HORIZON 2020 and EU support to cell therapy research

ISCT, Paris, 25 April 2014

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European Commission, Brussels
What is Horizon 2020?

- Responding to the economic crisis to invest in future jobs and growth
- Addressing people’s concerns about their livelihoods, safety and environment
- Strengthening the EU’s global position in research, innovation and technology

The EU’s 2014-20 programme for research & innovation

77 billion euros over 7 years
Three priorities

- Excellent science
- Industrial leadership
- Societal challenges
<table>
<thead>
<tr>
<th><strong>European Research Council (ERC)</strong></th>
<th>Frontier research by the best individual teams</th>
<th>13 095</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Future and Emerging Technologies</strong></td>
<td>Collaborative research to open new fields of innovation</td>
<td>2 696</td>
</tr>
<tr>
<td><strong>Marie Skłodowska-Curie actions (MSCA)</strong></td>
<td>Opportunities for training and career development</td>
<td>6 162</td>
</tr>
<tr>
<td><strong>Research infrastructures (including e-infrastructure)</strong></td>
<td>Ensuring access to world-class facilities</td>
<td>2 488</td>
</tr>
<tr>
<td><strong>Leadership in enabling and industrial technologies (LEITs)</strong>&lt;br&gt;(ICT, nanotechnologies, materials, biotechnology, manufacturing, space)</td>
<td>13 557</td>
<td></td>
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<tr>
<td><strong>Access to risk finance</strong>&lt;br&gt;Leveraging private finance and venture capital for research and innovation</td>
<td>2 842</td>
<td></td>
</tr>
<tr>
<td><strong>Innovation in SMEs</strong>&lt;br&gt;Fostering all forms of innovation in all types of SMEs</td>
<td>616 + complemented by expected 20% of budget of societal challenges + LEITs and 'Access to risk finance' with strong SME focus</td>
<td></td>
</tr>
</tbody>
</table>
# Societal challenges

<table>
<thead>
<tr>
<th>Challenge</th>
<th>Funding (2014-2020 in M€)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Health, demographic change and wellbeing</td>
<td>7 472</td>
</tr>
<tr>
<td>Food security, sustainable agriculture and forestry, marine and maritime and inland water research and the Bioeconomy</td>
<td>3 851</td>
</tr>
<tr>
<td>Secure, clean and efficient energy</td>
<td>5 931</td>
</tr>
<tr>
<td>Smart, green and integrated transport</td>
<td>6 339</td>
</tr>
<tr>
<td>Climate action, environment, resource efficiency and raw materials</td>
<td>3 081</td>
</tr>
<tr>
<td>Inclusive, innovative and reflective societies</td>
<td>1 310</td>
</tr>
<tr>
<td>Secure societies</td>
<td>1 695</td>
</tr>
<tr>
<td>Science with and for society</td>
<td>462</td>
</tr>
<tr>
<td>Spreading excellence and widening participation</td>
<td>816</td>
</tr>
</tbody>
</table>

HORIZON 2020
What's new?

• **Coupling research to innovation** – from research to retail, all forms of innovation

• **Focus on societal challenges** facing EU society, e.g. health, clean energy and transport

• **Fewer and broader topic descriptions**

• **20% SME participation target and special initiatives**

• **Simplified access and procedures:**
  - Participant portal for access
  - One project – one funding rate (research 100%+overheads 25%; innovation 70%)
  - Simpler rules, eg no time sheet for full-time personnel, fewer audit certificates
  - 8 month time to grant
Health, demographic change and wellbeing challenge

Builds on FP7 'Health' – the largest multi-national fund...

- ...for collaborative research (min 3 EU countries), global consortia, and public-private co-operation
- ...to fund excellent applied health and ageing research and innovation
- ...to bring together leading players from Europe and across the globe (incl US)
- ...to tackle key European and global health challenges

HORIZON 2020
Possible areas for cell therapy research in Horizon 2020 (1)

Societal Challenge: "Health, demographic change and wellbeing"

- PHC 14-2015 New therapies for rare diseases
  (Stage 1 deadline 14/10/2014)

- PHC 15-2015 Clinical research on regenerative medicine
  (Single stage deadline: 21/4/2015; second date tbc)

- PHC 16-2015 Tools and technologies for advanced therapies
  (Stage 1 deadline 14/10/2014)

Check other options in work programme!
Possible areas for cell therapy research in Horizon 2020 (2)

- Leadership in enabling and industrial technologies:
  - NMP 10-2014 Biomaterials for treatment of diabetes (Stage 1 deadline: 6/5/2014)
- ERC grants in basic science
- Marie Curie training actions
- Fast Track to Innovation, min 60% industry (2015)

Check other options in work programmes!
Innovative Medicines Initiative: Europe’s fast track to better medicines

