

Πανελλήνιο Συνέδριο Ασθενών

The role of patient organisations in a challenging environment

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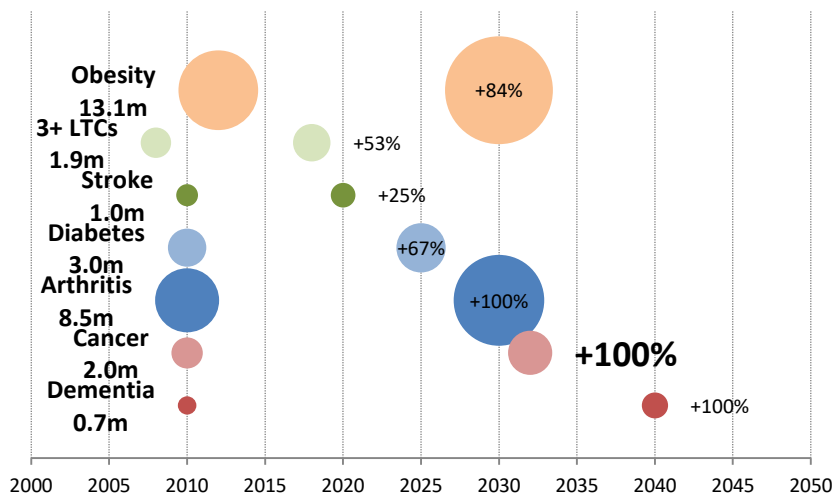


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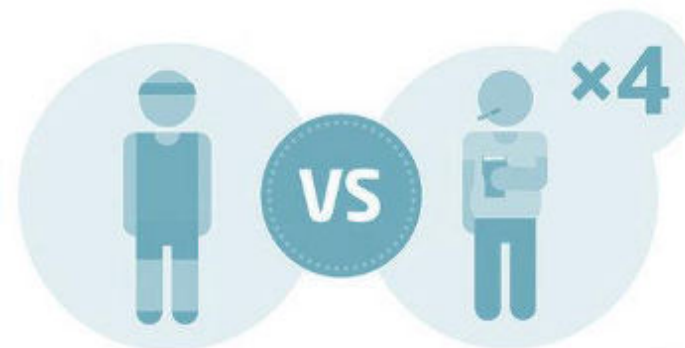
- Challenges to health system sustainability
- Why involve patients/patient organisations in HTA
- When and how can patients influence decisions about access to new treatments?
- What is patient evidence and how can patient organizations collect it?

Challenges for health systems: Meeting future needs will be very challenging

Increasing prevalence of chronic disease...



...and often multiple morbidity

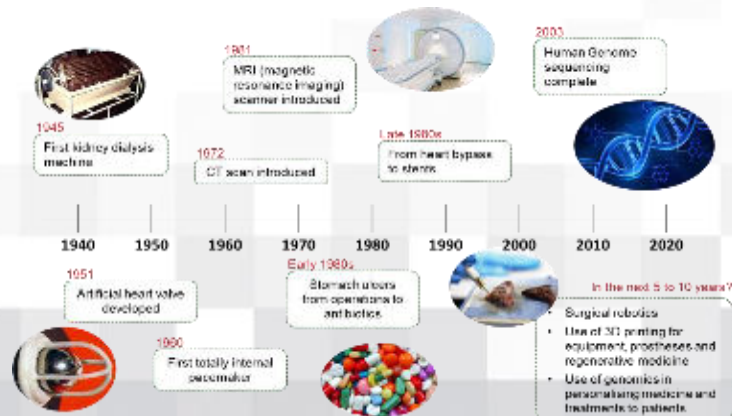


Someone in mid-life who smokes, drinks too much, exercises too little and eats poorly is four times more likely to die over the next 10 years

Diverse and changing expectations...

Early life	25- 44	45- 64	65- 84	Late life
A world of choice	Nowism and consumerist expectations	Multiple roles and demands	Degree of social isolation	Living situation and access to resources
Consumerist expectations	Always being connected	Diminishing social support	Wide variation in working patterns	Frailty
Genomic generation	Work – life convergence	Lifestyle choices and risky behaviours	Prevalence of health conditions	Differing desire and ability to engage in decisions

...and breath-taking technological change

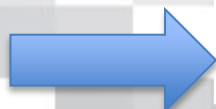


Challenge: High-need / high-cost patients



In developed countries, as much as 2/3 of health expenditure is spent on 5 - 10% of patients, a high-need and high-cost group characterised by high prevalence of chronic disease, multi-morbidity and complicating factors.

	Population (size; cost/year)	Total Cost per Year	Delivery System Needs
1	Healthy (~160m people)	\$130 billion	Prevention, primary care, public health
2	Maternal and infant health (~10m people)	\$60 billion	Prevention, primary care, public health
3	Acutely ill but mostly curable (~12m people)	\$300 billion	Acute care services, primary care, specialty care, medicines
4	Chronic conditions w/ generally "normal" function (~110m people)	\$800 billion	Self-management; Primary and specialty care in doctors offices; ER/acute care services, medicines
5	Significant disability but stable (~7m people)	\$290 billion	Home-based services (primary care, specialty); LTSS; environmental adaptation; caregiver support, rehab
6	"Dying" with short decline (~1m people)	\$50 billion	Home-based services and personal care services; Palliative care/hospice; caregiver training and support
7	Multiple chronic conditions w/ serious exacerbations, "advanced illness" (~2m people)	\$100 billion	Self-care support; at home services; 24/7 on-call access to medical guidance; caregiver support, medicines
8	Long course of decline from dementia and/or frailty (~6m people)	\$270 billion	Home-based services; LTSS; palliative care; DME; caregiver training and support;



