LeapsMag

You Saw the Grammys, But You Missed the More Important Awards

Future Frontiers Kira Peikoff
February 02, 2018

The 2018 Stem Cell Action Awards, honoring recipients for advocacy, leadership, and inspiration, presented by the Regenerative Medicine Foundation. (Courtesy RMF)

Last week in Miami, more than 450 researchers, physicians, lawyers, ethicists, and executives gathered from far-flung corners of the globe to share the latest updates in stem cell research and regenerative medicine. Sure, a science conference might not seem as glamorous as a celebrity-filled Madison Square Garden, but it's the place to be if you care about breakthroughs that could give you a longer and healthier life. Here are our top ten takeaways about what's hot and what's happening worldwide:

"The places you least expect will turn up to produce some really extraordinary things."

1) The future of stem cell treatment may involve the creation of a universal cell line that is genetically modified so every patient's immune system will accept it.

One of the leading scientists at the convention, Japanese stem cell pioneer Dr. Norio Nakatsuji, dubbed this quest a "very hot topic" right now. Being able to produce one safe cell line for everyone would be much cheaper and faster than having to create and grow patient-specific cells. "It is theoretically possible to genetically modify the lines so everyone can accept them," said Nakatsuji. A Seattle-based biotech company aptly named Universal Cells is leading the way in this promising area.

2) Japan was the world leader in stem cell research 10 years ago, but has since fallen behind the United States for reasons that some researchers find frustrating.

Japan is not a particularly religious society, so their culture does not object on principle to using donated human embryos for the creation of stem cells, and federal money can fund such research, unlike in the U.S. But the irony, according to Nakatsuji, is that the regulations for researchers are still very cumbersome. "We need to clear many probably unnecessary steps," he said. For example, before starting work in the field, new graduate students need special training and ethics lectures, and must be cleared by a committee; the process could take six months before an experiment can start, whereas in a country like Britain, scientists can immediately begin.

Also: back in 2006, a Japanese researcher who later won the Nobel Prize managed to reprogram 4 genes in adult cells and essentially turn back time, reversing the cells back to an embryonic state. The implications of this breakthrough were enormous, because destroying an embryo was no longer required to generate blank cells with unlimited potential—and these cells could now be created directly from a patient.

But then "a very unfortunate situation" happened in Japan, says Nakatsuji. There was a fever for these induced pluripotent (iPS) cells, and many Japanese researchers thought embryonic stem cell research was no longer important.

"This is a misconception," Nakatsuji lamented. "You do need both cell types." Embryonic stem cells, unlike their artificially made alternatives, are still safer and more reliable. A symbolic example, he said, is that groups in the U.S. and Europe are starting trials for Parkinson's disease that require dopamine-secreting neurons from stem cells. The researchers could have chosen iPS cells, but went with embryonic stem cells.

The main advantage now of iPS cells, Nakatsuji said, is not for therapeutic purposes, but for drug discovery and creating models of disease based on specific patient profiles.

Dr. Norio Nakatsuji receiving an award for international leadership from Bernard Siegel, the founder and director of the Regenerative Medicine Foundation.

3) In China, rampant stem cell tourism in 2009 led to disaster and a total government shutdown, from which the research field is only recently starting to recover.

Stem cell therapy in China "used to be totally unethical but then took a shock and is still recovering from that shock," said Dr. Wenchun Qu, a physician-researcher at the Mayo Clinic. Scam clinics profited off unapproved and unproven treatments which killed some patients until the total ban set in. Now, the research field is slowly coming back on board under strict regulation; there were only 35 clinical trial with stem cells in 2016, whereas in the U.S, there were more than 2000.

"A lack of public trust and deception is the number one factor" in China's falling behind, said Dr. Yen-Michael Hsu of Weill Cornell. "China is catching up trying to rebuild trust with the taxpayers."

As of last November, 102 designated institutions in China can conduct stem cell research only– not offer commercialized treatments. Bottom line: China is advancing fast in basic science and even leading in some areas, yet is trailing other countries in translational studies and clinical practice.

4) The Bahamas is emerging as a hub of legitimate research that is attracting innovative new trials.

A regulatory framework and National Stem Cell Ethics Committee were established around 2013, and since then, clinical research in the Bahamas has begun; the focus is on safety and efficacy, with standards high enough to satisfy the FDA, but also streamlined enough to allow for trials to proceed faster than they might in other countries.

One U.S.-based company, Advanced Regen Medical Technologies, is pursuing a proprietary cell culture that rejuvenates old cells by exposing them to young donor cells, with the goal of

extending healthy living. On May 24th, 2017, the company presented to the National Stem Cell Ethics Committee, and on December 15th, they treated their first patient.

“Here’s an indication that would be frankly impossible to get through the FDA and certainly not without many years of pain,” said Marc Penn, a leader of the company’s executive team. “We were able to get through the National Stem Cell Ethics Committee with all of us feeling good about the level of rigor within a seven-to-eight month span.”

Desiree Cox, the chairwoman of the Committee, stressed the selectiveness and rigor with which the Bahamas is approaching new trial applications. Of 20 proposed stem cell trials, they have approved only four.

