Policy statement

Stem cell research

Introduction

For some people who survive a heart attack, the damage to their heart muscle can lead to debilitating heart failure, for which there is no cure. Nearly 810,000 people are living with heart failure in the UK.[1] The body is not able to repair this damage. But in the future we may be able to use stem cells to help hearts repair themselves, bringing long-term benefits to people who have chronic heart failure.

Policy statement

Stem cell research provides real potential to combat heart and blood vessel diseases. We are committed to investing in pioneering research in the fight against cardiovascular disease, and currently fund research involving embryonic and adult stem cells.

Science cannot predict at this stage which types of stem cell will prove to be of most benefit, and so continued research on all types of stem cells remains necessary to improve our knowledge.

The BHF supports the current regulatory environment for stem cell research in the UK, and believes that this must be protected to make sure further breakthroughs in this field will continue.

To help ensure we see the full benefits of stem cell research for people living with heart failure, we are calling on all UK governments to develop the UK's stem cell research base by:

- maintaining at current levels funding that helps to reinforce the UK's international competitiveness in stem cell research
- reducing the regulatory overlap for those researchers seeking approval for research involving embryonic stem cells

The BHF does not endorse the use of unregulated direct to consumer stem cell therapies. These therapies are unproven and potentially dangerous. Greater regulation is needed internationally in this area to protect the public from misleading medical advice.

[1] General Practice Research Database (2010) Personal Communication. Applied to Office of National Statistics population estimates for 2008.

Background

Stem cells are immature cells in the body that exist to replace our old cells when we need new ones. They are pluripotent, meaning they are capable of developing into a variety of different types of specialised cell. Their versatility presents an exciting avenue for research whereby damaged tissue could potentially be replaced with healthy, functioning tissue – a process known as **regenerative medicine**.

For many heart patients, this could mean that in the future heart tissue could be repaired using replacement cells grown from stem cells. This field of research shows similar promise in other conditions such as Alzheimer's disease, diabetes and Parkinson's disease.

The main types of stem cell commonly used in research are:

- **embryonic stem cells**, which exist within early embryos and are the most pluripotent, capable of differentiating into any cell in the body
- **adult stem cells**, which exist in the body after birth and can only develop into a limited range of mature cell types
- **induced pluripotent stem cells**, which are adult cells that have been artificially made to take on the qualities of embryonic stem cells
- **umbilical cord blood stem cells**, which contain several types of adult stem cells and can be collected, frozen and stored in cord blood banks after birth.

To maximise the potential of stem cells, research into both adult and embryonic stem cells must be carried out.[2] By studying embryonic stem cells we will eventually understand their molecular properties and apply this knowledge to adult stem cells for the development of treatments. Research using adult stem cells holds great promise because the patient's own cells might be used to repair damaged tissues.

Past studies in animals have shown that stem cells can improve the function of a damaged heart. By improving our knowledge about the chemicals, genes, cells and tissues involved in repairing the heart, this could be ultimately translated into humans. To gain this fundamental understanding, we need to establish how to isolate and identify stem cells in the adult, how to stimulate them to turn into the cells we want, and how to make them incorporate and function safely and successfully as part of a tissue or organ.

Stem cells may also become more important in future drug development. Cell cultures grown from human stem cells could be useful for testing the safety and effectiveness of new drugs before they are given to people. A public-private consortium, Stem Cells for Safer Medicines, has been set up with the aim of helping to develop stem cell models for drug screening.[3] These developments may help to reduce the number of animals used in medical research. We may in the future also be able to improve the safety of new drugs by using stem cells from humans to study any potential toxicity.

Science cannot predict at this stage which types of stem cell will prove to be of most benefit, and so continued research on all types of stem cells remains necessary to improve our knowledge.[4]

[2] House of Lords Science and Technology Committee Report on Stem Cell Research (2002)
[3] www.sc4sm.org
[4] European Science Foundation (2010): Human Stem Cell Research and Regenerative Medicine

Stem cell research in the UK

The UK has been a leader in stem cell research since the 1970s, when the first mouse embryonic stem cell was isolated. Since 2000, a number of developments have advanced the stem cell research environment to help to develop this world-leading position.

In 2002 the UK Stem Cell Bank was created, the world's first such institute, to hold high quality stem cell lines from adult, foetal and embryonic tissues.[5] Jointly funded by the Medical Research Council and the Biotechnology and Biological Sciences Research Council, the first cell lines were deposited in 2004.[6]

The UK's position at the forefront of this research has been assisted by a supportive regulatory climate and favourable public opinion.[7] The strengths of the UK system present opportunities to lead the application of basic research to clinical benefits and attract talent and investment from abroad in stem cell research.[8] It is therefore important that the UK is taking full advantage of the favourable environment for stem cell research.

