

The role of patient advocates: how can patients influence policy making?

Jean Mossman

London, 24 February 2015

- When and how can patients influence decisions about access to new treatments?
- What is patient evidence and how can patient organizations collect it?

“It is more important to know what sort of person has a disease than to know what sort of disease a person has”

Hippocrates



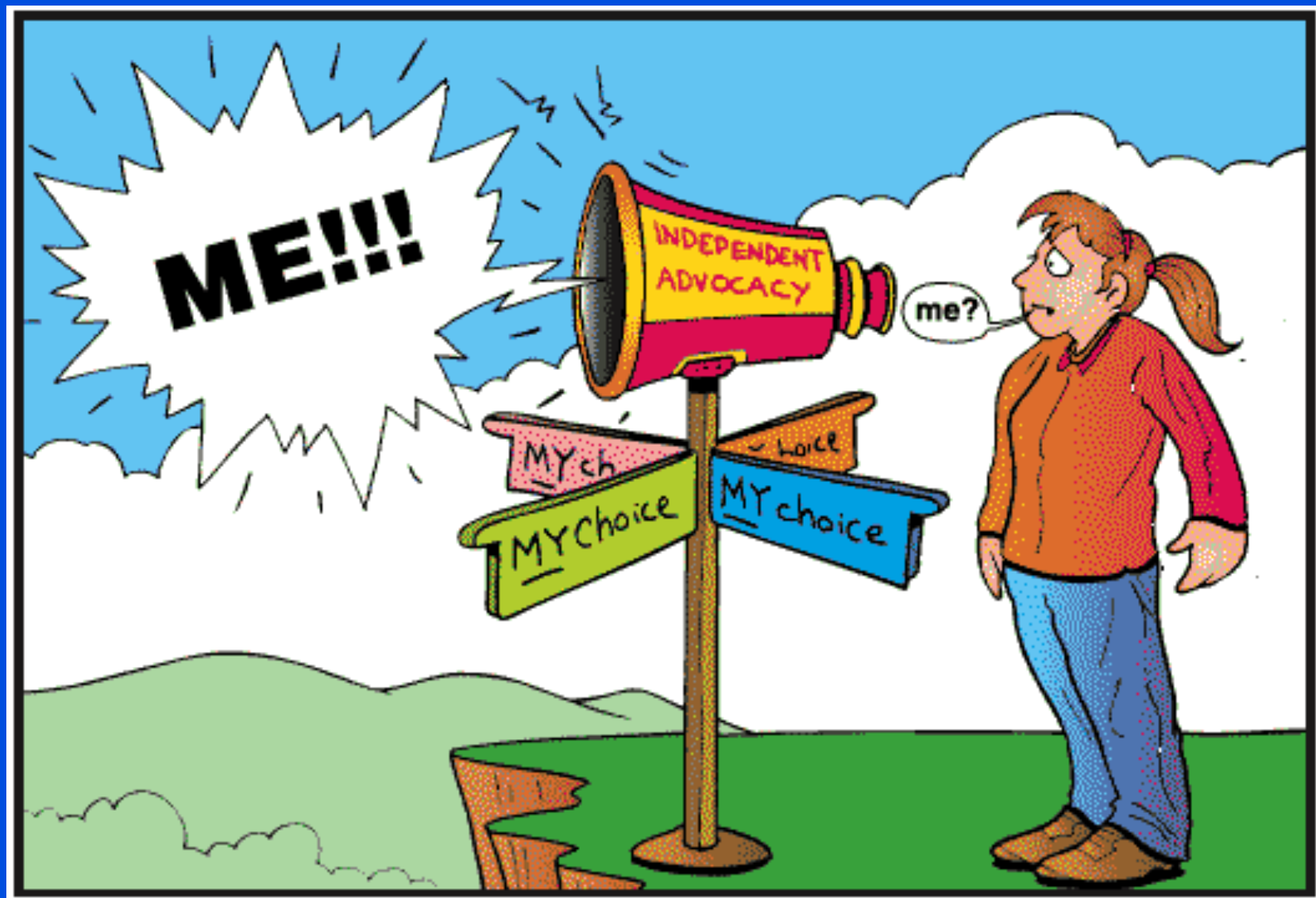
'It is also striking that no country has yet been successful in giving its citizens a truly central role in improving health and healthcare, preferring instead to rely almost exclusively on economic and professional levers.

