LE RÔLE
DES REPRÉSENTANTS DES PATIENTS

Comment les patients
participent à l'évaluation des technologies de santé

Journée Nationale des Innovations Hospitalières

Paris, 24 Juin 2019

Matteo Scarabelli

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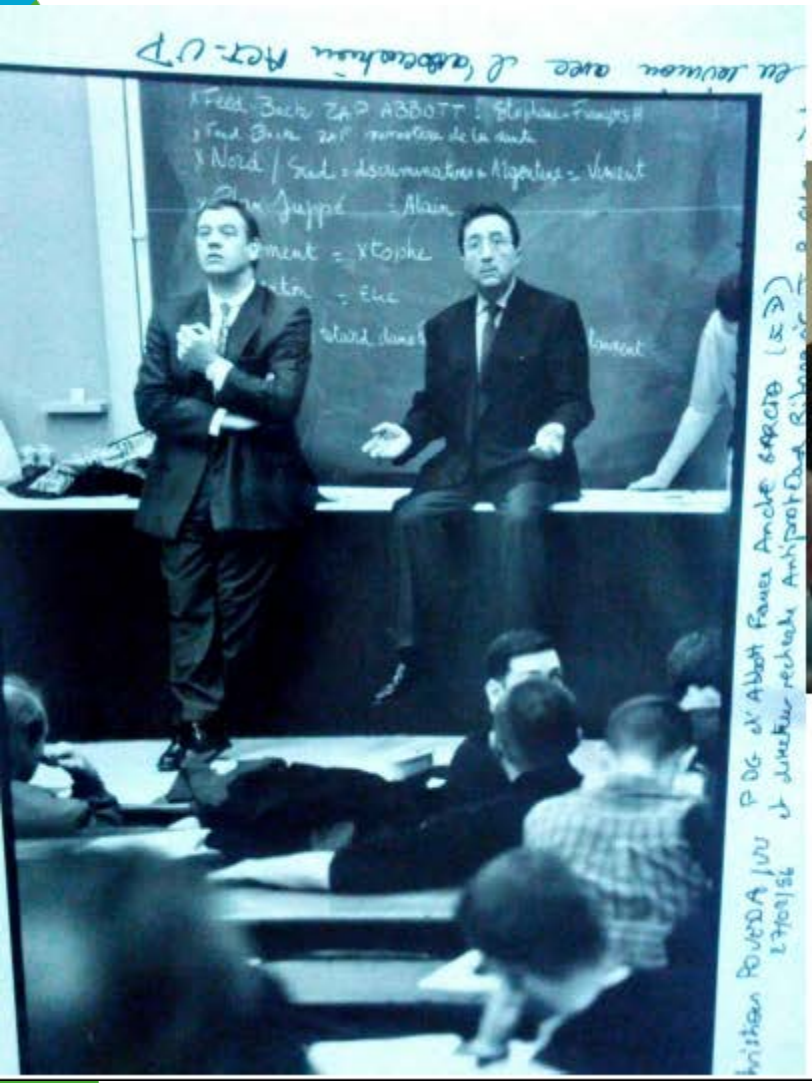
