

EMA Conference

10 yrs of EU OMP Regulation

Pharmaceutical Industry Perspective

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Joint Rare Diseases / Orphan Medicinal Products Task Force

A single voice for OMP developers in Europe

Vision:

Timely and equal access to therapies and care for all 30 million rare disease patients across Europe, regardless of their country of residence, and, regardless which one of the >7000 rare diseases they suffer from.

Joint TF Composition: From SME to 'Big Pharma'

47

European member companies of all sizes that have either developed or intend to develop orphan drugs

ebe
european
biopharmaceutical
enterprises

EUROPA BIO
The European Association for Bioindustries

Members of EBE and/or EuropaBio

4

Meetings per year

Joint OMP TF Priorities



- Contribute to the development of EU rare diseases-related policies and regulatory frameworks
- Work towards a harmonised implementation of EU legislation and regulatory guidelines for OMPs
- Improve timely access to therapies for all rare disease patients across the EU
- Raise awareness about the economic and social value of OMPs
- Provide expertise where needed

Congratulations!



To the rare disease Patient Associations, the Commission, the Parliament, the EMA, COMP and CHMP, and the industry associations for the **10th anniversary** of a successful healthcare legislation on Orphan Medicinal Products:

Regulation EC 141/2000



Successful Regulation



- Fruitful dialogue
- Patient oriented
- Uniformity of advice and procedures
- Free Protocol Assistance
- 10-year market exclusivity
- EMA: ‘one-stop shop’
 - Expertise bundling in the EU
 - SME support
 - Orphan designation
 - Protocol Assistance
 - Marketing Authorisations

Some Myths Still Linger...



- Developing an orphan drug is easier and quicker
- Limited clinical data required for approval
- Faster approval procedures for OMPs
- Incentives minimise drug development costs
- 10-year EU Market Exclusivity protection creates monopoly for first marketing authorisation
- OMPs are only developed by SMEs
- All OMPs are prohibitively expensive
- Patients have access to all orphan drugs as soon as they are approved

Orphan Drug Development Reality

Orphan drug development is often even more challenging:

- Few patients, geographically dispersed
- Often limited experience
- Standard study designs often not feasible
- Quality of data required is no different from non-orphans: needs to show positive benefit/risk
- Faster approval or granting of a conditional marketing authorisation is not guaranteed



Market Exclusivity for OMPs

Reality

- Orphan legislation only creates monopoly for a product in a given indication vs generics and similar products.
- Treatment options are approved if different molecules, mechanisms of action and benefits for the patients are demonstrated.
- Multiple OMPs have been approved for the same indication
- Example:
5 EU MAs for OMPs for Pulmonary Arterial Hypertension:

Tracleer[®]

Ventavis[®]

Revatio[®]

Thelin[®]

Volibris[®]

Orphan Drug Incentives Reality



- R& D Incentives
 - **Tax brakes:** Member State (MS) dependent and do not apply consistently across all MSs
 - **Grants for clinical trials:** 2H 2009 (DG research)
- EMA/Commission Incentives:
 - **10-year market exclusivity**
 - **Fee waiver protocol assistance**
 - +2 yrs market exclusivity for paediatric indications – what is the value to products with a SPC?
 - 50% Fee reduction for MAAs

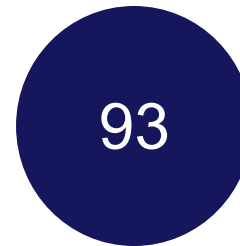
Intellectual Property Protection Is Critical for Orphan Drugs

- Of first 50 approved drugs, 26 have no patent protection*
- Designated orphan drug patent status in the EU:

No patent (or none identified)



