An aerial photograph of a large, intricate hedge maze made of green box hedges, with narrow dirt paths winding through it. The maze is the background for the entire slide.

# **1<sup>st</sup> EMEA Workshop on Small and Medium-sized Enterprises (SMEs)**

***“Navigating the Regulatory Maze”***

**EMA  
2 February 2007**

**Marisa Papaluca Amati, MD  
Safety and Efficacy Sector  
Pre-Authorisation Unit**





# Innovation

## Emerging Therapies and Technologies

**The EU political initiatives:** Pharmaceutical and health care industries cornerstone for the industrial EU competitiveness and for public health objectives (Lisbon 2000)

**The EU legal tools:** Implemented pharmaceutical legislation, Paediatric Medicines' legislation, Draft Regulation on Advanced Therapy medicinal products, Review of the Directive on Medical Devices

**The new EU 7<sup>th</sup> research framework:** IMI (2007-2013) – proposal for strategies aimed at the development of new and more effective medicines – Nano Strategic Research Agenda [*SMEs in focus*]

### **The EMEA Road Map to 2010:**

Long term commitment in support of innovation in liaison with stakeholders

→ Stimulation of research and innovation in the EU's pharmaceutical, biotechnology and healthcare industries, leading to the development of an adequate product development toolkit, able to address the bottlenecks during the development of innovative medicines



# **Innovation in Pharmaceuticals Objectives**

- Encourage creativity out of stagnation in the pharmaceutical arena**
- Expedite the development of drugs**
- Increase the rate of success of new developments**
- Provide for more efficient and safe targeted therapies (move towards diseases modifiers)**



# **Innovation**

## **Emerging Therapies and Technologies**

- **What steps are being taken in EU to facilitate the development and acceptance of new therapies and technologies?**
- **Which are the opportunities and benefits of the international collaboration?**
- **What are the next steps?**



# **Innovation**

## **Emerging Therapies and Technologies**

- **What steps are being taken at the EMEA to facilitate the development and acceptance of new therapies and technologies e.g. by SMEs?**
- **Create “hot spot” services**
  - **For general regulatory co-ordination, administrative and financial support: Establishment of the SMEs office**
  - **For science debate/exchange of views: establishment of the ITF**
  - **For bottlenecks identification and resolution recommendations: Innovation Think-tank**



# Innovation

## Emerging Therapies and Technologies

### EMA specific initiatives

Establishment of the **EMA Innovation Task Force** (2001) to facilitate early contacts with Sponsor (**EU soft landing zone**), to identify emerging science and technologies with potential regulatory impact and discuss informally bottlenecks and opportunities offered in the system





# Innovation

## Emerging Therapies and Technologies

### EMEA specific initiatives

#### EMEA Innovation Task Force (ITF)

Multidisciplinary permanent EMEA transversal group, including scientific, regulatory and legal competences

Complemented as required by additional specialised expertise (e.g. therapeutic group leaders, working parties, ad-hoc experts)

Open channel for knowledge acquisition and exchange [internal and, as appropriate, with stakeholders]



# EMA Innovation Task Force (ITF)

Emerging Therapies and Technologies: Introduction - Microsoft Internet Explorer

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Back Forward Stop Reload Home Search Favorites

Address <http://www.emea.europa.eu/htms/human/itf/itfintro.htm> Go Links

Google Search 74 blocked Check AutoLink AutoFill Options

European Medicines Agency **emea** New website address: [emea.europa.eu](http://emea.europa.eu) | Home | Sitemap | Links | help |

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## Emerging Therapies and Technologies

[Introduction](#)

[Areas of interest and available guidelines](#)

[How to get support from EMEA?](#)

[Related information sources](#)

### Introduction

In this part of the EMEA website you will find information on activities ongoing at the EMEA to support scientifically sound development of emerging therapies, new technologies and borderline and combination products, so that their benefits might timely be made available for public health.

To contribute to this objective the EMEA established the Innovation Task Force (ITF), a multidisciplinary group including scientific, regulatory and legal competences ensuring the EMEA-wide coordination in the areas of interest, and to provide a forum for early dialogue with applicants ([click here for ITF mandate](#)).

The EMEA ITF, within eight weeks from receipt of a request from the applicant, arrange (free of charge) briefing meetings, to facilitate informal exchange of information early in the development process, to provide early guidance and information, in liaison, as appropriate, with EMEA scientific committees, working parties and expert groups, also taking into account ongoing international activities.