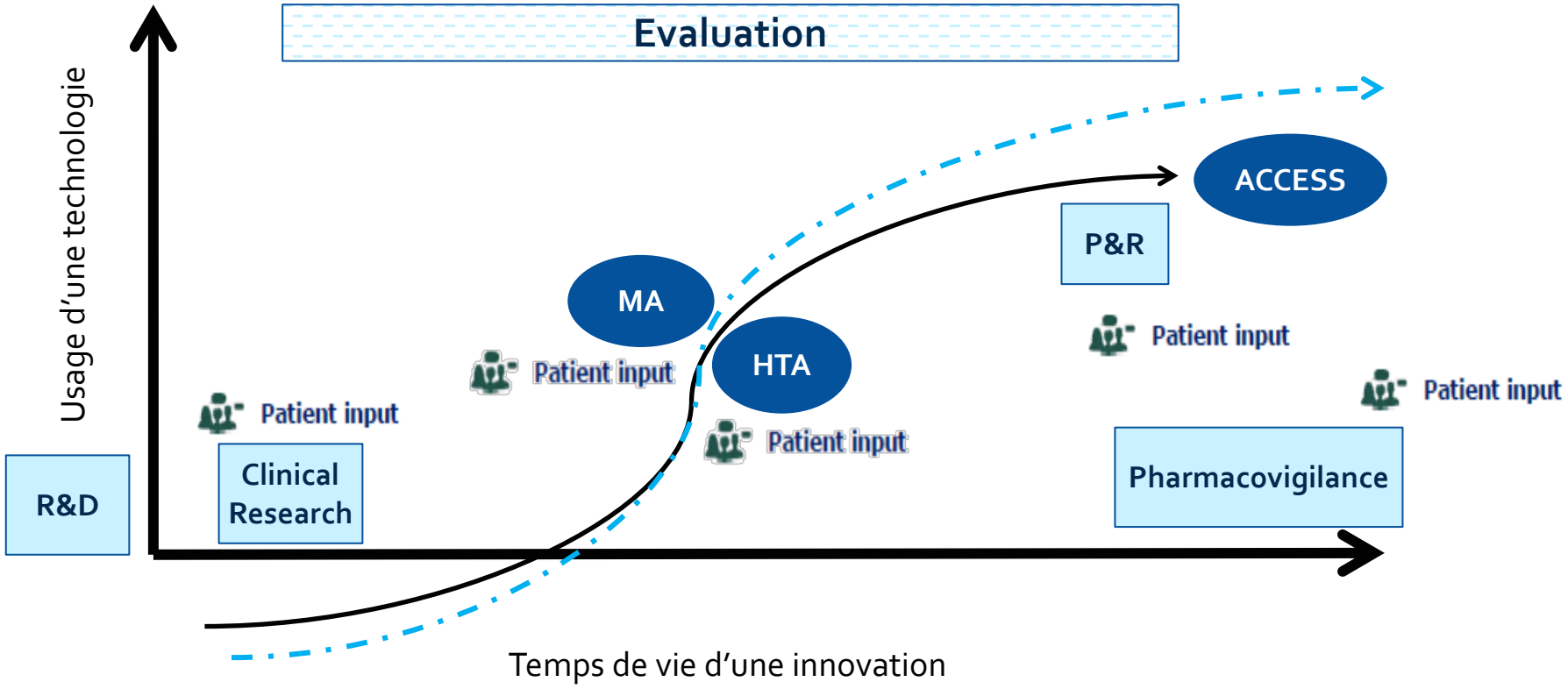
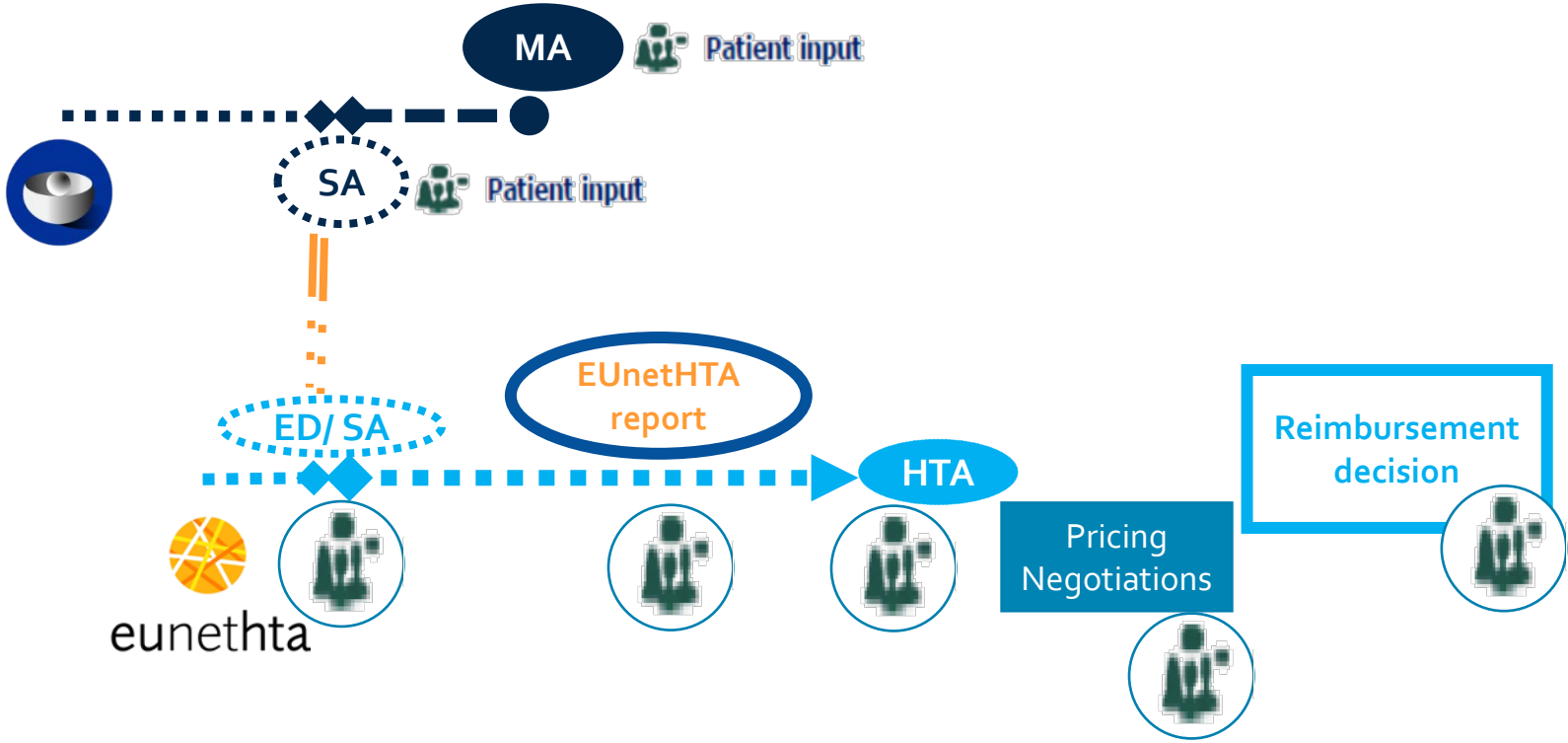
EURORDIS' Community Advisory Boards (CABs)



