EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

The contribution of CAT to the international development of ATMPs

CAT scientific symposium on ATMPs, 10 October 2024

Presented by Patrick Celis
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Content

1. ATMP cluster
2. International Pharmaceutical Regulators Programme (IPRP)
3. ICH
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6. Concluding remarks

CAT international collaboration

ATMP Cluster TCs

Started in 2008, before CAT

- EMA + FDA staff
- Since mid of 2009: CAT members/experts + FDA colleagues
- Extended participation by Health Canada (2012) and PMDA (2016)

5-6 ATMP cluster TCs/year

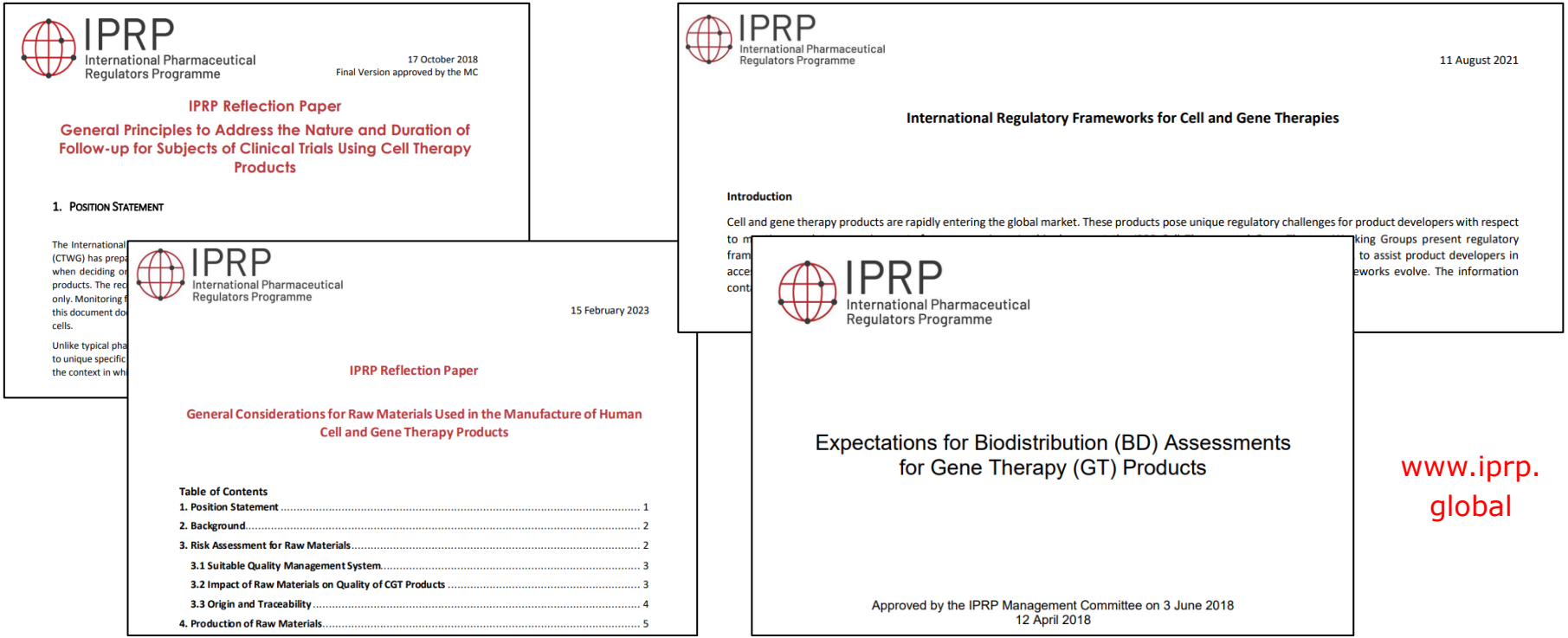
Scope of discussions:

- Regulatory issues, guidance documents, products under development (SA, IND), products under evaluation, issues with authorised products
- Already in Dec 2008: product discussion – cell products for cartilage repair

International Pharmaceutical Regulators Forum

- Main goal: information sharing & convergence/harmonisation activities
- Two working groups established
  - Cell therapy WG (2011)
  - Gene therapy WG (2012)
  - Recently merged to one group: Cell and Gene therapy WG
- International TCs: 2-3 / year & F2F mtgs (Auckland (2013), New Orleans (2015), London (2017), Vancouver (2024))
- Very broad participation: ICH members & observers, Authorities from Asia & South America, WHO

International Pharmaceutical Regulators Forum - outcomes



IPRP
International Pharmaceutical Regulators Programme

17 October 2018
Final Version approved by the MC

IPRP Reflection Paper

General Principles to Address the Nature and Duration of Follow-up for Subjects of Clinical Trials Using Cell Therapy Products

1. POSITION STATEMENT

IPRP
International Pharmaceutical Regulators Programme

15 February 2023

IPRP Reflection Paper

General Considerations for Raw Materials Used in the Manufacture of Human Cell and Gene Therapy Products