Patent expired



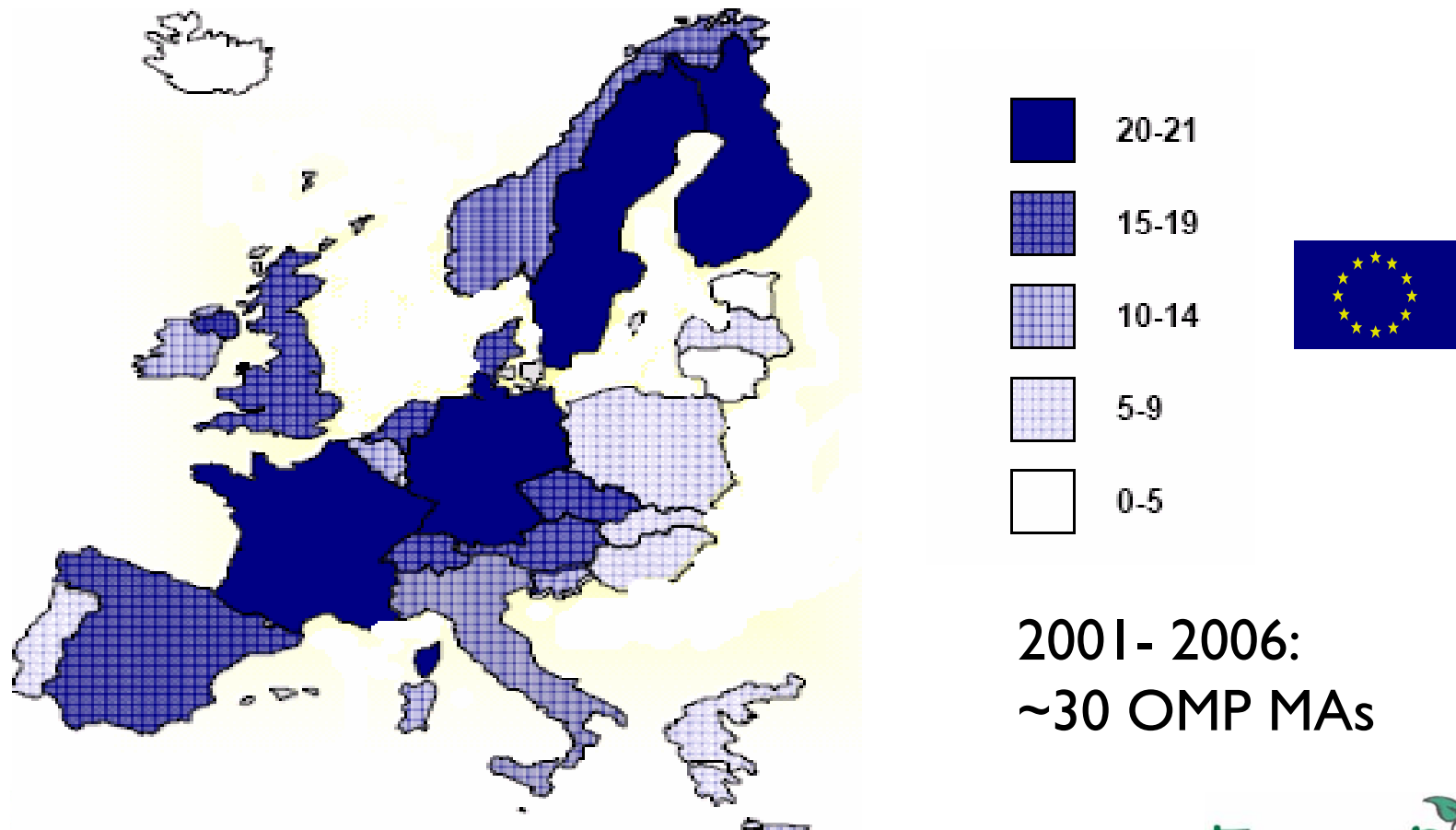
Patent protected



Intellectual property is a foundation of innovation including new therapies for rare diseases. Companies are reliant upon secure intellectual property protection to invest in clinical trials.