Briefing meetings are also meant to complement and reinforce existing formal regulatory procedures (e.g. designation of orphan medicinal products, CHMP scientific advice etc).

Upon request, the ITF in liaison with the Scientific Committee and, where appropriate with the European Commission, provides Regulatory Advice on the eligibility to the EMEA procedures as new medicinal products for emerging new therapies and borderline products. This advice is provided free of charge within 60 days from the receipt of a valid request from applicants ([click here for list of submission dates](#)).

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Send all queries regarding the Web content to: [info@emea.europa.eu](mailto:info@emea.europa.eu)  
Send all queries regarding the Web functionality to: [EMEAweb services](#)

### Contact Point...

Please send all queries in writing to  
[ITFsecretariat@emea.europa.eu](mailto:ITFsecretariat@emea.europa.eu)

For further contact information see [Contact points](#)

Start Local intranet 13:51

Microsoft PowerPoint... Emerging Therapie...



<http://www.emea.europa.eu/htms/human/itf/itfintro.htm>





# Innovation

## Emerging Therapies and Technologies

### ITF tasks in support to Sponsors

- **Briefing meetings [SOP/H/3044** <http://www.emea.europa.eu/pdfs/human/sop/SOP3044.pdf> ]

Arranged with ITF members, these meetings are meant to be an informal exchange of information early in the development process, to provide early guidance and information, in liaison, as appropriate, with EMEA scientific committee, working parties and expert groups

**Provide a new path for informal meetings to facilitate exchange of information at various stages of development and new voluntary processes to complement and reinforce existing procedures**

- **Regulatory advice on eligibility to the EMEA procedures as a medicinal product [SOP/H/3138** <http://www.emea.europa.eu/pdfs/human/sop/SOP3138.pdf> ]

Sponsors may request advice on whether their product can be considered a medicinal product, in that case being eligible for EMEA procedures. A classification report describing the scientific and regulatory criteria for the definition of a medicinal product is drafted by ITF members and finalised and adopted by the main scientific committee for the evaluation of human medicines, the CHMP, as appropriate



# Regulatory Advice (Classification) requests

Regulatory advice is provided *before access to EMEA procedures* (e.g. scientific advice, orphan medicinal product designation and marketing authorisation procedures) in the following cases:

- When there are **uncertainties** on whether the concerned therapeutic product(s) would qualify as medicinal product(s)
- **Borderline products**, having characteristics belonging to diverse legal frameworks, e.g. medicines and medical devices, food supplements
- For medicinal substances incorporated in medical devices for which the **ancillary functions are borderline**



# Regulatory Advice (Classification) procedure

- **Process**, deadlines and details publicly available (<http://www.emea.europa.eu/htms/human/itf/itfsupport.htm> )
- **A valid request** should provide sufficient information
- **Sponsors to make their “homework”** identifying the basis for the positioning of their product

**EMA co-ordinator**: member of ITF nominated to:

- Validate the request received by the sponsor
- Verify if precedents in the experience of the EMA
- Prepare the draft report on the advice reviewing the scientific arguments supporting the request for regulatory advice (including consultation of the ITF)



