EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

# Regulatory pathways to new medicines

EU 28: science, medicines, health – a regulatory system fit for the future
6 – 7 May 2013, Dubrovnik, Croatia

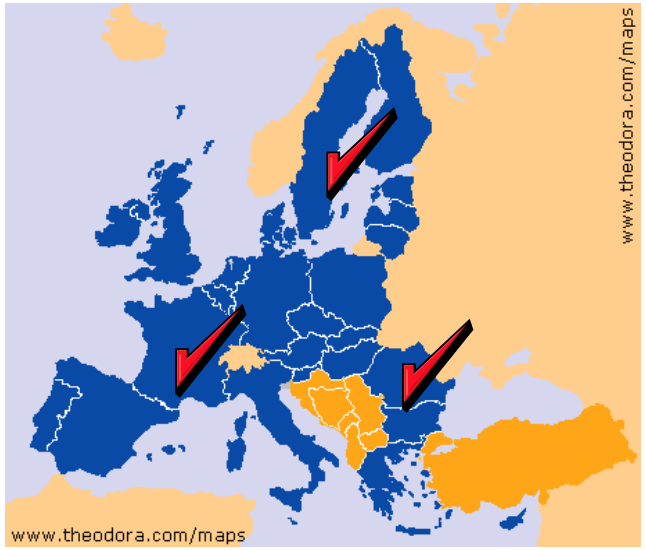
Presented by: Tony Humphreys
Head of Regulatory, Procedural and Scientific Committee Support
Patient Health Protection

