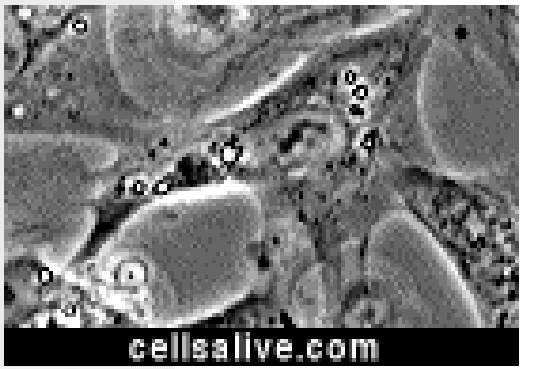
MINISTERIO DE SANIDAD Y POLÍTICA SOCIAL

agencia española de medicamentos y productos sanitarios

23 marzo 2010

Regulación de terapias avanzadas En la UE



Sol Ruiz - AEMPS

Regulation (EC) No 1394/2007

10.12.2007 EN Official Journal of the European Union L 324/121

REGULATION (EC) No 1394/2007 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL

of 13 November 2007

on advanced therapy medicinal products and amending Directive 2001/83/EC and Regulation (EC) No 726/2004

(Text with EEA relevance)

Specific rules regarding the authorisation, supervision and pharmacovigilance of ATMPs

Regulation (EC) No 1394/2007

(a) 'Advanced therapy medicinal product' means any of the following medicinal products for human use:

— a gene therapy medicinal product as defined in Part IV of Annex I to Directive 2001/83/EC,

— a somatic cell therapy medicinal product as defined in Part IV of Annex I to Directive 2001/83/EC,

— a tissue engineered product as defined in point (b).

Commission Directive 2009/120/EC

DIRECTIVES

COMMISSION DIRECTIVE 2009/120/EC

of 14 September 2009

amending Directive 2001/83/EC of the European Parliament and of the Council on the Community code relating to medicinal products for human use as regards advanced therapy medicinal products

(Text with EEA relevance)

scope

Advanced therapy medicinal products which are intended to be placed **on the market in Member States** and either **prepared industrially** or manufactured by a method involving an industrial process (Title II of Directive 2001/83).

scope

EXCLUDED from the scope of this Regulation:

advanced therapy medicinal products which are prepared on

- a non-routine basis according to specific quality standards,
- used within the same Member State in a hospital,
- under the exclusive professional responsibility of a medical practitioner in order to comply with an individual medical prescription for a custom made product for an individual patient

Key principles of the proposal

For products within the scope:

- Marketing authorisation required
- Demonstration of Quality, Safety & Efficacy
- Post-authorisation vigilance of S & E
- Centralised procedure mandatory

EL PROCEDIMIENTO CENTRALIZADO

Medicines for children
Medicines in Community referral procedures
Medicines for use outside the European Union
Withdrawn, suspended or revoked authorisations
Withdrawn applications
Press releases

Participation of patients and consumers in EMEA activities
Involve YOUR organisation
Eligible organisations
Patients' and Consumers' Working Party (PCWP)

Centralised procedure

The European Medicines Agency is responsible for the centralised procedure (also known as the 'Community authorisation procedure'). This procedure results in a single marketing authorisation (called a 'Community marketing authorisation') that is valid across the European Union. The centralised procedure is compulsory for human medicines that are:

- derived from biotechnology processes, such as genetic engineering;
- advanced-therapy medicines, such as gene-therapy, somatic cell-therapy or tissue-engineered medicines;
- intended for the treatment of HIV/Aids, cancer, diabetes, neurodegenerative disorders, autoimmune diseases and other immune dysfunctions, or viral diseases;
- officially designated 'orphan medicines' (medicines used for rare diseases).

For medicines that do not fall within these categories, companies may submit an application for a centralised marketing authorisation to the EMEA, as long as the medicine concerned is a significant therapeutic, scientific or technical innovation, or if its authorisation would be in the interest of public health.

Applications through the centralised procedure are submitted directly to the EMEA. Evaluation by the Agency's relevant scientific committee takes up to 210 days, at the end of which the committee adopts an opinion on whether the medicine should be marketed or not. This opinion is then transmitted to the European Commission, which issues a formal decision on the authorisation of the product.

Once a Community marketing authorisation has been granted, the marketing-authorisation holder can begin to make the medicine available to patients and healthcare professionals in all EU countries.

Decentralised procedure

Using the decentralised procedure, companies may apply for simultaneous authorisation in more than one EU country of medicinal products that have not yet been authorised in any EU country and that do not fall within the mandatory scope of the centralised procedure.

Mutual-recognition procedure

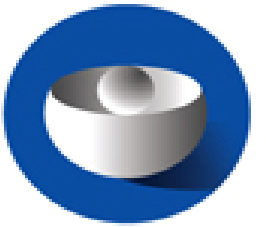
SOLICITUD A LA EMEA: CALENDARIO DE EVALUACION

DIA	
0	• INICIO DEL PROCEDIMIENTO
80	• DISTRIBUCION DE INFORMES DE EVALUACION
100	• COMENTARIOS MIEMBROS CHMP
115	• LISTA CONSOLIDADA DE OBJECIONES
120	• CHMP VALIDACION DE OBJECIONES.
121	• RESPUESTAS DE LA COMPAÑÍA 
150	• INFORME COMUN PARA EL CHMP
170	• COMENTARIOS DEL CHMP AL INFORME
180	• DEBATE DEL CHMP DECISION DE AUDIENCIA 

