

China's rare disease drug development landscape:

Overview and insights for global biopharmaceutical companies



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China's rare disease regulatory landscape is evolving rapidly, creating new opportunities for global biopharmaceutical companies. With 52% of imported orphan drugs approved through Clinical Waivers between 2019 and 2023, understanding the right regulatory pathway, clinical data package and evidence strategy is becoming increasingly important.

China's clinical development ecosystem has expanded rapidly over the past decade. In 2023, 55% of global clinical trials included an Asia-Pacific location, with China hosting 62% of trials conducted in the region. Since 2021, the number of trials initiated in Asia-Pacific has exceeded those initiated in the rest of the world.

Despite the growth in clinical trial activity and regulatory approvals across multiple product types and indications, substantial unmet medical needs for rare disease treatments remain in China. In response, China has adopted a multi-pronged approach to advance orphan drug development, creating significant opportunities for rare disease drug developers.

Key factors driving China's clinical development boom

China's rapid rise to become a global leader in clinical trials has been driven by a set of interconnected forces, with a supportive ecosystem spanning legislative, regulatory and listing reforms playing a central role in this growth:

- › **Regulatory reforms and efficiency:** China's pharmaceutical regulatory environment has undergone significant reforms, leading to significant efficiency improvements in China's National Medical Products Administration (NMPA) clinical trial and drug approval process. These reforms include measures such as Priority Review, Special Review, and Breakthrough Therapy designation for innovative products.

For example, the number of Investigational New Drug (IND) applications accepted in 2024 reached 3,073 — *three times the number accepted in 2017*. Similarly, the total number of New Drug Applications (NDA), Abbreviated New Drug Applications (ANDA), and biosimilar applications accepted in 2024 reached 5,978, *three times the total number accepted in 2020*. The on-time completion rate for drug reviews has remained above 95% since July 2020.

- › **Innovation focus:** Over the past decade, China has enacted legislation aimed at accelerating biopharmaceutical innovation. Investigational innovative therapies now represent a considerable share of clinical trials in China and include (but not limited to): multi-specific antibodies, immuno-oncology therapies, antibody-drug conjugates, cellular therapy products, and new chemical entities.
- › **Domestic industry leadership:** Chinese companies are the primary sponsors of local industry-sponsored clinical trials, accounting for approximately 95% of these trials. Their activity is particularly strong in innovative therapies such as cell therapy, gene therapy, and antibody-drug conjugates (ADCs). They are key drivers of China's increasingly large share of global clinical trials.

- **Revised stock exchange listing rules:** Changes to the listing rules allow pre-revenue and pre-profit biotech companies to be listed on the Hong Kong Stock Exchange (HKEX) or the Shanghai Stock Exchange's Science and Technology Innovation Board (STAR). These rules enable innovative, high-growth biotech companies to access capital markets and investment earlier and attract more investment to develop innovative assets.

Significant unmet medical needs for new rare disease treatments in China

Despite the growth in innovative drug discovery and clinical trials, substantial unmet medical needs for rare disease treatments remain in China.

According to the “China Rare Disease Drug Accessibility Status and Solutions Recommendations” report,² the *First Rare Disease Catalog* includes 121 rare diseases affecting approximately 3 million patients.

Among the 74 rare diseases with treatments approved in the US, EU, or Japan, significant gaps remain in China: no drugs are available domestically for 9 diseases, only off-label use of existing drugs is available for 22 diseases, and treatments for 13 diseases are not reimbursed by national medical insurance.

Furthermore, a 2014-2018 survey of 5,810 patients with rare diseases revealed that 42% received no treatment at all, and more than half of those who received treatment did not receive timely and adequate medication.

These findings highlight urgent unmet clinical needs among a substantial patient population and represent both a critical healthcare challenge and a significant opportunity for global rare disease drug developers.