Patients are reduced to being mere consumers in need of satisfying or passive patients in need of treatment or education.'

Nigel Crisp

Patient organisations need to re-frame the conversation

- Start with the person:
 - What matters to them
 - How their illness impacts their lives
 - What the technology offers
 - What more needs to be done



Why does it matter?





New Year's Eve 2004



Treatment costs for infusional vs oral 5FU

	Infusional £	Oral £
Drug cost	563	464
Administration	1500	113
Adverse events	22	131
One-off costs	12	7
Total	6255	2132

Patient costs for infusional vs oral 5Fu

Infusional

- 3 days in hospital each fortnight
- Adverse events
- Discomfort
- Shortage of veins
- Loss of dignity
- Boredom
- Frustration
- Little time for real life

Oral

- One outpatient visit every three weeks
- Adverse events
- Pills to take

Measure what matters:
defined by the patient and caregiver

**When and how can patients influence
decisions about access to new
treatments?**

Where patient evidence can be incorporated

- Regulatory process – getting the medicine approved
- HTA – demonstrating the medicine is value for money
- Local/regional drugs & therapeutics committees – getting the medicine on the formulary
- Supporting patients – taking medicine as intended so its full value is appreciated

MEDICINAL PRODUCTS FOR HUMAN USE

Go back to [Medicinal products for human use](#) > 50years

50 Years of EU Pharmaceutical Legislation



The EU has one of the safest and most advanced systems for monitoring the safety of medicines. Medicines authorised in the EU are of high quality and undergo a detailed assessment regarding their benefits and risk before being placed on the market.

2015 marks **50 years of pharmaceuticals legislation in the EU**: On 26 January 1965 the Council Directive 65/65 on the approximation of the law relating to medicinal products was adopted. This was the first piece of EU pharmaceutical legislation introducing some founding principles that are valid until today. Around those principles [a large body of legislation](#) has been developed over the last 50 years in order to guarantee high standards of quality and safety for medicinal products.

Additionally, in January 1995 the European Medicines Agency took up its responsibilities. Hence 2015 marks their [20th anniversary](#).



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

12 June 2014
EMA/24913/2005 - rev. 2
Stakeholders and Communication Division

Criteria to be fulfilled by patients' and consumers'
organisations involved in European Medicines Agency
(EMA) activities

Regulatory process – getting the medicine approved

- Work with clinicians and companies to ensure end points are valid (patient relevant outcomes)
- Work with EMA to help them understand what matters to patients

“The level of risk patients were prepared to take was quite illuminating...”

“It may be that patients’ acceptance of risk is higher than the regulator’s...”

Dr Ian Hudson, UK CHMP member



Providing some context

- Benefit and risk are inherent in any healthcare system

‘In the time it will take you to read this editorial eight patients will be injured, and one will die, from preventable medical errors’

EURO Millions

A black and white photograph of a smiling woman with long blonde hair, wearing a light-colored jacket, holding a glass of champagne. She is positioned in front of a large, stylized logo for 'EURO Millions'. The logo features the words 'EURO' and 'Millions' in a bold, sans-serif font, with 'EURO' in all caps and 'Millions' in title case. The text is surrounded by several large, five-pointed stars and curved motion lines, suggesting a sense of excitement and movement.

HTA: could it be EU?

