EUROPEAN CANCER PATIENT COALITION

Patient Involvement in HTA

An Online Guide for Patient Organisations

Thank you very much for your interest in this online guide!

This document is an in-progress draft of the content for the online guide. Once finalised, images and interactivity will be added. In this draft, shaded boxes like this one are used to describe the planned animated or interactive features.

If you have any comments on the proposed content of the guide that you would like to share with us, please get in touch with Alex Filicevas (alex.filicevas@ecpc.org)

EUROPEAN CANCER PATIENT COALITION

Chapter 1

Introduction

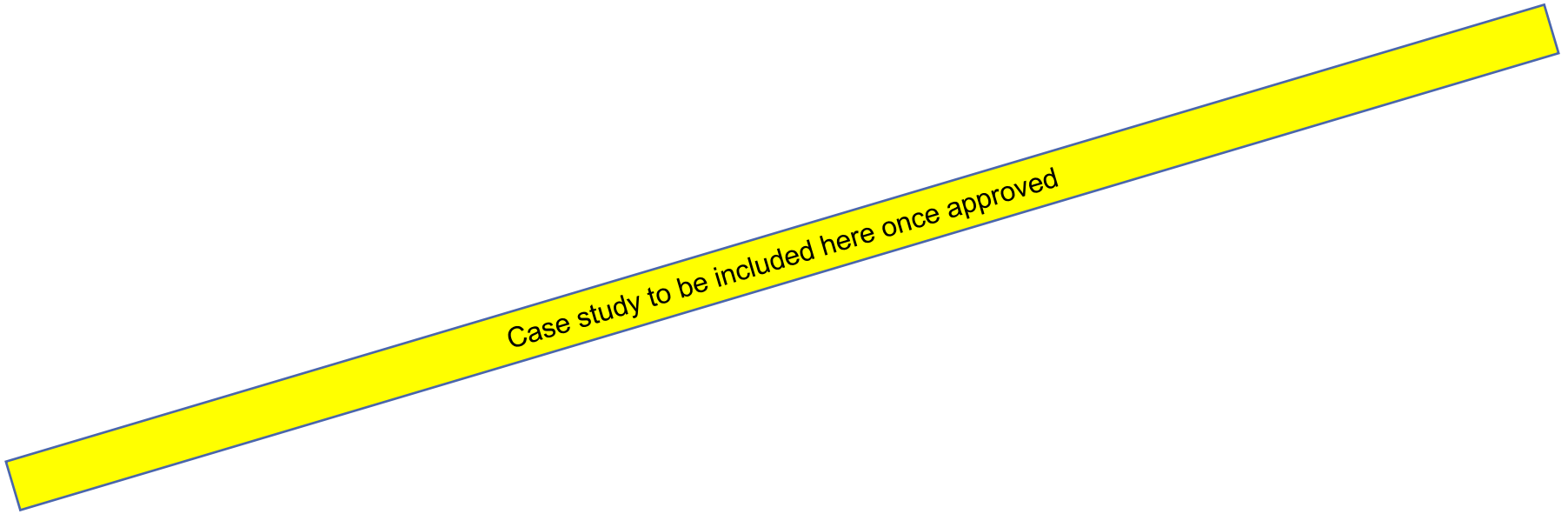
EUROPEAN CANCER PATIENT COALITION

Why is This Important for Cancer Patients?

- All patients are directly affected by the decisions of individual countries about whether to make new medicines available
- To make these decisions, the **value** of the new treatment is considered, and how this compares to the value of treatments that are already available
  - Measuring the value of a new treatment to inform these decisions is not easy. It is particularly challenging for cancer treatments, compared to other diseases such as arthritis or diabetes
  - 'Value' can mean different things to different people. People living with cancer and their carers can contribute important information about the need for new therapies and their impact on peoples' lives. Their unique knowledge, perspectives and experiences are crucial in determining the value of new treatments

(Interactivity) Click to see the case study

Having patient input in the decision making process could be the difference between a new cancer medicine being available to patients in a particular country or not



EUROPEAN CANCER PATIENT COALITION

Why is This Important Now?

