1st EMEA Workshop on Small and Medium-sized Enterprises (SMEs)

"Navigating the Regulatory Maze"

EMEA

2 February 2007

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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 7th 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)

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- **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?**

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- **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**

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EMEA specific initiatives

Establishment of the EMEA 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**

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EMEA specific initiatives

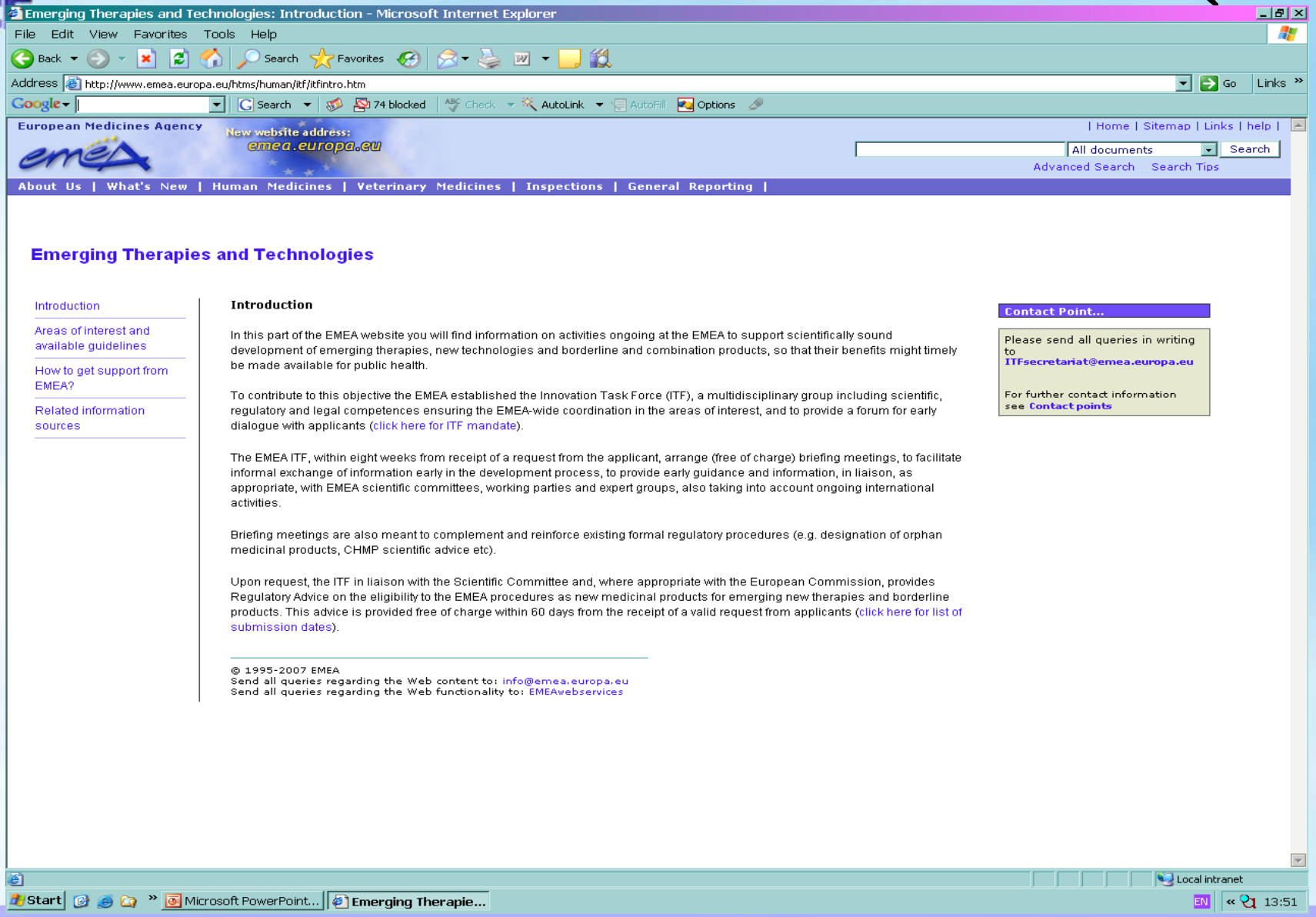
EMEA Innovation Task Force (ITF)

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]

EMEA Innovation Task Force (ITF)



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

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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 committees, 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

EMEA co-ordinator: member of ITF nominated to:

