUR ATTENTION!

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www.eu-patient.eu
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“A STRONG PATIENTS’ VOICE TO DRIVE BETTER HEALTH IN EUROPE”
Optimizing Patient Involvement in Payer Health Care Decisions to Access New Therapies
ISPOR Patient Representatives Roundtable Initiatives

• Patient Representatives Roundtable Series
• Patient Centered Special Interest Group
• Rare disease Special Interest Group
• Oncology Special Interest Group
• ISPOR Patient Representatives Strategy
This presentation considers the perspective of the payer and the Health Technology Assessment (HTA) agency.

- **HTA Agency**: Is the technology good value for money?
- **Payer**: Can we afford the new technology? What is the budget impact?
Expenditure on medicines in Ireland
(Community Drugs Schemes 1991 - 2014)

Over 45 cost containment measures introduced since 2006
Who is going to pay for the DAA’s for Hepatitis C or the new PCSK9 inhibitors for hyperlipidaemia etc etc ????
What about Key Opinion Leaders

or should that be Paid Key Opinion Leaders ???
Who funds Patients Groups Association with the Pharmaceutical Industry?
The NCPE conducts the health technology assessment (HTA) of pharmaceutical products for the Health Service Executive.

Information on 242 medication reviews for over 215 indications (www.ncpe.ie)
The process begins with the price application by the manufacturer.

Rapid review to determine whether a full HTA is required.

The pharmaceutical company is invited to submit an economic dossier to demonstrate that the product is value for money.

Full HTA with a 90 day time frame.

NCPE submission to the HSE – CPU.

National Drugs Committee.
So where does patient involvement come in?
We believe the Company has failed to demonstrate the cost-effectiveness of ipilimumab for the treatment of advanced melanoma in adult patients who received prior therapy. We cannot recommend reimbursement at the submitted price.

Price: € 85,000/patient
Budget impact: € 4,800,000 - € 7,400,000 per annum
Δ median overall survival = 3.6 months
Basecase ICER: € 147,899/QALY or € 92,443/LYG

September 2011
Final ICER ~ € 116,000/QALY
“First Ever Drug to Treat ‘Celtic Gene’ In Cystic Fibrosis Sufferers”

Ipilimumab (Yervoy) Ivacaftor (Kalydeco)
Cost-effectiveness of Ivacaftor (Kalydeco) for the treatment of cystic fibrosis in patients age 6 years and older who have the G551D mutation

Basecase ICER: € 449,035/QALY or € 443,825/LYG
Price: € 234,804/patient
Budget impact: € 28,000,000 per annum

January 2013
The Cystic Fibrosis Association of Ireland played a very important role during the Ivacaftor HTA process, particularly during the time between the NCPE recommendation and the reimbursement decision......
We are happy to receive input for patients at all stages of the HTA process but it is probably more helpful if that input occurs earlier in the process.
Formal

• HTA and decision training with patient groups through IPPOSI.

• Set up formal links with IPPOSI (Irish Platform for Patient Organisations, Science and Industry).

• Patient groups are informed of drug entering the process at the rapid review stage.

• Information provided by the patient group can then be included in the full report provided to the HSE.
Informal

• Regularly interact with patient groups providing advice, clarity and interpretation of our recommendations.

• The NCPE also seek advice from patient groups around certain aspects of the HTA particularly in relation to assumptions or unknown information.
The Process

Patient groups are notified via IPPOSI

NCPE interact with patient groups on queries

NCPE include patient submissions in report to HSE
Patients views are provided to us in many different ways and from a range of sources including.....
It is evident that simply concentrating on HTA methodological expertise is necessary but not sufficient for the successful conduct of the wider HTA process.

There is little doubt that patients have to be involved.

But how, at what stage and in what way ???
Don’t forget the elephant !!!!
Thank you
Industry

Veronica Foote, BA,
Head of Patient Relations & External Communications,
Novartis Oncology Region Europe, Surrey, UK
As **patients are our focus**, it is important that they know what to expect from Novartis

**Access to our Innovative Medicines:** We collaborate with others to help address some of the world’s greatest health challenges, and we work to find solutions to get the right treatment to the right patient at the right time as quickly as possible.

**Patient Safety:** We are committed to making quality products that are safe and effective to meet patient needs and demands.

**Respecting the Patient Perspective:** We believe in the active participation of patients and active citizens to improve health care services and outcomes for patients.

**Data Transparency & Data Integrity for Innovative Medicines:** We recognize that patients need to trust products from Novartis and may want to access information on their own regarding these products. We support clinical research adhering to the principles of ethics, governance and transparency.

**Clinical Trial Input:** We recognize that patient knowledge and experience with their disease or condition is valuable in the design of clinical trial protocols and outcomes.

**What Patients Can Expect from Novartis**

**We are inspired by patients**

- This inspiration motivates us to revolutionize the research, development and manufacturing of innovative, high-quality medicines that help people live longer, with a better quality of life, giving more time to do the things that matter to them.

- To do our best for patients, we do not accept the status quo. We work to reach more patients worldwide so that patients and society can benefit as quickly as possible.

- The depth and strength of our pipeline enables us to change the practice of medicine, and to bring more breakthroughs with real benefits to patients and society.

- We partner with people and organizations around the world because by working together we can make a greater difference.

- We continually challenge ourselves to the highest standards of compliance, integrity and performance in all that we do to ensure a sustainable future of innovation for patients, society and Novartis.
Questions?

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