THE A TO Z OF STEM CELLS

WORLD StemCells
Regenerative Medicine Congress 2014

THE A TO Z OF STEM CELLS

The regenerative medicine industry is booming, and significant growth is expected over the next decade. In light of the ongoing change and new developments, we have pulled together a list that represents what's happening in this space. This is our A-Z of stem cells, a complete look at the key considerations, recent breakthroughs and leaders in this sector.

Does it match your list? If not, tell us what we have missed out. Leave your comments in our LinkedIn group or tweet them to us @totalbiopharma #stemcells #wscrm

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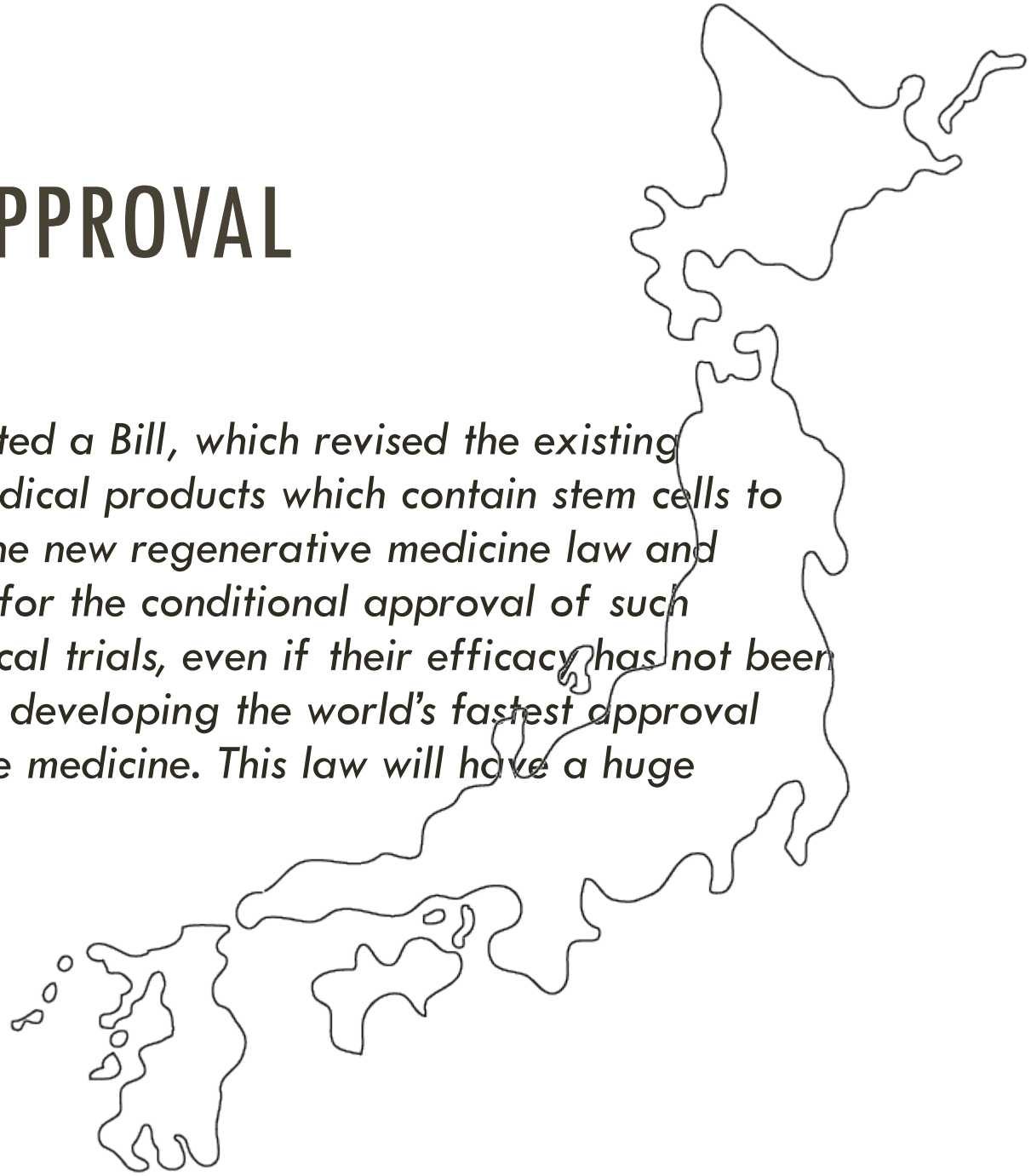
A IS FOR ACCELERATED APPROVAL