China's rare disease regulatory framework aims to accelerate orphan drug development

Unlike the US (Orphan Drug Act, 1983) and the EU (Regulation No. 141/2000, 2000), which have specific orphan drug legislation with a formal designation process, China uses the *Rare Disease Catalog* or scientific justification as a basis for regulatory oversight of drug development for rare diseases.

To incentivize orphan drug development, China has established a multifaceted framework that draws on international experience while adapting to its healthcare context and patient needs.

This framework includes new and updated legislation and regulatory policies, expanded guidance, and scientific flexibility, all aligned with international regulatory standards and practices and supporting China's inclusion in global rare disease drug development programs.

Additionally, specific tax credits are offered for clinical research and application fees for rare disease drug development programs.

1. Policy and legislative support to advance the development of orphan drugs:

New laws, regulations, and NMPA policies aim to facilitate and expedite orphan and other drug development and accelerate the regulatory review process.

a) Rare disease definition

- **Expanded rare disease catalog:** The expansion of China's Rare Disease Catalog from 121 diseases (First Catalog, 2018) to 207 diseases (with the Second Catalog added in September 2023) broadens the scope of diseases eligible for regulatory benefits and policy support. This expansion signals China's commitment to addressing a wider range of unmet medical needs and provides clarity for sponsors regarding which diseases qualify for expedited pathways.

b) NMPA policies and initiatives

Policy / Initiative	Details
Faster clinical start-up	Under the Clinical Trial Application (CTA) review process in China, there are two pathways: the standard 60 working-day (WD) process and the expedited 30 WD process. Projects that meet one of the following criteria are eligible for the 30 WD pathway: ➤ The drug is a key innovative product and demonstrates significant clinical value. ➤ It is included in the NMPA rare disease programs (CARE³ or SPARK⁴ plans; see below). ➤ It is part of a globally synchronized development program (Phases I–II) or international multicenter trial led by Chinese investigators (Phase III).

Priority review and accelerated approval pathways	Companies developing drugs for the prevention and treatment of rare diseases can apply for Priority Review and Approval, with a review timeline shortened to 130 WD; and 70 WD for orphan drugs approved outside China. Conditional Approval is available for drugs treating serious or life-threatening conditions with no effective treatment, including many rare diseases. This mechanism allows orphan drugs demonstrating preliminary efficacy and acceptable safety profiles to reach patients faster, with post-approval confirmatory studies required.
Clarified expedited pathways	More detailed operational guidance for Priority Review, Breakthrough Therapy designation, and Conditional Approval processes reduces regulatory uncertainty for sponsors
Acceptance of global clinical data	The Center for Drug Evaluation (CDE) offers “clinical waivers” (also known as clinical trial exemptions) when high-quality foreign clinical data is provided. For overseas-approved drugs treating serious, life-threatening, or rare diseases where no significant ethnic differences are expected, applicants can submit overseas clinical trial data to directly support marketing applications in China. This pragmatic approach, aligned with ICH E5 (Ethnic Factors) and E17 (Multi-Regional Clinical Trials) principles, significantly reduces development timelines and costs for global sponsors, while potentially eliminating the need for duplicative clinical trials.
Enhanced data acceptance standards	Updated provisions explicitly recognize the validity of real-world evidence (RWE) and innovative trial designs for rare diseases, acknowledging the practical challenges of conducting large randomized controlled trials in small patient populations.
Patient-centered initiatives	The “CARE Plan” (Patient-Centered Action for Rare Diseases Encouragement) and the “SPARK Plan” (Support Anti-Tumor Drug R&D for Kids) are innovative approaches designed to incorporate patient perspectives into orphan drug development while accelerating the development process. The CARE Plan facilitates early and close communication between the CDE and developers, incorporates patient perspectives into drug development and regulatory review processes, and allows for early review of pivotal clinical data (including top-line results), potentially accelerating approval timelines. Similarly, the SPARK Plan enables early and frequent interactions between developers and the CDE to obtain regulatory guidance on key technical issues related to pediatric anti-tumor drug development.