Health Technology Life-cycle




MA
Patient input
SA
Patient input
ED/ SA
EUnetHTA report
HTA
Pricing Negotiations
Reimbursement decision
eunethta

Idebenone (RAXONE) pour LHON
Scottisch Medicines Consortium

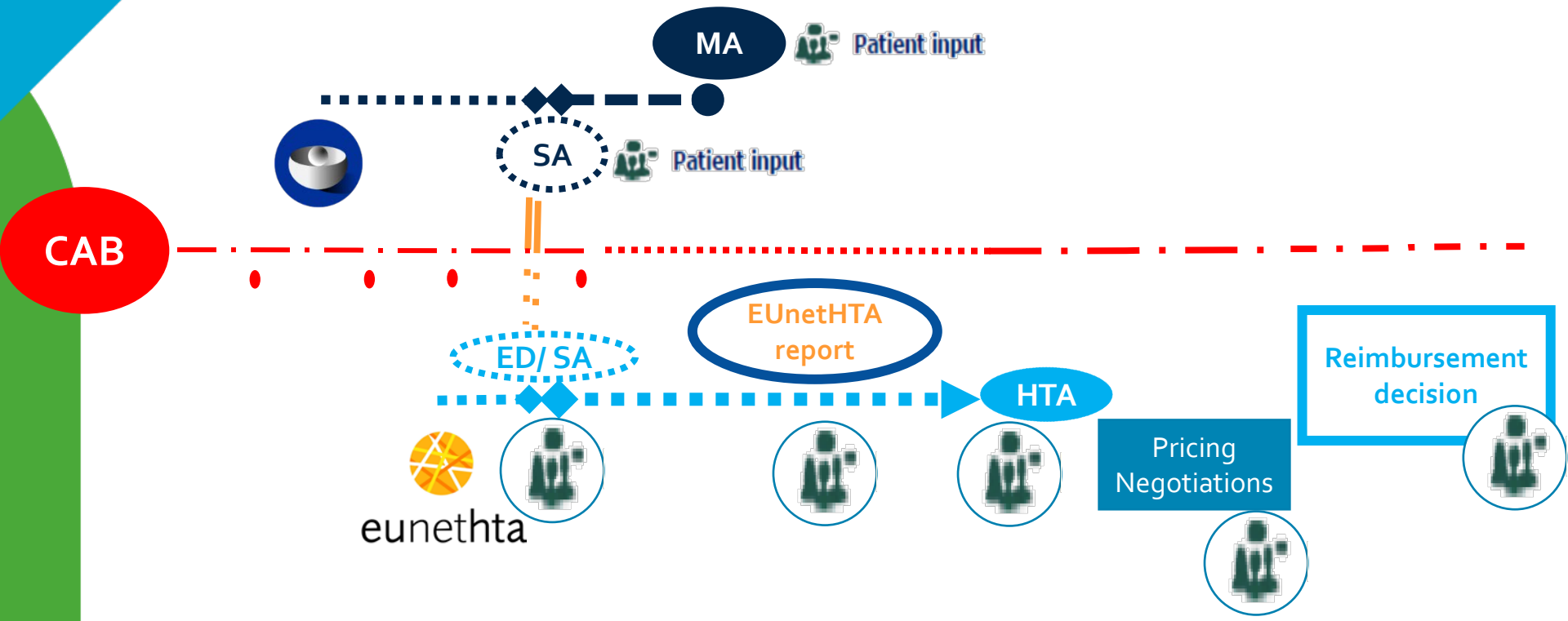
- **Indication**: déficience visuelle en adulte et adolescents avec neuropathie optique de **Leber** (et qui ne sont pas encore aveugles)
  - 300mg trois fois par jour (comprimés 150mg)
  - Censé prévenir et faire régresser la perte de vue

Résultat de l'évaluation:

- Les patients ont souligné l'importance d'approuver le premier traitement disponible, dont les bénéfice était indiqué dans l'essai
- Les même patients ont souligné que pour le bénéfice effectivement apporté, le médicament ne valait pas le prix propose par le laboratoire

L'avis des patients a permis à l'agence Ecossaise de négocier un prix à la baisse

(avec restriction d'indication dans le temps faute d'évidence plus robuste à fournir)



COMMUNITY ADVISORY BOARD (GROUPE DES PATIENTS EXPERTS)

- *Un cadre guidé par les patients pour interagir avec les développeur*
- *Pour soulever (et répondre) à toute question importante du début et le long du développement*

impact

- CAB meeting with Agouron, 1996 | Nelfinavir, new HIV protease inhibitor | CAB cost: $50,000 | RoI: >10,000 x

- Phase II results, study 510

1.5 g/day: effective
10% loose stools (grade 2)

2.25 g: more effective

3.00 g: even more effective
30% loose stools

- Agouron and clinicians chose 2.25 and 3.00 g or more for phase III

CAB disagreed
1.5 and 2.25 preferred
Finally 2.25 and 2.5 in phase III

#unique patient perspective

- Authorised in 1998 at 2.25 or 2.5/day

Children could be treated for 1st time, well tolerated. Demand > offer

Patients were staying longer on treatment as expected. Dose adjustment ++, several strengths

Surplus of 500 Mio$ sales 1st year compared to Agouron's estimates

impact

- Some CAB discussions about rare diseases

• Systemic sclerosis

Large capsules difficult to swallow

Different formulation? Dose adjustment? To be taken with some gel?

