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EMA/CAT/GTWP/394502/2011
CAT Gene therapy temporary working party

# Work plan for the Gene Therapy Working Party 2012

**Chair**

Maria Cristina Galli (Chair)

Matthias Renner (Vice-chair)

**Status**

December 2011 - adopted

## 1. Meetings scheduled for 2012

- 23-24 February 2012
- 3-4 May 2012 (virtual meeting)
- 25-26 October 2012 (to be confirmed)

Pending the workload, separate drafting group meetings may be organised by teleconference.

## 2. Guidelines

**Revision of the note for guidance on quality, preclinical and clinical aspects of gene transfer medicinal products (CPMP/BWP/3088/99)**

**Action:** Finalisation of the revision of the gene therapy parent guideline, to be released for public consultation.

**Comments:** In collaboration with BWP and SWP

**Guideline on the risk-based approach according to annex I, part IV of directive 2001/83/EC applied to advanced therapy medicinal products**

**Action:** Public consultation and revision of draft guideline jointly with CPWP

**Comments:** The Guideline on Cell-based medicinal products (EMEA/CHMP/410869/2006) as well as revision to Annex I, Part IV of Dir 2001/83/EC, as amended, introduce a risk based approach for the development of advanced therapy medicinal products (cell-based

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products and gene therapy products). This will provide further guidance on how to perform and present this analysis for initial marketing authorisation application.

In collaboration with BWP, SWP, and PhVWP

**Guideline on quality, non clinical and clinical aspects of medicinal products containing genetically modified cells (EMA/CHMP/GTWP/671639/2008)**

**Action:** Finalisation

**Comments:** In collaboration with CPWP and BWP

**Concept paper on the quality requirements for investigational gene therapy medicinal products before first clinical use**

**Action:** Consideration on the need to develop guidance on quality requirements for gene therapy investigational medicinal products.

**Comments:** The concept paper is intended to provide guidance to applicants developing gene therapy medicinal products on the minimal quality requirements for first-in-man clinical studies and to facilitate a harmonised approach in the EU.
In collaboration with BWP and the clinical trial facilitation group

**Concept Paper on insertional mutagenesis issues**

**Action:** Consideration on the need to develop a concept paper to be released for public consultation.

**Comments:** The concept paper is intended to discuss the quality, nonclinical and clinical tools to alleviate the risk of insertional mutagenesis and improve safety of gene therapy medicinal products.

**Concept Paper on DNA vaccines (lead by the VWP)**

**Action:** Provision of comments to the VWP.

**Comments:** Gene therapy medicinal products shall not include vaccines against infectious diseases. However, some principles and requirements of gene therapy apply to DNA vaccines against infectious diseases; therefore the GTWP will provide comments on this concept paper.

**Questions & Answers document on current topics in gene therapy**

**Action:** Initiation of a navigating map on EMA website to facilitate the search for GTWP applicable guidelines.

Addition of additional Q&A as appropriate

**Comments:** With reference to CAT focus group on 'navigating EMA/ATMP guidelines for gene therapy.

## 3. Awareness on Scientific Issues

Preparation of scientific reports on matters of public interest and emerging issues pertaining to gene therapy. In particular, the working party intends to address the following scientific topics:

- Consequences of pre-existing immunity of vectors

- Role and need of immunosuppression in conjunction with gene therapy

- Genetic technology methods to produce induced pluripotent stem cells (virtual expert meeting)

- Technology/methods for integration site directed gene therapy

- Technology/methods for sequencing

Information on regulators views on scientific issues specific to gene therapy (via commentary, or participation to/preparation of workshop / experts panel, regulators training subject to outcome publication, etc).

## 4. Other activities and activities at the request of CAT

5.1. Contribution to scientific advice, marketing authorisation evaluation, post-authorisation procedure evaluation, classification, certification, paediatric investigation plan evaluation and orphan designation on general and product specific matters related to gene therapy;

5.2. Contribution to briefing meetings related to gene therapy with external parties (pharmaceutical companies, academia, public/private partnership or patients' associations) through the participation of experts in close collaboration with the Innovation Task Force;

5.3. Liaison with interested parties (e.g. learned societies, trade organisations, industry, academia, patient's organisations) in close collaboration with the CAT;

5.4. International cooperation on gene therapy related matters, including participation to ICH group meetings when requested/if appropriate;

5.5. Collaboration with EDQM, together with CAT/BWP/CPWP, concerning standardisation of raw materials for production of Gene therapy medicinal products.

5.6. Contribute to training for quality, non-clinical and clinical assessment of gene therapy, and contribution to and organisation of external workshops and trainings related to gene therapy, where requested.

5.7. Constitute a rapid-acting crisis group to take on board specific issues related to gene therapy with the objective of exchanging information on a European level and to co-ordinate responses to the public in a timely manner, where requested;

5.8 Any other task delegated by the CAT for example liaison with the EC, other committees and working parties on matters relevant to gene therapy.