Streamlined post-approval changes	Simplified procedures for post-approval manufacturing changes and label updates reduce administrative burden and accelerate product optimization.
Market exclusivity protection	The policy⁵ introduces a defined 7-year market exclusivity period for eligible rare disease drugs, with the regulations proposing a potential maximum of 6-year regulatory data protection for innovative products over undisclosed clinical trial data and CMC, preclinical data.⁶ This protection mechanism, comparable to orphan drug exclusivity in the US and EU, enhances the commercial incentive to invest in rare disease R&D.

c) Financial incentives for developers⁷

- › **Innovation incentives:** Enhanced support for innovative drug development includes increased funding for translational research and robust intellectual property protection specifically targeting rare disease therapeutics.
- › **National medical insurance integration:** Through annual negotiations, China has progressively listed more rare disease drugs and offered preferential pricing policies in the National Reimbursement Drug List (NRDL). This integration improves patient access and creates a more predictable commercial pathway for approved products.
- › **Tax incentives:** Since March 2019, a simplified 3% value-added tax (VAT) rate has been applied to the production, sale, and import of rare disease drugs, significantly reducing the cost burden for manufacturers and improving patient affordability.
- › **Cross-border collaboration:** China has established policies that actively encourage international cooperation in rare disease drug development, facilitating partnerships between Chinese and foreign pharmaceutical companies, and promoting China's integration into global clinical development networks.

2. Regulatory guidance to advance orphan drug development:

To support orphan drug development in China, the CDE has issued a series of technical guidelines to provide clarity to sponsors. Most of the guidelines are derived from and align with global regulatory practices, such as those adopted by the FDA, EMA and ICH. The CDE guidelines cover:

- › **Disease understanding:** Emphasis is placed on conducting natural history studies, including in China, to identify patient populations, clinical outcome measures, and biomarkers.

- **Clinical trial design:** Flexibility is encouraged under unified standards. While randomized controlled trials (RCTs) are preferred, historical controls or other innovative designs may be acceptable under special circumstances. The requirement for “duplicate evidence” is not mandatory in all cases.
- **Endpoint selection:** Recognizing the challenge associated with many rare diseases, sponsors may consider Clinical Outcome Assessments (COAs) in RCTs. The selection of appropriate primary efficacy endpoints is crucial.
- **Model-informed drug development (MIDD):** Specific guidelines (e.g., the Technical Guidelines for Model-Informed Drug Development for Rare Diseases, 2025) encourage the use of modeling and simulation to optimize development.

Impact of China’s rare disease regulatory framework

Data and case studies⁸ from recent years indicate a clear and positive trend in orphan drug reviews and approvals by the NMPA:

1. Increasing the acceptance of global data:

The number of rare disease drug approvals in China has significantly increased (**Figure 1**). An analysis of imported orphan drugs approved by the CDE (2019-2023) shows that a majority (52%; 21 of 40 drugs) received “Clinical Waivers”, meaning that no new clinical trials were required in China to support registration (**Figure 2**). Among these, 3 drugs were approved solely on US/EU data, 13 drugs had Asian (non-Chinese) data, and 5 had Chinese data (mainland or elsewhere) (**Figure 3**). This demonstrates that the CDE has a pragmatic approach to data acceptance, one that aligns with ICH E5 (Ethnic Factors) and E17 (Multi-Regional Clinical Trials) principles and focuses on scientific justification rather than mandatory local trials.

Figure 1. Orphan drug approvals in China increased significantly between 2019 and 2024, while imported orphan drug approvals remained stable