#unique patient perspective

• Tuberculosis (TB)

Tuberculin test at inclusion

- 1 day at trial site
- Again after 48h to read results

→ Low recruitment

To send someone at home to read result (GP, community care trial)

#good sense

• Cystic Fibrosis

Pricing policy for new products

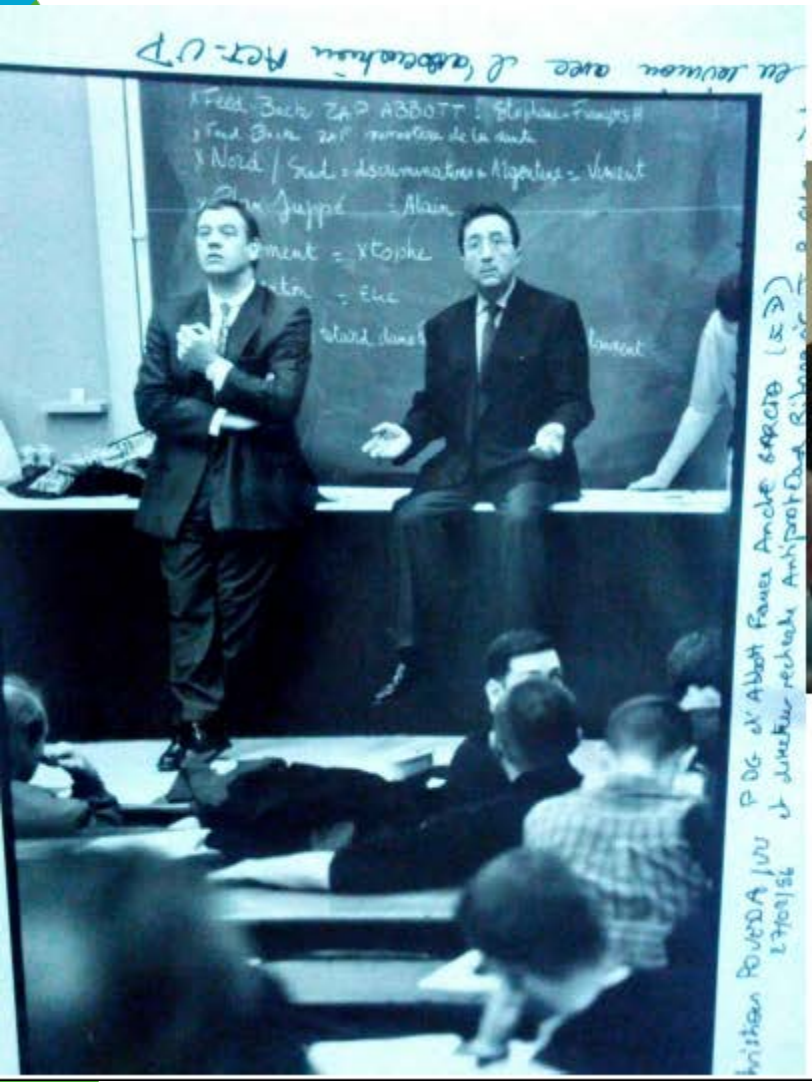
Training with a health economist on the concept of fair pricing / objective measure of price negotiation margin

#homework

QUELQUES ELEMENTS

- Comment les patients entre en contact avec les évaluateurs?
- Faire partie d'un réseau (organisation)
- Plus tôt les patients sont impliqués, plus haute les chances de succès
- Pertinence des contributions (et des questions).
- Aucune limites aux sujets à discuter avec les patients
- Feedback

EURORDIS' Community Advisory Boards (CABs)



Questions?

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RARE DISEASES EUROPE

Back-up slides

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RARE DISEASES EUROPE

EURORDIS funds system

European Medicines Agency

BOARD

Product Information

PRE-SUBMISSION

AUTHORISATION

POST-AUTHORISATION

Designation

Scientific Advice

Paediatric Plan

COMP

CAT

SAWP

PDCO

CHMP

S.A.G

PRAC

PCWP (Associations)

EURORDIS Open Academy

EURORDIS WINTER SCHOOL
on Scientific Innovation & Translational Research
F2F 11-15 March 2019, Paris
30 participants, patient advocates

EURORDIS LEADERSHIP SCHOOL
on Healthcare & Research - NEW
F2F 26-27 November 2019, Barcelona
60 participants, European Patient Advocacy Groups (ePAGs)

EURORDIS SUMMER SCHOOL
on Medicines Research & Development
F2F 10-14 June 2019, Barcelona
40 participants, patient advocates & researchers