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

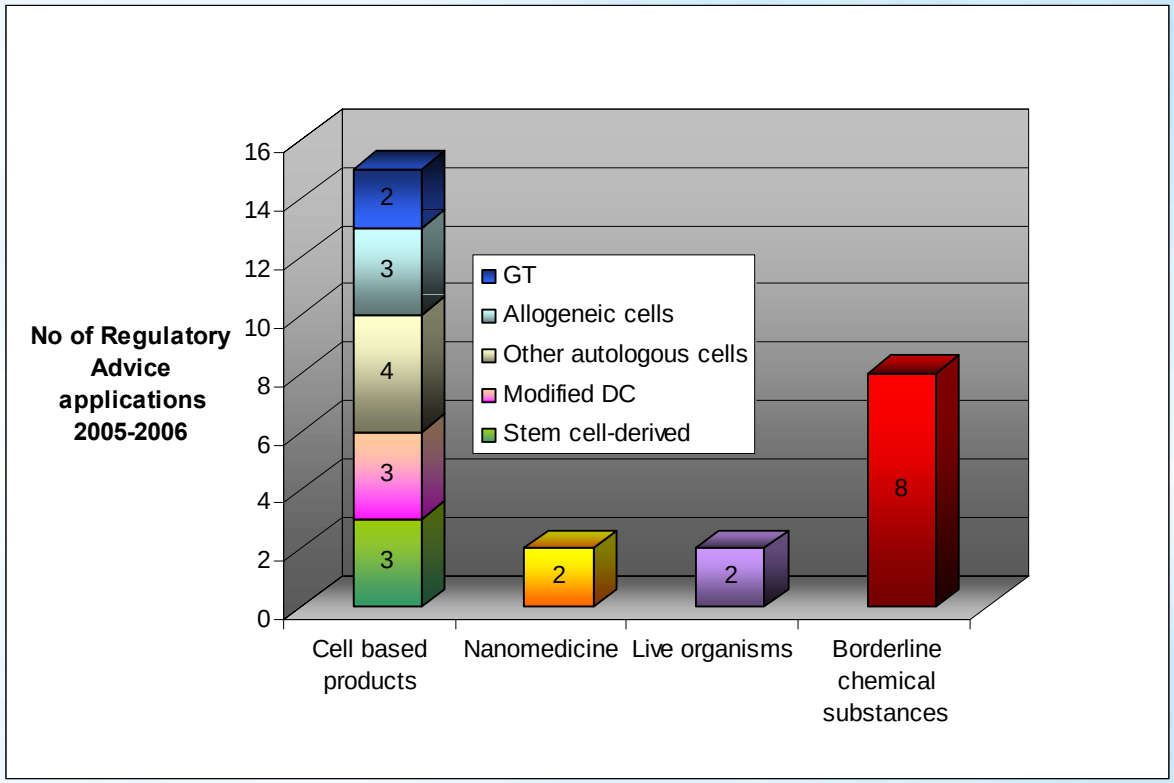
Regulatory Advice (Classification) procedure

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:

