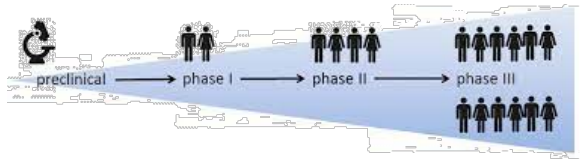
Can we set a Eurordis training on how patients can participate in HTA ?

Building on our knowledge
to best advocate for our treatments

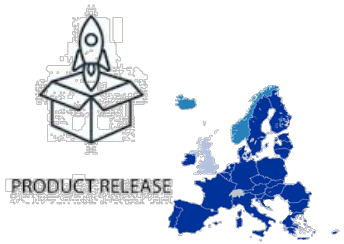
Council of European Federations | November 10th 2021

Matteo Scarabelli | EURORDIS

We all know how a product comes to the market



EMA



We know the process…

CABs

Scientific Advice

Interviews

Committees

Hearings

… when and how we can participate at different stages

Because we are prepared for it!

4 days of Summer School
dedicated to Development and Regulatory

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RARE DISEASES EUROPE

But when it comes to HTA?

Will my treatment be considered "good enough"?
Will it be "worth-to-pay for"?
Would it be ever reimbursed?

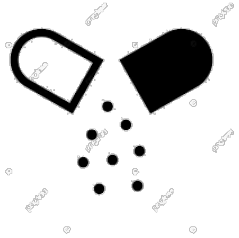
We know the general terms : what this is about
(thanks to 1 day of Summer School)

But patients often lack intelligence on the HTA systems:

- Don't know how to participate
- Cannot prepare, anticipate, or contribute to the decisions

Nitisinone (for Alkaptonuria, AKU)

Existing generic drug
(for Tyrosemia)
Licensed in 2005



Nick Sireau chair of AKU society

Child born with AKU 2003

Eurordis Summer School 2011

Patient-driven consortium
(AKU UK & FR)

2 consultants, 3 hospitals
2 Universities
+ Pharma company (SOBI)

Tested the drug for AKU
(EMA Scientific Advice)

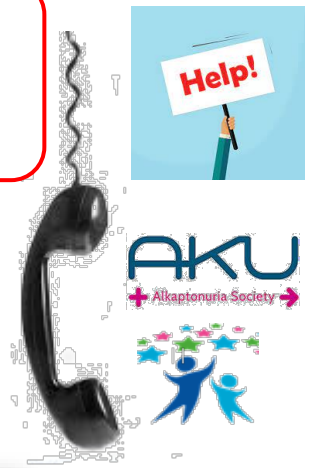
Obtained **funds from EU** H2020
2 studies **with the manufacturer**
(138 participants out of 1800 in EU)

(significant benefit)
Marketing Authorisation

Nitisonone **extended for AKU**
(3 November 2020)

Nitisinone (for Alkaptonuria, AKU)

Then came the time of HTA



July 2021

AKU contacted Eurordis as **the French HTA said there is no additional benefit** in Nitisonone for AKU:

What they realized

AKU patients **didn't know that HAS involves patients** during the time of the assessment (Open Call for Submissions/Questionnaires)

If there no call for patients, one can be opened "on demand", with **request for direct consultation**

Enduring a decision they **didn't see coming**

Didn't know whether an **appeal** was possible and **how to trigger it**

The HTA-genesis of a patient group

Spinraza
May 2017

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Patients endured the decision with no word to say

"All happened in a black box"

SMA Ireland come into existence
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First patient submission
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(BG, NL, LUX, AU, IRL)

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ongoing

SMA Ireland has already **established contact** with IR HTA

They know a Call for Patient Submission will fall **by end of November**

SMA Ireland is already **preparing the dossier,** based on the questions discussed with the HTAs

Is a training on participation in HTA worth it?

1 Few organisations have the chance to **test and fail** three times in five years (like SME Ireland)

2 Even those who **participated once**, don't necessary know how to **navigate the process**

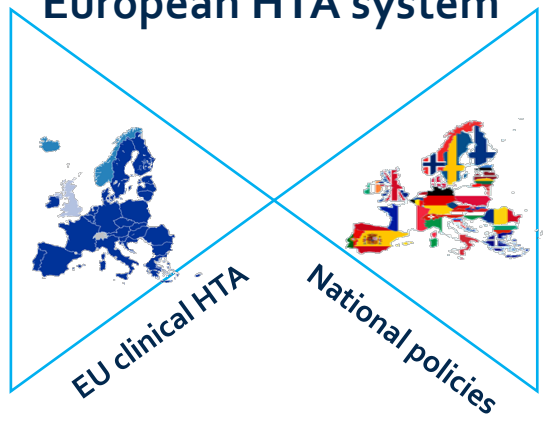
3 CAB members start to deal with **access issues** (HTA and pricing) since the **very first stages** with the companies they talk to

