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We are confident that 2020 and 2021 will be very relevant years for regenerative medicine in Japan

15.09.2020

Tags:

Japan, Regenerative Medicine, FIRM, Cell & Gene Therapy, Association, Asia-Pacific

Dr Ken-ichiro Hata, representative director and chairperson of Japanâ??s Forum for Innovative Regenerative Medicine (FIRM) outlines the organisationâ??s mandate, how Japan has been able to cultivate one of the worldâ??s most mature regenerative medicine ecosystems, and highlights key challenges including regulatory misalignment across Asia and the need for better manufacturing and commercialisation models.

Dr Hata, having been appointed the Chairperson of the Forum for Innovative Regenerative Medicine (FIRM) in 2019, could you introduce yourself briefly and share your mandate for the organisation moving forward?

My journey has been a very interesting and diverse one. I trained as a surgeon and started my career at the Nagoya University of Medicine as well as the Nagoya University Hospital. In 2004, I joined a regenerative medicine company, Japan Tissue Engineering (J-TEC) as Director of R&D, which was acquired by the FUJIFILM Group in 2014. In 2015, I became GM of FUJIFILMâ??s Regenerative Medicine Research Laboratories, and then in March 2016, GM of the Regenerative Medicine Business Division. Since November 2017, I have also been President and CEO of J-TEC. This unusual path has given me exposure, insights and clarity not only on academic work and

research but also industrial R&D, clinical development as well as the journey of a product from bench to market, including experience with the Japanese regulator, the Pharmaceuticals and Medical Devices Agency (PMDA).

As for FIRM, our objective remains unchanged: to foster the development of the regenerative medicine sector as a fully-fledged industry. This is why we have a vast membership of over 250 companies and corporations across many different niches, from pharmaceuticals and life sciences to logistics and manufacturing. I am also grateful that in our leadership team, we now have Dr Yoshitsugu Shitaka, Corporate Executive of Astellas Pharma and the President of the Astellas Institute of Regenerative Medicine (AIRM) as our Vice-Chairperson and Representative Director, which brings us closer to Big Pharma companies.

We want FIRM to be a platform where our members can gather their different technologies and expertise to deliver on the potential of regenerative medicine. My desire is to bring forth a new â??joyâ?• through regenerative medicine.

During my term as Chairperson, I hope that we will be able to firstly, achieve more collaboration with academia and researchers, and secondly, to support more bioventures and start-ups in developing and commercializing their products from the R&D stage to the market approval stage.

Japan is one of the countries with one of the most mature regenerative medicine ecosystems in the world, and FIRM has been a pioneer in this space. How do you evaluate the progress that has been made so far?

We are very pleased with the support that the Japanese government and the Japanese regulator, PMDA, have given to the area of regenerative medicine, particularly following the awarding of the Nobel Prize in Physiology or Medicine to Dr Shinya Yamanaka and his colleagues at Kyoto University for their research in induced pluripotent stem (iPS) cells in 2012. This brought a lot of attention to the important work being done in this space in Japan, both within Japan and internationally.

In 2014, the Ministry of Health, Labor and Welfare (MHLW) implemented the â??Strategy of SAKIGAKEâ??, as one of the action plans to implement â??Healthcare and Medical Strategiesâ?• adopted in 2013, consisting of the following two key measures:

â?? SAKIGAKE Designation System: promoting R&D in Japan aiming at early practical application for innovative pharmaceutical products, medical devices, and regenerative medicines.

â?? Scheme for Rapid Authorization of Unapproved Drugs: accelerating the practical application of unapproved/off-label use of drugs for serious and life-threatening diseases by expanding the scope of the Council on Unapproved Drugs/Off-label Use to include unapproved Western countries if it satisfies certain conditions and by improving the environment for companies to undertake the development of such drugs.

This strategy has been quite effective. In 2014, two important pieces of legislation relating to regenerative medicine were also enacted: the Act on the Safety of Regenerative Medicine (ASRM) and the revised Pharmaceutical Affairs Act (PMD Act). In particular, the revised PMD Act implemented the conditional and time-limited marketing authorization system for regenerative medicine products, if they meet a number of conditions, including the lack of any major safety concerns and the â??suggestiveâ?• findings of efficacy, can be sold on the market for up to seven years. This means that we no longer need to recruit large numbers of patients for clinical trials, which

can be very challenging. Following this conditional and time-limited approval, the safety and efficacy of the product need to be confirmed continuously in conjunction with postmarketing safety measures.

These measures position the Japanese ecosystem for regenerative medicine ahead of international competition. Today, Japan has nine regenerative medicine products on the market, and three of them are conditionally approved, which is very positive.

However, in terms of areas for improvement, from the regulator standpoint, we would like to see more flexibility in the evaluations of regenerative medicine therapies, particularly when it comes to â??efficacyâ??. Our experience through the various regulatory approval processes has indicated that demonstrating the safety of these therapies in clinical trials is feasible but demonstrating a standardized or consistent level of efficacy across patients is not necessarily possible, due to the heterogeneous nature of the therapies. We hope to work with the regulator to address the topic of â??efficacyâ?? through other approaches.

Ultimately, we would like to increase the available therapeutic indications for both existing and also upcoming products. The market size of these products is currently still rather limited. In order to cater to larger patient populations and address the significant unmet medical needs that still exist, we need to expand the reach of regenerative medicine products. From the industry standpoint, this also means we need to invest in more clinical trials.

Looking at cell and gene therapies, there are two major barriers to widespread adoption and utilization: manufacturing scale-up and commercialization models. What is your perspective on these two challenges?

These are both very important topics for the industry. In Japan, the regenerative medicine industry has developed around the concept of autologous regenerative therapy, i.e. using stem cells taken from the patientâ??s own body, as opposed to allogeneic regenerative therapy, i.e. using stem cells from donors. The approval pathways are perhaps faster for autologous therapies but the manufacturing, supply chain and commercial models are different.

The single-payer system in Japan has not resulted into a large market structure thus far but I am not sure how a multiple-payer approach will improve the way regenerative medicine therapies can be developed and brought to market, especially with regard to pricing.

Other Asian countries, especially South Korea and China, are also advancing in the field of regenerative medicine but there is a clear regulatory misalignment between various Asian countries that hinders regional collaborations. How can this be better addressed?

We must admit that we have not sufficiently promoted awareness of our achievements in the regenerative medicine space on the global scale but moving forward, we would like to focus on strengthening our collaborations with other entities within the Asia-Pacific region. This is not always straightforward because other countries also have their own agendas and priorities. Regulatory alignment is also a complex endeavor. However, we are happy to see that foreign companies have

found many opportunities and channels to come to Japan with work with our academics, researchers and industry members, which demonstrates that Japan is a leader in regenerative medicine.

What can we look forward to from FIRM and the Japanese regenerative medicine industry in the next couple of years?

Moving forward, we want to expand the range of therapeutic indications and launch more products successfully on the market, as well as to improve the industrialization and manufacturing of such therapeutics, to support these therapies becoming more mainstream solutions for patients in Japan and globally. We will also strive to form more fruitful collaborations with relevant stakeholders within Japan and internationally. We are confident that 2020 and 2021 will be very relevant years for regenerative medicine in Japan.

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