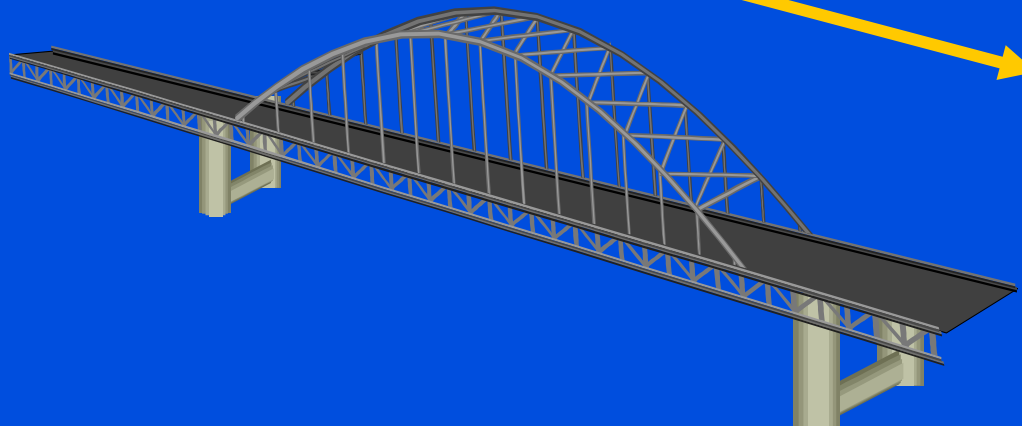
When in the HTA/reimbursement process should advocates be involved?

- Trials – design and as participant
- Scope – what is being assessed
- Experiential evidence
- Participating on an HTA committee
- Providing comments on draft reports
- Assessment reports – reflecting patient perspective
- Reviewing recommendations
- Ensuring that recommendations are available in plain language and reach the patient
- Evaluating the uptake of HTA recommendations

HTA – medicine is considered value for money

Information
(global/local)

Assessment



Decision

The advocates view: HTA is neither straightforward

$$1+1 = [(27/3)/3]-1$$

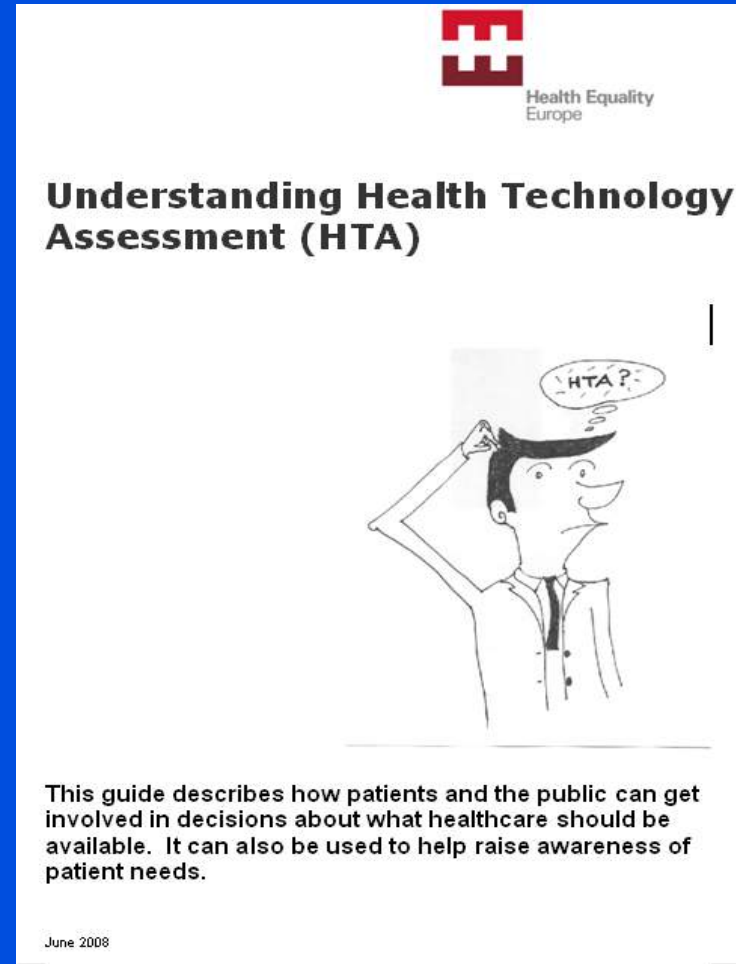
..... nor easy to navigate



Support for patient groups about HTA

Available in:

- English
- Mandarin
- Italian
- Polish
- Spanish
- Swedish
- Greek



<http://www.htai.org/index.php?id=744#c2840>

What do patients want from HTA?