4 Requests (like AKU case) are more and more frequent and will/may concern **all Eurordis members**

Building on the knowledge we already have

to design a training on how to engage country-by-country

To prepare our participation in the future European HTA system



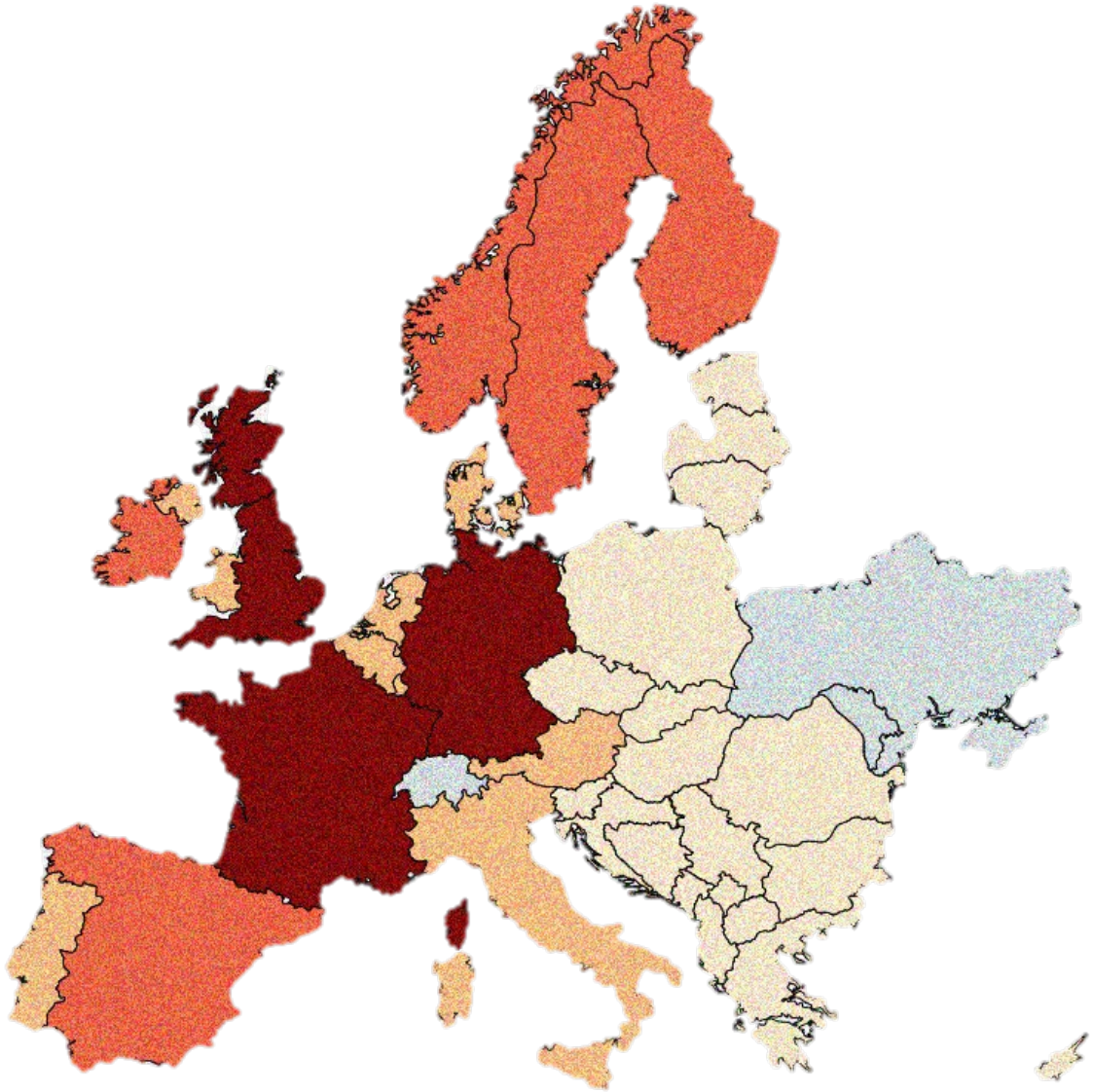
A picture of the situation as we know it

