Biobusiness briefs

https://doi.org/10.1038/d41573-025-00126-7

Trends in the development of cellular and gene therapy in China

Supported by governmental incentives and a reform-oriented regulatory framework, China has become an increasingly prominent player in the global cellular and gene therapy (CGT) field. Alongside these efforts, China has continued to optimize its foreign investment environment to encourage international collaboration in CGT. On the drug regulatory front, the National Medical Products Administration (NMPA) launched the Drug Regulatory Science Action Plan, which includes research on the technical evaluation of CGT products. In support of this, the Center for Drug Evaluation (CDE) under the NMPA has issued detailed technical guidelines covering key aspects of CGT development in the past 5 years, including chemistry, manufacturing and controls (CMC), non-clinical studies, clinical pharmacology and clinical studies (Supplementary Table 1). This article analyses trends in the CGT landscape in China, including the characteristics of CGT products in clinical trials and those that have been approved (see Supplementary information for details).

Trends in the CGT landscape in China

IND applications for CGT products. By the second quarter of 2025, the CDE had reviewed a total of 765 CGT investigational new drug (IND) applications, with CAR-T, stem cell and gene therapy products comprising the majority (Supplementary Fig. 1). Of these, 553 INDs were approved for clinical trials. Common reasons for non-approval of INDs included an insufficient scientific rationale, such as an unclear mechanism of action or the absence of demonstrated clinical need in the target population; the use of illegal or unethical starting materials; inadequate pharmaceutical or nonclinical data to support the conduct of clinical trials; safety concerns identified in investigator-initiated trials; and proposed indications or dosing regimens that were off-label relative to approved product labeling in combination therapy applications. Supplementary Fig. 2 shows a declining trend in IND terminations, reflecting enhanced data quality and strengthened CDE guidance and pre-IND engagement, all of which have contributed to a more supportive environment for CGT development.

Clinical trial landscape and characteristics of investigational CGT products. The development stage for CGT products in clinical trials in China is shown in Fig. 1. Among the various CGT modalities, CAR-T products are the most advanced overall, with a total of 72 products in exploratory trials, 15 in confirmatory trials, 7 under new drug application (NDA) review and 9 approved for marketing (Supplementary Table 2). An additional 45 INDs have been approved for products for which clinical trials have not yet been initiated.

The second most active area of clinical trial activity is for stem cell therapies, with 44 IND-approved products pending trial initiation, 87 in exploratory trials, 3 in confirmatory trials and 1 approved product (amimestrocel injection for graft-versus-host disease). The gene therapy area also has substantial activity, with 55 products in exploratory trials, 7 in confirmatory trials, 2 under NDA review, and 1 product approved for marketing recently (dalnacogene ponparvovec for haemophilia type B). Most other CGT modalities remain in early stages of development, but the data in Fig. 1 illustrate the diversifying CGT landscape in China.

The indications and targets for the three main therapy categories — somatic cell therapies (including CAR-T cell therapies, CAR-natural killer (NK) cell therapies and T-cell receptor (TCR) T cell therapies), stem cell therapies and gene therapies — were analysed further (Fig. 2).

Somatic cell therapies are primarily focused on cancers (both blood cancers and solid tumours). Blood cancer targets expressed on B-cells such as CD19 and BCMA are the most popular (Fig. 2b), and multi-target approaches are being explored. Encouraged by a pioneering clinical trial showing the potential of B-cell depletion with CD19-targeted CAR-T cell therapy to lead to treatment remission for patients with systemic lupus erythematosus (SLE), such therapies are now also being investigated for other non-oncological indications such as myasthenia gravis and systemic sclerosis.

Stem cell therapies in clinical trials have a broad spectrum of indications, with applications varying according to the specific functional properties of different stem cell types.

