# ICORD 2006

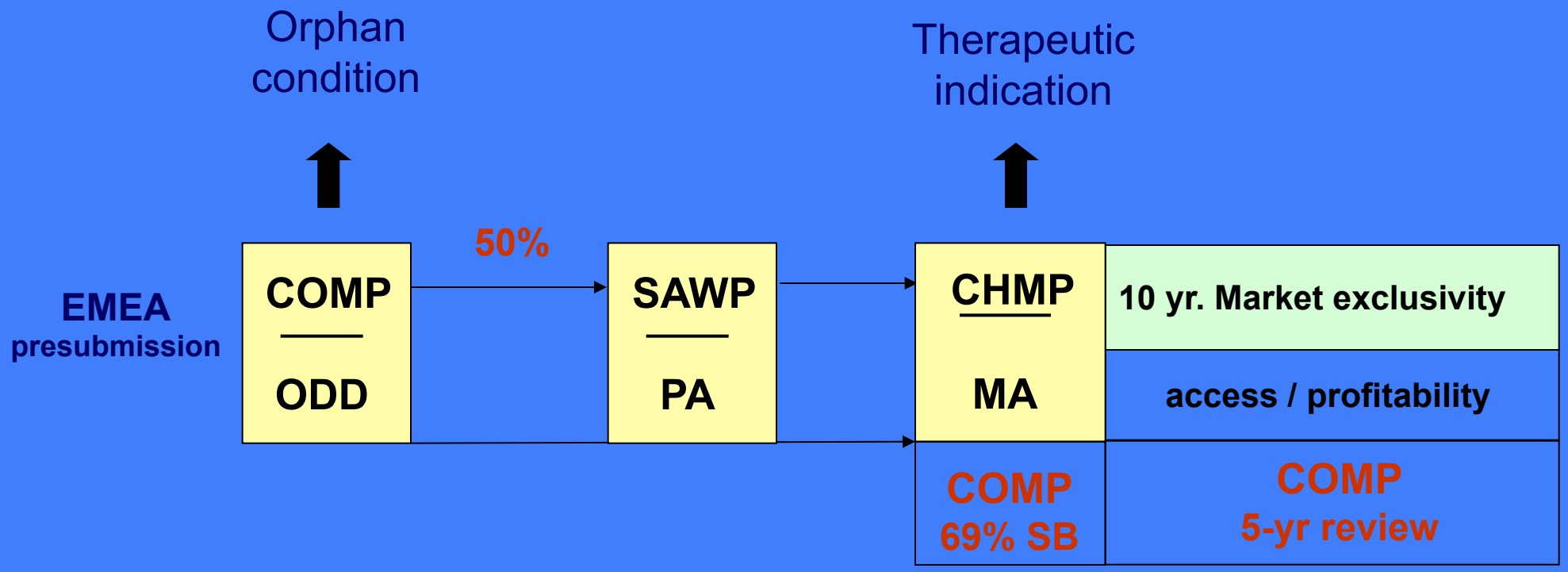
Kerstin Westermark

Md, PhD, Assoc. prof.

COMP Chairperson

# ORPHAN DRUG CONTINUITY POLICY

(From designation to MA and post-authorisation)



# The EU Orphan Legislation

# EU Orphan Regulations

- Regulation (EC) No 141/2000 of the European Parliament and of the Council on Orphan Medicinal Products of 16 December 1999
  - For medicinal products for human use only
  - Not for medical devices
  - Not for food or food supplements
  - Not for medicinal products for veterinary use
- Commission Regulation (EC) No 847/2000 of 27 April 2000

# Committee for Orphan Medicinal Products (COMP)

- COMP
  - 31 members (3 patient representatives) + Chairman
  - Tasks
    - Opinions on designation
    - Advising on general EU policies
    - International co-operation

# Criteria for Orphan Designation

"Prevalence" criterion

Prevalence

(< 5 / 10,000)

Insufficient return on investment

(costs > expected revenues)

