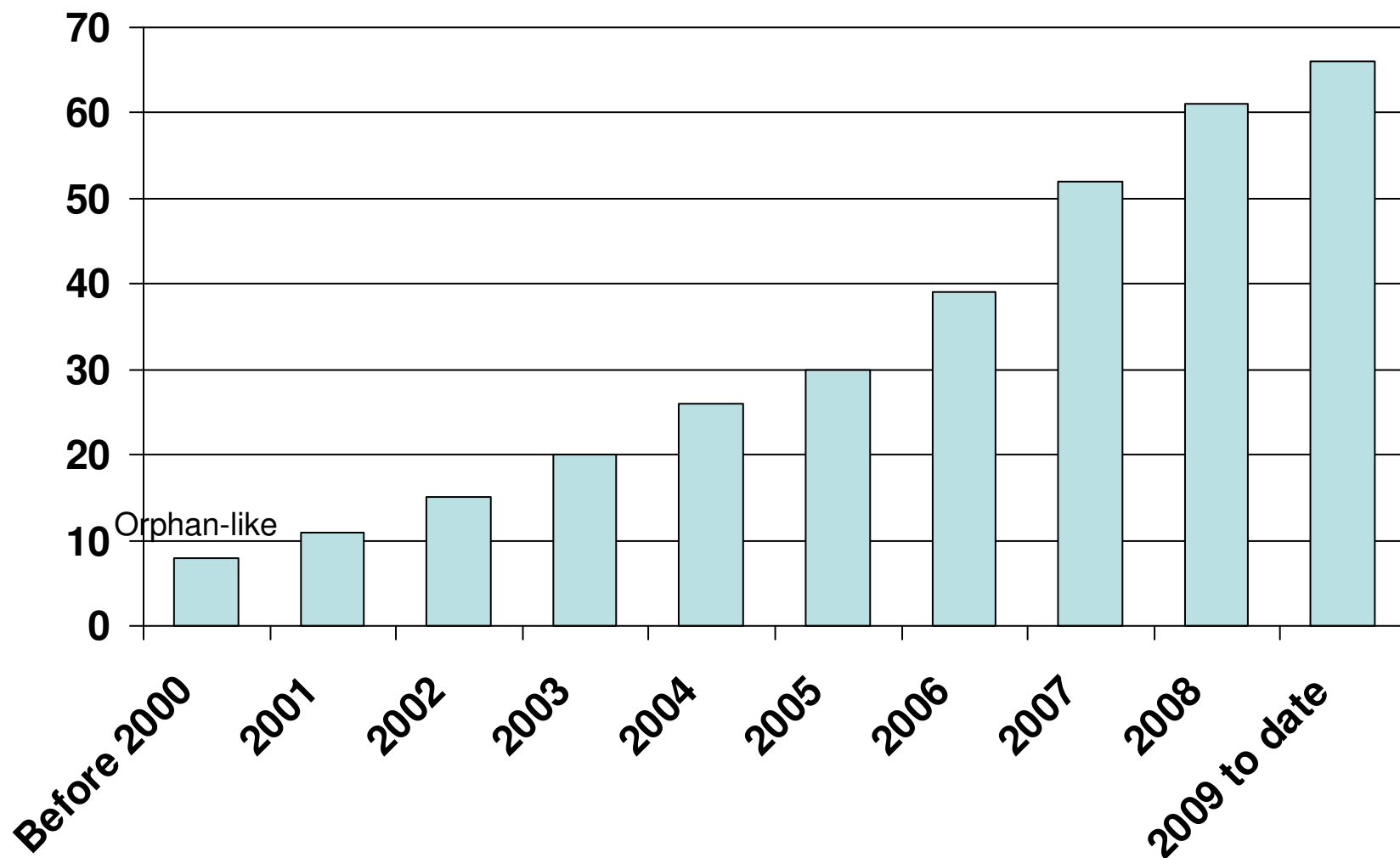


**How to provide
sustainable access to
Orphan Medicinal
Products at national level?**

*Making the most of current opportunities
for European cooperation to improve access*

Wills Hughes-Wilson
Senior Director Health Policy Europe, Genzyme

The legislation is working: cumulative number of orphan medicines with EU Positive Opinion



The problem



Treatment approved:

- unique or significant benefit

BUT

Small number of patients in Clinical Trial



Small number of patients in Clinical Trial = "not enough data to reimburse"

Governments:

- We need to know that what we are asked to pay for is something that works before we reimburse it

Sponsor:

- We need to develop the data but how to do it with no market access?