EURORDIS DIGITAL SCHOOL
on Social & Digital Media - NEW
F2F 8-9 October 2019, Gothenburg
25 participants, patient advocates

RareConnect

RareConnect.org
A EURORDIS INITIATIVE

- Launched in 2010
- Free-access online rare disease patient communities
- 140+ communities in partnership with 889 patient groups
- Supported by 3 full-time community managers and 407 volunteer moderators
- Translators offer free translations between 6 languages (English, French, German, Italian, Spanish, Portuguese, Russian)
- www.rareconnect.org
- Questions on starting a community?
  Contact: team@rareconnect.org

EUROPEAN REFERENCE NETWORKS

FOR RARE, LOW-PREVALENCE AND COMPLEX DISEASES

European Commission | Health

Connect clinical experts by groups of rare diseases to ensure knowledge sharing and care coordination across Europe.

- Digital consultations
- Patients representatives working with clinicians

EURORDIS RARE DISEASES EUROPE

24 European Reference Networks (ERNs)

ERNs	ePAGs
ERN BOND	Rare Bone dis.
ERN CRANIO	Cranofacial anomalies
Endo-ERN	Endocrine dis.
ERN EpiCARE	Rare Epilepsies
ERKNet	Renal diseases
ERN RND	Neurological dis.
ERNICA	GastroIntestinal dis.
ERN LUNG	Pulmonary dis.
ERN Skin	Skin diseases
ERN EURACAN	Solid Tumours
ERN EuroBloodNet	Haemato. Diseases & malignancies
ERN EURO-NMD	Neuromuscular
ERN EYE	Eye diseases

ERNs	ePAGs
ERN GENTURIS	Genetic Tumour Risk Syndromes
ERN GUARD-HEART	Rare Cardiac
ERN ITHACA	Congenital malformations/ intellect. disabilities
MetabERN	Metabolic disorders
ERN PaedCan	Paediatric cancers
ERN RARE-LIVER	Hepatic disorders
ERN ReCONNET	Connective Tissue / Musculoskeletal dis
ERN RITA	Immunodeficiency, Autoinflammatory / Autoimmune dis.
VASCERN	Mutli-systemic vascular dis.
UROGENITAL	Urogenital diseases
TRANSPLANT-CHILD	Paediatric transplantation

Mammaprint (*genomic test for early-stage breast cancer*) EUnetHTA

- **Indication**: test génétique sur un échantillon de cellules cancérogènes mesurant le risque des nouvelles métastases

Révision du projet d'évaluation (contributions des patients):

- Est-il pértinent d'inclure parmi les endpoints la survie globale ou la DMFS plutôt que privilégier la DFS?
- Pour prévenir et éviter la chimiothérapie nous sommes prêtes à accepter une plus grande incertitude quant à l'efficace
- Un article significatif n'apparait pas dans la littérature. Pourquoi?

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RARE DISEASES EUROPE

A NEW COOPERATION FOR A TRANSPARENT HTA

How patients see the EC Proposal for a Regulation on HTA Cooperation in EU

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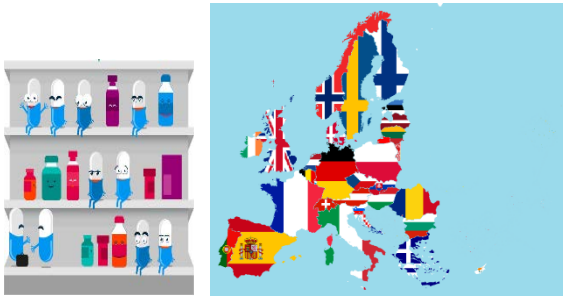
TODAY

Every year, new medicinal products and medical devices receive marketing authorisation or CE mark