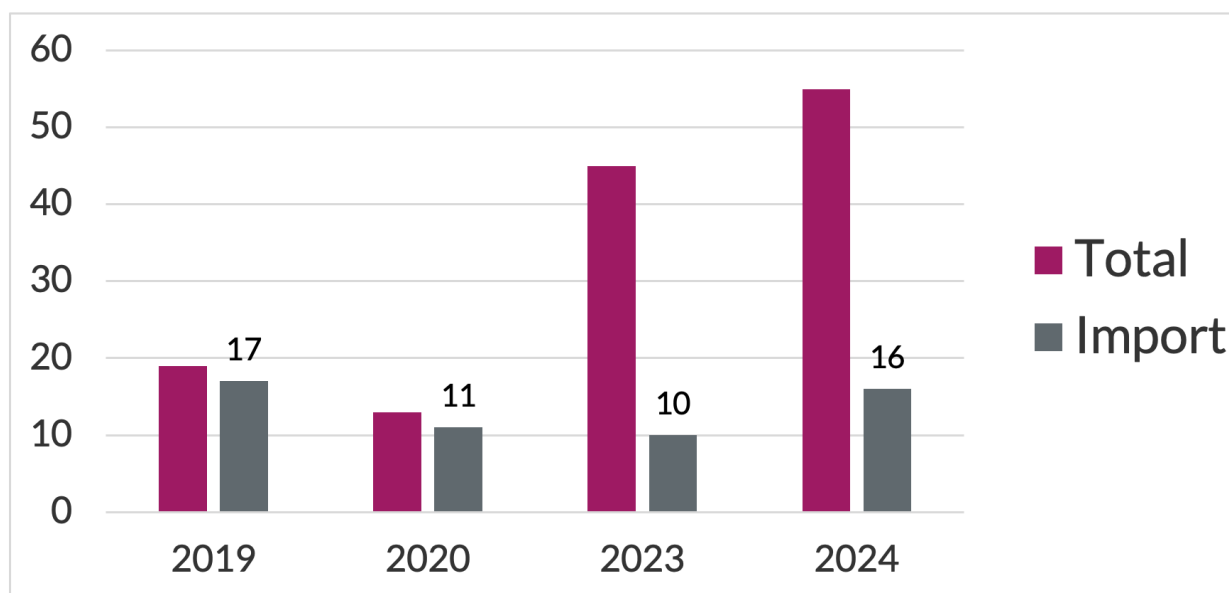
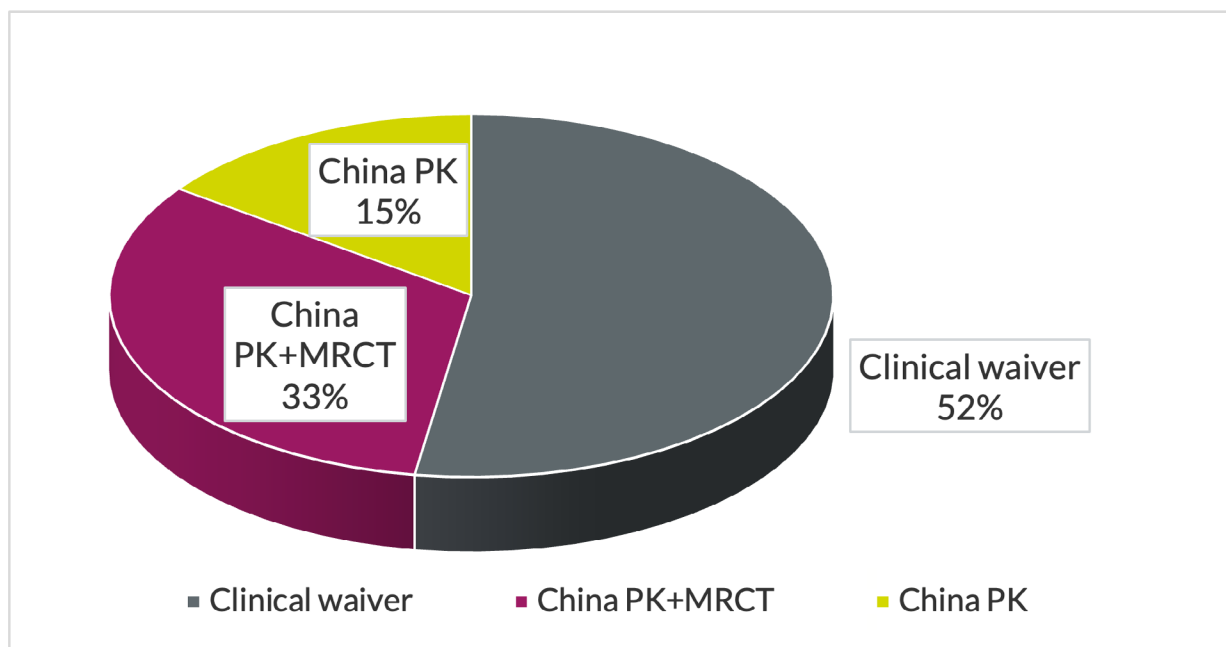
