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
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- Highlights from the Regulation on Advanced Therapies

- Implementation of the ATMP Regulation
Regulation on Advanced Therapies

- Regulation on Advanced Therapies
  - 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

Medical Devices 93/42/EEC

Regulation on Advanced Therapies

Medicinal Products 2001/83/EC

Science

Advanced Therapies

Medical Devices

Tissue Engineering

Cell Therapy

Gene Therapy

Biotech (e.g. insulin)

Pharmaceuticals (e.g. aspirin)

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

2007 – Adoption of ATMP Regulation

January 2009 – First CAT meeting

Almost there….

• Traceability Guideline
• Templates, SOPs etc

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