



# PATIENTS IN HTA

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**EURODIS.ORG**

## THE 3 STEPS

### Regulatory b/r

Can the product work?

In the context of a clinical trial

### REA

Does the product work and improve care?

In clinical practice

### Reimbursement decision & pricing

Is society willing to pay?

Based on REA and economic aspects

# Principles of patient and consumer engagement in HTA: Fair priority setting

A fair  
HTA  
process  
should  
ensure

Publicity

Availability of decisions to the wider public for scrutiny

Relevance

Stakeholders agreeing upon the “relevance” of the inputs for the decision

Appeals

Objections and contributions to the revision of decisions

Enforcement

“publicity”, “relevance”, “appeals” appropriately followed

Responsibility

Public (patients) agree with the methods, are consulted in an appropriate manner, adhere to the results

## You can engage in:

- HTA early dialogues
  - to minimise the risks that inadequate information are submitted at a later stage
- Scoping
  - which domains/topics/questions should be answered?
- Assessment
  - Providing the answers
- (Appraisal)
  - Making the decision to cover/reimburse

# HTA domains

Clinical domains	Economic domains
Description of the technology Intended use	Budget impact
Efficacy	Cost benefit analysis
Safety	Cost utility analysis
Relative effectiveness	Modelisation
Patient and social aspects	
Other domains	
Ethical aspects	
Organisational aspects	
Legal aspects	

