Recommendations for "Creating a Sustainable Ecosystem for Innovative Regenerative Medical Products"

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White Paper
Appendix

Summary

Regenerative medical products are those made by culturing or similar processing through the use of human or animal cells and tissues. They are used for reconstructing, repairing, and forming the structure and functions of the human body, treating and preventing diseases, and for gene therapy. These products offer new treatment possibilities for conditions that are difficult to cure with conventional methods and have the potential to significantly improve patient's quality of life.

Research and development of regenerative medical products in Japan are steadily progressing with the support of government policy and collaboration between industry and academia. However, compared to the global market, the Japan market has not grown sufficiently, and many of the products approved in Europe and the United States have not been developed in Japan. In order to overcome this stagnation of industrialization, more active investment in technology development and regulatory reforms are required.

This white paper focuses on CAR-T cell therapy and identifies specific challenges in the research, development, manufacturing and supply, and treatment of regenerative medical products, as well as in the healthcare insurance system that supports this entire process. The reasons for this focus include the significant clinical and societal value of CAR-T cell therapy, the complexity of its manufacturing and quality control process, given that it's a personalized medicine, and its approval and industrialization in Japan, the U.S., and Europe alongside other regenerative medical products.

CAR-T cell therapy [1]is a treatment that has the ability to harvest T cells from patients, genetically modify them, and attack cancer, and has been put into practical use in leukemia and lymphoma, and is expected to be a treatment that can aim for a cure with a single dose. However, in Japan, where there is no sufficient pricing system and medical fee system to evaluate the advanced and complex process of CAR-T cell therapy, sufficient investment in new research and development is currently underway. Furthermore, since the number of countries and regions that can produce CAR-T cells is limited, there are concerns that Japan will face the issue of drug lag and drug loss from the perspective of manufacturing priorities.

In order to improve this situation and develop regenerative medical products sustainably, it is important to build an ecosystem where more patients can receive innovative medical care. In formulating this white paper, we will deepen discussions with experts on the medical economy, technology development of regenerative medical products, and medical treatment, clarify the barriers to the practical application of regenerative medical products and the formation of an ecosystem that supports them, and publish this white paper as a proposal for solutions.

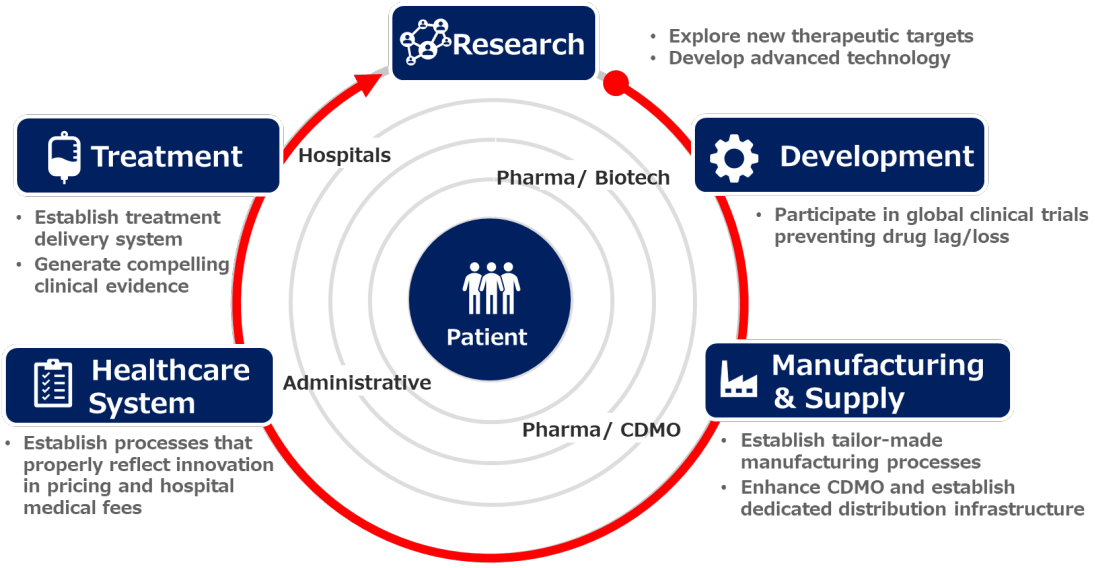
The need to build an ecosystem for regenerative medicinal products

