The role of SMEs in therapeutic innovation

EFPIA’s vision

Ruth McKernan
CSO Pfizer Neusentis
Vice Chair of Research Directors Group
SMEs are more important in Pharma today

- The changed ecosystem
- The relationship between SMEs and Pharma
- SMEs in public/private partnerships
  - EBiSC as an example
The Pharma Ecosystem: The old model

Large Vertically Integrated Sites

Internal
- Regulatory
- Pharmacovigilance
- QA/QC
- Development Ops
- Clinical Dev
- Experimental Medicine
- Supply Chain
- Final Manufacturing
- Bulk Synthesis
- Drug Safety
- Tableting
- Metabolism
- Screening
- Biologic Target Validation
- Medicinal Chemistry
- Exploratory Research

External
- Development Functions
  - Enabling Lines/Functions
  - Research Functions
- Research Grants
- In-License & M&A
The Pharma Ecosystem: The new model

Dynamic ecosystem today

**Internal**

- Global support network
  - Regulatory and Supply
  - Platform functions

- Empowered Research Unit
  - Biologic Target
  - Validation/exploratory research
  - Identify small molecule and biologic NMEs
  - Proof of concept P2

**External**

<table>
<thead>
<tr>
<th>Service providers</th>
</tr>
</thead>
<tbody>
<tr>
<td>Clinical trials – Parexel, Quintiles</td>
</tr>
<tr>
<td>QA/QC – Parexel, Quintiles</td>
</tr>
<tr>
<td>Dev Ops – Parexel, Quintiles</td>
</tr>
<tr>
<td>Experimental medicine – Tessella, P1vital, Imanova, Cantab</td>
</tr>
<tr>
<td>Supply Chain – Fisher</td>
</tr>
<tr>
<td>Final Manufacturing – Lonza, Aptuit</td>
</tr>
<tr>
<td>Bulk Synthesis – Peakdale, Lonza</td>
</tr>
<tr>
<td>Drug Safety – Huntingdon Life sciences, Covance, Chantest</td>
</tr>
<tr>
<td>Tabletting – AMRI, aptuit</td>
</tr>
<tr>
<td>Metabolism – Quotient, York, Covance</td>
</tr>
<tr>
<td>Screening – Wuxi, Galapagos, Evotec, Essen</td>
</tr>
<tr>
<td>Med Chem – Wuxi, Peakdale</td>
</tr>
</tbody>
</table>

**Development**

**Spin-outs**

SME
License/
Partner/
Acquire
SMEs are more important to Pharma

- Pharma have VC Investment groups
- Research – decision makers
- Cycling of assets
- Number of deals increasing
- More ‘virtual’ Research Units - External Innovation

Empowered Research Unit

- Biologic Target Validation/exploratory research
- Identify small molecule and biologic NMEs
- Proof of concept P2

Spin-outs

SME
License/
Partner/
Acquire
SMEs are more important to Pharma

Taking it to the next level

Empowered Research Unit

Biologic Target
Validation/exploratory research

Identify small molecule and biologic NMEs

Proof of concept P2

SME
License/
Partner/
Acquire

Spin-outs

Non
EBiSC – European BANK of induced pluripotent Stem Cells

- Generates & distributes standardized, QC, iPS cell lines
  - International distribution capability
  - Catalogue of 3,000-10,000 cell lines
  - Can generate bespoke lines, provide services
- Launched 1st Jan 2014, IMI/Pharma funded.
- 3 year duration → self-sustaining
- 26 partners: Co-ordinated by Pfizer and Roslin Cells Ltd

6 SMEs

Roslin Cells (Consortium Manager),
ARTTIC, (project administration),
Douglas Connect (info mgmt), European Screening Port (automation)
Definigen (functional assays),
Bioneer (iPS cell line generation)

8 universities

Edinburgh,
Cologne, Bonn,
Newcastle,
Charité (Berlin),
Hannover,
University College London,
Hubrecht Institute.
Plus other banks and university entities

6 pharma

Pfizer
Novo Nordisk
AZ, UCB
Janssen
Lundbeck
Contributions and Impact

Pharma
- Discovery
  - Relevant human tools
target validation and improved screening
- Cpd selection
  - Validation of genetic variation for precision medicine
- Phase I
- Phase II
- Phase III
- Post market
  - Addressing safety issues post rare event reporting

SME
- Assimilate lines from IMI initiatives academics and other sources
- QC, standardise, expand and ship

Academia
- Clinicians
  - Provide access to invaluable cohorts of patients
  - Rare & genetic diseases
- Stem cell Scientist
  - Understand Biology of iPS cell differentiation
  - Understand biology of disease
- Bio-engineer
  - Develop automation methods for iPS cell culture and biobanking

Stem Cell Bank

Share collective knowledge on cells used
Become self-sustaining
Enable new businesses
Why SMEs?

- Project management /commitment
- Used to dealing with Pharma / milestones
- Operational plans fit for changing science
- Realistic BD expectations
- Investable spin-offs better led by SMEs
- Longer lasting benefit for EU