Scientifically-based Regulatory System and Value-Based Care: Critical Factors for Pharmaceutical Innovation

Takeda Pharmaceuticals International

Executive Summary

China has made incredible strides over the last twenty years in reforming and improving its healthcare system, helping millions of people and patients enjoy longer, healthier lives. However, as medical innovation in China and elsewhere in the world accelerates, there is a need for further reforms to ensure China remains at the forefront of research and development (R&D) into new therapies. Reforms should also continue to facilitate access for Chinese patients to the latest innovative treatments.

China has always placed innovation at the core of its overall development goals, both generally and explicitly within healthcare. The Chinese government is taking great care of its citizens through continuing healthcare reforms. At present, China is the second largest pharmaceutical market globally, and is growing at a fast pace. Medical innovation is the best way to confront the combined challenges of unmet medical needs, changing demographics, improved lifestyles and the resulting increase in chronic diseases. Inclusive economic growth, significant advances in diagnostic and medical innovation, and better access to healthcare enable people across the globe to live longer and more productive lives.

Nowadays, we are witnessing the onset of an unprecedented trend of medical innovation that is expected to reach patients in the coming years. For example, immunotherapies and cell and gene therapies could provide essential breakthroughs for patients with unmet needs, including those with rare or complex diseases. The effort to find solutions for these patients must continue and the challenge is enormous: about 8.000 rare diseases exist, of which only a small portion have effective treatments today. In recent years, China's pharmaceutical sector has made major progress in terms of innovation. This includes research and development of new drugs, going from 'me-too' to 'me-better', and then to 'first-in-class'. However, there is still work to be done in order to ensure China becomes a top innovative country in the field of medical R&D.

Since 2015, China has unveiled a series of policies and measures aimed at significantly reforming drug regulation and patient access. First, the introduction of Marketing Authorization Holder （MAH） system enhanced regulatory efficiency by starting a dialogue between industry and the government evaluation center on R&D for drugs, accelerating evaluation and approval processes, and initiating collaboration with The International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use （ICH）. The gap with the international community has thereby been narrowed.

Furthermore, the establishment of National Healthcare Security Administration and the implementation of a dynamic adjustment mechanism of the National Drug Reimbursement List (NDRL) has put an end to the fragmentated management of China's health insurance system. It also promoted volume-based purchasing reforms, and further regulated the market prices of drugs in a way that supports patient access to innovative drugs.

Benefiting from this improved regulatory environment, especially the reform of the drug approval system, the pharmaceutical sector in China is steadily becoming more innovative. In the next decade or two, the key to enhancing pharmaceutical innovation is building a sustainable innovation ecosystem. Creating a favorable policy environment will also support foreign investment in China. Drug researchers with vision and experience will come to China to develop breakthrough therapies. This will support the government's aim of becoming an international leader in this field.

To allow for further development and safe use of advanced therapies for patients in China, Takeda sees opportunities for the Chinese government to even further accelerate progress in two areas. **The first is to continue the development of an open, scientifically-based regulatory system and the acceleration of marketing approvals for innovative drugs with critical clinical needs. The second is to promote reforms in pricing, reimbursement & procurement using a value-based approach, to improve patient access to innovative drugs, and to foster innovation.** With experience gained from running pharmaceutical businesses worldwide, Takeda would like to make the following detailed proposals:

Regulatory system: First, supporting measures should be improved to ensure effective implementation of the fast track system, particularly for drugs which are launched in China first, best-in-class drugs for high unmet medical needs, and innovative drugs for diseases for which no effective treatment is available in China. New regulatory proposals related to the fast track system should be announced as early as possible, and a package of reform measures put in place to create a new scientific review and approval system. This system should provide manufacturers with regulatory guidance at the beginning of the process and ongoing dialog with CFDA during the review and until a final decision is made.

Second, further progress should be made in promoting the utilization of real-world evidence (RWE) to support approval decisions. The RWE pilot research projects ongoing in the City of Boao Lecheng should be expanded so that data standards, technical regulations and operational management of RWE can be established, and a regional real-world healthcare data platform can be constructed. Further consideration should be given to expanding this pilot into other selected cities / free trade zones in different parts of China to enable more data collection and patient access to fulfil the potential of RWE in healthcare decision-making.

Thirdly, the research on technical review and regulatory system of cell and genetic therapy products should be accelerated with the aim to comprehensively speed up - in all dimensions - the clinical translation and marketing of advanced cell and gene therapy products from both domestic and international sources. This process should involve promoting the development of cutting-edge technologies, gradually lifting restrictions on the import and export of cell and gene therapeutic

products and designating related regulatory authorities to ensure uncompromising quality standards of products during clinical trials and after their entry into the market. Relevant import and export procedures should be studied and formulated to support cross-border R&D and manufacturing as well.

Fourthly, in areas showing remarkable technical development but in which regulations have not kept up, updates and revisions of policies should be embarked upon, for example, in the field of innovative plasma-derived products that can be used to treat various rare and complex diseases. The possibility of conditionally lifting restrictions on the importation of some blood products (e.g., immunoglobulin products) should be explored. Takeda recommends the creation of a joint working group to discuss policy changes for certain advanced therapies and the scope for orderly and safe development of blood products.

Patient access: First, value-based and evidence-based regulations on NRDL product pricing and patient access should be established, along with regular reviews of value assessment methods to ensure the system remains fit for purpose for innovative therapies or therapies that address high unmet medical needs (for example, rare disease therapies and cell and gene therapies). The fact that for such therapies clinical data may be less robust at the beginning and the economic case not immediately clear should be recognized yet not form an insurmountable hurdle to access. Second, the patient access process should be optimized to facilitate the transition from marketing approval and NRDL listing of innovative therapies, to improve patient access upon, or shortly after regulatory approval, particularly for therapies indicated for diseases with high unmet medical needs. Thirdly, further exploration is recommended for an innovative multilayer funding system especially multi-channel fund-raising and innovative access schemes centered around basic medical insurance. The latter should be complemented by an orderly development of a commercial insurance system, which could ease the burden on the Basic Medical Insurance System.

Conclusion

Multi-stakeholder dialogue and engagement in the reform process offers great opportunities for China to shape the policy environment, including internationally, and become a thought leader. Takeda is committed to working with the Chinese government, healthcare providers, regulatory agencies and patient groups to establish an optimal regulatory framework and a multi-layer payment model that will enable timely and affordable access for patients. Implementation of such frameworks will both support China’s goals in its ongoing healthcare reform efforts and benefit patients.