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Overview of current policy for rare disease in China

Introduction

2018 was regarded as a milestone of rare disease management in China as, for the first time after decades of negligence in this area, China published the First List of Rare Diseases (FLRDs). This was a key step in improving and standardising the regulatory system of rare disease management, orphan drug development, and review and approval.

In this FLRDs, 121 diseases were included and officially defined as rare diseases. Since then, a series of regulations and policies have been released and implemented to support the development of rare disease treatment and management.

Regulatory updates for rare disease management in China since 2018 cover a wide range of topics including the definition of rare disease, releases, and updates on lists of rare diseases, rare disease drug R&D, drug registration, market access of rare disease drugs, and supply of orphan drugs.

The Chinese Government showed a strong commitment to encouraging innovation of rare disease treatment. In May 2019, the Center for Drug Evaluation (CDE) announced that real-world evidence (RWE) can be leveraged as a method to evaluate effectiveness and safety when clinical trials are not feasible. In addition, the CDE published a public notice to solicit opinions to the Technical Guidelines for the Research and Development of Rare Disease Drugs in October 2021, further improving the R&D efficiency of rare disease drugs.

Aside from the regulations encouraging R&D, the Government also introduced several policies such as reducing the VAT tax of imported orphan drugs; as of 2022, there have been 34 rare disease drugs that are entitled to this tax reduction. Despite these regulations and implementations, the development of the regulatory system of rare disease management in China still lags when compared to countries and regions such as Japan, the EU and the US, with the average launch time of most orphan drugs in China 9.5 years behind those within the EU and US.

Key takeaways

Several key trends on the current policy and market dynamics for rare disease in China are outlined in this paper. With strong expertise and deep understanding of the rare disease field in China, Deallus is well positioned to support Pharma to fully understand the current landscape and maximise success in the market.

- **A series of regulations and polices targeting rare diseases are in place, as well as the First List of Rare Diseases (FLRDs), allowing the standardisation of the regulatory system of rare disease product development and management.**
- **Several regulatory pathways can be leveraged to accelerate rare disease product approval, with priority review being the major one.**
- **Overseas clinical trial data for New Drug Application (NDA) is acceptable if applicants can prove no efficacy or safety differences in ethnicity.**
- **Current pricing of rare disease drugs has no established or transparent methods to date, where the cost varies largely across difference diseases.**
- **While central market access through the National Reimbursement Drug List (NRDL) is available, it has been putting great pressure on Pharma to reduce the prices of the drug, although this is essential for patients to afford the out-of-pocket payments for rare disease products.**
- **Pharma companies as well as provincial/city-level local governments have made further attempts to increase patient access, including commercial insurance and regional special fund to cover high-value rare disease drugs outside of NRDL.**

Contents

Regulatory pathways of rare disease drug approval

There are several regulations that accelerate the review and approval process of drugs for rare diseases in China.

For orphan drugs that have been launched overseas, clinical trial data from overseas can be directly used for New Drug Application (NDA) if applicants can prove there is no difference on ethnicity after investigation. If the clinical trial data obtained from overseas trials are defined as partially acceptable after evaluation, the drug can be included under conditional approval, which refers to gathering further safety and efficacy data of the drug after launch for evaluation.

Another is priority review, a drug regulatory pathway that grants a prioritised review compared to the traditional pathways, and which can accelerate the approval of innovative drugs and urgently needed drugs. For the orphan drugs granted priority review, which includes those needed in clinical practice and have been launched overseas, the review time should be no more than 70 days, compared to more than a year for a normal review period.

Generally speaking, the Chinese Government has shown supportive attitudes towards the R&D and introduction of rare disease treatments in the China market, aiming at creating a more friendly environment for R&D and granting accelerated regulatory approval for orphan drugs.

Regulations such as technical guidelines can help to increase the efficiency and visibility of clinical trials, and with the acceptance of RWD and trial data from the overseas market, the restrictions and challenges related to patient recruitment have also been mitigated significantly.

These regulations open doors for the orphan drugs that have been launched in the overseas markets to enter the China market with an accelerated timeline. For companies who wish to introduce their rare disease treatments to China, it is highly recommended to understand these favourable policies to adopt a more efficient launch strategy.

Drug price setting and reimbursement

Orphan drug price setting follows the same process as other innovative drugs, with no established or transparent methods to date.

Though value-based pricing (VBP) has become the golden standard of pricing for innovative drugs in developed countries, most orphan drug pricing is set using the cost-plus pricing (CPP) method in China, which is a cost-based method for setting the price of pharmaceutical products by assessing production costs and adding a profit margin.

Without a uniform pricing method, rare disease treatment cost varies largely across different diseases, ranging from less than $6,000 to over $500,0000 per year per patient. Currently, 61% of orphan drugs cost less than $100,000 annually, with the largest proportion of drugs costing between $6,000 and $50,000 per year.