Patient Access to Approved Orphan Drugs - Reality

Nb of orphan drugs available in EU markets in 2006*



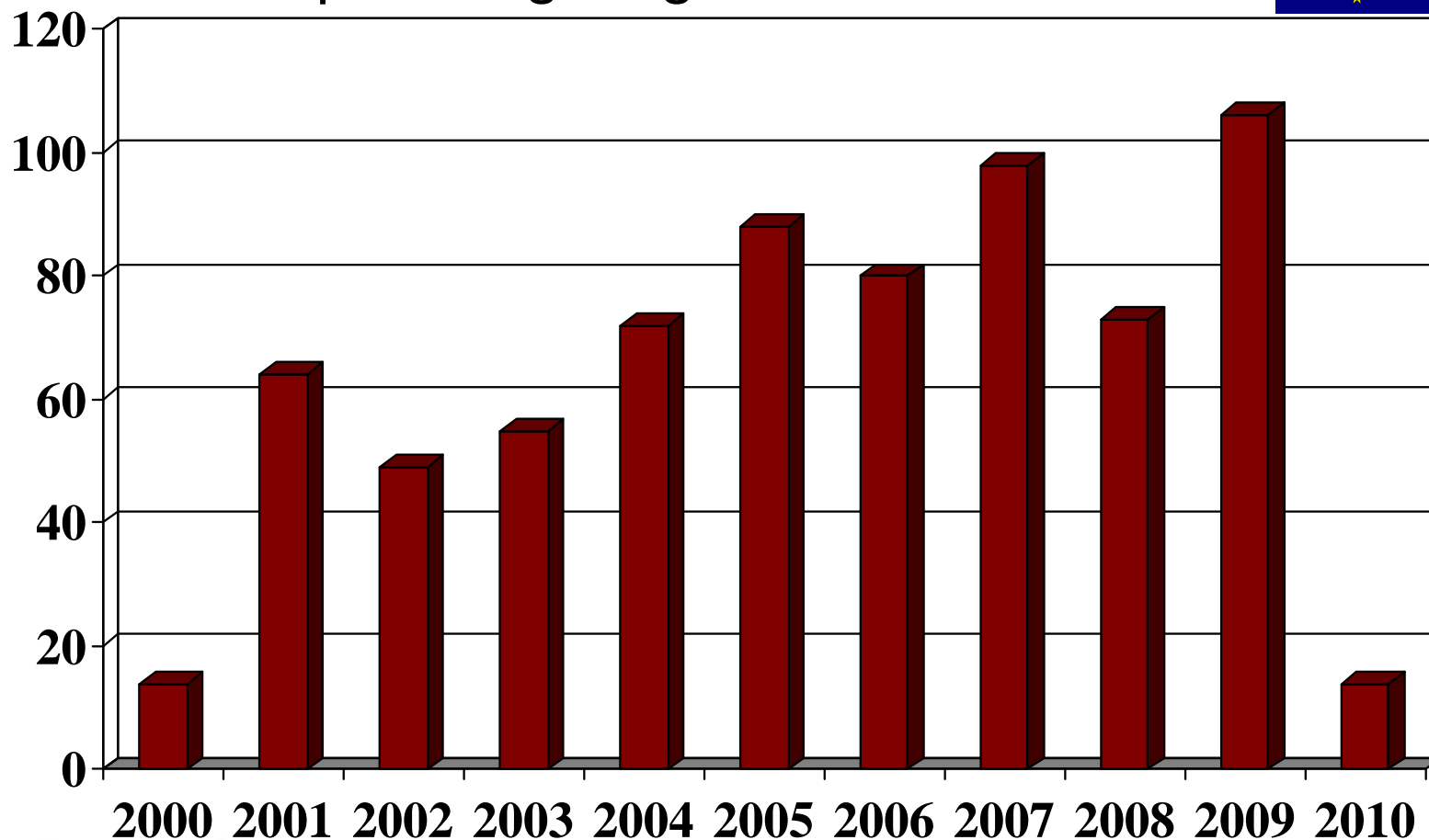
The Perceived Problem



Orphan Drugs