- With the development of new medicines, the treatment of many cancers is advancing. Research and development offers promising and life-changing innovative medicines, which come at higher and higher costs. To ensure the sustainability of healthcare systems, there is an increasing need to measure the value of these innovative medicines
- Patient access to innovative medicines is disparate across European Union. Research published in 2015 showed that delays in access to new treatments range from a few months to up to 10 years in some cases.[1] One of ECPC's core goals is to fight and reduce these increasing inequalities
- Educating and empowering patient organisations to participate in the healthcare decision making process in their country may help factors that are important to patients but are not currently taken into account by health systems when deciding the value of new treatments, such as social and economic benefits of treatment, to be included in future

Animation: scroll to review the next paragraph
(Reference) 1: Europe of Disparities", ECPC 2015
(Pop-up) Click to learn more about the ECPC

A Stronger European Union Cooperation in Health Technology Assessments (HTA) Could Help in Reducing Inequalities in Access to Innovative Technologies, in Particular in Cancer Treatments

2014

The European Cancer Patient Coalition (**ECPC**) is a key member of the European Cancer Concord (ECC), representing cancer patients alongside eight other organisations of patients and consumers. ECPC worked closely in partnership with ECC to launch The European Bill of Cancer Patient's Rights on World Cancer Day in 2014. The aim of this bill was to foster closer cooperation on Health Technology Assessment (HTA) in Europe to accelerate the reimbursement process and give patients faster access to new treatments. It was further supported with a Written Declaration in the European Parliament and supported by more than 250 Members of the European Parliament

2015

ECPC published a White Paper in 2015 "Europe of Disparities", showing the enormous disparities in access, the time-to-access to innovative drugs in the European Union sometimes is from a few months up to 10 years

2016

In 2016, the ECPC was accepted as a member of the European Commission's HTA Assessment Network Stakeholder Pool, representing cancer patients, alongside eight other organisations of patients and consumers

The ECPC amplified the momentum on the European Union cooperation on HTA during the process of amending the regulation 726/2004 ("*Laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency*"), which was voted by the ENVI Committee and the European Parliament in the first quarter of 2016. The European Cancer Patient Coalition supported European Medicines Agency Director Prof Guido Rasi, as well as MEPs Elisabetta Gardini and Alberto Cirio, authors of amendments to the Regulation 726/2004 that called for further integration of HTA systems in Europe. In 2017, the European Parliament passed a new report on the "Options for Improving Access to Medicines", which contains most of the amendments proposed in the European Cancer Patient Coalition's position paper on this topic

2017

The ECPC responded to the European Commission's 2017 consultation on "Strengthening of the EU cooperation on Health Technology Assessment", stating that increased EU cooperation will cut costs and duplication of efforts across HTA bodies, decrease the delay in access to innovative health technologies, facilitate participation of patient organisations in the HTA decision making process, and facilitate access to the market for industry. The response to the public consultation was followed by a meeting between representatives from the ECPC and the European Commission's unit in charge of the work on HTA.

2018

EUROPEAN CANCER PATIENT COALITION

Overview of this Guide

- This online guide is divided into 5 chapters:
  - What is Health Technology Assessment (HTA)?
  - Involvement of Cancer Patient Organisations in the HTA process
  - Why the HTA of Cancer Treatments is Different to that of Treatments for Other Diseases
  - Defining Survival, Quality of Life and Safety in People With Cancer
  - Why Some Countries Reimburse a Cancer Treatment and Others Do Not
- Where you see the (click) symbol, click to reveal more information! If words are (**bold**), click to see more information.
- The progress bar shows how far you are through a certain chapter. You can leave and renter the guide whenever you like. Clicking on the navigation bar on the left navigates to a different chapter
- We hope you find this guide useful. Further information expanding on the topics covered is available as a booklet, which is available for download from the ECPC website (link).

Chapter 2

What is Health Technology Assessment?

EUROPEAN CANCER PATIENT COALITION

New Medicines: From Discovery to Patient

Discovery and Development | **Clinical Trials** | **Regulatory Approval** | **Reimbursement by Health System** | **Medicine Available to Patients**

- Medical interventions, such as drugs, procedures, devices and prevention programmes, are known as ‘health technologies’. This guide focuses on medicines for the treatment of cancer
- It usually takes ten years or more for a new medicine discovered in the laboratory to become widely available to patients. Click on the icons to learn more about each step in this process

Researchers identify promising compounds to be developed into potential new treatments.

