The Cell Therapy Catapult UK Clinical Trials Database as of April 2013

The UK Clinical Trials Database covers cell therapy clinical trial activity that the Cell Therapy Catapult believes to be ongoing in the UK as of April 2013. It supercedes the database of November 2012.

It has been compiled and verified by the Cell Therapy Catapult team, and includes:

- academic and commercial trials
- ongoing trials in the UK, regardless of the nationality of the sponsor
- all trials involving cells as therapeutic agents*

* excluding trials of established haematopoietic stem cell transplantation regimens and gene therapy vectors for genetic modification in vivo, but now including ex vivo genetic modification of cells which are then administered to patients

Significant changes versus the previous version of the database are discussed in more detail below.

The database will be updated regularly, depending on feedback and new information. Its accuracy and comprehensiveness relies as much on the input of the cell therapy community as that of the Cell Therapy Catapult. We welcome your updates, additions and corrections, which you can send to us here.

A Cell Therapy Catapult UK Preclinical Research Database has also been compiled.

The purpose of the Cell Therapy Catapult UK Clinical Trials Database

The Cell Therapy Catapult has been established to develop and grow a thriving cell therapy industry in the UK, and will be progressing a portfolio of projects. The UK Clinical Trials Database forms an important part of the mechanism by which the Cell Therapy Catapult identifies potential programmes for investment or partnership, by indicating which areas and approaches are advancing through the clinic.

In addition, since the focus of the Cell Therapy Catapult is to promote translational activities towards commercialisation, clinical trial activity is a highly relevant measure of progress. By providing a snapshot of the UK landscape at a given time, the database enables us to measure our progress. Finally, the Cell Therapy Catapult hopes that the database will be of use to academics, researchers and commercial organisations operating in the cell therapy space by allowing them to understand the extent of cell therapy activity in the UK.

The interesting trends the database highlights are discussed below.
UK Cell Therapy Clinical Trials Database - commentary on key findings

There are 34 verified cell therapy clinical trials ongoing in the UK, according to the findings of the Cell Therapy Catapult database.

Majority - 76% - of UK clinical trials are sponsored by a research institution

Of the six different commercial sponsors, four are UK companies

Approximately 2:1 split between autologous and allogeneic therapies

Diverse cell types in trials - bone marrow cells predominate\(^1\), followed by T cells

\(^1\) Bone marrow mononuclear cells, CD34+ bone marrow stem cells, CD133+ bone marrow stem cells and mesenchymal stem/stromal cells are all bone-marrow derived

\(^2\) 3 of the 7 CD34+ stem cells shown as bone marrow derived can also be blood or cord derived (see database)
Traditional dominance of transplant and oncology indications is being challenged

4 of the 9 oncology clinical trials have a dual classification of Oncology/Blood in the clinical trials database. For the purpose of this pie chart they have been classified only as Oncology. See database for details.

In addition, there is significant activity in cardiovascular and neurological indications.

The majority of cell therapies are in Phase I/II or II trials

The trials in the UK Cell Therapy Database are mainly early stage, as follows:
- Phase I - 2 trials
- Phase I/II - 17 trials
- Phase II - 12 trials
- Phase II/III - 1 trial
- Phase III - 2 trials

How does the UK cell therapy sector compare with those elsewhere?

In a recent analysis of advanced therapy medicinal product (ATMP) development in Europe*, the findings were very consistent with those of the Cell Therapy Catapult database. The European study identified 318 clinical trials of 250 ATMPs during the period between 2004 and 2010, of which around 75% were cell-based. It also found:

- Clinical trial sponsors were academic groups, charities or small companies
- Academic groups and charitable organisations sponsored over 50% of trials
- Early trials dominate, as with the UK translational/commercialisation gap
- Oncology trials dominated, followed by cardiovascular and haematology
- Cell type breakdown reflected that in the Cell Therapy Catapult database
- UK, Germany and Spain were the leading countries developing ATMPs

* Maciulaitis et al, Molecular Therapy Volume 20, No 3, March 2012, pp 479-382
Other studies draw similar conclusions (Bonfiglio, 10th Stem Cell Summit April 2013).

**UK Cell Therapy Clinical Trials Database - changes since previous database**

<table>
<thead>
<tr>
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<th>November 2012</th>
<th>April 2013</th>
<th>Change</th>
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<tr>
<td>Trials</td>
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<td>34</td>
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<tr>
<td>Commercial sponsors</td>
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<td>6</td>
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</tr>
<tr>
<td>UK commercial sponsors</td>
<td>3</td>
<td>4</td>
<td>1</td>
</tr>
<tr>
<td>Autologous/allogeneic split</td>
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<td>2:1</td>
<td></td>
</tr>
<tr>
<td>Early-stage trials (Phases I, II/II or II)</td>
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<td>31</td>
<td>12</td>
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<tr>
<td>Later-stage trials (Phase II/III onwards)</td>
<td>2</td>
<td>3</td>
<td>1</td>
</tr>
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</table>

The 10 trial changes in the Clinical Trials Database can be classified as follows:

* Four trials involving ex vivo genetically modified cells
* Seven other trials newly notified to us
* One previously unverified trial has been removed

The additional trials have led to a significant increase in the number of Phase I/II and Phase II trials, and account for the dominance of autologous therapies in the database (versus an approximately equal split previously).

New cell types entering the database include pancreatic islets and antigen-presenting cells.

**UK Cell Therapy Clinical Trials Database - conclusions**

There is a high quality but nascent cell therapy industry in the UK, with significant potential for the Cell Therapy Catapult to increase the number of UK companies translating research towards commercialisation through clinical trial activity. In addition, the Cell Therapy Catapult has identified a rich pipeline of therapies which are likely to enter clinical development over the next two years, as detailed in the Preclinical Research Database. More later-stage trials and products progressing towards approval will be a further indication of the Cell Therapy Catapult’s success, leading to the generation of better health and wealth in the UK.

**May 2013**