1995-2009

EUROPEAN MEDICINES AGENCY

2010–



EUROPEAN MEDICINES AGENCY

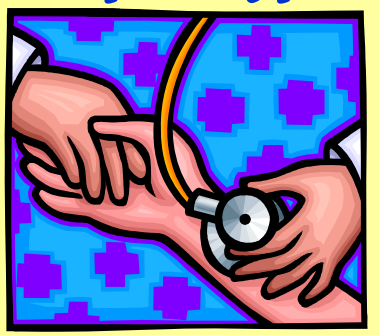
SCIENCE MEDICINES HEALTH

www.ema.europa.eu

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

COMITÉS CIENTÍFICOS

CHMP



Hu

CVMP

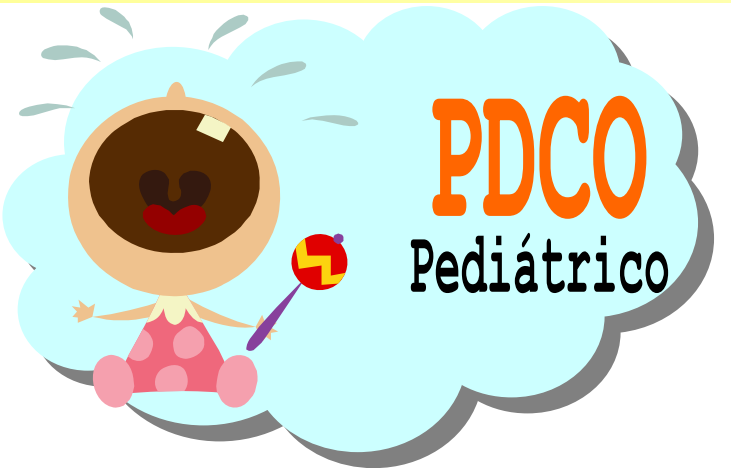
Vet

COMP

Huérfanos

HMPC

Plantas med



PDCO
Pediátrico

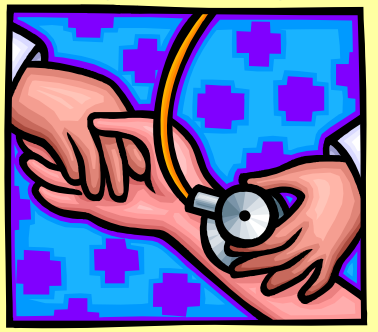
cat

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

COMITÉS CIENTÍFICOS

atmp

CHMP



Human Med

propuesta

cat

dictamen final

The European Medicines Agency marks its 15th anniversary with a short film

Published 26/01/2010

Inaugurated on 26 January 1995, the European Medicines Agency has now been working with its partners in the EU Member States to promote and protect human and animal health across the European Union for 15 years.

As part of its 15th anniversary celebrations, the Agency has commissioned a short film that provides a flavour of the ever-evolving environment in which it operates.

Watch the 5-minute film (30MB)

Latest Press Releases

See **Press Office** for archived press releases

26/02/10	COMP	FDA and EMA Agree to Accept a Single Orphan Drug Designation Annual Report
19/02/10	EMA	United Therapeutics Europe Ltd withdraws its marketing authorisation application for Tyvaso
19/02/10	CHMP	Press Release from the CHMP February meeting
19/02/10	EMA	European Medicines Agency updates on pandemic influenza
18/02/10	EMA	European Medicines Agency recommends contraindication for Reqranex in patients with any pre-existing cancer - *See also:* Questions and Answers
16/02/10	EMA	European Medicines Agency and EUnetHTA Joint Action start collaboration on European Public Assessment Report (EPAR) contribution to relative effectiveness assessments
15/02/10	EMA	European Medicines Agency and Swissmedic agree sharing of information on H1N1 pandemic medicines

See **Press Office** for archived press releases

Meeting highlights from the Committee for Medicinal Products for Human Use (CHMP)

15-18 February 2010

5th pandemic vaccine recommended for approval

The Agency's Committee for Medicinal Products for Human Use (CHMP) recommended the granting of a conditional marketing authorisation for a fifth pandemic vaccine, **Humenza** (split virion, inactivated, AF03 adjuvanted influenza H1N1 pandemic vaccine), from Sanofi Pasteur SA, intended for the prophylaxis of influenza in an officially declared pandemic situation. This recommendation was made using an emergency procedure which fast-tracks evaluation of new vaccines developed during a pandemic.

More information on pandemic medicines is available in a separate press release.

Other positive opinions for new medicines adopted

The Committee adopted a positive opinion, recommending the granting of a conditional marketing authorisation, for **Votrient** (pazopanib), from Glaxo Group Ltd, intended for the treatment of patients with advanced renal cell carcinoma. The review for Votrient began on 25 March 2009 with an active review time of 210 days. Votrient is the **63rd orphan medicinal product** to receive a positive opinion by the CHMP.

A marketing authorisation under conditional approval means that further evidence on the medicinal product is awaited. In the case of Votrient this relates to clinical data of pazopanib in comparison with sunitinib in the treatment of patients with advanced renal cell carcinoma. The European Medicines Agency will review new information within one year and update the product information as necessary.

The summaries of opinion for this medicine, including the full indication, can be found here.

Background

A-Z Listing of EPARs

EPARs for authorised medicinal products for human use

Please click on a letter to view an indexed list of products:

A B C D E F G H I J K L M N O P Q R S T U V W X Y Z

A

Name	INN	Revision	Published	Additional Information
Abilify	*INN: aripiprazole*	Rev. 19	21/12/09	
Abraxane	*INN: paclitaxel (as paclitaxel albumin)*	Rev. 3	20/08/09	
Abseamed	*INN: recombinant human erythropoietin alfa*	Rev. 8	04/02/10	
Aclasta	*INN: zoledronic acid*	Rev. 9	08/07/09	
Acomplia	*INN: rimonabant*	Rev. 8	30/01/09	Marketing authorisation withdrawn (30/01/09) Press Release (23/10/08) and Questions and Answers Press Release (19/07/07)

Product Overview

Name of the Medicinal Product
ChondroCelect

Marketing Authorisation Holder
TiGenix NV
Romeinse straat 12 bus 2
BE-3001 Leuven
Belgium

Active Substance
characterised viable autologous cartilage cells expanded ex vivo expressing specific marker proteins

International Nonproprietary Name or Common Name
characterised viable autologous cartilage cells expanded ex vivo expressing specific marker proteins

Pharmaco-therapeutic Group
--

ATC Code
--

Therapeutic Indication
Repair of single symptomatic cartilage defects of the femoral condyle of the knee (International Cartilage Repair Society [ICRS] grade III or IV) in adults. Concomitant asymptomatic cartilage lesions (ICRS grade I or II) might be present. Demonstration of

ChondroCelect H-C-878

European Public Assessment Report

Published 16/11/09

BG	ES	CS	DA	DE	ET	EL	EN	FR	IT	LV	LT	HU	MT	NL	PL	PT	RO	SK	SL	FI	SV	See Glossary of language codes...
◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	Summary for the public
◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	All Authorised Presentations
							◆															Public assessment report (6)
							◆															Procedural steps taken and scientific information after authorisation (8)

Product Information*

05/10/2009 ChondroCelect-H-C-878-00-00

BG	ES	CS	DA	DE	ET	EL	EN	FR	IT	LV	LT	HU	MT	NL	PL	PT	RO	SK	SL	FI	SV	See Glossary of language codes...
◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	

Conditions imposed on member states for safe and effective use

05/10/2009

BG	ES	CS	DA	DE	ET	EL	EN	FR	IT	LV	LT	HU	MT	NL	PL	PT	RO	SK	SL	FI	SV	See Glossary of language codes...
◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	◆	

(*)This document includes:

Annex I - Summary of product Characteristics
Annex IIA - Manufacturing Authorisation Holder responsible for Batch Release
Annex IIB - Conditions of the Marketing Authorisation
Annex IIIA - Labelling
Annex IIIB - Package Leaflet
Please note that the size of the above document can exceed 50 pages.
You are therefore advised to be selective about which sections or pages you wish to print.