Structured involvement

Involvement but no knowledge

No information about any involvement

No involvement



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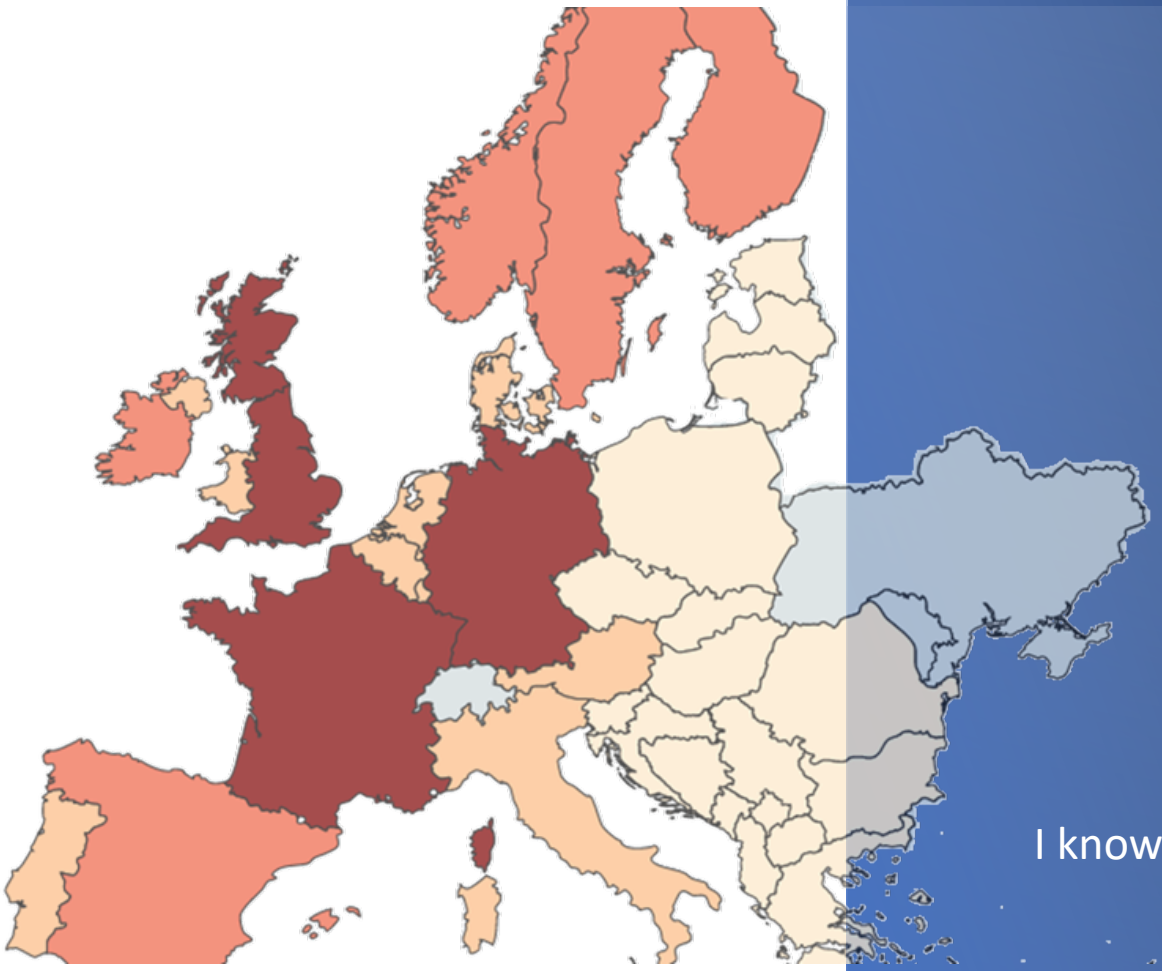
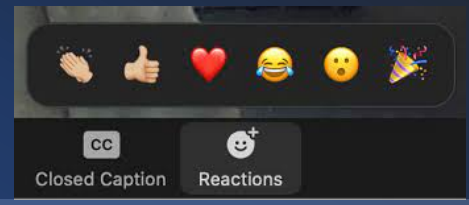
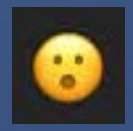


And you… what do you know?



My organisation contributed to HTA in one (or more) country ?

There is no possibility of involvement in my country (or in the countries covered by my federation) ?

