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# The role and importance of European Scientific-Regulatory advice mechanisms – incentives for SME's and academics

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

**Prof. Dieter Deforce**
University Gent, Belgium
Faculty of Pharmaceutical Sciences
Laboratory for Pharmaceutical Biotechnology
Member of SAWP of EMA

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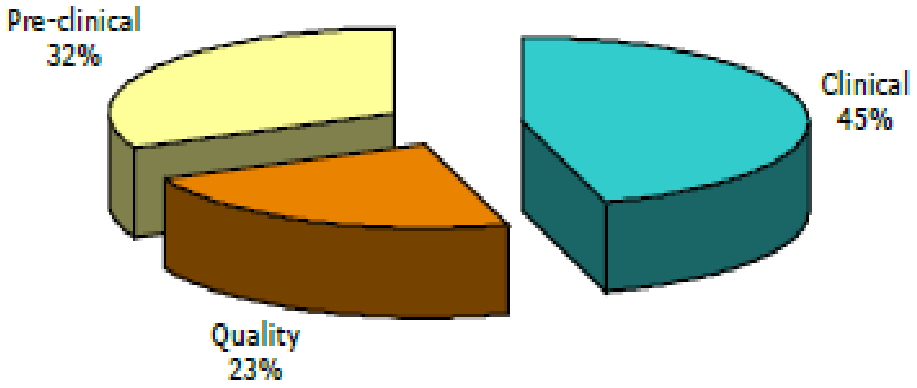
# The role and importance of **European <u>Scientific</u>**-Regulatory **<u>advice</u>** mechanisms – incentives for SME's and academics

# Scientific Advice

- Scientific Advice:
  - Strictly confidential
  - During any stage of development
  - On all aspects of the development
    - Quality
    - Non-clinical
    - Clinical



# Scientific Advice

- Scientific Advice:
  - Where no guidance exists (or can be interpreted differently)
  - Where deviation from guidance is sought
  - Short process 40-70 days

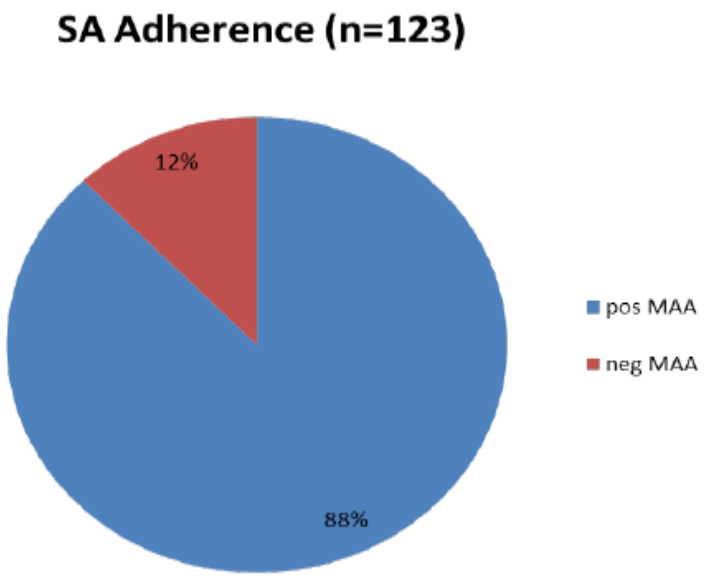
# SAWP: How does it work?

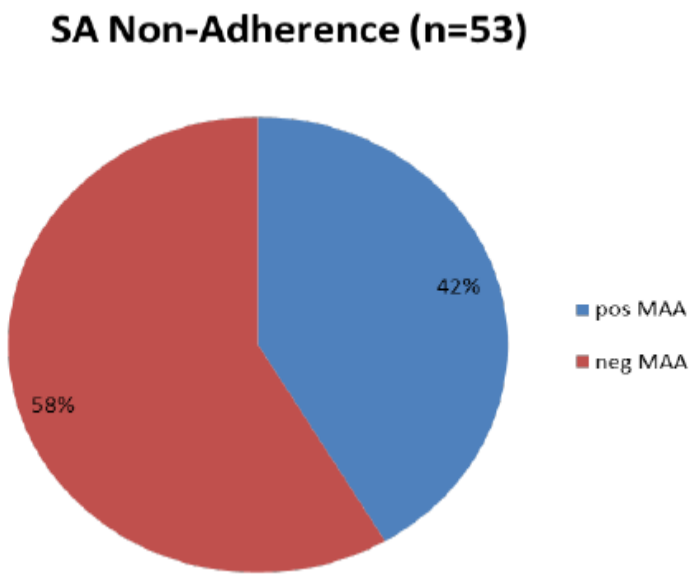
- 2 coordinators from Scientific Advice WP
  - Internal and external experts
- Involvement of:
  - Committee for Advanced Therapies
  - Quality WP
  - Biologics WP
  - Vaccine WP
- Possibility for Discussion Meeting
- Discussion in SAWP and CHMP

