ICH
harmonisation for better health

Cell & Gene Therapy Discussion Group (CGTDG)

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International Council for Harmonisation of Technical Requirements
for Pharmaceuticals for Human Use

ICH CGTDG Members

Regulatory/Administrative Authorities

- EU Commission, Europe
- FDA, USA
- MHLW/PMDA, Japan
- ANVISA, Brazil
- EDA, Egypt
- HSA, Singapore
- MFDS, Republic of Korea
- MHRA, UK
- NMPA, China
- SFDA, Saudi Arabia
- TFDA, Chinese Taipei
- Health Canada, Canada
- Swissmedic, Switzerland
- ANMAT, Argentina
- ANPP, Algeria

Industry Associations

- EFPIA
- JPMA
- PhRMA
- BIO
- IFPMA
- IGBA

Other/International Associations

- EDQM
- USP
- IPRP
- WHO

25 member organizations representing 45 countries

Remit of CGTDG

- Serve as technical discussion forum for issues related to ICH harmonisation efforts in the field of advanced therapy medicinal products (ATMP)
  - Focus on more mature modalities: *in vivo* viral vector-based gene therapy products and *ex vivo* genetically modified cells
- Align on high level principles where there are special considerations for ATMPs
- Develop strategic framework for future harmonisation efforts
  - Provide recommendations to guide ICH activities in the ATMP field across clinical, nonclinical, and quality disciplines
  - Assess existing ICH guidelines (Q, S, E, and M) for applicability to ATMPs
- Generate strategic roadmap of recommendations for new ICH guidelines or revisions/annexes to existing guidelines, as well as suggestions for ATMP examples in training materials

ICH CGT DG by the Numbers

- Kick-off meeting: **October 24th, 2023**
- **50** Individuals comprising the DG: **25** orgs and **45** countries
- **37** ICH CGT DG Group Meetings (**~ 60** online hours together!)
- **101** Meetings (including subteam and DG Leads meetings!)
- **4** Days of In-Person meetings (Montreal, Nov 2024) (**~ 32** hours)
- **801** Documents generated on our Sharepoint Site
- Final meeting for endorsement of final deliverables: **Oct 21, 2025**
- **2 years later... a 28-page <u>Final Recommendation Paper</u> (excluding Annex II) with 7 prioritized recommendations**

ATMP Product Terminology and Classification

WHO TRS 1048 Annex 3 (2023): “Considerations in developing a regulatory framework for human cells and tissues and for **advanced therapy medicinal products**”

From executive summary of WHO workshop (May 2024 in Oman):

"Cells, tissues, and gene therapy products (CGTPs) represent biological products across a spectrum of complexity from those that require minimal oversight (such as a simple allogeneic skin graft) to those (such as chimeric antigen receptor T-cells) which require significant regulatory evaluation to demonstrate product safety and efficacy before marketing authorization."

"A risk-based approach for regulation of CGTPs, classification, terminology for CGTPs and the importance of global harmonization especially on terminology were discussed."

Global ATMP Regulatory Framework

CGTDG built common understanding of existing regulatory frameworks across regions.

From DG members and observers: More than **300** national/regional ATMP regulations/guidelines as of December 2024

- Annex II Explanatory Note: The Regulations and Guidance listed in Annex II were considered interim deliverables of the ICH CGTDG that were intended to illustrate the global landscape of ATMP-relevant regulatory frameworks (e.g., regulations, guidance documents, guidelines) as of December 2024. Members of the ICH CGTDG listed these documents from their respective countries/regions. This listing should not be considered comprehensive or compiled in a standardized way given regional differences in product classification and regulatory frameworks for ATMPs. Annex II is simply included as a reference here as supportive information to the ICH CGT DG work and may also be a useful centralization of such information. There is no expectation for ICH to maintain or update this list in the future.

Strategic Roadmap

Recommendations with regard to future ATMP related guidelines

1. Annex to ICH Q5E: Comparability of ATMPs subject to changes in their manufacturing process – endorsed by ICH MC in May 2025

2. New ICH E guideline on long-term follow up (LTFU)

3. Annex to ICH Q11 on development and manufacture of ATMPs

4. New ICH S guideline on nonclinical evaluation of ATMPs

5. Annex to ICH E8 on considerations for early phase/exploratory clinical trials evaluating ATMPs

6. Annex to ICH Q5A(R2) to address viral safety for cell-based ATMPs

7. Case studies in training materials for ICH Q2 and Q14 for unconventional analytical method development and validation

The CGTDG Recommendation Paper is published here on the ICH website

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Thank You

Q&A

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