INTERNATIONAL CONFERENCE ON HARMONISATION (ICH)

European Medicines Agency (EMEA)

European Society of Gene and Cell Therapy (ESGCT)

EMEA/ICH WORKSHOP ON VIRAL/VECTOR SHEDDING

Tuesday **30 OCTOBER 2007 (1100-1800)**

in conjunction with:

The XVth Annual Congress of the European Society of Gene and Cell Therapy
27-30 October 2007 Rotterdam, The Netherlands

Date 8 October 2007
Doc. Ref:EMEA/CHMP/GTWP/583410/2007

Background

The ICH Steering Committee (ICH SC) at their meeting in Chicago (October 2006) agreed on the Gene Therapy Discussion Group's (GTDG) proposal to hold an ICH Workshop on Shedding in EU in conjunction with the annual meeting of the XVth Annual Congress of the European Society of Gene and Cell Therapy.

The European Society of Gene and Cell Therapy (ESGCT http://www.esgct.org/index.cfm), accepted to host the Workshop and the subsequent two-day meeting of the ICH Gene Therapy Discussion Group. The Network of Excellence CLINIGENE (http://www.clinigene.eu/), set up within the framework of the European Commission Research activities, kindly agreed to support the workshop financially.

The objective of the workshop is to discuss the data available on the relationship between bio-distribution and shedding of diverse vector systems, the impact on shedding of vector design and the potential shedding-associated safety concerns to be considered in clinical development. "Shedding" in the field of gene therapy means dissemination of the gene therapy product through excreta of the treated subject or patient. The ICH GTDG discussed writing an ICH Considerations document on virus / vector shedding. The purpose of this document would be to address patient safety and public health concerns related to third party exposure.

The expected outcome of this workshop would be to draft "consensus principles" outlining the recommendations of the ICH regions regarding the design of relevant studies for the diverse vectors and safety issues. In addition, information gathered at this workshop will provide a better understanding of the contribution of shedding studies to the risk/benefit assessment of gene therapy products.

Speakers and participants

Speakers (regulators, industry, academia) from different Regions (e.g. Japan, Europe, US, Canada, Switzerland etc.) will participate in the workshop and contribute to the scientific debate supported by their appointing authority.

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PROGRAMME

11.00-12.30 **<u>SESSION 1</u> (Chair: Klaus Cichutek)**	
11.00-11.10 **Introduction** ▪ What is viral shedding and why is it important? ▪ Theoretical risks to patients, their families and friends, healthcare workers and the environment	**Klaus Cichutek (10mins)** Paul-Ehrlich-Institute, Germany EMEA, EU
11.10-12.10 Current regulatory requirements for viral shedding studies for gene therapy vectors	**Teruyo Arato (20mins)** Pharmaceuticals and Medical Devices Agency, Japan **Sharon Longhurst (20mins)** Medicines and Healthcare products Regulatory Agency UK EMEA, EU **Daniel Takefman (20mins)** FDA, USA **Andreas Marti (20mins)** Swiss Agency for Therapeutic Products, Switzerland (EFTA)
12.10-12.30 **Panel discussion (20mins)**	
12.30-13.30 **LUNCH BREAK**	
13.30-15.30 **<u>SESSION 2</u> (Chair: Stephanie Simek)**	
13.30-13.40 **Introduction**	**Stephanie Simek (10mins)** FDA, USA
13.40-15.10 **Non clinical aspects:** Animal models (suitability of the animal model for the particular vector, influence of application route,	**Edwin van Amersfoot (30mins)** Amsterdam Molecular Therapeutics, The Netherlands

biodistribution, time of shedding, samples, possibility for bridging studies with similar vectors but different transgene)

Clinical aspects: — **Samuel Wadsworth (30mins)**, Genzyme, US

Set up and performance of clinical studies

- Define the scope and the extent of shedding studies in man: how pre-clinical data inform clinical studies: translational issues – AAV specific issues pertaining to vector shedding in gene therapy clinical trials

- Shedding studies: moving from phase I. studies through to phase III: sample collection, shipping examples, analysis and methods, preparing for launch of the product — **Didier Guilhem (30mins)**, World Courier France, France

15.10-15.30
Panel discussion (**20mins)**

Shedding studies: are we ready for harmonisation?
With participation of Stephanie Simek (FDA)

15.30-16.00
COFFEE BREAK

16.00-18.00
SESSION 3 (Chair: Teruhide Yamaguchi)

16.00-16.10
Introduction — **Teruhide Yamaguchi (10mins)**, National Institute of Health Sciences, Japan

Should all Viral Vectors be treated in the same way in relation to shedding?

- Review of experience of shedding data for in vivo gene therapy

  - **Adenoviruses** — **Toshi Fujiwara**, Okayama University, Japan; **David Eckland**, Ark Therapeutics, United Kingdom; **Ingrid Boltje**, Genzyme, US
  - **AAVs** — **Caroline Le Guiner**, Inserm, France (ESGCT); **Janneke Meulenberg**, Amsterdam Molecular Therapeutics, The Netherlands
  - **Seneca Valley Virus** — **Paul Hallenbeck**, Neotropix, US

• **Shedding of viral vectors during clinical gene therapy**	**Ellen Schenk-Braat (5mins)** Erasmus MC, The Netherlands
17.40-18.00 **Panel discussion** (**20mins)** With participation of Alan Boyd (Alan Boyd consultants)	
18.00-18.20 **CONCLUSIONS**	**Klaus Cichutek**