

The Cell Therapy Catapult UK Clinical Trials Database as of November 2012

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 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*

It will be updated annually, or more often if required. 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 is expected to be established in due course.

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 as the Cell Therapy Catapult starts operations, the November 2012 database is a benchmark against which progress can be measured. 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.

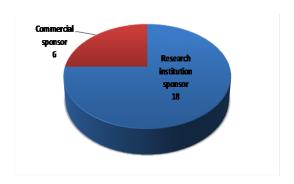
^{*} excluding trials of established haematopoietic stem cell transplantation regimens and cells acting as a delivery system for a gene therapy vector

UK Cell Therapy Clinical Trials Database - commentary on key findings

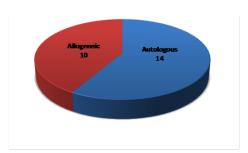
There are 21 verified cell therapy clinical trials ongoing in the UK, according to the findings of the Cell Therapy Catapult database, with verification of three more pending. All 24 are included in the analyses below, except where indicated.

The majority (75%) of UK clinical trials are sponsored by a research institution

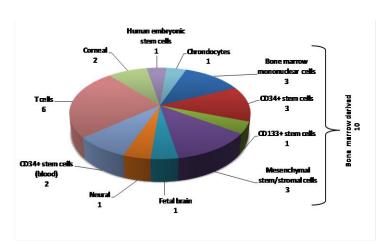
Of the four commercial sponsors, three are UK companies



Approximately equal split between autologous and allogeneic therapies

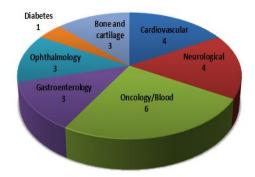


Diverse cell types in trials - bone marrow cells predominate*, followed by T cells



^{*} Bone marrow mononuclear cells, CD34+ bone marrow stem cells, CD133+ bone marrow stem cells and mesenchymal stem/stromal cells are all bone-marrow derived

Traditional dominance of transplant and oncology indications is being challenged



In addition, there is significant activity in cardiovascular and neurological indications, followed by gastroenterology and ophthalmology.

The majority of cell therapies are in Phase I/II or II trials (excluding unverified trials)

The trials in the UK Cell Therapy Database are mainly early stage, as follows:

- Phase I 3 trials
- Phase I/II 11 trials
- Phase II 5 trials
- Phase II/III 1 trial
- Phase III 1 trial

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-stage trials featured heavily, in line 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

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. 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.

December 2012