Studies are run to assess:
- Safety
- Quality- Each dose of a drug manufactured should contain the same concentration of active ingredient and have minimal impurities
- Efficacy- How well a treatment works in a clinical study

Based on the outcomes of the clinical trials, the regulatory authority (**European Medicines Agency** in Europe) decides whether to grant a Marketing Authorisation for the new medicine; this means the product can be sold in Europe

National health systems decide if they will fund (**reimburse**) a new treatment based on the cost of the drug and how well it works.
Depending on the country, treatments can be funded by either:
- National health systems: Healthcare is financed and provided by the government through tax payments
- Insurers: People pay into an insurance program which provides healthcare coverage. Doctors and hospitals tend to be private in these countries
- Co-payments: Patients cover some of the costs of the new treatment and the remainder is funded by the national health system

(Interactivity) Click to learn more about each step in this process

EUROPEAN CANCER PATIENT COALITION

Difficult Decisions

Discovery and Development

Clinical Trials

Regulatory Approval

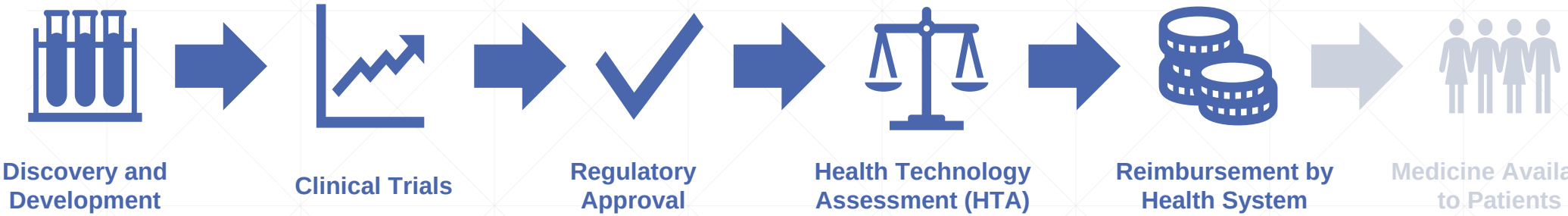
Reimbursement by Health System

Medicine Available to Patients

- With a limited budget for healthcare, a decision to pay for a new treatment for some patients is effectively a decision not to pay for a different treatment for others. This is called the ‘opportunity cost’ of paying for a certain treatment
- To illustrate, imagine you have €1m. It may be spent either to vaccinate 10m people against measles, pay for medicine that alleviates the symptoms of 100,000 people with arthritis, or pay for 50 life-saving kidney transplants, how would you choose to spend the money?
- Policy makers have to choose how best to use their resources to benefit patients. What is considered the ‘best’ way to allocate budget can be different between countries (or even regions within a country), depending on the priorities and values of each health system

(Animation) Section of the image is highlighted

What is Health Technology Assessment?



- **Health technology assessment (HTA)** is a process that evaluates the clinical, economic and societal implications of a new treatment
- The information provided by HTA gives policy makers a measure of the added value of funding a new treatment compared to the current standard of care. This can support policy makers in their decisions about whether new treatments should be introduced
- HTA is done differently in different countries; some countries do not have a formal HTA process
- Click here to learn how the European Union Network of Health Technology Assessment organisations defines HTA

(Animation) HTA appears as part of the pathway; (Pop-up) Next slide

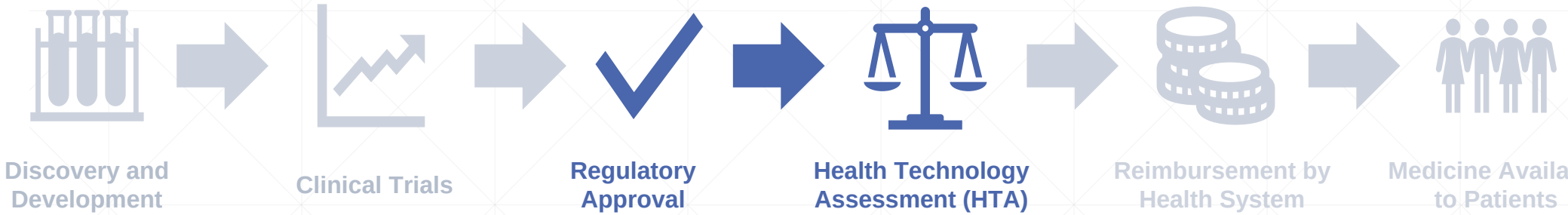
EUROPEAN CANCER PATIENT COALITION

Health Technology Assessment…

"… a multidisciplinary process that summarises information about the medical, **social, economic and ethical issues related to the use of a health technology in a systematic, transparent, unbiased, robust manner.**

It's aim is to inform the formulation of safe, effective health policies that are **patient-**focussed and seek to achieve best value"

Regulatory Approval and HTA: What's the Difference?