Table of Contents

IPRP
International Pharmaceutical Regulators Programme

11 August 2021

International Regulatory Frameworks for Cell and Gene Therapies

Introduction

Cell and gene therapy products are rapidly entering the global market. These products pose unique regulatory challenges for product developers with respect

Groups present regulatory

to assist product developers in

eworks evolve. The information

IPRP
International Pharmaceutical Regulators Programme

Expectations for Biodistribution (BD) Assessments for Gene Therapy (GT) Products

Approved by the IPRP Management Committee on 3 June 2018
12 April 2018

www.iprp.global

ICH

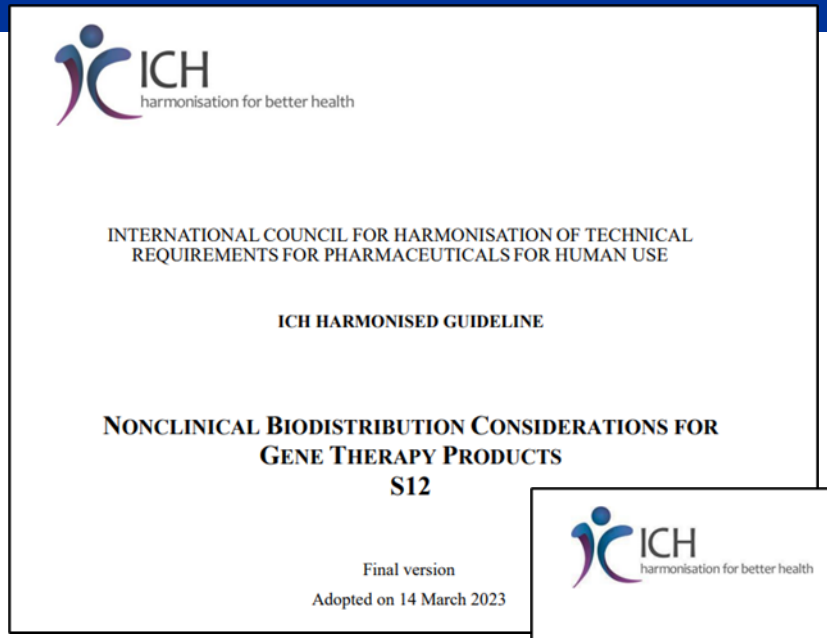
- Gene therapy discussion group
  - Established in Sept 2002 → involvement of GT experts from EU / gene therapy expert group
  - CAT-GTWP involvement since 2009
  - Discontinued in Sept 2011
  - Key document developed by ICH GTDG
    1. General Principles to Address Virus and Vector Shedding (June 2009)
    2. ICH considerations on Oncolytic Viruses (September 2009)
    3. General Principles to Address the Risk of Inadvertent Germline Integration of Gene Therapy Vectors (October 2006)

ICH

www.ich.org

Support of CAT members to recent/ongoing ICH activities:

- ICH S12 guideline



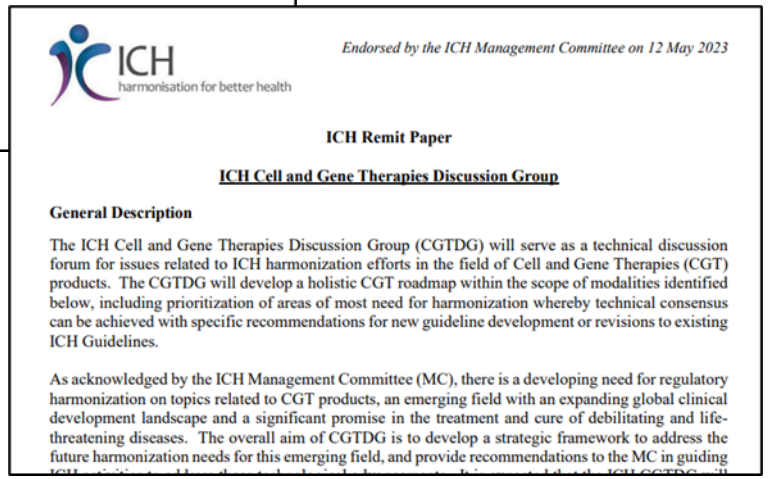
ICH
harmonisation for better health

INTERNATIONAL COUNCIL FOR HARMONISATION OF TECHNICAL REQUIREMENTS FOR PHARMACEUTICALS FOR HUMAN USE

ICH HARMONISED GUIDELINE

NONCLINICAL BIODISTRIBUTION CONSIDERATIONS FOR GENE THERAPY PRODUCTS
S12

Final version
Adopted on 14 March 2023



ICH
harmonisation for better health

Endorsed by the ICH Management Committee on 12 May 2023

ICH Remit Paper

ICH Cell and Gene Therapies Discussion Group

General Description

The ICH Cell and Gene Therapies Discussion Group (CGTDG) will serve as a technical discussion forum for issues related to ICH harmonization efforts in the field of Cell and Gene Therapies (CGT) products. The CGTDG will develop a holistic CGT roadmap within the scope of modalities identified below, including prioritization of areas of most need for harmonization whereby technical consensus can be achieved with specific recommendations for new guideline development or revisions to existing ICH Guidelines.