# Post Nov 2005 Three European Systems

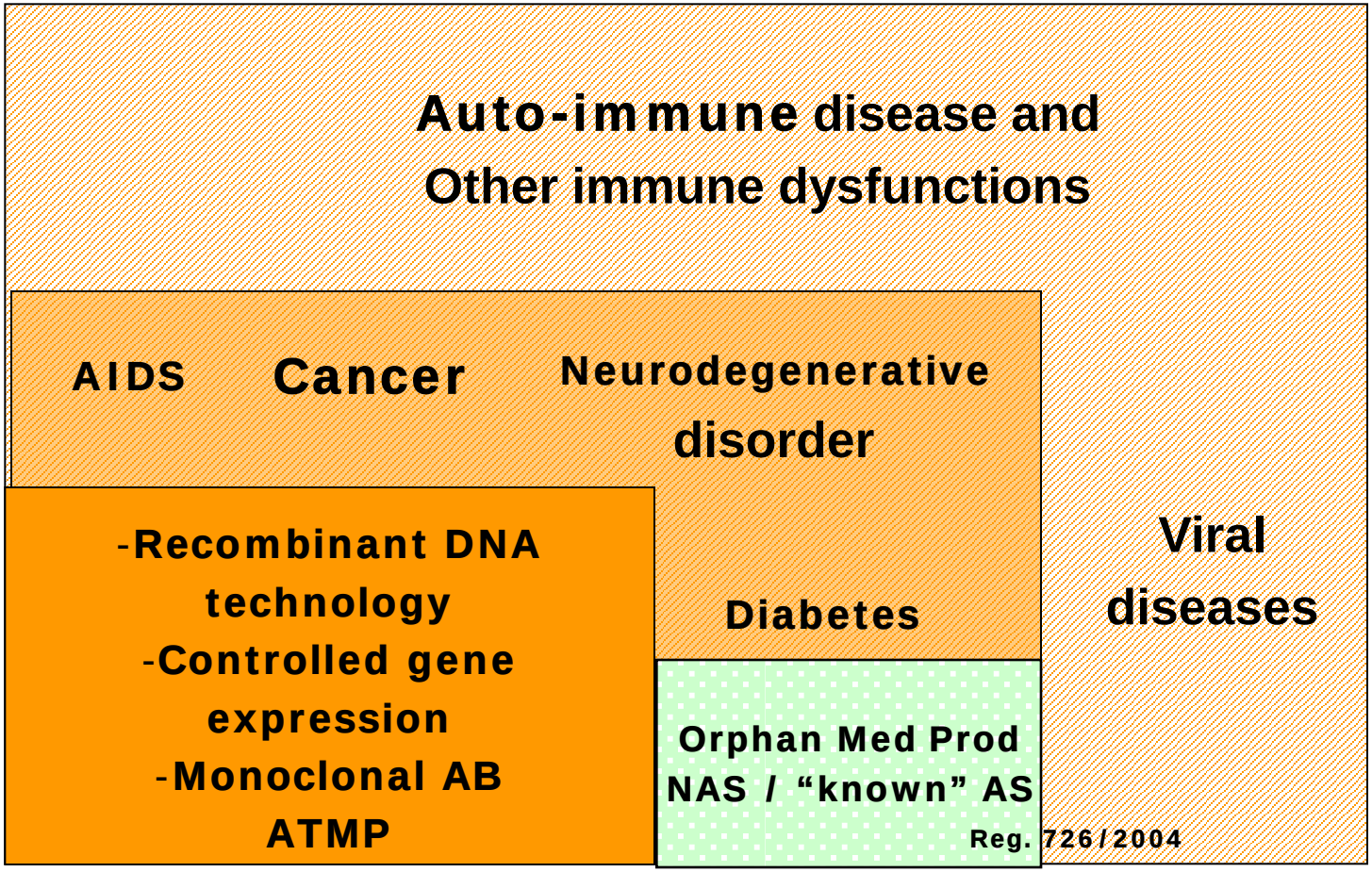
**Centralised Procedure (via EMA)**

**Mutual Recognition procedure**

**Decentralised Procedure**



# Mandatory Scope of Centralised Procedure



# Optional Scope of Centralised Procedure

**Art. 3(2) of Regulation (EC) No 726/2004**

**Art. 3(2)(a)**

New Active Substances

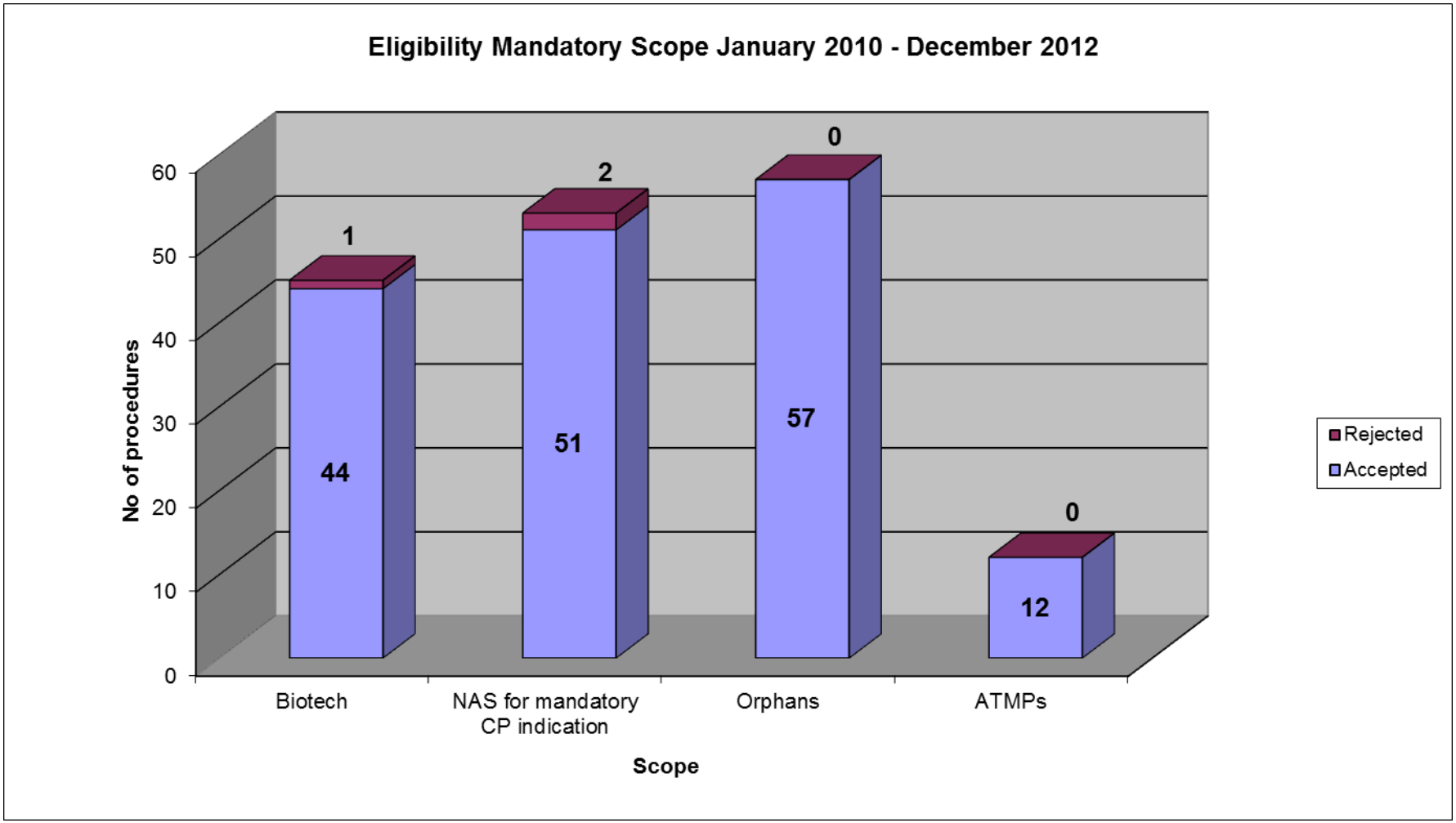
**Art. 3(2)(b)**

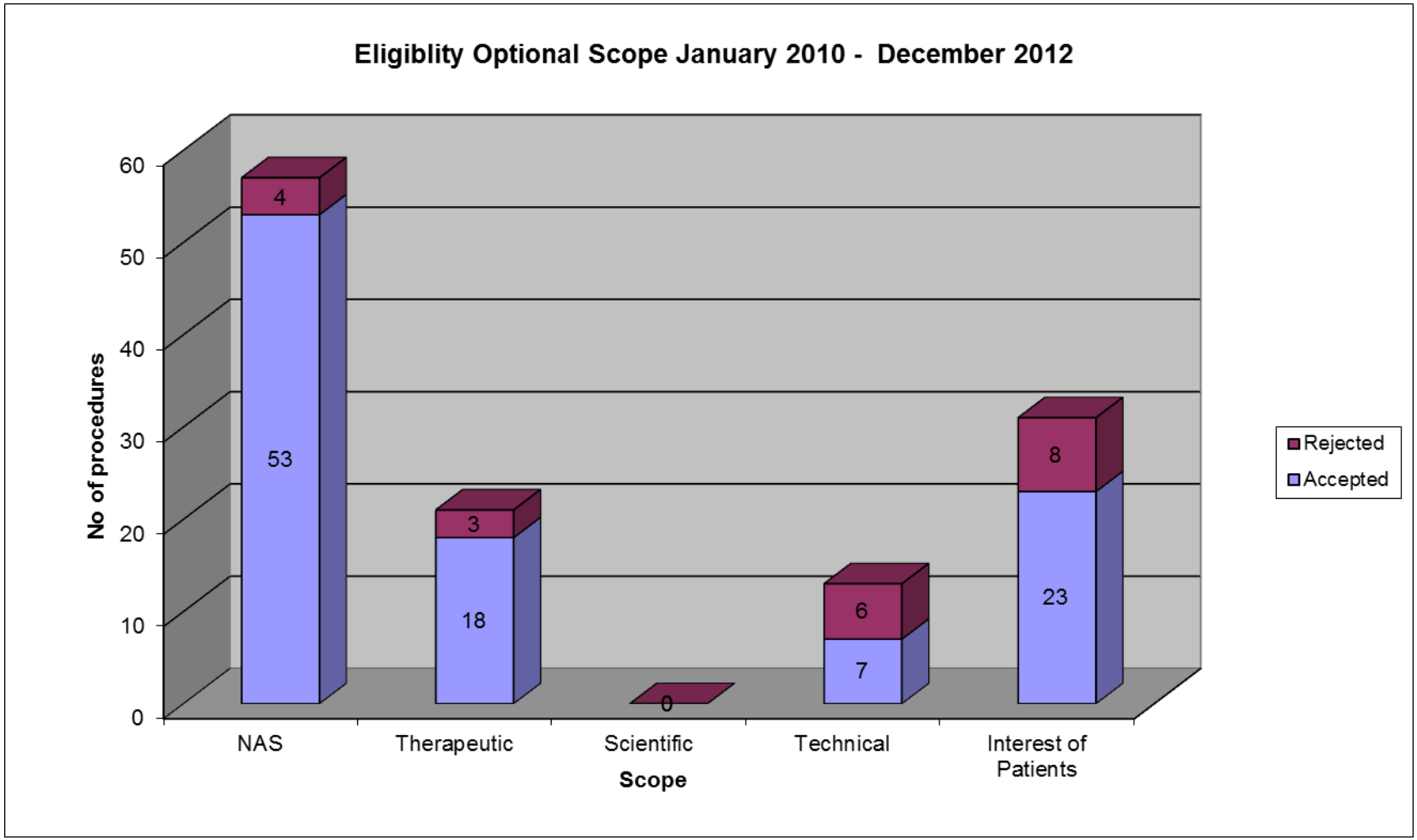
Significant Innovation

-Therapeutic &/or Scientific &/or Technical