Send all queries regarding the Web content to: info@emea.europa.eu
Send all queries regarding the Web functionality to: EMEAwebservices

Glossary

BG = bălgarski
ES = español
CS = čeština
DA = dansk
DE = Deutsch
ET = eesti keel
EL = elliniká
EN = English
FR = français
IT = italiano
LV = latviešu valoda
LT = lietuvių kalba
HU = magyar
MT = Malti
NL = Nederlands
PL = polski
PT = português
RO = română
SK = slovenčina
SL = slovenščina
FI = suomi
SV = svenska

www.ema.europa.eu/humandocs/Humans/EPAR/chondrocelect/chondrocelect.htm

European Medicines Agency
Evaluation of Medicines for Human Use

EMEA/724428/2009

ASSESSMENT REPORT

FOR

ChondroCelect

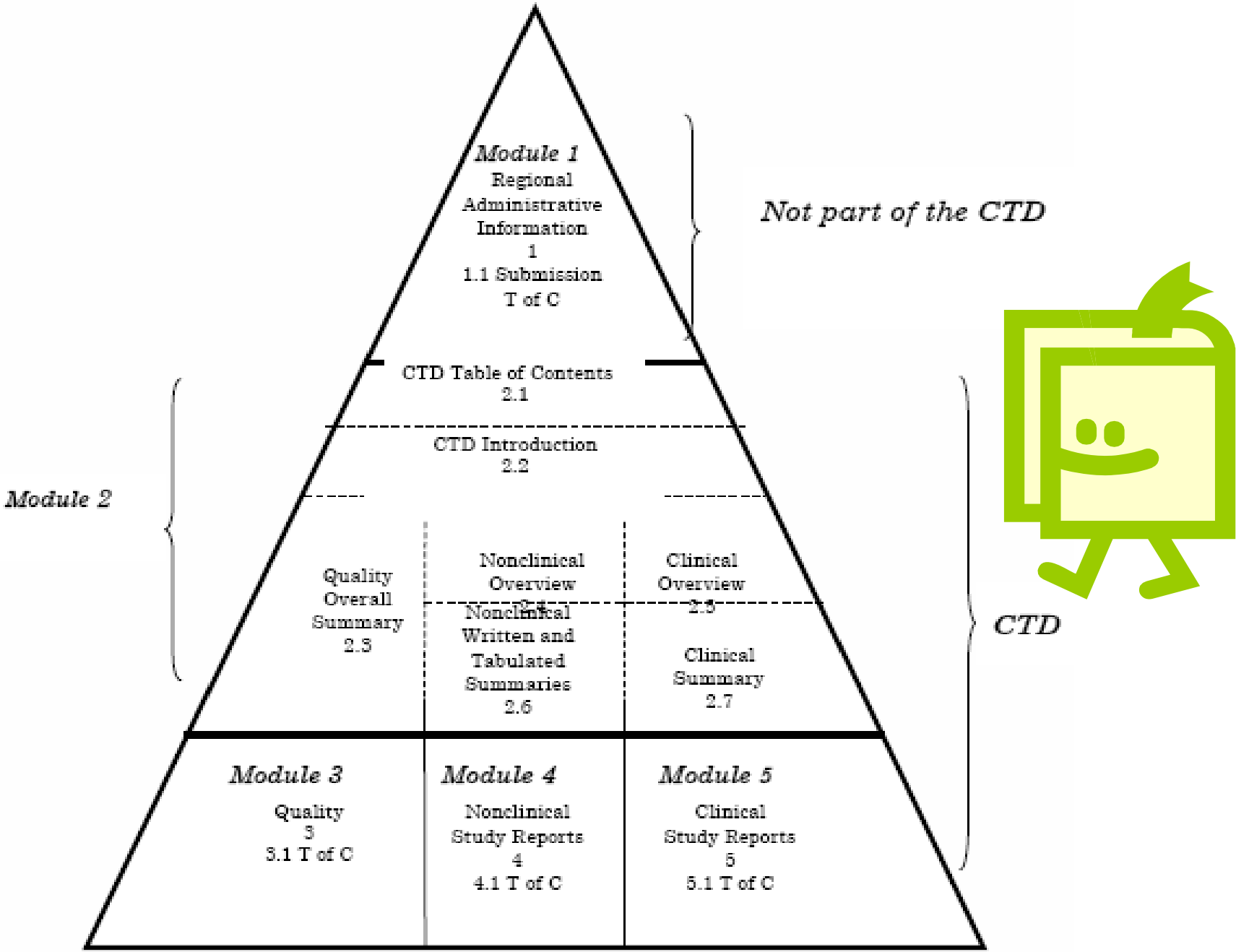
Common name: characterised viable autologous cartilage cells expanded ex vivo expressing specific marker proteins

Procedure No. EMEA/H/C/000878

Assessment Report as adopted by the CHMP with
all information of a commercially confidential nature deleted.

7 Westferry Circus, Canary Wharf, London E14 4HB, UK
Tel. (44-20) 74 18 84 00 Fax (44-20) 74 18 84 16
E-mail: mail@emea.europa.eu http://www.emea.europa.eu

Diagrammatic Representation of the Organization of the ICH CTD
Common Technical Document



European Medicines Agency

February 2004
CPMP/ICH/2887/99

ICH Topic M 4
Common Technical Document for the Registration of Pharmaceuticals for Human Use – Organisation CTD

Step 5

COMMON TECHNICAL DOCUMENT FOR THE REGISTRATION OF PHARMACEUTICALS FOR HUMAN USE: ORGANISATION OF COMMON TECHNICAL DOCUMENT

(CPMP/ICH/2887/99)

CTD Modules

No. of modules : 5

* Module 1 - Regional Information
* Module 2 – Quality overall summary
  Nonclinical overview
  Clinical overview
  Nonclinical written summaries
  Clinical summaries
* Module 3 - Quality
* Module 4 – Safety (Nonclinical)
* Module 5 – Efficacy (Clinical)

http://www.ema.europa.eu/htms/human/ich/ichmulti.htm

Background
Efficacy
Multidisciplinary
Quality
Safety
Considerations
ICH Public Meetings

International Conference on Harmonization (ICH)

Multidisciplinary

	Reference Number	Publication Date	Effective Date
M 2 Electronic Common Technical Document (e-CTD) - Questions and answers	CPMP/ICH/1840/01	November 2003	May 2004
M 2 Business Requirements	CHMP/792476/09	Dec 2009	Dec 2009
M 3 (R2) Non-Clinical Safety Studies for the Conduct of Human Clinical Trials for Pharmaceuticals	CPMP/ICH/286/95	June 2009	December 2009
M 4 Common Technical Document for the Registration of Pharmaceuticals for Human Use - Organisation of CTD	CPMP/ICH/2887/99	November 2003	February 2004
M 4 Common Technical Document for the Registration of Pharmaceuticals for Human Use Questions and Answers	CPMP/ICH/5552/02	June 2004	June 2004
M 4 Q Common Technical Document for the Registration of Pharmaceuticals for Human Use - Quality	CPMP/ICH/2887/02	February 2003	July 2003
M 4 Q Location issues for Common Technical Document for the Registration of Pharmaceuticals for Human Use - Quality Questions and Answers	CPMP/ICH/4680/02	July 2003	August 2003

