



Healthcare Decisions

Working with decision-makers

Sjogren's Syndrome

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Health Technology Assessment International (HTAi)

Patient Focused Medicines Development (PFMD)

What perspective am I bringing?



HTA Bodies / Regulators



Patient groups



Industry



Researchers



<https://htai.org/interest-groups/pcig/>

Past chair and current steering committee member

Working internationally with HTA bodies and patient groups

Research and building resources



<https://imi-paradigm.eu>

One of the lead consortium members

Focus on patient involvement in Early Dialogues with HTA

Built tools and resources to aid patient involvement processes



PATIENT FOCUSED MEDICINES DEVELOPMENT

<https://patientfocusedmedicine.org/>

Current board member of the PFMD consortium

Building practical tools to aid patient involvement

Focus on aligning needs of regulators and HTA bodies

Patient advocate in the field of HIV from 1995

Member of the patient access function at an international pharmaceutical company in the early 2010s

The flow of today's webinar



Policy
Decision
Making



Regulatory
decisions



Access and
reimbursement
decision making



Summary and
key takeaways

Policy Decision-making

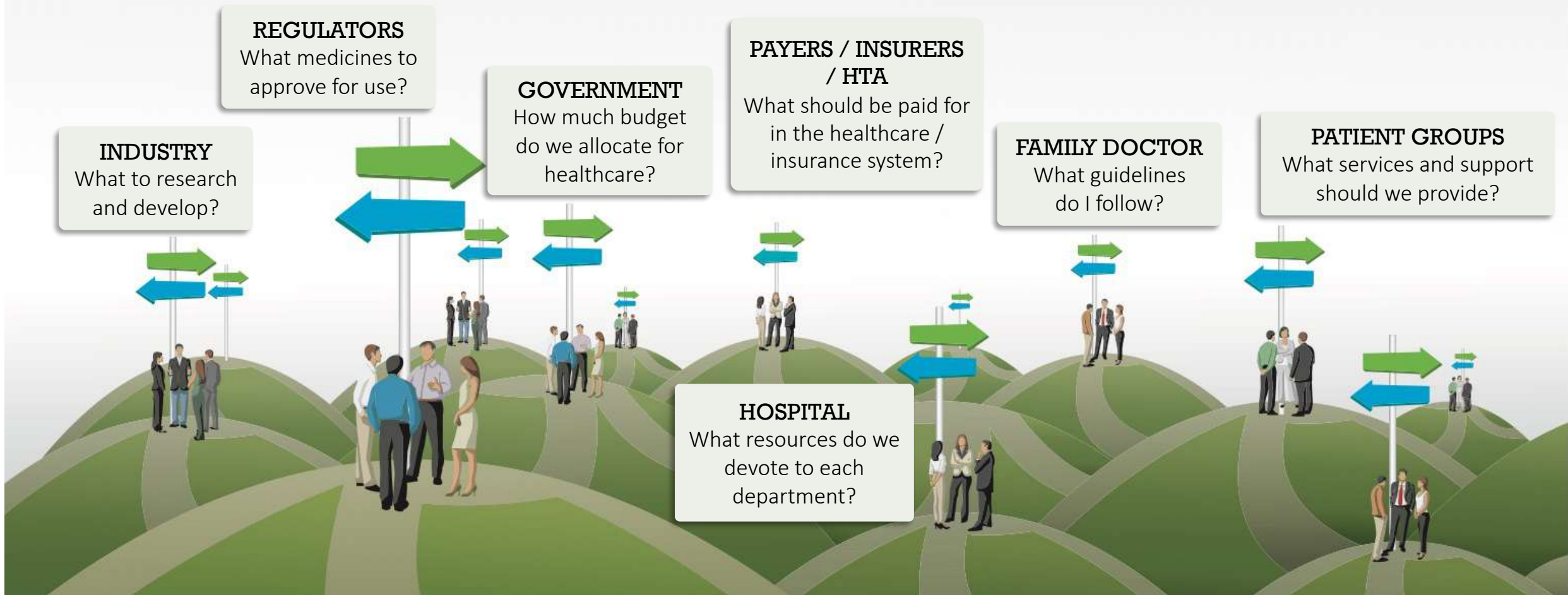


To understand how policy decisions affect us
and easy ways of getting involved



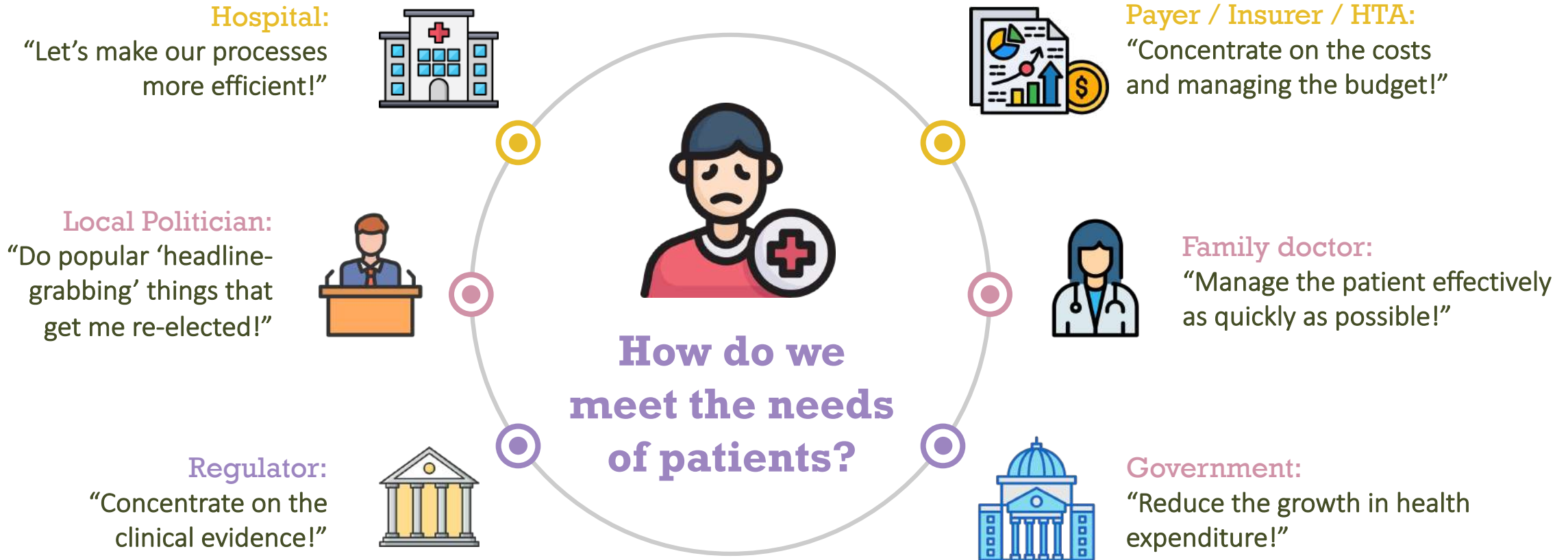
Our healthcare is shaped by decisions made by others

All have the same ultimate goal: To improve the lives and outcomes for patients



The challenge is that not everyone agrees on what is best

Illustrative examples:



Each stakeholder has a valid point of view on where they are looking for value and outcomes from healthcare

Value to the healthcare system



Efficient use of resources

Reducing complexity

Minimising procedures

Maintain/increase quality and outcomes

Value to the physician



Better clinical outcomes

More productive consultations

Satisfied patients

Payment incentives?

Value to society



Productive members of community

Able to support others (e.g. children, parents)

Adds skills and capabilities to society

Develops and evolves with the society

Value to the patient



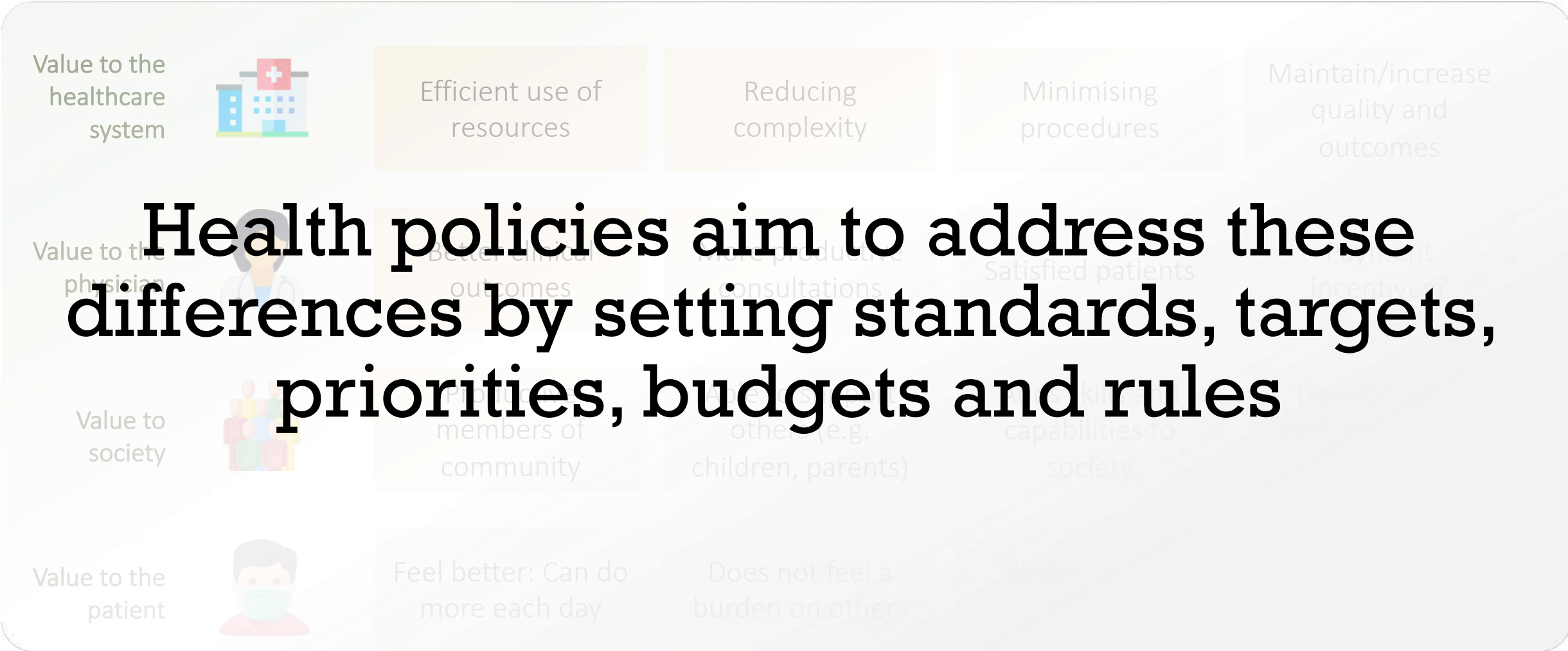
Feel better: Can do more each day

Does not feel a burden on others

Better mental health

Condition and treatment do not 'get in the way'

Each stakeholder has a valid point of view on where they are looking for value and outcomes from healthcare



Our role as patient advocates?