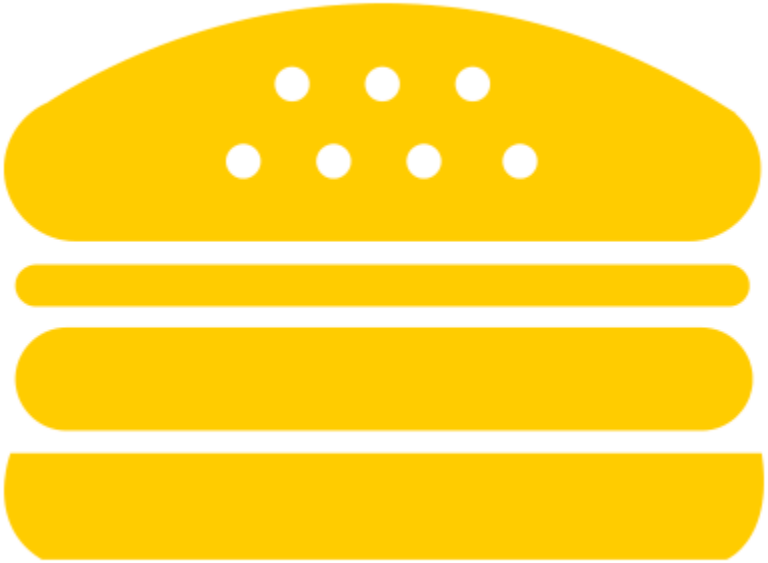
*"Japan's parliament, 'the Diet', recently enacted a Bill, which revised the existing Pharmaceutical Affairs Law to define new medical products which contain stem cells to be termed regenerative medicine products. The new regenerative medicine law and revised pharmaceutical affairs law will allow for the conditional approval of such products if safety has been confirmed in clinical trials, even if their efficacy has not been fully demonstrated. Leading the way towards developing the world's fastest approval process specifically designed for regenerative medicine. This law will have a huge implications for the industry globally."**

*SOURCE: http://www.stemcellsresearch.me/tag/japan



B IS FOR BURGER

"A beef burger grown from cow stem cells in a laboratory was put to the taste test in August 2013. Mark Post and his team at Maastricht University in the Netherlands developed the burger from the muscle cells of two organic cows. The cells were put into a nutrient solution to help them develop into muscle tissue, growing into small strands of meat. Its hopes that making meat in labs could eventually help feed the world and fight climate change. Watch the cultured meat space in 2014."



*SOURCE: http://www.cbsnews.com/news/burger-grown-from-cow-stem-cells-in-laboratory-put-to-taste-test-in-london/

C IS FOR CELL THERAPY CATAPULT

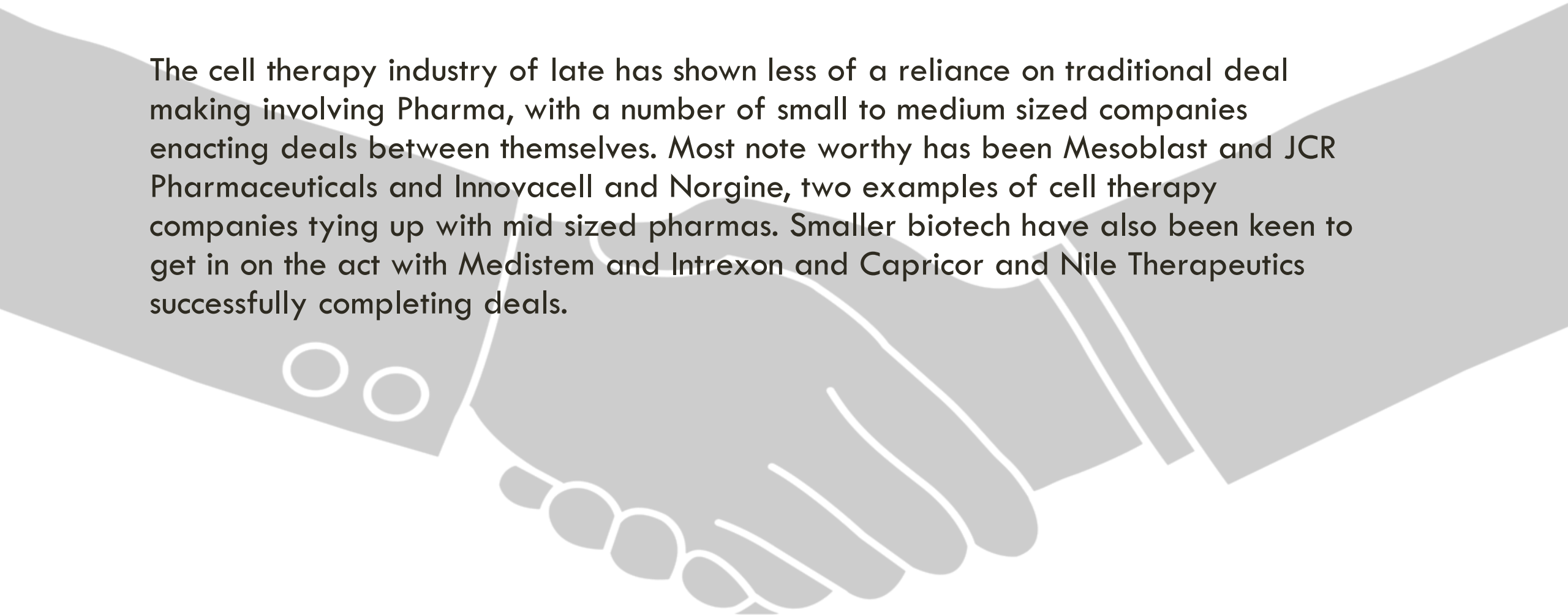
*"The Cell Therapy Catapult's vision is for the UK to be a global leader in the development, delivery and commercialisation of cell therapies, and a place where businesses can start, and confidently grow. It was established in recognition of the need for concerted long-term translational activities in order to realise the full value of cell therapies, and operations will grow rapidly throughout 2014. The Cell Therapy Catapult will take products into the clinic, de-risking them for further investment, provide clinical expertise and access to NHS clinical partners, be a source of regulatory expertise, provide technical expertise and infrastructure to ensure products can be made to GMP and delivered cost effectively, generate national and global opportunities for collaboration and provide access through our network to business expertise, grants and investment finance."**