- That the impact considered is broader than the health service
- That the impact of an illness and its treatment on the patient and family is understood
- That illness is given a priority to reflect its burden
- That a true reflection of a drug's value is assessed
- That the assessors accept that all evidence has been generated with a particular view in mind

That the impact considered is broader than the health service

- Burden on economy
 - Staying in the workplace
- Burden on social services
 - Staying independent
- Burden on families and friends
 - Staying active and mobile

That the impact of an illness and its treatment on the patient and family is understood



That a true reflection of a drug's value is assessed

Herceptin for the treatment of people with breast cancer

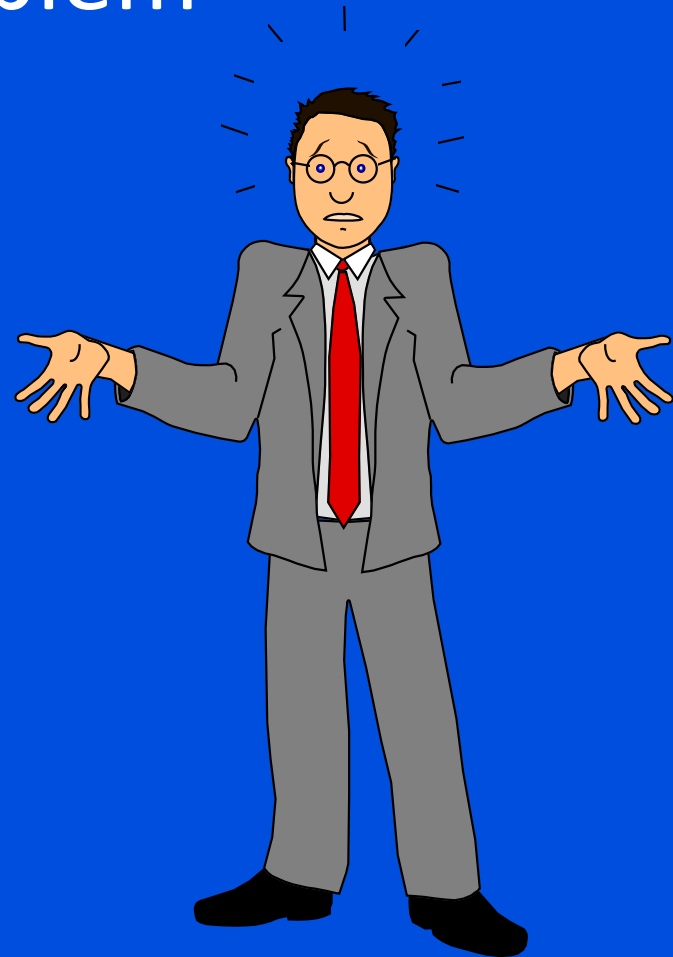
- ICER for patients with metastatic disease £37,500 [TA 34]
- ICER in the adjuvant setting between £2,387 (manufacturer) and £18,000 (evidence review group) [TA107]

Drugs & Therapeutics Committees (DTCs)

- DTCs operate at local (for one NHS Trust, say) or regionally (for a Comprehensive Cancer Network, for example)
- Some DTCs have members of the public/patients among the membership
 - Explore what is possible in your area
- The sort of experience-based information that patients and the public contribute to HTA processes can be used to make the case to include a medicine on a local/regional formulary

Health economists/payers view: 'Patients are the problem'

- Limited resources
- Unlimited “demands”
- **Choosing** between which
‘wants’ we can ‘afford’
given our resource
‘budget’



Health economists/payers view (2)

Regulators, payers & prescribers



**Evidence-based
Rational
Objective**

Patients & patient organisations



**Passionate
Emotional
Subjective**

Where has patient input had influence?