"Seriousness" criterion

Life-threatening or chronically debilitating

Life-threatening, seriously debilitating or serious and chronic

Available "methods" for diagnosis / prevention / treatment

"Sign. benefit" criterion

NO

YES

Significant benefit / non satisfactory

# Incentives for Designated Products

## Main EU Incentives

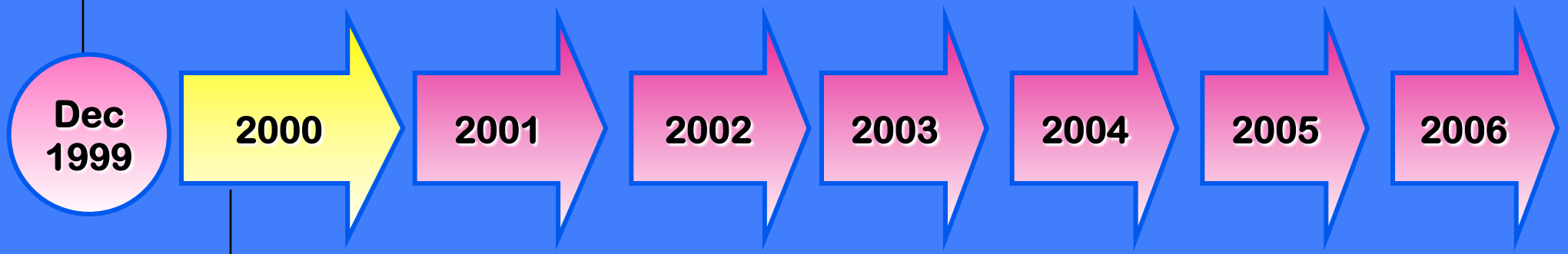
- Ten years exclusivity from the date of marketing authorisation
- Protocol assistance from the EMEA
- Direct access to Centralised Procedure
- Fees reduction for centralised applications
- Priority access to EU research programs

## National Incentives

- Inventory published on Commission Web-site

# Orphan Designation

# Major Milestones




Apr 00

1st Meeting of COMP

Regulation 847/2000 adopted

1st Application for Orphan Designation received on 28 April

Aug 00

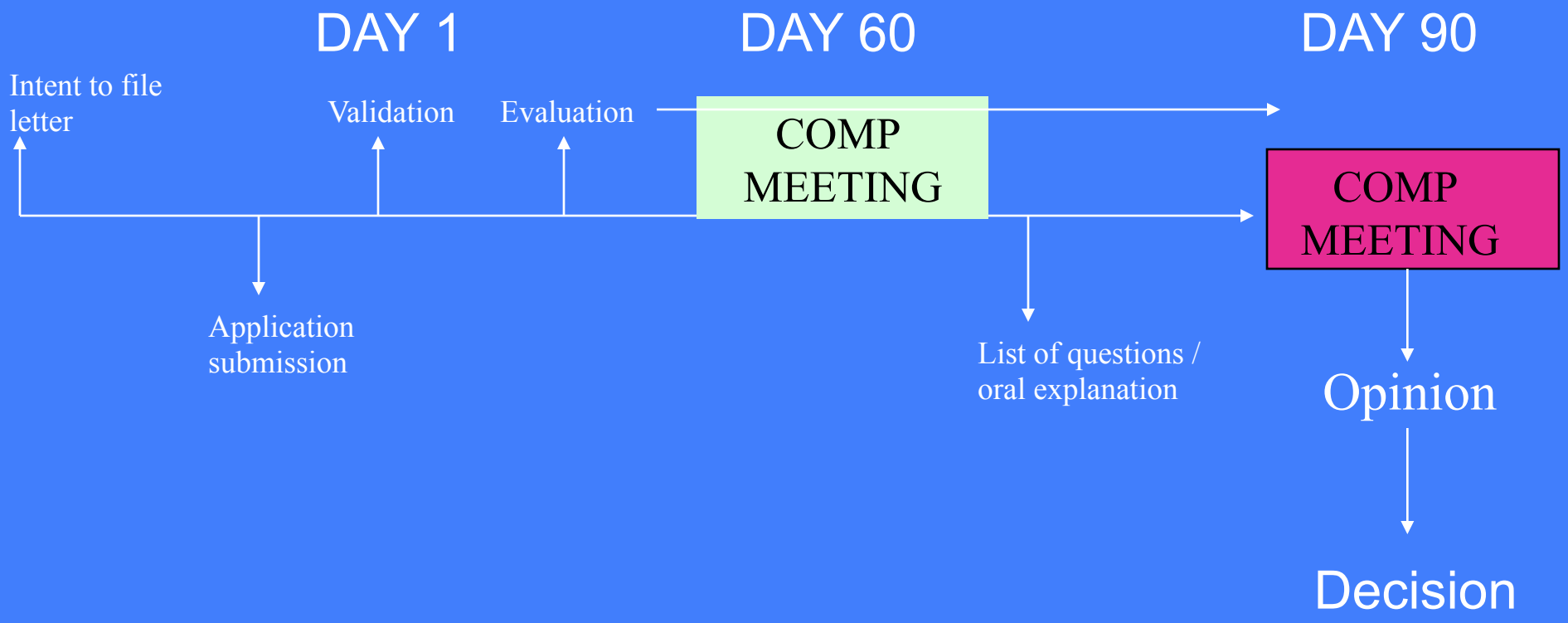
1st Orphan Medicinal Products Designated