*SOURCE: https://www.catapult.org.uk/cell-therapy-catapult

CATAPULT
Cell Therapy

D IS FOR DEAL MAKING

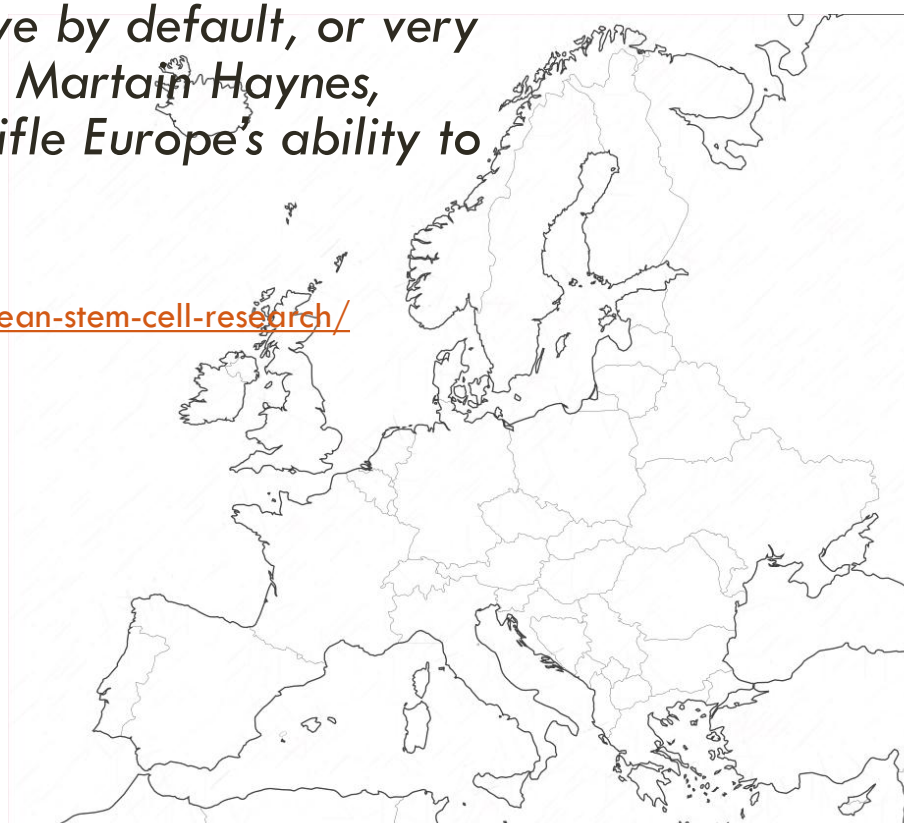
The cell therapy industry of late has shown less of a reliance on traditional deal making involving Pharma, with a number of small to medium sized companies enacting deals between themselves. Most note worthy has been Mesoblast and JCR Pharmaceuticals and Innovacell and Norgine, two examples of cell therapy companies tying up with mid sized pharmas. Smaller biotech have also been keen to get in on the act with Medistem and Intrexon and Capricor and Nile Therapeutics successfully completing deals.



