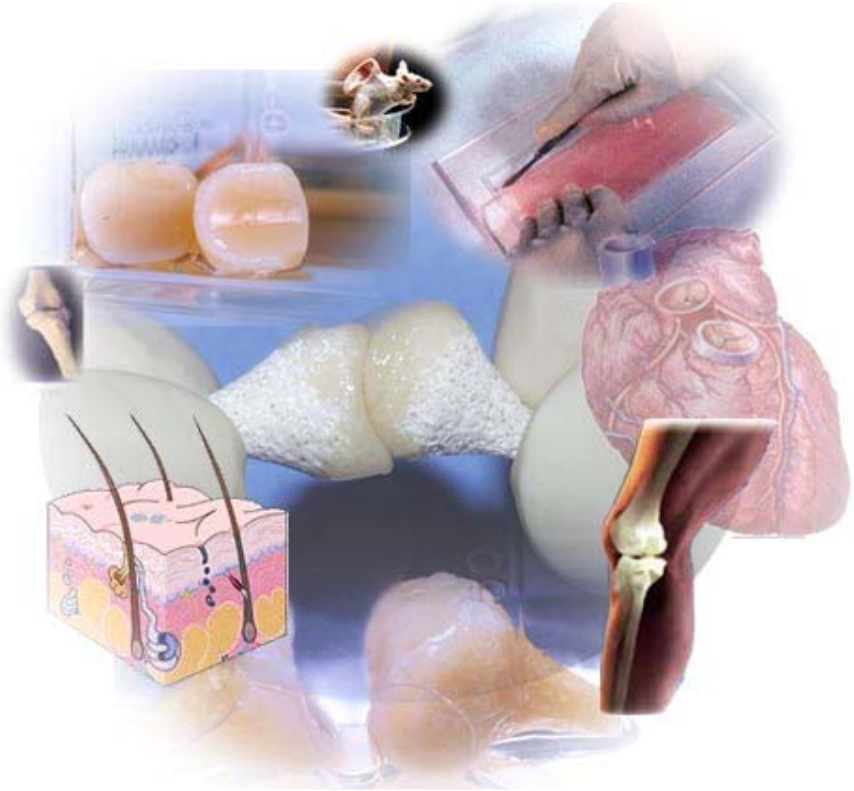
Advanced Therapies Regulation Introduction & Implementation



First Workshop on ATMP

3 April 2009

Patrick Celis

Agenda

- Advanced Therapy Regulation: Introduction
- Highlights from the Regulation on Advanced Therapies
- Implementation of the ATMP Regulation

- **Advanced Therapy Regulation: Introduction**
- Highlights from the Regulation on Advanced Therapies
- Implementation of the ATMP Regulation

Regulation on Advanced Therapies

- **Regulation on Advanced Therapies**
  - » Regulation (EC) No 1394/2007 of 13 November 2007
  - » Published on 10 December 2007
  - » Applicable from 30 December 2008

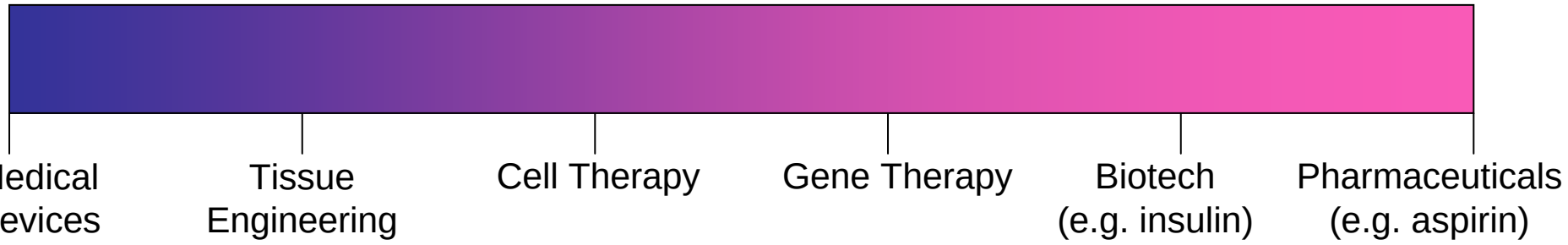
- **For further reading:**
  http://ec.europa.eu/enterprise/pharmaceuticals/advtherapies/index.htm

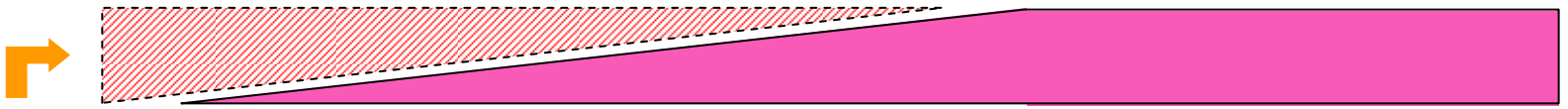
Bridging the gap

Legislation

Science

Advanced Therapies





NEW Committee for Advanced Therapies (CAT) Specific expertise

CHMP expertise

- Advanced Therapy Regulation: Introduction

- **Highlights from the Regulation on Advanced Therapies**

- Implementation of the ATMP Regulation

What are advanced therapy medicinal products?