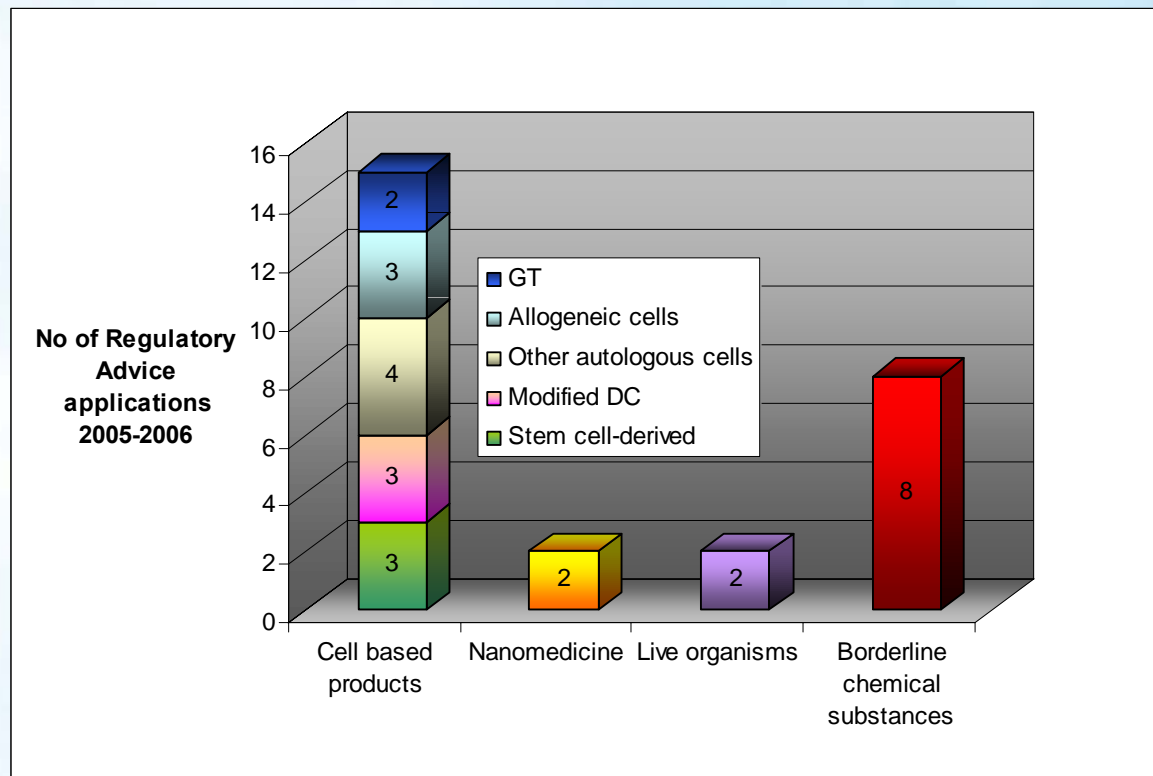
# **Regulatory Advice (Classification) procedure**

**CHMP co-ordinator(s)** appointed when there is not clear-cut precedent case and there is a need of an in depth scientific discussion at CHMP level

**Builds on the draft preparatory work of the ITF:**

- Commenting the draft report