E IS FOR EUROPE

*"A report published by the European Science federation (ESF) has given 27 different countries one of four possible statuses based on the permissiveness of their stem cell policies. With the possibility of being deemed either very permissive with regards to human Embryonic Stem Cells, permissive with restrictions, restrictive by default, or very restrictive, the authors of the paper, Professor Stigordahl and Mr Martain Haynes, suggest that those countries with restrictive agendas could well stifle Europe's ability to retain its key role in the stem cell industry."**

*SOURCE: http://www.totalbiopharma.com/2013/11/11/germany-italy-standing-progress-european-stem-cell-research/



F IS FOR FUNDING

*"Supporters of California's multibillion-dollar stem cell program plan to ask for $5 billion more to bring the fruits of research to patients. Robert Klein, a leader of the 2004 initiative campaign that established the program, is planning on talking with California voters about the proposal. If the public seems receptive, backers will work to get an initiative on the 2016 ballot to extend funding for the California Institute for Regenerative Medicine."**

*SOURCE: http://www.utsandiego.com/news/2014/Feb/20/robert-klein-cirm-stem-cell-billion-2016/

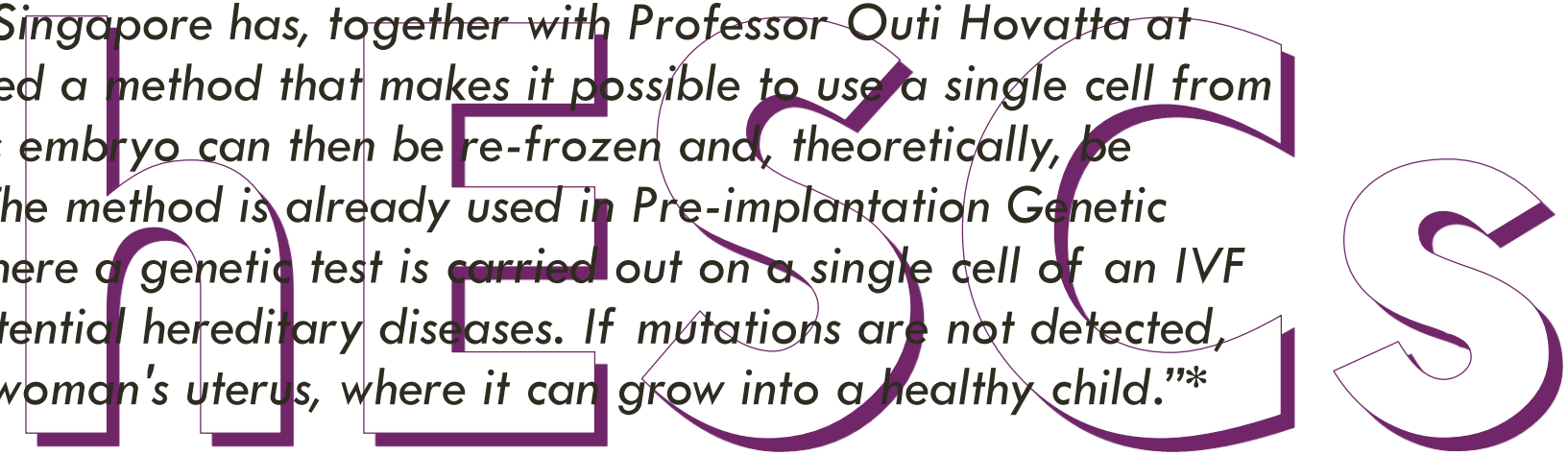
G IS FOR GENE THERAPY

*"Gene therapy has generated exceptional excitement and interest over its potential for over three decades, but actual clinical progress has been painfully slow and frustrating. Bluebird Bio (BLUE) (and I am choosing to capitalize the name, even though it is officially lower case) may be on its way to transforming at least part of the dreams and hopes of gene therapy into reality. Bluebird has developed a platform that management believes will allow the company to deliver therapies via lentivirus vectors to treat single-gene rare/orphan disorders. The company's lead compound addresses a small market, but the second could be a potential billion-dollar therapy. The risks here are definitely elevated, but Bluebird may be in the early days of establishing a leadership position in a major therapeutic class."**

*SOURCE: http://seekingalpha.com/article/2053683-bluebird-bio-may-turn-dreams-into-reality

H IS FOR HESCS

*"A new method allows for large-scale generation of human embryonic stem cells of high clinical quality. It also allows for production of such cells without destroying any human embryos. The discovery is a big step forward for stem cell research and for the high hopes for replacing damaged cells and thereby curing serious illnesses such as diabetes and Parkinson's disease. An international research team led by Karl Tryggvason, Professor of Medical Chemistry at Karolinska Institutet and Professor at Duke-NUS Graduate Medical School in Singapore has, together with Professor Outi Hovatta at Karolinska Institutet, developed a method that makes it possible to use a single cell from an embryo of eight cells. This embryo can then be re-frozen and, theoretically, be placed in a woman's uterus. The method is already used in Pre-implantation Genetic Diagnosis (PGD) analyses, where a genetic test is carried out on a single cell of an IVF embryo in order to detect potential hereditary diseases. If mutations are not detected, the embryo is inserted in the woman's uterus, where it can grow into a healthy child."**



