Annex 1: Description of the GoCART Coalition work packages

The GoCART Coalition organises its activities through its work packages. The work packages cover an area of interest of the Coalition and ensure the implementation of the mission and vision of the Coalition and the strategy on which the Executive Committee has agreed.

This document includes a description of the context and main aims of the GoCART Coalition work packages. It will be updated from time to time to reflect the ongoing development of the work groups and activities.

- **WP1: Data harmonisation**
    - Context: In Europe, clinical data from patients treated with gene and cellular therapies are reported to many registries, each built for a limited purpose, with different governance rules and specific software tools managing the data. This results in siloed data, inefficiencies and duplication of efforts.
    - Overall aim: Create a central EU data registry for the harmonised collection of clinical data on patients treated with cellular therapies to support collaborative studies and regulatory decision making.
    - Current work groups/activities:
        - Review and update the EBMT Cellular Therapy form
        - Align with CIBMTR to create a common data set and harmonised definitions for gene and cellular therapies, alignment with other registries could also be assessed;
        - Develop a harmonised gene therapy form

- **WP2: Standards of care**
    - Context: gene and cellular therapies are inherently complex products and treatment administration is restricted to qualified centres. With the rapid developments and pending product approvals, there is a need for developing treatment guidelines and harmonising centre qualification procedures across pharmaceutical companies, accreditation bodies and national requirements.
    - Overall aims: 1) to develop harmonised guidelines on patient and product management for health care professionals. 2) to reduce inspection burden and redundancies by developing and implementing consensus-driven requirements and qualification standards for clinical teams delivering gene and cellular therapies from cells and tissues of hematopoietic origin.
    - Current work groups/activities:
        - Harmonise centre qualification strategies by leveraging the JACIE qualification scheme
        - Harmonise labelling, documentation and packaging requirements
        - Develop guidelines on patient and product management, topics of interest include:
            - Guidance for hospital pharmacy on the implementation and operational management of CAR T-cell therapies
            - Recommendation guidelines on apheresis for ATMP manufacture

- **WP3: Education and Training**
    - Context: Gene and cellular therapies are complex products that require comprehensive and ongoing training of health care professionals as well as patients and caregivers. A plethora

of training courses are already offered by MAHs as well as health organisations, which can lead to considerable overlap.

- Overall aim: develop harmonised educational programmes for different groups of health care professionals and patients.
- Current work groups/activities:
  - Webinar series
    - GoCART Clinical Case Discussion series for physicians. In this monthly webinar two clinical cases are discussed with a panel of experts
    - GoCART Nurses Best Practice Sharing session. In this monthly webinar nurses explore different aspects of the patient journey with an nurse expert
  - Map CART manufacuter's educational training requirements for HCPs and develop a core "CART passport".
  - Develop CAR T-cell therapy Clinician Checklists to contribute to improvements in the quality and safety of medical care.

- **WP4: Scientific excellence**
  - Context: Scientific research on gene and cellular therapies increased substantially over the last years. With Real World Data becoming increasingly available, many scientific questions, from different perspectives, can be explored. Only by working together can we leverage enough data to conduct meaningful research. GoCART wants to maximise the use of data collected in the central Registry as well as data available to other stakeholders, and to facilitate further collaboration between stakeholders. While strongly protecting confidentiality, the guiding principle should be 'collect once, use often' to advance our knowledge in the field of gene and cellular therapies, support better decision making and drive efficiencies for all stakeholders.
  - Overall aim: stimulate scientific discussion across stakeholders, facilitate the set-up of joint research projects and avoid duplication of scientific efforts.
  - Current activities:
    - Set up yearly calls for research proposals to promote the conduct of collaborative studies

- **WP5: Policy and advocacy**
  - Context: Gene and cellular therapies are subject to EU and national regulations affecting their preparation, administration and patient access. These new therapies challenge the regulations which were designed for more traditional pharmaceutical products and health authorities are assessing how they will adapt.
  - Overall aim: represent and promote the interests of the GoCART coalition and its stakeholders in EU policy making by engaging with EU institutions and other relevant stakeholders.
  - Activities to be developed:
    - Develop a shared policy and advocacy strategy
    - Advocate key coalition positions with policy makers
    - Contribute to European policy making on topics concerning gene and cellular therapies from cells and tissues of hematopoietic origin
    - Specific topics of interest include but are not limited to: regulation on Substances of Human Origin and Hospital Exemption.