# Recommendations

- **The Sc Ad procedure is NOT:**
  - a pre-evaluation of the dossier to be submitted
  - For getting an approval or assessment of the quality of a product for clinical trials => National competences
- **Quality of the responses** provided is largely dependent upon
  - the relevance and quality of the question(s) put
  - and the documentation provided to support the Company position

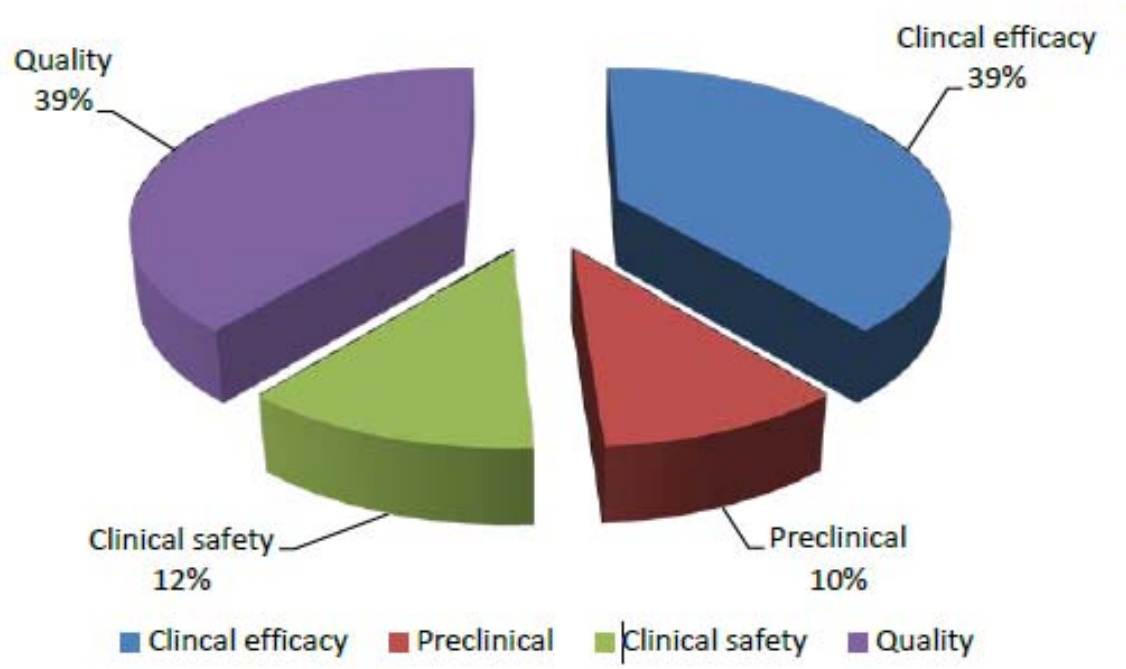
# SA adherence and impact on MAA outcome



