Japan's venture in regulating regenerative medicines

Original publiction:
Accelerating Regenerative Medicines and Cellular Therapies: The Japanese Experiment in Ethics and Regulation
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The regulations on stem cells and regenerative medicines that Japan passed in 2013 were intended to increase patients' access to promising new medical treatments. Economic, safety and ethical issues are raised by the creation of a legal pathway for public and private institutions to use unapproved regenerative medicine treatments.

What questions & challenges are raised?

For years researchers have discussed the potential for stem cells (SCs) and regenerative medicines (RMs) to deliver a profoundly new way to treat and potentially cure injuries and disease. The clinical trial process for testing new drugs and treatments examines both safety and efficacy, but for many this process is perceived as too slow and laborious. To meet the demands of patients, clinicians and biomedical companies, many countries are considering or have already implemented new regulations to allow RMs to be given to patients - even if treatments have not yet been proven to work. Japan passed two laws in 2013 in an effort to promote RM research and address the growing demand for access to SC treatments. Dr Tamra Lysaght from the National University of Singapore identifies several ethical and economic issues of Japan's new RM regulation in her paper "Impacts of Japan's Framework for Regenerative Medicine". Dr Lysaght points out how frameworks of this kind are vulnerable to exploitation, give credibility to treatments with unproven efficacy and can potentially damage the reputation of SC research and RMs in Japan and around the globe.

What insight & direction does this give for research policies?

Emerging RMs are often labour intensive and costly. Dr Lysaght point out that policies like what Japan has adopted give businesses the opportunity to shift financial risks onto health care providers and patients. It is important for the long-term success of government health programs and private health insurers that costs of RMs are appropriately evaluated against benefits backed by scientific evidence. Government healthcare systems that provide ineffective treatments would further strain their limited finances and unjustly distribute money away from supplying proven treatments to citizens. Dr Lysaght points out that regulators should strictly enforce time-frames that clinically unapproved treatments are permitted and quickly remove treatments that fail to show benefits from the market. Another important consideration raise by Dr Lysaght is that RM treatments that are able to go through the standard clinical trial process should do so unless there are substantial reasons why such trials are not realistic. (Such reasons might include a limited numbers of patients due to disease rarity.) Finally, a greater global effort should be made to protect consumers from unsubstantiated claims by businesses offering SC treatments. This could be done by implementing and enforcing international standards for SC products (which could be developed with guidance from organisations like the World Health Organisation and World Medical Association) and prohibiting SC treatments to patients outside of clinical trials (except under exceptional circumstances) and in line with guidelines set out by the International Society for Stem Cell Research.

What background and point are discussed?

Japan's 2013 regulations create two legal pathways for using non-clinically approved SC and RM treatments in public and private medical centres. One pathway permits academic and industry sponsors of RM treatments to put products on the market for a seven-year period while clinical trials are underway to collect safety and efficacy data. Under this pathway, sponsors may also be able to have the costs of providing their treatment paid for by Japan's National Health Insurance (NHI). The other pathway permits RM treatments to be provided through private practice or in the context of clinical research, as long as safety risks are evaluated and pass review by certified RM committees (and the Health Sciences Council in 'high-risk' cases). Dr Lysaght points out that these policies appear to place a disproportionate emphasis on safety over clinical benefit. For example, two RM treatments have been granted listing on the NHI Drug Price List, yet the effectiveness of these treatments were studied on insufficiently small group sizes (only 7 and 25 individuals). Effectively, patients and the NHI could be paying lots of money for treatments that may be safe but don't have any benefits. Dr Lysaght also presents a strong argument that allowing unproven treatments on the market lessens the incentive for sponsors to run well-designed clinical trials. Studies in the US show that once treatments are on the market, testing their safety and effectiveness is often delayed or doesn't happen at all. Additionally, once companies are allowed to market products, they are typically slow to stop selling them if they are shown to be ineffective or unsafe. While Japan's new policies provide some level of monitoring the safety of SC treatments being offered in private medical practice, they do not regulate the price or advertising methods. This presents businesses with the opportunity to make misleading claims regarding the benefits and safety of SC treatments, a practice that occurs even in countries with strict customer protection systems, like the US. Additionally, legalising un-proven treatments can give the impression of credibility to treatments when there may be no significant scientific proof they work. Whether intentional or not, businesses may mislead patients about treatments, ultimately undermining the public's trust in legitimate RM research as well as science and medicine at large.