

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 and Gene Therapy Conference
EDQM 25 Feb. 2003

1

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

Cell and Gene Therapy Conference
EDQM 25 Feb. 2003

2

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

Cell and Gene Therapy Conference
EDQM 25 Feb. 2003

3

Safety tests

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

Cell and Gene Therapy Conference
EDQM 25 Feb. 2003

4

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

Cell and Gene Therapy Conference
EDQM 25 Feb. 2003

5

Methods

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

Cell and Gene Therapy Conference
EDQM 25 Feb. 2003

6

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

Cell and Gene Therapy Conference
EDQM 25 Feb. 2003

7

Potency assays

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

Cell and Gene Therapy Conference
EDQM 25 Feb. 2003

8

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

Cell and Gene Therapy Conference
EDQM 25 Feb. 2003

9

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**

Cell and Gene Therapy Conference
EDQM 25 Feb. 2003

10

Definition/nomenclature

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

Cell and Gene Therapy Conference
EDQM 25 Feb. 2003

11

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

Cell and Gene Therapy Conference
EDQM 25 Feb. 2003

12
