

2022 Work Plan

Gene Therapy Working Group (GTWG)

Date: 12 October 2021

Chair: Judith Arcidiacono, FDA, United States

1. KEY MILESTONES AND DELIVERABLES

1.1. Current status of key milestones and deliverables

| Past completion date | Objective | Key Milestone or Deliverable |
|-----------------------------|------------------------------------|--|
| October 2019 | Regulatory Framework Project (RFP) | In collaboration with the gene therapy working group, frameworks from 11 regulatory agencies were collected. |
| October 2020 | Regulatory Framework Project (RFP) | Tabulated results |
| September 2021 | RFP | Published Regulatory Frameworks on IPRP website |
| October 2021 | Raw Materials Survey | Survey tabulated |

1.2. Future anticipated key milestones

| Expected future completion date | Objective | Key Milestone or Deliverable |
|--|---|--|
| May 2022 | Compilation of Guidance, Guideline and Reflection Paper | Work with the GTWG to publish tables on RPFs |
| May 2022 | Publish journal manuscript introducing availability of FRP outcomes | Publication of manuscript in journal |
| June 2022 | Raw Materials Project | Draft Reflection Paper |

| | | |
|---------------|---|---|
| November 2022 | Compile list of marketed products for participating regions | Publish list of marketed products on IPRP website |
|---------------|---|---|

2. TIMELINE FOR SPECIFIC TASKS

| Beginning date | End date | Task / Activity | Details |
|----------------|------------------|---|--|
| May 2021 | November 2022 | Raw Materials Reflection Paper | <ul style="list-style-type: none"> a. Create survey b. Draft Outline c. Establish drafting committee d. Prepare draft e. Review draft and collect comments f. Submit draft to MC g. Publish reflection paper to IPRP website |
| October 2021 | May 2022 | Publish list of Guidance/Guidelines/Reflection Papers (GGRP) | <ul style="list-style-type: none"> a. Create table for guidelines/guidance b. Request update to information originally collected in 2019 c. Submit to MC for review d. Publish list on IPRP website |
| October 2021 | February 2022 | Finalization of RFP tables | Regulators review RFP tables for accuracy and completeness |
| January 2022 | To be determined | Initiate Reflection Paper (RP) on Long-Term Follow-up (LTFU) for patients receiving gene therapy products | <ul style="list-style-type: none"> a. Comparison of EC/EMA, Europe and FDA, United States guidance on LTFU b. Identify drafting committee c. Draft outline for RP d. Draft RP e. Review RP by participants f. Submit RP to MC g. Publish RP on IPRP website |
| February 2022 | | WG teleconference #24 | |

| | | | |
|---------------|------------|--|---|
| March 2022 | April 2022 | Publish List of Marketed Products | <ul style="list-style-type: none"> a. Compile list b. Review list c. Submit list to MC d. Publish list on IPRP website |
| November 2022 | May 2023 | Update Frameworks, GGRP, and Marketed products | <ul style="list-style-type: none"> a. Request updates from participants b. Compile updates c. Submit updates to MC d. Publish updated information on IPRP website |