LifeCell – Daily News Update
April 30 , 2009

Key International Research News:

Publication	Pharmaceutical Technology
Headline	**China creates Asia's largest stem cell bank**
Gist of the article	China is seeking to make its mark in the field of stem cell research by starting work on a new facility that will house the country's first comprehensive regenerative medicine technology centre and Asia's largest stem cell bank. The Stem Cell Regenerative Medicine Industrial Project of National Bio-Industry Base (NBPD) is part of a multistage project aimed at providing a central research zone for China's regenerative medicine and biomedical industry. The work was initiated by the Jiangsu government's China Medical City and Shenzhen Beike Biotechnology Co. Ltd (China), which hope that the facility will lay the foundations for China to develop into a world leader in the biotechnology industry. "In the 1970s, the US government provided intellectual property support, and venture capital companies offered the funding that allowed the US to leapfrog past Europe to become a leader in biotechnology and pharmaceuticals," said Sean Hu, Chairman and CEO of Beike Biotechnology. "China's Medical City is now doing the same and we are starting to see the results of these efforts." The 20-m2 facility, which will be located in Taizhou, will house four centres: a technology transfer centre to help take research to the clinic, a testing centre, a clinical support service centre and Asia's largest stem cell bank.

Key International Research News:

Publication	Irishtimes.com
Headline	**Can stem cells change the world?**
Gist of the article	How true are the claims that stem-cell research could one day lead to cures for a range of debilitating diseases and life-threatening conditions? asks DICK AHLSTROM SUPERMAN ACTOR Christopher Reeve was tragically paralyzed on May 27th, 1995, the day he tumbled from a horse and broke his neck. The riding accident left him paralyzed and bound to a wheelchair, but it also gave the world a champion who campaigned tirelessly in support of stem-cell research.

Medical therapies based on the use of stem cells hold great promise for the treatment of currently intractable conditions such as Alzheimer's disease and multiple sclerosis. Researchers also believe they may one day be used to cure the spinal-cord damage that so changed Reeve's life before his eventual death in October 2004.

Reeve left behind a foundation that has put €60 million into spinal-injury research, including work using stem cells.

Similar support for Parkinson's disease research has seen €110 million invested by the Michael J Fox Foundation. The actor launched the foundation after he was diagnosed with Parkinson's almost 20 years ago, and it too is emphasizing the potential of stem-cell research to deliver a cure for the disease.

Irish researchers are more cautious, however, about treatments based on stem cells. While their use offers great promise, we are unlikely to see treatments for serious conditions in less than five to 10 years, they say. Many are also concerned about fraudulent claims being made for stem-cell treatments that might actually cause more harm than good.

The subject arose at a conference on stem-cell research in Dublin last week. It discussed the decision by the parents of eight-month-old Gretta Kieran Cullen to travel to China so that their daughter can receive controversial stem-cell treatments. The infant suffers from septo-optic dysplasia, a genetic condition that has left her blind and suffering from other symptoms. Her treatments began this week and will continue for about a month.

Trinity College Dublin-based research fellow Dr Stephen Sullivan organized the conference and he fears a "backlash" against this important technology when patients who undergo unproven stem-cell treatments fail to get a positive result.

"There is a huge amount of money in offering therapies to people with terrible diseases," he says. "But it is important not to be offering them false hope."

Many such treatments are being advertised on the internet, but they have not undergone proper assessment, he says. "People often talk about embryonic stem cells being transplanted into the patient. This is five to 10 years away. If people come to you offering a cure and they haven't done clinical trials then they have no evidence it will work."

There are a number of tell-tale signs that a stem-cell treatment is unproven and possibly unsafe, he adds. "The first and most important thing is if they are asking for money up front."

This is not to say, however, that stem-cell technology won't find its

way into useful treatments. We already have treatments based on the use of adult stem cells, a form of stem cell recovered from mature tissues, Sullivan adds. “At the moment, when it comes to stem cells, the only real treatments are from adult stem cells like bone marrow transplants.”

Researchers at NUI Galway’s regenerative medicine institute, Remedi, hope to begin testing adult stem-cell treatments in humans as early as next year.

“Our focus is on the development of therapies using [adult] stem cells,” says Prof Frank Barry, Remedi’s professor of cellular therapy. “We are very much translational and our objective has always been that we want to develop something which can be given to a patient.”

His group, which ranges in size from 50 to 70 researchers, has already set up a “stem-cell manufacturing facility” that will allow large numbers of adult stem cells to be grown up in the lab.

The group’s plan is to target two areas, osteo-arthritis of the knee and the reduced blood flow in the feet and hands seen in those with diabetes. The hope is that, once injected, the stem cells will help to reverse the symptoms associated with these two conditions, according to Barry.

THERE HAS BEEN a huge amount of public disquiet about the conduct of a particular type of stem-cell research, the type based on the use of stem cells taken from human embryos, a process which always causes the destruction of the embryo.

Researchers shied away from their use here because of the ethical problems associated with embryonic stem cells until October 2008, when the governors of University College Cork voted by just 16 to 15 to introduce a code of practice that would allow this research.

No such research has started so far, states UCC’s Dr Tom Moore, a principal investigator in the Biosciences Institute, who would like to use these cells for research. The vote, he says, “was to produce a mechanism for getting approval for the research. It has been a lengthy process and it is still ongoing”.

