



CAT Stakeholders workshop

*Focus Groups: a model for a fruitful interaction
between CAT and its stakeholders*

Interactive flow-chart for Gene Therapy guidelines

Pablo de Felipe

London, 12 January 2012

European Medicines Agency - Multidisciplinary - Multidisciplinary: Cell therapy and tissue engi - Microsoft Internet Explorer

Archivo Edición Ver Favoritos Herramientas Ayuda

Atrás Búsqueda Favoritos

Dirección http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general_content_000405.jsp&mid=WC0b01ac058002958a

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Text size: [A](#) [A](#) [A](#) Site-wide search

Home Find medicine **Regulatory** Special topics Document search News & events Partners & networks About us Quick links

Human medicines
 Pre-authorisation
 Post-opinion
 Post-authorisation
 Product information
 Scientific advice and protocol assistance
 Scientific guidelines
 Search guidelines
 Quality
 Q&A on quality
 Biologicals
 Non-clinical
 Clinical efficacy and safety
 Multidisciplinary
 Paediatrics
 Cell therapy and tissue engineering
 Vaccines
 Biosimilar
 Gene Therapy
 Herbal medicinal products

Home Regulatory Human medicines Scientific guidelines Multidisciplinary Cell therapy and tissue engineering

Multidisciplinary: Cell therapy and tissue engineering

This page lists the scientific guidance documents on **cell therapy** and **tissue engineering**.

If you have comments on a document which is open for consultation, please use the [form for submission of comments on scientific guidelines](#).

Topic	Documents	Reference number	Publication date	Effective date	Remarks
CHMP/CAT position statement on Creutzfeldt-Jakob disease and advanced therapy medicinal products	Adopted guideline Overview of comments Draft guideline	CHMP/CAT/BWP/353632/2010	June 2011	June 2011	
Reflection paper on stem cell-based medicinal products	Overview of comments Adopted reflection paper Draft reflection paper	CAT/571134/09	February 2011	January 2011	
Development of a guideline on the risk-based approach according to annex I, part IV of Directive 2001/83/EC applied to advanced therapy medicinal products	Concept paper	CHMP/CPWP/708420/09	Released for consultation December 2009		Deadline for comments March 2010
Reflection paper on <i>in-vitro</i> cultured chondrocyte containing products for cartilage repair of the knee	Overview of comments Draft reflection paper Adopted reflection paper	CAT/CPWP/568181/2009	May 2010	April 2010	
Potency testing of cell based immunotherapy medicinal products for the treatment of cancer	Overview of comments Adopted guideline Draft guideline	CHMP/BWP/271475/06	December 2007	May 2008	
Guideline on xenogeneic cell-based medicinal products	Adopted guideline Draft guideline Concept paper	CHMP/CPWP/83508/09	December 2009	January 2010	
Human cell-based medicinal products	Overview of comments Adopted guideline	CHMP/410869/06	June 2008	September 2008	

Internet

Adv. Ther. guidelines at the EMA webpage

European Medicines Agency - Multidisciplinary - Multidisciplinary: Gene Therapy - Microsoft Internet Explorer

Archivo Edición Ver Favoritos Herramientas Ayuda

Atrás Búsqueda Favoritos

http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000410.jsp&mid=WC0b01ac58002958d



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Text size: Site-wide search GO

Home Find medicine **Regulatory** Special topics Document search News & events Partners & networks About us Quick links

Home > Regulation > Scientific guidelines > Multidisciplinary > Gene Therapy

Multidisciplinary: Gene Therapy

If you have comments on a document which is open for consultation, please use the form for submission of comments on scientific guidelines.

Topic	Documents	Reference number
CHMP/CAT position statement on Creutzfeldt-Jakob disease and advanced therapy medicinal products	Draft guideline	EMA/CHMP/CAT
Quality, pre-clinical and clinical aspects of medicinal products containing genetically modified cells	Draft guideline Concept paper	CHMP/GTWP/6 71639/2008
Development of a guideline on the risk-based approach according to annex 1, part IV of directive 2001/83/EC applied to advanced therapy medicinal products	Concept paper	CHMP/CPWP/7 08420/09
Questions and answers on gene therapy	Adopted guideline	CHMP/GTWP/2 12377/08
Revision of the note for guidance on the quality, pre-clinical and clinical aspects of gene transfer medicinal products	Concept paper	CHMP/GTWP/2 34523/09
ICH Considerations General Principles to Address Virus and Vector Shedding	Concept paper	CHMP/ICH/449 035/09
Quality, non-clinical and clinical issues relating specifically to recombinant adeno-associated viral vectors	Overview of comments Adopted guideline Concept paper	CHMP/GTWP/5 87488/07
ICH Considerations - Oncolytic Viruses	Adopted guideline Draft guideline	CHMP/GTWP/6 07698/08

