IPRP
International Pharmaceutical Regulators Programme

Mandate Document

Gene Therapy Working Group (GTWG)

Version 1.0

endorsed by the MC on 9 June 2015

Document History

Version number	Action	Date of endorsement
v1.0	First version of the Mandate document (dated 18 Nov 2014) presented to the MC for endorsement at the meeting in Fukuoda, Japan in June, 2015.	**09 June 2015**

1. General Considerations

1.1. Statement of the Perceived Problem

Consistent international assessment of gene therapy products in an environment where regulations vary in specific regions is essential to enhance the global availability of safe and effective GT products has been lacking.

1.2. Expected Benefits

The GTWG provides an international networking forum to discuss emerging scientific developments and concerns for the regulation of gene therapy products. This open forum allows for the less experienced regulators to learn from the more experienced regulators and supports the efforts of APEC, PAHO and ICH.

1.3. Background to the proposal

The field of gene therapy is undergoing rapid scientific and clinical innovation worldwide. The Gene Therapy Discussion Group (GTDG) within the ICH framework was initially established in 2000 to *prospectively* work towards harmonization in an emerging product field rather than undertake the more difficult exercise of harmonization after regional policies and guidance documents were in place. The GTDG produced three ICH considerations papers to put forth harmonizing principles for gene therapy topics. In 2009, the M6 Expert Working Group (EWG) was formed to write an ICH guideline on virus/vector shedding. Due to insufficient fielding of representatives from the full ICH Steering Committee membership, the GTDG and M6 EWG were discontinued. However, the number of gene therapy clinical trials conducted worldwide has not diminished and the numbers of reported breakthroughs in the field of gene therapy have increased.

Since the ICH GTDG is no longer operating under the conventional ICH mechanism, FDA proposed that an alternative venue to engage global regulators in the area of gene therapy products be considered. On 11 October 2012, 9 members of the global regulatory community including Brasil ANVISA, European Medicines Agency (EMA), Health Canada, India National Institute of Biologicals (NIB), Japan Ministry of Health, Labour and Welfare(MHLW)/Pharmaceutical and Medical Devices Agency (PMDA), South Korea Ministry of Food and Drug Safety (previously known as Food and Drug Administration (KFDA)), Singapore Health Sciences Authority (HSA), Swissmedic, and U.S. Food and Drug Administration (US FDA) convened via teleconference. The Agencies agreed to establish the Regulators Forum Gene Therapy Discussion Group (RFGTDG), allowing the continuation of the ICH GTDG's activities in an adapted form. With the integration of the RFGTDG into the IPRF organization, the name of the group was changed to the IPRF GT Working Group (IPRF GTWG). Effective November, 2017, the IPRF and International Generic Drug Regulators Programme (IGDRP) were consolidated into the International Pharmaceutical Regulators Programme (IPRP).

2. SCOPE

- Viral vectors, oncolytic vectors and genetically modified bacterial vector based GT products, genome editing technologies
- Non-proprietary information regarding regulatory experiences with GT products including focused topic discussions to enable sharing of views and regulatory best practices among international regulators.
- Topics that with adequate rationalization are potentially suitable for regulatory convergence or harmonisation

3. OBJECTIVES AND KEY DELIVERABLES

The primary goals of the Working Group are information sharing and regulatory convergence.

3.1. Objectives

- Objective 1: Retain knowledge of regulatory activities in participating regions

  At each meeting or teleconference, conduct a roll call of regulatory updates to include new regulations, new guidance or guidelines, and recently approved products

- Objective 2: Identify Topics for regulatory convergence or harmonization

  Identify possible topics for harmonization or regulatory convergence by conducting scoping exercises to identify commonalities and differences in regulatory approaches.

- Objective 3: Collaborations and information sharing with other international and regional bodies

  Explore information-sharing processes with other international (ICH) and regional bodies (APEC, PANDRA, etc.) and collaborate in terms of training of international regulators

3.2. Key deliverables

- Deliverable 1: Post results of projects on IPRP website

  Provide meeting summaries and results of specific projects to the MC for posting on the IPRP website.

- Deliverable 2: Draft Reflection Papers

  Reflection Papers will be drafted on topics for regulatory convergence or to communicate to stakeholders the current state of regulation for gene therapy products.

- Deliverable 3: White Papers and Journal Articles

  The GTWG will publish articles in trade journals and peer-reviewed journals as appropriate topics are developed

4. COMPOSITION

The GTWG is comprised of international regulatory authorities and persons representing harmonization initiatives (APEC, WHO, and PAHO), who are interested in the convergence of regulatory approaches for gene therapy products.

5. SPECIFIC ORGANISATION

5.1. Designation of a Chair Supporteur

The Chair Supporteur will provide secretariat support services, including setting up teleconferences, writing agendas and reports. The individual serving in this role will be decided by the GTWG members annually.

5.2. Organisation of meetings

Teleconferences will be held quarterly depending on the availability of participants. Face to face meetings will be held on an ad hoc basis. Meeting chairs and co-chairs will rotate with a schedule that is decided on by the GTWG members.

5.3. Contact with stakeholders

Contact with stakeholders includes participation in society meetings and publications in peer-reviewed journals.