He believes there is nothing to compare with embryonic stem cells because these are the cells in the embryo that transform to become all the tissues of the body.

“They are pluripotent, almost totipotent. You can differentiate them into all the different cell types,” he says.

He is less concerned about using them for quick cures than for the remarkable power they offer for medical research. They allow scientists to study the changes that take place when disease occurs

	and compare this with healthy cells. “Therapies are a long way down the line, but being able to use them is good for basic science. Even if stem cells were never used in human therapies, it would still be hugely valuable to facilitate the study of disease mechanisms,” he says. He mentions his own studies of a cell type found in the base of the brain, called the purkinje cell. Although rare, it is very important because of its potential link to autism.Moore has identified a gene of interest in these cells, but it is a very difficult thing to acquire certain rare cell lines for study, including the purkinje cell. “It would really be valuable for us to be able to make that cell line to study the gene,” he says. The manipulation of embryonic stem cells would permit that closer study. SO TOO MIGHT an astounding research discovery made in Japan. An announcement came in November 2007 that scientists had successfully “re-programmed” an ordinary skin cell to become something akin to an embryonic stem cell. These “induced pluripotent stem cells” seem to be identical to true embryonic cells, and while comparative studies continue, researchers believe they will allow stem-cell technology to advance quickly in the absence of the ethical burden. If they perform like the real thing, then research groups around the world, including Ireland, will be able to exploit the technology to study disease. “This is really a platform knowledge,” says Sullivan. “If you can make every cell type in the body, you can study every disease that affects humans.”

Key International Industry News:

Publication	**Pharmaceutical technology**
Headline	**Big guns give stem cell therapy financial backing**
Gist of the article	Pioneering stem cell therapy, developed by British researchers to tackle the ophthalmic disorder age-related macular degeneration (AMD), has received the financial backing of Big Pharma giant, Pfizer Inc., which is expected to pump in approximately 55.9 million euro during the next 3–5 years. Pfizer Regenerative Medicine will collaborate with the UK's University College London (UCL) to gain a better understanding into how to develop stem cell-based therapies for certain ophthalmic conditions. "We are excited to be working with pioneers in the field of stem cell

ophthalmology from UCL," said Ruth McKernan, Chief Scientific Officer of Pfizer Regenerative Medicine. "While we have much to learn about how stem cells can be used therapeutically, we are confident that this relationship will increase that understanding and help us advance to a time when our work may benefit patients worldwide."

The collaboration brings together the pioneering work of the university researchers in cell-based therapies and Pfizer's expertise in the design and delivery of therapeutics.

What does Pfizer have to offer?
Pfizer's contributions will include expertise in the design and execution of clinical studies and interaction with global regulators, as well as in product manufacturing techniques. The collaboration will examine how human embryonic stem (hES) cells differentiate into retinal pigment epithelium (RPE), with the goal of developing stem cell-based therapies primarily for wet and dry AMD.

Under the terms of the agreement, Pfizer will provide funding to UCL to enable research into the development of stem cell-based therapies for AMD, as well as other retinal diseases. Pfizer is granted exclusive worldwide rights to develop and commercialize an RPE stem cell-based therapeutic in the ophthalmology field. After the completion of preclinical safety studies, Pfizer will have the option to conduct clinical trials to determine efficacy of treatment and commercialize any resulting product. Pfizer already develops and markets medical therapies for ophthalmologic disease and has active efforts in retinal diseases, glaucoma and dry eye syndrome.

"We have not only the benefit of Pfizer's experience of the regulatory process and their expertise in stem cell technology, but the ability, if this works, to produce on a much larger scale. It has huge implications, not only for our project, but for the field of regenerative medicine as a whole," said Professor Pete Coffey, UCL Institute for Ophthalmology and Director of the London Project.

In November 2008, Pfizer launched the Pfizer Regenerative Medicine research unit. This independent research organization will build on the company's experience in this field and recent scientific progress in understanding the biology of stem cells and the opportunity it provides to discover and develop a new generation of medicines for major unmet medical needs. Initial research in this area by Pfizer was focused on the development in drug discovery tools and now expands into developing a new generation of regenerative medicines for major medical needs that could benefit millions of patients worldwide.

The agreement with UCL adds to a range of collaborations and licences with public and private institutes involving experts in this emerging area of biomedical research. These collaborations support

	the evaluation of alternative cell sources, explore the control of stem cells with small molecules and seek to optimize the production of certain cell types for potential use as therapies. Nick Higgins, the CEO of Intercytex Group, a developer of regenerative medicine products that acquired the UK stem cell company Axordia in December 2008, has endorsed the news: "We welcome this significant endorsement by Pfizer of the potential of stem cell research. While there are existing therapies to treat the less common wet form of AMD, there are currently no therapies to treat dry forms of the disease." Intercytex is working with the London Project to Cure Blindness, a collaboration between the UCL's Institute of Ophthalmology, Moorfields Eye Hospital and the Centre for Stem Cell Biology. Through applying regenerative medicine technologies, the project aims to develop a cure for AMD, the leading cause of blindness in the elderly. Dry AMD is known to be associated with defects in the RPE cells. In exchange for research funding, and royalties, Axordia is providing UCL with the embryonic stem cell line (SHEF-1) for differentiation into RPE cells. The London Project to Cure Blindness aims to restore vision by replacing a patient's RPE cells in the macular of the eye with new RPE cells derived from SHEF-1.