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Stem cells: the future of medicine

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Imagine being able to take cells from your skin, transform them into other types of cells, such as lung, brain, heart or muscle cells, and use those to cure your ailments, from diabetes to heart disease or macular degeneration. To realise this, however, challenges still remain, Professor Janet Rossant, a pioneer in the field, says.

All across the world, scientists have begun clinical trials to try and do just that, by making use of the incredible power and versatility of stem cells, which are special cells that can make endless copies of themselves and transform into every other type of cell.

While human embryos contain embryonic stem cells, which help them to develop, the use of those cells has been controversial. The scientists are using induced pluripotent stem cells instead, which are other cells that have been reprogrammed to behave like stem cells.

"There are still significant challenges that we need to overcome, but in the long run we might even be able to create organs from stem cells taken from patients. That would enable rejection-free transplants," said Professor Janet Rossant, a pioneer in the field.

The mouse that changed everything

A speaker at the recent Commonwealth Science Conference 2017 held in Singapore and organised by Britain's Royal Society and Singapore's National Research Foundation, Prof Rossant gave an overview of stem cells' origins, history, uses and potential.

Now a senior scientist at The Hospital for Sick Children (also known as Sick Kids) in Toronto, Canada, after a decade as its chief of research, she was the first scientist to demonstrate the full power of stem cells in mice.

In the early 1990s, scientists believed that stem cells could only become certain types of cells and carry out limited functions. Based on her own research and that of others, however, Prof Rossant believed that they were capable of far more.

Working with other scientists, she created an entire mouse out of stem cells in 1992, upending the conventional wisdom. "We went on to create many baby mice that were completely normal, and completely derived from stem cells grown in a petri dish," she said.

"That was an amazing experiment, and it was instrumental in making people believe that human embryonic stem cells could have the full potential to make every cell type in the body," she added.

When scientists learned how to remove stem cells from human embryos in 1998, however, controversy ensued. Many lobbied against the cells' use in medical research and treatment due to the moral implications of destroying even unwanted embryos to gain the cells.

In Canada, Prof Rossant chaired the working group of the Canadian Institutes of Health Research on Stem Cell Research, establishing guidelines for the field. These guidelines helped to keep the field alive in Canada, and were influential well beyond the country's borders.

In 2006, Japanese researchers succeeded in taking skin cells from adult mice and reprogramming them to behave like embryonic stem cells. These revolutionary, induced pluripotent stem (IPS) cells allowed scientists to sidestep the ongoing controversy.

The challenges in the way

While stem cells have been used for medical treatment in some cases – bone marrow transplants, for example, are a form of stem cell therapy – there are several challenges that need to be overcome before they can be used more widely to treat diseases and injuries.

"We need to get better at turning stem cells into the fully mature cells that you need for therapy. That's going to take more work. Another issue is that of scale-up. If you're going to treat a patient, you need to be able to grow millions of cells," said Prof Rossant.

She added: "Safety is another concern. One of the most exciting things about pluripotent stem cells is that they can divide indefinitely in the culture dish. But that's also one of the most scary things about them, because that's also how cancer works.

"Furthermore, because we need to genetically manipulate cells to get IPS cells, it's very hard to know whether we've got completely normal cells at the end of the day. These are all issues that need to be resolved."

She noted that some scientists are working on making "failsafe" IPS cells, which have a built-in self-destruct option if they become dangerous. "Bringing stem cells into regenerative medicine is going to require interdisciplinary, international collaboration," she said.

In the meantime, stem cells have been a boon to medical research, as scientists can use them to create an endless supply of different cells to study diseases and injuries, and test drugs. "That's the biggest use of IPS cells right now," Prof Rossant said.

Sick kids – and how to help them

At SickKids, which is Canada's largest paediatric research hospital, she has been using stem cells to study cystic fibrosis, a frequently fatal genetic disorder that causes mucus to build up and clog some organs such as the lungs. It affects primarily children and young adults.

SickKids discovered the CFTR gene that, when mutated, causes the disease. It was also the first to produce mature lung cells, from stem cells, that can be used to study the disease and test drugs against it.

Even better, Prof Rossant and her team were able to turn skin cells from cystic fibrosis patients into IPS cells and then into lung cells with the genetic mutation specific to each of them. This is critical to personalising treatment for each patient.

"Drugs for cystic fibrosis are extraordinarily expensive, and patients can have the same mutation and yet respond differently to the same drug," Prof Rossant explained. "With our work, we can make sure that each patient gets the right drug at the right time."

In 1998, Prof Rossant also discovered a new type of stem cell in mice, now called the trophoblast stem cell. These surround an embryo and attach it to the uterine wall, eventually becoming the placenta. She is using such cells to study placenta defects and pregnancy problems.

By using IPS cells to create heart cells and other cells, pharmaceutical companies can also test their new drugs' effectiveness and uncover potential side effects, as well as develop personalised medicines.

"There are still huge amounts of opportunities in pluripotent stem cells," said Prof Rossant, who has won numerous awards for her research, including the Companion of the Order of Canada and the 2016 Friesen International Prize in Health Research.

She is also president and scientific director of the Toronto-based Gairdner Foundation, which recognises outstanding biomedical research worldwide, and a professor at the University of Toronto's molecular genetics, obstetrics and gynaecology departments.

"Meetings like the Commonwealth Science Conference are a fantastic opportunity for scientists to come together, learn about each other's work and establish new relationships, which will help to push science forward, including in stem cell research," she said.

She noted: "The world of science is becoming increasingly interdisciplinary, so this kind of meeting of minds across nations, cultures and scientific fields is really the way of the future."

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