The biggest public/private partnership in Life Science aiming to:

- Make drug R&D processes in Europe more innovative and efficient
- Enhance Europe’s competitiveness
- Address key societal challenges

Features:

- 1:1 funding, joint decision making
- All EU funds go to SMEs, academia, patient organisations and regulatory agencies
- Large pharmaceutical industry, represented by EFPIA, contributes in-kind

HORIZON 2020

European Commission
EU support to cell therapy research in FP7 (1)

Organ regeneration
- Nerve, eye, skin
- Wound, muscle
- Heart, valve, artery
- Bone and cartilage
- Liver, kidney, pancreas

Cancer immunotherapy
Transplant tolerance
Immunomodulation
Autoimmune diseases

Technologies
- Media and biomaterials
- Cell characterisation, identification and selection
- Gene transfer
- Standardisation
- Expansion
- Bioreactors and miniaturisation
- Imaging

Cell types
- All, incl haemato, adult, hES, iPS
EU support to cell therapy research in FP7 (2)

**Targets:**
- New therapies
- Supporting technology
- Science underpinning regulation

**Trends:**
- Broader terms of reference and 2-stage application process to encourage innovation and SMEs
- More deliverables-orientated, notably clinical trials and technology
Conclusions:
• Active support from EU – c. €350 million contribution over 7 yrs
• Research is multi-disciplinary; collaboration needed
• Science moving rapidly
• Therapies more complex than initially imagined
• Increasing number of examples coming forward
• Moving to the clinic is a real bottleneck, notably:
  • Diversity of business/therapeutic models
  • Lack of experience of procedures, regulatory pathway, etc
  • Resources - "Valley of death"

Future priorities?
### Examples of EU translational cell therapy projects

<table>
<thead>
<tr>
<th>Project</th>
<th>Description</th>
<th>Website</th>
</tr>
</thead>
<tbody>
<tr>
<td>Optistem</td>
<td>Optimisation of stem cell therapy for clinical trials of degenerative skin and muscle diseases</td>
<td><a href="http://www.optistem.org">www.optistem.org</a></td>
</tr>
<tr>
<td>The One Study</td>
<td>A unified approach to evaluating cellular immunotherapy in solid organ transplantation</td>
<td><a href="http://www.onestudy.org/index.html">www.onestudy.org/index.html</a></td>
</tr>
<tr>
<td>Espoir</td>
<td>European clinical study for the application of regenerative heart valves</td>
<td><a href="http://www.espoir-clinicaltrial.eu">www.espoir-clinicaltrial.eu</a></td>
</tr>
<tr>
<td>RegenAR</td>
<td>Allogeneic eASCs Phase 1B/2A clinical trial for treating rheumatoid arthritis</td>
<td><a href="http://www.regenar.eu">www.regenar.eu</a></td>
</tr>
<tr>
<td>Cell-PID</td>
<td>Advanced cell-based therapies for the treatment of Primary ImmunoDeficiency</td>
<td><a href="http://cell-pid.eu">cell-pid.eu</a></td>
</tr>
<tr>
<td>Attack</td>
<td>Adoptive engineered T cell Trials to Achieve Cancer Killing</td>
<td><a href="http://www.attack-cancer.eu">www.attack-cancer.eu</a></td>
</tr>
</tbody>
</table>

And [www.eurostemcell.org](http://www.eurostemcell.org)
## Examples of EU cell therapy technology projects

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<td>PurStem</td>
<td>Utilisation of the mesenchymal stem cell receptome for rational development of uniform, serum-free culture conditions and tools for cell characterisation</td>
<td><a href="http://www.purstem.eu">www.purstem.eu</a></td>
</tr>
<tr>
<td>BEST-stem cells</td>
<td>Biochemically equivalent substitutive technology for stem cells</td>
<td><a href="http://www.beststemcells.ed.ac.uk">www.beststemcells.ed.ac.uk</a></td>
</tr>
<tr>
<td>BIO-COMET</td>
<td>Bioreactor-based, clinically orientated manufacturing of engineered tissue</td>
<td><a href="http://www.biocomet.eu">www.biocomet.eu</a></td>
</tr>
<tr>
<td>InnovaLiv</td>
<td>Innovative strategies to generate human hepatocytes for metabolic liver diseases: tools for personalised cell therapy</td>
<td><a href="http://www.innovaliv.eu">www.innovaliv.eu</a></td>
</tr>
<tr>
<td>TissueGEN</td>
<td>Production of a 3D human tissue disease platform to enable regenerative medicine therapy development</td>
<td><a href="http://www.tissuegen.org">www.tissuegen.org</a></td>
</tr>
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</table>
Horizon 2020 Participant Portal

Your one-stop-shop for EU funding

http://ec.europa.eu/research/participants/portal
Thank you for your attention

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