Aug 01

1st Orphan Medicinal Products Authorised

401 OMP Designated and 30 OMP Authorised Oct 06

# Procedure for Designation

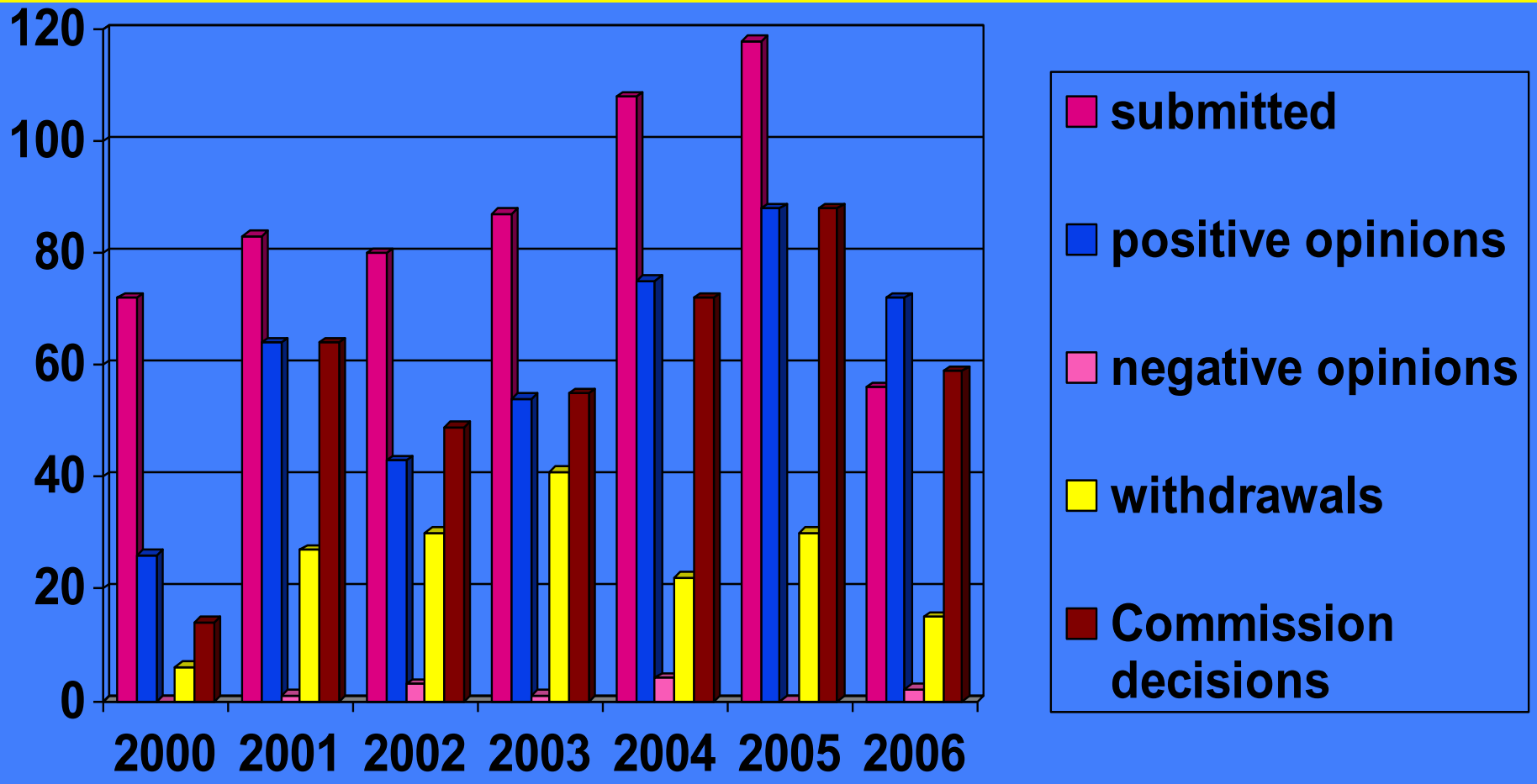


# Status of Orphan Applications

| | 2000 | 2001 | 2002 | 2003 | 2004 | 2005 | 2006 | Total |
|---|---|---|---|---|---|---|---|---|
| No. of applications submitted | 72 | 83 | 80 | 87 | 108 | 118 | 86 | 634 |
| Positive COMP Opinions | 26 | 64 | 43 | 54 | 75 | 88 | 72 | 422 |
| Commission Decisions | 14 | 64 | 49 | 55 | 72 | 88 | 59 | 401 |
| Final Negative COMP Opinions | 0 | 1 | 3 | 1 | 4 | 0 | 2 | 11 |
| Withdrawals | 6 | 27 | 30 | 41 | 22 | 30 | 15 | 171 |

Up to 17 October 2006

# Status of Orphan Applications




Up to 17 October 2006

# Protocol Assistance for OMP

# Protocol Assistance

**Protocol Assistance (PA) = Scientific Advice (SA)**

**(Article 6 of Regulation (EC) No 141/2000)**

- Sponsor can ask questions on
- Quality aspects
- Preclinical development
- Clinical development
- Significant benefit
- Regulatory aspects

# NEW FRAMEWORK FOR SA & PA
## Legal requirements

New regulation of European Parliament and Council Regulation (EC) 726/2004 - **Main key aspects for SA**

- Scientific Advice Working Party
- Modernised structures allowing the development of advice for companies, in particular, small and medium sized enterprises
- **More** general and in-depth **SA**
- **SA for the development of** new therapies
- **Contacts with in particular** patient organisations and health-care professionals' **associations**