Patients: Not just advocates who believe their voice should be heard



Four arguments in favour of engaging the public in health care policy were identified by the University of Toronto Priority Setting in Health Care Research Group:

- The public is the most important stakeholder in the health care system
- Engaging the public is in keeping with the principles of a democracy
- Members of the public can provide insights on the values and priorities of their communities
- Engaging the public can lead to improved public trust and confidence in the health care system

Bruni et al., 2008

Patients: Not just advocates who believe their voice should be heard (cont'd)



- The WHO has stated that patient involvement in their health care is a social, economic and technical necessity
- Patients are responsible for many decisions about their health:
 - They decide *when* to seek medical advice
 - They decide *whether* to accept that advice
 - They decide *whether* to take the prescribed medicines
 - They decide *whether* they will take complementary medicines and adjust their lifestyle (if appropriate)

Where patients & patient groups can contribute



- Identifying healthcare priorities
- Identifying research priorities
- Clinical trial design, implementation and interpretation
- Contributing to designing services & setting standards
- Auditing whether standards are being met
- Regulatory and reimbursement decisions
- Training healthcare professionals
- Improving patient safety
- Providing information to patients and the public
- Patient engagement is not just a 'nice to have'

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



- **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 realised

Regulatory process – how the patient voice impacts decisions

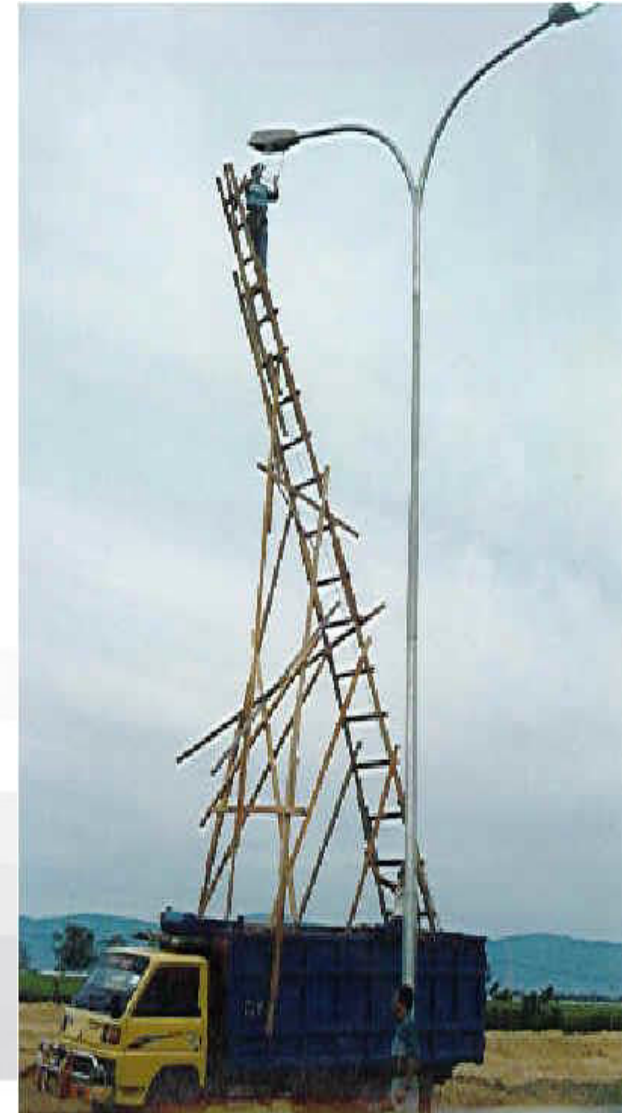


- Patient groups can work with clinicians and companies to ensure end points are valid (patient relevant outcomes)
- Work with EMA to help them understand what matters to patients

“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



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



- Clinical 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

What do patients want from HTA and value assessment processes?



- That the impact considered is broader than the health service
 - Burden on the economy and the benefit of staying in the workplace
 - Burden on social services and the benefit of staying independent
 - Burden on families and friends and the benefit of staying active and mobile
- 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

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 NICE that the location of the flare-up (e.g. face or joints) was more significant

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



1. Patients and carers provide ‘experiential’ evidence



- Saying you were sick five times each day is less meaningful than explaining what this 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
- Recording that the fatigue caused by existing treatments is so severe that it means you have to lie down all day and so cannot look after your children
- Explaining that a pill is more acceptable than an intravenous treatment not just because it means less trips to hospital but because it allows you to continue living a more normal life
- Describing the effect a treatment has on your daily life – such as, ‘it makes it impossible to stand on my feet all day, which means I cannot work’

2. 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

For a universal template for patient group submissions see: <http://www.htai.org/interest-groups/patient-and-citizen-involvement/resources/for-patients-and-patient-groups.html>

3. 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?

4. 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

Conclusion



- Many challenges for health care systems impacting sustainability
- Within this difficult framework, the patient and caregiver perspective is uniquely relevant to assessing the value of a medicine
 - Can *'distinguish your medicine from the crowd'*
- Patient organisations have the reach to provide experiential evidence of the disease, available treatment options and the role of self management
- It has been shown that patient evidence can shape decisions on reimbursement in a way that safeguards health policy objectives such as equity and efficiency

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

Hippocrates

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