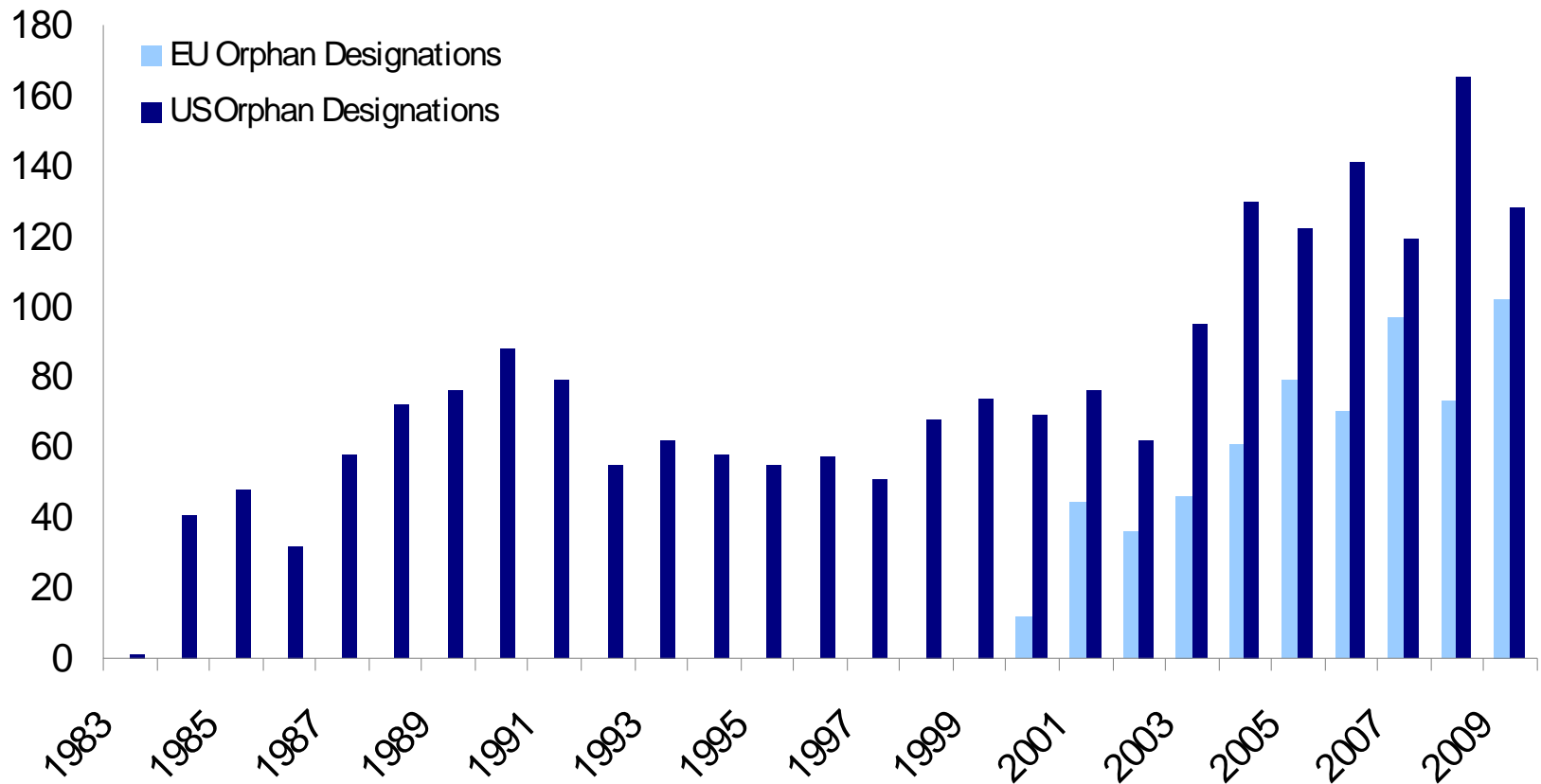
EU Orphan Drug Designations Have Increased Over Time

EU orphan drug designations: 2000 - 2009



Orphan Drug Designations in US (since 1983) & EU (since 2000)