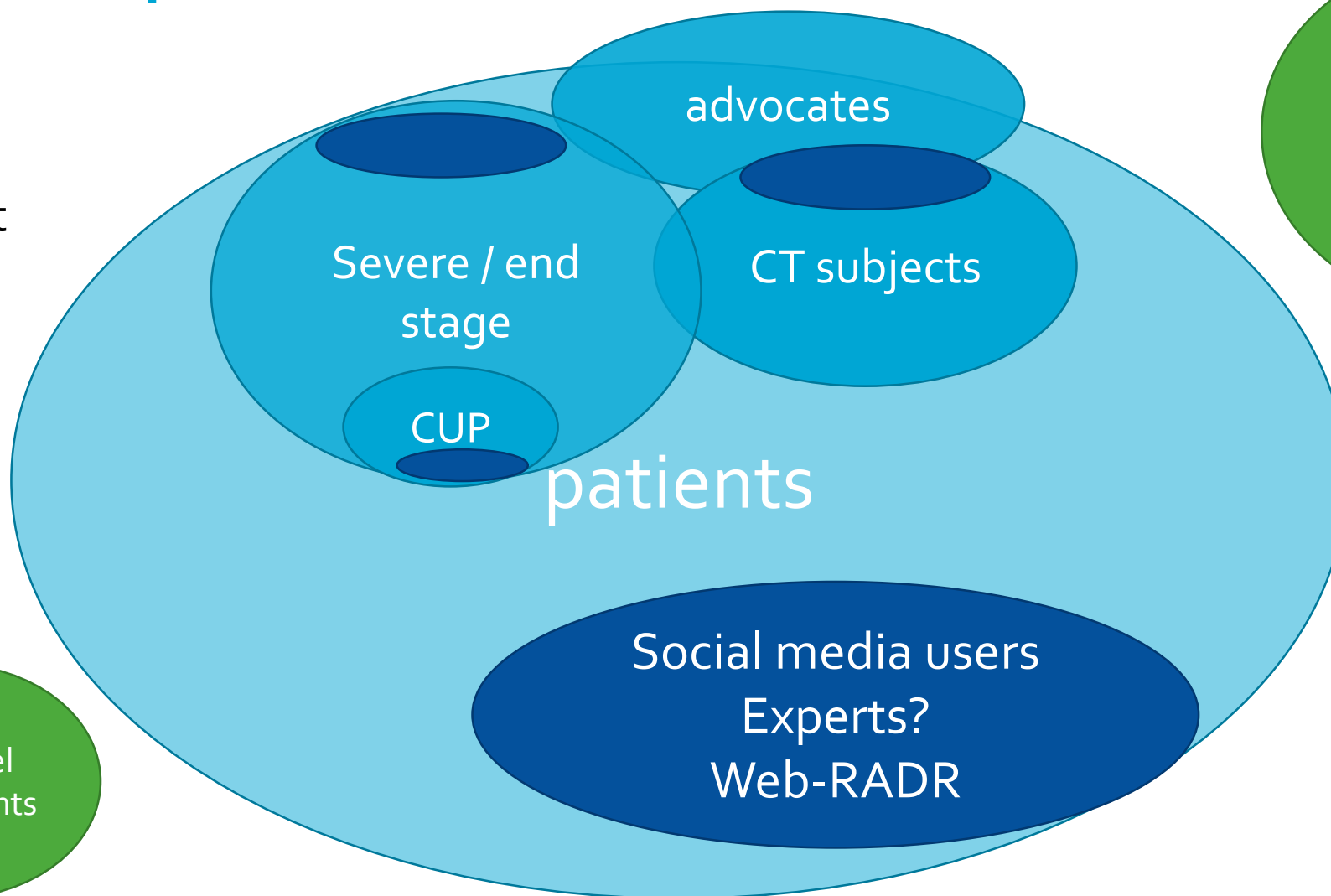
# PICO

- Population
- Intervention
- Comparator
- Outcome

# You said patients?

Similar but  
different  
condition?  
Carer?  
Siblings?

Off-label  
use patients



# Difficulties finding patients. HTA early dialogues (SEED/EMA/EUnetHTA) 13

patients invited for 22 seats (59%)  
35 contacted (37%), 57 organisations, 284+ emails (+ phone)

Date	Condition	Type	Technology	Patients attended / contacted	POs contacted
18 Sept. 2014	Non-small C lung cancer	SEED	Medicine	0 / 1	1
8 Oct. 2014	Confidential	EMA-HTA	Medicine	1 / 2	1
3 Dec. 2014	Myasthenia Gravis	EMA-HTA	Medicine	0 / 3	2
15 Jan. 2015	Heart failure	SEED	Implantable device	2 / 2	2
22 Jan. 2015	Confidential	SEED	Medicine	2 / 5	5
12 Feb. 2015	Asthma	SEED	Medicine	1 / 4	11
13 Feb. 2015	Thyroid cancer	SEED	Diagnostic test	2 / 5	10
10 Mar. 2015	Discogenic back pain	EMA-HTA	Medicine	1 / 4	14
14 Apr. 2015	Implantable heart	SEED	Implantable device	1 / 2	2
29 June 2015	Sanfilippo syndrome	EUnetHTA	Medicine	1 / 4	4
7 July 2015	Haemophilia A	EMA-HTA	Medicine	1 / 2	2
7 Sept. 2015	Insulin dependent diab.	EUnetHTA	Device	1 / 1	3