European Medicines Agency

July 2003
CPMP/ICH/2887/99 - Quality

ICH Topic M 4 Q
Common Technical Document for the Registration of Pharmaceuticals for Human Use - Quality

Step 5

COMMON TECHNICAL DOCUMENT FOR THE REGISTRATION OF PHARMACEUTICALS FOR HUMAN USE QUALITY OVERALL SUMMARY OF MODULE 2 AND MODULE 3: QUALITY

(CPMP/ICH/2887/99 - Quality)

European Medicines Agency

July 2003
CPMP/ICH/2887/99 - Safety

ICH Topic M 4 S
Common Technical Document for the Registration of Pharmaceuticals for Human Use - Safety

Step 5

COMMON TECHNICAL DOCUMENT FOR THE REGISTRATION OF PHARMACEUTICALS FOR HUMAN USE NONCLINICAL OVERVIEW AND NONCLINICAL SUMMARIES OF MODULE 2 ORGANISATION OF MODULE 4

(CPMP/ICH/2887/99 - Safety)

European Medicines Agency

July 2003
CPMP/ICH/2887/99 - Efficacy

ICH Topic M 4 E
Common Technical Document for the Registration of Pharmaceuticals for Human Use – Efficacy

Step 5

COMMON TECHNICAL DOCUMENT FOR THE REGISTRATION OF PHARMACEUTICALS FOR HUMAN USE
CLINICAL OVERVIEW AND CLINICAL SUMMARY OF MODULE 2
MODULE 5: STUDY REPORTS

(CPMP/ICH/2887/99 - Efficacy)

European Medicines Agency

May 2005
CHMP/ICH/175860/2005

ICH Topic M 5 EWG
Routes of administration
Controlled Vocabulary

Step 5

ROUTES OF ADMINISTRATION CONTROLLED VOCABULARY
(CHMP/ICH/175860/2005)

COMMISSION DIRECTIVE 2003/63/EC

of 25 June 2003

amending Directive 2001/83/EC of the European Parliament and of the Council on the Community code relating to medicinal products for human use

'ANNEX I

ANALYTICAL, PHARMACOTOXICOLOGICAL AND CLINICAL STANDARDS AND PROTOCOLS IN RESPECT OF THE TESTING OF MEDICINAL PRODUCTS

PART I

STANDARDISED MARKETING AUTHORISATION DOSSIER REQUIREMENTS

we work for you

EMEA STRUCTURE

:: Overview
:: Mission Statement
:: Organigramme
:: Management
:: Staff
:: European Experts

EMEA COMMITTEES

:: Management Board
:: CHMP
:: CVMP
:: COMP
:: HMPC
:: PDCO
:: CAT

CONTACT & LOCATION

:: General Enquiries
:: Press Office
:: Pharmacovigilance
:: Product Defects
:: EMEA Certificates
:: Documentation
:: European Experts
:: IQM/Audits
:: Business Hours
:: EMEA holidays 2009
:: How to Find Us

MEETINGS & EVENTS

:: Meetings
:: Events

RECRUITMENT

:: Recruitment Policy
:: Job Opportunities

PUBLISHING SERVICES

:: Online Mailing service
:: Important legal notice
:: Copyright Policy

SEE ALSO

:: Calls for Tender
:: Fees Payable to EMEA

EMEA Information on A/H1N1 Influenza Outbreak

Updates on A/H1N1 Outbreak | Information for Visitors

Survey on the design of the EudraCT clinical-trials website

Published 9/6/2009

Following changes to European legislation, the European Medicines Agency is now able to make public some of the information in EudraCT — a database that contains information on clinical trials in Europe.

The set of data that will be made available has been set out in guidelines published by the European Commission. We are interested in knowing which aspects of this information are of most interest to you, how you might use this information and how you would want to access it.

Responses to this survey will help us to design a website that is appropriate for the needs of the public and other interested parties. The deadline for completing the survey is 23/6/2009.

Please take our very short web survey here

EMEA appoints new Head of Internal Audit

Published 2/6/2009

The European Medicines Agency (EMEA) has appointed Dr Edit Weidlich as Head of its Internal Audit Sector.

Edit, who started her new role on 1 May 2009, is responsible for:

- implementing the Agency's annual internal audit programme;
- advising the Agency's senior management on internal/external audit findings;
- related activities to improve the functioning of the Agency and its internal audit operations.

Edit brings to the post extensive experience and knowledge acquired throughout her career, particularly from her previous position as Vice President of the Government Audit Office of Hungary.

Latest Press Releases

See Press Office for archived press releases

08/06/09 EMEA Press Release EU-wide recall of Raptiva (efalizumab) to be initiated

Fast track to a topic...

PRODUCT INFORMATION

:: Human Medicines
:: Veterinary Medicines
:: Safety Announcements
:: Withdrawals and Refusals
:: Summary of Opinions
:: Opinions for Orphan Designation
:: Opinions for medicines used outside the EU

MEDICINES FOR CHILDREN

PATIENT GROUPS

MEDICINES FOR THE ELDERLY

enepp

SME OFFICE

ADVANCED THERAPIES

MEDICINES AND EMERGING SCIENCE

EU ENLARGEMENT

NEW EU LEGISLATION

ROADMAP 2010

EU TELEMATICS

:: EudraPharm Website
:: EudraCT Website

Advanced Therapies

Introduction

Advanced therapy medicinal products (ATMPs) are medicinal products for human use, and are based on gene therapy, somatic cell therapy or tissue engineering. They offer groundbreaking new treatment opportunities for diseases and injuries of the human body. The regulatory framework for ATMPs is established by Regulation (EC) No 1394/2007 on advanced therapy medicinal products which is designed to ensure the free movement of these medicines within the European Union (EU), to facilitate their access to the EU market, and to foster the competitiveness of European pharmaceutical companies in the field, while guaranteeing the highest level of health protection for patients. Regulation (EC) no 1394/2007 also establishes the new expert Committee on Advanced Therapies (CAT).

- Questions and answers on the regulation of advanced therapy medicinal products

Further information relating to EU legislation on advanced therapies is available on the European Commission's Pharmaceuticals website.

Related documents:

- Dossier requirements of centralised applications for CAT members NEW *Updated on 12/08/2009*

Contact Point

Questions relating specifically to the authorisation of advanced therapy medicinal products may be submitted to:
AdvancedTherapies@emea.europa.eu

Introduction

Advanced Therapies Regulation

Regulatory and Procedural Guidance

Special procedures designed for ATMPs

ATMP Classificaton

Certification Procedure

Scientific guidelines

How to get support from the EMEA

Interested parties

See also:

Committee for Advanced Therapies

CAT Monthly Report

Advanced Therapies - Scientific Guidelines

Scientific Guidelines

Scientific guidelines are drawn up with a view to helping applicants in preparing marketing-authorisation applications for medicinal products for human use.