Regulatory approval and health technology assessment ask different questions

Regulatory approval
Does the treatment work and is it safe?

HTA:
Does the treatment represent value for money?

EUROPEAN CANCER PATIENT COALITION

What Happens After a Medicine is Approved by the EMA?

After regulatory approval of a new medicine, health systems decide if they will reimburse a new treatment. How exactly the decision is made varies from country to country.

The following things may be considered:

- The burden of the condition on patients and the health system
- The management of the condition and treatments currently available
- The evidence of the new treatment being considered and the interpretation of the evidence
- The value for money of the new treatment

Click here to learn more

Policy makers also consider the evidence in light of wider factors related to the local context of their healthcare system.

(Pop-up) Next slide

EUROPEAN CANCER PATIENT COALITION

Clinical evidence

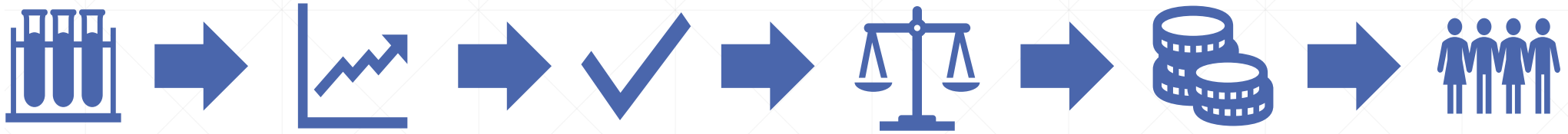
- How much the new medicine does more good than harm, compared with one or more alternative treatments that would normally be given (known as '**relative effectiveness**')

Non-clinical evidence

- The impact the new treatment may have on the national health system's budget (known as a '**budget impact evaluation**')
- The balance of costs and benefits associated with introducing a new therapy to the health system (known as '**cost effectiveness analysis**')
    - The benefit of the medicine can be measured in many ways, often focusing on if treated patients live longer or if their quality of life improves
    - As well as the cost of the new medicine, costs incurred by the hospital or use of other health resources used throughout the patients life as a consequence of giving the medicine are considered
    - Potential savings should also be considered, for example if a new medicine means a patient needs fewer trips to hospital
- A more expensive treatment could be considered better value for money than a less expensive treatment, provided it provides a sufficiently greater benefit to patients

EUROPEAN CANCER PATIENT COALITION

What Happens Next?



Discovery and Development

Clinical Trials

Regulatory Approval

Health Technology Assessment (HTA)

Reimbursement by Health System

Medicine Available to Patients

Based on the evidence available, policy makers then decide if they will reimburse a new treatment. A report may be produced to explain how the decision was made

It may be decided to limit the group of patients for whom the new medicine is recommended, or prioritise the order of treatment options that should be tried. For example, a new medicine may be recommended for use only after alternative treatments have been unsuccessful

If there is not enough evidence but the new treatment that appears promising, the health system may reimburse the treatment but require further clinical trials or collection of evidence in the future

If health systems think the new treatment is too expensive, they may negotiate with the manufacturers on the price

EUROPEAN CANCER PATIENT COALITION

The System is Different in Different Countries

- Not all EU member states have an official HTA process. In countries that do, the process and the organisation(s) responsible is often different between countries
- All HTA processes consider the added clinical benefit of a new medicine, however the emphasis on cost-effectiveness varies