Yes, we can campaign, protest, and draw attention to the needs of the patient community

But that is often not how change happens...



Our role as patient advocates?

Or we can work in partnership with decision-makers to collaborate with them on the solutions

This is usually how lasting change happens





It is this
collaboration
that we will go
through today

Lots of levels of policy and decision-making

From the global, to the regional to the national, to the city, to the district...



Organisations like the **World Health Organisation** set global health priorities and consider the needs of developed, middle income and resource poor healthcare settings

Tends to focus on the big, common drivers of health

Lots of levels of policy and decision-making

From the global, to the regional to the national, to the city, to the district...



Organisations like the **European Union** and **regional chapters of the WHO** set regional health priorities

Tend to focus on processes that remove disparities between countries or that facilitate cross-border healthcare within the region

Lots of levels of policy and decision-making

From the global, to the regional to the national, to the city, to the district...



Ministries of health and finance set national priorities, budgets and plans for particular disease areas

National medical associations set guidelines and protocols

National payers determine what is covered by public healthcare or insurance schemes

Lots of levels of policy and decision-making

From the global, to the regional to the national, to the city, to the district...



Individual states or regions within a country may control many aspects of healthcare

Often set resources such as how many hospitals are in the area, how is the ambulance service resourced, and usually have control over social care and how this is resourced

Lots of levels of policy and decision-making

From the global, to the regional to the national, to the city, to the district...



Individual hospitals and clinics in the city will set their priorities, processes, patient pathways and staffing levels

Often it is at this level that real change can happen quickly! Processes can be easier to change and it is much easier to arrange joint collaborations with local institutions such as clinics to pilot new pathways that benefit patients

Lots of levels of policy and decision-making

From the global, to the regional to the national, to the city, to the district...

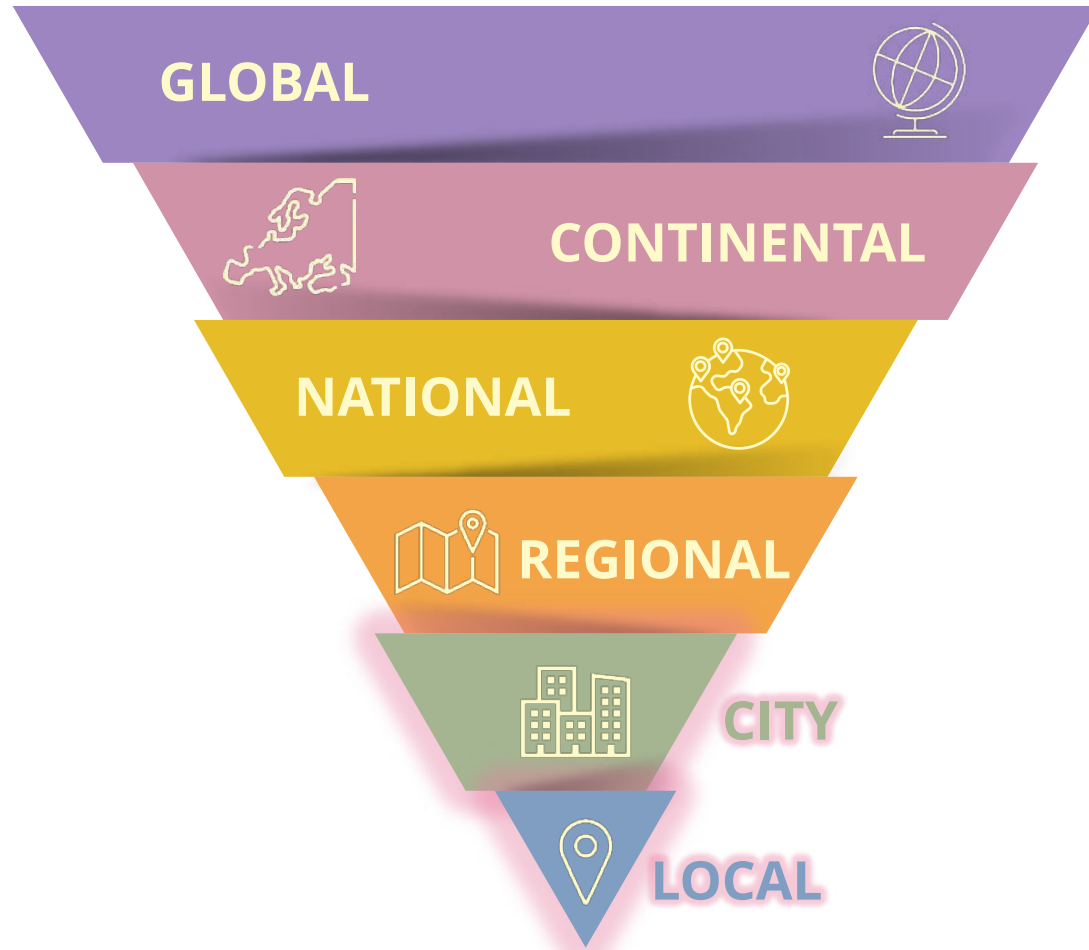


A single doctor's practice is the first line of the patient experience, and it can have the biggest impact on diagnosis and referral to specialists

Often, it is here that change can be most effective. Relationships already exist and there are simple steps that can be taken to pilot new ways of working with patients

TOP TIPS – You don't have to solve this whole pyramid!