- Leading the forthcoming internal discussions at CHMP level
- Leading the discussions in case of Oral explanations
- Finalising in conjunction with the EMEA coordinator the conclusions

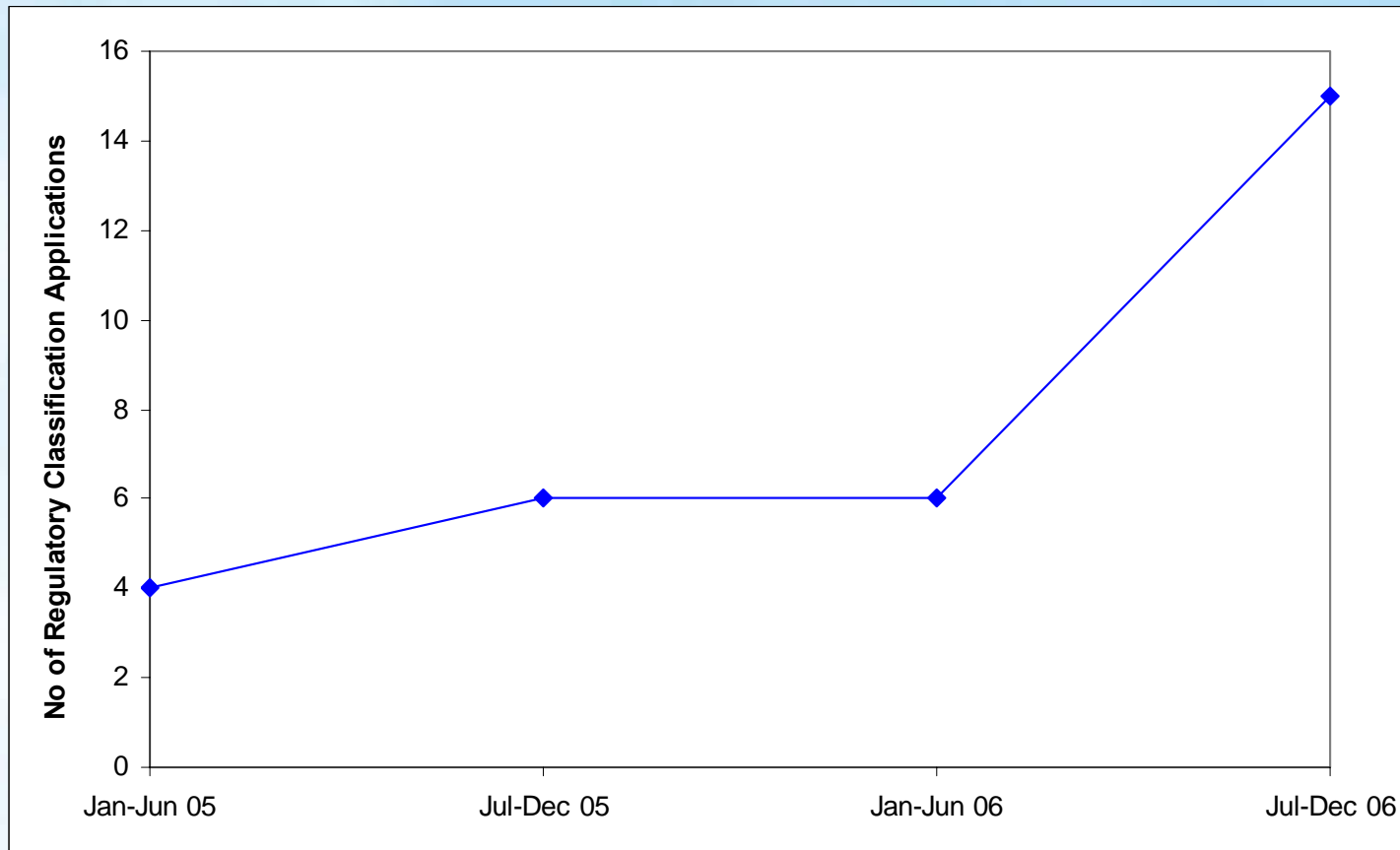
# Product categories under Regulatory Advice