# NEW FRAMEWORK FOR SA & PA Procedure

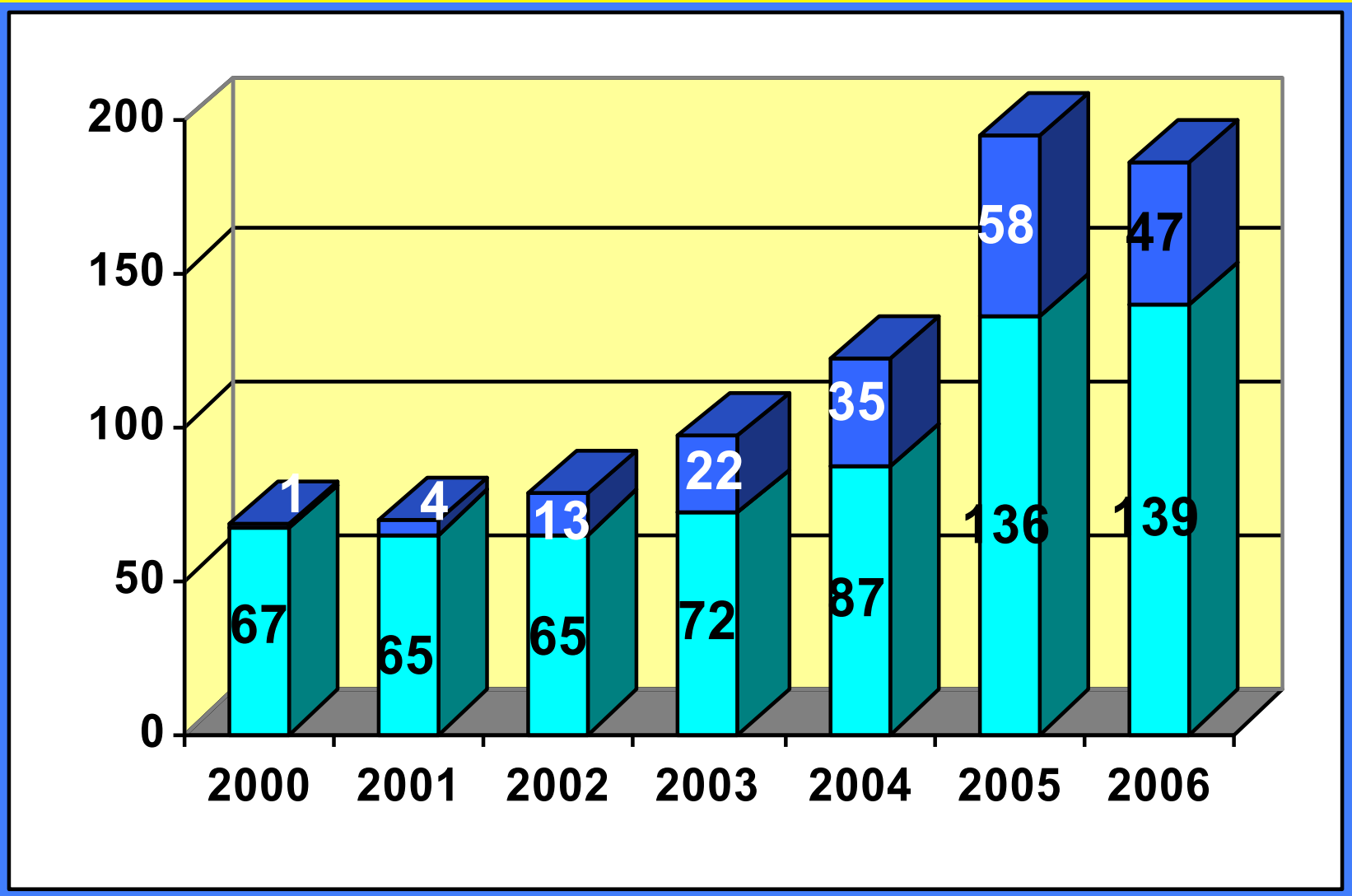
- **A streamlined 70-day procedure with possible finalisation in 40 days**
- **Planning phase with:**
  - Earlier appointment of coordinators/experts
  - Presubmission meeting (optional) to:
    - Receive early feedback
    - Increase quality of request before start of procedure
    - Recommended for first time users of SA, for Protocol assistance, for SMEs, for SA on “specific types of medicinal products and therapies”, and “broad and more general advice”.

# Summary of New Framework for SA / PA

- Extended scope to provide also broader and more general advice
- New faster procedure with involvement of experts already in the pre-submission phase
- Broad definition of follow-up
- Increased transparency and communication

# Scientific Advice / Protocol Assistance

## Procedures (Sept 06)



# Guidance Documents

- Guideline on format and content of applications for designation as orphan medicinal products and on the transfer of designations from one sponsor to another

  /ENTR/6283/00

- EMEA guidance for companies requesting scientific advice (SA) and protocol assistance (PA)

  EMEA-H-4260-01-Rev.3

# Guidance Documents (cont)

- Points to consider on the calculation and reporting of the prevalence of a condition for orphan designation EMEA/COMP/436/01
- Guideline on the elements required to support the medical plausibility and the assumption of significant benefit for an orphan designation (EMEA/COMP/66972/2004)
- Guideline on clinical trials in small populations CHMP/EWP/83561/2005

# Orphan Drug Achievements in the EU – Information

- COMP report to the Commission in relation to Article 10 of Orphan Regulation 141/2000 on Orphan Products

  **EMEA/35218/2005 Final**
- General report on the experience acquired with the application of Regulation (EC) No 141/2000 on orphan medicinal products during the first five years of publication

  http://ec.europa.eu/enterprise/pharmaceuticals/orphanmp/doc/orphan_en_06-2006.pdf
- 7/9/06: Inventory of Community and Member States' incentive measures to aid research, marketing, development and availability of orphan medicinal products. *Revision 5*

  http://ec.europa.eu/enterprise/pharmaceuticals/orphanmp/doc/inventory_2006_08.pdf