Yuzo Toda – Chairman, Forum for Innovative Regenerative Medicine (FIRM), Japan



FIRM acts as a form of translational bridge between academia and the market, bringing the results of academia to the patient

27.11.2017

Tags: Japan, FIRM, Fujifilm, Association, Regenerative Medicine, R&D, Innovation, Funding

Yuzo Toda, chairman of the Forum for Innovative Regenerative Medicine (FIRM) in Japan, discusses how the organization acts as a translational bridge between academia and industry, how the paradigm of regenerative medicine differs from that of traditional pharmaceuticals, and why Japan has taken the lead in this exciting new field.

What is FIRM's *raison d'être*, mandate and main objectives?

FIRM is a private association active in the business strategy, regulatory approval, manufacturing, and marketing of regenerative medicine. Our mission is to pave the way for real, practical breakthroughs in regenerative medicine by pursuing broad, industry-led partnerships with governments, universities, and the private sector. We also engage closely with the media, for the purpose of building a greater consensus on the commercialization process and the application of new regenerative medicine techniques in the community. FIRM acts as a form of translational bridge between academia and the market, bringing the results of academia to the patient.

How would you characterize FIRM's membership profile and how does a regenerative medicine association's member profile differ from that of a traditional pharma

association?

These are very much two altogether different industries. The predominant paradigm in the pharmaceutical industry is palliative care. Of course, new drugs are being developed and new scientific ideas are being implemented, but most of the time, the pharmaceutical industry aims to attack the symptoms rather than the base mechanism of the disease.

However, in regenerative medicine, we harness a different approach. We require a lot of technical know-how and a many supporting industries; not only cell scientists. Therefore, FIRM possesses a wide variety of members from pharma, biotech and other associated, supporting and auxiliary industries including the machinery, chemicals, reactants and even transportation, insurance and banking sectors.

In pharmaceuticals, the value chain is already very well established and mature. However, as regenerative medicine is a very new field, each piece needs to be built up from scratch.

Overseas, the Japanese regulatory system for regenerative medicine is regarded as very advanced. How has this system evolved?

The Japanese regulatory framework for regenerative medicine has a long heritage and, though it may seem simple, is actually very scientific. In traditional pharmaceutical drug approval, patients, once diagnosed, have their diversity more or less denied. Once you are diagnosed by a doctor, your treatment then starts with no variation from that point onward. Your medication is the same, regardless of whether you are younger, older, bigger or smaller. Scientifically that is very strange.

In regenerative medicine, we take a different approach. The human body is a kind of cell processing system that differs dramatically from person to person. Therefore, the traditional pharma approval system is not applicable and this is why we have implemented a conditional approval system; the most important part of the new regulatory framework. This system takes into account people’s diversity. Conditional approval is the main tenet of the system, followed by safety, efficacy, and speed.

After that, we have a post-marketing data collection system that depends on the disease or condition; we make a contract with the government showing how many patients are receiving treatment and how diverse they are. After a certain period of time, if we can show evidence of efficacy, then we will get full approval. In principle, the system is the right one but we are still in a learning process.

The samples of real life evidence in regenerative medicine are very small. How do you go about building cases with such small pools of evidence?

You are right in saying that there is not a lot of evidence, but there are more than 60 projects in the new Japanese regulation system. In that sense, within one or two years, a lot of visible results will come.

The capacity to finance projects is important. Who is the financial muscle behind regenerative medicine in Japan?

Within Japan, there is a reluctance to fund sectors that are potentially risky. Of course, for a new industry such as regenerative medicine that is a formidable problem. To mitigate this, we have already started programs to foster venture capital investment into our industry; organizing 'crossroad' meetings to introduce venture capitalists from both inside Japan and abroad to our members.

We also hope that Japan's advanced regulatory system for regenerative medicine will act as an incentive for investment into the industry and make doing business easier.

Large Japanese enterprises like Fujifilm [where Mr. Toda is CTO and senior executive vice president – Ed.], can also supply both money and technology. This is not a simple industry, so we need the different types of technology that large enterprises are able to supply.

Furthermore, Japanese banks are also becoming interested in funding regenerative medicine.

Regenerative medicine, however, by its very nature, must remain local. Is there therefore another discrepancy here, between being at the forefront of innovation and having extremely local applications?

I see regenerative medicine internationalizing. Of course, in principle, it is true that regenerative medicine is a 'locally manufactured, locally consumed' industry. However, a cell is a living thing and bringing it into the global system, will trigger new technological development. Consequently, in that sense, I am rather optimistic.

There are, of course, moral and ethical issues around stem cells. What is the general Japanese view on this topic?

The view in Japan is similar to that in Europe and the US. As always with new science and new technology, people are unsure as to how it fits in the existing social system. It is just a matter of time. If people understand that the technology is profitable or meaningful for society, mindsets will change.

The economic impact of medication is extremely serious. Thus, there is not only a ‘push’ from academic results, but also a social ‘pull’ on how to solve medical problems. The existing medication paradigm is no longer valid for the current needs of society.

I do however understand the ethical issues and we must be very careful or we will miss a great opportunity.

For regenerative medicine to become affordable, who will the payer be in the mid-term?

This is a big question for the reimbursement system. We have to shift our mindset from the pharmaceutical payment system. In the pharma industry, once prices are set, that is the official price. This system is not valid for regenerative medicine. We have already started some discussions to implement an industrial product price setting system. For instance, in the beginning stage, prototypes were prohibitively expensive, but, depending on the sales volume, the price decreased. This is still a very topical discussion point because if we do not modify the status quo and define new price reimbursement methodologies, we cannot easily bring these sorts of products onto the market. If you take the example of industrial products, in the beginning upfront costs are also high. Venture capital needs this money to be invested in order for them to break even. We need to consider lessons learned from other industries and sectors. There is a case for saying that the state should cover the shortfall to get some of these therapies off the ground initially.

The capacity to pay will be very important in terms of implementation. There are various models in the world: single payer, multiple payers. In the past, the multiple payer approach has been more successful in terms of technology adoption. For regenerative medicine, what will be the faster track?

Multiple payers. It is more flexible.

There is no doubt that Japan is the global leader in regenerative medicine. What role does Japan want to play in this field moving forward?

We need to know more about the function of the cell itself. Before coming to preventive medicine, we need early diagnostic systems. This is a really a scientific challenge. Without an early detection system, we have to diagnose in advance before the disease. Then if we can get some direction, we need some guidelines on how to live life to prevent disease.

We also need a less invasive method where, once a patient is diagnosed, if they live according to a particular lifestyle, they can take preventive medication. Such a preventive approach will become more important.

> People should not misunderstand; this is a new economy. The existing pharmaceutical industry paradigm is not applicable here

Japan will be at the forefront of this. All the diseases we try and cure, such as cancer and CNS diseases are not simple. People should not misunderstand; this is a new economy. The existing pharmaceutical industry paradigm is not applicable here. This is a big opportunity for a new industry to be created.

What is your final message to our international audience?

Regenerative medicine is one name of the application. The central idea is cell science based medicine. Around this core idea, cell therapy, regenerative medicine, tissue engineering, cures, drug discovery and drug development will be revolutionarily changed. It is very difficult to utilize an automatic system for this new field, so we will see the emergence of a new system, with control dispersed throughout. For instance, we can make a cell robot, heart robots, or kidney robots that can be utilized both for treatments as well as for drug discovery and development.

See more interviews