European Medicines Agency - Multidisciplinary - Multidisciplinary: Gene Therapy - Microsoft Internet Explorer

Archivo Edición Ver Favoritos Herramientas Ayuda

Atrás Búsqueda Favoritos

http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000410.jsp&mid=WC0b01ac58002958d

Pharmacogenomics
Miscellaneous
ICH
Innovation Task Force
Regulatory and procedural guidance
SME office
Paediatric medicine
Orphan designation
Herbal products
Referral procedures
Article 58 applications
Compassionate use
Pharmacovigilance
Advanced therapies
Inspections
Product defects and recalls
Parallel distribution
Pandemic influenza
Non-pharmaceutical products
New countries/EFTA
Fees
Veterinary medicines

Non-clinical studies required before first clinical use of gene therapy medicinal products	Overview of comments Adopted guideline Draft guideline Concept paper	CHMP/GTWP/1 25459/06	May 2008	Nov 2008
Follow-up of patients administered with gene therapy medicinal products	Overview of comments Adopted guideline Draft guideline Concept paper	CHMP/GTWP/6 0436/07	Nov 2009	May 2010
Scientific Requirements for the Environmental Risk Assessment of Gene Therapy Medicinal Products	Overview of comments Adopted guideline Draft guideline Concept paper	CHMP/GTWP/1 25491/06	May 2008	Nov 2008
Non-Clinical testing for Inadvertent Germine transmission of Gene Transfer Vectors	Overview of comments Adopted guideline Draft guideline	EMA/273974/05	Dec 2006	May 2007
Development and Manufacture of Lentiviral Vectors	Adopted guideline	CHMP/BWP/245 8/03	May 2005	Nov 2005
Quality, Preclinical and Clinical Aspects of Gene Transfer Medicinal Products	Adopted guideline	CHMP/BWP/308 8/99	Apr 2001	Oct 2001

General

- “Note for guidance on the quality, preclinical and clinical aspects of gene transfer medicinal products” (CPMP/BWP/3088/99) Q NC C
- “Questions and answers on gene therapy” (CHMP/GTWP/212377/08) Q NC

Certification (only for Q or for Q+NC)

- “Procedural advice on the certification of quality and non-clinical data for small and medium sized enterprises developing advanced therapy medicinal products” (EMA/CAT/418458/2008/corr.) Q NC
- “Guideline on the minimum quality and non-clinical data for certification of advanced therapy medicinal products” (EMA/CAT/486831/2008/corr.) Q NC

First in human

- “Guideline on the non-clinical studies required before first clinical use of gene therapy medicinal products” (EMA/CHMP/GTWP/125459/2006) NC

Marketing authorisation application (MAA) (1/2)

Vectors

- “Guideline on development and manufacture of lentiviral vectors” (CHMP/BWP/2458/03) **Q**
 - “ICH considerations: oncolytic viruses” (CHMP/ICH/607698/08) **Q NC C**
- “Reflection paper on quality, non-clinical and clinical issues related to the development of recombinant adeno-associated viral vectors” (CHMP/GTWP/587488/07) **Q NC C**
- General chapter of the Eur. Ph.: “Gene transfer medicinal products for human use” (01/2010:51400) **Q**

Gene therapy products with modified somatic cells

- “Guideline on quality, non-clinical and clinical aspects of medicinal products containing genetically modified cells” (CHMP/GTWP/671639/2008) **Q NC C Draft**

Risk of germline transmission

- “Guideline on non-clinical testing for inadvertent germline transmission of the gene transfer vectors” (EMA/273974/2005) **NC**
- “ICH Considerations: General principles to address the risk of inadvertent germline integration of gene therapy vectors” (CHMP/ICH/469991/2006) **NC**

Marketing authorisation application (MAA) (2/2)

Post-administration

- “Guideline on follow-up of patients administered with gene therapy medicinal products”
(**EMA/CHMP/GTWP/60436/2007**) **C**