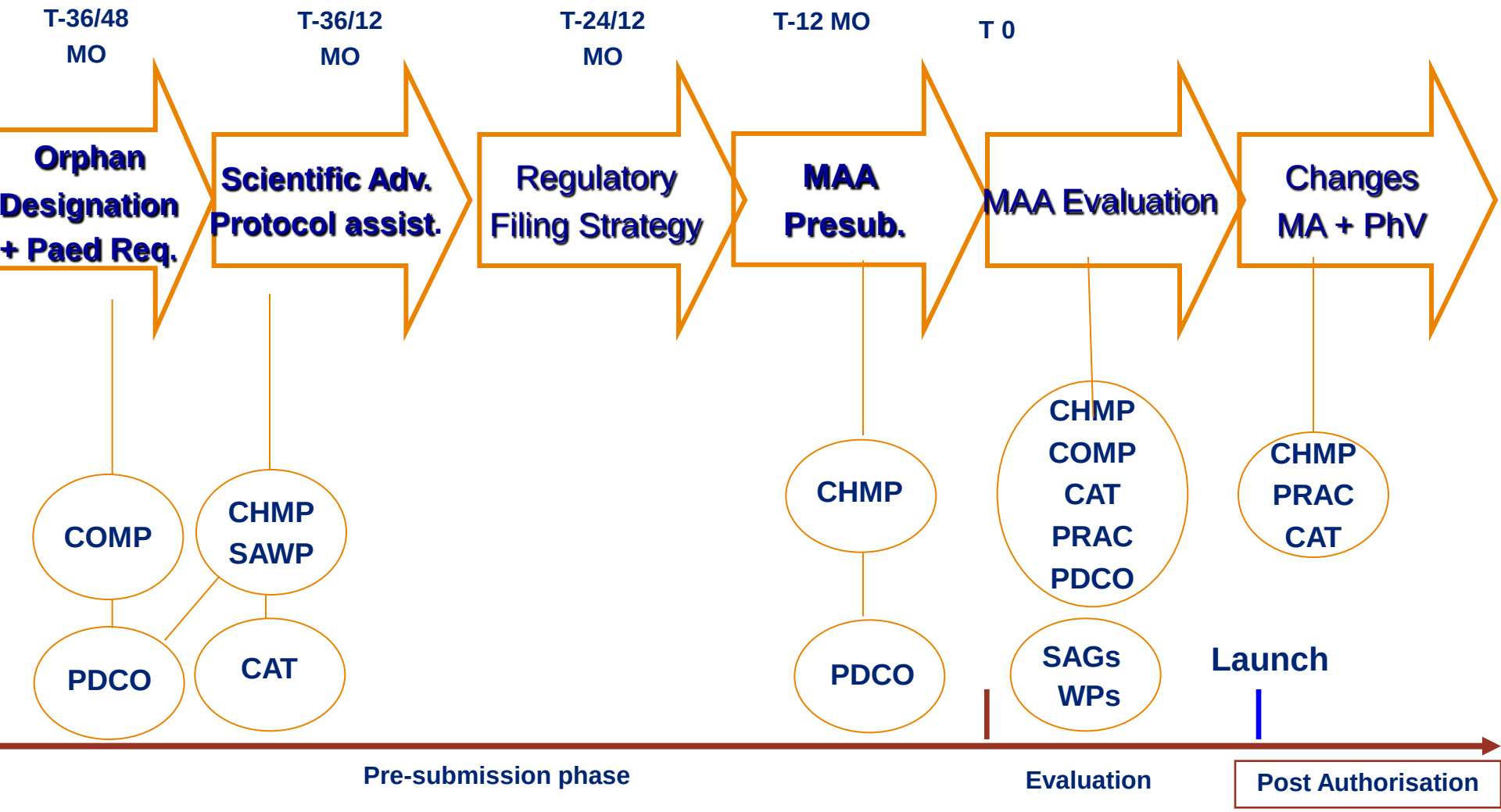
**OR**

Interest of Patients at Community Level

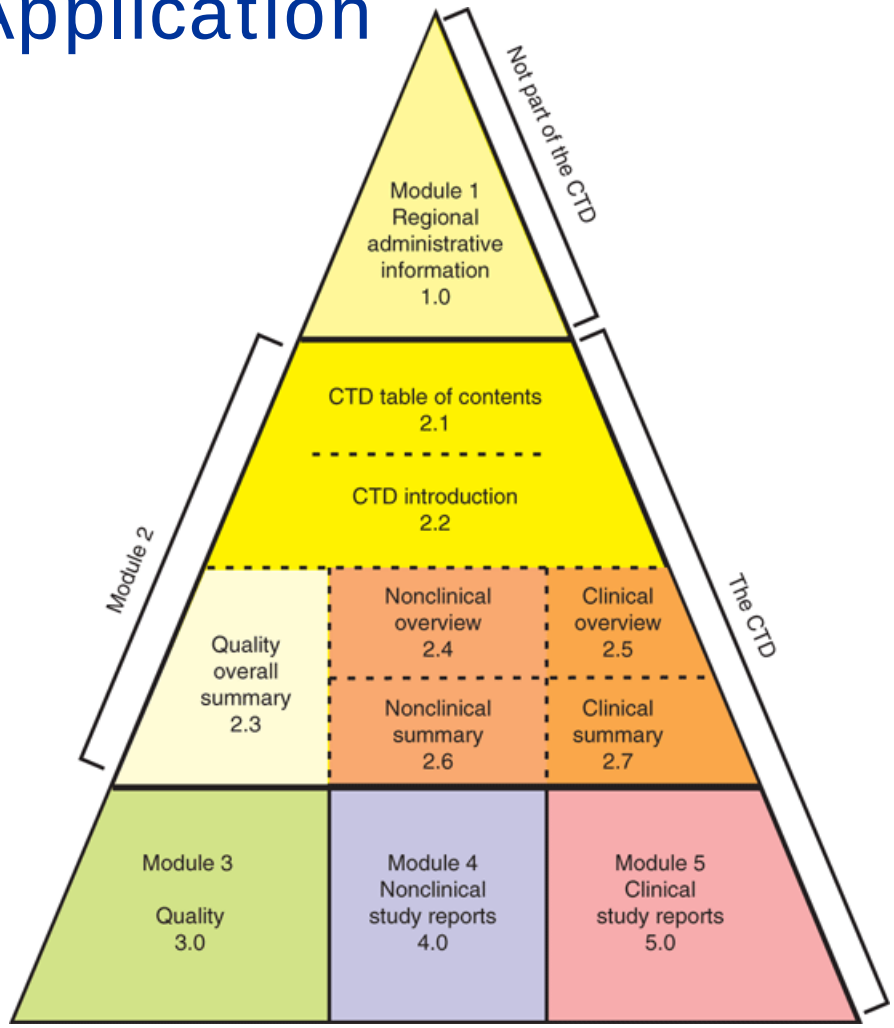

Eligibility Mandatory Scope January 2010 - December 2012
No of procedures
60
50
40
30
20
10
0
1
2
0
44
51
57
0
12
Biotech
NAS for mandatory CP indication
Orphans
ATMPs
Scope
Rejected
Accepted


Eligiblity Optional Scope January 2010 - December 2012
No of procedures
60
50
40
30
20
10
0
4
53
3
18
0
6
7
8
23
NAS
Therapeutic
Scientific
Technical
Interest of Patients
Scope
Rejected
Accepted