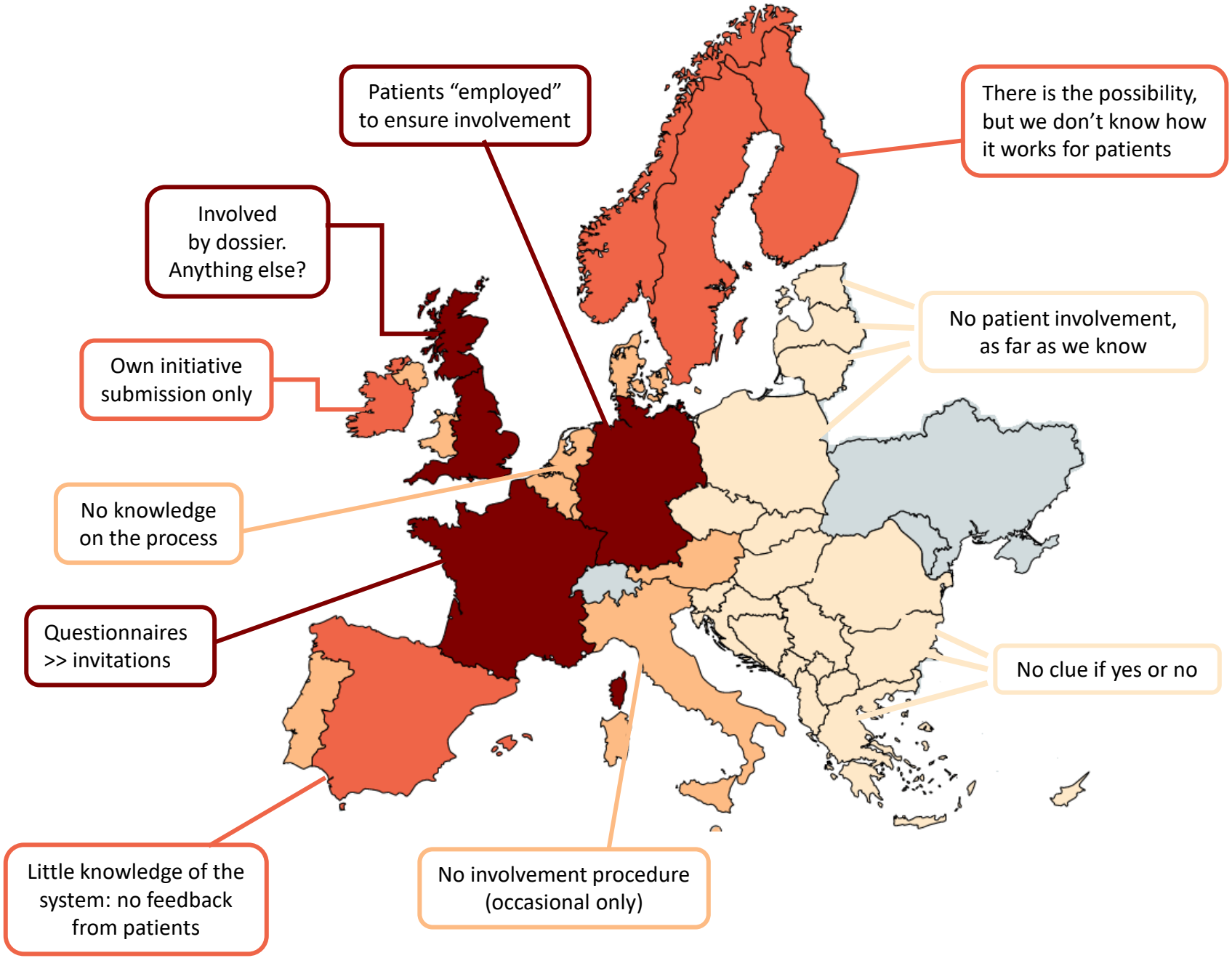


There is this possibility, but I don't know more ?

I know someone in my Fed, that knows someone… who may have knowledge of it ?

What do we know.

Does it reflect your knowledge?



Do you think such training would be worth

?

Share with us the contact of the person within your Federation who can help us making it real and meaningful

To Anja Helm | anja.helm@eurordis.org

To Matteo Scarabelli | matteo.scarabelli@eurordis.org

To François Houÿez | francois.houyez@eurordis.org

Kick-off of

INVITATION

EUnetHTA 21

Fill the gap over 2 years
between end of the EUnetHTA Joint Action 3 (2016-2020) and the European Cooperation to start under the new law:
preparing the guidelines!

Kick-off meeting

To prepare the participation of stakeholders and patients especially in EUnetHTA 21
December 3th
10:30am – 12:00pm
(online)

European Cooperation (HTA Regulation)

In 2024, the HTA Regulation will entry into force and the EU Cooperation will start to operate

Thank you

Matteo Scarabelli | Patient Engagement Manager HTA

matteo.scarabelli@eurordis.org

SCRIPT

- discutere della necessità di un training per tutti i nostri membri
  - Discuss together the need of a training focused on how patients can participate in HTA country by country

- Presenterò due casi dove possiamo indovinare la necessità di tale training
  - I will present a couple of case to illustrate where we see this need coming from

- Cominceremo a vedere dove sono le conoscenze su cui costruire
  - We will start to look at where we can together find the intelligence we need to build this training
- **Faremo un esercizio per vedere se ciò che sappiamo corrisponde a quello che voi sapete**
  - **We will make an exercise on what do we know and whether this matches with your knowledge of the situation in your country or through your federation**

