Access: the key to successful European commercialisation – rising to the challenge(s)

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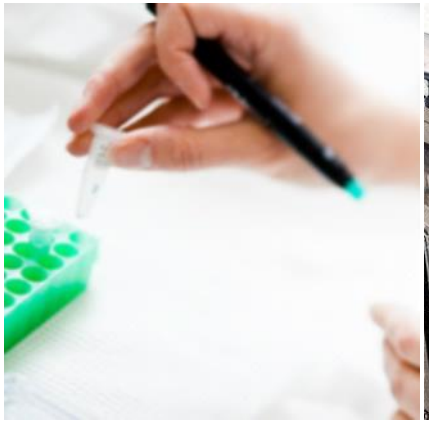
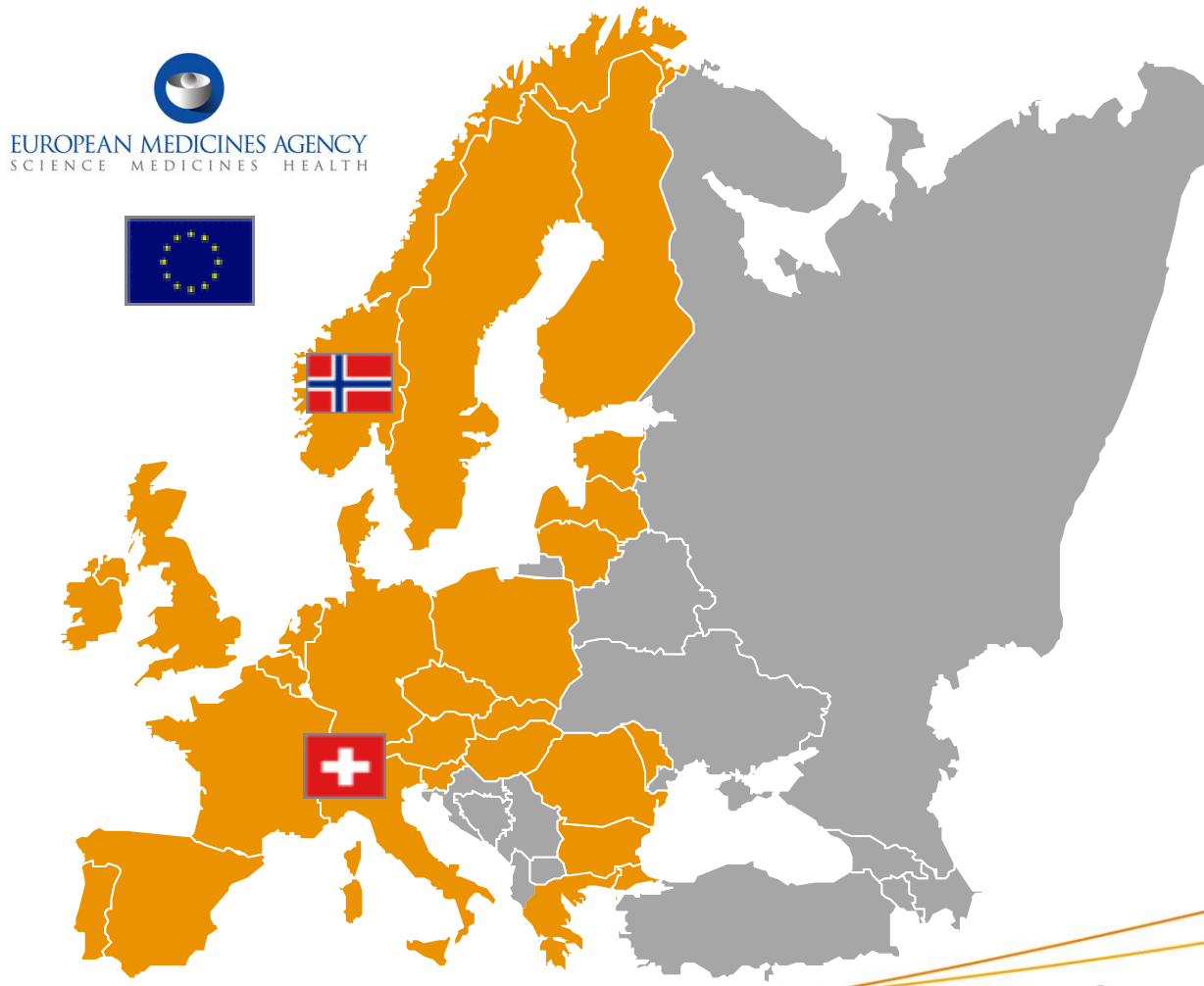