Start from what you know, who you know, and look out for opportunities



Impacting health policy is often a bottom-up approach that starts locally

- Start with an institution or clinic you already know well
- Talk to the people you are already in contact with
- Ask open questions such as “What can be improved?”
- Listen to them to understand the decisions that they are in control of and those that are above them
- Be clear with them on the challenges you are hearing from your patient members
- Work with them on the decisions that they control
- Offer to input into decision that are above them (e.g. can you provide them with patient insights to bring to the meetings that they attend?)

TOP TIPS

Be opportunistic – look out for events, issues and topics that align with your aims



News stories, local elections, local events

- If there is a news story that illustrates challenges with healthcare locally, can you follow up with any decision-makers quoted in the article to arrange a meeting with them to discuss the issues?
- National and local elections provide a fertile ground for setting up meetings with prospective candidates to discuss your priorities
- Is there an event such as the opening of a new clinic or hospital department that you can attend. These are an ideal setting to make new contacts
- *TOP TIP: Don't jump in with your demands in social situations, instead signal that you have some interesting patient insights to share and ask to arrange a meeting to discuss*
- *Take a collaborative tone "working together we can achieve so much!"*

Policy Making Advocacy: DON'T THINK LIKE THIS



THINK LIKE THIS!



Pause for questions



Regulatory decisions



To understand how medical interventions such as drugs are approved and how patient advocates can input



The two main steps to therapies becoming available

REGULATORY ASSESSMENT

HTA / PAYER / INSURANCE ASSESSMENT



Focus is on the new therapy
(often not compared to others)

“Does the evidence show that the benefits outweigh any potential harms?”

Focus is on the **difference between the new therapy and current approaches**
(always compared to others)

“Does the evidence show that therapy brings additional benefits compared to current treatments or methods, and are those benefits big enough and important enough to justify the investment?”

Both regulators and payers/HTAs increasingly seek input from the patient community

They want to improve the quality of their decision-making by understanding the needs, experiences and preferences of patients



Example Regulatory: EMA

ATMP = Advanced Therapy Medicinal Products
 COMP = Committee for Orphan Medicinal Products
 CAT = Committee for Advanced Technologies
 CHMP = Committee for Human Medicinal Products

SAWP = Scientific Advice Working Party
 PDCO = Paediatric Committee
 PRAC = Pharmacovigilance Risk Assessment Committee
 SAG = Scientific Advisory Groups