SCRIPT

- We had the summer school
  - we know about clinical research
  - We know about the authorisation, and we can follow what happens, and we know how to participare
- We don’t have a summer school for what happens next (HTA)
  - Will be considered “good enough” and “worth-to-pay”? Would we ever get it reimbursed?
  - We can’t anticipate what might happen: we don’t know what to do, decision comes and we find out when everything is done

SLIDES

- Foto in un angolo della summer school
- Il developer fa il suo sviluppo, va all'EMA: tutti, inclusi I pazienti possono consigliare (CAB, scientific committee, invited as experts), e poi razzo "prodotto lanciato": *conosciamo il processo e* sappiamo come partecipare
- (warning: le cose sono più complesse di così)
- E dopo? Linea su HTA: riassunto del processo, comparative effectiveness and money: non c'é conoscenza/partecipazione
- Esempio Nitisinone [non sapevano e sono rimasti fregati]
  - Obtained 6Mo€ research funds from EU Horizon programme
  - Run 2 studies for repurposing of Nitisnone for AKU
  - Partnering with the Sobi, the holder of the drug (150+ trial participants)
  - The product obtained a marketing authorisation (extension of indication) for AKU
- Esempio SMA: hanno dovuto imparare sulla loro pelle

SCRIPT

- Allora forse, c'é bisogno di un training in HTA nei vari paesi

- I CABs ce lo chiedono, vogliono preparsi (con il manufacturer: quali sono gli elementi HTA important, il prezzo), e i nostri membri anche ci chiedono come
- Ma é vero che non sappiamo niente? **Ma non sappiamo abbastanza: possiamo creare un training**
- Esempio SMA: hanno imparato sulla loro pelle
- [visual dei paesi: cosa sappiamo]

- Possiamo creare un training: partendo da quello che sappiamo
- [coinvolgere sul visual dei paesi]: and you, what do you know?
- Share the contacts

SLIDES

- Fare vedere chi ci chiede
- ESEMPIO SMA: 3 steps, passaggi con diversa partecipazione, fallimenti e successi, ma ancora partecipano in modo esteriore
- **Altra slide: Possiamo create un training per HTA: primo abbozzo di visual**
- Altra slide: é vero che non sappiamo niente? [visual dei paesi: cosa sappiamo]: creiamo un training da quello che sappiamo
- Visual per coinvolgere loro