World Orphan Drug Congress USA | Washington DC | 24 April 2015

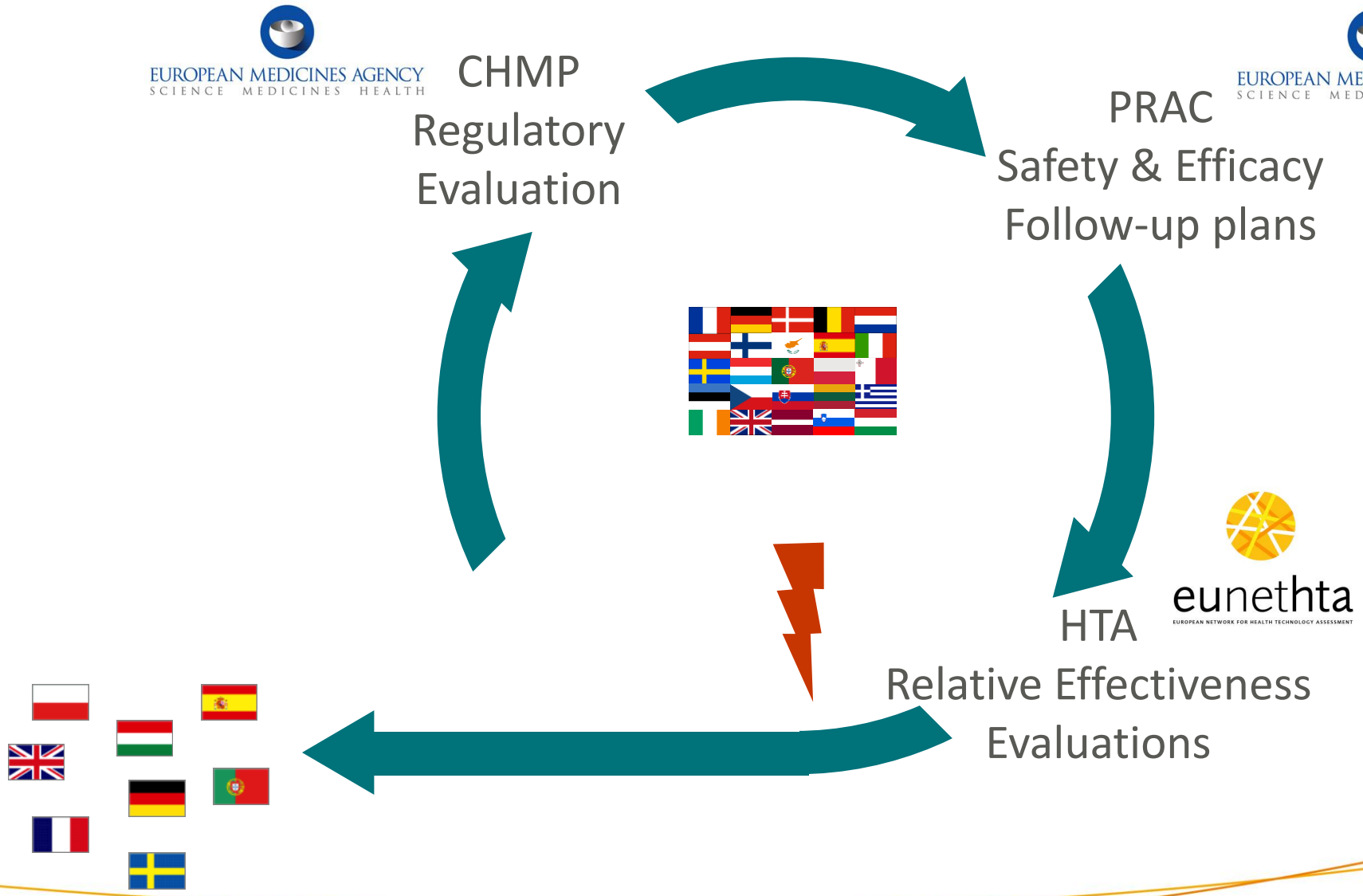
Pioneer in Rare Diseases

Europe: a large, diverse set of systems

- EU 28 countries: 500+ million people
- Non EU countries
- 1 EU Marketing Authorisation…
- …28+ national, regional, local systems for
  - HTA evaluations
  - Individual Pricing & Reimbursement decisions
- Different healthcare traditions & systems
- Increasing access issues?



