



# The Essential Guide to Stem Cells

Gain an overview of the stem cells industry

Understand how stem cells are commercialised and what is driving today's drug discovery market

Discover what the future holds

# Introducing Mr Erik Miljan



Mr Erik Miljan has over 10 years experience in the Stem Cell Biotech sector, from intellectual property (IP) portfolio management through product development to regulatory approval. He initiated, implemented and delivered the operational and management strategy leading to the first ever stem cell based product approved for clinical trial in the UK, in addition to delivering non-therapeutic stem cell products to market. He has experience in international settings across both academia and industry in the US, Japan, Australia and the European Union.

This e-book, the [12 hour MBA in Stem Cells](#) and the [3 day MBA in Stem Cells](#) are all specifically designed for those who are new to the industry. They provide an overview of the industry helping you to get up to speed quickly. Click [here](#) to find out more.

# The essential guide to Stem Cells

In this essential guide to stem cells you will:

1. Understand why stem cells are special and where adult stem cells are found
2. Discover what is driving today's drug discovery market
3. Explore the different types of treatment strategies and the conditions that have been helped by stem cell therapies
4. Take a look at how the stem cell market is composed
5. Learn how companies engaging in stem cell therapy are financed
6. Explore the human stem cell debate
7. Look at future trends in stem cells and which therapeutic applications will be the market frontrunners

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# Who, What, When and Where?

Stem cells are at the forefront of science. Although the field is still in its infancy there is constant advancement.

Your body contains over 250 different cell types, each customised for a particular role. So how do we get so many cells to make up our body? Cells divide by multiplying, in a process called cellular division. It goes through the cell cycle, the S phase (synthesis of DNA), then progresses through to G1 and then G0. This creates a new cell.

Stem cells are special as they can renew themselves through mitotic cell division and differentiate into a diverse range of specialized cell types. Stem cell division either divides symmetrically or by asymmetric division.

Classically, stem cell potency is the measure of the number of differentiated offspring that can be generated from a particular stem cell.

There is a hierarchy in stem cells:

**Totipotent** - able to generate any cell type, as well as a placenta

**Pluripotent** - can generate all three germ layers within the body and generate all other types of stem cells as well

An adult stem cell is an undifferentiated cell, found among differentiated cells in a tissue or organ that can renew itself and can differentiate into major specialized cell types of the tissue or organ.

Adult stem cells are found in many more tissues than was originally thought possible:

- Hematopoietic stem cells (HSC)
- Bone marrow stromal stem cells (also called mesenchymal stem cells) (MSC)
- Neural stem cells (NSC)
- Skin stem cells
- Adipose stem cells

“Stem cells are special as they can renew themselves through mitotic cell division and differentiate into a diverse range of specialized cell types”

# Stem Cells in Drug Discovery

## Q and A time!

### Q1. What is the basis for stem cell drug discovery?

A1. Stem cells are powerful tools in drug discovery. “Long ago it became evident that the key to every biological problem must finally be sought in the cell; for every living organism is, or at some time has been, a cell.” — E.B. Wilson (1856–1939)

### Q2. What is driving today's drug discovery market?

A2. There are many factors driving today's drug discovery market. They include:

- The huge potential of stem cells to model human normal and disease physiology in a screening/assay paradigms
- Biotech and Pharma frontloading human relevant toxicity testing in preclinical drug discovery
- Limitations of current cell models - abnormal tumour cells and inaccessible human tissue are preventing old methods moving forward
- Replace animal models for ethical and cost reasons
- Commercial availability of tools and technologies

### Q3. Some would say that stem cells are the ideal drug discovery tool. Why?

A3. Stem cells are a renewable source of human cells and they can be readily differentiated into functional human mature cell types.

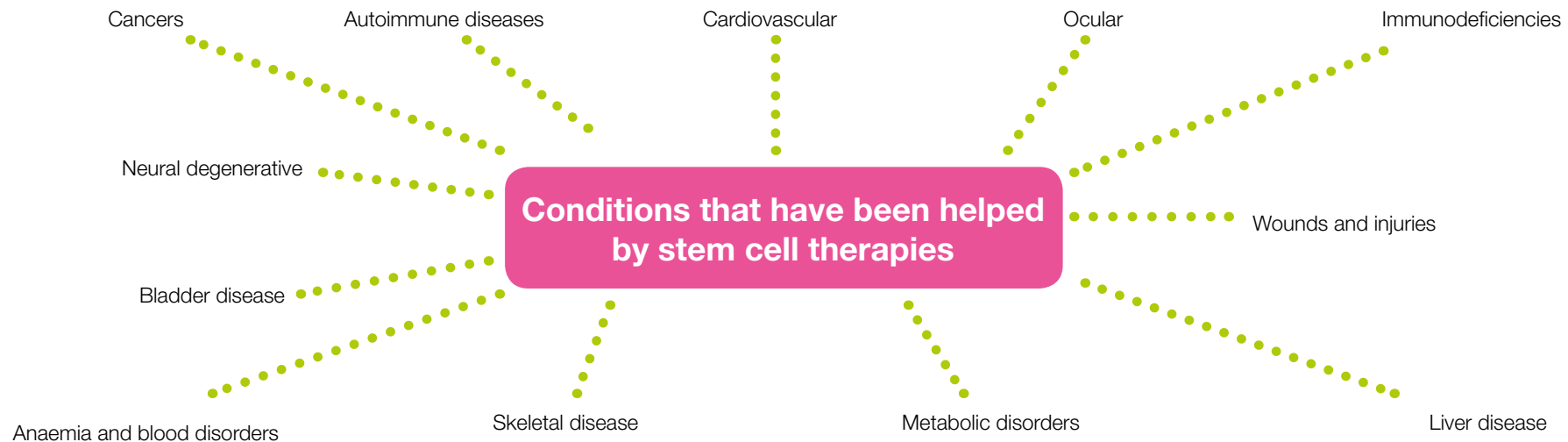
### Q4. Stem cell-based modelling of diseases in a dish is becoming increasingly popular. Why?