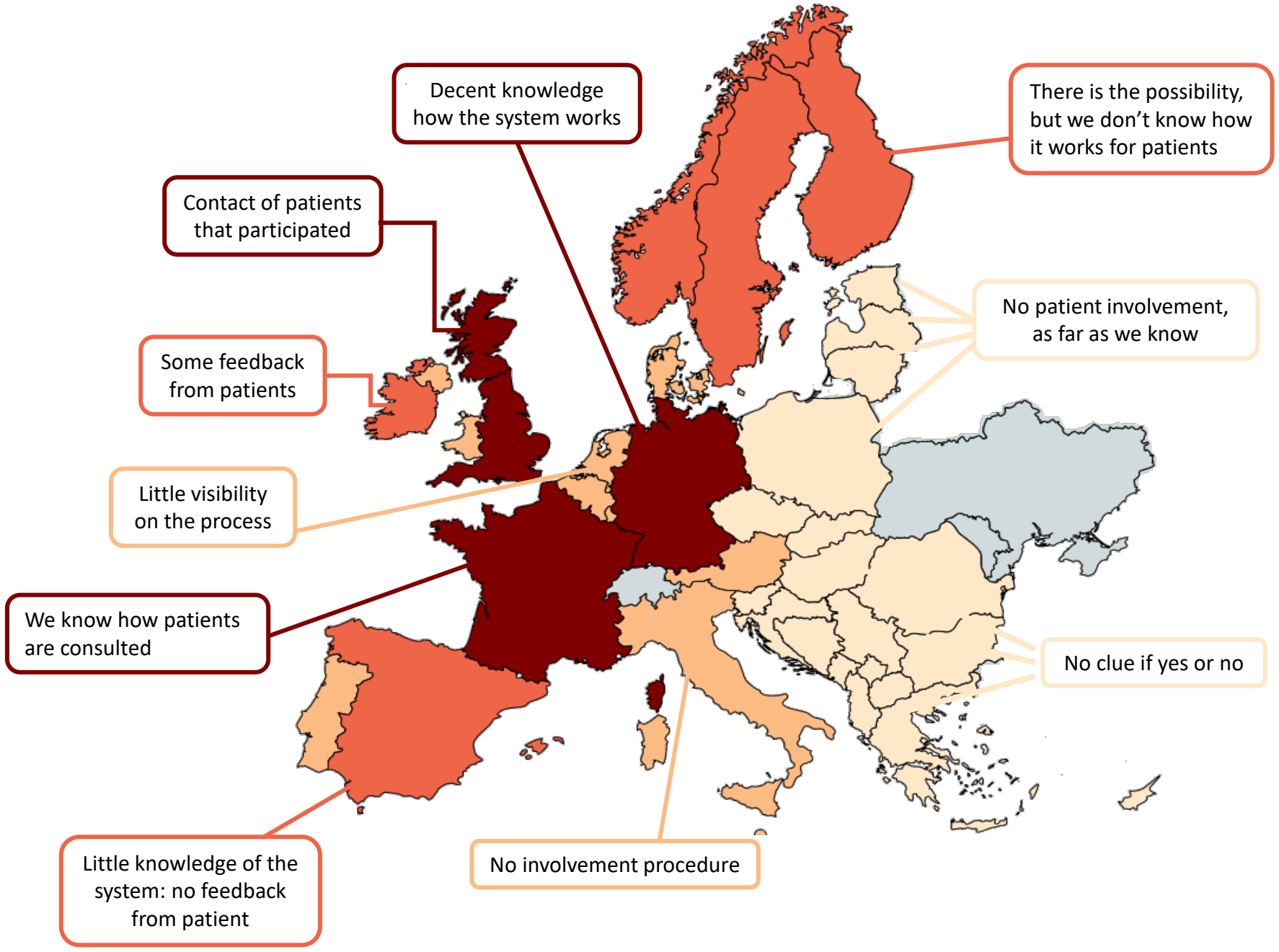
ESEMPI

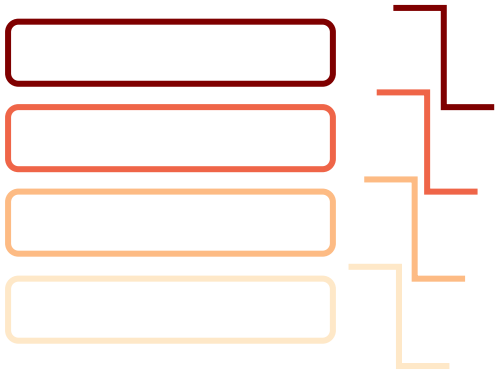
Gli irlandesi du SMA si sono trovati a fare questo percorso da soli, ci hanno messo 5 anni e 3 prodotti: se non avete la fortuna di avere questo tempo e questa frequenza di sviluppo. Meglio essere preparati
Quelli di nitisinone, si sono trovati con la decision della francia, senza sapere che potevano contrivuire. Hanno avuto una decision negativa e stanno cercando di rimediare Zynteglo: il manufacturer ha lasciato il mercato

- Spinraza EC 30 may 2017
  - SMA Ireland came after the first refusal of spinraza in Ireland Dec 2017 - -,5 months
- 2019 (confidential price negotiations: green light) 2 years later
- Zolgensma EC 18 may 2020
  - Not recommended in April 2021 (11 months)
    - Beneluxa October 2021
  - They were consulted (the issue was the price)
- Risdiplam EC 26 march 2021
  - They know who reach out to to be contacted: what questions stand?
  - They know a call will fall before end of november
  - They are already preparing a patient submission

SCRIPT

- Finale: metteteci in contatto con chiunque consociate
- Per fare un training su HTA nei vari paesi: solo la conoscenza diretta può dare il risultato
- Finale: cooperazione EU
  - Dovremo costruire un modello nuovo:
  - ci saranno due livelli integrati: a) EU come EMA (dove sarà più facile essere riconosciuti), e b) a livello nazionale (che potrà fare di più, pushed up verso l'alto dal Sistema UE)
  - Sarà più facile perché


Decent knowledge how the system works
There is the possibility, but we don't know how it works for patients
Contact of patients that participated
No patient involvement, as far as we know
Some feedback from patients
Little visibility on the process
We know how patients are consulted
No clue if yes or no
Little knowledge of the system: no feedback from patient
No involvement procedure