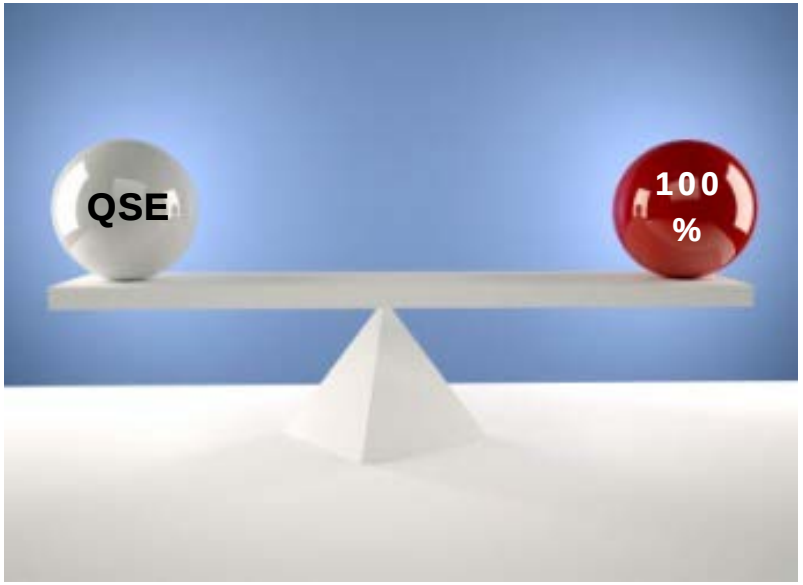
# Committees Co-ordination



# Assembling your Marketing Authorisation Application




All Marketing Authorisation applications must demonstrate the same standard of



# Applicants “choice” of legal basis

## Innovator pharma

**Art. 8(3) “Full”**
100% non clinical / clinical studies

**Art. 8(3) “Mixed”**
> 0% - < 100% Non clinical / clinical studies
100% balance bibliography

## All Pharma

**Art. 10 (c)**
Informed Consent

**Art. 10 (a)**
Well Established Use
100% bibliographic

**Art. 10 (b)**
Fixed Combination Dose forms
> 0% - < 100% Non clinical / clinical studies

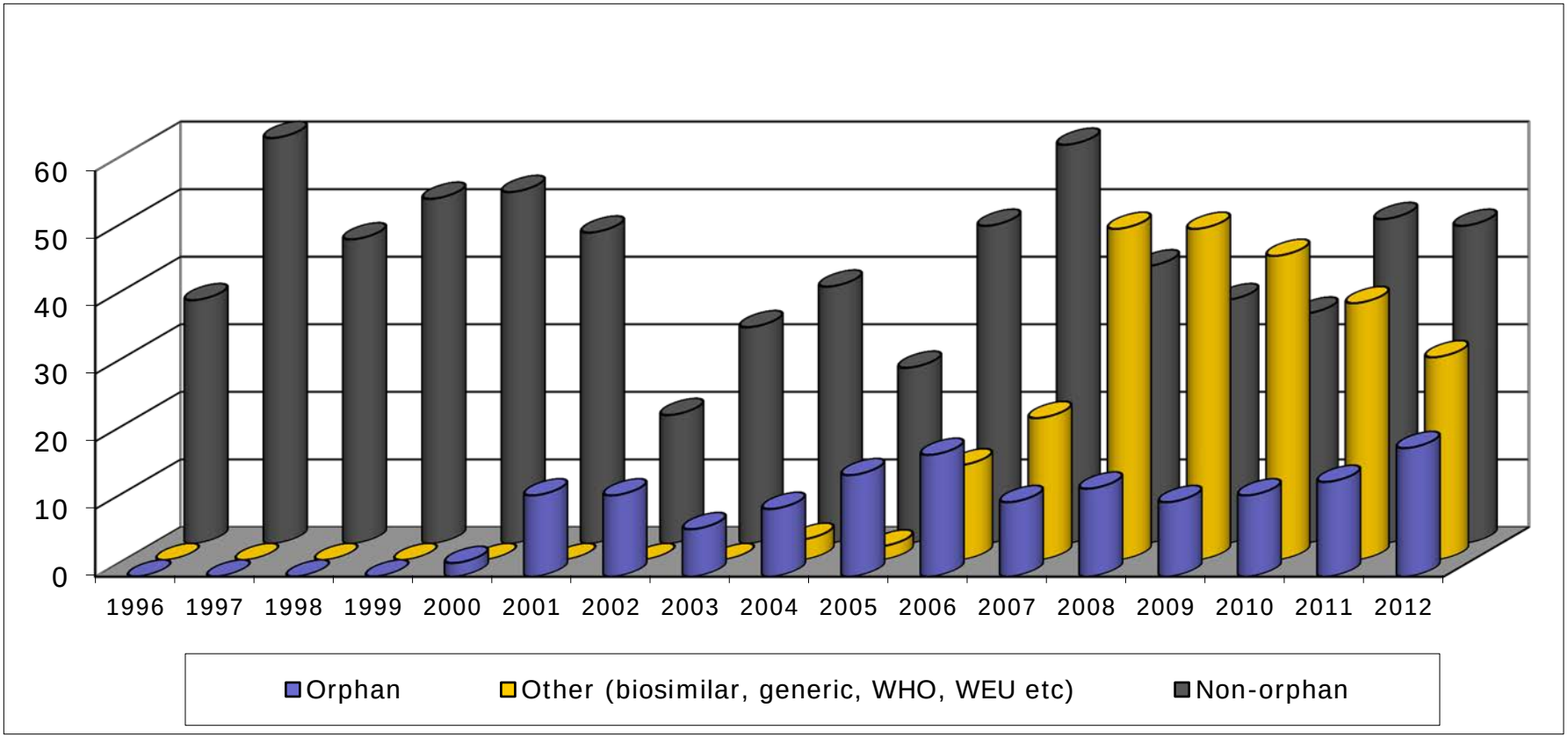
## Generic / Biosimilar Pharma

**Art. 10 (1)**
Classic “generic”
Bioequivalence +/- biowaiver

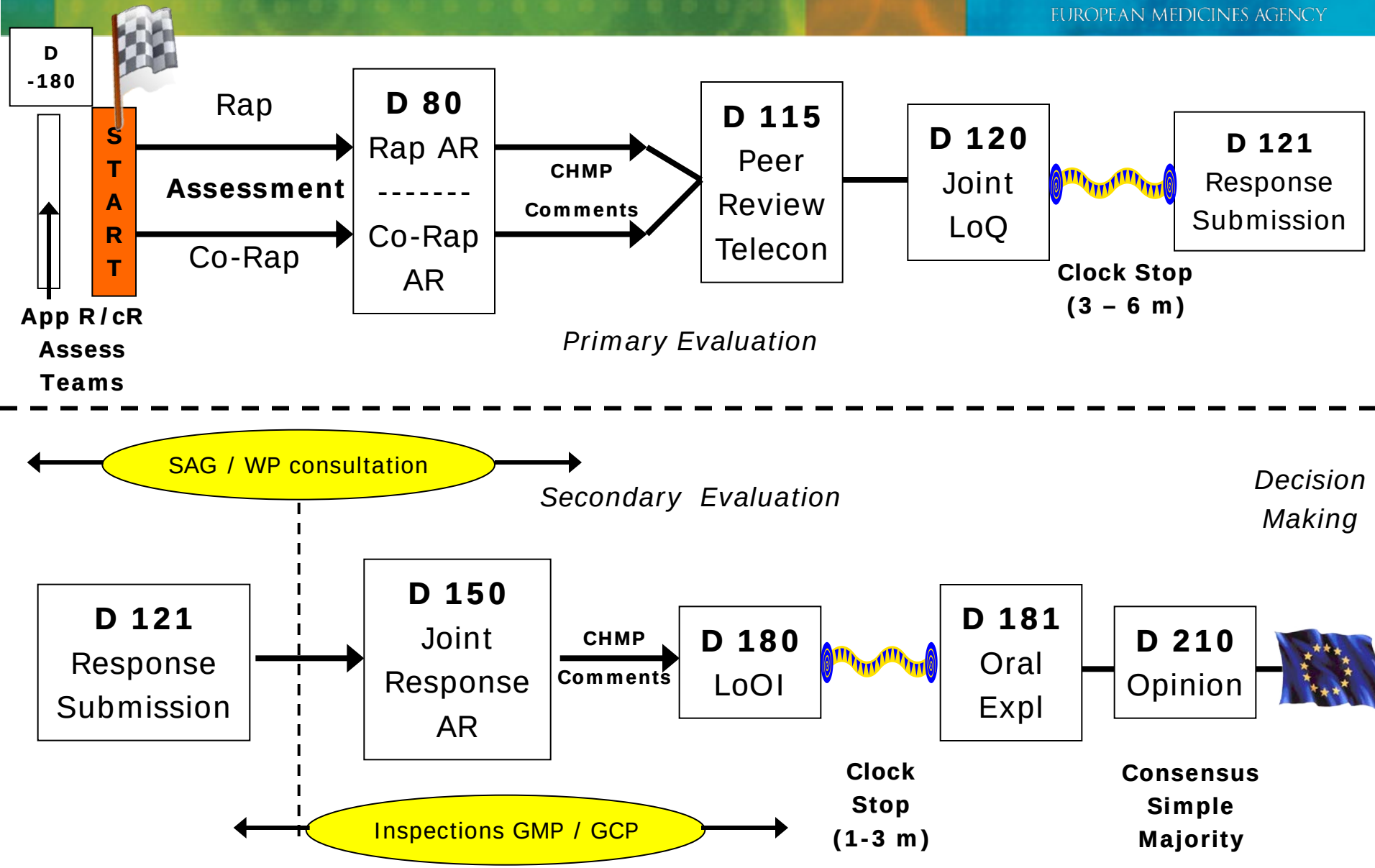
**Art 10 (3)**
Complex “generic”
> 0% - < 100% Non clinical / clinical studies