- Example 1: Age-related Macular Degeneration (AMD)
 - Vision in one or two eyes: Evidence suggested that loss of sight in one eye impacts little difference on quality of life (because what had been tested in clinical research was the loss of vision in both eyes).
 - Patient organisations, patients and carers clearly indicated that there were significant negative effects of loss of binocular vision on daily activities and quality of life.

Where has patient input had influence?

- Example 2: Psoriasis
 - Clinical research indicated that the amount of psoriasis was what most affected the quality of life.
 - Patients told us that the location of the flare-up (e.g. face or joints) was more significant.

value \neq price

The value of a medicine should not be measured by cost alone, but by the benefit it brings to individual patients and to society as a whole

What is patient evidence and how can patient organizations collect it?

Patients and caregivers provide ‘experiential’ evidence

- Explaining:
 - Being sick five times each day means you cannot manage to go to work, or that it happens so quickly that you cannot make it to the toilet and have to clean up after yourself
 - The fatigue caused by the illness - and not relieved by existing treatments - is so severe that it means you have to lie down all day and so cannot look after your children
 - The effect a treatment has on your daily life – ‘it makes it impossible to stand on my feet all day, which means I cannot work’

The disease and its impact

- The nature of the illness:
 - Acute? Chronic? Life threatening?
 - Symptoms that are difficult to live with
- The limitations it imposes on:
 - Daily life
 - Ability to work
- The impact on a person's mental wellbeing
- Whether the illness prevents people from fulfilling their chosen role in life

The benefits and risks of the technology

- What benefits does it bring?
 - How do they impact on a patient's daily life?
- What unwanted effects does the technology cause?
 - How tolerable are they?
 - How do they impact on the patient's daily life?
- How easily does the technology fit into patients' daily life?
 - Do they have to go to hospital to receive it or take time from work?
 - Does the technology prevent them from doing anything routine?
 - Is anyone else affected, such as a family member accompanying the patient?
- What would happen to patients if there was limited access to the technology?

The caregivers' experience

How caregivers are affected by the person's illness:

- Poor health because all their energy goes into caring for the patient
- Taking time off work to care for the person
- Paying for a carer for the patient or for childcare because the patient cannot look after the child/children
- Financial hardship because they reduce working hours
- Distress, watching the patient suffer

How patient groups can gather patient evidence

- Existing data may already be available
 - Survey/questionnaires
 - Review of helpline questions
 - Social media
- Qualitative Evidence
 - Patient stories
 - Social networking
 - Interviews
 - Focus groups
- Quantitative evidence
 - Robust surveys

Partnering with academic departments:

Developing evidence

Table 2 Frequent subjects of enquiry from prostate, male and female colorectal cancer patients

