

# ICH Cell and Gene Therapy Discussion Group (CGT DG) Work Plan

## February 13, 2025

**Topic Adoption date:** *August 2023*

**Rapporteur:** *Dr. Kathleen Francissen, BIO*

**Regulatory Chair:** *Dr. Melanie Eacho, FDA, United States*

**Last Face-to-Face Meeting:** *Montreal, Canada, November 2024*

### 1. Key milestones

#### 1.a. Current status of key milestones

| Past completion date | Milestone                                                                                                                                                                                                                                     |
|----------------------|-----------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| <b>October 2023</b>  | <i>ICH CGT DG Kick-Off Meeting</i>                                                                                                                                                                                                            |
| <b>December 2023</b> | <i>Provide review to EWG of ICH Q1/Q5C Revision (Annex on ATMPs)</i>                                                                                                                                                                          |
| <b>February 2024</b> | <i>Reached consensus on use of “ATMP” and associated terminology put forth by WHO for ICH CGT DG business</i>                                                                                                                                 |
| <b>May 2024</b>      | <i>Build common understanding of existing high level regulatory frameworks across regions: Regulators verify the accuracy of map of global CGT regulatory frameworks.</i>                                                                     |
| <b>May 2024</b>      | <i>Compiled information gathered by trade organizations (via survey of their member companies) on gaps and/or areas of divergence encountered while trying to conduct global clinical trials and/or commercialize CGT products globally</i>   |
| <b>June 2024</b>     | <i>Consolidate and prioritize areas for harmonization: Prioritization discussion based on regulatory framework gap assessment in combination with industry-led survey results</i>                                                             |
| <b>October 2024</b>  | <i>Consolidated high-level principles document based on: a) Ex vivo genetically modified immune cell products (including both autologous and allogeneic), and b) In vivo viral vector-based gene therapy products. Consolidated document.</i> |

#### 1.b. Key Deliverables

| Approval Date      | Deliverable                           |
|--------------------|---------------------------------------|
| <b>August 2023</b> | <i>High Level Principles Document</i> |

|                    |                                                                                                                                                                |
|--------------------|----------------------------------------------------------------------------------------------------------------------------------------------------------------|
| <b>August 2023</b> | <i>Strategic Roadmap of areas in most need of harmonization with recommendations for revisions to existing ICH guidelines and/or new guideline development</i> |
| <b>August 2023</b> | <i>Recommendation Paper summarizing roadmap</i>                                                                                                                |

### 1.c. Future anticipated key milestones

| <b>Expected future completion date</b> | <b>Milestone</b>                                                                                                                                                                                                    |
|----------------------------------------|---------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| <b>Apr 2025</b>                        | <i>Review existing ICH guidelines (Q, S, E, M) in terms of their applicability to CGT product classes in scope and identify areas where harmonisation already exists and areas for improvement in harmonisation</i> |
| <b>June 2025</b>                       | <i>Holistic CGT roadmap w/ prioritization of areas in most need of harmonization with recommendations for revisions to existing ICH guidelines and/or possibly new guideline development</i>                        |
| <b>October 2025</b>                    | <i>Recommendation paper summarizing high level principles and roadmap described above, for ICH MC and Assembly review and endorsement</i>                                                                           |

## 2. Timeline for specific tasks

**Bold = tasks; Nonbold = subtasks that lead to the task**

| <b>Beginning date</b> | <b>End date</b>   | <b>Task / Activity</b>                                                                        | <b>Details</b>                                                                                                                                                                                                                     |
|-----------------------|-------------------|-----------------------------------------------------------------------------------------------|------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| <b>Aug. 2024</b>      | <i>Apr. 2025</i>  | <i>Internal consultation for revisions to consolidated high level principles</i>              | <i>Upon completion of prior activity of identifying and prioritizing topics for harmonization, the ICH CGT DG shall review the consolidated High Level Principles document with additional insights and update as appropriate.</i> |
| <b>January 2025</b>   | <i>March 2025</i> | <i>Analyses of prioritized issues/topics with added relevant national/regional guidelines</i> | <i>List regional guidelines for each topic</i>                                                                                                                                                                                     |
| <b>February 2025</b>  | <i>April 2025</i> | <i>Outline/Draft recommendations</i>                                                          | <i>Outline of recommendations</i>                                                                                                                                                                                                  |
| <b>March 2025</b>     | <i>April 2025</i> | <i>Assess harmonization/divergence of current</i>                                             | <i>In subteams and collectively as DG; identify interdisciplinary areas</i>                                                                                                                                                        |

|                       |                     |                                                                                                                                                    |                                                                                                                                                                                                                                                                                           |
|-----------------------|---------------------|----------------------------------------------------------------------------------------------------------------------------------------------------|-------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
|                       |                     | <i>expectations – flag areas of divergence</i>                                                                                                     |                                                                                                                                                                                                                                                                                           |
| <b>August 2024</b>    | <b>April 2025</b>   | <b><i>Review existing ICH guidelines (Q, S, E, and M) with the high level principles and the prioritized topics for harmonization in mind.</i></b> | <b><i>Subteams to review guidelines and develop specific recommendations on revisions to existing ICH guidelines and/or possibly new guideline development (where, for example, specific guidance may be needed due to the nature of the CGT product and/or technical necessity).</i></b> |
| <b>May 2025</b>       | <b>June 2025</b>    | <b><i>1<sup>st</sup> Draft Recommendation Paper</i></b>                                                                                            | <b><i>Initial compilation of prioritized topics with recommendations</i></b>                                                                                                                                                                                                              |
| <b>August 2024</b>    | <b>June 2025</b>    | <b><i>Holistic CGT roadmap</i></b>                                                                                                                 | <b><i>Generate holistic CGT roadmap w/ prioritization of areas in most need of harmonization with recommendations for revisions to existing ICH guidelines and/or new guideline development</i></b>                                                                                       |
| <b>June 2025</b>      | <b>July 2025</b>    | <b><i>2<sup>nd</sup> Draft Recommendation Paper</i></b>                                                                                            | <b><i>DG review and feedback</i></b>                                                                                                                                                                                                                                                      |
| <b>July 2025</b>      | <b>July 2025</b>    | <b><i>Informal consult to EWGs and DG Member Agencies for review</i></b>                                                                           | <b><i>Send to active EWGs working on ATMP topics to review and provide comments to ensure consistency on Recommendation Paper</i></b>                                                                                                                                                     |
| <b>July 2025</b>      | <b>Sept 2025</b>    | <b><i>3<sup>rd</sup> Draft Recommendation Paper</i></b>                                                                                            | <b><i>DG finalized draft w/DG consensus</i></b>                                                                                                                                                                                                                                           |
| <b>September 2025</b> | <b>October 2025</b> | <b><i>Final Recommendation Paper with DG Sign-Off</i></b>                                                                                          | <b><i>Recommendation paper summarizing high level principles and roadmap described above, for ICH MC and Assembly review and endorsement</i></b>                                                                                                                                          |