Biomarkers at the EMEA
New initiatives
Expectations
15 Dec 2006

Bruno FLAMION, MD, PhD
Chair, Scientific Advice Working Party (SAWP)

15 Dec 2006

- **Biomarkers as a tool**
- **Conditional approval**
- **How to interact with regulators (now – then)**
- **Innovative Medicine Think Tank Group**
- **“Briefing meetings” concept**
- **Looking at the future**
- **Acknowledgment**

Biomarkers are a tool for focusing drug development programs

… and understanding diseases

Not all biomarkers will turn out as surrogate endpoints (especially across-products endpoints)

Biomarkers at SAWP

- **Oncology:**
  - **RR**
  - **PSA**
  - **Gene signatures**
  - **Other markers of progression and severity**
- **Proteinuria, kidney size**
- **Cardiovascular BMs**

Conditional approval (1)

Conditional approval

A strange mixture of concepts

→ A flexible tool?

Conditional approval (2)

A strange mixture of concepts…

1. Seriously debilitating or life threatening diseases (or orphan medicinal product)
2. Unmet medical need
3. Presumed positive B/R balance:
   - surrogate marker?
   - interim analysis?
   - population/biomarker-selective approval?
4. Fast access
5. Specific Obligations = further studies

Conditional approval (3)

Conditional approval:

1. Based on biomarker: what is the likelihood that outcome data will be confirmatory?
2. An incentive to demonstrate added benefit of NCE
3. Should it be extended?

Industry's views on EMEA

From the Innovative Drug Development Think-Tank Group meetings:

- Scientific interactions with EU regulators are slow, impersonal and formal

EMEA think-tank group

Innovative drug development

- Review Strategic Research Agenda (EC 7th Program)
- Hearings with big and small pharma (12-2005 to 06-2006) on evolving science, new technologies, new methodologies
- Hearings with academia (09-11 2006)
- Internal consultations
- Public conclusions: ~ Spring 2007

PGx Working Party – Briefing Meetings

Main topics discussed

1. Chosen design and rationale for genomic data
2. Population selected for PG studies (variables related to phenotype)
3. Size of selected population ; power to detect association
4. Statistical methodology ; corrections for multiple testing
5. Positive and negative predictive values of PG biomarkers (clinical trials experience)
6. Assumptions on clinical utility (benefit in using predictive PG testing *vs* other predictive biomarkers ; use of PG biomarker as segregation marker)

Looking at the future (1)

EMEA – Industry – Academia collaborative work (on biomarkers):

Clear goals and focus

A framework

→ Representativeness

→ SAWP – Ad Hoc Working Groups – SAGs - CHMP

Looking at the future (2)

Regular discussion fora / briefing meetings

Extended conditional approval (?)

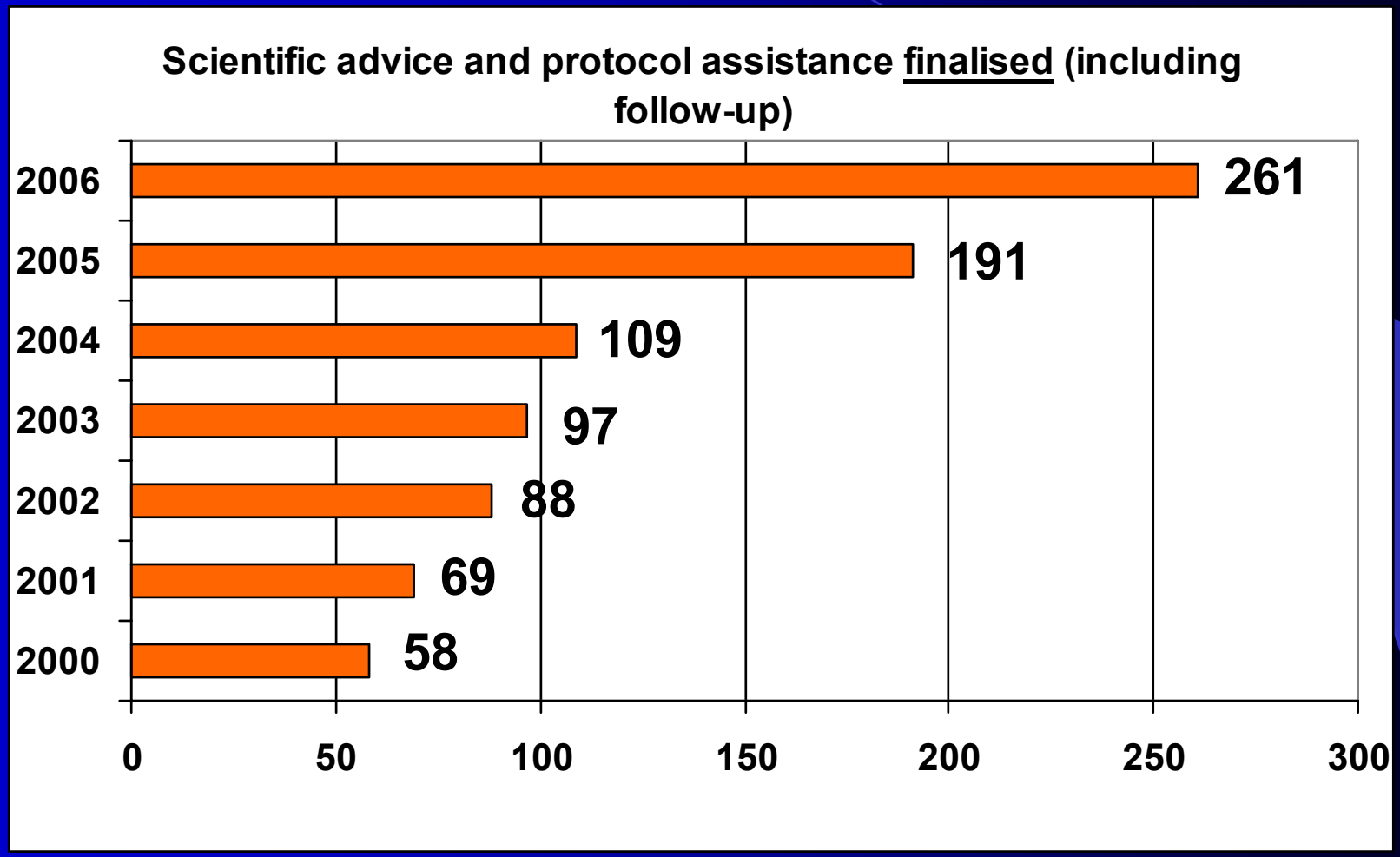
Life cycle approach

Thank you to the EMEA Scientific Advice Working Party

Spiros VAMVAKAS, MD, PhD
Acting Deputy Head of Sector

… and to EFPIA

Number of Scientific Advices



SAWP composition

Angeles Alonso Garcia
Fernando de Andrés Trelles
Minne Casteels
Dieter Deforce
Pierre Demolis
Hans-Georg Eichler
Jens Ersbøll
Christine Gispen-de Wied
Bertil Jonsson
Sheila Killalea
Andrea Laslop
N.
N.
Sif Ormarsdóttir
Hans Ovelgönne
Markku Pasanen
Mira Pavlovic
Cristina Sampaio
Christian Schneider
Beatriz Silva Lima
Stefano Vella
John Warren

COMP members

Brigitte Blochl-Daum
Rembert Elbers
Kerstin Westermark

Observers