“We’re interested in first-in-man studies, things that are breaking the boundaries, going beyond what is already done elsewhere, linking to predictive analytics,” she said. “The places you least expect will turn up to produce some really extraordinary things.”

Another active clinical trial there is a phase 1 study for Aging Frailty run by a Miami-based start-up called Longeveron. “Our experience is it comes as a huge relief to many people to have the opportunity to go to such a program rather than wait for a drug to be approved in the U.S.,” said Dr. Joshua Hare, the director of the Interdisciplinary Stem Cell Institute at the University of Miami and the co-founder and Chief Science Officer at Longeveron.

“The challenge right now is the effective translation and development of viable stem-cell based therapies."

5) Researchers are working on building an artificial heart with stem cells, but technology is not the only hurdle.

A group at the Texas Heart Institute in Houston is experimenting with this strategy: stripping a real heart organ of its cells, then repopulating it with blood-forming stem cells, and implanting it. In cows, this approach has worked successfully. But one problem, said Dr. Doris Taylor, the director of Regenerative Medicine Research at the Institute, is educating regulators, since this kind of treatment is not a drug and not a device.

That said, when will we see someone order a heart off the shelf?

“I think in the next two years,” she said, “you will see exciting things happening at least at the level of congenital heart disease, if not adult hearts.”

6) Cost is a major barrier to regenerative medicine’s success.

“It’s not about whether you can get enough of the cells you need, it’s about whether you can get them for less than one million dollars,” Taylor said wryly.

Cell therapies intended for patients must be manufactured in a special facility to generate the quantity necessary for treatment. Some experts expressed concerned that these bio-manufacturing facilities are like “the Wild West” right now because there is no standard for pricing.

Some companies are “getting away with murder,” said Dr. Camillo Ricordi, director of the Diabetes Research Institute. “This doesn’t happen in most of the rest of the world.”

7) Media hype has caused the premature (and potentially dangerous) commercialization of unproven stem cell therapies.

There are now over 570 such clinics operating in the U.S., with hot spots in Florida and California, which offer up stem cells for everything from sports medicine and vitamins to beauty products and pet health.

In fact, according to the FDA, the only stem cell-based products currently approved for use consist of blood-forming stem cells derived from cord blood. Everything else, for now, is still experimental.

While plenty of legitimate research is moving ahead in clinical trials, consumers may be confused by the plethora of scam clinics. But since last August, the FDA has begun cracking down, issuing three enforcement actions.

Also worth noting: what the marketplace refers to as “stem cells” are in fact products that contain a very low amount of concentrated adult stem cells derived from fat or bone marrow. There are no *pure* stem cell products out there.

“The challenge right now is the effective translation and development of viable stem-cell based therapies,” said Dr. Shane Shapiro, a sports medicine physician at the Mayo Clinic.

What constitutes a genetically modified organism? Europe is in the process of deciding.

8) An exciting coming trend is induced tissue regeneration.

The company AgeX, run by gerontologist and stem cell pioneer Dr. Mike West, is in preclinical trials for a treatment that can reset the regenerative potential of mature tissue.

This ability is lost in the early stages of life to help prevent cancer, but AgeX is interested in figuring out a way to restore it with pluripotent stem cells in adult tissue, to correct the damage incurred by aging. West said he expects the program to reach human clinical trials in the next five years.

9) Stem cells alone are not the whole story.

The future of cell therapy will involve cell derivatives—the things that cells secrete, like exosomes, microRNA, and viruses, that can be better controlled than the cells themselves.

Exosomes, which are extracellular vesicles released from cells, act as fingerprints that are useful for diagnosis and therapy, said Dr. Li Chen, the head of the Human Liver Cell Lab at the University of California-San Diego. Because exosomes are smaller than cells, they can also cross the blood-brain barrier.

Europe is the leading place for exosome research. Recently, a 21-year-old boy suffering from brain cancer there was treated with stem cell therapy, which failed, but then subsequently he received surgery with exosomes applied to his tumor, and he survived.

10) The European Union is in the process of deciding what legally constitutes a "genetically modified organism" – and the stakes are high.

The European Court of Justice, the EU's highest court, is considering this question: If a modification brought about by genetic engineering technology could also have occurred naturally, should the resulting organism be considered a GMO?

Just last week, an advocate general of the court proposed that whenever an organism is manmade that *could* theoretically occur naturally, it should not be considered a GMO, and therefore should not be subjected to such regulations.

If the Court agrees with the advice of its advocate general later this year, then the decision would have huge implications for biotech agriculture across Europe, paving the way for gene-edited crops to hit the market.

Kira Peikoff

Editor-in-Chief

Kira Peikoff is a journalist whose work has appeared in the New York Times, Newsweek, Nautilus, Popular Mechanics, The New York Academy of Sciences, the Hastings Center Report, and other outlets. She is also the author of three suspense novels that explore controversial issues arising from scientific innovation: Living Proof, No Time to Die, and Die Again Tomorrow. Peikoff holds a B.A. in Journalism from New York University and an M.S. in Bioethics from Columbia University. She lives in New Jersey with her husband and son.
view full profile