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Regulatory Harmonization Steering Committee Advanced Therapy Products Priority Work Area

Core Curriculum

This is a high level core curriculum that the roadmap will cover towards regulatory convergence for advanced therapy products in the APEC region. Specific topics and details will depend on the objectives of the Centre of Excellence (CoE) workshop and will be discussed by the programme and steering committees.

The trainees would include multiple stakeholders; regulators, academic professionals, companies and healthcare providers. Targeted training to specific stakeholders may be provided by CoE, as necessary.

Objectives:

1. Understand the science and regulations behind advanced therapy product development
2. Awareness of key considerations for Clinical, non-clinical, quality aspects (including GMP) of advanced therapy products
3. Understand regional and country-specific requirements for regulating advanced therapy products
4. Understand factors affecting the access of advanced therapies to patients
5. Understand the post-approval activities required to ensure and monitor the long-term safety and clinical effectiveness of advanced therapy products

Curriculum:

1. Science/Technology
    a. Overview of specific product categories (e.g., differentiating autologous vs allogeneic, vectors, gene editing techniques for somatic cells, risks/benefits)
2. Development of Advanced Therapies
    a. Product classification
    b. Target product profile
    c. Strategic implications
    d. Phases of development
    e. Product life cycle management
3. Manufacturing and Analytical Quality
    a. Process development and optimization (i.e., comparability)
    b. Quality attributes and analytical methods (i.e., method development and validation)
    c. Supply chain
    d. Risk management
    e. Platform technology
    f. National and international standards
        i. Reference materials
        ii. Documentary Standards
    g. GMP specifics to advanced therapy products
    h. Site change management
    i. Cleaning methods/disposable equipment/contamination

    j. Inspections and audits
    k. Post-approval quality variations and product life cycle management
4. Non-clinical
    a. Pharmacology, pharmacokinetics (distribution, persistence), pharmacodynamics (specificity, activity)
    b. Toxicology (animal model, off target, immunogenicity, tumorigenicity)
    c. *In silico* models
5. Clinical
    a. Trials for advanced therapies
        i. Early phase
        ii. Nontraditional clinical development (i.e., accelerated, RWE, etc.)
        iii. Patient selection and screening
        iv. Patient populations/candidates for treatment (i.e., what risk benefit assessment in gene therapy)
    b. Statistical implications of protocol design, standard of care, epidemiology, among others
    c. Risk benefit analysis (Sponsor, Regulator, Patient)
        i. Route of administration implications (drug, device)
        ii. Dose
        iii. Acceptability of global vs regional data
    d. Risk mitigations (these are strategies that are part of the clinical development plan)
    e. Post approval risk mitigation strategy – include long term follow up and life cycle management
    f. Expanded indications
    g. Familiarity/training of facility/hospital staff/physicians/investigators
6. Product life cycle management
    a. Optimising post-approval controls
    b. Stakeholder training, engagement and collaboration
7. Regulatory Policy and Framework, and Advanced Therapy specific special access/accelerated schemes
    a. Overview of policy and framework (APEC and other economies such as EU)
    b. Accelerated development and schemes for product developers (e.g., Fast Track, Breakthrough Designation, Regenerative Medicine Advanced Therapy Designation, Orphan Designation, Sakegaki)
    c. Platforms and ongoing collaborative projects