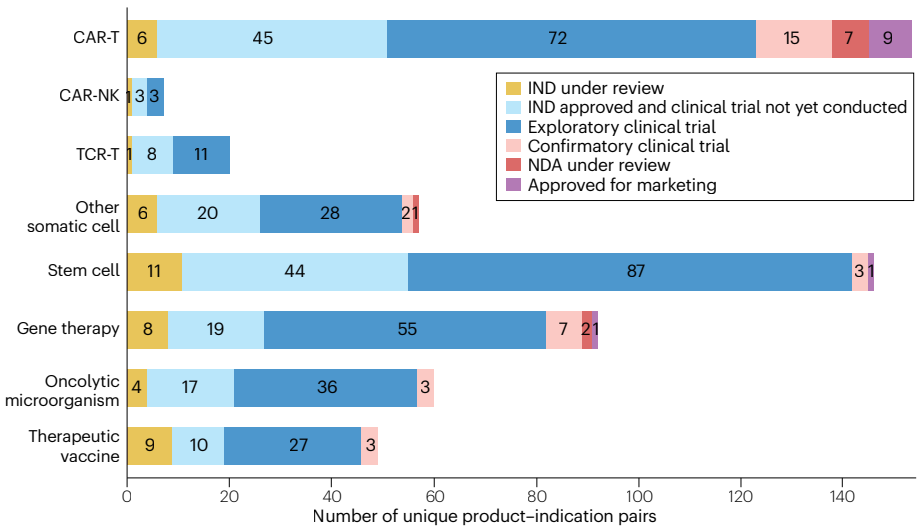

Fig. 1 | Clinical development status for investigational cell and gene therapy products in China. Distribution of investigational cell and gene therapy products across various clinical development stages: investigational new drug (IND) application under review, IND approved and clinical trial not yet conducted, exploratory clinical trial, confirmatory clinical trial, new drug application (NDA) under review and approved for marketing. Data are categorized by therapeutic type: CAR-T, CAR-NK, TCR-T, other somatic cell, stem cell, gene therapy, oncolytic microorganism and therapeutic vaccine. The *x*-axis represents the total number of indications across all products. See Supplementary information for details.

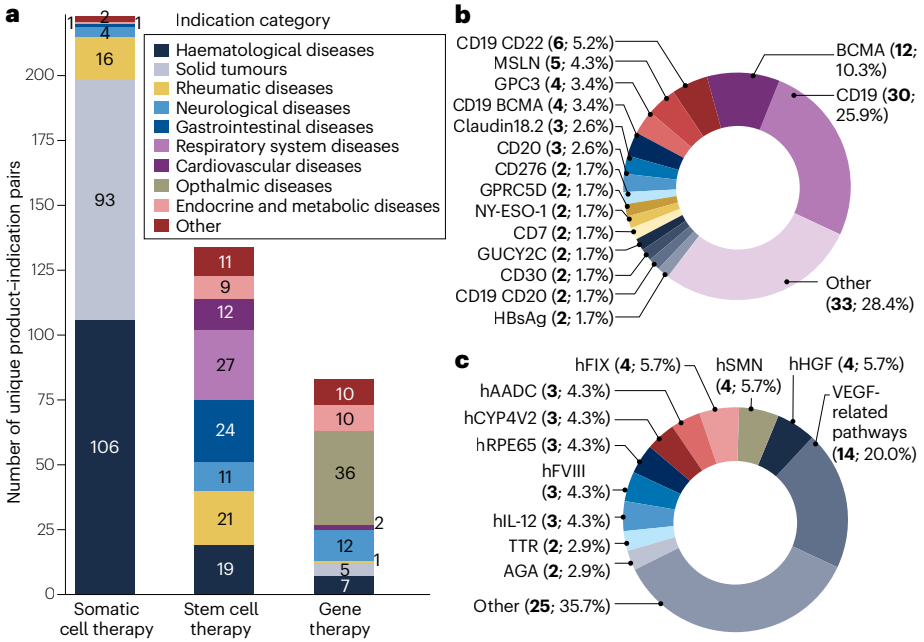

Fig. 2 | Distribution of therapeutic indications and targets for investigational cell and gene therapy products in China. a, The indication analysis includes somatic cell therapies, stem cell therapies and gene therapies. The indication count follows the same criteria as used in Fig. 1. **b**, Target analysis of somatic cell therapies. **c**, Target analysis of gene therapies. Targets are counted based on the number of products with approved investigational new drug applications. Products targeting dual or multiple targets are counted as a single combination and are not listed separately.

