Korea okays stem cell therapies despite limited peer-reviewed data

South Korea is positioning itself in the vanguard of stem cell commercialization, with three adult stem cell treatments approved in the country over the past eight months. Yet, although officials inside the Korea Food and Drug Administration (KFDA) maintain that the therapies have been vetted according to international best standards, outside experts worry that the country's drug agency may be moving too quickly with its regulatory decisions.

"It took a lot of people by surprise that the KFDA was willing to approve these ahead of any country in the world," says Douglas Sipp, a stem cell policy researcher at Japan's RIKEN Center for Developmental Biology in Kobe. "There has not been a lot of information released about the details of these studies or the regulatory approval process in English."

The first approval came in July 2011 when the KFDA approved Hearticellgram-AMI for treating heart attacks. Developed by Seoul-based Pharmicell, the therapy consists of mesenchymal stem cells cultured from a patient's own bone marrow that are injected into the coronary artery to improve heart function. Traditional transplants of blood-forming stem cells from bone marrow and peripheral blood are regulated under separate terms from other human cell and tissue products in many countries, and so Hearticellgram is the first stem cell 'product' approved by a regulatory authority anywhere in the world. (The second approval came just four months later when the US Food and Drug Administration legalized its first stem cell product, a cord blood stem cell–based therapy called Hemacord, from the non-profit New York Blood Center.)

The KFDA then forged ahead in January with stem cell go-aheads for two more Seoul-based companies: one for Medipost's Cartistem, which uses banked umbilical cord blood stem cells to help regenerate knee cartilage for people undergoing orthopedic surgery and the other for Anterogen's Cupistem, an injection of fat stem cells derived from a patient's own abdomen or thigh that is intended to help people with Crohn's disease who suffer from painful anal fistulas.

Notably, Hearticellgram-AMI and Cartistem are the first licensed stem cell treatments that use bone marrow– or cord blood–derived stem cells to treat diseases outside of the blood and immune systems. Yet, some researchers worry about the efficacy of products of this sort, given the lackluster data released publically to date—and what is known about these agents is mostly through press releases by the KFDA and presentations, not peer-reviewed journals.

Cartistem's results look the most promising. In an 89-person Korean clinical trial, 26% more people who received the Medipost treatment experienced an improvement in knee function on a widely used cartilage repair assessment scale compared with those who underwent knee surgery alone. Meanwhile, a 59-person trial with Hearticellgram-AMI found that people who received the stem cell product could eject around 6% more blood out of their left ventricles at the end of the six-month study, compared with just 2% improvements in people who received standard medical therapy.

Show me the data

Company officials say that peer-reviewed results are coming soon. According to Francis Han, Medipost's head of research and development strategy, "the doctors running the clinical trials are now gathering more long-term follow-up data on the patients for publication." Similarly, Anterogen spokesperson Kim In-ok says that the company is in the process of preparing a journal article based on findings presented at last year's European Society of Coloproctology annual meeting in Copenhagen.

The KFDA, for its part, stands by its record. Ahn Gwang-soo, deputy director of the agency's cell and gene therapy products division, points to the strict procedures in place to ensure that medicines are safe and effective. "Although there were some companies that conduct stem cell treatments outside of Korea," he says, "they are not permitted to administer those treatments within Korea."

D Yvette Wohn

Corrected after print 22 May and 21 June 2012.

Survey says: too many PhDs

The biomedical workforce is increasingly plagued by job shortages for young scientists, according to a January report—the first public offering from a working group created last year by the US National Institutes of Health (NIH) to study strategies for maintaining a sustainable pool of researchers in the country.

The report compiled information from an online survey conducted last year that asked participants to rate the importance of eight workforce issues and invited them to include comments. Around half of the 219 primary investigators, institutional administrators and research trainees who responded identified 'supply and demand' as one of the most pressing concerns, with issues surrounding the characteristics of PhD and post-doctoral training programs not far behind.

Now that the problems have been flagged, attention is shifting to finding potential solutions. Creating more jobs to tackle the demand side of the equation may be difficult, however. "NIH budgets haven't been growing, and with government austerity measures now, the prospects for future spending increases don't seem very encouraging," notes economist Paula Stephan, who studies trends in the biomedical workforce at Georgia State University in Atlanta. Indeed, US President Barack Obama's budget request for fiscal year 2013, released on 13 February, proposed to keep the NIH's budget level at $30.7 billion.

So, rather than relying on increased funding for the NIH, the working group is considering suggestions that include reducing the number of training grants for PhD students and post-doctoral fellows, creating more programs for biomedical master's degrees rather than full-blown doctorates and providing preparation for jobs outside tenure-track academic research, according to the panel's co-chair Sally Rockey, who is also the NIH's deputy director for extramural research. But, at this point, the panel is still gathering further data on training and career options. "We would like to know as much information as possible about the potential careers open to physician-scientists and PhDs," Rockey says.

Researchers, government officials and analysts all agree: something needs to change if the biomedical workforce is to remain a viable and attractive career option. "We need to rethink the approach we take to research in order to create a better balance between training and productivity," says Howard Garrison, deputy executive director for policy at the Federation of American Societies for Experimental Biology, a Washington, DC–based advocacy group. "Everyone is looking forward to seeing the NIH recommendations." The working group is scheduled to present its full set of proposals to the NIH director's advisory committee in June.

Rebecca Hersher

Corrections

In the March 2012 issue, the article entitled "Korea okays stem cell therapies despite limited peer-review data" (*Nat. Med.* **18**, 329, 2012), failed to convey that the 26% improvement in knee function was an additional gain compared to the control group. The sentence should have read: "In an 89-person Korean clinical trial, 26% more people who received the Medipost treatment experienced an improvement in knee function on a widely used cartilage repair assessment scale compared with those who underwent knee surgery alone." The piece also referred to Francis Han by her Korean name, Han Sung-ho, and did not specify that she is in charge of research and development strategy. The error has been corrected in the HTML and PDF versions of the article. Additionally, as a clarification, the cells referred to as 'banked' in the piece are frozen-stored and do not require donor-recipient matching.

In the March 2012 issue, the article entitled "Korea okays stem cell therapies despite limited peer-review data" (*Nat. Med.* **18**, 329, 2012) incorrectly stated that the Hearticellgram-AMI therapy results related to 80 individuals. In fact, the number is 59, and the difference in left ventricular ejection fraction changes between the treatment and control group was statistically significant. Additionally, the information about this therapy was available through poster presentations, not exclusively through press releases by the Korea Food and Drug Administration, as originally stated. The errors have been corrected in the HTML and PDF versions of the article.