The EU orphan designation rate is catching up with US

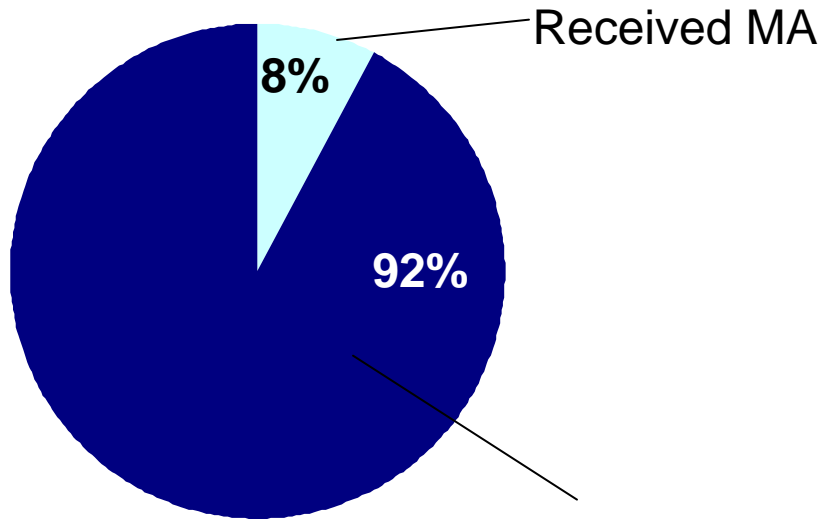


Few Designated Orphan Products Receive Market Authorisation

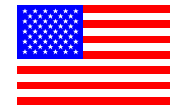


EU

8% of products given orphan designation (2000 – 2009) have since received MA

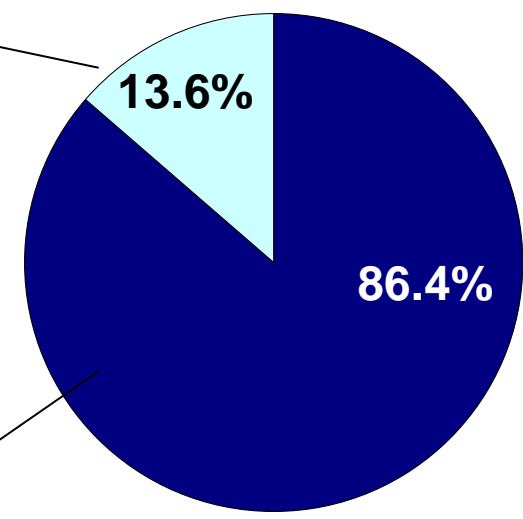


Source: EMEA (2009)



