Session IV

The Future

15:25-16:30

CELL / GENE THERAPY Action Plan

Quality, safety testing, standardisation, methods, monographs, guidelines…

Reference materials,

Definition, nomenclature …

CELL / GENE THERAPY Action Plan

A global package complementing each others

Licence, inspection, pharmacopoeia, control labs (OMCL)

Still evolving field

Timely: first products are emerging

But basically 2 types of operators:

-industry different scales

- academics/ hospitals

Safety highest priority

- What else than the existing PhEur test methods
  - Sterility, mycoplasma, LAL , adventitious agents….
  - NAT existing requirements
- Are they adapted for the purpose?
  - Need for customisation
- Q- PCR ?
  - Need for specific SOPs/ guidance

Safety tests

- Exhaustive list of virus ?
- Validation models?
- Role of production methods
  - Serum free medium culture

Reference Materials

- Adenovirus
  - Need for common reference methods SOPs
- What else
  - Lentivirus ...
  - AAV
  - Non viral vectors
  - Common reference methods for use...
- Other ref material
- Regional working standards

Methods

- Guidance vs detailed SOPs
- specificity
- Validity criteria/suitability
- Confidence limits/precision
- Reagents, patent issues ....

Potency assays

- Room for development (ie chondrocytes..)
- Need for development of study models for efficacy/safety
- Other key issues
  - Customised QC vs IPCs
  - Role of outsourcing

Potency assays

- I e Adenovirus
  - Standard SOPs
    - Strength particle numbers
    - Infectivity plaque assay
    - Specific potency assay
- Retrovirus
- AAV
- Pox virus
- Plasmid

Quality

- Upstream vs end product testing
- Place of QA,
- Inspection GMP
  - clear rules
  - no double standards.
  industry vs academics/ hospitals
- Role of independent testing by OMCLs
- Transfer of technology
  - Training/qualification

Monographs

- Define scope
- General guidance vs detailed methodology
- Product/group of product(type) specific
- Flexible standardisation approach?
- Horizontal guidance vs vertical approach/guidance?
- Source material:reagents
  - FCS requirements

Definition/nomenclature

- Implication on the rules and regulations applicable to the products
  - Controls
  - License
  - Inspections
  - etc
- GT/ CT different approaches?

- On going dialogue, advice and open consultation
- Close collaboration with
  - BWP, GTEG ...
- And international partners