Below are links to specific scientific guidelines that have been developed for ATMPs in area of gene therapy and cell therapy and tissue engineering.

Specific guidance documents concerning pharmacovigilance for ATMPs are also published here below.

Further scientific guidelines relevant to the development of human medicinal products are located in the central repository 'Scientific guidelines for human medicinal products', where they are categorised under quality, non-clinical and clinical sections.

Other information on ongoing activities for ATMPs can be found in the sections of the Gene Therapy Working Party (GTWP) and the Cell-based Products Working Party (CPWP)

Scientific Guidelines

- Gene therapy
- Cell therapy and tissue engineering
- Pharmacovigilance
    - EMEA/149995/08 Guideline on Safety and Efficacy Follow-up - Risk Management of advanced therapy medicinal products
        - Overview of comments received on the above draft

Contact Point

Questions relating specifically to the authorisation of advanced therapy medicinal products may be submitted to: AdvancedTherapies@emea.europa.eu

Gene Therapy								
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	◆				CHMP/CPWP/708420/09	Release for consultation Dec 2009		Deadline for comments 31 Mar 2010
Questions and answers on gene therapy			◆		CHMP/GTWP/212377/08	Dec 2009	Dec 2009	
Revision of the note for guidance on the quality, pre-clinical and clinical aspects of gene transfer medicinal products	◆				CHMP/GTWP/234523/09	Release for consultation Dec 2009		Deadline for comments 31 Mar 2010
ICH Considerations General Principles to Address Virus and Vector Shedding	◆				CHMP/ICH/449035/09	July 2009	July 2009	
Quality, non-clinical and clinical issues relating specifically to recombinat adeno-associated viral vectors	◆				CHMP/GTWP/587488/07	Release for consultation Mar 2009		Deadline for comments Sep 2009
ICH Considerations - Oncolytic Viruses		◆	◆		CHMP/GTWP/607698/08	Oct 2009		
Development of a guideline on the quality, pre-clinical and clinical aspects of medicinal products containing genetically modified cells	◆				CHMP/GTWP/405681/06	Release for consultation May 2007		Deadline for comments Aug 2007
Non-clinical studies required before first clinical use of gene therapy medicinal products	◆	◆	◆	◆	CHMP/GTWP/125459/06	May 2008	Nov 2008	
Follow-up of patients administered with gene therapy medicinal products	◆	◆	◆	◆	CHMP/GTWP/60436/07	Nov 2009	May 2010	
Scientific Requirements for the Environmental Risk Assessment of Gene Therapy Medicinal Products	◆	◆	◆	◆	CHMP/GTWP/125491/06	May 2008	Nov 2008	
Non-Clinical testing for		◆	◆	◆	EMEA/273974/05	Dec 2006	May 2007	

Cell therapy and tissue engineering								
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	◆				CHMP/CPWP/708420/09	Release for consultation Dec 2009		Deadline for comments 31 Mar 2010
Reflection paper on *In-Vitro* cultured chondrocyte containing products for cartilage repair of the knee	◆				CAT/CPWP/288934/09	Release for consultation Sep 2009		Deadline for comments 31 Dec 2009
Potency testing of cell based immunotherapy medicinal products for the treatment of cancer		◆	◆	◆	CHMP/BWP/271475/06	Dec 2007	May 2008	
Guideline on xenogeneic cell-based medicinal products	◆	◆	◆		CHMP/CPWP/83508/09	Dec 2009	1 Jan 2010	
Human cell-based medicinal products		◆	◆	◆	CHMP/410869/06	Jun 2008	Sep 2008	

European Medicines Agency

London, 21 May 2008
Doc. Ref. EMEA/CHMP/410869/2006

COMMITTEE FOR MEDICINAL PRODUCT FOR HUMAN USE (CHMP)

GUIDELINE ON HUMAN CELL-BASED MEDICINAL PRODUCTS

DRAFT AGREED BY CPWP AND BWP	November-December 2006
ADOPTION BY CHMP FOR RELEASE FOR CONSULTATION	25 January 2007
END OF CONSULTATION (DEADLINE FOR COMMENTS)	31 July 2007