US

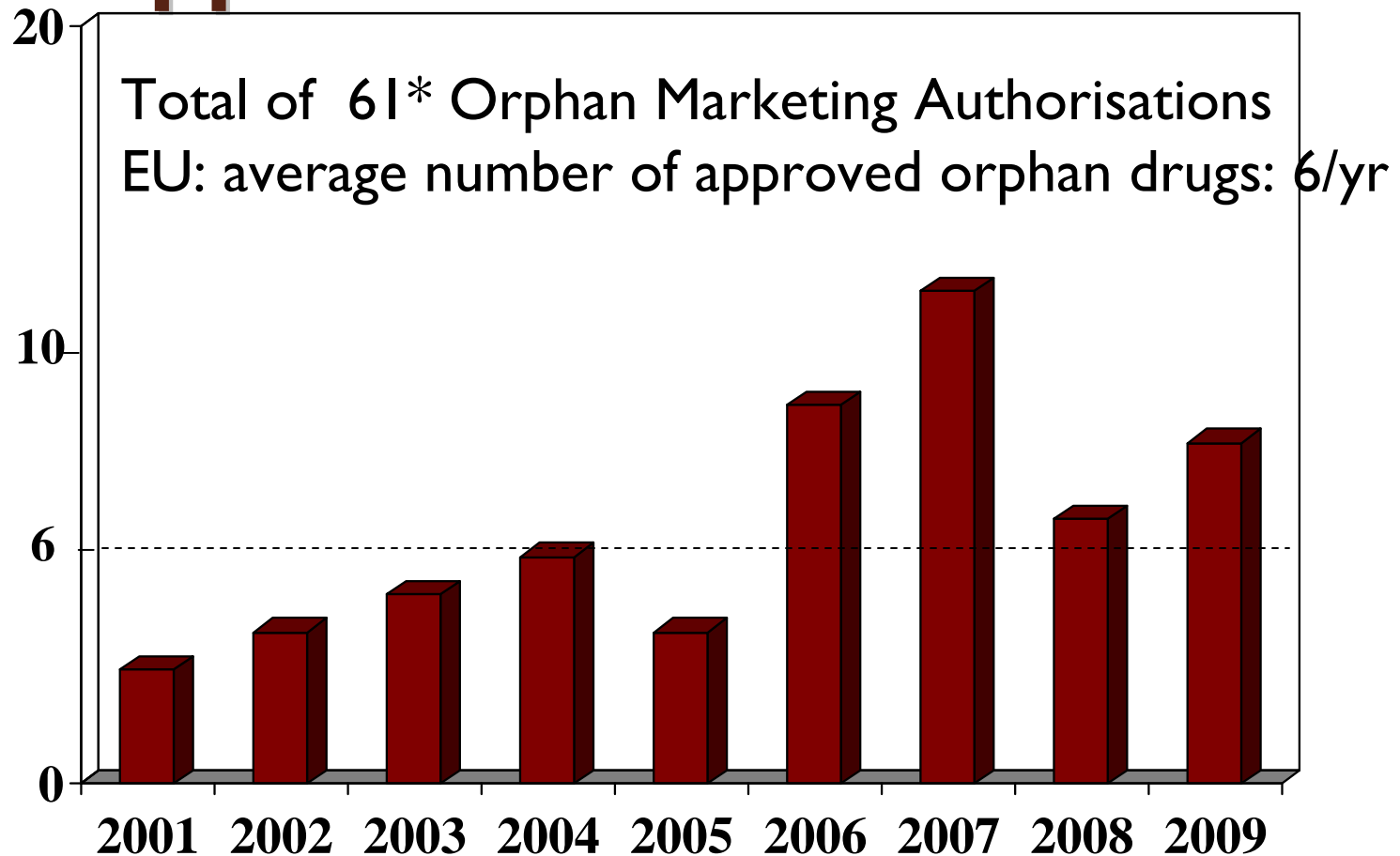
13.6% of products given orphan drug designation (1983 -1993) have since received MA



Source: FDA (2009)