In the meantime, central market access through the National Reimbursement Drug List (NRDL) negotiation has introduced cost-effective medicines to benefit the people who have joined the national medical reimbursement system.

Referring to the negotiation of 2021 NRDL, the Government authority demanded a significant price concession for high-value products (with annual costs above RMB 500K/USD 77k in China) to be listed. The most phenomenal case is Biogen's Spinraza, with a 95% price cut after nine rounds of negotiation to become the first high-value rare disease product listed in NRDL.

Under Government pressure to reduce the drug prices, rare disease drug pricing and the market access environment may not be optimal, especially for Multinational Companies in China. To maximise the market uptake, a deep understanding of the local market dynamics and a trade-off between price and volume needs to be made.

Patient payment & regional variation in access

Without any reimbursement, out-of-pocket (OOP) payment of rare disease products generally is unbearable to most families.

At the national level, the reimbursement for rare disease products is the same as with other innovative drugs (e.g. oncology, chronic disease). Through entering NRDL, typical coverage is 60-70% of the total cost meaning the patients only need to pay the additional 30-40% out-of-pocket. Considering that high-value products already take a significant price concession when entering NRDL, the OOP is considered quite affordable to most families and the willingness to pay has been significantly increased.

If we take a look at the two high-value rare disease drugs listed in 2021 NRDL as an example, after the NRDL listing, patients only needed to pay 1% and 8% of the pre-NRDL marketed price for Spinraza and Replagal, respectively. (See table below)

Product	Pharma	Indication	Marketed price before NRDL/vial	Listing price NRDL/vial	Patient OOP after NRDL/vial	% (OOP/listed price)	% (OOP/ marketed price)
Spinraza	**Biogen**	**Spinal Muscular Atrophy (SMA)**	**RMB 700,000 ($107,700)**	**RMB 33,000 ($5,100)**	**RMB 10,000 ($1,500)**	**30%**	**1%**
Replagal	**Takeda**	**Fabry Disease**	**RMB 12,180 ($1,874)**	**RMB 3,100 ($477)**	**RMB 930 ($143)**	**30%**	**8%**

Apart from the NRDL listing, Pharma companies are also exploring alternative avenues to increase patient access, including drug donation activities and commercial healthcare insurance (i.e. Hui Min Bao in Beijing, Shanghai).

At the provincial/city-level, some local governments have made further attempts to widen the coverage of rare disease drugs to increase access. In Zhejiang provice, for example, the government has set a special fund to cover a few high-value rare disease products in addition to NRDL coverage, including Fabrazyme (Fabry disease), Cerezyme (Gaucher disease), Myozyme (Pompe disease), and Kuvan (hyperphenylalaninemia, HPA).

With multiple national and regional market access avenues, the economic burden on patients and their families has been significantly relieved. Apart from NRDL listing, Pharma has the opportunity to engage with regional market access programs to optimise the market uptake, with continued efforts to support funding allocation and reimbursement decision across multiple stakeholders. However, to successfully negotiate these avenues, it is imperative that Pharma fully understands the current landscape, who the key stakeholders are, and understanding of what other companies are doing, and what potential options officials may be open to at the provincial level.

Case Study

Clinical development tracking of rare disease pipelines in China

CLIENT

Our client is a global marketing team of a biopharma, dedicated to the research and development of novel therapeutics for rare diseases. It needed to understand the global clinical trial activity of two rare diseases to inform their strategic plans, clinical development and regulatory strategy of the in-house asset for these indications.

SOLUTION

Deallus undertook a competitive landscape evaluation and conducted a systematic secondary research map to develop a strong understanding of the clinical trial landscape, product snapshots and implications analysis.

We also conducted an in-depth assessment of competitor activities including market opportunities & threats analysis to identify competitors' and newcomers' strategic plans and timelines and their trial timelines, as well as assess the competitive threat to the client's in-house product.

Deallus also completed brand and launch planning to identify key stakeholder and patient segments. Deallus provided recommendations on launch standardisation across different geographic markets, and facilitated internal alignment and communication on the standardised launch approach.

Geographies of interest in APAC: China, Japan, South Korea, Australia.

OBJECTIVE

The client asked key questions outlining what they wanted to understand to succeed.

- Who are our competitors and how would that impact our launch?
- What are the optimal brands and launch plans for sustained success in launched and unlaunched markets?
- How could the launch plan be implemented and facilitated?
- How do we align the overall launch strategies to create synergies across all markets?
- (Core question) what are the overall strategies and competitor insights we need in order to successfully launch and how should we plan, implement and execute these strategies?

VALUE

Deallus delivered comprehensive clinical trial trackers for the indications of interest and quarterly reports summarising all market and competitor findings as well as forecast on launch sequence of competitor products.