CHAPTER 7

COMMITTEE FOR ADVANCED THERAPIES

Article 20

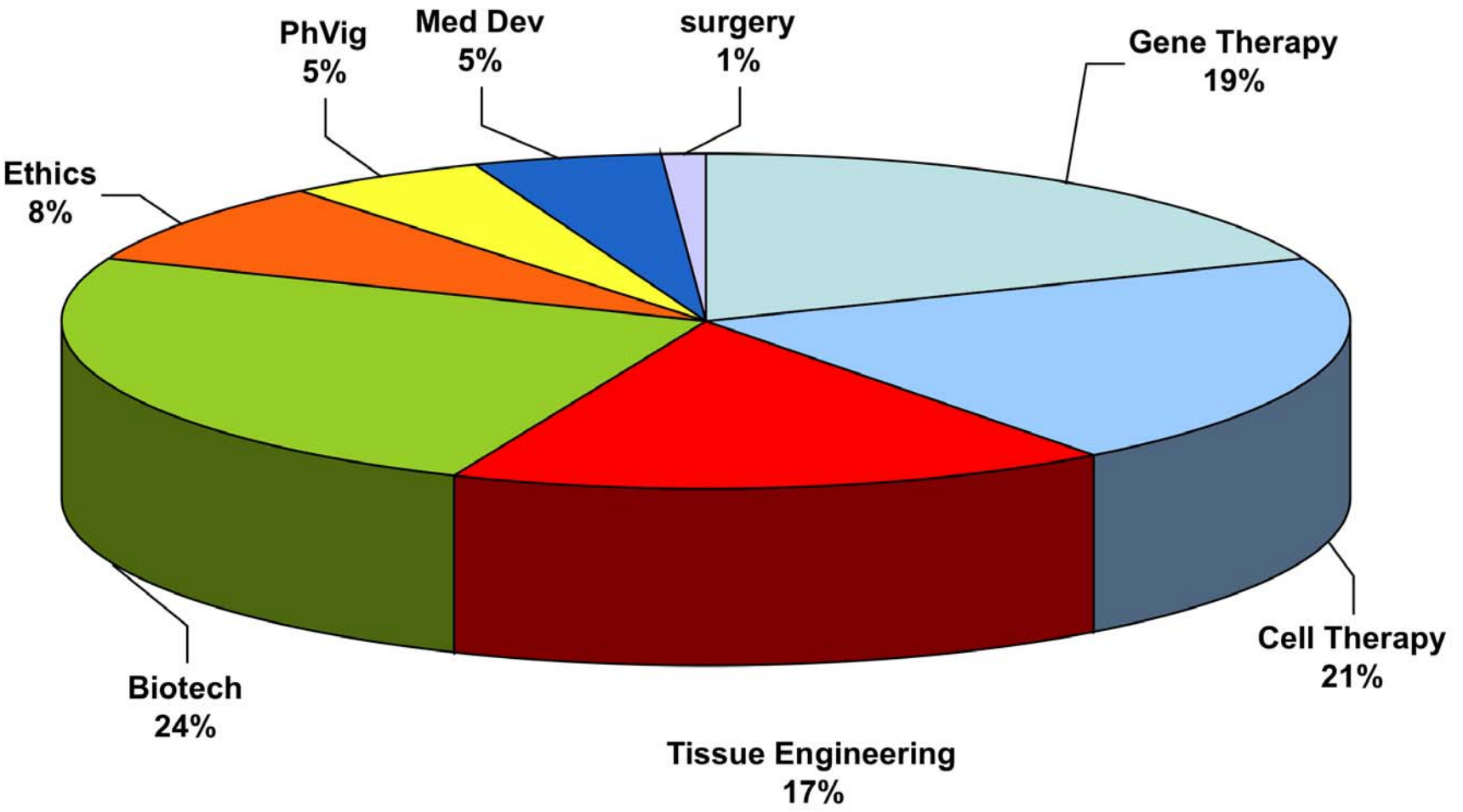
Committee for Advanced Therapies

1. A Committee for Advanced Therapies shall be established within the Agency.

- **5** CHMP members (co-opted or not) with their alternates (from the MS or identified by the CHMP)
- **1** M (1A) per MS from MS not represented through CHMP
- **2** M (2A) representing clinicians
- **2** M (2A) representing patients' associations
- Should include at least 2 M (2A) with expertise in MD

- List of expertise: medical devices, tissue engineering, gene therapy, cell therapy, biotechnology, surgery, pharmacovigilance, risk management and ethics

Expertise within CAT members



Declared expertise as of 15-12-08

Artículo 23

Tareas del Comité de terapias avanzadas

El Comité de terapias avanzadas realizará las siguientes tareas:

a) elaborar un proyecto de dictamen sobre la calidad, la seguridad y la eficacia de un medicamento de terapia avanzada para

su aprobación final por parte del Comité de medicamentos de uso humano y asesorar al mismo sobre cualquier dato obtenido al desarrollar un medicamento de este tipo;

b) asesorar, en virtud del artículo 17, sobre si un producto entra dentro de la definición de medicamento de terapia avanzada;

c) a petición del Comité de medicamentos de uso humano, asesorar sobre cualquier medicamento para el que puedan precisarse, a efectos de la evaluación de su calidad, seguridad o eficacia, conocimientos y experiencia en alguno de los ámbitos científicos mencionados en el artículo 21, apartado 2;

d) brindar asesoramiento en todo lo relativo a medicamentos de terapia avanzada, a petición del director ejecutivo de la Agencia o de la Comisión;

e) prestar asistencia científica para la elaboración de todo documento relacionado con el cumplimiento de los objetivos del presente Reglamento;

f) a petición de la Comisión, brindar conocimientos, experiencia y asesoramiento científicos sobre cualquier iniciativa comunitaria relativa al desarrollo de terapias y medicamentos innovadores que lo requieran en alguno de los ámbitos científicos mencionados en el artículo 21, apartado 2;

g) contribuir a los procedimientos de asesoramiento científico contemplados en el artículo 16 del presente Reglamento y en el artículo 57, apartado 1, letra n), del Reglamento (CE) nº 726/2004.

when the rubber hits the road

- **Chondrocelect** (autologous chondrocytes): marketing authorization granted (July 2009)
- **Cerepro** (AdV-HSVtk): negative opinion on the second marketing authorization application
- **Advexin** (AdV-p53): withdrawn by the applicant before the final opinion

11 March 2010
EMA/151854/2010
Press Office

Press release

Ark Therapeutics Ltd withdraws its marketing authorisation application for Cerepro (sitimagene ceradenovec)

The European Medicines Agency has been formally notified by Ark Therapeutics Ltd of its decision to withdraw its application for a centralised marketing authorisation for the advanced therapy medicinal product Cerepro (sitimagene ceradenovec).

Cerepro received an orphan designation on 6 February 2002 and was intended for the treatment of patients with high-grade operable glioma.

Following the adoption of a negative opinion for Cerepro by the Committee for Medicinal Products for Human Use (CHMP) in December 2009, the company had requested a re-examination of the opinion. This procedure started on 18 February 2010.

Orphan Drug Designation

- Based on Regulation (EC) No 141/2000
- May be obtained at any stage of development
- No fees

European Medicines Agency

New URL: ema.europa.eu

Agencies of the European Union

STRUCTURE
:: Overview
:: Mission Statement
:: Organisational Structure
:: European Experts

COMMITTEES
:: Management Board
:: CHMP
:: CVMP
:: COMP
:: HMPC
:: PDCO
:: CAT

CONTACT & LOCATION
:: General Enquiries
:: Press Office
:: Pharmacovigilance
:: Product Defects
:: Certificates
:: Documentation
:: European Experts
:: IQM/Audits
:: Business Hours
:: Holidays 2010
:: How to Find Us

MEETINGS & EVENTS
:: Meetings
:: Events

RECRUITMENT
:: Recruitment Policy
:: Job Opportunities

PUBLISHING SERVICES
:: Online Mailing service
:: Important legal notice
:: Copyright Policy

SEE ALSO
:: Calls for Tender
:: Fees Payable to the European Medicines Agency

Information about pandemic influenza vaccines

Világméretű influenzajárvány elleni vakcinákra vonatkozó információk

Informazzjoni dwar il-vaċċini għall-influwenza pandemika

Informatie over vaccins tegen pandemische griep

Focetria | Pandemrix | Celvapan | Arepanrix

Visit the Agency's pandemic influenza (H1N1) website

Latest pandemic pharmacovigilance report

European Medicines Agency and Swissmedic agree sharing of information on H1N1 pandemic medicines

Published 15/02/2010

The European Medicines Agency and Swissmedic will from now on be able to exchange confidential information about the authorisation and safety of medicines used in the context of the H1N1 pandemic influenza.

The confidentiality arrangement was agreed between the European Medicines Agency and the Swiss Agency for Therapeutic Products, Swissmedic, on 12 February 2010.

Press Release

New presentation of monthly figures on centralised procedures for human medicines

Published 08/02/2010

The European Medicines Agency has started publishing monthly figures related to the centralised procedure activities for human medicines in a new way.

These monthly figures will provide current information related to the volume and evaluation of marketing authorisation and post-authorisation applications for medicinal products for human use received by the European Medicines Agency. They are complementary to the CHMP monthly report and will be published on the Agency's website within two weeks following the end of the

Fast track to a topic...

New visual identity
More information...