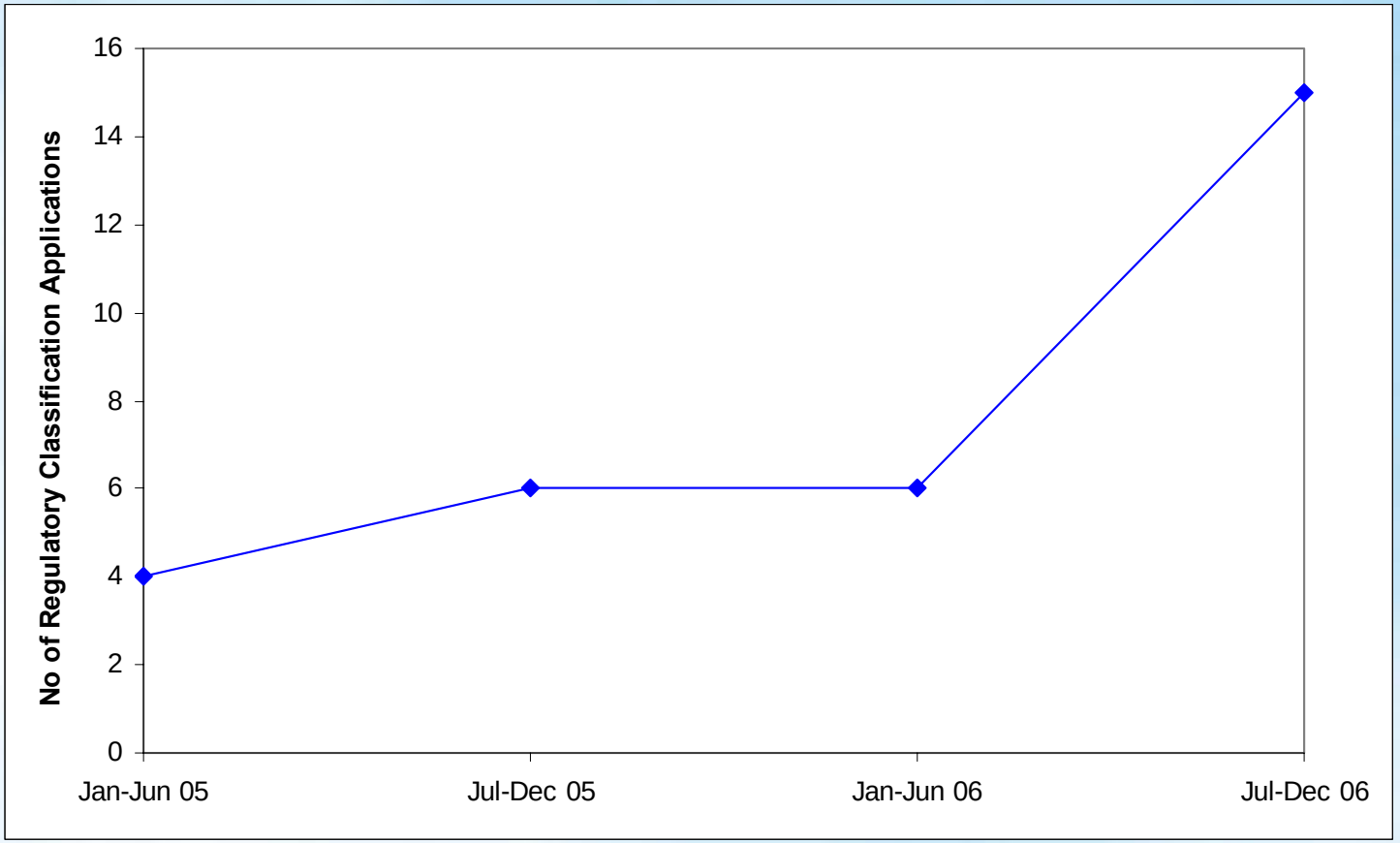
- Commenting the draft report
- Leading the forthcoming internal discussions at CHMP level
- Leading the discussions in case of Oral explanations
- Finalising in conjunction with the EMEA coordinator the conclusions