Increasing collaboration, impact on end-result?



Orphan drugs face amplified challenges

- Regulatory risk:benefit – unmet medical need
- Uncertainty of evidence – strength, statistical significance
- New and emerging technologies – “taking it on trust”?
- Techniques may be part of treatment – limited centres

BUT

- Acknowledgement of shared challenges
- Systematic inclusion of stakeholders
- Willingness to collaborate to find solutions
- Opportunities at every step of the way
- Charting a path

Right dialogue at the right time:
Where we are will determine what could be useful

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- Innovation Task Force
- Business Pipeline Review
- Adaptive Pathways
- The future…?

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Patients | Payers | Treatment guidelines

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European Medicines Agency
licensing pilot project

"This is not so much an adaptive system as an adaptive mind-set"

"It's a <u>prospective plan</u>, <u>agreed with all stakeholders</u>, to <u>progressively reduce uncertainty</u> + <u>grant access</u> to needed therapies"

The European Medicines Agency (EMA) is inv
adaptive licensing pilot project. Companies w
pilot are requested to submit ongoing medici
consideration as prospective pilot cases.

A framework to guide discussions of individual pilot studies has been published.

The adaptive licensing approach, sometimes called staggered approval or progressive

- Leaflets
- RSS feeds
- Newsletters
- Social media

To initiate a pilot case:
adaptivelicensing@ema.europa.eu

Where we are will determine what could be useful

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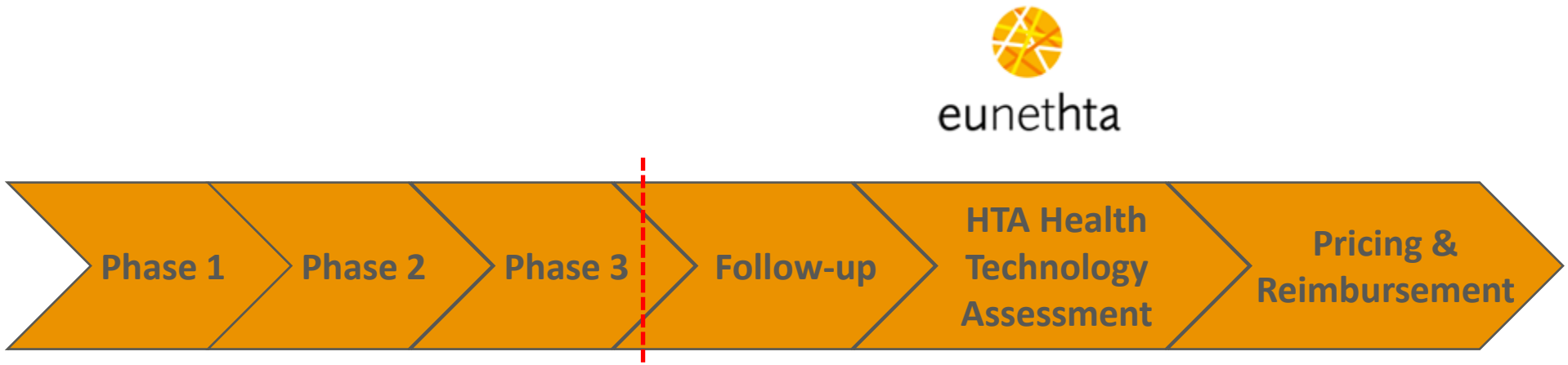
Parallel Regulatory + HTA Scientific Advice

- Individual countries
- European Medicines Agency (EMA) “Parallel” Scientific Advice
  - Since 2010
  - 25+ procedures already by 2013
  - Well-established opportunity – guidance, best-practice
- SEED – Shaping European Early Dialogues for Health Technologies
  - 14 HTA bodies, coordinated by HAS, France
  - Funded by EU: October 2013-August 2015
  - 10 early dialogue projects – 7 medicines, 3 medical devices
  - “Reduce the risk”

Where we are will determine what could be useful



Aligning on Health Technology Assessments (HTAs)

- Since 2005 collaboration – 30 countries in “Joint Action 2”
- Co-funded EU and HTA bodies
- Collaboration for evidence-based decision-making
- Reduce duplication, share knowledge
  - Efficient use of resources
  - Sustainable system of knowledge-sharing
  - Best practices
- 10 pilots, “production” – an interest in orphans
- Uptake and use by Member States
- Participation in the development of the future systems, tools
- Permanent network of HTA bodies – 2011 Cross-Border Healthcare Directive

Where we are will determine what could be useful

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Follow-up measures – safety **+ efficacy**

- Pragmatic regulatory decisions since earliest days of Orphan Regulatory framework
- Uncertainties at time of Marketing Authorisation – follow-up
- Conditional Marketing Authorisation since 2006
- EU's Pharmacovigilance Regulatory framework 2012 & 2014: Pharmacovigilance & Risk Assessment Committee (PRAC)
  - Post-Authorisation Safety Studies (PASS)
  - Post-Authorisation Efficacy Studies (PAES)

The final frontier?