Documents for the public

Public summaries of opinion



Product information



Safety communications



Pre-submission

Evaluation

Post-authorisation

Regulatory Procedure

Orphan Designation / ATMP classification

Scientific advice and protocol assistance

Paediatric Investigation Plan

Marketing Authorisation Application Evaluation

Post Marketing Authorisation

Committees and working parties

COMP
CAT



CHMP
SAWP



PDCO



CHMP, CAT,
PRAC, COMP, SAG

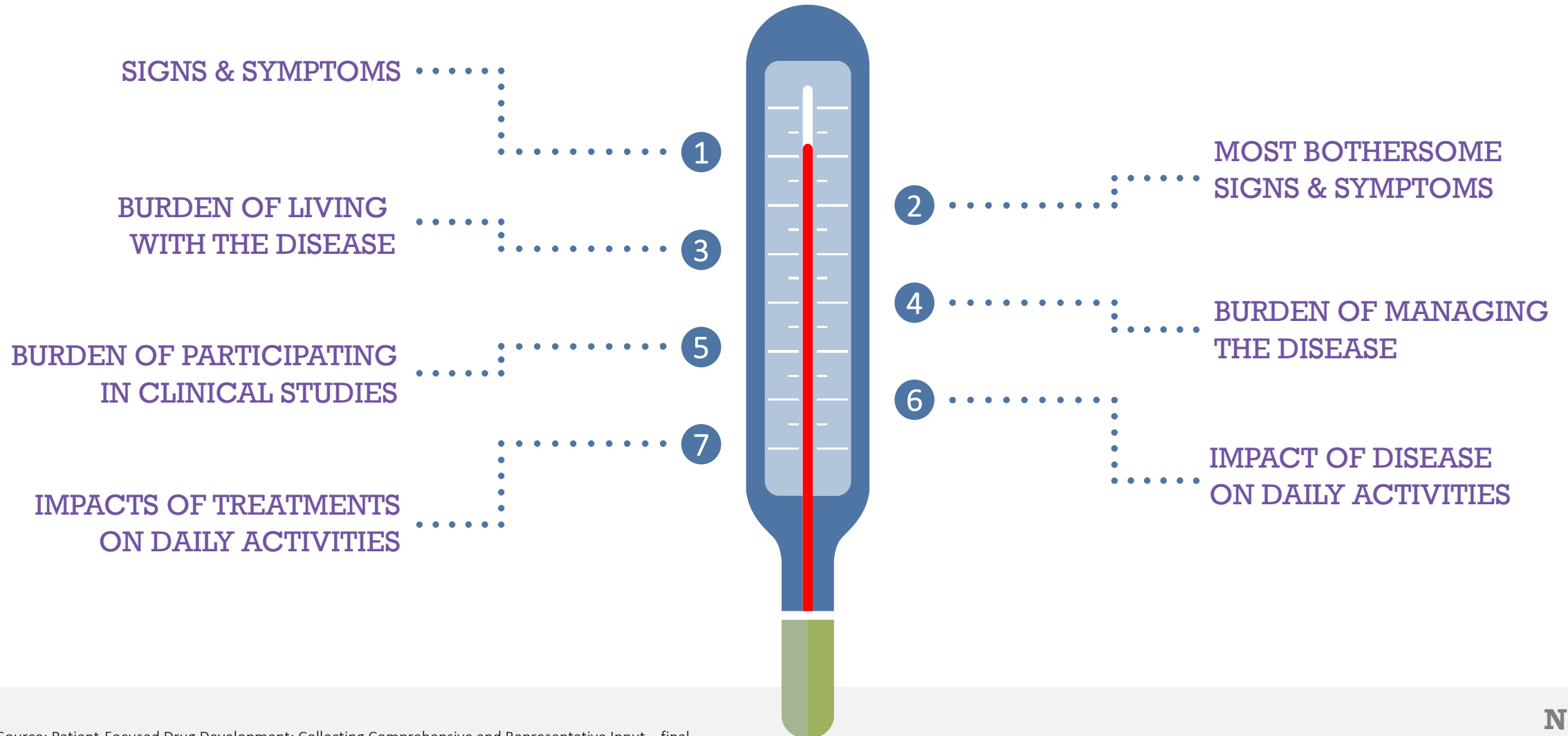


CHMP,
PRAC, SAG



Example FDA Patient Focused Drug Development Program

Looking to understand the impact of the disease and its treatment on patients



TOP TIPS FOR INTERACTING WITH REGULATORS

Let them know you exist!



Usually based on being invited, so...

Outside of any assessment of a medicine in your disease area send an email to the regulator (search website for patient engagement department or person)

- *Outline your organization, its remit, geographical reach*
- *Emphasize your connection to patients with the disease*
- *Your willingness to be part of any future relevant discussions*
- *Your ability to come with evidence from the patient community*

If an assessment is upcoming, send a short reminder that you are there to help and request to be invited. Note that only one or two patient representatives can attend some kinds of meetings and so do not take rejection personally.

TOP TIPS FOR INTERACTING WITH REGULATORS

Come armed with the facts, figures and insights from your patient community



Often there is only a few minutes to present your point of view...

- Focus on the patient perspective, not the clinical evidence (other stakeholders bring clinical evidence to regulators). Focus on:
 - *Burden of the disease and how this is not being addressed currently*
 - *Impact of disease on daily life (be specific)*
 - *Symptoms that are most bothersome*
 - *Challenges with current disease treatment and management*
 - *Key areas that are not met by current available medicines*

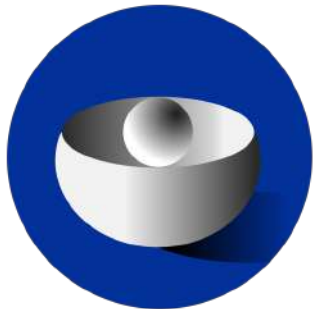
Back up your statements with evidence (e.g. from surveys of patients or interviews), and practice in front of a mirror or with a friend to get your points sharp and quickly across in the time allotted

CASE STUDY

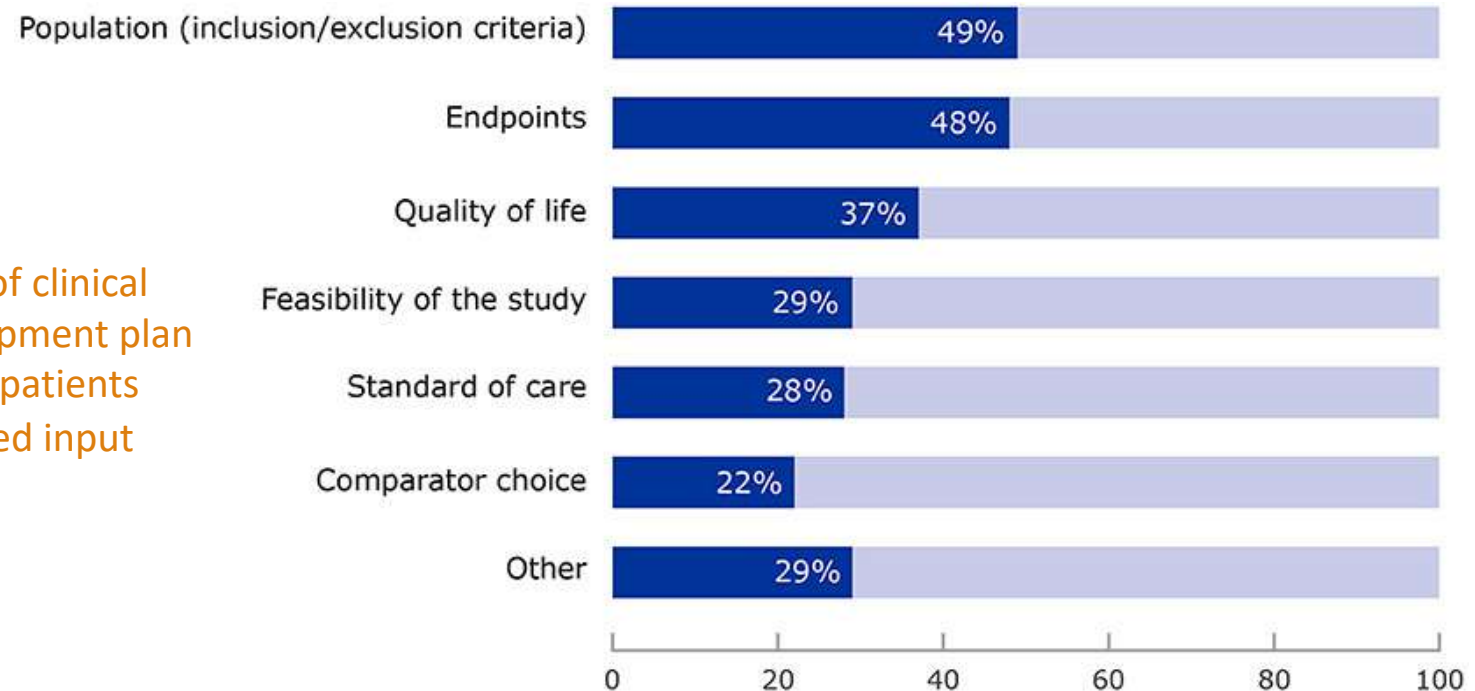
The Added Value of Patient Engagement in Early Dialogue at EMA: Scientific Advice