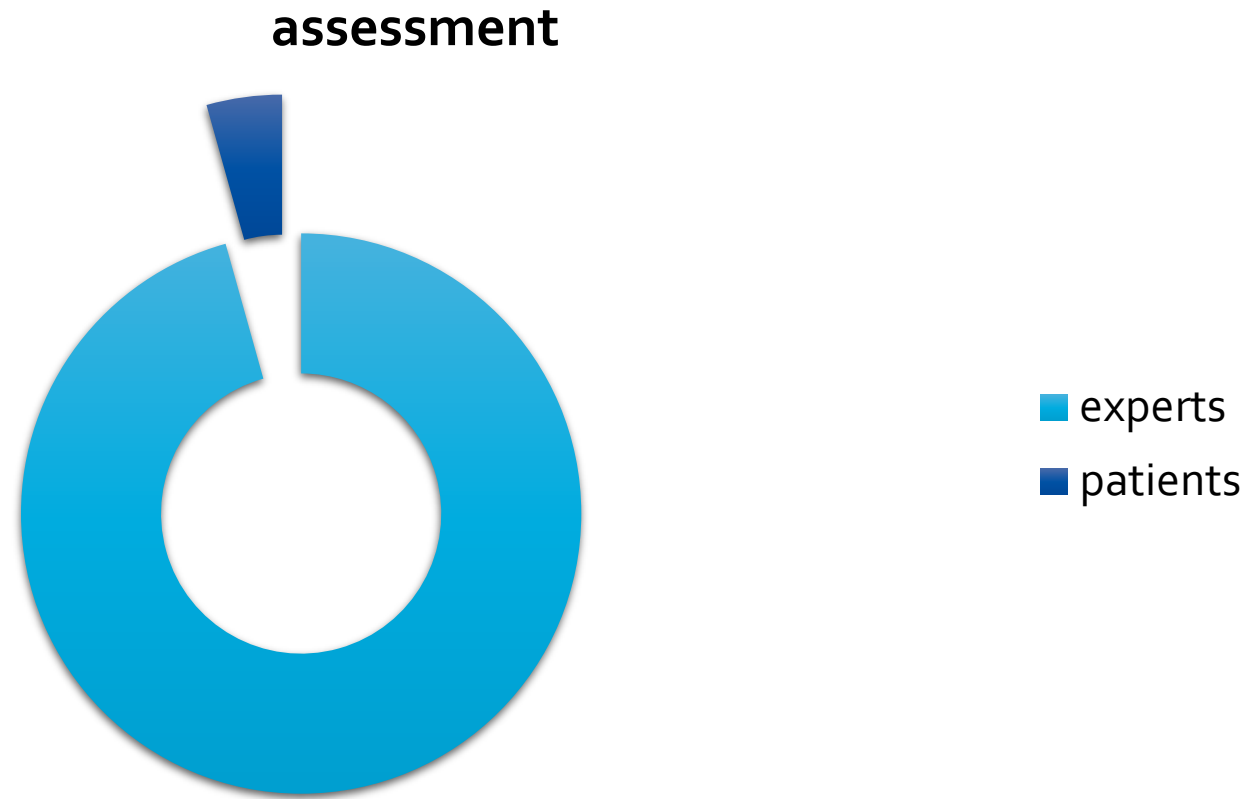


## Assessment



■ HTA experts

2 among many



10 among many

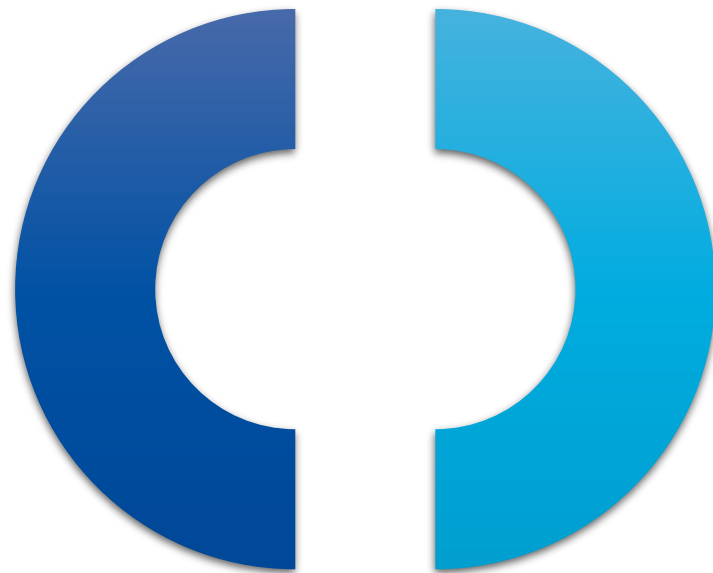
assessment



■ experts  
■ patients

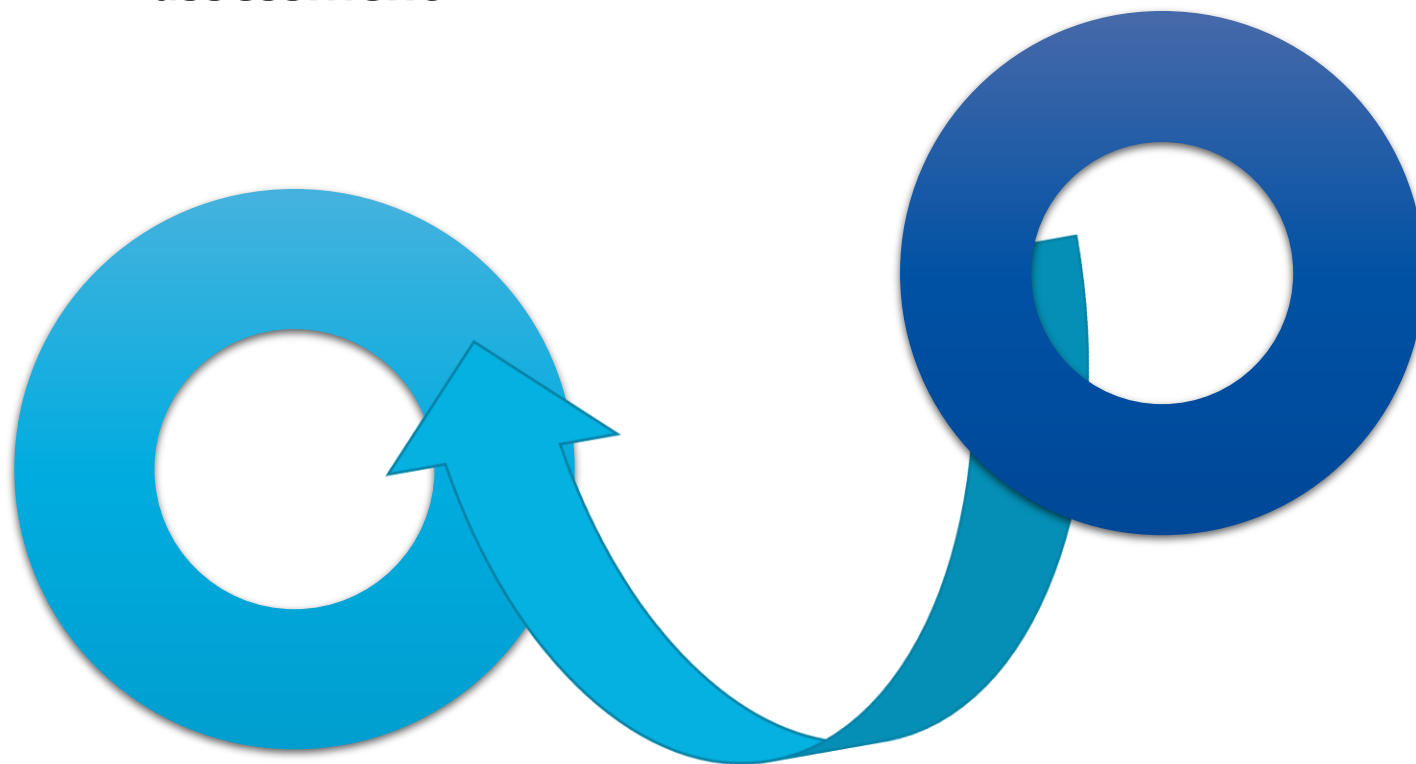
many among many

assessment



- HTA expert
- patients

many in parallel to many  
assessment



## 2 rapporteurs among many



# HOW?

- Questionnaires ? Better use them as a guide for discussions or semi-guided interviews
- Focus groups
  - 8 – 10 patients
  - Clinical trial or not
  - Record discussions, make report
  - Ask what changed in their lives on treatment, all relevant aspects, clinical, societal, Quality of Life...
  - Compare the claim as proposed by the company with your own experience
  - Discuss which patients can benefit the most
  - An HTA expert can be invited to guide the discussion

# How? Patient jury

- 12-15 patients
- Invite an HTA expert or not
- A representative of the company
- Ask them to present the pros/contras
- vote



# what to do

Can scientific research on monkeys be justified?  
First, record where your moral view lies, by marking a cross on the line opposite.

Can scientific research on monkeys be justified?

Definitely 'yes'

Definitely 'no'

Now go through the arguments below. Tick the ones you agree with and put a cross against those that you disagree with. Fill in the 'Your argument' boxes if you wish. Record your views at the end of each section. Then go to the Final Conclusion section.

## MORALS

### facts

- Monkeys are similar to humans in ways that other laboratory animals (such as rodents) are not. For example, they are similar in their brain structure, thinking ability, behaviour and immune system.
- Monkeys can suffer (e.g. pain, stress and anxiety) like humans.
- Monkeys' thinking abilities are not as developed as humans'. For example, there are differences in level of understanding, communication and problem solving.

Is it acceptable to use monkeys in research that would not be allowed on humans?

YES

NO

A1 Humans matter more (to humans) than monkeys do.