Country	Type of Health System	HTA Agency	Pricing and Reimbursement Considerations	Information
Austria	Social insurance	The Federal Ministry of Health, Family and Youth (BMGFJ)	Pharmacological analysis, medical-therapeutic evaluation, Economic considerations	Link
Denmark	Tax-based	The Danish Medicines Agency	Therapeutic effect, value added, side effects, price comparisons and economic eva	Link
France	Health insurance	The National Authority for Health (HAS)	The medical benefit (SMR), improvement of medical benefit (ASM ligible for treatment	Link
Germany	Social health insurance	The Institute for Quality and Efficiency in Health Care (IQWIG) and The Federal Joint Committee (G-BA)	Therapeutic classification, comparability with ffectiveness	Link
Greece	Mixture of tax, social insurance and private expenditure	Social insurance funds are responsible for reimbursement and pricing is set by the Ministry of Development	Reimbursement d on disease severity and socioeconomic conditions of the beneficiary	Link
Hungary	Funded by a mixture of tax and social insurance	The Health Technology Assessment Office	ffectiveness, pharmacoeconomic data, budget impact calculations	Link
Ireland	Private health insurance and Community Drug Schemes	The Health Informati y (HIQA)	Cost effectiveness, budget impact, innovative nature of the technology, costs and benefits to society	Link
Italy	Tax-based	dicines Agency (AIFA) and The Pricing and mbursement Committee (CPR)	Therapeutic benefit, cost effectiveness, risk-benefit ratio, economic impact on health system	Link
The Ne s		The National Healthcare Institute (ZIN)	Additional clinical benefit, cost effectiveness, budgetary impact	Link
Poland	Social insurance and private insurance	Health Technology Assessment Agency (AOTM)	Safety, importance of drug, influence of drug on medical costs, affordability, risk-benefit ratio, economic impact on health system	Link
Portugal		The National Authority of Medicines and Health Products (INFARMED)		
Spain	Public health care insurance	Instituto de Salud Carlos III (ISCIII) at the national level and several regional HTA organisations	Efficacy, cost, efficiency, effectiveness, safety and therapeutic utility	Link

Table to be completed with all EU member states and presented as pop-ups from an interactive map

EUROPEAN CANCER PATIENT COALITION

Chapter 3

Involvement of Cancer Patient Organisations in the HTA Process

EUROPEAN CANCER PATIENT COALITION

Why is the Involvement of Patients in HTA Important?

- All patients are directly affected by HTA decisions. For example, the outcome of a HTA could result in:
  - Delayed access to a new treatment
  - Limited access to a certain population
  - No access if the new treatment is not approved for reimbursement
- The information patients can provide information and insight about the impact of their condition and treatments on their lives is important because it can't usually be obtained from anywhere else (such as from clinical trials).
- Patient involvement is particularly valuable in the assessment of new cancer treatments. The reasons for this will be covered in chapters 4 and 5 of this guide.

EUROPEAN CANCER PATIENT COALITION

How Can Patients be Involved in HTA?

- There are many ways that patients can be involved in the decisions about access to medicines, even in countries where there isn't a formal process for HTA
- Patient organisations should try to build up a relationship with the relevant organisations in their country to ensure they are told about the opportunities for patient involvement
- In this guide, we will focus on two ways that patients can provide their expert input into the decision making process for a new medicine

Identifying potential topics for HTA

Serving as members of HTA boards, committees and workgroups

Providing **expert input** to an appraisal committee

Helping to design and prepare patient-friendly HTA report summaries

Identifying patient-related health outcomes and other impacts e.g. economic, social, to be assessed

Submitting evidence, known as a **patient group submission**

Reviewing draft HTA reports and recommendations

Dissemination of HTA findings to policymakers, patient groups and other target groups

EUROPEAN CANCER PATIENT COALITION

Patient Group Submissions

In some countries, patient groups can submit evidence, known as a patient group submission, to be considered in the HTA process. The purpose of this is to identify the priorities and preferences of patients. Use the buttons below to see guidance on what might be useful to include in a patient group submission*

Do

- ✓ Mention how information was obtained and number of participants
- ✓ Mention patient and carer experience
- ✓ Report on experiences of many people living with this condition
- ✓ Report on current therapies and anticipated impact of medicine in patients who have never used the medicine being assessed
- ✓ Explain what side effects patients are prepared to live with
- ✓ Describe experiences of patients who have used the medicine being assessed

Don't

- ✗ Reference literature cases
- ✗ Include scientific evidence
- ✗ Include input from clinicians or other healthcare providers or pharmaceutical manufacturers

*Before submitting any evidence, please check with the relevant organisation what should be provided, who may send a template for patients to complete and submit