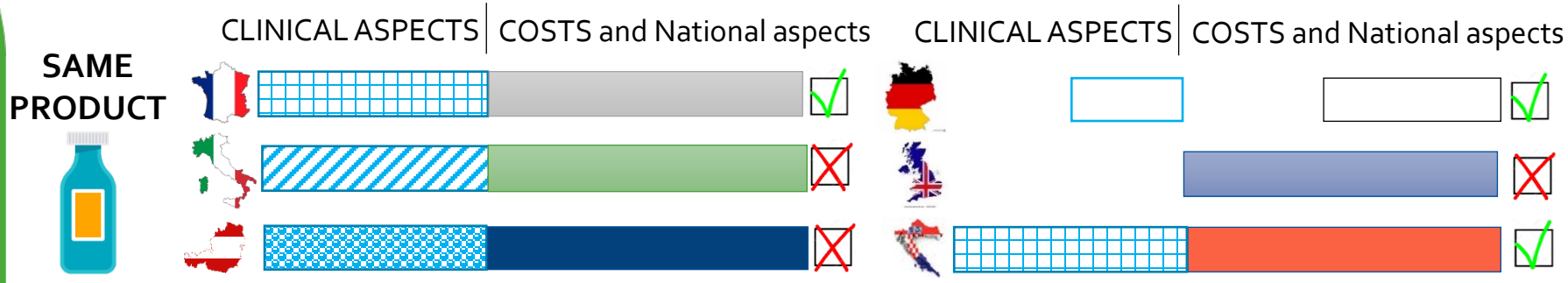


For every single technology, each country should decide about coverage and reimbursement

Each country carry out HTA to decide how much it is worth, on the basis of:

- CLINICAL ASPECTS and EVIDENCE (Relative Effectiveness Assessment REA)
- COSTS and National-related aspects

TODAY

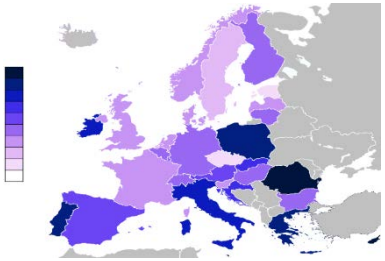


- CACOPHONY IN THE ASSESSMENT OF THE SAME CLINICAL ASPECTS
- THE RATIONALE BEHIND THE FINAL DECISION ISN'T CLEARLY UNDERSTANDABLE

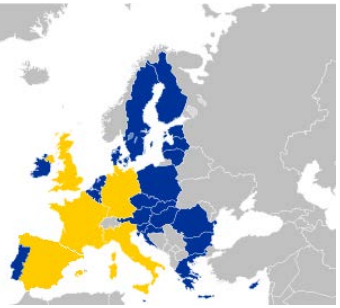
DUPLICATION OF WORK

RAISING COSTS

INEQUALITIES



Companies address mainly the biggest countries



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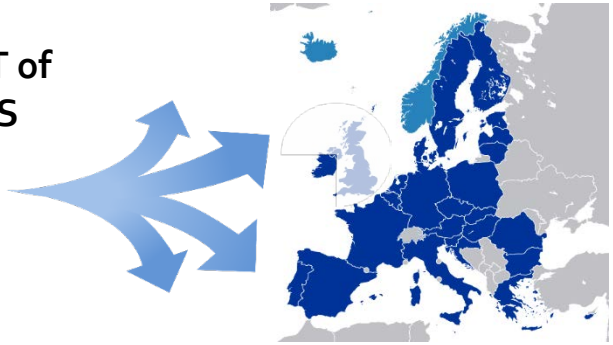
TOMORROW:
proposed Regulation for a permanent HTA cooperation

JOINT ASSESSMENT of CLINICAL ASPECTS



MANDATORY UPTAKE:

obligation to re-use the joint clinical assessment at national level

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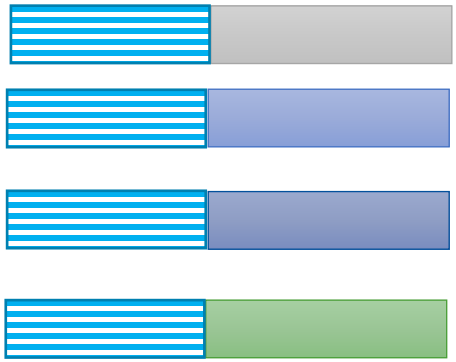
NATIONAL LEVEL:

complete the evaluation with national-related elements

JOINT HTA ✚ COSTS and National aspects



NATIONAL DECISION MAKING on pricing and reimbursement

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