Designated but did not received MA

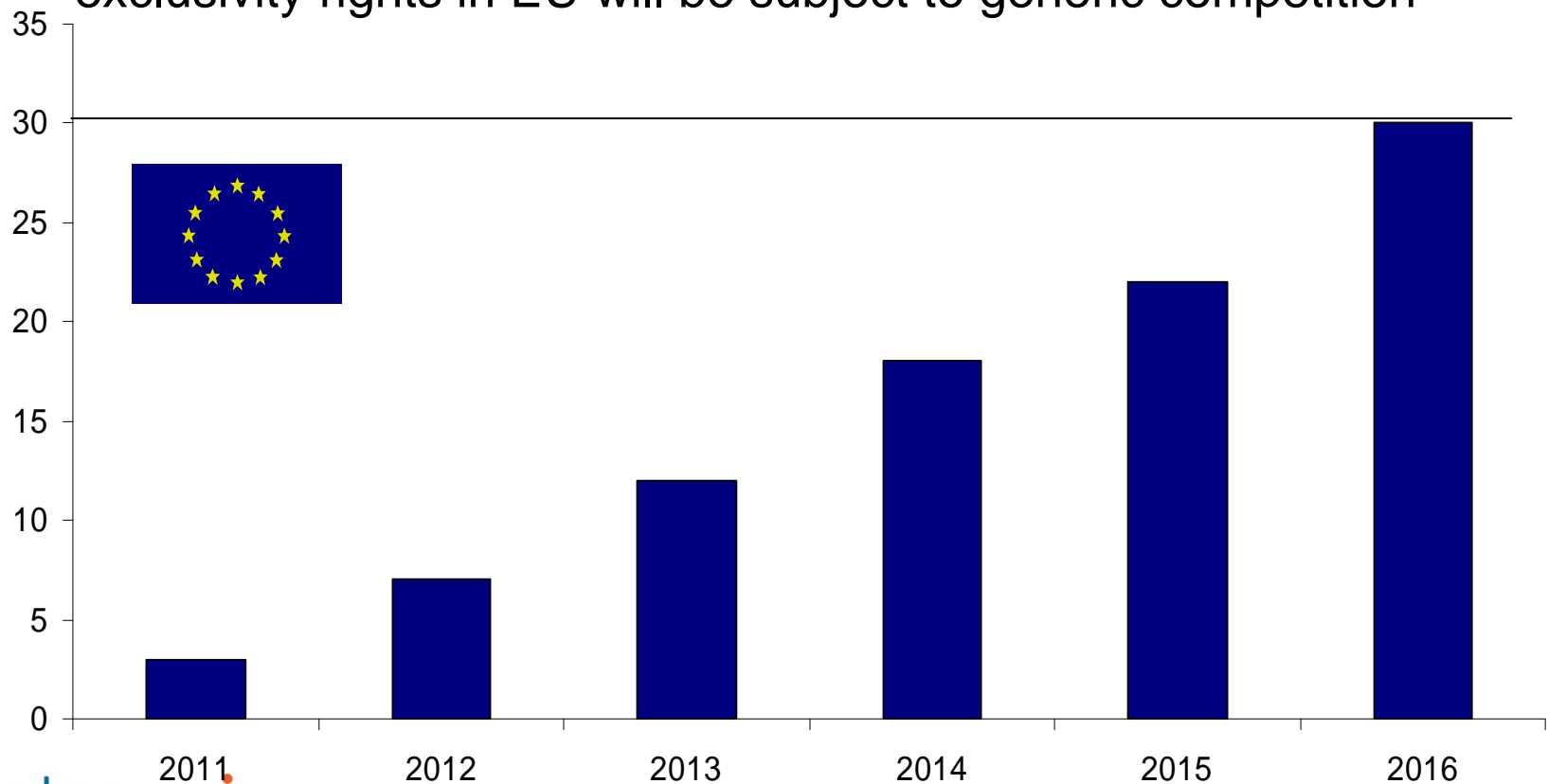
EU MAs for Orphan Drugs Appear to Have Peaked