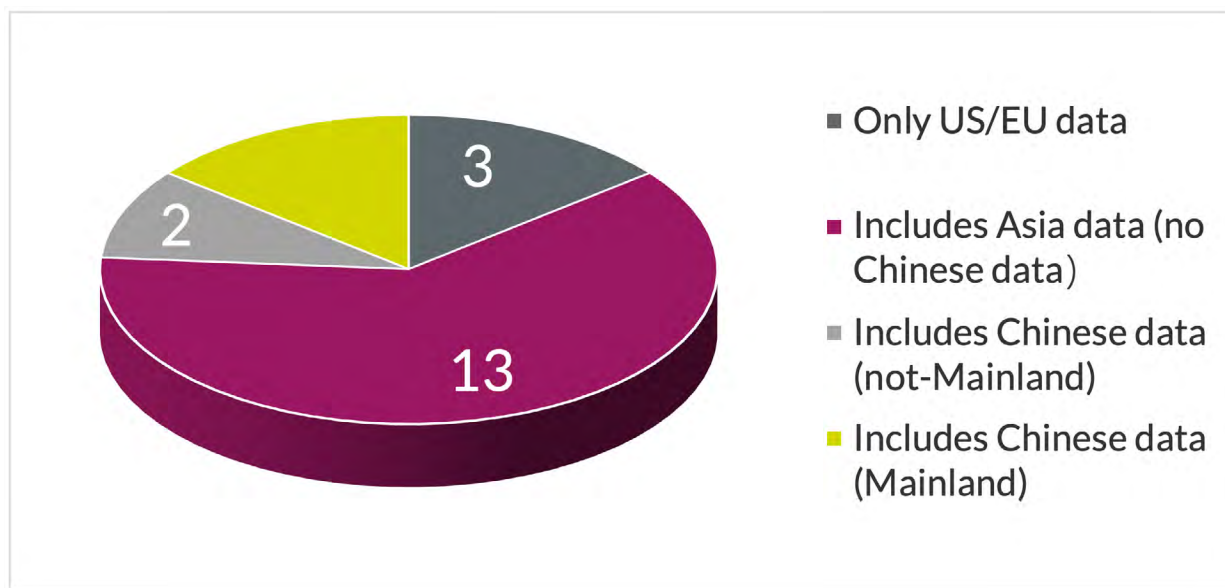