EMA / European Commission:

- Single EU Marketing Authorisation but 27+ different in-use follow up plans?

Patients:

- Why can we not get access to an approved treatment?

Proposal

Clinical Added Value Reports

1. Acknowledge “unique or significant benefit” concept in EU Orphan development
2. Working Group at EMEA to prepare report for Member States on clinical value and role of treatment at time of Marketing Authorisation
3. Member States to grant access
4. In-use data gathering to build up experience of product clinical value



EU High Level Pharmaceutical Forum – October 2008

Acknowledged the challenges in the field of orphan drugs & proposed 4 elements to address them



November 2008

EU Commission Communication
on Rare Diseases: Europe's Challenges

Recognition & Visibility of Rare Diseases

Develop European Cooperation
& Improve Access to
High-Quality Healthcare for Rare Diseases

International Cooperation

Governance & Monitoring

June 2009



Council Recommendation on a European
Action in the Field of Rare Diseases

National Plans for Rare Diseases

Definition, codification & inventorying

Research

Centres of Expertise & Reference Networks

Gathering expertise at European level

Empowering patients' organisations

Sustainability



EU High Level Pharmaceutical Forum – October 2008



November 2008

EU Commission Communication
on Rare Diseases: Europe's Challenges

June 2009



Council Recommendation on a European
Action in the Field of Rare Diseases

Develop European Cooperation
& Improve Access to
High-Quality Healthcare for Rare Diseases

Gathering expertise at European level



EU High Level Pharmaceutical...

“Something needs to be done about orphan drugs”



November 2008

June 2009



EU Commission Communication on Rare Diseases: European Strategy

“We will set up a working party with Member States”

Council Recommendation on a European Union in the Field of Rare Diseases

Develop European Cooperation & Improve Access to High-Quality Healthcare for Rare Diseases

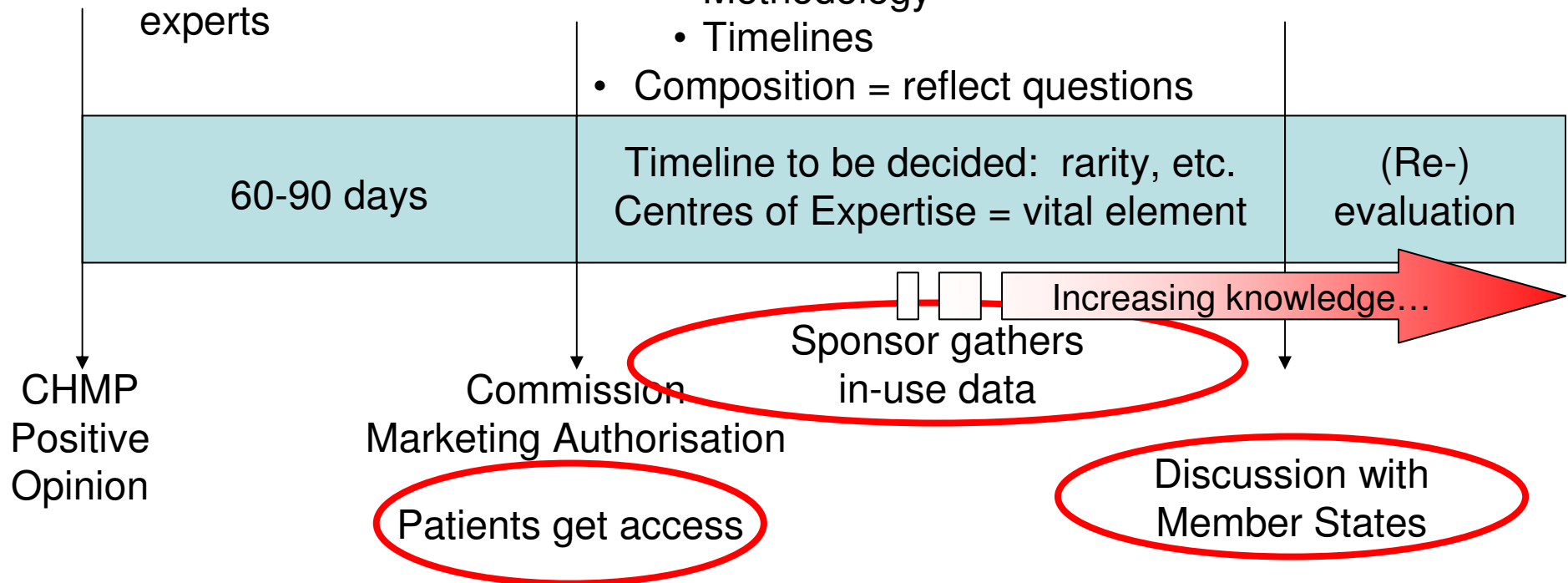
“We will work together”