**Art. 10 (4)**
Biosimilars
“Comparability” QSE data package

# Trends in EU marketing authorisation applications 1995-2012



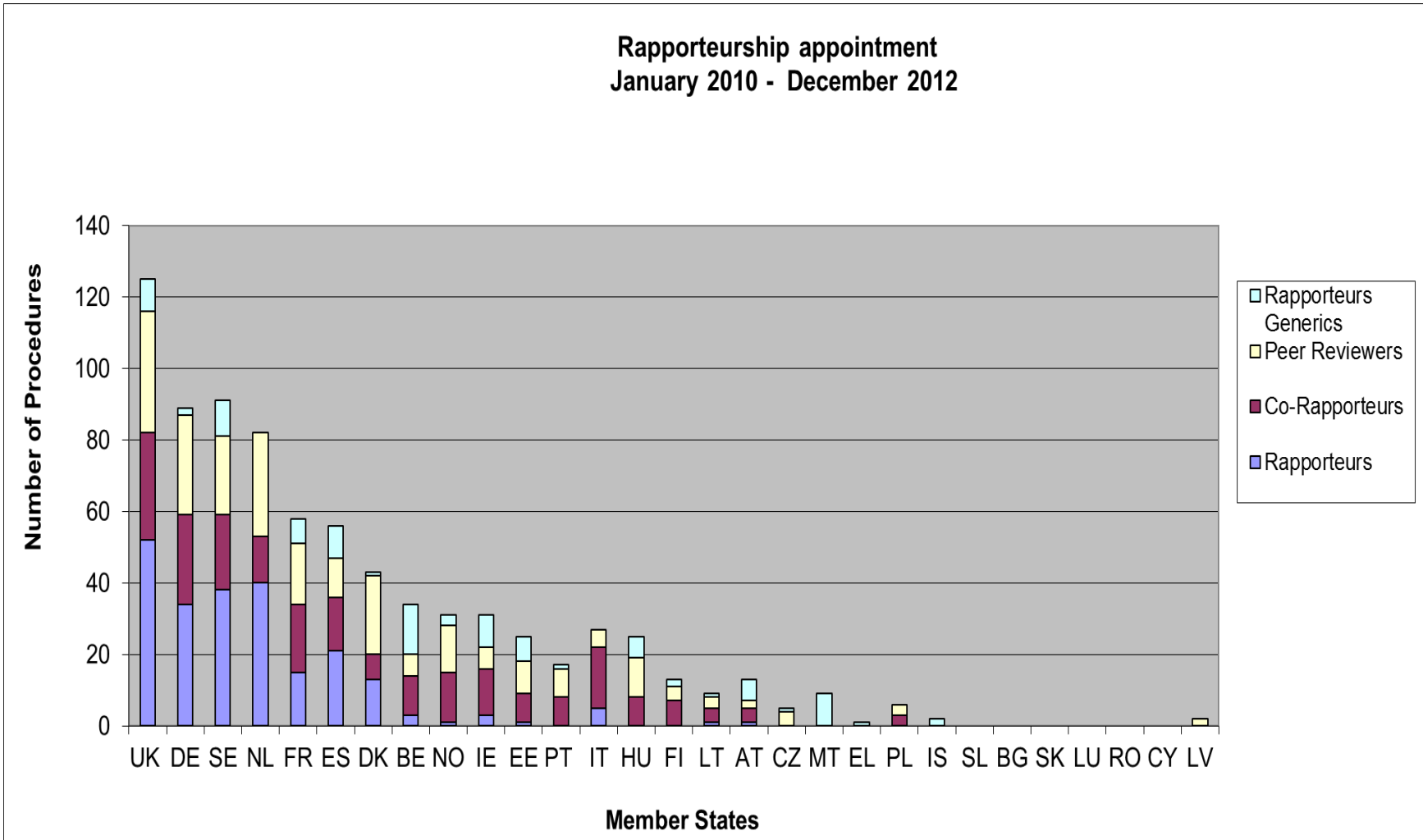
# Centralised Procedure Overview



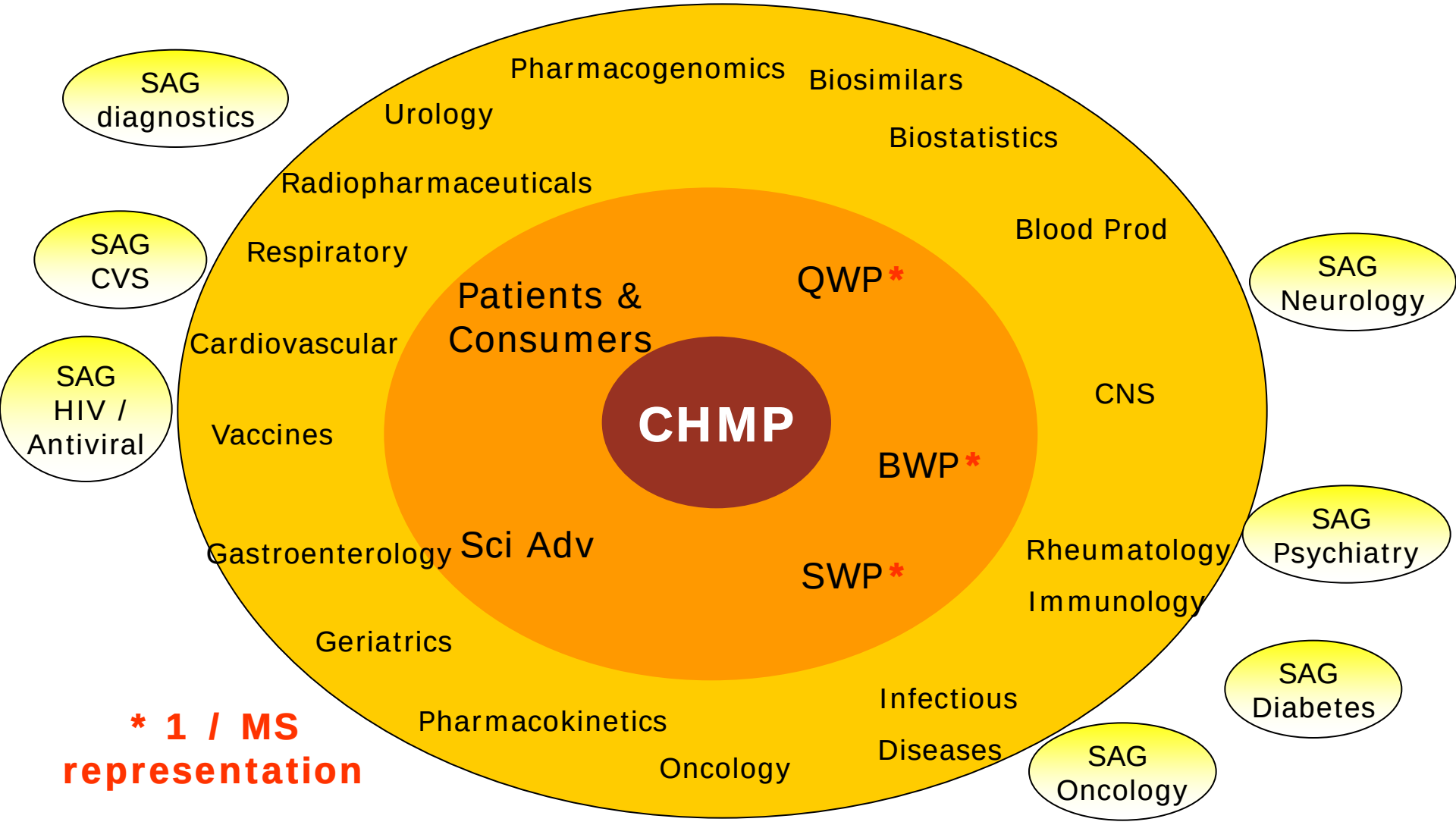
# Accelerated Assessment – Regulation 726 / 2004 Art. 33

In order to meet, in particular, the legitimate expectations of patients and to take account of the increasingly rapid progress of science and therapies, **accelerated assessment** procedures should be set up, reserved for medicinal products of major therapeutic interest, and procedures for obtaining **temporary authorisations** subject to certain annually reviewable **conditions.**

Soliris
Isentress
VPRIV
Pumarix
Zytiga
Incivo
Victrelis
Kalydeco


Rapporteurship appointment
January 2010 - December 2012
Number of Procedures
140
120
100
80
60
40
20
0
UK DE SE NL FR ES DK BE NO IE EE PT IT HU FI LT AT CZ MT EL PL IS SL BG SK LU RO CY LV
Member States
Rapporteurs Generics
Peer Reviewers
Co-Rapporteurs
Rapporteurs

# SAG / Working Party Constellation



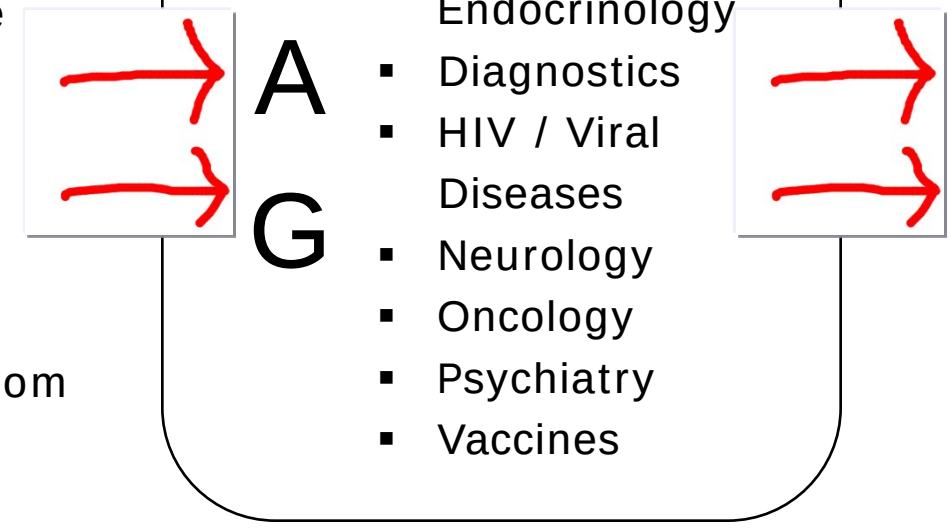

*** 1 / MS representation**

# Scientific Advisory Groups: input

*To deliver independent recommendation to specific questions*

Rapporteur / Co-Rapporteur: present the issue and provide additional information on the dossier