Where we are will determine what could be useful

Multiple opportunities to interact with payer bodies

- Talking with individual countries
  - Planning, budgeting
  - “No surprises” – for anyone!
- MEDEV group of payers exploring MoCA recommendations
  - Set of recommendations about joint evaluation of orphan drugs signed by 11 EU Member States, April 2013
  - Payers “club” since 1998, now inviting orphan drug manufacturers for dialogue on a voluntary basis
  - 1 pilot initiated, 2 further pilots initiated
- Monday 20 April 2015 – Belgium + Netherlands formally announce intent to collaborate on orphan pricing negotiations
  - Develop practical action points to enable joint discussions with companies
  - Intent to be operational by 2016
  - “Represent more patients, can negotiate a lower price”

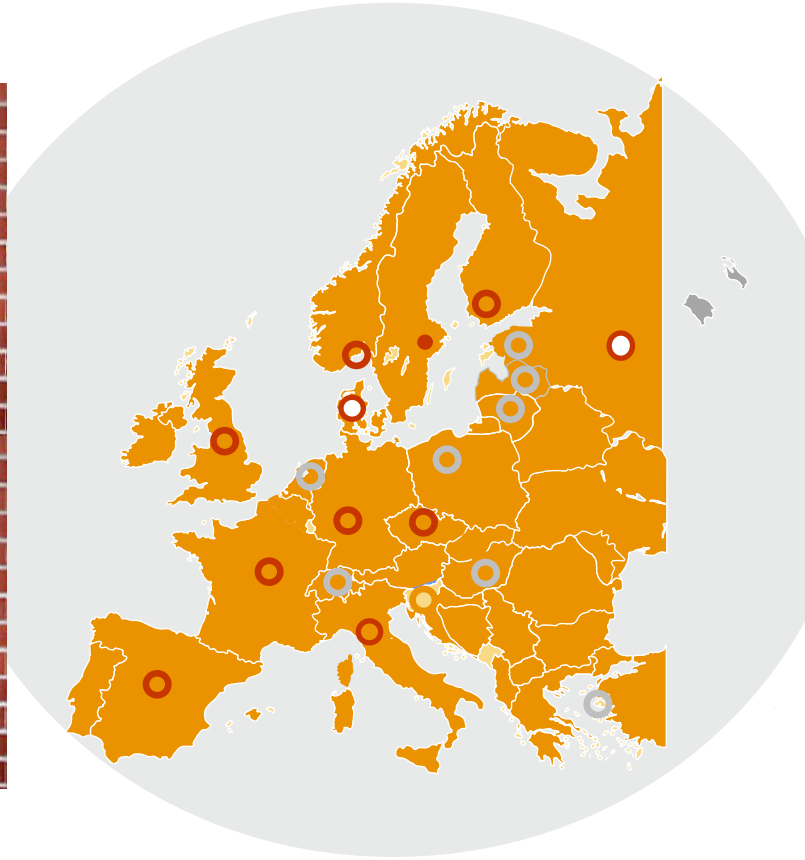
But if Europe comes late in the thinking, what then?

Small number of specialised companies

Ex-EU company

- 1 niche product
- +/- 300 patients in EU
- Ph3 or later
- No EU capabilities

- Costs/capital
- Regulatory
- Pricing & Patient Access
- Pharmacovigilance
- Compliance
- Supply network
- Marketing capabilities
- QA release
- KOL network
- IT
- Insurances
- Offices
- …



Estimated start-up cost in EU $50 million!