Product categories under Regulatory Advice



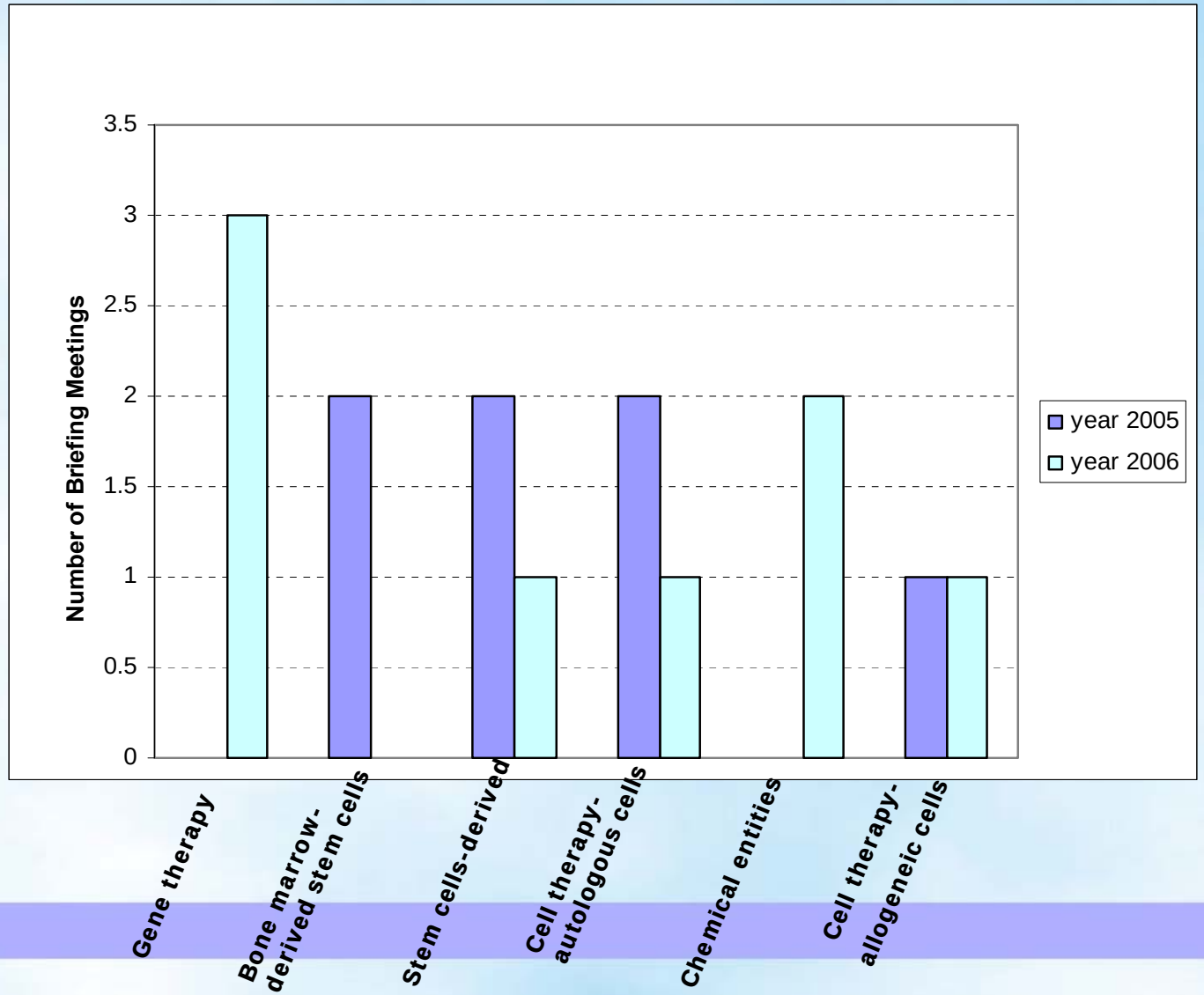
- ✓ Cell-based products represent the majority of products in Regulatory Classification requests
- ✓ Cell-based gene therapy products indicate an emerging trend in new therapies
- ✓ Borderline Chemical substances for innovative diagnostic/therapeutic strategies
- ✓ Nanomedicine, and in particular, therapeutic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



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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**

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EMEA specific initiatives at international level

– **International workshops putting together regulators and stakeholders to discuss emerging issues**

– **Establishment the pilot EMEA/FDA confidentiality arrangements (2004): confirmed March 2006**

– **Contribution to ICH opening to new sciences (e.g. Gene therapy and Pharmacogenomics)**

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•EMEA Road Map and FDA critical path initiative pointing in the same direction: each region implementing new opportunities, also jointly

- FDA/EMEA 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)

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CONCLUSIONS

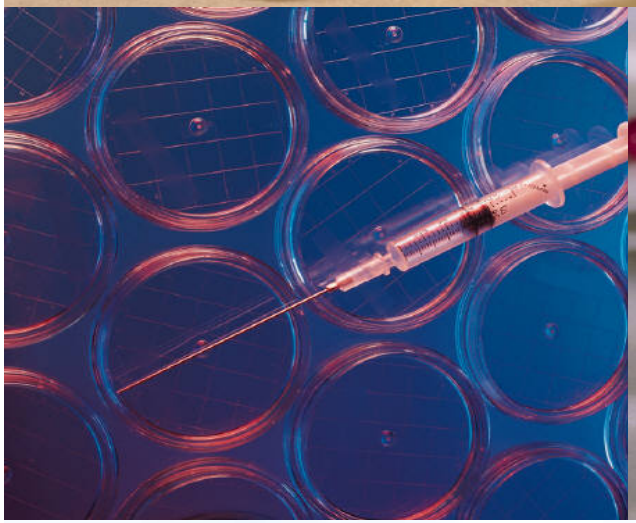
- Positive general EU environment for innovation
- New processes, competences and scientific panels established at the EMEA: SMEs Office and ITF as entry points
- Mechanisms for Regulatory input in to joint academia/Industry platforms being developed within the EU

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?



Acknowledgements:
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Tom Cardy, Business Pipeline Manager, S&E Sector (tom.cardy@emea.europa.eu)