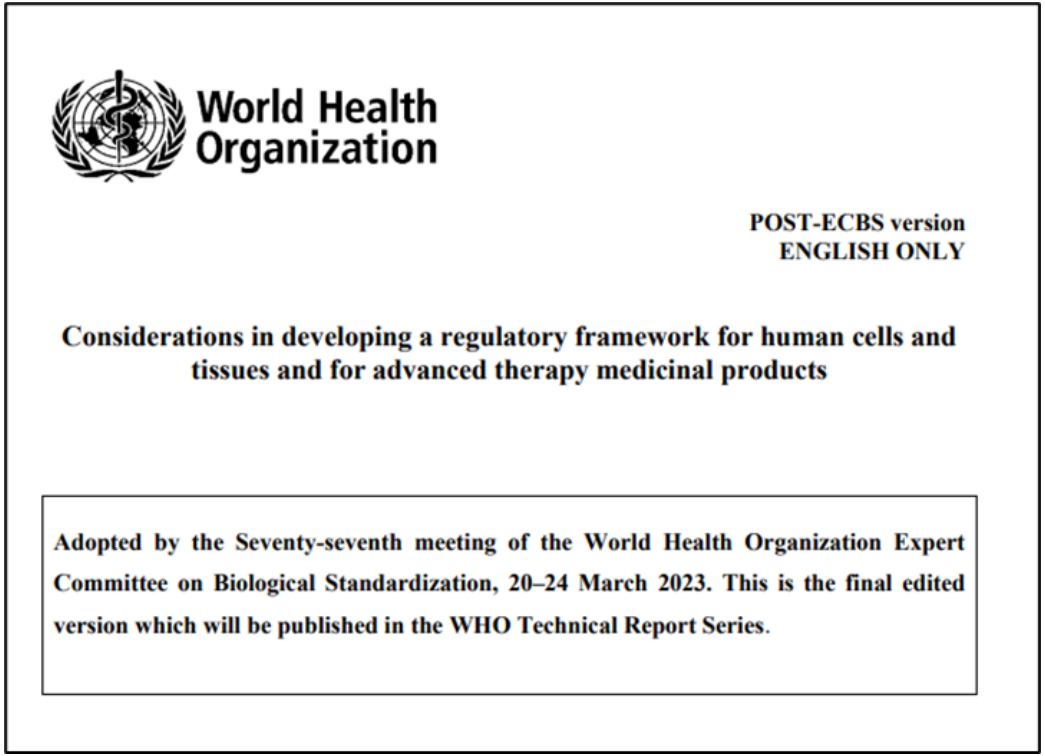
As acknowledged by the ICH Management Committee (MC), there is a developing need for regulatory harmonization on topics related to CGT products, an emerging field with an expanding global clinical development landscape and a significant promise in the treatment and cure of debilitating and life-threatening diseases. The overall aim of CGTDG is to develop a strategic framework to address the future harmonization needs for this emerging field, and provide recommendations to the MC in guiding

- Cell and Gene Therapy discussion group (May 2023)

WHO

CAT members/experts also contributed to the recent WHO initiatives to support the development of legal/regulatory framework in low/medium income countries

- WHO considerations (2023)
- Training session (Oman, 2023)



World Health Organization

POST-ECBS version
ENGLISH ONLY

Considerations in developing a regulatory framework for human cells and tissues and for advanced therapy medicinal products

Adopted by the Seventy-seventh meeting of the World Health Organization Expert Committee on Biological Standardization, 20–24 March 2023. This is the final edited version which will be published in the WHO Technical Report Series.

annex-3---hcts-atmps-regulatory-considerations---clean-for-posting---12-may-2023.pdf (who.int)

Bilateral interactions and collaborative initiatives

- Ad-hoc bilateral TC with international regulators (mainly with FDA)
  - Products under development (SA, IND)
  - Products under evaluation (MAA, BLA) & authorised ATMPs
- Exchange of documents such as SA reports, IND meeting minutes, LoQ …
- CoGenT pilot for ultra-rare GTMPs
  - Feasibility pilot to scope out possibilities for even closer interactions of EMA & FDA during BLA/MAA evaluation
  - (Very) limited number of products

Concluding remarks

- Need for harmonisation of requirements for ATMPs recognised by CAT
  - international dimension of ATMP development recognised by CAT from the very beginning
- CAT is playing an important role in harmonisation
  - at EU level: CAT meetings act also as safe-harbour for members to discuss national ATMP issues outside of CAT's remit (e.g. clinical trials, HE)
  - involvement in international harmonisation activities
  - bilateral interactions with other regulators
- CAT ambassadors: role of CAT members & experts in contributing and giving talks at international meetings

Thanks for your attention!

Further information

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