PRODUCT INFORMATION
:: Human Medicines
:: Veterinary Medicines
:: Safety Announcements
:: Withdrawals and Refusals
:: Summary of Opinions
:: Opinions for Orphan Designation
:: Opinions for medicines used outside the EU

TRANSPARENCY POLICY
MEDICINES FOR CHILDREN
PATIENT GROUPS
MEDICINES FOR THE ELDERLY
enepp
SME OFFICE
ADVANCED THERAPIES
MEDICINES AND EMERGING SCIENCE
EU ENLARGEMENT
NEW EU LEGISLATION

Introduction

Orphan designation

Application guidance and related information

Annual report on development

Transfer of orphan designation

Maintenance of orphan designation criteria

Summaries of opinion on orphan designation

Orphan incentives

COMP

Overview

Meeting dates

Reporting

See also:

Community register of orphan medicinal products

Human medicines - Orphan medicinal products

Summaries of opinion on orphan designation

Please use the mail box to comment on summaries of opinion on orphan designation. Please note, however, that the EMEA will not answer individual e-mails received.

Note: Draft status is awarded to a title when the document is pending comments from patients' representative organisation

Search by name	Search by orphan condition	Positive/Negative	Date
gene			Search
adeno-associated viral vector containing modified U7 snRNA gene	Duchenne muscular dystrophy	Positive	11/10/2005
adeno-associated viral vector containing porphobilinogen deaminase gene	acute intermittent porphyria	Positive	12/05/2009
adeno-associated viral vector containing the human alpha sarcoglycan gene	alpha sarcoglycanopathy	Positive	02/04/2009
adeno-associated viral vector containing the human calpain 3 gene	calpainopathy	Positive - Draft	24/02/2009
adeno-associated viral vector containing the human gamma sarcoglycan gene	gamma sarcoglycanopathy	Positive	11/10/2005
adeno-associated viral vector serotype 5 containing the human ABCA4 gene NEW *Updated on lunes, 22 de febrero de 2010*	Stargardt's disease	Positive - Rev 1	22/02/2010
adenoviral vector containing human p53 gene	Li-Fraumeni syndrome	Positive	02/04/2009
adenovirus associated viral vector serotype 4 containing the human RPE65 gene	Leber's congenital amaurosis	Positive	22/01/2008

GT products with orphan drug status

- AAV vector expressing lipoprotein lipase - lipoprotein lipase deficiency
- AdV – HSV-tk gene - high-grade glioma (+ ganciclovir sodium)
- HSV-tk and truncated low affinity NGFR transfected donor lymphocytes - haematopoietic cell transplantation
- Autologous $CD34^{+}$ cells transfected with a RV vector - ADA gene - SCID due to ADA deficiency

Introduction

Orphan designation

Application guidance and related information

Annual report on development

Transfer of orphan designation

Maintenance of orphan designation criteria

Summaries of opinion on orphan designation

Orphan incentives

COMP

Overview

Meeting dates

Reporting

See also:

Community register of orphan medicinal products

Human medicines - Orphan medicinal products

Summaries of opinion on orphan designation

Please use the mail box to comment on summaries of opinion on orphan designation. Please note, however, that the EMEA will not answer individual e-mails received.

Note: Draft status is awarded to a title when the document is pending comments from patients' representative organisation

Search by name	Search by orphan condition	Positive/Negative	Date
cell			Search
allogeneic *ex vivo* expanded umbilical cord blood cells	acute lymphoblastic leukaemia	Positive	30/10/2009
allogeneic *ex vivo* expanded umbilical cord blood cells	acute myeloid leukaemia	Positive	30/10/2009
allogeneic *ex vivo* expanded umbilical cord blood cells	chronic myeloid leukaemia	Positive	20/10/2009
allogeneic *ex vivo* expanded umbilical cord blood cells	Hodgkin's lymphoma	Positive	11/09/2009
allogeneic *ex vivo* expanded umbilical cord blood cells	myelodysplastic syndromes	Positive	20/10/2009
allogeneic human umbilical cord tissue-derived cells	retinitis pigmentosa	Positive	10/07/2008
anti-epithelial cell adhesion molecule / anti-CD3 monoclonal antibody	ovarian cancer	Positive - Rev 1	27/10/2005
autologous CD34+ cells transfected with lentiviral vector containing the human arylsulfatase A cDNA NEW *Updated on lunes, 22 de febrero de 2010*	metachromatic leukodystrophy	Positive - Rev 1	22/02/2010
autologous CD34+ cells transfected with retroviral vector containing adenosine deaminase gene NEW *Updated on lunes, 22 de febrero de 2010*	severe combined immunodeficiency (SCID) due to adenosine deaminase (ADA) deficiency	Positive - Rev 1	22/02/2010

donor lymphocyte preparation depleted of functional alloreactive T-cells	Graft-versus-Host disease	Positive	24/04/2009
ex vivo cultured adult human mesenchymal stem cells	graft-versus-host-disease	Positive - Rev 1	19/11/2009
ex vivo expanded autologous human corneal epithelium containing stem cells	corneal lesions, with associated corneal (limbal) stem cell deficiency, due to ocular burns	Positive	24/04/2009
expanded human allogeneic mesenchymal adult stem cells extracted from adipose tissue	anal fistula	Positive	20/10/2009
genetically modified allogenic (human) tumour cells for the expression of IL-7, GM-CSF, CD80 and CD154, in fixed combination with a DNA-based double stem loop immunomodulator (dSLIM)	renal cell carcinoma	Positive	24/04/2009
herpes simplex virus lacking infected cell protein 34. 5	glioma	Positive	23/10/2003
heterologous human adult liver derived stem cells	Crigler-Najjar syndrome	Positive	02/07/2008
heterologous human adult liver derived stem cells	ornithine-transcarbamylase deficiency	Positive	10/07/2008
HLA-A2 restricted CD8 T-cell line expressing MART-1 T-cell receptor	MART-1 positive malignant melanoma in HLA-A2 positive patients	Positive	15/06/2009
human anti-intercellular adhesion molecule-1 monoclonal antibody	multiple myeloma	Positive	15/06/2009
human autologous bone-forming cells derived from bone marrow stem cells	non-traumatic osteonecrosis	Positive	17/01/2008
human autologous mesenchymal adult stem cells extracted from adipose tissue	anal fistula	Positive - Rev 2	22/10/2009

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

16 February 2010
EMA/COMP/247430/2005 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

autologous CD34+ cells transfected with retroviral vector containing adenosine deaminase gene for the treatment of severe combined immunodeficiency (SCID) due to adenosine deaminase (ADA) deficiency

On 26 August 2005, orphan designation (EU/3/05/313) was granted by the European Commission to Fondazione Telethon, Italy, for autologous CD34+ cells transfected with retroviral vector containing adenosine deaminase gene for the treatment of severe combined immunodeficiency (SCID) due to adenosine deaminase (ADA) deficiency.