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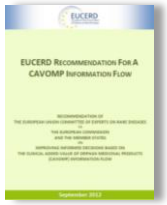
A wealth of initiatives to address uncertainties, build collaboration

Tapestry Networks

SEED
Shaping European Early Dialogues



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Horizon Scanning + Payers + Early Dialogue

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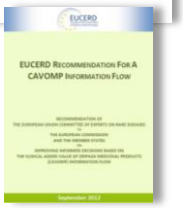
Conditional Marketing Authorisation



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Initial Value Assessment

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Post-Authorisation Safety & Efficacy

Real-World Patient Outcomes

EMINET

Managed entry agreements for pharmaceuticals:
The European experience[1]

Conditional Reimbursement

Conditional Value-Based Pricing

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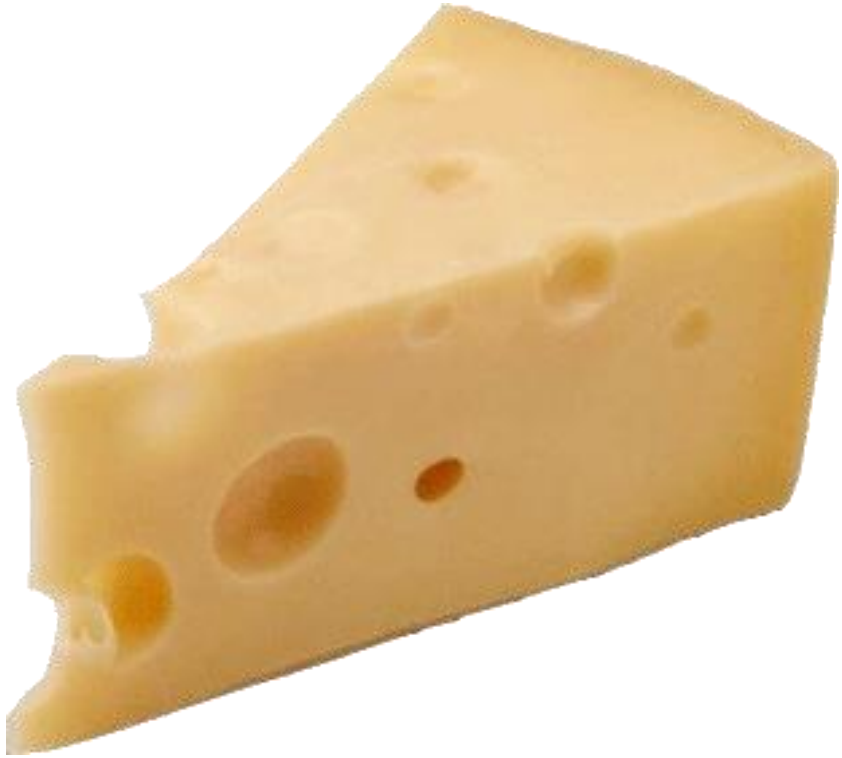
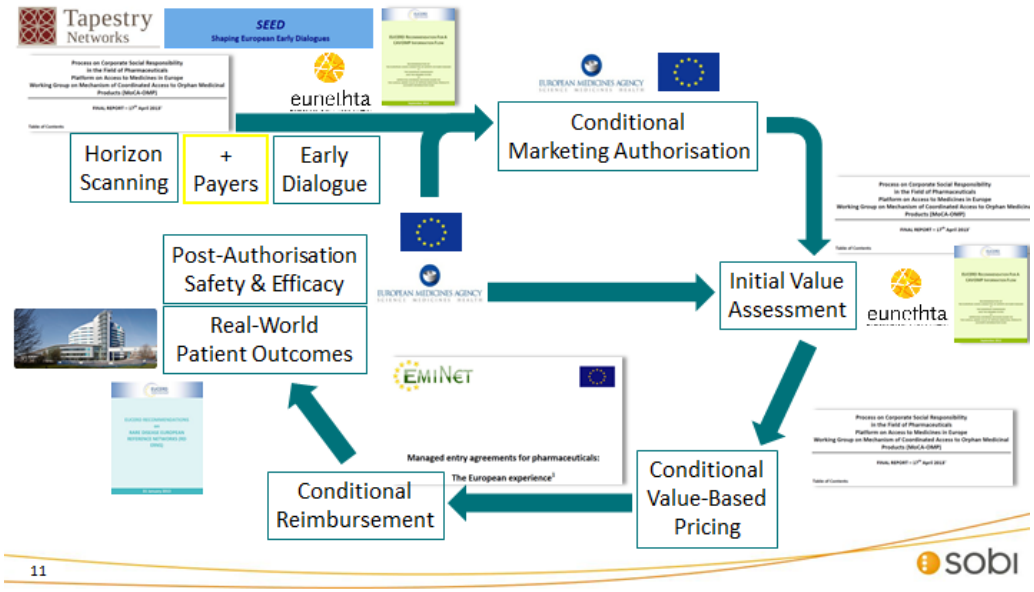


Whose responsibility is it?