We also pressure-tested internal thinking of key competitors' strategic intent and provided advice on optimal clinical development strategy, regulatory strategy, and potential patient pool to the client.

Conclusion

China is undeniably a vast market that presents an appetising opportunity for rare disease drug developers, not only due to the large patient population but also due to infancy of the rare disease market, meaning that competition is still limited. While domestic companies may have an advantage in terms of local presence, established relationships and local data, many MNCs have the advantage of an established rare disease portfolio already launched in other markets which can be expanded into the Chinese market, potentially without too many hurdles.

The update in regulations and policies since 2018 suggest that rare disease management has become more of a priority for the Chinese Government, and the hope is that this will continue moving forward, hopefully reducing the drug lag between China and other major markets. Regional governments are also taking the initiative to widen the coverage beyond just being national. While this does somewhat complicate the landscape, it also strongly suggests that this focus is likely to continue, paving the way for more rare disease drugs to enter the Chinese market.

The largest question for MNCs whether to enter the market is undeniably around achievable price. Recent examples have shown that the NRDL negotiation scheme has been successful in China, resulting in prices that would be unimaginable in other markets being accepted in China in order to gain national access to the market. However, it is important to note that there are other routes available to access the Chinese market. As a result, MNCs must carefully consider which route to market serves them best, after understanding the trade-off between price and volume, and how this will impact their potential return on investment.

Overall, the future looks optimistic - more patients are and will continue to have access to efficacious treatments, an achievement thanks to the Government, patient groups and pharmaceutical companies. For MNCs who want to enter the China market with orphan drugs, the timing is optimal, and a comprehensive understanding of the local market is always the key to success.

Overall, the future looks optimistic - more patients are and will continue to have access to efficacious treatments, an achievement thanks to the Government, patient groups and pharmaceutical companies.

Contacts.

Neal Somchand
Principal and Head of Japan

+81[0]3-6807-5857
Neal.somchand@deallus.com

Haoyu Liu
Senior Associate

haoyu.liu@deallus.com

Yi Jin
Senior Associate

yi.jin@deallus.com

George Gu
Principal and Head of China

+86-13311771871
George.gu@deallus.com

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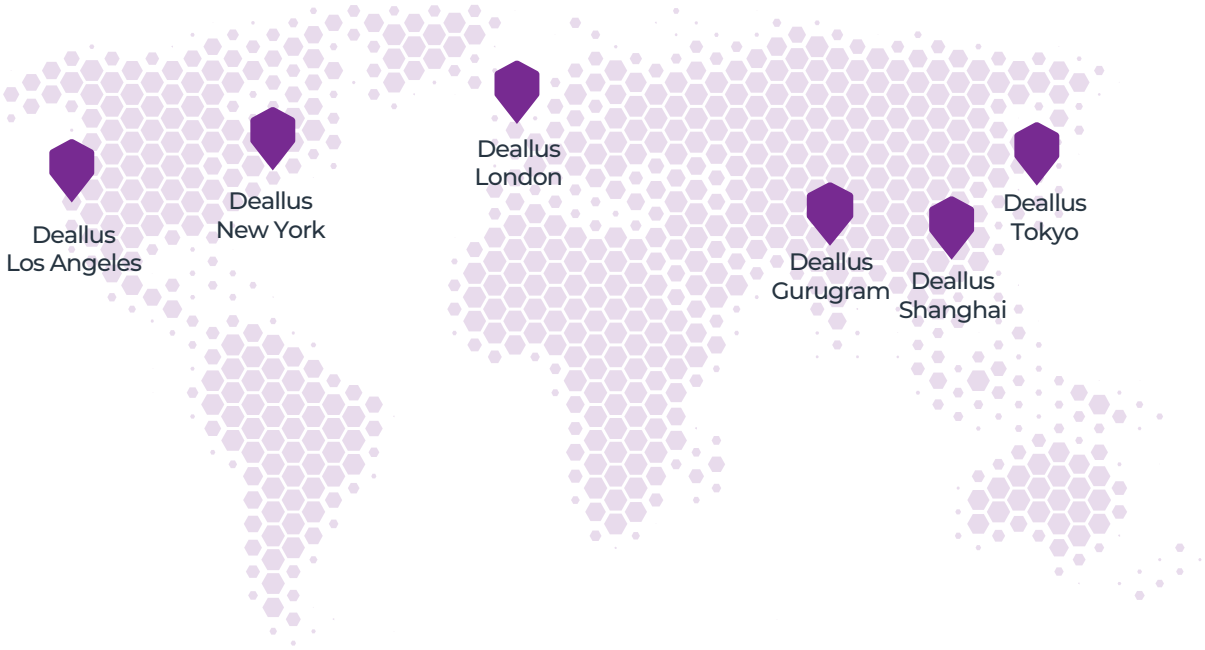

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