# Major objections in MAA

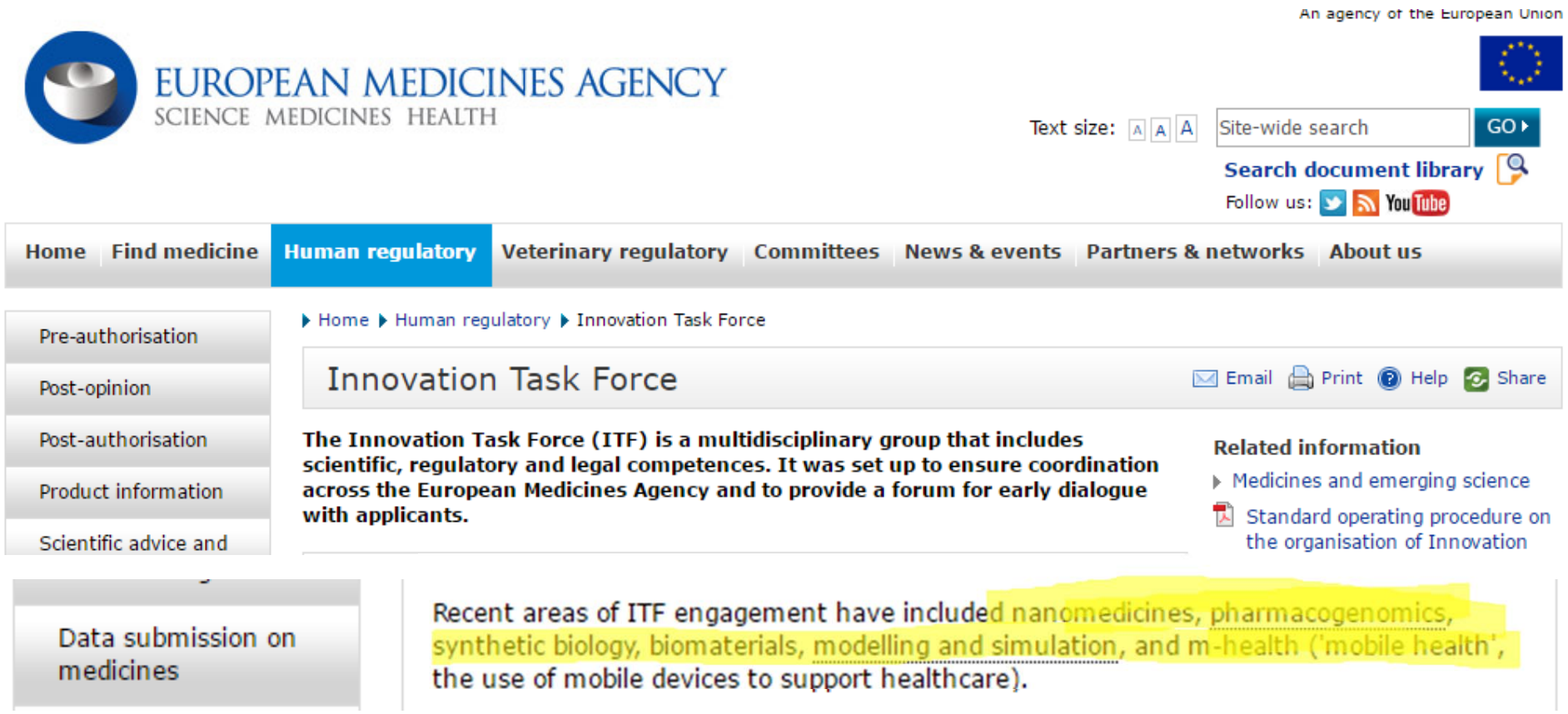
Overall success rate for SMEs 53% vs 78% for all companies



# Joint Scientific – HTA advice

- Health Technology Assessment (reimbursement)
  - Early involvement of HTAs
  - Important for planning clinical trials
    - Endpoints
    - Comparators
- Discussion meeting with SAWP and HTAs