Creating a prospective, agreed system



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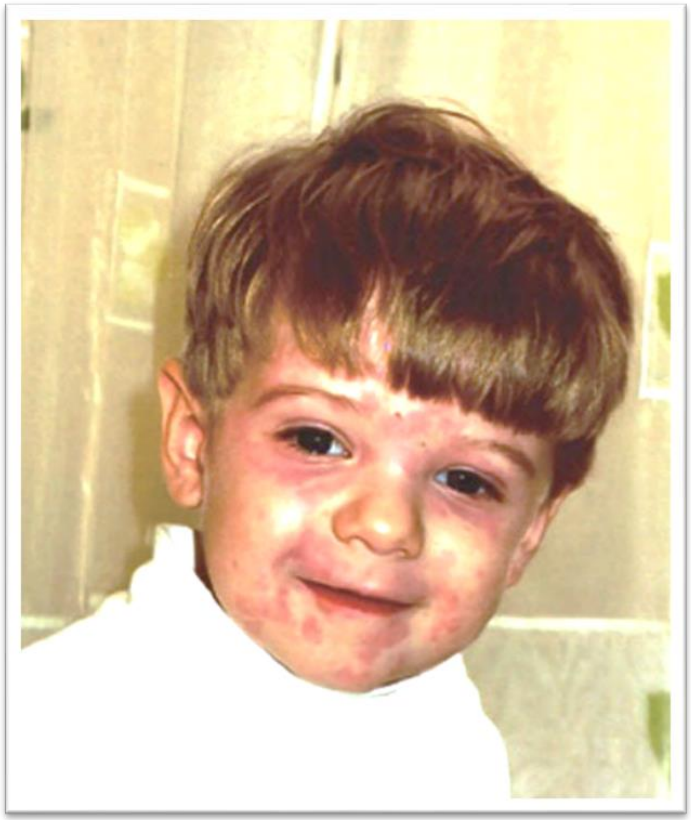
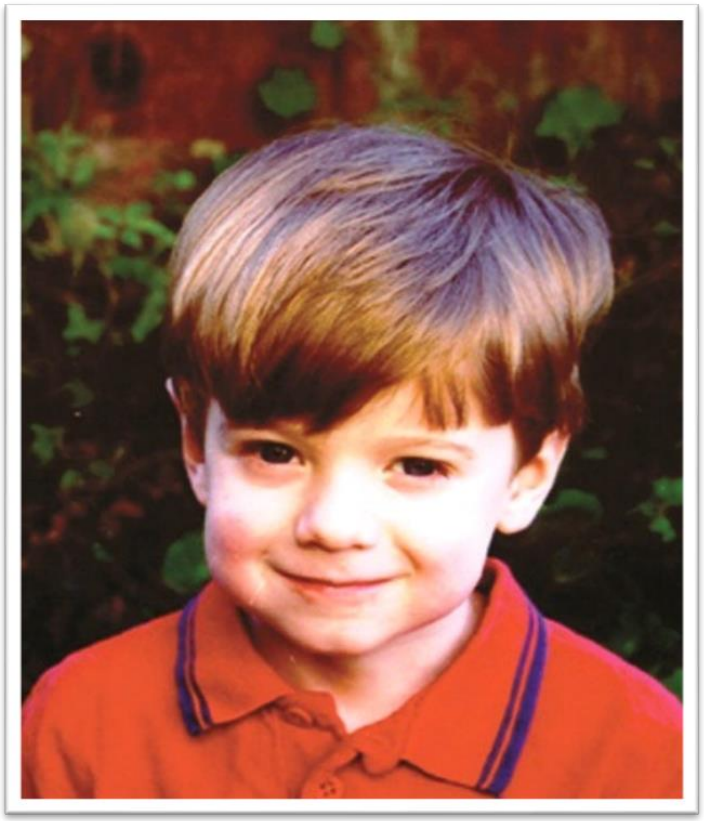
If we have a shared goal, we can find solutions





Rising to the challenge(s)

- It should be part of your first move
  - Have the end in mind from the very beginning – securing [reimbursed] access will be a critical key to commercialisation + avoiding delays
- The innovative product is just the start – need similar innovation in your team approaching the European market(s)
  - Am I making the most of everything at my disposal?
  - Engagement, adaptive designs + methods, discussing at European / collaborative levels
- It's never too late to start a dialogue – with whom will depend on where you are in the process + it's [almost] never too late
- People across the system are genuinely open to find collaborative solutions if you are genuinely seeking the same outcome

Thank you!