Government support for stem cell research

The UK Stem Cell Initiative was set up by the UK Government in 2005 to conduct a review to create a ten-year vision for UK stem cell research. The Initiative highlighted the critical importance of continued investment in stem cell research, and the report was endorsed by the Government. The recommendations included:

- increased government and research council funding for basic stem cell research over the next decade
- redirecting the UK's world class academic researchers in developmental biology to stem cell research
- more funding and development of the UK Stem Cell Bank
- the development of research council resourced Centres of Excellence, to ensure sufficient training opportunities and incentives are available for post-graduate and post-doctoral researchers and to keep existing stem cell researchers in the UK
- establishing a specialised research ethics committee for stem cell clinical research
- requiring researchers using in vitro embryonic stem cell lines to register with, and submit an annual research summary report to, the UK Stem Cell Bank
- creating a national network for scientists, clinicians, ethicists, policy makers, regulators and commercial organisations with an interest in stem cell research

The UK National Stem Cell Network was created in 2006 to address some of the recommendations. This is funded by four research councils.

The UK Government pledged to increase basic research funding into stem cell research to £100m between 2006-08.[9] In 2007-08 the Government provided over

[5] www.ukstemcellbank.org.uk
[6] BBC Online: "UK opens pioneer stem cell bank" (19/05/04)
[7] Medical Research Council, Biotechnology and Biological Sciences Research Council, Sciencewise (2008): Stem Cell Dialogue
[8] Medical Research Council (2008): Stem cell science in the UK – key facts about research, regulation and funders
[9] Department of Health (2007), Government response to the UK Stem Cell Initiative report and recommendations

£60 million of public funding for stem cell research - £24.7 million of which focused on research relating to adult stem cells and £14.7 million on embryonic stem cells.[10]

In 2008, the Medical Research Council (MRC) opened a Centre for Regenerative Medicine to develop therapies for major diseases including cancer, heart disease, diabetes, and degenerative diseases, using stem cells as a starting point.

Translational funding

In addition to being a world leader in basic stem cell research, the UK aims to lead in the translation into clinical therapies. While funding is available for trials in small numbers of patients, it has been difficult for researchers to obtain funding for the large-scale clinical trials required before a treatment can be licensed.[11] The time and cost of such trials mean these often require industry funding. But there is a lack of market appeal for some potential treatments, for example where a patient's stem cells could be used for their own treatment.[12]

The UK Government, in response to the House of Lords Science and Technology Committee Report on Stem Cell Research in 2002, recognised the need to provide long term investment to ensure the potential of basic research is realised.[13]

In 2010, the House of Commons Science and Technology Committee urged the Government to take a greater role in early stage translation for stem cell research and provide greater funding. In stem cell research, this would mean providing funding until it is possible for a regenerative medicine product to demonstrate that it is safe and effective.[14]

The UK Government has begun to invest more in regenerative medicine, and has announced a "RegenMed" programme to aid regenerative medicine businesses in the UK. Managed by the Technology Strategy Board, this investment totals an initial £21.5 million.[15] The Medical Research Council also announced a new translational stem cell research programme, aiming to fund £10 million per year through the Translational Stem Cell Research Committee.[16]

At present, stem cell research into cardiovascular disease is not yet at a stage to take full advantage of these opportunities, but as basic science in this area develops further there may be advancements towards translation.

Regulation in the UK

The UK provides a progressive regulatory environment that has enabled a variety of research to take place.

The Human Fertilisation and Embryology Act (1990) laid the foundation for regulating embryonic stem cell research. The Act established the Human Fertilisation and Embryology Authority (HFEA) to regulate the creation, use, storage and disposal of embryos produced through in vitro fertilisation (IVF).

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[10] House of Lords response to written question, Lord Drayson, Science Minister (29/06/09)
[11] Parliamentary Office of Science and Technology (2009), Postnote on Regenerative Medicine
[12] Ibid
[13] Department of Health (2002), Government Response to the House of Lords Science and Technology Committee Report on Stem Cell Research
[14] House of Commons Science and Technology Committee (2010), Report on Bioengineering
[15] Department of Business, Innovation and Skills (2010), Life Sciences 2010: Delivering the Blueprint
[16] www.mrc.ac.uk/Fundingopportunities/Grants/TSCRC/index.htm

Embryonic stem cells used in research are donated by couples that have undergone IVF treatment. They have chosen to donate embryos that would have otherwise been destroyed, so that these can be used in stem cell research. By law, only embryos at a very early stage of development – up to 14 days – can be used.