The most frequent subjects of enquiry	Prostate cancer patients		Male colorectal cancer patients		Female colorectal cancer patients		Prostate patients versus male colorectal Significance ^a	Male colorectal versus female colorectal Significance ^a
	n = 411	(%)	n = 162	(%)	n = 217	(%)		
Site specific information ¹	140	(34.1)	43	(26.5)	64	(29.5)		
Emotional support / narratives ²	154	(37.5)	66	(40.7)	104	(47.9)		
Emotional support and reassurance	137	(33.3)	57	(35.2)	96	(44.2)		
Narratives and catharsis	17	(4.1)	9	(5.6)	8	(3.7)		
Publications / booklist ³	133	(32.4)	65	(40.1)	76	(35.0)		
Specific therapy enquiries group ⁴	196	(47.7)	80	(49.4)	104	(47.9)		
Chemotherapy	11	(2.7)	55	(34.0)	72	(33.2)	<i>P</i> < 0.001	
Complementary or alternative therapies	5	(1.2)	4	(2.5)	16	(7.4)		<i>P</i> < 0.05
Hormonal therapy	125	(30.4)	0		0		<i>P</i> < 0.001	
Radiotherapy	123	(29.9)	13	(8.0)	16	(7.4)	<i>P</i> < 0.001	
Surgery	69	(16.8)	24	(14.8)	34	(15.7)		
Treatment enquiries								
General treatment enquiry	39	(9.5)	10	(6.2)	5	(2.3)		
Treatment side effects	89	(21.7)	34	(21.0)	60	(27.6)		
Research or clinical trials	15	(3.6)	14	(8.6)	11	(5.1)	<i>P</i> < 0.05	
Treatment centres or doctors	19	(4.6)	7	(4.3)	11	(5.1)		
Other medical enquiries								
Clarification of information	65	(15.8)	21	(13.0)	34	(15.7)		
Diet and nutrition	11	(2.7)	15	(9.3)	35	(16.1)	<i>P</i> < 0.001	
Prognosis	37	(9.0)	19	(11.7)	10	(4.6)		<i>P</i> < 0.05
Recurrence	30	(7.3)	11	(6.8)	16	(7.4)		
Symptom control	31	(7.5)	10	(6.2)	17	(7.8)		
Other support								
Health professional communications	47	(11.4)	16	(9.9)	23	(10.6)		
Sexuality and sexual problems	21	(5.1)	3	(1.9)	1	(0.5)		

Reflecting what matters to patients

Eur J Cancer Care (Engl). 2012 Sep;21(5):565-80. doi: 10.1111/j.1365-2354.2012.01370.x. Epub 2012 Jun 4.

Quality of life assessments in advanced breast cancer: should there be more consistency?

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Abstract

Quality of life (QOL) measures have assumed increasing importance in assessing the impact of therapeutic drugs and interventions on patients and in making judgements about their cost-effectiveness. Important treatment decisions and crucial funding strategies involve QOL data and, for patients with a disease such as advanced breast cancer that impinges on their life expectancy, QOL can become a hugely important consideration. Yet, despite this, there is a lack of consensus on what defines an appropriate QOL measure, and inconsistency in the instruments that are chosen to measure it. The National Institute for Health and Clinical Excellence (NICE) is seen as a model for appraising the value of new treatments and NICE approval is required for treatments to be funded in the UK. In order to compare different disease conditions they use a generic measure, preferring the EQ-5D. We have performed a literature search of clinical trials in advanced breast cancer to establish which QOL measures have been used. Our findings show marked heterogeneity in terms of which QOL tools are used. It is suggested that there should be more consensus on which QOL instruments are used, not only between researchers, but between them and the bodies that approve funding.

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PMID: 22672416 [PubMed - indexed for MEDLINE]

Publication Types, MeSH Terms



Responsiveness of the EQ-5D to clinical change

Journal:	<i>International Journal of Technology Assessment in Health Care</i>
Manuscript ID:	IJTAHC-12-139.R1
Manuscript Type:	Methods
Date Submitted by the Author:	n/a
Complete List of Authors:	Tordrup, David; London School of Economics, LSE Health Mossman, Jean; European Federation of Neurological Associations, Kanavos, Panos; London School of Economics, LSE Health
Keywords:	QALY, responsiveness, health technology assessment, patient participation, EQ-5D
	Objectives In many economic evaluations and reimbursement decisions, Quality Adjusted Life Years (QALY's) are used as a measure of benefit to assess effectiveness of novel therapies, often based on the EQ-5D 3-level

And the future?

Personalised medicine will need a different approach



*“Although we can’t go back and
make a new beginning, we can start
now and make a new ending”*

Chico Xavier

Conclusion

- The patient and caregiver perspective is uniquely relevant to assessing the value of a medicine
- Patient organisations have the reach to provide experiential evidence of the disease, available treatment options and the role of self management
- Sharing knowledge and experience avoids duplication of effort