- “Guideline on safety and efficacy follow-up - risk management of advanced therapy medicinal products” (**Doc. Ref. EMA/149995/2008**) **C**

- “ICH Considerations: General principles to address virus and vector shedding” (**CHMP/ICH/449035/09**)
C

Environmental risk

- Guideline on scientific requirements for the environmental risk assessment of gene therapy medicinal products” (**EMA/CHMP/GTWP/125491/2006**)

- “Guideline on environmental risk assessments for medicinal products consisting of, or containing, genetically modified organisms (GMOs)” (**EMA/CHMP/BWP/473191/2006-corr**)

Other relevant guidelines (1/3)

Cell therapy guidelines

- Guideline on human cell-based medicinal products (EMEA/CHMP/410869/2006)
- Guideline on xenogeneic cell-based medicinal products (EMEA/CHMP/CPWP/83508/2009)
- Reflection paper on stem cell-based medicinal products (EMA/CAT/571134/2009)

Reagents/Materials

- ICH Q5B: note for guidance on quality of biotechnological products: analysis of the expression construct in cell lines used for production of r-DNA derived protein products (CPMP/ICH/139/95)
- Note for Guidance on the Use of Bovine Serum in the Manufacture of Human Biological Medicinal Products (CPMP/BWP/1793/02)
- ICH Q5D: Quality of Biotechnological Products: Derivation and Characterisation of Cell Substrates Used for Production of Biotechnological/Biological Products (CPMP/ICH/294/95)
 - Position Statement on the Use of Tumourigenic Cells of Human Origin for the Production of Biological and Biotechnological Medicinal Products (CPMP/BWP/1143/00)
- Use of Transgenic Animals in the Manufacture of Biological Medicinal Products for Human use (3AB7A)

Development

- Development pharmaceuticals for biotechnological and biological products (CPMP/BWP/328/99). Annex to Note for guidance on development pharmaceuticals (CPMP/QWP/155/96)



Other relevant guidelines (2/3)

Viral safety related guidelines (for biological and biotechnological products)

- Note for guidance on virus validation studies: the design, contribution and interpretation of studies validating the inactivation and removal of viruses (CPMP/BWP/268/95 or 3AB8A)
- ICH Topic Q5A: Note for guidance on quality of biotechnological products: viral safety evaluation of biotechnology products derived from cell lines of human or animal origin (CPMP/ICH/295/95)
- “Guideline on quality, non-clinical and clinical aspects of live recombinant viral vectored vaccines”
(EMA/CHMP/VWP/141697/2009)

TSE/CJD safety related guidelines

- Note for guidance on Minimising the Risk of Transmitting Animal Spongiform Encephalopathy Agents via Human and Veterinary Medicinal Products (EMA/410/01 Rev. 3) (Official Journal of the European Union - 2011/C 73/01)
- CHMP/CAT position statement on Creutzfeldt-Jakob disease and advanced therapy medicinal products (EMA/CHMP/CAT/BWP/353632/2010) Draft

Changes in the manufacturing process

- ICH Topic Q5E: Note for guidance on biotechnological/biological products subject to changes in their manufacturing process (CPMP/ICH/5721/03)
- Reflection paper on changes during development of gene therapy medicinal product () Draft

Other relevant guidelines (3/3)

Testing

- Tests on samples of biological origin (3AB11A)
- ICH Q6B: Specifications: Test Procedures and Acceptance Criteria for Biotechnological/Biological Products (CPMP/ICH/365/96)
- Guideline on potency testing of cell based immunotherapy medicinal products for the treatment of cancer (EMA/CHMP/BWP/271475/2006)
- ICH 5QC: Quality of Biotechnological Products: Stability Testing of Biotechnological/Biological Products (3AB5A or CPMP/ICH/138/95)