*SOURCE: http://www.eurekalert.org/pub_releases/2014-01/ki-nmi012414.php

I IS FOR IPO

Signs of life are appearing in the stem cell and cell therapy IPO market. An unprecedent number of Biotechs' have recently followed through on successful initial public offerings. Blubird Bio topped this exclusive list with EUR 87M raised followed by the likes of Fate Therapeutics, CDI and Cardio3 BioScience with a combined EUR 110M raised. Other noteworthy IPOs include KadimaStem, Regeneus, ReproCell and Vital Therapies. All eyes could be on Health Point Inc. in 2014.

bluebirdbio™

Fate Therapeutics

Cellular Dynamics international

CARDIO3 BIOSCIENCES

We care. We cure.

J IS FOR JOHNSON & JOHNSON

Following Capricor Therapeutics $20 million injection from the California Institute for Regenerative Medicine (CIRM), Johnson & Johnson is stepping up with a $12.5 million upfront to buy itself an option and a front-row seat on the program. J&J will have 60 days following delivery of the 6-month follow-up to Phase II to decide whether it wants to take control of the program and carry it through late-stage studies. And if J&J takes the next step, it will be committed to a $325 million package of milestones, plus royalties on any sales. Could this mark the start of a rare Big Pharma injection on a field that is trying hard to mount a comeback.*

Johnson & Johnson

*SOURCE: http://www.fiercebiotech.com/story/jj-gambles-125m-rare-big-pharma-bet-stem-cell-therapy/2014-01-06

K IS FOR KIDNEYS

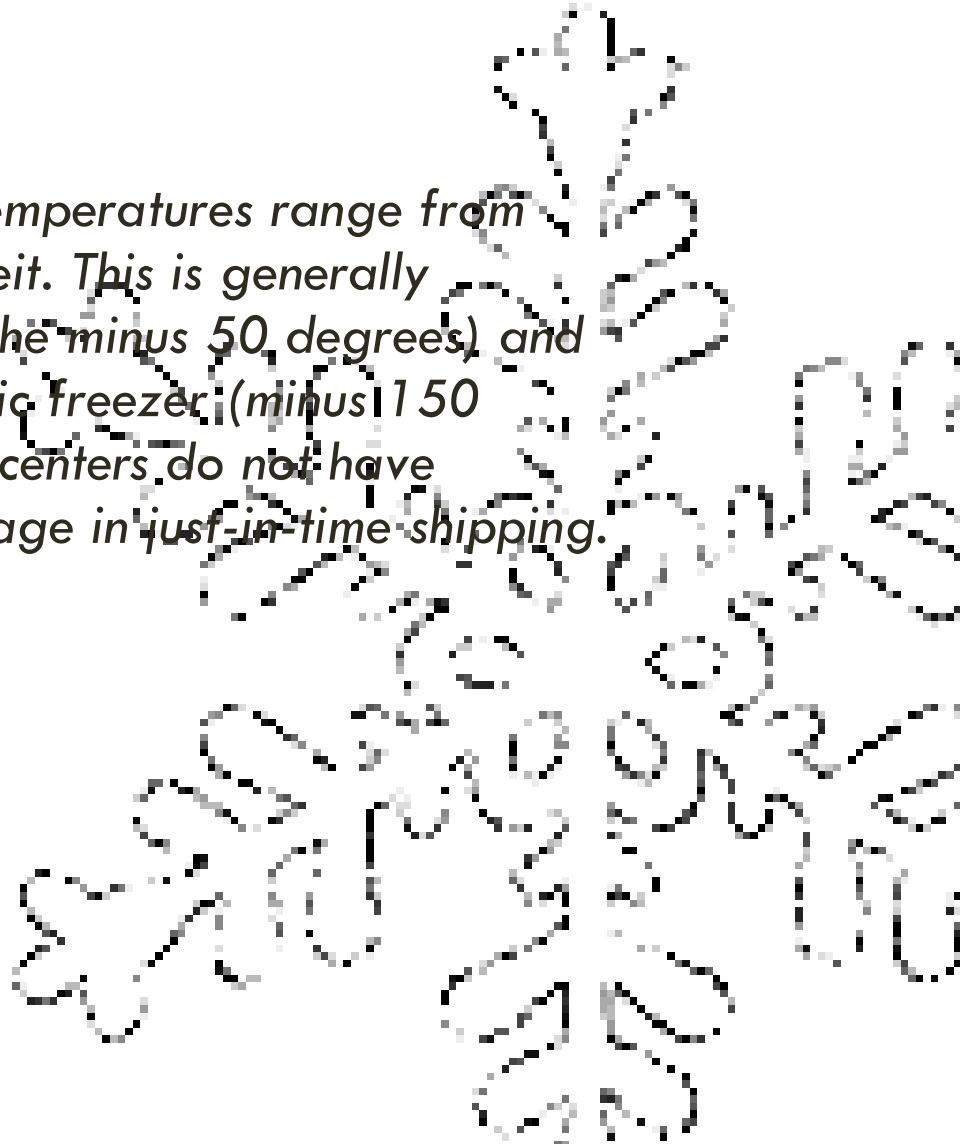
*"Scientists in Australia have grown the world's first kidney from stem cells – a tiny organ which could eventually help to reduce the wait for transplants. The breakthrough, published in the journal Nature Cell Biology, followed years of research and involved the transformation of human skin cells into an organoid – a functioning "mini-kidney" with a width of only a few millimetres. Scientists are hoping to increase the size of future kidneys and believe the resulting organs will boost research and allow cheaper, faster testing of drugs. Within the next three to five years, the artificial organs could be used to allow doctors to repair damaged kidneys within the body, rather than letting diseases develop before proceeding with a transplant."**