EUROPEAN CANCER PATIENT COALITION

Patients' Experiences of Being Involved in HTA

Case studies to be included here once approved

EUROPEAN CANCER PATIENT COALITION

Top Tips

- Reach out to the organisation conducting the HTA for guidance and clarity on the submission process and deadlines
- Collect as much patient information as possible to support the submission
- After handing in a submission, make an effort to attend the discussion meetings. Having patients give their individual perspective at these meetings can add a lot of value and make a real difference to the committee's decision about whether to consider a treatment for reimbursement
- If you are able, showcase your understanding of the scientific evidence by contributing during discussion meetings.
- Above all, do not let lack of capacity limit your involvement!

How to Collect Patient Information

- Click on the buttons below to view further information on each step

1. Identify what information you will collect

Qualitative: Thoughts, experiences, stories and feelings of patients and carers

- Challenges of managing condition
- Difficulties in receiving or accessing treatment
- How treatment has or has not improved quality of life
- Side effects

Quantitative:

- How much time spent travelling to appointments
- How long treatment works for
- How many therapies patients have been on

2. Identify who to collect information from

Consider patients with different:

- Educational background
- Socio-economic status
- Geographic location
- Age

3. Decide on method to recruit participants

- Patient database
- Connect with external groups or partner organisations
- Use meetings or conferences
- Use social media
- Ask clinicians running trials

EUROPEAN CANCER PATIENT COALITION

Patient Involvement in the HTA Process Does Not Guarantee that a New Medicine Will Be Made Available

- Providing patient evidence does not ensure that the new medicine will be made available to patients in your country, but it may help ensure your voice is heard
- There may be an opportunity to appeal the decision if you do not agree with the recommendations
- It can be frustrating if you feel that your views have not been heard but it may be that other factors were considered to be more significant

EUROPEAN CANCER PATIENT COALITION

Chapter 4

Why Cancer Treatments are Different from Treatments for Other Diseases

EUROPEAN CANCER PATIENT COALITION

What is Different About Cancer?

- In total, there are more than 100 types of cancer. Specific treatments must be developed for the different cancer types. This makes treating cancer difficult as ‘one-size-fits all’ therapies are less realistic for cancer treatments than treatments for other diseases such as arthritis or diabetes
- There is a need to speed up access to new treatments for cancer patients as there might be few treatment options available. Reduced or delayed access to cancer medicines has a very real impact on patient survival
- Many cancer treatments are expensive and may only offer a small extension to life

EUROPEAN CANCER PATIENT COALITION

Clinical Trials

- New treatments have to be thoroughly tested. Medical research studies involving people are called clinical trials. Trials aim to find out if a new treatment or procedure:
  - Is safe
  - Has side effects
  - Works better than the currently used treatment
  - Helps patients feel better
- Randomised controlled trials are the gold standard for determining the efficacy and safety of new, innovative treatment regimens for patients with cancer and other diseases
- Patients in the control arm will not receive the new treatment, to provide a comparison to see how the innovation compares against no treatment or the current standard treatment

EUROPEAN CANCER PATIENT COALITION

HTA of Cancer Medicines

- For a number of reasons, collecting evidence to demonstrate the effectiveness of cancer treatments is often more difficult that for treatments for other diseases. Therefore, evaluating the value of new medicines and making decisions about whether to reimburse a medicine can be more difficult.
    - In some cancers, the number of patients who are eligible to take part in clinical trials is small. Researchers need a certain number of patients to allow them to be confident in the conclusions they draw from the results of clinical trials
    - In cancer trials with a placebo control, patients in the placebo group may be offered the opportunity to ‘cross over’ into the group being given the new medicine if their disease progresses. Again, this reduces the strength of any conclusions that can be made based on the results of the trial
    - Cancer treatment trials commonly use the standard treatment as the comparator due to ethical issues with using a placebo control. However, most trials may take years before the results are reported and therefore the standard treatment may not be relevant by time of the HTA
    - In third line or rescue cancer therapy, there is often no other treatment options to serve as an appropriate comparator

EUROPEAN CANCER PATIENT COALITION

Chapter 5

Defining Survival, Quality of Life and Safety in People With Cancer

EUROPEAN CANCER PATIENT COALITION

What do Clinical Trials Measure?