Regenerative medical products are groundbreaking technologies that offer innovative treatments, but there are numerous challenges that need to be overcome from the research stage through to actual patient treatment. For example, during the research and development phase, it is necessary to address various issues related to cell and tissue processing. Additionally, it is essential to establish a specialized system for the stable supply of these products, one which requires unique processes such as cell culture and handling. Once a stable supply system is established, these products must undergo evaluation by various regulatory authorities to ensure that they can be provided by medical institutions.

To sustainably operate this entire process as an "ecosystem," it is essential to reinvest the outcomes from actual clinical practice, as well as the insights and revenue gained, into the research, development, and manufacturing of the next cycle. This enables the development of next-generation products. Ensuring this ecosystem cycle operates sustainably will make it more likely that innovative medical treatments can be delivered to more patients, while simultaneously

[1] CAR-T cell therapy: A completely personalized treatment method in which T cells, a type of lymphocyte from a patient, are collected and reprogrammed to have the function technology, and the cells are proliferated and administered to the patient himself.

achieving growth of the sector and advancements in medical care.



Benefits of establishing an ecosystem of regenerative medical products

Benefits for Japan's healthcare environment

Producing regenerative medical products domestically for Japanese patients can address global manufacturing and storage limitations, ensuring a sustainable supply. This will bring many benefits to patients and their families. Since a single dose can provide a high therapeutic effect, it is expected that patients who have required long-term hospitalization can return to society as soon as possible, improve their quality of life, increase treatment options, and reduce physical, mental, and economic burdens. Moreover, it is expected to meet the current demand as well as the therapeutic needs of various diseases such as autoimmune diseases in the future.

Benefits for human resource activity

The spread of regenerative medical products can create a place for highly skilled human resources related to biotechnology in Japan to play an active role in the fields of R&D and manufacturing. It can alleviate the burden on healthcare workers, allowing them to focus on treating other diseases, and enable patients who have been cured of severe illnesses—and their families—to become active again, contributing immeasurably to society and the economy.

Benefits for international competitiveness

Technologies related to regenerative medical products have the potential to become the foundation of Japan's biotechnology industry, leading to the acceleration of research and development starting in Japan. This is expected to develop into opportunities for international leadership, expanding Japan's influence in Asia and globally.

Actions and Policies Necessary for the Dissemination and Sustainability of Regenerative Medical Products

Recommendation 1:

Reform of the pricing system and medical fee system to promote the spread of regenerative medical products

Japan's healthcare insurance system operates on a principle of universal health coverage, providing an environment conducive to receiving advanced treatments. By keeping prices low, Japan facilitates rapid and extensive insurance reimbursement, whereas in Europe, high pricing set by companies often results in delayed reimbursement due to cost-effectiveness evaluations. However, setting prices too low could reduce the incentive for companies to invest in research and development, potentially leading to drug lags or losses. Implementing systemic reforms that reflect the innovation and characteristics of regenerative medical products could position Japan as a global leader in this field, thereby delivering the benefits of treatment to more patients.

Ensuring sustainable access to regenerative medical products requires comprehensive reform of the healthcare insurance system. First, it is necessary to review pricing to properly assess innovation and reconsider market expansion repricing[2]. Additionally, in order to utilize limited medical resources efficiently, strategies to reduce medical costs, such as curbing excessive medication usage and ensuring appropriate use, should also be considered.

- **Adequate reflection of the usability rating in the price**

Regenerative medical products not only offer clinical value over long periods but are also a benefit to society by aiding patient reintegration into society and reducing the burden on caregivers. It is important to consider how these contributions over an extended timeframe can be reflected in the form of adjustments to premiums

[2] Market expansion recalculation: A system to reduce drug prices for drugs whose annual sales value has increased relative to the expected sales amount set by the indications and effects at the time of drug price listing.

within the healthcare insurance system. Furthermore, using this system as an incentive, it is necessary to promote the collection and analysis of real-world clinical data and reverse translational research[3] for verification.