The EMA looked at the impact of having patients involved in scientific advice meetings (between the EMA and the medicine developer) between 2017 and 2020



Areas of clinical development plan where patients provided input



Percentage of EMA coordinators who reported that patients provided input into this area

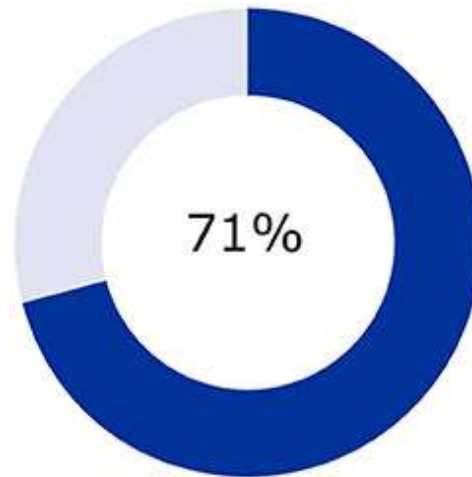
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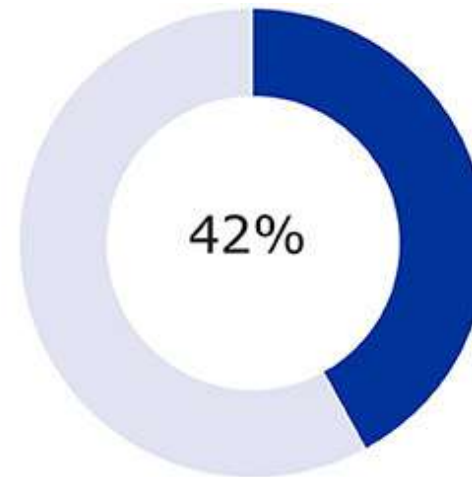


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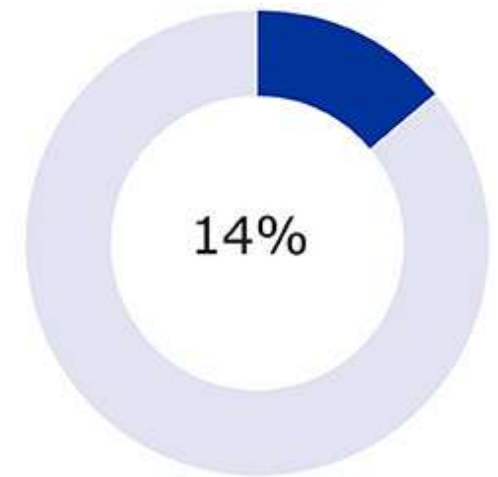
Additional inputs reported by the EMA project coordinators



Bringing the real-life experience



Offering a different perspective



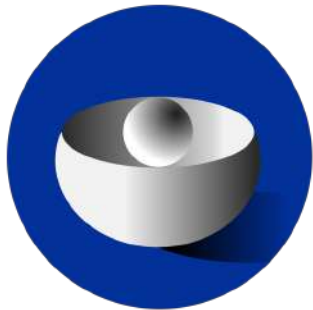
Raising issues that had not previously been considered

CASE STUDY

The Added Value of Patient Engagement in Early Dialogue at EMA: Scientific Advice



The EMA looked at the impact of having patients involved in scientific advice meetings (between the EMA and the medicine developer)



“While regulators and other experts can provide guidance on many aspects of the complexities of medicine development, **the day to day experience of living with a condition and its treatment can only be addressed by someone with first-hand experience.** The data presented here offers unique insights as it is the first time that such impact data is being presented by a regulatory body. We have highlighted how **patients fill an important gap by providing real-life experience of the conditions and their treatments, in addition to providing input into the clinical aspects of the development plans.**”

Pause for questions



Reimbursement decisions



To understand how medicines are assessed for reimbursement and different ways that patient advocates can be involved



Working with payers and Health Technology Assessment bodies

Let's start with the basics of understanding the role of reimbursement authorities and Health technology Assessment



Health Technology Assessment - HTA



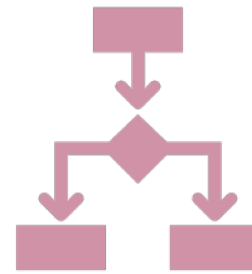
Different in each country

Considers evidence about a new technology (e.g. medicine) compared with the standard of care used in the national system

The purpose of HTA is to inform:

- **If a health technology should be used**
- **How best to use it**
- **Which patients will benefit most from it**

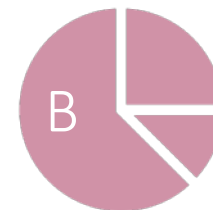
Why do we need a process that assesses the value of medicines? *It has become impossible to pay for everything...*



So how can systems decide who gets what?