- An endpoint is something which is measured in a clinical trial or study
- A clinical trial will usually define or specify a primary endpoint as a measure that will be considered success of the therapy being studied
- A useful treatment is generally considered to be one that improves the quality and/or increases the length of patients lives. Therefore, clinical trials of cancer treatments need to measure the survival, quality of life and safety of the participants

Use of Surrogate Outcomes in Clinical Trials

- The time from a patient joining a study to death (from any cause) is known as “overall survival” (OS). Due to the nature of cancer clinical trials, complete information on overall survival (OS) is not available until the last person in the trial dies. Therefore, using survival as a primary endpoint in cancer trials means that long follow-up periods are needed before the trial is completed, potentially delaying patient access to beneficial treatment
- For some cancers such as chronic lymphocytic leukaemia, it sometimes take years to have the survival data. This is good for patients, however health systems may need to see this data before they decide to reimburse the medicine
- A surrogate outcome is an outcome that is observable within the trial period or soon after treatment has finished, and has a clear link to the final outcome (such as overall survival). Examples of surrogate endpoints for overall survival are:
    - Disease-free survival (DFS) – time from randomisation until tumour recurrence or death from any cause
    - Progression-free survival (PFS) – time from randomisation until objective progression or death from any cause
- In order to speed up access to new cancer medicines, regulatory agencies accept the use of surrogate outcomes to demonstrate efficacy of new medicines. However, use of surrogate outcomes makes it difficult for HTA to assess whether the medicine is likely to offer real benefit to patients due to the lack of mature data on overall survival

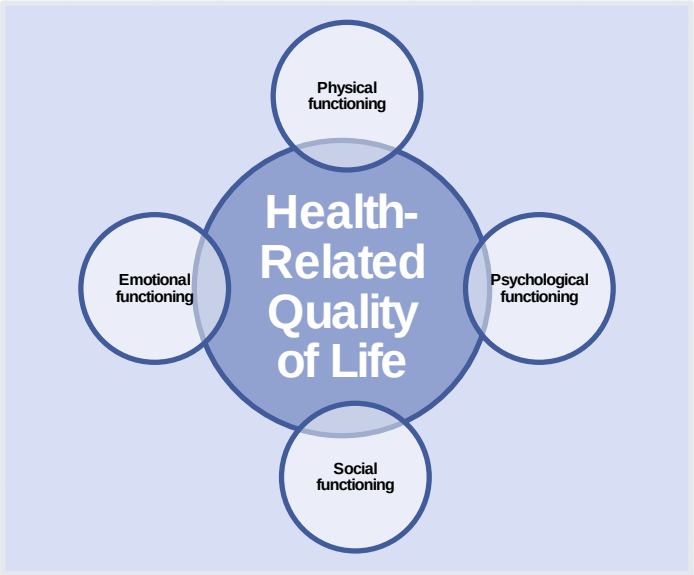
EUROPEAN CANCER PATIENT COALITION

Challenges to Measuring Survival

- In order to measure survival it is important to state what the event is (death or relapse) and when the period of observation starts and finishes
- This data might be difficult to collect for all patients in cancer clinical trials because:
  - Some patients will be lost to follow-up
  - Some patients will go on other treatments after the one being evaluated
- Therefore, for some patients, the true time to event will be unknown which makes analysis of survival difficult

What is Quality of Life?

- Quality of life (QOL) is a broad concept including subjective evaluations of both positive and negative aspects of life. QOL can be defined in many ways which makes it difficult to measure, therefore in clinical trials health-related quality of life (HRQOL) outcomes are measured.



- **Many cancer treatments may only offer a small extension to life. Therefore improvements in quality of life are very important when deciding whether to recommend a treatment for reimbursement**

EUROPEAN CANCER PATIENT COALITION

Measuring Health-Related Quality of Life

- There is not one, standard way to measure health-related quality of life
- Questionnaires are often used, which can include questions such as if a patient is having problems in walking about, or if they are feeling worried or depressed
- Some questionnaires have been designed specifically to capture the impact of cancer and cancer treatments on a person's quality of life
- However, because health systems have to make decisions across different diseases, they generally prefer questionnaires that are not specific to one disease. These 'generic' questionnaires might not be as good at recording the impact of cancer and cancer treatments on a person's quality of life