A3 Humans and monkeys matter equally.

A3 There are significant differences (e.g. in thinking ability) that can justify treating monkeys differently from humans.

A4 The thinking between humans and monkeys (e.g. ability to understand things) is so similar that monkeys deserve the same treatment as humans.

A5 It is sometimes acceptable to do things to monkeys that we would not be prepared to do to humans, when this is the only way to reduce or prevent human suffering (e.g., when developing and testing new medicines).

A6 We would not do things to monkeys that we are not prepared to do to ourselves. To do so is 'speciesist' (just as treating people of a different race differently from ourselves is racist).

A7 Your argument

A8 Your argument

So, is it acceptable to use monkeys in research that would not be allowed on humans?

Mark a cross on the line below

Definitely 'yes'

Definitely 'no'

## BENEFITS

### facts

- In the UK, 95% of research using monkeys is to develop new medicines; 10% is to gain scientific knowledge, for example about the nervous system. Monkeys are never used to test cosmetics.
- Scientists used monkey experiments in developing (for example) anti-rejection drugs for organ transplants; life-support systems for premature babies; medicines for asthma; and advanced methods of kidney dialysis.
- There is little systematic information on the overall impact of monkey studies on scientific and medical advances.

Are monkey experiments worth it?

YES

NO

B1 New medicines sometimes have to be tested in monkeys before regulators will allow the medicines to be tested in humans and later sold.

B2 The information required by the medicines regulators should be gained in other ways - e.g. using cells, tissues or computer models.

B3 Using monkeys to gain scientific knowledge is acceptable because the knowledge may lead to new treatments.

B4 Using monkeys is only to gain scientific knowledge is acceptable. If experiments on monkeys are to be done at all, they should at least have direct health care benefits.

B5 The laboratory's ethics committee + the government + the funder of the research all have to agree that the likely benefits from the research justify using monkeys.

B6 There is not enough independent assessment of the benefits of and justification for research on monkeys.

B7 Your argument

B8 Your argument

So, do the benefits justify using monkeys in research?

Mark a cross on the line below

Definitely 'yes'

Definitely 'no'

### facts

- UK law says that animals must not be used in research if there is an alternative method that could achieve the objective of the experiment. Monkeys should only be used when no other species of animal will do.
- Alternatives to monkeys could include cells, tissues, computer modelling, and studies using human volunteers.

Is the use of monkeys necessary to achieve the benefits claimed?

YES

NO

C1 Monkeys are valuable in research because they are so similar to humans.

C2 Differences between humans and animals, including monkeys, make many animal tests unreliable.

C3 Scientists accept that animal tests may not show up all the side effects. Monkey experiments can help to weed out unsuitable medicines before they progress to human trials.

C4 Sometimes medicines have side effects that are not seen in monkeys, but which are seen in humans.

C5 Scientists already use alternatives wherever possible, so a ban on the use of monkeys in research would slow medical progress and could drive work abroad to countries where animal welfare conditions are poor.

C6 Banning the use of monkeys in research would force scientists to think of other methods, and so lead to the development of more, and better, alternatives.

C7 Your argument

C8 Your argument

So, do the benefits justify using monkeys in research?

Mark a cross on the line below

Definitely 'yes'

Definitely 'no'

## HARMS

### facts

- UK law on animal experiments requires that the suffering of animals, and the number used, must be minimised. For example, anaesthetics and pain relief are used whenever appropriate.
- Currently, there are no published data on the level of suffering that monkeys actually experience in experiments.

Can the level of harm caused to the monkeys be justified?

YES

NO

D1 Any substantial suffering caused to monkeys must be matched by high benefits (e.g. monkeys given Parkinson's Disease in order to develop new treatments for the disease).

D2 No amount of benefit to humans can outweigh the fact that monkeys regularly die as a result of being kept in cages in laboratories (due to health issues).

D3 The harms are minimised as far as possible, and modern methods of laboratory housing and care of monkeys ensure high standards of welfare.

D4 Monkeys are treated as a means of research in laboratory (e.g. they are well designed and, in some cases, during long journeys to the UK from overseas labs).

D5 Monkeys can be trained to co-operate in experiments (e.g. to present their arm for blood sampling).