*SOURCE: http://www.telegraph.co.uk/news/worldnews/australiaandthepacific/australia/10520058/Kidney-grown-from-stem-cells-by-Australian-scientists.html

L IS FOR LOGISTICS

*"Stem cells must be cryo-preserved. Storage and shipping temperatures range from minus 50 degrees Fahrenheit to minus 150 degrees Fahrenheit. This is generally accomplished by shipping stem cells in dry ice (maintaining the minus 50 degrees) and then either using immediately or storing briefly in a cryogenic freezer (minus150 degrees). Most physician offices, pain clinics or ambulatory centers do not have cryogenic freezers. To use stem cell therapies, they must engage in just-in-time shipping. As soon as the dry-ice container arrives, use the cells."**

*SOURCE: http://ryortho.com/2014/02/new-york-stem-cell-summit-2014-preview/



M IS FOR MARKET WORTH

The market is predicted to see robust growth in the years ahead, driven primarily by the ongoing advancements in the cell therapy industry along with considerable developments in the world of medicine. The overall cell therapy market is forecast to register a 21% CAGR on average in the upcoming years; and is anticipated to amount to GBP 5 billion by 2018. A number of cell-based products and technologies, which are currently in the R&D pipeline, are expected to enter the market during the next five years, thus encouraging an increase in the growth rate of the cell therapies market. The expanding prevalence of diseases together with the lack of adequate effective treatment option for these illnesses is most likely to spur the advances in the cell-based therapeutics market both in developed and developing countries. The development of sophisticated automation devices is the most prominent emerging trend in the overall cell based therapy market.*

*SOURCE: http://www.prweb.com/releases/2014/03/prweb11645282.htm

N IS FOR NANOTECHNOLOGY

*"Nanotechnology represents a fascinating new outlook on regenerative medicine that could promote extensive research and lead to the realization of interesting and innovative tools to improve and restore tissue function. The integration of nanotechnological biomimetic materials and translational medicine could provide the chance to produce surfaces (e.g., bone, vasculature, heart tissue, cartilage, bladder tissue and brain tissue), structures and systems with nanoscale features that can mimic the natural cellular environment and quickly promote cellular events, such as adhesion, mobility and differentiation. Further improvements, stemming from the optimization of nanomaterials by the continuous introduction of nanotechnology platforms, will boost the development of innovative cell-based therapeutics."**