EUROPEAN CANCER PATIENT COALITION

Safety

- Cancer treatment can cause side effects. Additionally, many cancer patients have non-cancer comorbidities and receive multiple medications. Potential drug interactions with drugs administered for non-cancer comorbidities may lead to harmful adverse events
- Adverse events will have detrimental impacts on quality of life and will also incur costs, both of which need to be considered when evaluating the value of a new treatment

EUROPEAN CANCER PATIENT COALITION

Chapter 6

Why Some Countries Reimburse a Cancer Treatment and Others Do Not

EUROPEAN CANCER PATIENT COALITION

"A Europe of Disparities"

- There are imbalances and inequalities in the ability of cancer patients to access cancer medicines, cancer care and therapies in Europe
- These differences are likely due to a number of factors, one of which is how reimbursement decisions are made in each country
  - If a new treatment is not reimbursed by a country's health system, the vast majority of patients will be unable to access it
  - The time until decisions are made on whether to reimburse new treatments can also vary between health systems, meaning that new medicines may be available to patients in some countries before others

EUROPEAN CANCER PATIENT COALITION

Why Some Countries Reimburse a Cancer Treatment and Others Do Not

Differences in the structure, function, scope and approaches of organisations responsible for HTA and policy making	*For example:* • *Some countries use formal methods when assessing cost-effectiveness, whilst others do not* • *Some countries assess treatments for rare cancers differently to treatments for other types of cancers*
Different priorities for healthcare	*For example, if the severity of the condition is a priority for a health system, it may have a higher willingness to pay for treatments for severe conditions*
How much money the country has chosen to spend on public health	*Countries which spend more on healthcare tend to accept more treatments into their national health systems than countries which spend less*
Price of new medicines	*Each healthcare system can negotiate independently with manufacturers*
The type of healthcare system	*Studies have found that more treatments are approved in social health insurance systems than tax-based systems*

EUROPEAN CANCER PATIENT COALITION

Supporting Access to Cancer Medicines

- A major challenge in access to new treatments stems from the different ways value of a treatment is measured by the European Medicines Agency when approving a new treatment, and each Member State when making reimbursement decisions. Therefore, improved EU cooperation in assessing new medicines could help.
- Increased cooperation between EU and observer non-member states in HTA could also avoid duplication of efforts across health systems
- As such, efforts to harmonise HTA at the European level are ongoing *(link to further information).* ECPC is part of the HTA Network Stakeholders Pool, representing cancer patients and contributing to policy development related to HTA at EU level
- Some countries are addressing the challenge of patient access to innovative cancer medicines by putting in place national cancer plans that may include special funding for the medicines to be accessed outside of the usual system

EUROPEAN CANCER PATIENT COALITION

Call to Action!

- Patient organisations have a role to play in improving access to innovative treatments for patients in their country
- Participating in HTA can be challenging and time consuming but it is a real opportunity to influence the delivery of healthcare and help to determine whether new treatments are made available to patients
- We hope you found the information in this guide useful. If you'd like to learn more, further information expanding on the topics covered in the guide is available in a booklet available for download from the ECPC website (link)

EUROPEAN CANCER PATIENT COALITION

Glossary

- **Adverse effect**: A negative or unwanted side effect that results from a treatment or intervention.
- **Clinical efficacy**: The benefit of a test, treatment or procedure gained under ideal conditions, such as in a clinical trial. Efficacy can be assessed at treatment launch based on global data.
- **Clinical effectiveness**: How well a treatment works in the 'real world' rather than in a carefully controlled clinical trial. Health systems are interested in the effectiveness of a new treatment for patients in the health system of interest. Effectiveness can only be assessed properly after years on the market and needs national data.
- **DNA:** part of cells that contains the information needed to run and repair the body.
- **Health technologies**: Medical interventions, such as drugs, procedures, devices, prevention programmes, are known as 'health technologies'. The health technologies covered in this guide are medicines for cancer treatment.
- **Health technology assessment (HTA)**: a multidisciplinary process that summarises information about the medical, social, economic and ethical issues related to the use of a health technology in a systematic, transparent, unbiased, robust manner. It's aim is to inform the formulation of safe, effective health policies that are patient-focussed and seek to achieve best value" (EUnetHTA definition)
- **Reimbursement**: Health systems fund a new treatment by paying the manufacturers the cost of the treatment. This enables access for patients.