Cell Therapy Development
Past, present and future

20th Annual ISCT Meeting

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cat.catapult.org.uk
Cell Therapy Past: Pioneering

http://www.fda.gov/BiologicsBloodVaccines/CellularGeneTherapyProducts/ApprovedProducts/
“Backward looking statements”

Great pioneers.....use the state of the art at the time

Mallory and Irvine 1924

Tenzing and Hillary 1953
What did we learn?

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<tr>
<th>Cell therapy products do work in humans</th>
<th>There is a way through the regulatory maze</th>
<th>You can get paid</th>
<th>You can achieve a high price</th>
<th>There can be demand from clinicians</th>
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<td>The market is value sensitive</td>
<td>Clinicians need more data</td>
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<td>Product still has to be sold</td>
<td>No special treatment</td>
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<td>Cost of manufacture and supply is locked in</td>
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Present: applying the learning...
Early development of the value proposition

Business models
Healthcare economics
Reimbursement strategies
Intellectual property strategy
Collaborations

Immunomodulation
Regulatory, Clinical trial design, business models

Planning of reimbursement strategy for Europe (Big 5)
Preparing the route to reimbursement

*Top-level roadmap to market access for licensed: ATMPs, non-medicinal therapies, medical devices (England)*

- **Horizon Scanning Centre**
- **NICE / DoH / NHS**
  - Topic Selection for NICE Assessment
- **NICE Assessment**
- **NHS Commissioning**
  - mainly by “Specialised Services”
- **Rare Diseases Advisory Group**
- **Clinical Reference Groups**
- **Hospitals**

**Cell Therapy manufacturers should engage successfully with all above stakeholders in order to maximise therapy uptake**

* Equivalent to NICE assessments in Scotland are undertaken by the Scottish Medicines Consortium (SMC) and in Wales by the All Welsh Medicines Strategy Group (AWMSG); The Rare Diseases Advisory Group advises NHS England, NHS Scotland, NHS Wales, NHS Northern Ireland

Abbreviations: CT (Cell Therapy), NICE (National Institute for Clinical and Care Excellence)
Early process and assay development

Assay Development
Process Development
Facility Design
Storage and shelf life
Transport and logistics

Phase 2 clinical trial
ready process
Scale up, Assays,
Freezing and
distribution of cells

Large scale
pluripotent cell
process development
Cost effectively delivering the evidence

- Quality
- Safety
- Efficacy
- Comparative Clinical & Cost-Effectiveness; Budget Impact

REGULATORY APPROVAL

MARKET ACCESS

Clinical trial design and management
Regulatory interaction
Collaboration and Platforms

Pre-competitive innovation and co-operation:
- GMP raw materials
- Logistics
- Shelf Life and Transport Standards, etc

Sharing of expertise
Collaboration on in-house projects

GMP iPSC cell bank
Future: Delivering value to the patient and provider
Where are we now?

Pioneers
Showing the way

2nd Generation
Adopting the best available
And making it work

3rd Generation
Step change to newly developed technologies

We are somewhere here

4th Generation
Major technological leaps
Future of cell therapy business

Sales led

Budgeted

Value based

Competitive

Rapid deployment
Future of manufacturing

- Closed Systems and Bio-reactors
- Modular automated design
- Central data driven control
- Common platforms and standards
- Cost
Large Scale Manufacturing Centre, a manufacturing hotel to support industry growth

Design Approach

- 5000m²
- Flexible replicable manufacturing pods
- Autologous / Allogeneic
- Cell based gene therapies

Location

- Ability to supply time critical products to EU markets
- Resilience of supply
- Supply chain
- Workforce
- Client access and expansion
- Catapult support and operability

£55m investment over 5 years

Capital and initial operating costs

Supporting TSB programmes
Future of Products

High unmet need

High efficacy

Patient benefit

Near patient devices

Shorter development cycles
A future in collaboration
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