The UK's progressive position on the use of embryonic stem cells in research has been reinforced in subsequent reforms. In 2008, the Government passed a new Human Fertilisation and Embryology Act that included provisions for the creation of human admixed embryos – embryos containing human and animal material. As there are a limited number of human embryos available for research, this could provide an alternative source for stem cells more in the future. Types of admixed embryo include:

- chimeras, formed by merging human and animal embryos
- hybrids, by fertilising a human egg with an animal sperm, or vice versa
- cybrids, by inserting human DNA into an animal egg from which the nucleus has been removed.[17]

The HFEA has oversight where embryos are created and used as a source for embryonic stem cell lines. When the embryo is destroyed the Human Tissue Authority (HTA)'s regulatory remit begins. The HTA also regulates the use of adult stem cells. The Medicines and Healthcare products Regulatory Agency (MHRA) becomes involved when a cell therapy is intended to be a medicinal product or an investigational medicinal product.[18]

Current regulation, though conducive to enabling stem cell research, provides a complex route for stem cell research. To develop a therapy from embryonic stem cells, a research establishment would need approval or licensing from eight different organisations: the HFEA, HTA, Research Ethics Committee, NHS Research and Development office, UK Stem Cell Bank, Home Office Animal Licensing Directorate, MHRA, and the European Medicines Agency.

Critics of the system highlight that different parts of the system do not work together as well as they could, and there are currently gaps and overlaps between the agencies involved.[19] The MHRA and HTA indicated in 2010 that they are developing a memorandum of understanding to eliminate regulatory overlaps. The Government also launched the Stem Cell Tool Kit in December 2009, an online resource to help researchers navigate their way through the complexities of the UK regulatory process.[20] In July 2010 the Government announced that it would seek to bring the regulatory functions of both the HFEA and HTA together within a new single research regulator by 2014.

Another area of complexity that could hinder further development of stem cell treatments concerns induced pluripotent stem cells. These cells show promise as they are derived from the patient that would be treated. However, under current European and US rules, each new set of cells for each patient would have to be approved independently. The associated evidence required for a single cell line that's

[17] Henderson, M., Science Editor, Times Online: "Q&A: Human Fertilisation and Embryology Bill" (26/03/08)
[18] House of Commons Science and Technology Committee (2010), Report on Bioengineering
[19] Ibid
[20] www.sc-toolkit.ac.uk

intended for therapy has led to suggestions that patient-specific iPS cells for treatment are impractical.[21]

International competitiveness

The UK has been host to significant breakthroughs in stem cell research, and is widely renowned as a world leader. However, there are concerns that the UK's position is in danger of being left behind by international challenges.

The US Government's moratorium on federal funding for embryonic stem cell research provided an opportunity for the UK to attract expertise and funding. Despite this, some US states were able, through their own state law, to continue funding stem cell research including embryonic stem cells. The State of California for example allocated $3 billion for stem cell research between 2004 and 2009.[22] In addition, the US National Institutes of Health have spent considerable amounts on other types of stem cell research in the past decade.[23]

Commercial stem cell companies

As stem cell research continues to develop, treatments that use stem cells will become available for a number of different conditions including cardiovascular disease. Currently, no stem cell therapies for cardiovascular disease are licensed for use within the UK. The range of other diseases for which there are proven treatments based on stem cells is also still extremely small.[24]

A small number of private clinics abroad claim to offer effective stem cell therapies. [25] These operate without rigorous regulation, and offer untested therapies that have not undergone the thorough animal testing or phased clinical trials that are required for licensed therapies elsewhere.

The UK National Stem Cell Network has described these treatments as at best ineffective, positively dangerous, and potentially fatal.[26] These experimental treatments, which have not been properly tested and approved for human use, often involve a type of stem cell being injected into a diseased area of the body. Without knowing the consequences, this places a significant risk to the patient. There have been examples in the past where tumour lesions developed in patients following unlicensed treatments involving injected stem cells, which have led to fatality.[27]

The International Society for Stem Cell Research has created a website that provides the public with information evaluating the claims made by clinics on stem cell therapies.[28]

[21] Henderson, M., Science Editor, The Times: "Medical potential of IPS stem cells exaggerated says world authority" (17/02/10)
[22] Parliamentary Office of Science and Technology (2009), Postnote on Regenerative Medicine
[23] Medical Research Council (2008): Stem cell science in the UK – key facts about research, regulation and funders
[24] International Society for Stem Cell Research (2008): Patient Handbook on Stem Cell Therapies: Appendix I of the Guidelines for the Clinical Translation of Stem Cells
[25] Coghlan, A., New Scientist: "Death revives warnings about rogue stem cell clinics" (17/06/2010)
[26] www.uknscn.org/downloads/stem_cell_tourism.pdf
[27] Nagy, A. & Quaggin, S.E. (2010), Stem Cell Therapy for the Kidney: A Cautionary Tale. J Am Soc Nephrol 21: 1070–1072, 2010
[28] www.closerlookatstemcells.org

BHF activity

The BHF currently provides funding for over £16 million of research that involves stem cells, including embryonic and adult cells. These consist of project, programme and fellowship grants, representing a major commitment to this pioneering area of research in the UK.

As part of the BHF's 50th anniversary, we are seeking to raise £50 million to fund a vital new programme of regenerative medicine. From the Mending Broken Hearts appeal we intend to develop up to two Centres of Regenerative Medicine to facilitate this groundbreaking work

The BHF is also committing £1 million in partnership with the MRC (who are funding £1.5 million) to fund up to three centre development grants to strengthen the infrastructure for cardiovascular research involving stem cells.