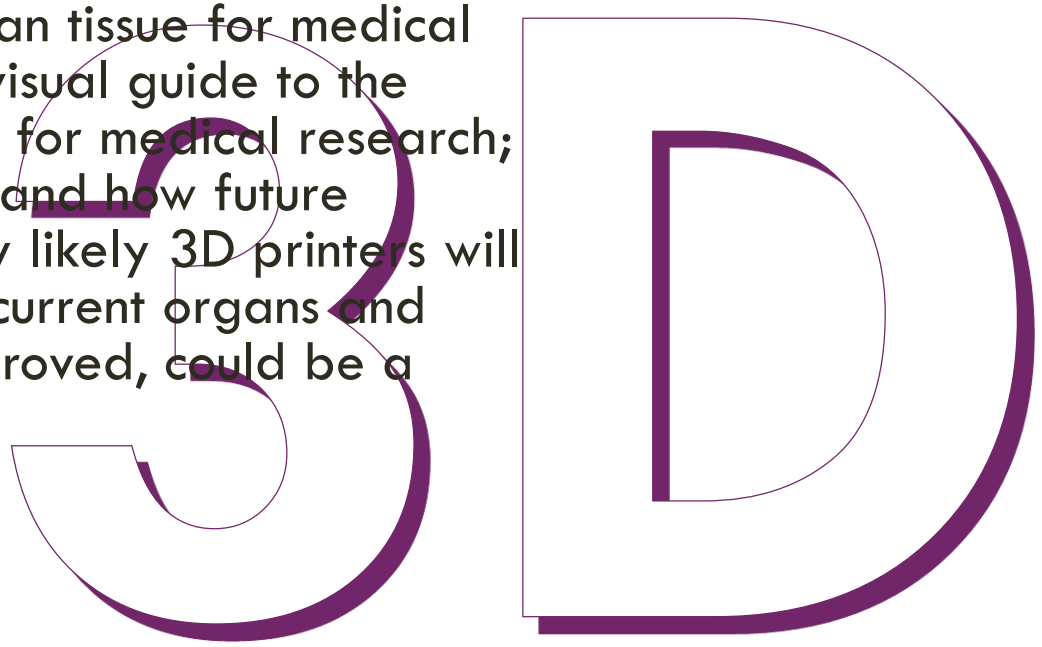
*SOURCE: http://www.medscape.com/viewarticle/780791

O IS FOR ORGAN GROWTH

Paul Knoepfler of the University of California, Davis School of Medicine sees one of the hottest trends in 2014 as organ/tissue growth from stem cells as was seen in the 2nd half of 2013 with livers, brains, kidneys and such. Some of the hottest publications in 2014 to date are on growth of actual organs and tissues that are functional, with many more to follow throughout the year.

P IS FOR PRINTING (3D)

Printing, or the process of creating human tissues through 3D printers, is a highly contested area of technological innovation. From a technological perspective, the rise and development of 3D printing and its capabilities will play an undeniable part in our future lives. UK-based company PrinterInks has teamed up with US startup Organovo, a company specialised in designing and printing functional human tissue for medical research and therapeutic applications, to create a visual guide to the subject. Currently printed tissues are generally used for medical research; introducing disease to monitor how the tissue reacts and how future treatments may be developed. In the future, it's very likely 3D printers will be used to create simple tissues for implanting into current organs and partial organs. The printing of whole organs, if approved, could be a reality within the next decade.*



*SOURCE:
http://www.telegraph.co.uk/technology/news/10629531/The-next-step-3D-printing-the-human-body.html

Q IS FOR QUOTES

"When I saw the embryo, I suddenly realised there was such a small difference between it and my daughters. I thought, we can't keep destroying embryos for our research. There must be another way."
Shinya Yamanaka

R IS FOR REPETITION

*"In an effort to bring clarity to one of the most controversial and confusing scientific findings in recent memory, three Japanese scientists have released a detailed protocol explaining step by step how to create stem cells with a simple acid bath. A leading stem cell scientist at Boston Children's Hospital is working directly with the scientist who led the work to try and repeat the technique. The surprising report in January by Boston and Japanese scientists that stem cells, with the ability to develop into any cell in the body, could be created with the seemingly straightforward technique sparked a raging and very public debate in the scientific community. Within a month, a problem with images in one of the papers was revealed, which the scientists have said will be corrected. Other possible problems have been pointed out on online forums. An investigation by the Japanese scientists' institution has been ongoing, spurred by questions raised by outside scientists."**

*SOURCE: http://www.bostonglobe.com/news/science/2014/03/06/efforts-repeat-controversial-stem-cell-technique-intensify/P3mYc8vsGg7UcAln09SFgM/story.html

S IS FOR SPINAL CORD

"A team of doctors at the University of Calgary has, for the first time in North America, successfully performed a stem cell transplant in a spinal cord injury patient, a procedure that could offer a glimmer of hope to patients whose injuries have long been considered untreatable. The doctors injected the neural stem cells into the spine of a 29-year-old paraplegic, who will now be monitored to determine whether implanting those cells is safe. Later studies will look at whether it is possible to regenerate new tissue and repair the man's injury."

*SOURCE: http://www.ctvnews.ca/health/start-of-stem-cell-study-offers-hope-to-patients-with-spinal-cord-injuries-1.1633993

T IS FOR TRANSDIFFERENTIATION

The process by which stem cells from one tissue differentiate into cells of another tissue. Many in the stem cell community are just as excited about transdifferentiation as they are about iPS cells in 2014. Transdifferentiation is likely to continue to raise eyebrows in the coming year as you see more activity in this area. However, when it comes to a trade off between transdifferentiation and iPS cells, is likely that many in the community want to see both in equal measures.