Decent knowledge

Little knowledge

No information

No involvement

Patient representatives are consulted

Little knowledge:
no feedback from patients

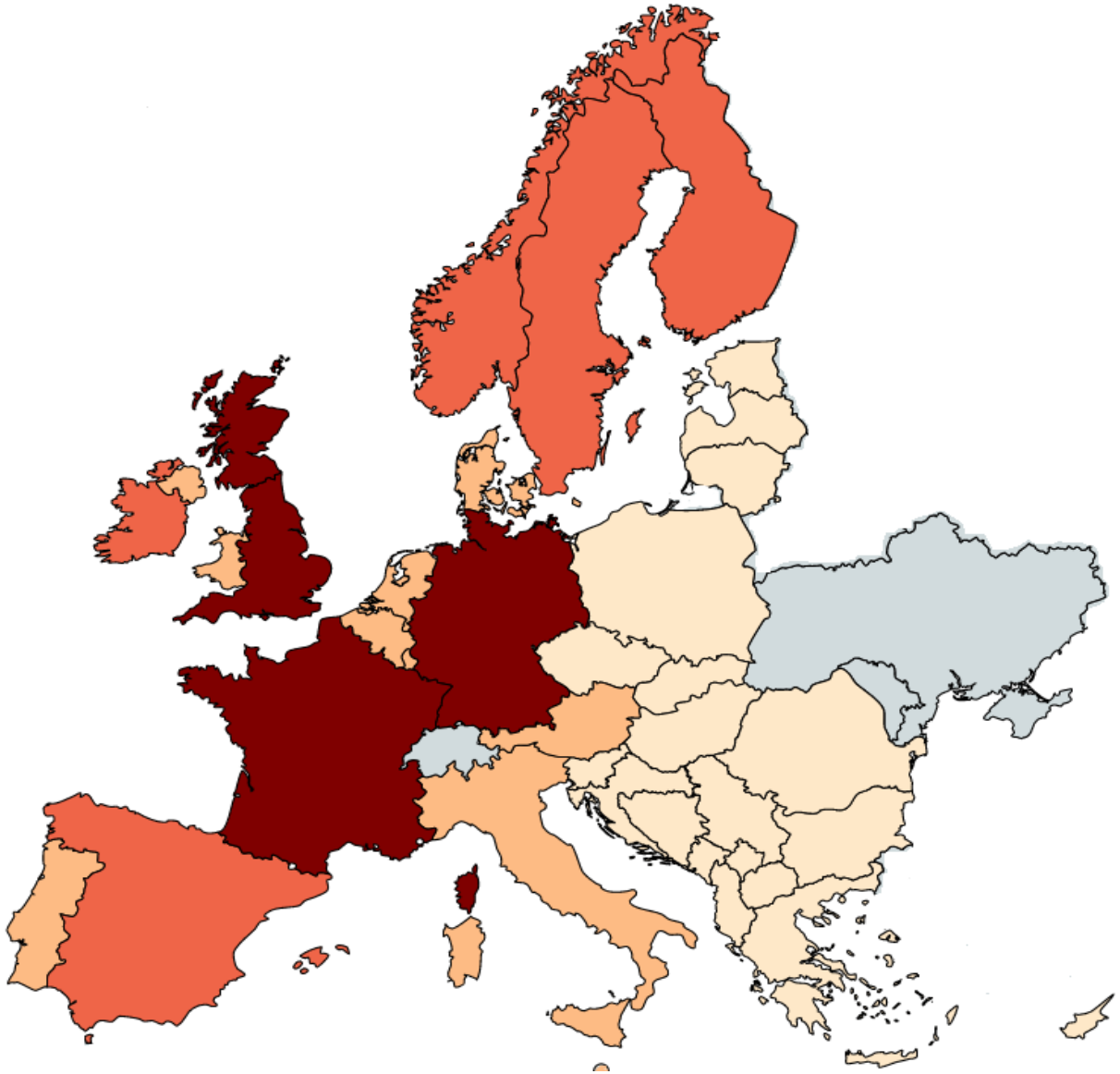
No patient involvement, as far as we know

No clue if yes or no

Do we know enough?

Decent knowledge how process works

How patient representatives are consulted



Each country looks for the best treatment (effectiveness) to pay for (costs)