- ✓ Cell-based products represent the majority of products in Regulatory Classification requests
- ✓ Cell-based gene therapy products indicate an emerging trend in new therapies
- ✓ Borderline Chemical substances for innovative diagnostic/therapeutic strategies
- ✓ Nanomedicine, and in particular, therapeutic nanoparticles as one of the newest cancer-specific treatments



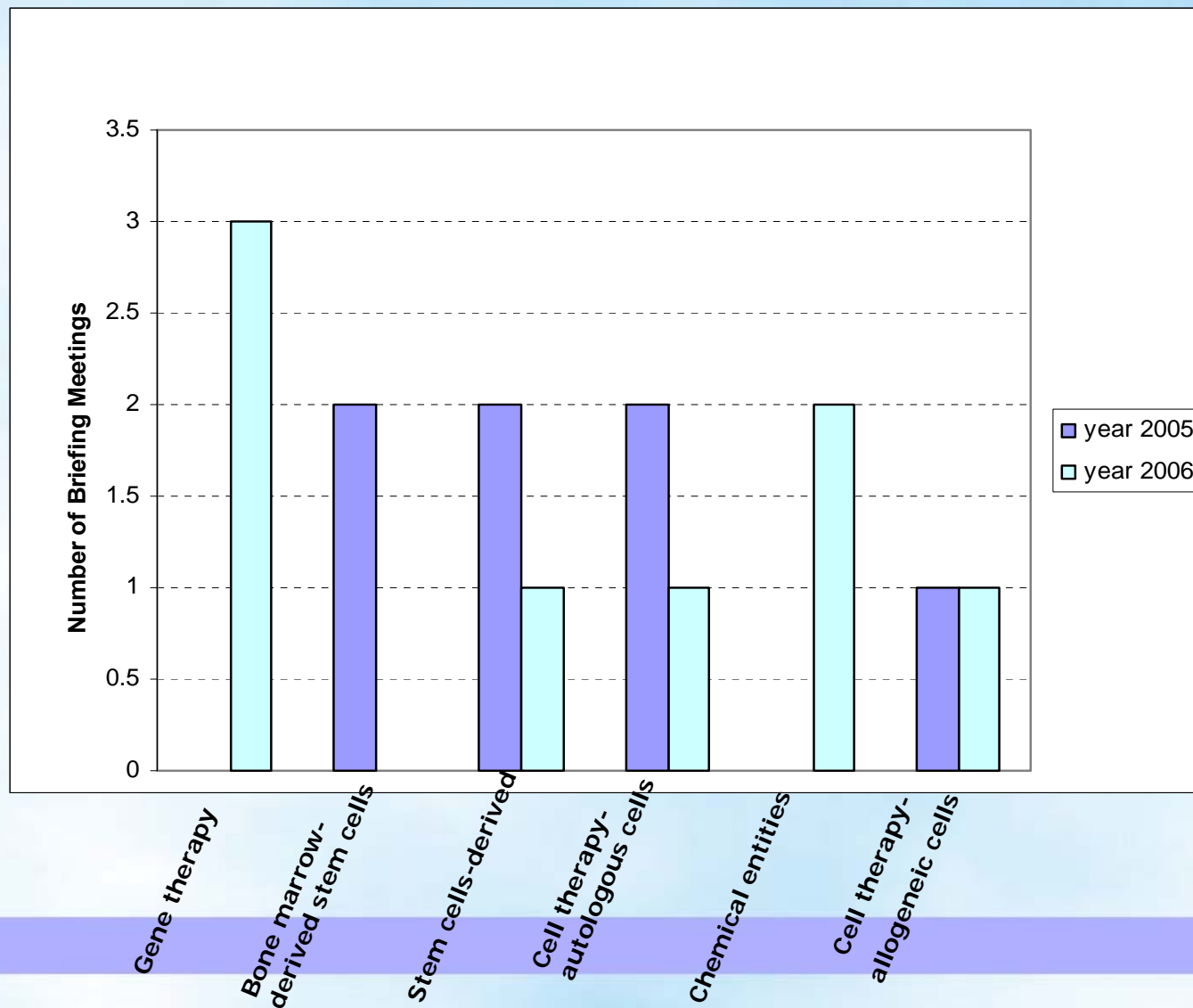
# ITF REGULATORY ADVICE



Graph shows the increasing number of Regulatory Classification applications on advanced and emerging therapies received by the ITF during the last two years.



# Briefing Meetings with sponsors





# **Innovation Emerging Therapies and Technologies**

## **EMEA specific initiatives**

- Establishment of specialised working parties (e.g. Gene therapy working party, Cell-based Products Working Party, Pharmacogenomics working party)
- Establishment of the EMEA **Innovation Think-Tank** (2004) steering EMEA/CHMP activities on Innovation



# **Innovation**

## **Emerging Therapies and Technologies**

### **EMA specific initiatives at international level**

- International workshops putting together regulators and stakeholders to discuss emerging issues**
- Establishment the pilot EMA/FDA confidentiality arrangements (2004): confirmed March 2006**
- Contribution to ICH opening to new sciences (e.g. Gene therapy and Pharmacogenomics)**





# **Innovation**

## **Emerging Therapies and Technologies**

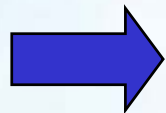
- **EMA Road Map and FDA critical path initiative pointing in the same direction: each region implementing new opportunities, also jointly**
- **FDA/EMA confidentiality arrangements already proved to be a suitable forum for discussing new developments. Further Joint activities being explored**
- **ICH positive approach to innovative areas (e.g. Gene therapy, Pharmacogenomics)**

# **Innovation**

## **Emerging Therapies and Technologies**

### **CONCLUSIONS**

- **Positive general EU environment for innovation**
- **New processes, competences and scientific panels established at the EMEA: SMEs Office and ITF as entry points**
- **Mechanisms for Regulatory input in to joint academia/Industry platforms being developed within the EU**



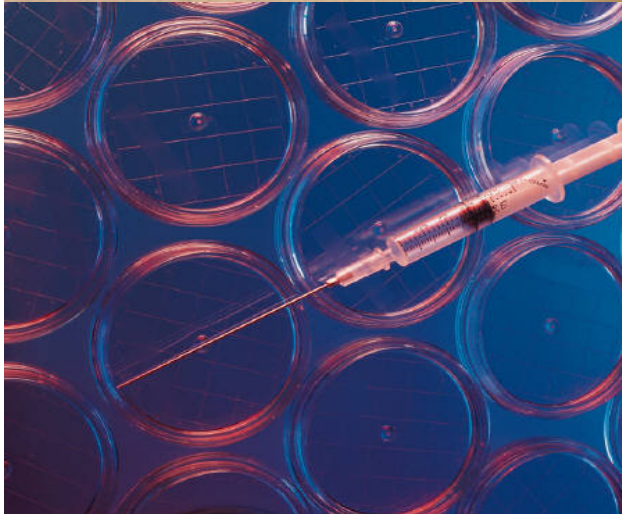
**the EMEA “model of interaction” is encouraging innovation at all levels:**

**Sponsors have the opportunity to use the services to further promote their innovative approaches**



Thank you  
for your attention.

Any Questions?



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