By giving those who shout loudest or have the best connections what they want?



HTA

By reviewing the evidence and making a judgement based on this evidence?

Policy making versus HTA

“ Health technology assessment is a **multidisciplinary** process that uses explicit methods to determine the value of a health technology at different points in its lifecycle. The purpose is to **inform decision-making** in order to promote an equitable, efficient, and high-quality health system.¹ ”

Myths

HTA bodies decide which medicines and similar health technologies are available

Determining access to medicines becomes a purely evidence-based technical process

It is politicians who ultimately decide



HTA provides **evidence-based input** to the policy-making processes concerning the use of technology in health services and thereby **seeks to promote** evidence informed policy-making. ... HTA offers a **bridge** between the research and policy communities



Policy makers decide:



The overall resources available to healthcare



The priorities for healthcare spending



The resources (in staff and budget) available to a HTA body



The focus of the assessment processes



Illustrative example

In the early 2010s, most cancer medicines reviewed by NICE under the standard HTA processes failed to demonstrate enough value to recommend reimbursement within the UK's NHS

The political answer:

A separate '**Cancer Drug Fund**' that would be used to pay for cancer therapies not recommended by NICE

"A very modern triumph of political expediency over rationality" *The Lancet*¹

But this money soon ran out



Tories unveil £400m cash boost to extend controversial cancer drugs fund

Controversial drugs scheme to be extended despite criticism from doctors

Oliver Wright | Saturday 28 September 2013 11:57 | comments



1: The Lancet: Editorial: Aug 7 2010: New £50 million cancer fund already intellectually bankrupt. [https://doi.org/10.1016/S0140-6736\(10\)61202-0](https://doi.org/10.1016/S0140-6736(10)61202-0)

2: The Independent: 28 September 2013 <https://www.independent.co.uk/news/uk/politics/tories-unveil-ps400m-cash-boost-to-extend-controversial-cancer-drugs-fund-8845178.html>

So a key opportunity for patient groups to change or adapt systems better care:

Understand the landscape and the system and have a view on how it can be improved

Identify partners across the patient and physician communities who can collaborate together on making specific requests for change

Reach out to the key policy makers (e.g. within health and finance ministries) that can make change happen to start the dialogue

Continue the dialogue and expand the partners to create momentum behind the proposed changes

Working with payers and Health Technology Assessment bodies

Why and how does patient involvement happen in these reimbursement decisions?



The approach before patients were engaged



Considering the evidence and these viewpoints do we recommend to reimburse, and if so for which patients, and how?

BUT: The evidence never tells the full story



CLINICAL STUDIES PROVIDE ONLY PART OF THE STORY

- Controlled patient population – how will patients in the real world benefit?
- Limited duration of trials – will the benefits be long-lasting?



HOW WILL THE QUALITY OF PATIENT'S LIVES BE IMPROVED?

- Standard quality of life measures often miss important patient impacts
- Missing data is often a big problem in analysing these measures



APPROPRIATENESS OF EVIDENCE AGAINST COMPARABLE TREATMENTS

- Clinical trial evidence often missing comparative data to best alternatives
- Lack of evidence about the relative performance against recent options

UNCERTAINTIES

So, the traditional approach breaks down...



What is the meaningful benefit to patients?

What outcomes are important?

Are there particular needs for sub-populations?

What do these changes in disease scores really mean for patients?

How does any change in clinical practice impact patients?

Who will benefit most from this technology?

What is the 'real' value of this treatment?

Patients are the missing link in these deliberations

They can:

- Explain the impact of often poorly understood conditions on their daily lives
- Translate the endpoints and PRO measures into **specific** meaningful impacts they experience every day
- Highlight the positive impact that changes in these measures mean to their lives
- Clearly articulate, **with specifics**, how their needs are not being met with current treatments and standards of care



Today we see patients involved in many ways



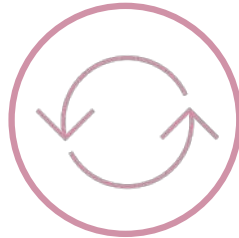
BUT: Important to remember the central purpose of HTA



**To protect the
healthcare
system for
everyone**



Healthcare systems
buy health outcomes,
not drugs



Needs a
repeatable and
consistent
approach



So the ultimate
purpose of patient
involvement is to...