# Innovation Task Force



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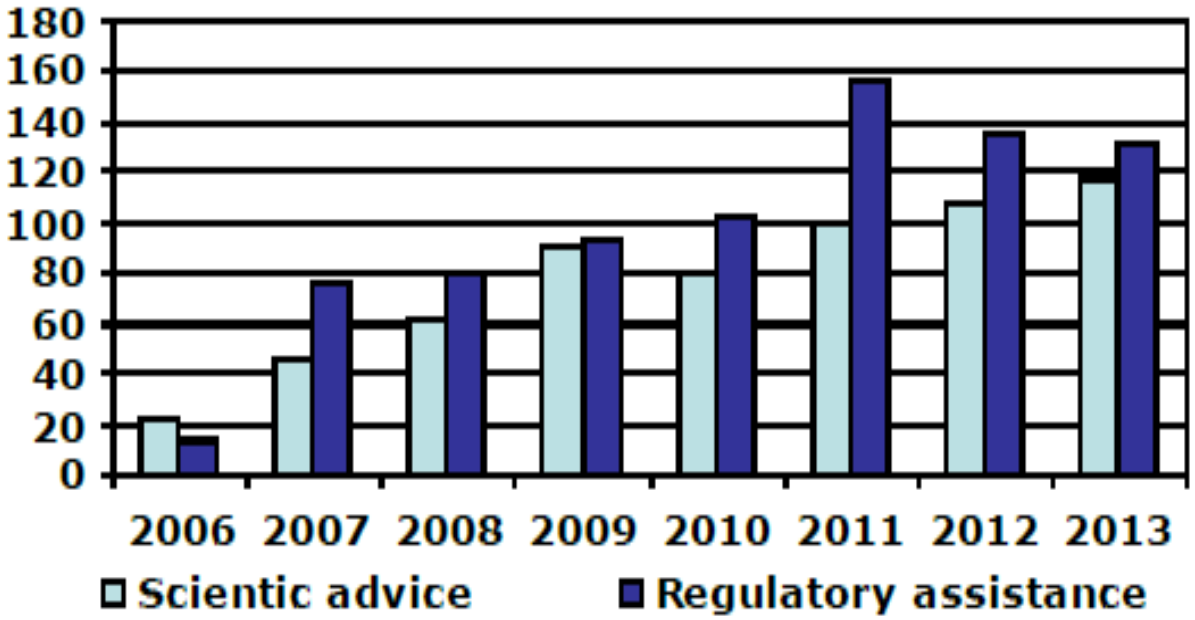
# Guidelines


www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000043.jsp&mid=WC0b01ac
Apps
Web of Knowledge
Eudralink
Aanbevolen websites
Web Slice-galerie
Customize Links
Safe Search
DEZE PAGINA IS VEILIG
SAFE IS GESLOTEN
DELEN VIA FACEBOOK
An agency of the European Union
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH
Text size:
Site-wide search
GO
Search document library
Follow us:
Home
Find medicine
Human regulatory
Veterinary regulatory
Committees
News & events
Partners & networks
About us
Pre-authorisation
Post-opinion
Post-authorisation
Product information
Scientific advice and protocol assistance
Home ▸ Human regulatory ▸ Scientific guidelines
Scientific guidelines
Email
Print
Help
Share
The European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) prepares scientific guidelines in consultation with regulatory authorities in the European Union (EU) Member States, to help applicants prepare marketing-authorisation applications for human medicines. Guidelines provide a basis for practical harmonisation of how the EU Member States and the Agency interpret and apply the detailed requirements for the demonstration of quality, safety and efficacy that are in the Community directives.
Related information
Search for scientific guidelines
The rules governing medicinal products in the European Union
Directive 2001/83/EC
Procedure for European Union

# SME office – Regulatory advice

- **Direct assistance**:
  - Queries dealt with by SME office: e-mail/telecon
  - Briefing meetings/telecon on regulatory strategy
- Published **SME user guide** on regulatory procedures

# SME SA – RA


180
160
140
120
100
80
60
40
20
0
2006
2007
2008
2009
2010
2011
2012
2013
Scientic advice
Regulatory assistance

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# Fee Reductions for SMEs

90% reduction on :