Possible hearing from applicant

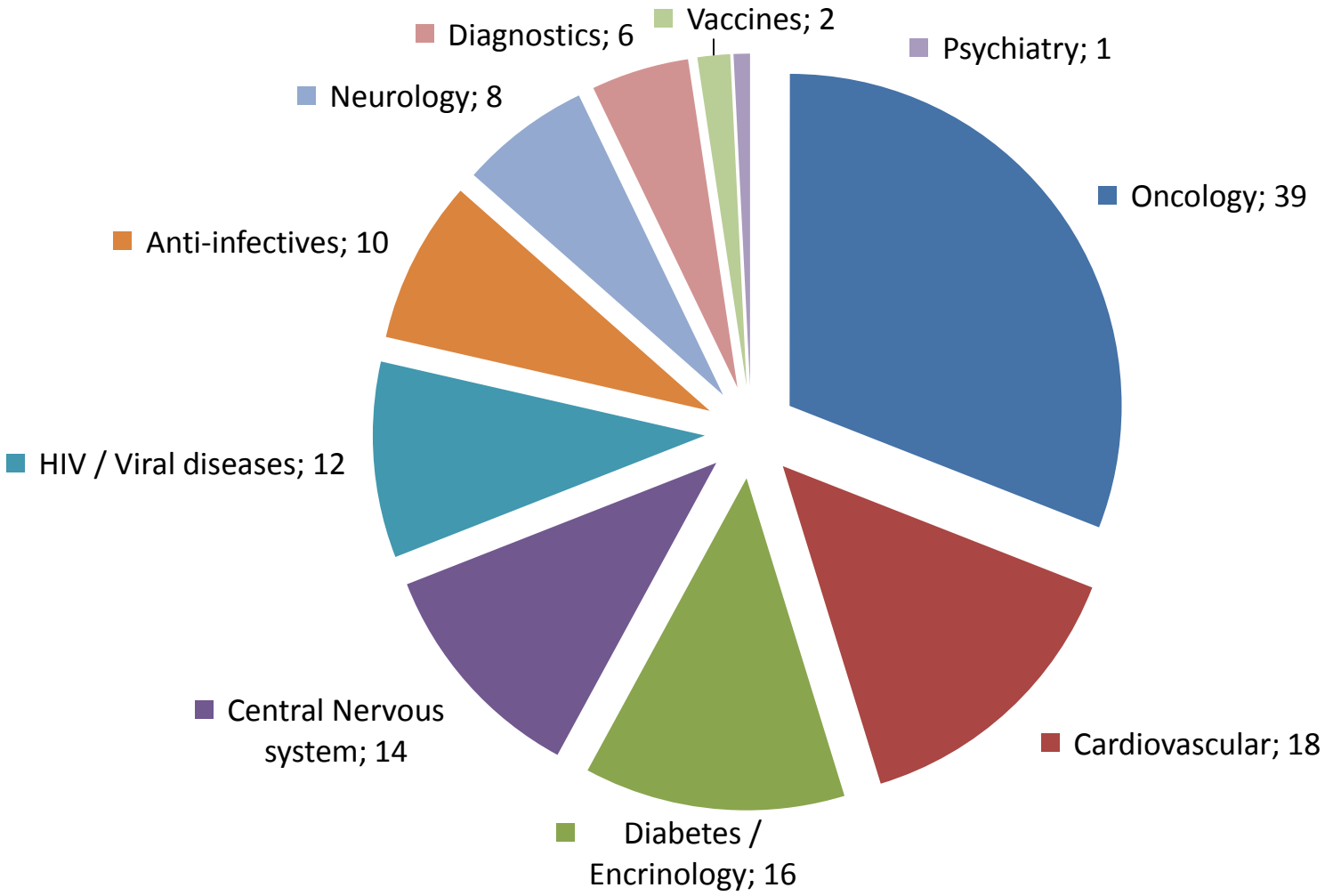



Provides answers to each question

If no consensus: majority views recorded together with any divergent position in document "SAG answers and comments to the CHMP"

Position is reflected in CHMP Assessment Report

# Scientific Advisory Groups (SAGs) 2006 - 2013



# CHMP Voting Rules

32 members eligible to vote (27 MS/NCAs + 5 co-opted)

Norway and Iceland recorded separately


Abstention!