It is the strength of the evidence that the promised health outcomes will be realised that drives much of the decision

So that citizens can see that the same process is applied, no matter what the disease and that lobbying is not unfairly changing the rules for some

... improve the quality of HTA decisions:
Not lobbying, but ensuring patient evidence is considered as an important part of the decision

A range of methods used

Just a few examples:

Informal discussions with patient groups on an ad-hoc basis

Open public consultations where patients, physicians and members of the public can comment

Formal processes with written submissions from patient groups as part of the considered evidence

Representation at committee meetings as patient experts to give testimony and answer questions

A common approach looks like this:



Scoping phase

To determine the areas of focus for an appraisal



Patient input

Consultation and workshops

Evidence Submission

To collect evidence from affected stakeholders



Patient input

Patient organisations and patient expert written submissions

Committee Meeting 1

To make a draft recommendation



Patient input

Patient experts attend and answer questions

Public members involved in decision making discussions

Consultation

To allow input on the draft recommendation



Patient input

Patient organisation and patient experts comment

Committee meeting 2

To discuss the comments



Patient input

Patient experts exceptionally invited back

Public members in the decision making discussions

Final Appraisal Decision / Recommendation

The final recommendation



Patient input

Comment on factual accuracies

Can appeal

What if no patient involvement in a country's HTA?

Some countries do have HTA but they don't involve patients

OPPORTUNITIES

To use examples from other countries and the body of evidence that supports patient involvement to call for the formal inclusion of patients into the HTA process

1. Understand the system, who controls it, who sits on committees and who gives input
2. Short-term: Align with those who do give input to ensure the patient view is included in their input
3. Longer-term: Join other disease areas to call for a formal patient input into the process

TOP TIPS FOR INTERACTING WITH HTA / PAYERS

Understand what questions they care about...



1. How does this condition affect the day-to-day lives of people living with it?
2. How well do medicines which are currently available help patients manage this condition?
3. Have you (the patient organization) been able to consult with patients who have used this medicine?
4. Would this medicine be expected to improve the patient's quality of life and experience of care, and if so, how?
5. What kind of impact would treating a patient with this medicine have on the patient's family or carers?
6. Are there any disadvantages of the new medicine compared to current standard treatments?

Tips to remember when answering these questions



Be specific and be focused on patient experience and impacts



Focus on the patient perspective



Be specific



Pure emotion does not help the decision

Do not repeat information they get from others such as the evidence from the clinical studies. Focus on patient impacts and experiences

Highlight the day to day and long-term impacts on what patients experience from the disease and its treatment – use examples and evidence

Emotion is a powerful tool, but HTA bodies hear this from all disease areas. Psychosocial impacts related to the disease specifics are best.

Tips to remember (2)

Work with the system you have, not the system you would like



Be specific and be focused on patient experience and impacts



Find or build evidence that supports your points



Highlight any patient types that are particularly impacted



Look at submissions from previous/other HTA submissions

Patient surveys, quotes, stories with specifics, are valuable tools to substantiate the unmet needs. Many HTA bodies accept evidence from outside the country too (e.g. from other patient groups)

A key question HTA bodies are trying to assess is 'who will benefit most from this treatment'. If there are certain kinds of patients whose needs are not being met, then highlight these.

Many HTA bodies publish the input from patient groups. Take a look at some past submissions and the evidence used – can any of this be re-used?

Remember, even though you may want access to a therapy:

***It is not the patient
group's responsibility to
secure access***

*HTA is a multidisciplinary process
with many reasons why a treatment
may not be recommended*

*Many of these reasons are outside
the control of the patient community*



A negative assessment is not the end of the story...

It is the beginning of a process that often leads to reimbursed access:

Managed entry agreements can be negotiated that allow for access for a small group of patients while further evidence is collected

Companies can also resubmit with new evidence that answers some of the HTA concerns

Many systems allow for an appeal if stakeholders (including patient groups) believe that evidence has not been appropriately considered

CASE STUDY

Patient input into a potential new treatment for eczema



Key points raised:

- Living with eczema can be significantly life challenging
- The incessant itch of eczema can be intolerable - difficult to carry out day-to-day tasks
- Simply getting dressed (and finding skin friendly clothing) can be very difficult
- More severe eczema can be painful with damaged skin cracks and bleeds
- Holding a pencil, typing, or holding your baby will be extraordinarily painful if hands are affected
- A visible condition still often perceived as infectious or a result of poor personal hygiene
- For those whose eczema is unresponsive to topical treatments (topical steroids and topical calcineurin inhibitors) the options are quite limited
- Worries about the potential adverse effects of topical steroids and frightened at the prospect of using any of the currently available systemic options
- Systemic treatments are quite limited and all suppress the immune system

CASE STUDY

Patient input into a potential new treatment for eczema



Evidence collected by a patient group and provided to the HTA

Supplied a Patient Survey:

- 305 people were surveyed
- 58% said it impacts on their personal relationships
- 10% spent over 30 days a year managing their eczema e.g. by applying creams
- 86% said that the management of the condition impacts their day to day activities
- 7 in 10 said their sleep was affected
- 1 in 10 consume more alcohol when their eczema is at it's worst
- Over 70% reported feeling depressed
- Nearly 1/4 missed more than 6 days of work per year due to their condition, whilst approx. 15% missed 16 or more days

The treatment was recommended for reimbursement in patients with moderate to severe eczema for whom standard systemic therapies do not work or are not tolerated



Pause for questions

Summary and key takeaways



Be realistic, start with what you know, take it a step at a time

Policy makers, regulators, HTA professionals are all human beings too

Building trusted relationships is no different than building friendships – it starts small



DISCUSSION



An illustration of a woman with dark hair, wearing a yellow sweater, reaching upwards with her right hand towards a red puzzle piece. The background is a light yellow with scattered pink dots and several other puzzle pieces in orange, blue, and teal. A white rectangular box is positioned on the left side of the image, containing the text 'Thank you!' in a brown, sans-serif font.

Thank you!