- **scientific advice**
- inspections
- scientific services

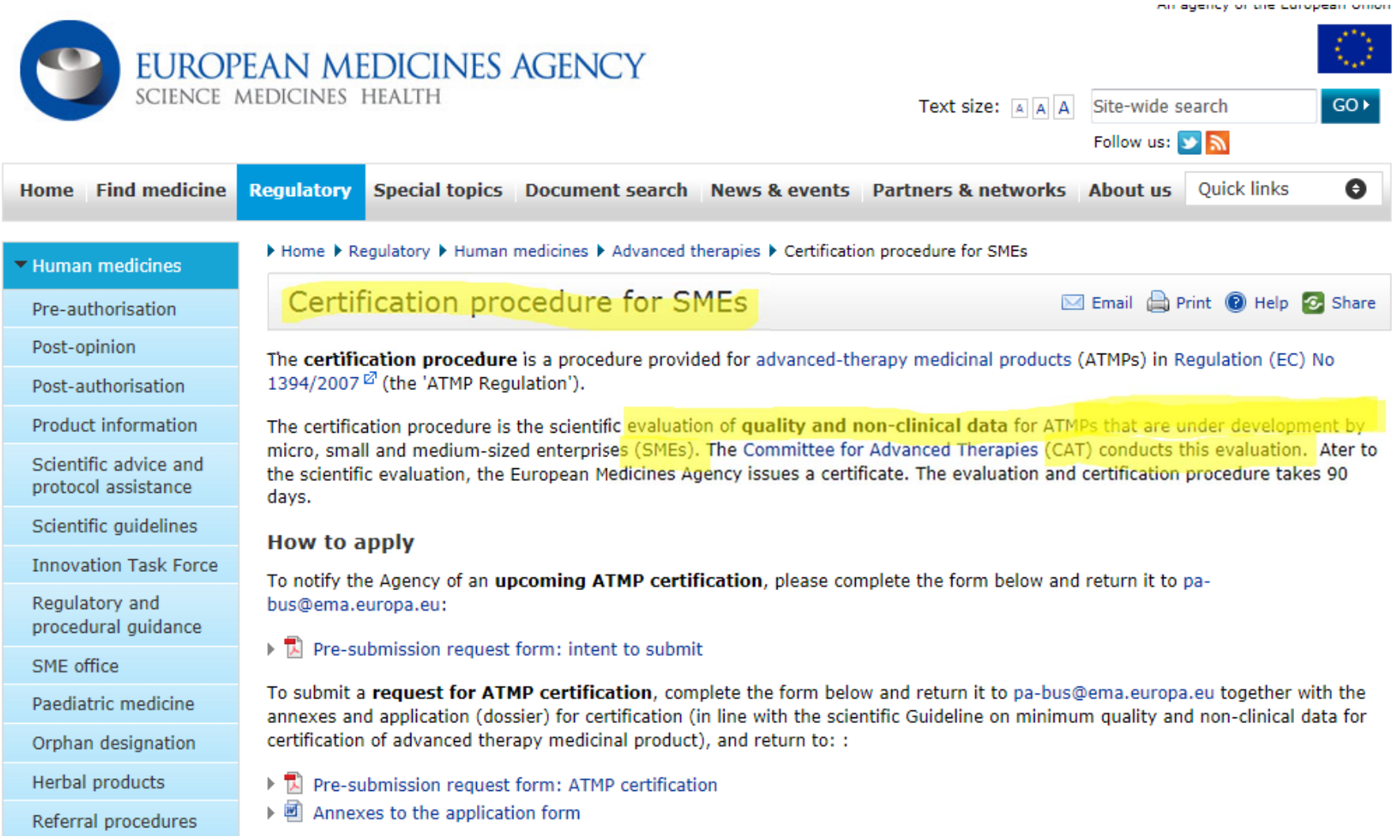
# Tailoring assistance to SMEs

To promote innovation and development of new medicines by SMEs

SME Office:

- SME assignment, public SME register
- **regulatory assistance**
- Translations of product information
- Facilitate communication

# **Certification** of Quality / Non-clinical data for ATMP



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# PRIME - PRIORITY MEDICINES


## Special support for SMEs and academia

Micro-, small- and medium-sized enterprises (SMEs) and applicants from the academic sector can apply for PRIME at an earlier stage of development when they have compelling non-clinical data and tolerability data from initial clinical trials. They may also request a fee waiver for scientific advice.

# PRIME

- Medicines addressing Unmet Medical Need
- Preliminary data must be available
- **Major** therapeutic advantage!
- Early
  - appointment of CHMP/CAT rapporteur
  - Interaction, SAWP
  - Earlier on the market?

# Topics of special interest for SMEs:

## -Personalised Medicine

## -Biomarkers

Disease areas
Transparency
Releasing clinical-trial data
Antimicrobial resistance
Safety monitoring of medicines
Medicines for children
Clinical trials
Medicines for rare diseases
Advanced therapies
Falsified medicines
Medicines for older people
Generic medicines
Biosimilar medicines
Regulatory science
Medicines and emerging science
Nanotechnology
Pharmacogenomics and personalised medicine



# Pharmacogenomics and personalised medicine



**Pharmacogenomics** is the study of how the variability of the expression of genes between people leads to differences in susceptibility to disease and responses to medicines.

