

2020 Work Plan

Gene Therapy Working Group (GTWG)

Date: 29 October 2019

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1. KEY MILESTONES AND DELIVERABLES

1.1. Current status of key milestones and deliverables

Past completion date	Objective	Key Milestone or Deliverable
June 2019	ICH Guideline	ICH accepted Japan's proposal to further develop the GTWG Reflection Paper on Biodistribution of Gene Therapy Vectors
October 2019	Gather regulatory frameworks from participating regulators.	In collaboration with the Cell Therapy Working Group (CTWG), frameworks from 10 regulatory agencies were collected.
October 2019	Teleconference	Discuss next steps for framework project, BIO's draft reflection paper, and new topic for convergence.

1.2. Future anticipated key milestones

Expected future completion date	Objective	Key Milestone or Deliverable
August 2020	Communicate the outcome of the framework project to the public.	Working with the CTWG, publish a manuscript on the frameworks project.
February 2020	Initiate project on convergence on approaches for Long Term Follow-up (LTFU) for patients receiving gene therapy products.	Identify a drafting committee.

2. TIMELINE FOR SPECIFIC TASKS

Beginning date	End date	Task / Activity	Details
February 2020		Teleconference	Topics: Identify drafting committee for LTFU topic, regulatory updates
February 2020	March 2020	Drafting committee teleconference	Outline the goals and outcomes of the LTFU project.
April 2020		Working group teleconference	Collect information on LTFU from participating regulators, discuss status of the framework manuscript.
March 2020	June 2020	Drafting of manuscript on framework project.	Develop draft manuscript by drafting committee.
June 2020	August 2020	Circulate framework manuscript among regulators	Commenting and editing of draft manuscript.
July 2020		Working group teleconference	Sharing regulatory updates, status discussions on LTFU project and framework project.
October 2020	December 2020	Clearance of framework manuscript by regulatory authorities.	Circulate final draft for permission to publish from regulatory authorities.
Expected January 2021		Acceptance of the manuscript by IPRP MC	Manuscript to be submitted to MC and then journal for publication.

3. SUMMARY OF ANY CURRENT ISSUE

The GTWG is seeking advice on adding new member countries to the working group. It is important to note that for this working group adding new countries/new individuals to the working group fulfils the mandate to provide an open forum to 1) Maintain knowledge of regulatory activities in participating regions; 2) Identify topics for regulatory convergence; 3) Collaboration and information sharing with other international (ICH) and regional bodies (APEC, PANDRH, etc) and to collaborate in terms of training of international regulators.

4. NECESSITY FOR FACE-TO-FACE MEETING

To further the preparation of a manuscript on the framework project and to initiate a new topic, the GTWG may meet in-person with the along with the drafting committee for the framework project from the CTWG on the margins of the International Society for Cellular Therapy (ISCT) Annual Meeting in Paris, May 27 – 30, 2020. This will be discussed after the CTWG's the November 2019 teleconference.