D6 Monkeys do not choose to be involved in experiments.

D7 Your argument

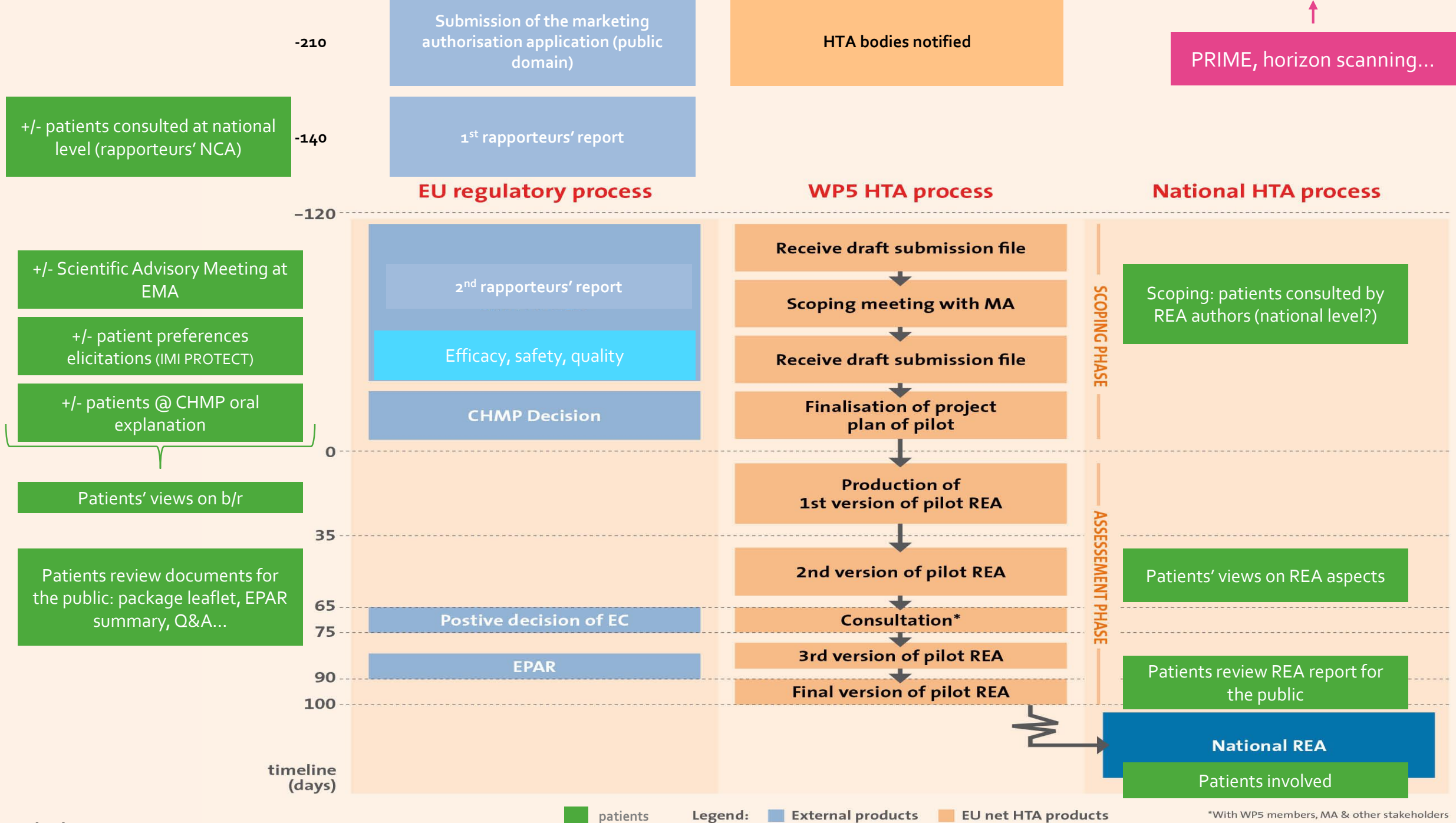
D8 Your argument

So, can the level of harm caused to the monkeys be justified?

Mark a cross on the line below

Definitely 'yes'

Definitely 'no'



Thank you!



*"When we want your opinion,  
we'll give it to you."*