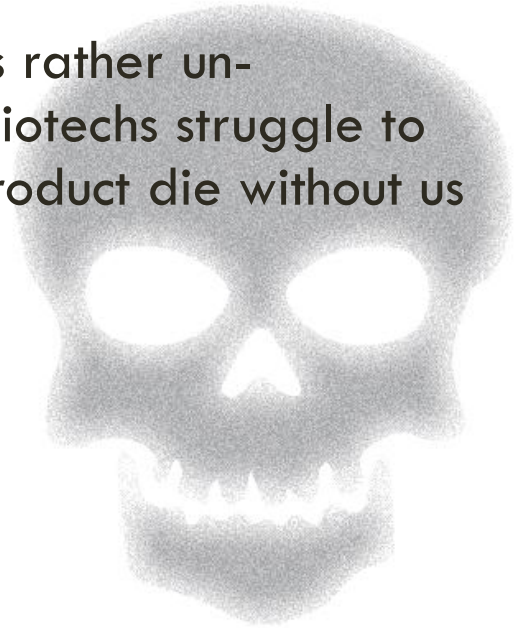
U IS FOR UMBILICAL CORD

*"A man who was given 18 months to live after being diagnosed with leukaemia has said his life has been saved by stem cells taken from umbilical cords. The man was given a transplant of cells donated following births in France and the US. The treatment for patients with cancers such as leukaemia uses donated blood stem cells, usually from adult donors, to replace damaged ones. Blood stem cell transplantation is used to restore cells destroyed by some types of cancer and other blood diseases, such as sickle cell anaemia. After being treated with radiation or high-dose drugs, the patient receives the harvested stem cells, which travel to the bone marrow and begin to produce new blood cells."**

*SOURCE: http://www.bbc.co.uk/news/uk-england-manchester-26150300

V IS FOR VALLEY OF DEATH

The period between basic research and a Phase I clinical trial is rather un-affectionately known to as the *"Valley of Death"*. A time when biotechs struggle to find enough funding to bridge that gap. A time when many a product die without us ever realising its potential.



W IS FOR WORLD STEM CELLS & REGENERATIVE MEDICINE

Europe's leading congress for C-suite stem cell and cell therapy executives returns for its 9th edition in May in London, UK. Continuing to be the 'go to' event for stem cell therapy developers, pharma and investors in Europe it provides an unmatched educational and networking opportunities with high-level executives and influential decision makers. Visit the congress website for more information.

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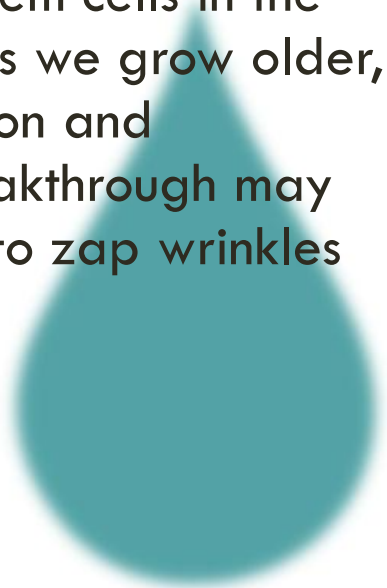
X IS FOR XENOTRANSPLANTATION

*"With the shortage of human organs available for transplant, some work has been done to use pig and nonhuman primate tissues and organs instead. Some critics charge that this could lead to new, dangerous forms of disease if a pathogen that now only affects animals becomes communicable among humans. The use of Stem Cells will eradicate this potentially fatal problem."**

* SOURCE: http://dictionary.reference.com/browse/xenotransplantation

Y IS FOR YOUTH

The secret to the fountain of youth lies in awakening '*sleeping*' stem cells in the skin, according to new research. A computer model found that as we grow older, we lose the ability to trigger these 'master cells' to kick into action and regenerate damaged skin. British and U.S. scientists say the breakthrough may open the door to the development of better beauty treatments to zap wrinkles for good.*



*Source: http://www.dailymail.co.uk/health/article-2349712/The-secret-eternal-youth-Awakening-sleeping-stem-cells-skin-say-scientists.html

Z IS FOR ZEBRAFISH

The Zebrafish or Danio rerio is a tropical freshwater fish famous for its regenerative abilities and has been modified by researchers to produce several transgenic strains.

Not only this, but it is a very popular aquarium fish, sold under the trade name zebra danio.

We’d love to meet you…

WORLD StemCells
Regenerative Medicine Congress 2014

The 9th World Stem Cells & Regenerative Medicine Congress is Europe’s leading scientific and business development event for the stem cell and cell therapy industry.

World Stem Cells & Regenerative Medicine Congress is a proven conference and exhibition that delivers the innovators, disruptors and influencers from across the complete value chain.

Having brought together over 2200 stakeholders in its 8 year history, this event combines translational and clinical research with commercialisation and business development.

Visit the event website