Regenerative Medicine and Advanced Therapies

Open Info Day
8 July 2016

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DG Research and Innovation
Regenerative medicine and advanced therapies

Programme for this afternoon:

• Introduction and forthcoming calls
• Experiences, pitfalls to avoid and wise words (2 speakers)
• Discussion
ATMP development: targeting different points on the innovation chain

Risk level

- **Discovery, PoC**
- **Preclinical**
- **Upscaling Manufacturing**
- **Clinical Phases 1-3**
- **Uptake Delivery**

**Resources required**

PUSH → PULL

**Tools and technology (incl. SME-Instrument)**

- IMI (iPSC)
- ERC
- Gene transfer
- Stem cell differentiation and proliferation
- Clinical research on regenerative medicine
Regenerative medicine and advanced therapies

Two topics open in forthcoming calls for proposals...
1. Clinical research on regenerative medicine

Main points

- **SC1-PM11-2016-2017**
- **Clinical research – any stage; proof of regulatory engagement/autorisation**
- **Collaborative research – min 3 EU/assoc countries**
- **Indicative budget: €30 million (2017)**
- **~ €4-6 million per project – depending on work**
- **Deadline: 11 April 2017**
Clinical research on regenerative medicine – results so far

<table>
<thead>
<tr>
<th>Year</th>
<th>Proposals Received</th>
<th>Proposals Eligible</th>
<th>Proposals Supported</th>
<th>Success rate %</th>
<th>Budget € million</th>
</tr>
</thead>
<tbody>
<tr>
<td>2014</td>
<td>30</td>
<td>30</td>
<td>6</td>
<td>20</td>
<td>35.2</td>
</tr>
<tr>
<td>2015</td>
<td>48</td>
<td>46</td>
<td>7</td>
<td>15</td>
<td>39.1</td>
</tr>
<tr>
<td>2016</td>
<td>32</td>
<td>30</td>
<td>?</td>
<td>?</td>
<td>30.0</td>
</tr>
</tbody>
</table>
Clinical research on regenerative medicine (2014 – 2015)

<table>
<thead>
<tr>
<th>Project Acronym</th>
<th>Area covered/Condition</th>
<th>Approach/Technology</th>
</tr>
</thead>
<tbody>
<tr>
<td>TREGeneration</td>
<td>GvHD (chronic)</td>
<td>Allogeneic Treg lymphocytes</td>
</tr>
<tr>
<td>RETHRIM</td>
<td>GvHD (acute)</td>
<td>Allogeneic MSC (bone marrow-derived)</td>
</tr>
<tr>
<td>SCIENCE</td>
<td>Ischemic heart disease</td>
<td>Allogeneic adipose-derived MSC</td>
</tr>
<tr>
<td>ADIPOA2</td>
<td>Osteoarthritis</td>
<td>Autologous adipose-derived MSC</td>
</tr>
<tr>
<td>S0806</td>
<td>Spinal cord injury</td>
<td>Recombinant growth factor + device</td>
</tr>
<tr>
<td>ARISE</td>
<td>Heart valve</td>
<td>Allogeneic decellularized aortic valve</td>
</tr>
<tr>
<td>NISCI</td>
<td>Spinal cord injury</td>
<td>Antibodies against Nogo-A</td>
</tr>
<tr>
<td>BOOSTB4</td>
<td>Osteogenesis imperfecta</td>
<td>Allogeneic MSC (fetal-derived)</td>
</tr>
<tr>
<td>RESSTORE</td>
<td>Stroke</td>
<td>Allogeneic adipose-derived MSC</td>
</tr>
<tr>
<td>SEPCELL</td>
<td>Sepsis</td>
<td>Allogeneic adipose-derived MSC</td>
</tr>
<tr>
<td>TETRA</td>
<td>Trachea (severe airway disease)</td>
<td>Autologous MSC + decellularized trachea scaffold</td>
</tr>
<tr>
<td>BetaCellTherapy</td>
<td>Type-1 diabetes</td>
<td>Encapsulated progenitor cells (hESC-derived)</td>
</tr>
<tr>
<td>BIO-CHIP</td>
<td>Knee cartilage injury</td>
<td>Autologous nasal chondrocytes</td>
</tr>
</tbody>
</table>

"To allow an adequate coverage in the field of regenerative medicine, proposals should take into account the projects previously funded under this topic in Horizon 2020"
2. Cell technologies in medical applications

Main points

- **SMEInst-05-2016-2017** "Supporting innovative SMEs in the healthcare biotechnology sector"
- SME Instrument – EU/Assoc SME(s) only (can subcontract)
- Phase 1 – Feasibility study (€50,000 lump sum)
- Phase 2 – Research/Innovation (~€1-5 million depending on work – 100% reimbursement)
- Indicative budget:
  - 2016: €35 million
  - 2017: €80 million (includes biomarkers and diagnostic medical devices, Phase 2, first cut-off)
- Multiple deadlines
3. Other topics that might support regenerative medicine and advanced therapies

SC1 Two-stage call – stage 1 closing 4 October 2016

• SC1-PM-02-2017 "New concepts in patient stratification"
• SC1-PM-08-2017 "New therapies for rare diseases" [read texts carefully]

Others

ERC, FET, Marie Skłodowska-Curie actions...
PARTICIPANT PORTAL

Unique entry point

http://ec.europa.eu/research/participants/portal

Information available on the participant portal includes all you have to know about our programmes and about submitting a proposal.

New evaluators welcome.
Thank you for your attention

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