- **Revision of the application of the market expansion repricing**

Regenerative medical products undergo a complex and personalized manufacturing and distribution process, which requires a lot of money for highly specialized personnel, intellectual property, facilities and equipment, special quality control, transportation, and post-manufacturing operations. Additionally, these products are individually manufactured for each patient, lacking economies of scale, and making cost reduction through mass production difficult.

Applying market expansion repricing, which assumes economies of scale, could severely impact company revenues, and hinder continued patient access. Therefore, reviewing current market expansion repricing rules, which assume economies of scale for conventional pharmaceuticals, is necessary. At the very least, establishing a mechanism that prevents excessive price reductions during scope expansions would help maintain an environment where companies can supply innovative treatments in a sustainable manner.

- **Improvement of the medical fee system to properly reflect medical costs**

To enable the sustainable provision of regenerative medical products, support for facility investment and human resource development at medical institutions is crucial. Thus, when setting reimbursement rates, it is necessary to evaluate the costs of medical provision systems, including the personnel and facilities required for the provision of regenerative medical products, sufficiently within reimbursement rates. Additionally, it is important to incorporate systems that allow medical institutions to recoup their investment in facilities and personnel.

Recommendation 2:

[3] Reverse translational research: A research method that feeds back problems and questions observed in clinical practice to basic research and obtain new discoveries and findings.

Review Ecosystem Challenges and Continue with Policies that will Enhance Next-Generation Cycles

To develop an ecosystem for regenerative medical products, it is essential to make ongoing efforts to identify challenges, improve processes, and refine these. This will help enhance the next generation of medical technologies. While the government currently supports the establishment of research, development, manufacturing, and supply systems for regenerative medical products, transient investments alone cannot sustain the ecosystem. To ensure Japan becomes a more attractive market, it is essential to balance regulatory system enhancement, reinvestment of profits gained from initial cycles by private companies, and the further expansion of government investment funds from a bio-industry development perspective.

- **Promote measures to strengthen stakeholder collaboration**

In innovative technology development, it is crucial to introduce research findings swiftly and efficiently to the market. Bridging research plays a vital role in this process. The government and relevant ministries should promote policies that facilitate the transfer of university-originated technologies to startups and push rapid product development.

To ensure smoother provision of regenerative medical products, it is urgent to build systems and strengthen networks to link referring facilities with treatment-providing facilities more effectively. By enhancing collaboration among medical institutions, clarifying the positioning of regenerative medical products in clinical guidelines, and boosting incentives for patient referrals, a network should be developed to ensure that patients requiring regenerative medical products receive treatment quickly and appropriately.

To improve patient awareness and ensure their access to treatment, patient organizations, academic societies, medical institutions, and development companies need to collaborate in building forums for sharing and discussing the latest information on diseases and the various treatment options, including immunotherapy, and create comprehensive and easy-to-understand guidance.

Furthermore, evidence derived from real-world clinical data should be accumulated and fed back into next-generation research and development. With the participation of treatment-providing facilities, academia, development companies, and patients, it is important to verify the long-term value of regenerative medical products and build the kind of foundations that will help ensure their further development.

- **Continue to reduce costs and develop infrastructure**

The manufacturing, quality control, and supply of regenerative medical products remain in the early stages of refinement, with many processes relying on manual labor by highly skilled personnel, resulting in high manufacturing costs. However, with technological advancements such as automation and robotics, future cost reductions are anticipated, allowing more patients to benefit from new regenerative medical treatment opportunities across various diseases.

The government should aim to nurture highly specialized human resources, promote facility development, enhance supply chains, and establish a domestic ecosystem, solidifying Japan's position as a base for expansion into Asian countries.

<Attribution of this Recommendation>

This proposal is a compilation and presentation of opinions by the Healthcare and Business Creation Group of the Research and Consulting Division of the Japan Research Institute, Inc., with a fair and equitable perspective in mind, based on the desire to contribute to society from a medium- to long-term perspective.

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