HTA don't look only at the therapy's efficacy…

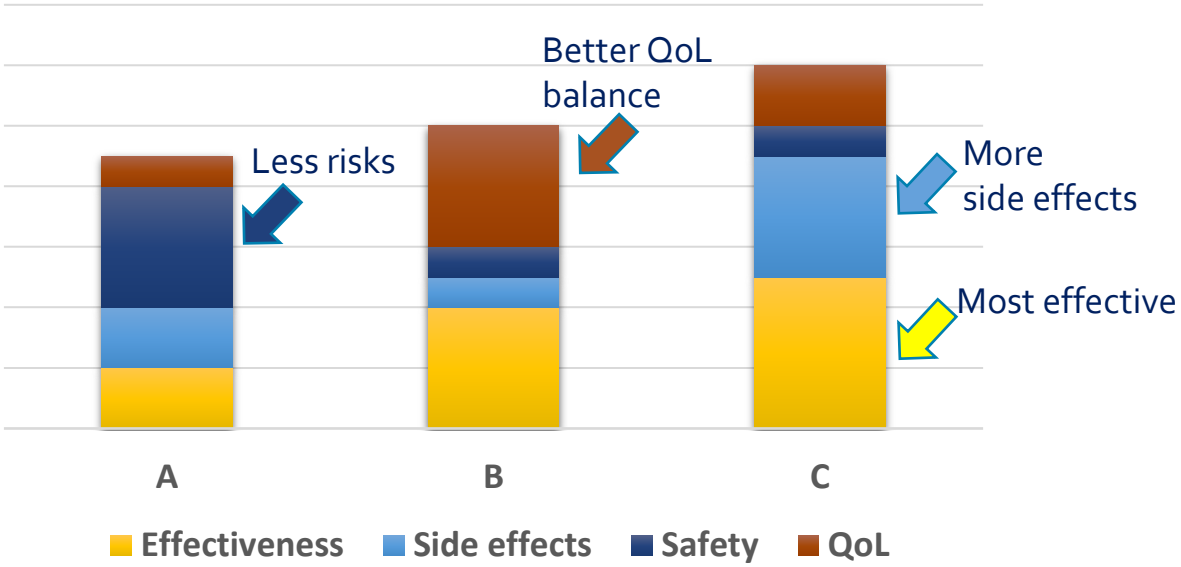
VS

1. It compares to all the other options to find the best solution

2. for the most appropriate expenses

Each country looks for the best treatment (effectiveness) to pay for (costs)

vs



Patients have **direct knowledge**:

- which side effects are manageable
- what's the impact of a certain degree of effectiveness
- the qualitative meaning of QoL improvements

Patients **may prefer** the treatment less effectives with less side effects, or the most effective as the side effects are manageable

→ That's why patients have their rightful place in HTA procedures and discussions

Diff countries, Diff HC systems:
Diff ways of allocating ressources -> Diff ways of doing HTA

European Countries have different HTA Institutions

which reflects different ways of dealing with patients involvement

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AIFA AGENZIA ITALIANA DEL FARMACO

Agència de Qualitat i Avaluació Sanitàries de Catalunya

Gemeinsamer Bundesausschuss

NHS National Institute for Health and Clinical Excellence

HAS HAUTE AUTORITÉ DE SANTÉ

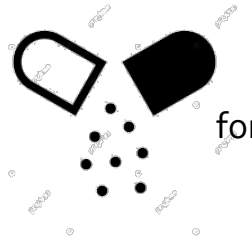
Zorginstituut Nederland

NICE

KCE Belgian Health Care Knowledge Centre

eunethta

Nitisinone (for Alkaptonuria, AKU)



Existing generic drug
for Hereditary tyrosinaemia type 1

patient-driven consortium (AKU UK & FR)

2 consultants, 3 hospitals
2 Universities
+ Pharma company (SOBI)

Tested an existing drug
(EMA Scientific Advice)

1° study **funded by EU** Horizon 2020
2° **with the manufacturer**
(138 participants out of 1800 in EU)

(significant benefit)
Marketing Authorisation

Nitisonone – already authorised for Tyrosemia – **extended for AKU (3 November 2020)**

Nick Sireau
chair of AKU society

Child born with AKU 2003

Eurordis Summer School 2011

July 2021

AKU contacted Eurordis as **the French HTA denied added-value** to Nitisonone for AKU: stick to price of the off-label generic

The French HTA didn't get the benefit demonstration of the development as advised by the EMA

AKU society didn't know that

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Nitisinone (for Alkaptonuria, AKU)

Then came the time of HTA

July 2021

AKU contacted Eurordis as **the French HTA said there is no additional benefit** in Nitisonone for AKU:

1. French HTA probably misunderstood the efficacy as measured in the study designed following the EMA Scientific Advice

2. "no additional benefit" means that France cannot pay the new drug more than the existing generic

3. All that despite the treatment existed only off-label

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2. If there no call for patients, that can be opened "on demand", with **request for direct consultation**

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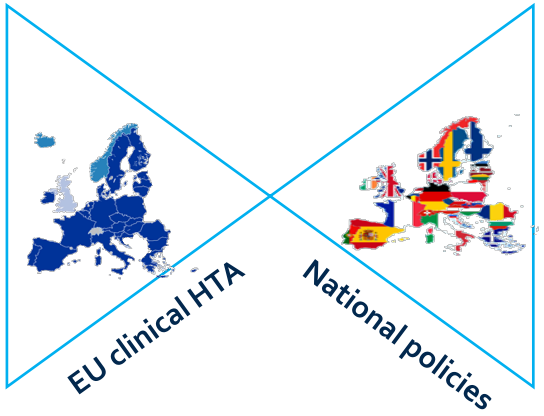

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