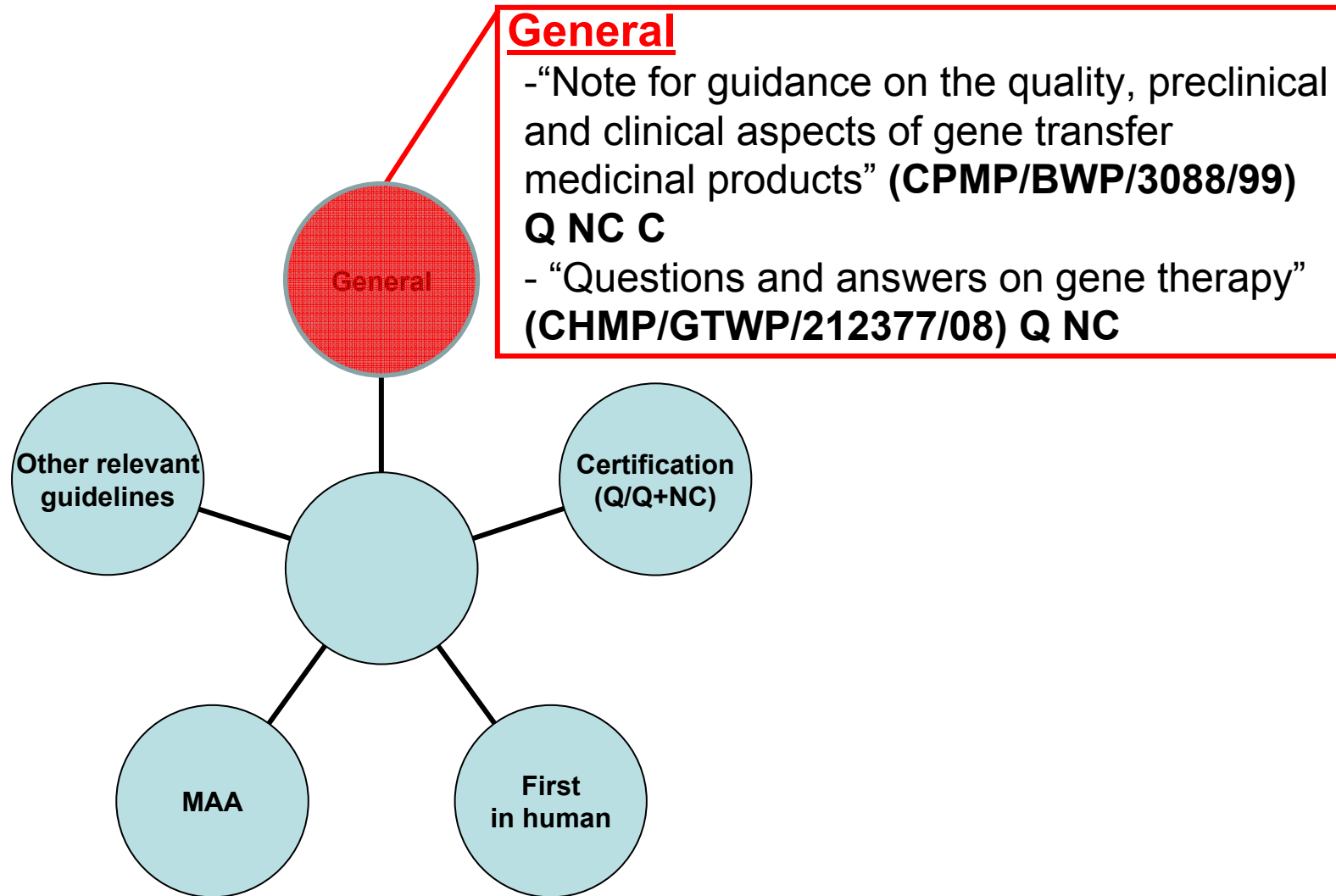


NOTE:

This flowchart only includes the currently applicable guidelines. It is acknowledged that in the EMA website all historical guidelines can be found.