Gathering expertise at European level

CAVOD & the new proposals: A solution?

- Task 1: Clinical Added Value Report prepared by Committee @ EMEA
- Composition = orphan experts

- Task 2: Annex / “Roadmap” – minimum data set required to continue reimbursement
- Stakeholders:
 - What do Member States want / need to see?
 - What is realistic for sponsor to develop
 - Role & involvement of patients
 - Treating physicians
 - Methodology
 - Timelines
- Composition = reflect questions



CHMP
Positive
Opinion

Commission
Marketing Authorisation

Patients get access

Sponsor gathers
in-use data

Increasing knowledge...

Discussion with
Member States

60-90 days

Timeline to be decided: rarity, etc.
Centres of Expertise = vital element

(Re-)
evaluation

Critical Success Factors

1. Clear mandate = to facilitate timely access
2. Should not add additional time or hurdles
3. Composition of the evaluator group – must be expertise- / relevance-based
4. Member States would need to engage with the process / take note of the evaluations



EU High Level Pharmaceutical Forum – October 2008



November 2008

EU Commission Communication
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National Plans for Rare Diseases

Develop European Cooperation
& Improve Access to
High-Quality Healthcare for Rare Diseases



Centres of Expertise & Reference Networks

Gathering expertise at European level



Swedish EU Presidency Pilot Project on follow-up effectiveness of Orphan Drugs?

Who would need to act in order to make this happen?*



EMA European
Medicines Agency



European Commission
DG SanCo?
DG Enterprise?



Member States

Sponsors

genzyme

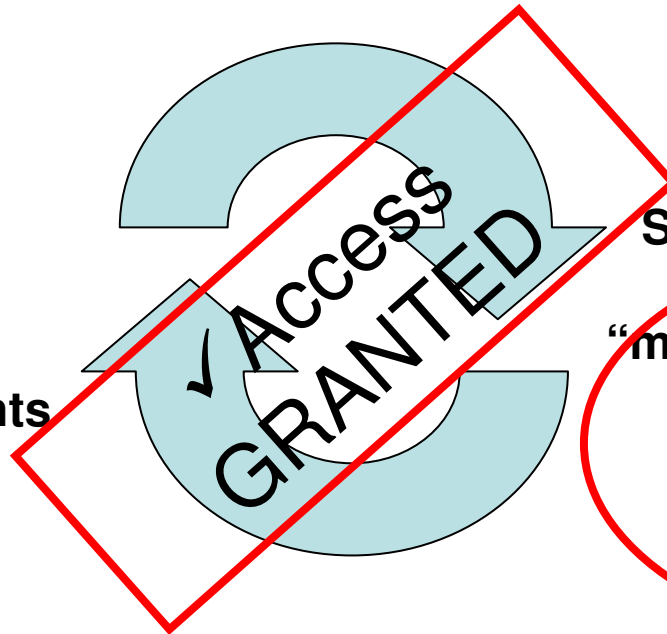
Patients

*** Pretty much everyone! Will need to be a cooperative and coherent set of actions**

The problem – solved?



Treatment approved:
- unique or significant benefit
Acknowledging just small number of patients in Clinical Trial



Small number of patients
in Clinical Trial =
“maybe not enough data yet
to make definitive
reimbursement
decision...
...but we have a plan”

Governments:

- ✓ Get to know that what they are asked to pay for is something that works

Sponsor:

- ✓ Get realistic timeline, methodology to develop in-use data with wider patient access (N.B. Prof. Cox's comments yesterday)

Patients:

- ✓ Get immediate access to an approved treatment

Thank you!