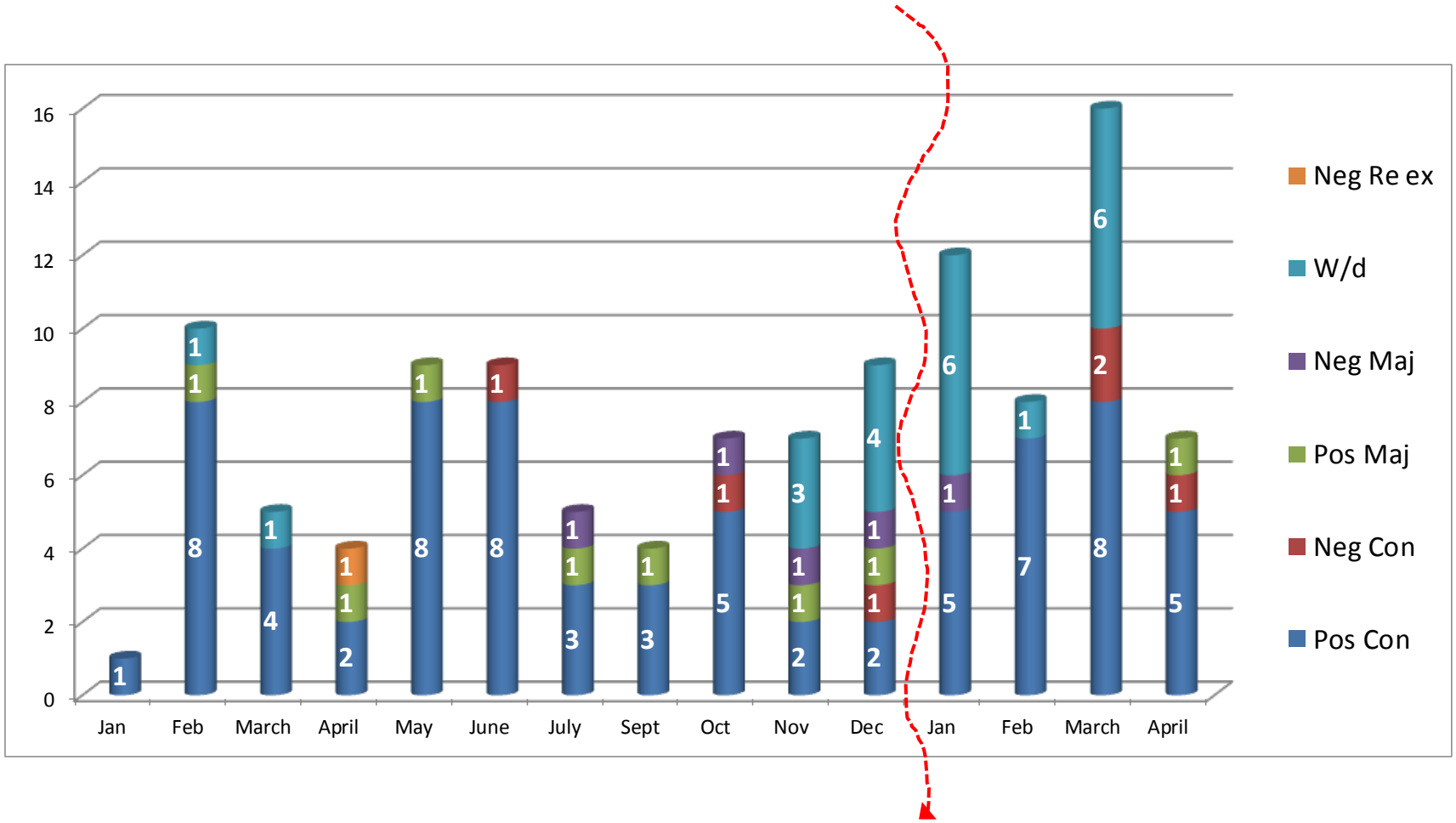
Quorum = 21


Voting

Simple Majority: 17 to sustain a positive or negative opinion

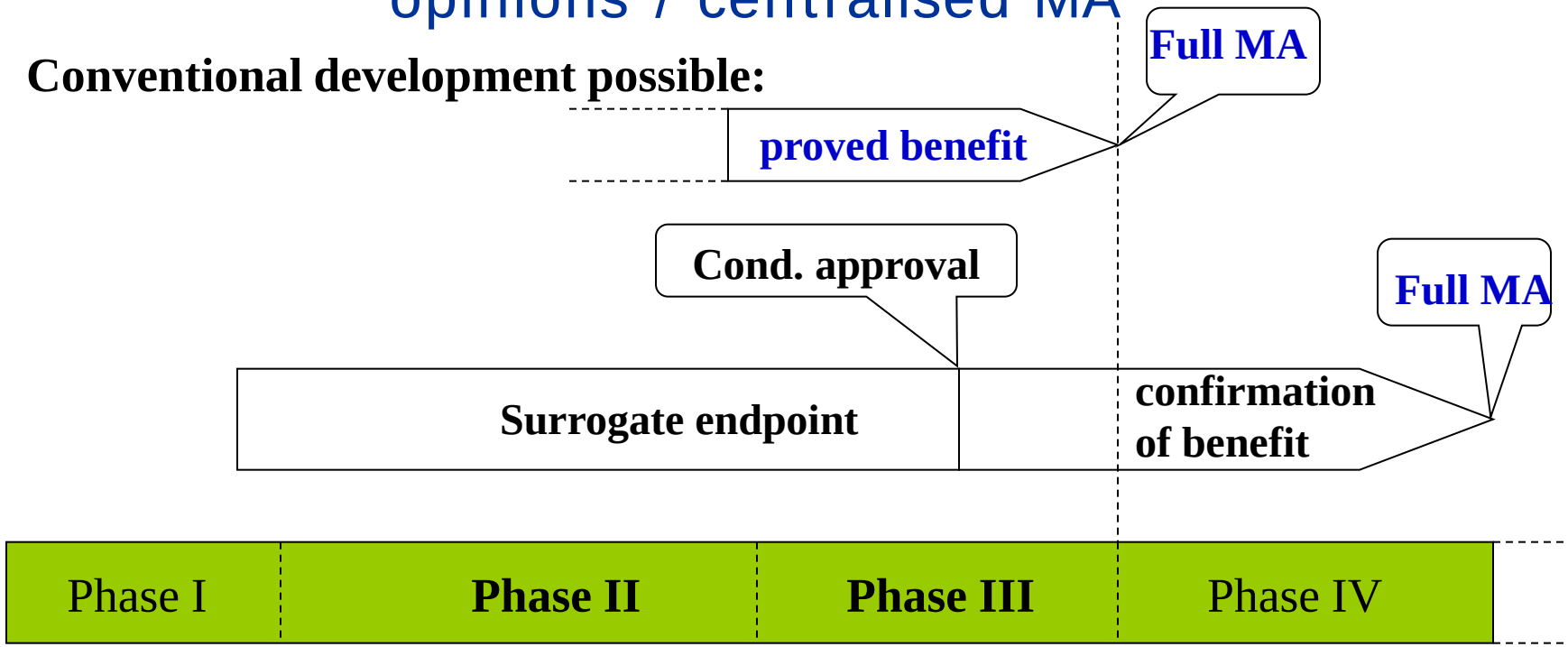
No pre-determined MS position CHMP capacity scientific member, hence vote personal / individual

# CHMP Outcomes 2012 - 2013

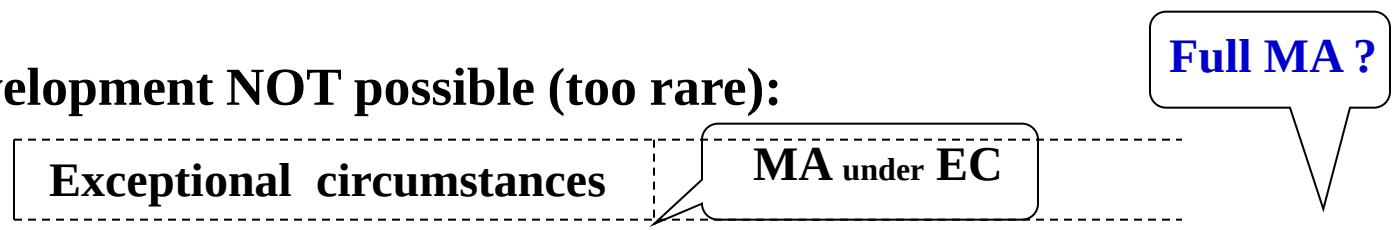


# Drug development and data criteria for different type of opinions / centralised MA

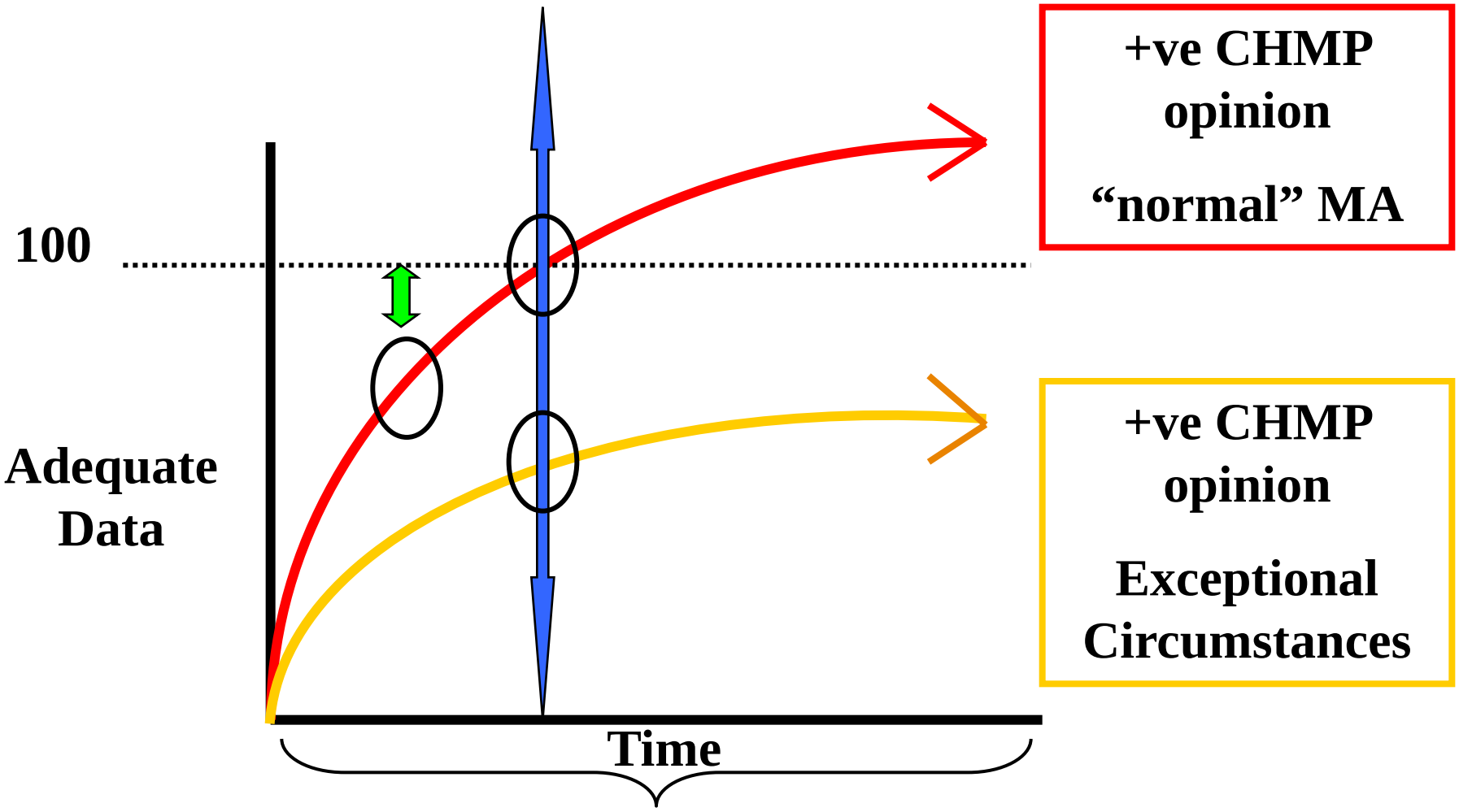
- **Conventional development possible:**