Research into pharmacogenomics began following observations that not all people respond in the same way to the same medicines. This revealed that differences between individuals is caused by their genetic profile, at least in part.

Pharmacogenomics is an important new technology that promises to optimise the use of medicines, by targeting medicines to patient's individual genes. This is called '**personalised medicine**'. Many pharmaceutical companies are also now integrating pharmacogenomics into their development strategies for medicines.

## Activities at the European Medicines Agency

The European Medicines Agency has a Pharmacogenomics Working Party, a group of European experts in pharmacogenomics that gives recommendations to the Committee for Medicinal Products for Human Use (CHMP).

The Agency also hosts meetings on pharmacogenomics and related areas:

- Report to the Committee on Proprietary Medicinal Products on the European Medicines Agency seminar on the use of pharmacogenetics in the drug development process - 5 June 2000
- European Medicines Agency/European Federation of Pharmaceutical Industries and Associations workshop: Integrating pharmacogenomics early into drug Development: Pharmacokinetics as a working example (18/12/2008)
- European Medicines Agency workshop on pharmacogenomics: from science to clinical care (08-09/10/2012)

The Agency also publishes scientific guidelines on pharmacogenomics. These intend to help companies design and carry out studies in pharmacogenomics.

**Related information**

- Pharmacogenomics Working Party
- Scientific guidelines: Pharmacogenomics

**How useful is this page?**

**Average rating:** Based on 3 ratings

**Add your rating:**

# Biomarkers

- **QUALIFICATION OF NOVEL METHODOLOGIES FOR DRUG DEVELOPMENT**
  - **CHMP Qualification Opinion**
    - **public**
  - **CHMP Qualification Advice on future protocols and methods for further method development towards qualification**
    - **confidential**
  - **Possibility to apply in parallel to the EMA and FDA**

Disease areas
Transparency
Releasing clinical-trial data
Antimicrobial resistance
Safety monitoring of medicines
Medicines for children
Clinical trials
Medicines for rare diseases
Advanced therapies
Falsified medicines
Medicines for older people
Generic medicines
Biosimilar medicines
Regulatory science
Medicines and emerging science
Nanotechnology
Pharmacogenomics and personalised medicine
Biomarkers



# Biomarkers



The European Medicines Agency pays close attention to research into the use of **biomarkers** in the **development of medicines**.

Biomarkers are tests that can be used to follow body processes and diseases in humans and animals. They can be used to predict how a patient will respond to a medicine or whether they have, or are likely to develop, a certain disease. For example, the levels of chemicals in the fluid surrounding the brain may be able to predict the likelihood that a patient with mild memory problems will go on to develop dementia due to Alzheimer's disease.

Biomarkers are playing an increasingly important role in the development of new medicines. The Agency expects that their use in research will contribute to **faster public access** to new medicines.

## Activities at the Agency

On request, the Agency can give an opinion on the **qualification** of the use of a biomarker, to indicate its acceptability for a specific use in pharmaceutical research and development.

▸ For more information, see qualification of novel methodologies and biomarkers.

The Agency has also concluded a joint qualification process for biomarkers together with the United States Food and Drug Administration (FDA). The qualification followed submission of data by the Predictive Toxicology Consortium (C-Path PSTC) of pharmaceutical companies, and qualified the use of seven biomarkers of drug-induced kidney toxicity in preclinical drug development.

▸ See the final conclusions on the pilot joint EMEA/FDA VXDS experience on qualification on of nephrotoxicity biomarkers.

The Agency has hosted two workshops on biomarkers in the development of new medicines:

▸ 2006 EMEA/EFPIA workshop on biomarkers
▸ 2005 EMEA/CHMP workshop on biomarkers

**How useful is this page?**

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### Related information

▸ Scientific advice and protocol assistance
▸ Qualification of novel methodologies and biomarkers
▸ Scientific guidelines: pharmacogenomics
Final conclusions on the pilot joint European Medicines Agency / Food and Drug Administration VXDS experience on qualification of nephrotoxicity biomarkers (22/01/2009)

# Closing Remarks

- Consult available guidance, procedural and scientific
- **Early Scientific** advice is strongly encouraged
- Address any regulatory, procedural and administrative questions to SME office