What is severe combined immunodeficiency (SCID) due to adenosine deaminase (ADA) deficiency?

Medicines and Emerging Science

How to get support from the EMEA?

There are a number of opportunities available for interaction with the EMEA, depending on the key features and development status of your product/technology. For further information, select from the links below:

- Innovation Task Force (ITF)
- SME Office (dedicated assistance for micro, small and medium-sized enterprises)
- Orphan medicinal product designation
- EMEA scientific advice and protocol assistance
- Pre-submission guidance for users of the centralised marketing procedure

Contact Point

For further contact information see Contact points

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

Medicines and Emerging Science

Innovation Task Force

The Innovation Task Force (ITF) is a multidisciplinary group that includes scientific, regulatory and legal competences, set up to ensure EMEA-wide coordination in the areas of interest and to provide a forum for early dialogue with applicants (click here for ITF mandate).

The ITF, within eight weeks of receipt of a request from an applicant, arranges free-of-charge briefing meetings to facilitate the informal exchange of information and the provision of guidance early in the development process. Where appropriate, this is done in liaison with EMEA scientific committees, working parties and expert groups, and takes into account ongoing international activities.

Briefing meetings are also meant to complement and reinforce existing formal regulatory procedures (e.g. designation of orphan medicinal products, CHMP scientific advice etc).

The ITF — in liaison with the CHMP and, where appropriate, the European Commission — provides regulatory advice on whether new medicinal products for emerging therapies and borderline products are eligible for EMEA procedures. This advice is provided free of charge within 60 days of receipt of a valid request from an applicant.

The scope of the briefing meetings covers regulatory, scientific and other issues arising from the development of new therapies and technologies and borderline products. The applicant's information is kept confidential. EU scientific experts may participate as appropriate.

Briefing meetings may also be the first step for regulatory advice concerning those medicinal products for which confirmation is needed, with regard to their status and the applicability of pharmaceutical legal provisions.

To request a briefing meeting, please complete the request form below and return it to: ITFsecretariat@emea.europa.eu

- Briefing meeting request form
- List of submission dates

Contact Point

For general queries on matters covered by this page please send an e-mail to: ITFsecretariat@emea.europa.eu

All other queries should be sent to: info@emea.europa.eu

briefing meetings

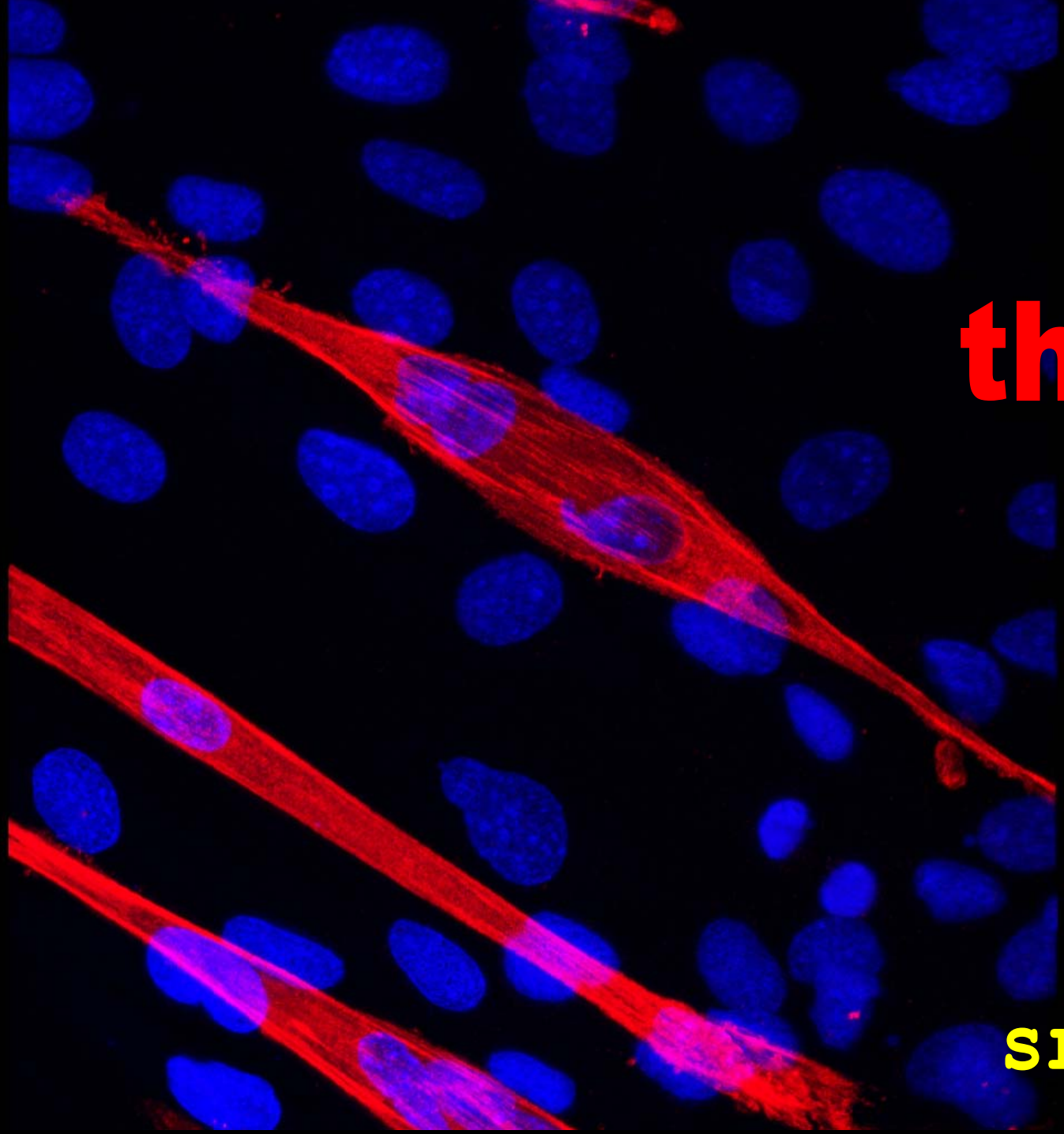
European Medicines Agency
Standard Operating Procedure

Title: Organisation of Briefing Meetings		
PUBLIC		Document no.: SOP/H/3044
Lead Author	Approver	Effective Date: 30-OCT-06
Name: Constantinos Ziogas	Name: Marisa Papaluca-Amati	Review Date: 30-OCT-08
Signature: On file	Signature: On file	Supersedes: N/A
Date: 23-OCT-06	Date: 23-OCT-06	

1. Purpose

This SOP describes the procedure for managing EMEA briefing meetings.

Applicants developing innovative emerging therapies and technologies are encouraged to request informal briefing meetings with the EMEA at different stages of product(s) development and when planning for any of the EMEA available procedures.



thank you!

sruiz@aemps.es