*2 withdrawn from community register of orphan drugs

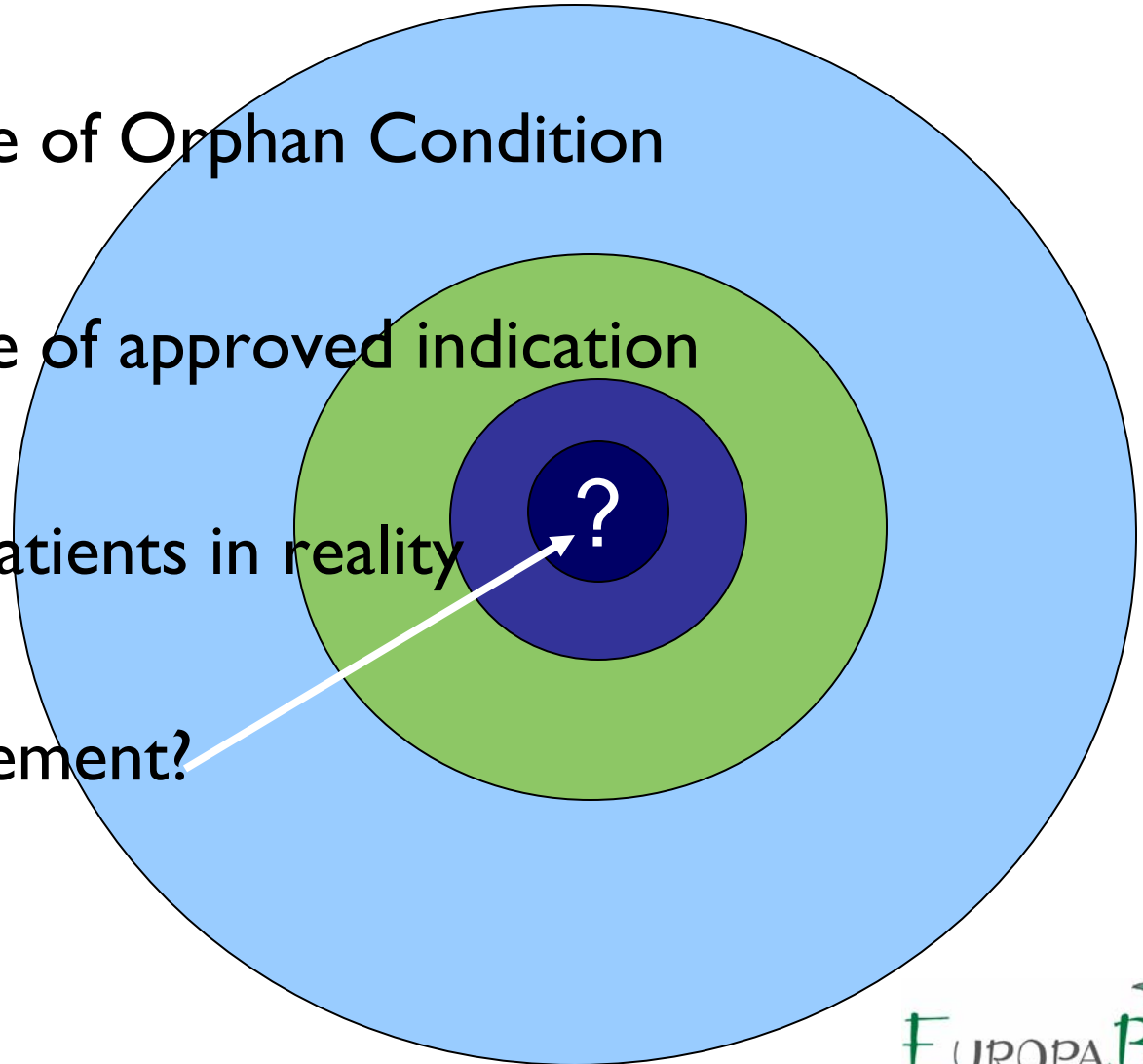
After 2011: EU Orphan Drugs Start Losing Market Exclusivity

By 2016, 30 orphan drugs that currently have market exclusivity rights in EU will be subject to generic competition



The Number of Treated Patients Reality

- Prevalence of Orphan Condition
- Prevalence of approved indication
- Treated Patients in reality
- Reimbursement?



Orphan Drug Development Challenges and Rewards

- Clinical
- Regulatory
- Risk Mgmt
- Market Access
- Resources



- IP protection
 - Market Exclusivity
- Grants
- Tax Breaks

With appropriate protections and incentives, companies will continue their commitment to change the course of rare diseases by developing innovative science and medicines for patients around the world

What Will it Take to Get More Orphans Drugs on the EU Market?

- **Maximise incentives**

- Research grants and tax breaks
- Fee reductions
- 10 years market exclusivity – or more?
- Accelerated approval/CMA
- Reimbursement

- **Educate the uninformed**

- Designation prevalence vs orphan population treated
- One approved orphan drug may lead to another
- Support products throughout life cycle:
Research – Development – Approval – Reimbursement

How do We all Ensure a Bright Future for Rare Diseases and Orphan Medicinal Products in the EU?

- Find creative solutions to address specific rare disease/ orphan drug challenges to ensure:
 - swift approvals
 - consistent reimbursement structures
 - rapid patient access to new treatments
- Increase multi-stake holder brain storming, discussions and exchanges even more
- This workshop is a good example a big thank you to the organisers!