Figure 2. China approval of imported orphan drugs, 2018-2023



PK: pharmacokinetic data; MRCT: multi-regional clinical trial

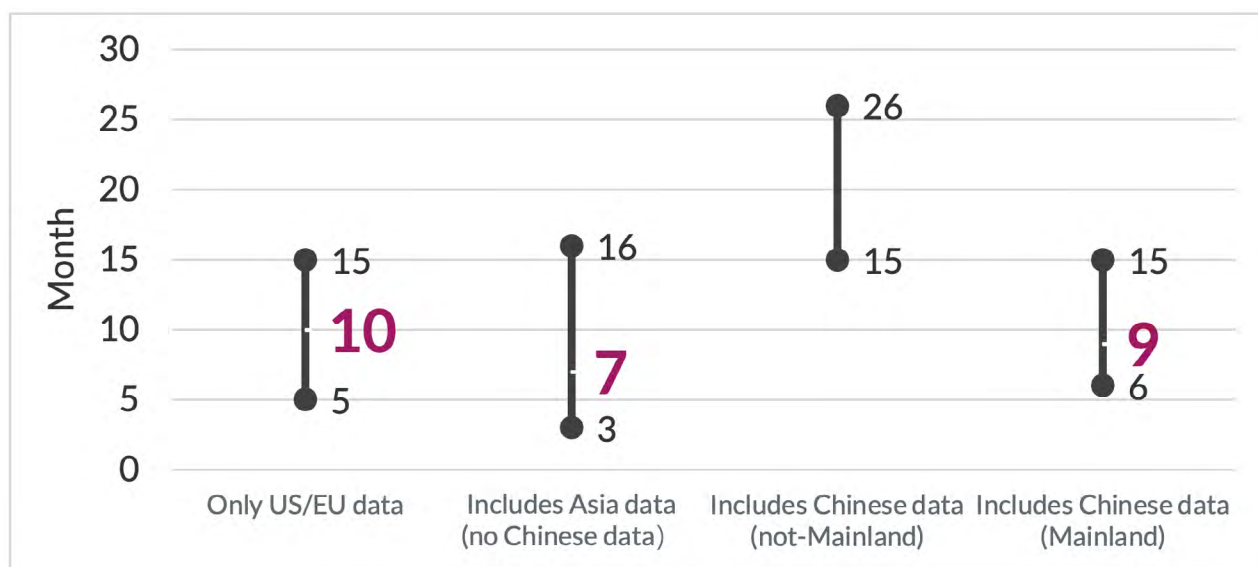
Figure 3. Clinical data packages for imported orphan drugs approved via “Clinical Waiver,” 2018-2023



2. Efficiency gains:

The median CDE approval time for imported orphan drug marketing applications (via Clinical Waivers) has been competitive. For drugs addressing significant unmet medical needs, the median approval timeline (7-10 months) is comparable to major global regulatory authorities (**Figure 4**). The RDPAC (R&D based Pharmaceutical Associations Committee) has suggested using the 12-16-month Priority Review timeline in 2023 as an industry benchmark for comparison with the current NDA/BLA application timeline.

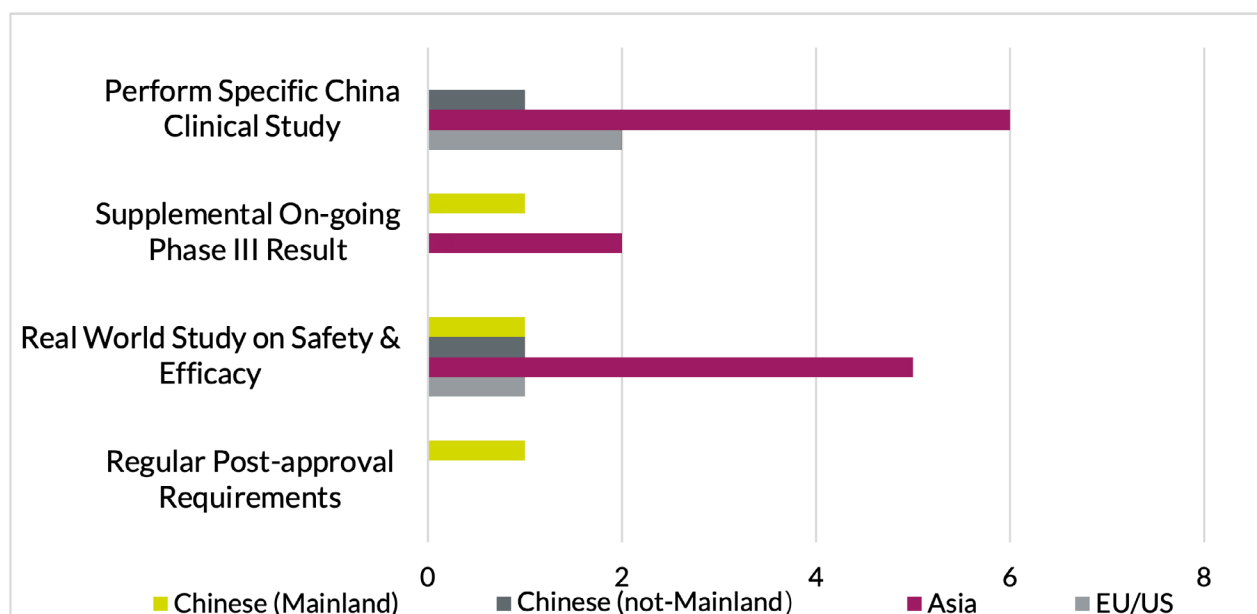
Figure 4. CDE NDA/BLA review timelines for orphan drugs approved via “Clinical Waiver” with different clinical data packages, 2018-2023