- **Advanced Therapy medicinal products (ATMP)**
  - » Gene therapy products
  - » Somatic Cell therapy products
  - » Tissue engineered products

- **TEP → Defined in new legislation (Regulation 1394/2007)**
- **Gene therapy and somatic Cell therapy MP → Annex I to Dir 2001/83 (under revision)**

Highlights from Regulation (1)

- **Definition of Advanced Therapy MP**
- **Definition of Tissue engineered product**
  - » TEP now considered MP
- **Principles of existing legislation on medicines apply to advanced therapies:**
  - » marketing authorisation
  - » demonstration of Quality, Safety & Efficacy
  - » post-authorisation vigilance
- **Centralised procedure mandatory:**
  - » pooling of Community expertise
  - » harmonised requirements & evaluation
  - » ensure uniform and direct access to market

Highlights from Regulation (2)

- **New Committee for Advanced Therapies (CAT)**
  - » Legislation defines composition and necessary expertises (e.g. surgery, medical device, ethics)
  - » 1 member per member state (+ 1 alternate)
  - » 5 joint CHMP-CAT members
  - » 2 members representing patient organisations (+2 alternates)
  - » 2 members representing doctors (+ 2 alternates)

Highlights from Regulation (3)

- **Pre-authorisation requirements**
  - » For products incorporating medical devices: compliance with ‘Essential Requirements’
  - » Specific guidelines on GMP (Good Manufacturing Practice) and GCP (Good Clinical Practice)
  - » Specific rules for labelling/packaging
- **Post-authorisation requirements**
  - » Follow-up of efficacy and adverse reactions, and risk management:
    - – obligation for EMEA to inform relevant device/tissue national authorities
  - » Traceability

Highlights from Regulation (4)

- **Tasks of CAT**
  - » Initial **evaluation**, re-examination, post-marketing activities for ATMPs → Draft opinion to CHMP
  - » **Classification** procedure: is a product an ATMP? → Scientific recommendation from CAT
  - » **Certification** procedure: Q/N-C review, for ATMP only → CAT opinion → EMEA certification
  - » **Scientific Advice** for ATMP
    - CAT actively involved in all SA for ATMP (via SAWP)
  - » **Other task**, eg consultation by CHMP on non-ATMPs, advice to Commission

Highlights from Regulation (5)

Incentives for industry:

- **Scientific Advice:**
  - » 90% fee reduction for SMEs, 65% for others
- **Scientific recommendation on advanced therapy classification: 60 days**
- **SMEs: Certification of quality and non-clinical data**
- **Additional Fee reduction if applicant is SME or hospital and can prove there is a particular public health interest in the Community**

- Advanced Therapy Regulation: Introduction
- Highlights from the Regulation on Advanced Therapies
- **Implementation of the ATMP Regulation**

Implementation activities

- **Already before the final adoption of the ATMP Regulation (Dec 2007), EMEA initiated its implementation actions**
  - » Specific Advanced Therapies Task Force was set up
  - » Identification of Tasks / Subtasks and timings

- **Scientific Guidelines for ATMPs**
  - » Developed by BWP-GTWP-CPWP
  - » This started long before adoption of ATMP Regulation

2008 – The year of implementation!

- **Scientific challenges:**
  - » Revision of Annex I to Dir. 2001/83/EC
  - » GCP specific for ATMPs (feedback to EC)
  - » GMP specific for ATMPs (ongoing)
  - » Certification
    - – Scientific Guideline on minimum dossier requirements (ongoing)
  - » Guideline on PM safety & efficacy follow-up and RMP for ATMPs
  - » Traceability of ATMPs (feedback to EC)

2008 – The year of implementation!