Gene therapies are largely focused on rare genetic diseases, including haemophilia types A and B, inherited retinal diseases, spinal muscular atrophy and inborn errors of metabolism. Gene therapies for eye diseases targeting VEGF-related pathways are particularly popular, with 14 IND-approved products (Fig. 2c).

Approved CGT products in China and post-marketing management. So far, the NMPA has approved six CAR-T cell therapies for nine indications (including seven under conditional approval and two with full approval), along with one conditionally approved stem cell therapy and one fully approved gene therapy, each for a single indication (Supplementary Table 2).

Five CAR-T cell therapy products have been approved in China based on single-arm clinical trials. These trials used tumour response at no less than three months after CAR-T infusion as the primary endpoint, in line with the CDE's requirement to assess the durability of response. All products demonstrated a favourable benefit–risk profile, with efficacy results significantly superior to existing therapies. Given the limited sample sizes in these studies, marketing authorization holders are required to conduct post-marketing studies to further verify product efficacy and safety. For conditionally approved products, confirmatory clinical trials should be completed within a required period of time from the date of approval, up to a maximum of four years. Long-term follow-up as well as real-world studies are required to monitor patients receiving CAR-T therapies.

Additionally, one CAR-T product that had already been approved overseas, axicabtagene ciloleucel, was evaluated in China through a bridging study and foreign clinical studies. The primary endpoint of the bridging trial was consistent with that of the foreign study. The acceptance of foreign studies in future applications remains subject to communication with the CDE.

Post-marketing risk management for CAR-T cell therapies in China is characterized by a comprehensive, lifecycle-wide approach that rigorously safeguards patient safety and ensures therapeutic efficacy, with particular attention to the surveillance of secondary malignancies, such as T-cell lymphomas. Although international investigations have detected CAR transgenes in malignant T-cell clones in a limited number of cases, no instances of secondary T-cell lymphoma have been reported in China. In practice, risk control measures are embedded throughout the development and marketing process. Clinical trial documentation emphasizes vigilant monitoring for secondary malignancy risks; product labels explicitly warn of these risks and require lifelong patient follow-up; and, upon detection of any malignant events, standardized protocols for the timely collection and molecular analysis of pre- and post-treatment biological samples are activated.

Regulatory outlook for CGT in China

The regulatory landscape for CGT in China is evolving rapidly to meet the growing demand for advanced therapeutic medicinal products (ATMPs). The CDE has preliminarily proposed a classification and description system for ATMPs in China and is studying policies to accelerate their development. Looking ahead, China's approach is expected to emphasize unified deployment while following the principles of "early involvement, one enterprise one policy, whole-process guidance, and research–review linkage". The CDE may adopt a case-by-case review approach, offering tailored strategies for different products based on their specific risks, benefits and manufacturing complexities. As the CGT field evolves rapidly, China is set to continually refine its regulatory framework to support innovation.

Yantong Liu[1], Chenghao Ge[2], Xiaoyuan Chen[2,3], Shuang Lu[1] ✉ & Tao Wang[1] ✉

[1]Center for Drug Evaluation, National Medical Products Administration, Beijing, China. [2]Clinical Trial Center for Drugs and Medical Devices, Beijing Tsinghua Changgung Hospital, School of Clinical Medicine, Tsinghua Medicine, Tsinghua University, Beijing, China. [3]Tsinghua Clinical Research Institute, Tsinghua Medicine, Tsinghua University, Beijing, China.

✉e-mail: lush@cde.org.cn; wangtao@cde.org.cn

Acknowledgements

The authors sincerely acknowledge X. Liu, H. Wang, Y.Huang, C. Zhao, J. Wang, G. Song, H. Wang and X. Wu from the Center for Drug Evaluation (CDE) for their valuable contributions and support. This work was supported by the Key Project for the Drug Regulatory Science Action Plan (grant no. RS2024S003).

Author contributions

Y.L and C.G. contributed equally, and S.L. and T.W. contributed equally.

Competing interests

The authors declare no competing interests.

Additional information

Supplementary information The online version contains supplementary material available at https://doi.org/10.1038/d41573-025-00126-7.