3. Post-approval requirements:

For drugs approved solely on foreign data, post-approval commitments are common requirements to gather safety and efficacy data in Chinese patients. These may include China-specific clinical studies, additional data requirements from an ongoing Phase III trial, or real-world studies. There is an inverse relationship between the proportion of Chinese clinical data in the regulatory submission and post-approval requirements (**Figure 5**): orphan drugs with a lower proportion of Chinese-origin data are more likely to face post-approval study obligations.

Figure 5. Post-approval requirements for orphan drugs approved via “Clinical Waiver” with different clinical data packages, 2018-2023



The x-axis shows the number of projects; and the different color shows the geographic origins of clinical data. “Regular” post-approval means that the orphan drug is subject to standard pharmacovigilance activities required for all newly approved drugs, without any additional or enhanced post-approval monitoring requirements.

Conclusion

China presents compelling opportunities for global biopharmaceutical companies engaged in rare disease drug development: it offers a large patient population, significant unmet needs in rare disease treatments, an efficient and innovation-friendly regulatory system, and increasing acceptance of global clinical data based on a robust scientific framework. By engaging early with Chinese regulators on the updated guidance and expedited pathways, global biopharmaceutical companies can significantly improve patient outcomes and capture the opportunities in this fast-growing market.

Ready to discuss including China in your orphan drug development plan? Our ex-NMPA/CDE regulators are [always available for a conversation](#).

References

1. White Paper_Clinical Trials Roundup APAC Deep Dive, Citeline, October 2024 https://www.citeline.com/-/media/citeline/resources/2024/10/exploring-the-asia-pacific-clinical-trials-landscape/white-paper_clinical-trials-roundup-apac-deep-dive.pdfhttps://www.citeline.com/-/media/citeline/resources/2024/10/exploring-the-asia-pacific-clinical-trials-landscape/white-paper_clinical-trials-roundup-apac-deep-dive.pdf
2. China Rare Disease Drug Accessibility Status and Solutions Recommendations, China Food and Drug Administration Journal, 2019, Vol. 2
3. National Medical Products Administration. Patient-Centered Action for Rare Diseases Encouragement – “CARE Plan.” https://english.nmpa.gov.cn/2025-09/05/c_1138504.htm
4. National Medical Products Administration. Support Anti-Tumor Drug R&D for Kids – SPARK Plan. https://english.nmpa.gov.cn/2025-09/05/c_1138509.htm
5. Implementation Regulations of the Drug Administration Law of the PRC (January 2026) <https://www.nmpa.gov.cn/xxgk/fgwj/flxzhfg/20260127172639127.html>
6. Pharmaceutical Product Data Protection Regulation (Trial), May 2026 <https://www.nmpa.gov.cn/xxgk/ggtg/ypggtg/ypqgtgtg/20260515145324185.html>
7. Several Measures to Support the High-Quality Development of Innovative Drugs https://www.gov.cn/zhengce/zhengceku/202507/content_7030260.htm
8. 40 published orphan drug review reports from China CDE <https://www.cde.org.cn/main/xxgk/listpage/b40868b5e21c038a6aa8b4319d21b07d>

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