- **Procedural:**
  - Setting up of new Committee (Committee for Advanced Therapies)
    - Development of Rules of Procedure
    - Procedure for evaluation of ATMPs (pre/post)
    - Appointment of CAT members/alternates
  - New procedures to be developed
    - Certification procedure
    - Classification as ATMP
      - Procedure based on existing ITF procedure
      - Procedure will be published shortly on EMEA Website

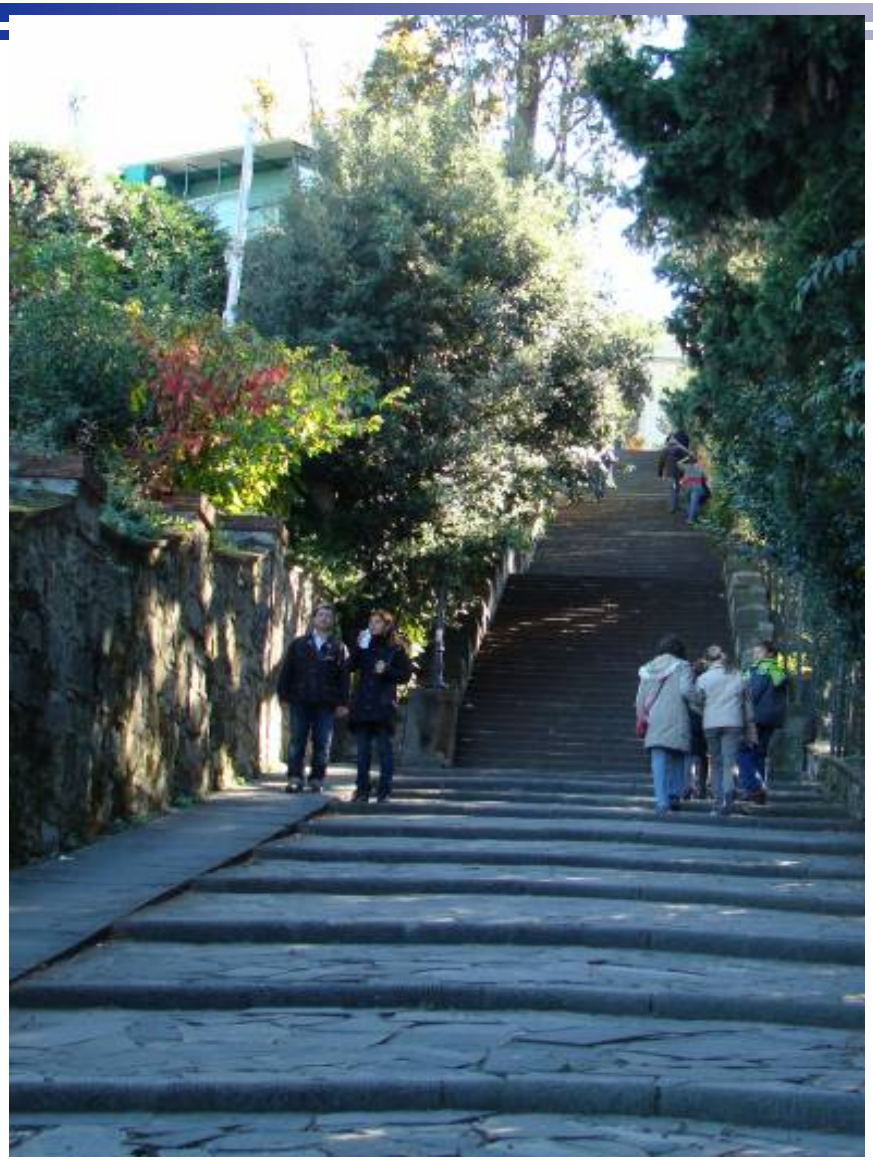
2009: The year of consolidation

- **Inaugural CAT meeting: 15-16 January 2009**
- **February CAT meeting: Election of CAT chair: Dr Schneider and CAT vice-chair: Dr Salmikangas**
- **Product-related discussions**
  - » Marketing authorisation applications
  - » CAT contributions to Scientific Advice procedures for ATMPs
  - » Classification requests
  - » Certification procedures (awaited, from May onwards)

What is still to be done?

- **Preparation of various templates, SOPs etc**
- **Preparation, on behalf of EC, the Guideline on Traceability**
- **Finalisation of Guideline on GMP for ATMPs**
  - » Part of revision of Annex 2 to GMP Guide
- **Integration of the work of GTWP and CPWP in CAT**

Implementation of ATMP Regulation



- Integration work GTWP-CBWP in CAT
- Traceability Guideline
- Templates, SOPs etc

→ Almost there….

→ January 2009 – First CAT meeting

- Scientific Guideline (BWP-GTWP-CPWP)
- Setting up of CAT
- Development of new Procedures
- Revision of Annex I to Dir 2001/83/EC

→ **2007 – Adoption of ATMP Regulation**

- Scientific Guideline (BWP-GTWP-CPWP)

Any Questions?

Thank you for your attention

Patrick Celis

CAT Secretariat

Tel. +44 207 418 8656

Patrick.celis@emea.europa.eu

For general queries on ATMPs / CAT:

AdvancedTherapies@emea.europa.eu