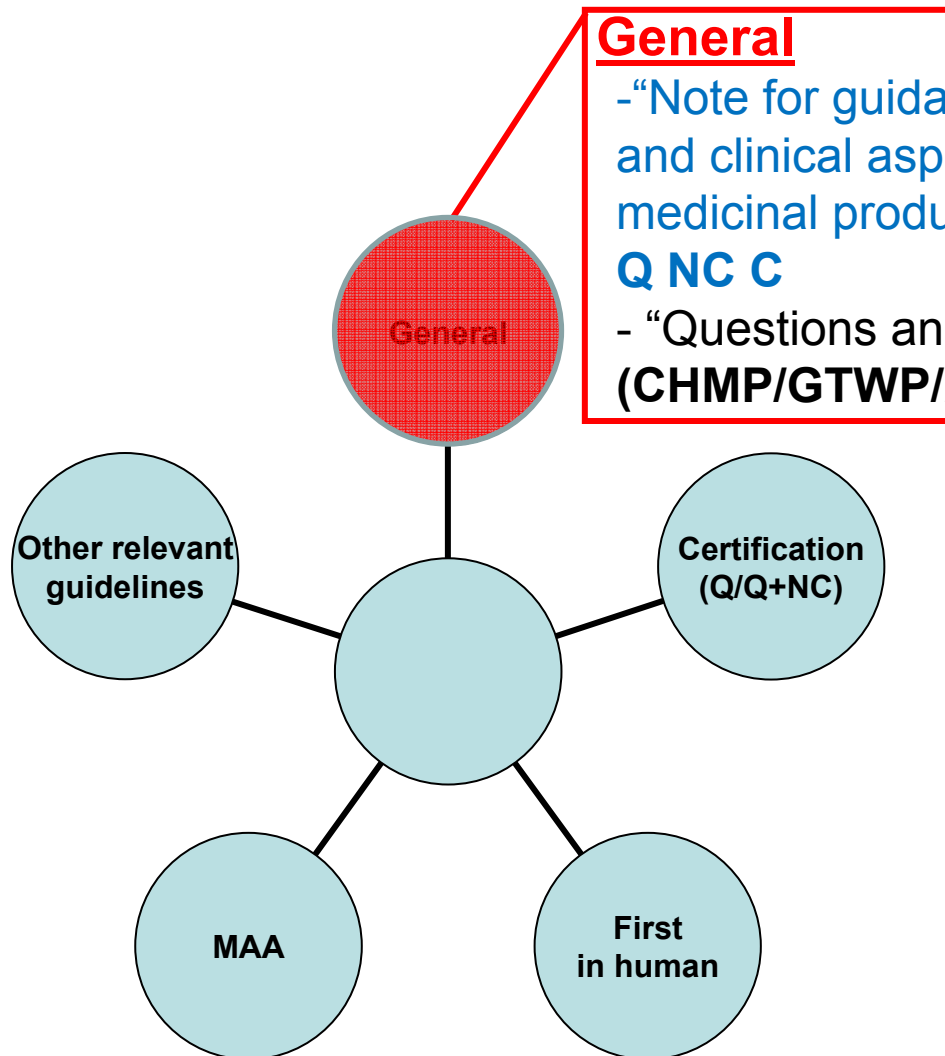
The guidelines included in this flow chart are those specific for gene therapy medicinal products and those related with biotechnological and biological products where similar principles can be applied to GTMPs. Other general ICH and EMA guidelines, in particular for non clinical, clinical aspects, paediatrics and orphans as well as European Pharmacopeia monographs and chapters might be applicable.

Flow-chart 2nd step: proposal of arrangement





Flow-chart 2nd step: proposal of arrangement



General

-“Note for guidance on the quality, preclinical and clinical aspects of gene transfer medicinal products” (CPMP/BWP/3088/99)

Q NC C

- “Questions and answers on gene transfer medicinal products” (CHMP/GTWP/0000000000)



The European Agency for the Evaluation of Medicinal Products
Evaluation of Medicines for Human Use

London, 24 April 2001
CPMP/BWP/3088/99

COMMITTEE FOR PROPRIETARY MEDICINAL PRODUCTS
(CPMP)

NOTE FOR GUIDANCE ON THE QUALITY, PRECLINICAL AND
CLINICAL ASPECTS OF GENE TRANSFER MEDICINAL
PRODUCTS

DISCUSSION IN THE BIOTECHNOLOGY WORKING PARTY (BWP)	June – December 1999
DISCUSSION IN THE SAFETY WORKING PARTY (SWP)	June 1999
DISCUSSION IN THE EFFICACY WORKING PARTY	July – November 1999
TRANSMISSION TO CPMP	December 1999
RELEASE FOR CONSULTATION	December 1999
DEADLINE FOR COMMENTS	June 2000
DISCUSSION IN THE EFFICACY WORKING PARTY (EWP)	September 2000
DISCUSSION IN THE SAFETY WORKING PARTY (SWP)	February 2001
DISCUSSION IN THE BIOTECHNOLOGY WORKING PARTY (BWP)	March 2001
WRITTEN PROCEDURE WITH SAFETY WORKING PARTY (SWP)	April 2001
TRANSMISSION TO CPMP	April 2001



¡THANK YOU!

Interactive flow-chart for Gene Therapy guidelines

Pablo de Felipe

London, 12 January 2012