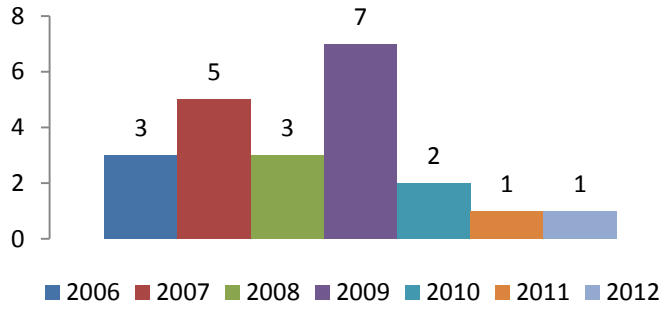

- **Conventional development NOT possible (too rare):**



# Conditional Approval



# Exceptional Circumstances 2006 – 2012




**Anti-Infectives**

Daronix

Focetria

Pandemrix

Celvapan

PIV – H5N1(X2)

Foclivia

Pumarix

**Metabolism**

Elaprase

Vedrop

Orphacol

Glybera

**Blood**

Atryn

**Oncology**

Evoltra

Atriance

Yondelis

Ceplene

Arcalyst

Ilaris

**Hormonal**

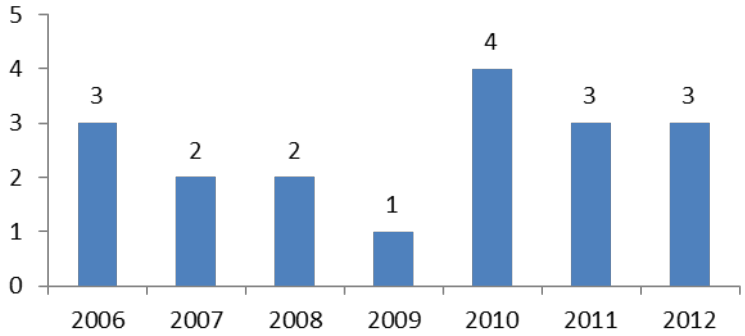
Increlex

**Nervous System**

Zenas

Vyndaqec

# Conditional Approval 2006 – 2012




**Oncology**

Sutent ***Orph***

Vectibix

Tyverb

Arzerra

Votrient ***Orph***

Votubia ***Orph***

Caprelsa

Xalcori

Adcentris

Pixuvri

**Anti-Infective**

Prezista

Isentress

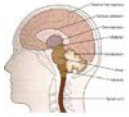
Intelence

Cayston

Arepanrix

Humenza

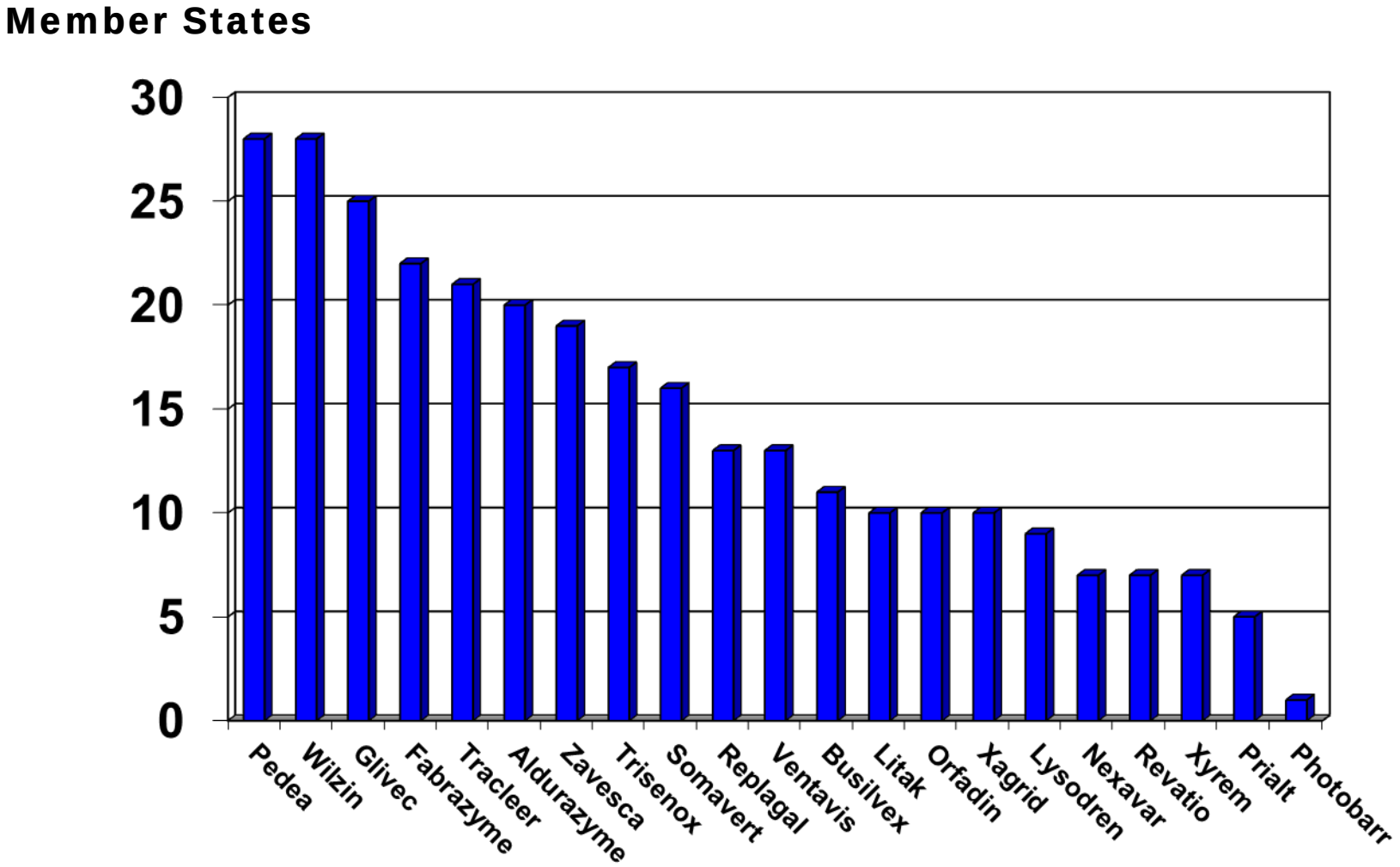
**CNS / NDD**



Diacomit ***Orph***

Fampyra

# Real access to products?



# Article 1

"The provisions of this Regulation shall not affect the powers of Member States' authorities as regards setting the *prices* of medicinal products or their *inclusion* in the scope of the *national health system* or *social security schemes* on the basis of health, economic and social conditions.

In particular, Member States shall be *free to choose* from the particulars shown in the marketing authorisation those *therapeutic indications* and *pack sizes* which will be covered by their security bodies."

## Neudexta

dextromethorphan hydrobromide + quinidine sulphate

- First treatment pseudobulbar affect (uncontrolled emotional expression)
- **CHMP opinion:** +ve by consensus
- **Eligibility:** significant therapeutic innovation
- **Legal basis:** Art. 10(6)

## Sancuso

Granisetron PONV

- Transdermal formulation for patients with difficulty swallowing tablets
- **CHMP Opinion:** +ve by majority
- **Eligibility:** significant innovation / patient interest
- **Legal basis:** Art. 10(3)

## MACI

Matrix induced antologous chondrocyte implantation

- First combined tissue engineered medicine in EU
- **CHMP Opinion:** +ve by consensus based on draft CAT opinion
- **Eligibility:** ATMP
- **Legal basis:** Art. 8(3)

## Imatinib Accord

Imatinib

- Generic of Glivec = CML adults and paediatrics
- **CHMP Opinion:** +ve by consensus
- **Eligibility:** Art. 3(3) Generic CAP
- **Legal basis:** Art. 10(1)


Hvala lijepa.
Imate li kakvo pitanje?