A4. Traditionally animal models of disease have enabled enormous advances in understanding. However, animal models have shortcomings; often they are artificially induced and do not represent the true underlying disease state in humans. Here we can use human disease specific stem cell lines, which can overcome these challenges, at least in part.

# Therapeutic Applications of Stem Cells in Regenerative Medicine

Stem cells are at the very heart of cell therapy and regenerative medicine.

But what is regenerative medicine? Regenerative medicine is a process which replaces or regenerates human cells, tissues or organs to restore or establish normal function.



There are three different types of treatment strategies from the stem cell source:

1. Autologous – stems cells that are derived from oneself
2. Allogeneic – stems cells that are derived from a related species but not one of an identical genetic makeup
3. Xenogeneic – stem cells that are derived from a different species, e.g. an animal that are transplanted back into a human



# Landscape of the Stem Cell Industry

Stem cells have become an integral part of almost every facet of life science research across academia, biotech and pharma.

The diverse application of stem cells is ever growing and includes:

- Stem cells in basic research
- Stem cells in drug discovery
- Stem cells as therapies

How is the stem cell market composed?

- Stem cell products (adult stem cells, human embryonic stem cells and others)
- Stem cell services (stem cell banking, stem cell acquisition and testing, drug discovery, isolation/characterization services and molecular biology)
- Stem cell technologies (stem cell acquisition, stem cell production, cryopreservation)
- Stem cell applications (regenerative medicine and therapy)

How is the stem cell market segmented?

- Stem cell research
- Stem cells for drug discovery
- Cell therapies

Did you know...adult derived stems have been researched for number of years and now there is an emerging market for commercialisation. However, recent interests from major pharmaceutical companies have shown, instead of the use of drugs for incurable diseases, stem cell therapies can be used to repair, regenerate and replace the diseased tissues in the body.

# How do we Commercialise Stem Cells?

Stem cell therapy is extremely cash consuming when compared with the rest of the bio-tech industry. It requires a constant flow of financing. Currently the sector is fragmented and poorly financed compared to other parts of the biotech industry.

## Financing Sources

Generally, companies engaging in stem cell therapy are financed by:

- Government programs
- Private funds (venture capital) and grants
- Initial public offerings (IPOs)
- Funding from universities (although this is largely unknown. In the US and Canada universities have funding to invest in their own R&D projects)

## Stem Cell Patent Landscape

It's worth remembering that a key aspect of obtaining investment in any biotechnology field is the availability of patent protection.

# Ethics and Government of Stem Cells

Ethics and government of stem cells is broken into two categories:

1. Ethics of regulated stem cell clinical trials (conducted at a national level)
2. Ethics and government of stem cell research (conducted at a national level concerning stem cell types, cloning and reprogramming)

In the UK, GTACC approval must be sought before MHRA approval will be given. GTACC can stop the progress of an application. In the US it must be reviewed by the Recombinant DNA Advisory Committee before FDA approval.

## The Human Stem Cell Debate

Derivation of pluripotent human stem cell (hSC) lines from oocytes and embryos is fraught with disputes regarding the onset of human personhood and human reproduction. With any human stem cell research there are difficult dilemmas including:

- Consent to donate materials for hSC research
- Early clinical trials of hSC therapies
- Oversight of hSC research

It's widely argued that adult stem cells do not attract ethical issues like human embryonic stem cells.

# Future Trends in Stem Cells

Stem cell based products are following a similar pattern of growth to the monoclonal antibodies market. Large pharmaceutical, biotechnology and medical device companies are investing in stem cell research and EMEA regulations and guidelines concerning stem cell research are dynamically evolving. Stem cell therapy holds the highest potential in the field of regenerative medicine, and is likely to experience widespread application by early/mid 2017.

## Future Predictions

- Hematopoietic stem cell transplants will continue on the same clinical track, with more emphasis on allogeneic
- Therapeutic applications for skin, eye and orthopaedic conditions to be the market frontrunners
- Stem cells in drug discovery and toxicology to become widespread. Human functional hepatocytes and cardiomyocytes are now available on the market

## CROs and CMOs will experience high growth

- The CROs and CMOs market for stem cells is predicted to grow rapidly in the next 5 years
- Increasing demand to outsource R&D by large pharma

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# We'd love to hear your views on all of this...

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# Wondering if you know all there is to know about Stem Cells?

Why not try our 12 Hour MBA in Stem Cells.

The 12 Hour MBA in Stem Cells will take you through the modules covered in this E-Book (and more!) in much more depth. Erik Miljan will walk you through the fundamentals of stem cells in an easy to manage online training course.

For more information contact Evon Wong on: +44 (0) 207 608 7028

Or visit: <http://www.terrapintraining.com/training/12-Hour-MBA-in-Stem-Cells>

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# We'd love to meet you too...

We also run a 3 day MBA in Stem Cells in various locations across the globe.

Alternatively, we can come to you. We can tailor the course to your needs and deliver the course for you and your team.

For more information contact Evon Wong on: +44 (0) 207 608 7028

Or visit: <http://www.terrapintraining.com/training/3-day-mba-in-stem-cells>

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