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Supplementary information

Trends in the development of cellular and gene therapy in China

In the format provided by the authors and unedited

Data sources and curation

The dataset provided in the Excel spreadsheet (see Supplementary Data) was compiled using publicly available information from the Center for Drug Evaluation (CDE) of China. This includes data from the CDE's drug registration and clinical trial platforms, as well as publicly released reports and announcements. Product information was cross-validated across these multiple sources to ensure consistency and accuracy. Target and indication data were further verified using commercial databases, within the scope of publicly disclosed information. The analysis includes applications reviewed since January 1, 2017, to June 30, 2025, which marks the publication of key technical guidelines for cell and gene therapy (CGT) products and the beginning of systematic review efforts by the Office of Clinical Evaluation of Biological Products, established in 2016.

Supplementary Table 1 | Technical guidelines for CGT products in China (as of June 30, 2025)

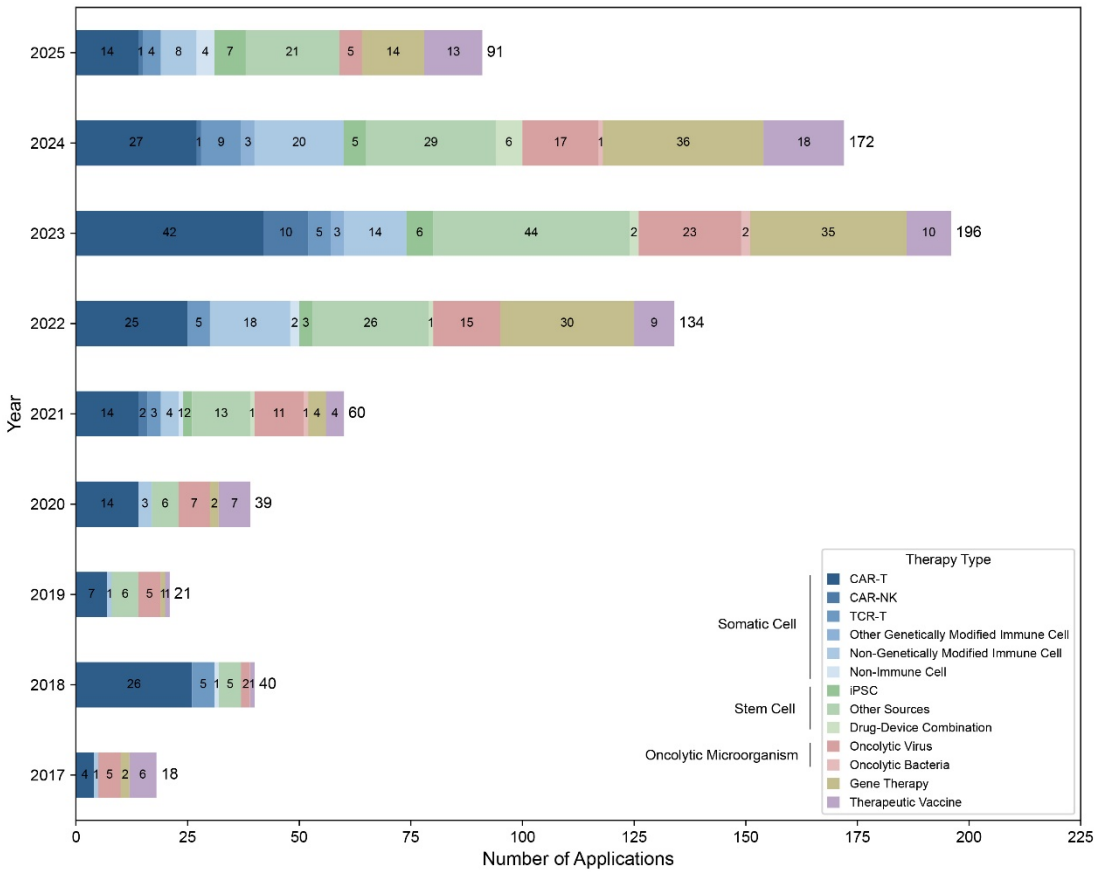
Subject	Year	Document
CMC	2022	Guideline for Chemistry, Manufacturing, and Control (CMC) Studies and Evaluation of Immune Cell Therapy Products (Trial)
	2022	Technical Guideline for the Chemistry, Manufacturing, and Control (CMC) Study and Evaluation of ex vivo Gene Modification System (Trial)
	2022	Technical Guidelines for the Pharmaceutical Study and Evaluation of in Vivo Gene Therapy Products (Trial)
	2023	Guidance for Pharmaceutical Research and Evaluation of Oncolytic Virus Products (Trial)
	2023	Guidance for Research and Evaluation on Chemistry, Manufacture and Control of Human Stem Cell Products (Trial)
	2023	Question and Answers on the Manufacturing Changes of Autologous CAR-T Cell Therapy Products
	2024	Technical Guidance for CMC Studies and Evaluation of Recombinant Adeno-associated Virus Vector in vivo Gene Therapy Products Investigational New Drug Applications
	2024	Common problems and technical requirements of replication competent lentivirus detection
Non-clinical studies	2021	Technical Guidance for Nonclinical Studies and Evaluation of Genetically Modified Cell Therapy Products (Trial)
	2021	Technical Guidance for Nonclinical Studies and Evaluation of Gene Therapy Products (Trial)
	2024	Technical Guidance for Nonclinical Studies of Human-Derived Stem Cell Products
	2024	Technical Guidance for Nonclinical Studies of Adeno-associated Virus Vector Gene Therapy Products
Clinical pharmacology studies	2024	Technical Guidance for Clinical Pharmacology Research on Cell Therapy Products (Trial)
Clinical studies	2021	Technical Guidance for Clinical Trials of Immune Cell Therapy Products (Trial)
	2021	Technical Guidance for Clinical Trials Design of Oncolytic Virus Drugs (Trial)
	2021	Technical Guidance for Clinical Studies with Long-Term Follow-up of Gene Therapy Products (Trial)
	2022	Technical Guidance on Clinical Risk Management Plan of Biologics License Application for Chimeric Antigen Receptor (CAR) T Cell Products
	2023	Technical Guidance for Clinical Trials Design of Gene Therapy Products for Hemophilia
	2023	Technical Guidance for Clinical Trials of Therapeutic Cancer Vaccines (Trial)
	2023	Technical Guidance for Clinical Trials of Human-Derived Stem Cells and Derived Cell Therapy Products (Trial)
	2023	Technical Guidance for Clinical Communication of Cell and Gene Therapy Products
	2024	Technical Guidance for Clinical Trials of Gene Therapy Products for Rare Diseases (Trial)
	2024	Technical Guidance for Clinical Trials of Mesenchymal Stem Cells for Graft-Versus-Host Disease (Trial)
	2024	Technical Guidance for Clinical Trials of Chimeric Antigen Receptor (CAR) T Cell Products for Hematological Malignancies (Trial)
	2025	Technical Guidelines for Clinical Trials of Gene Therapy Products for Thalassemia (Trial)

Supplementary Table 2 | Summary of NMPA-approved CGT products and clinical data

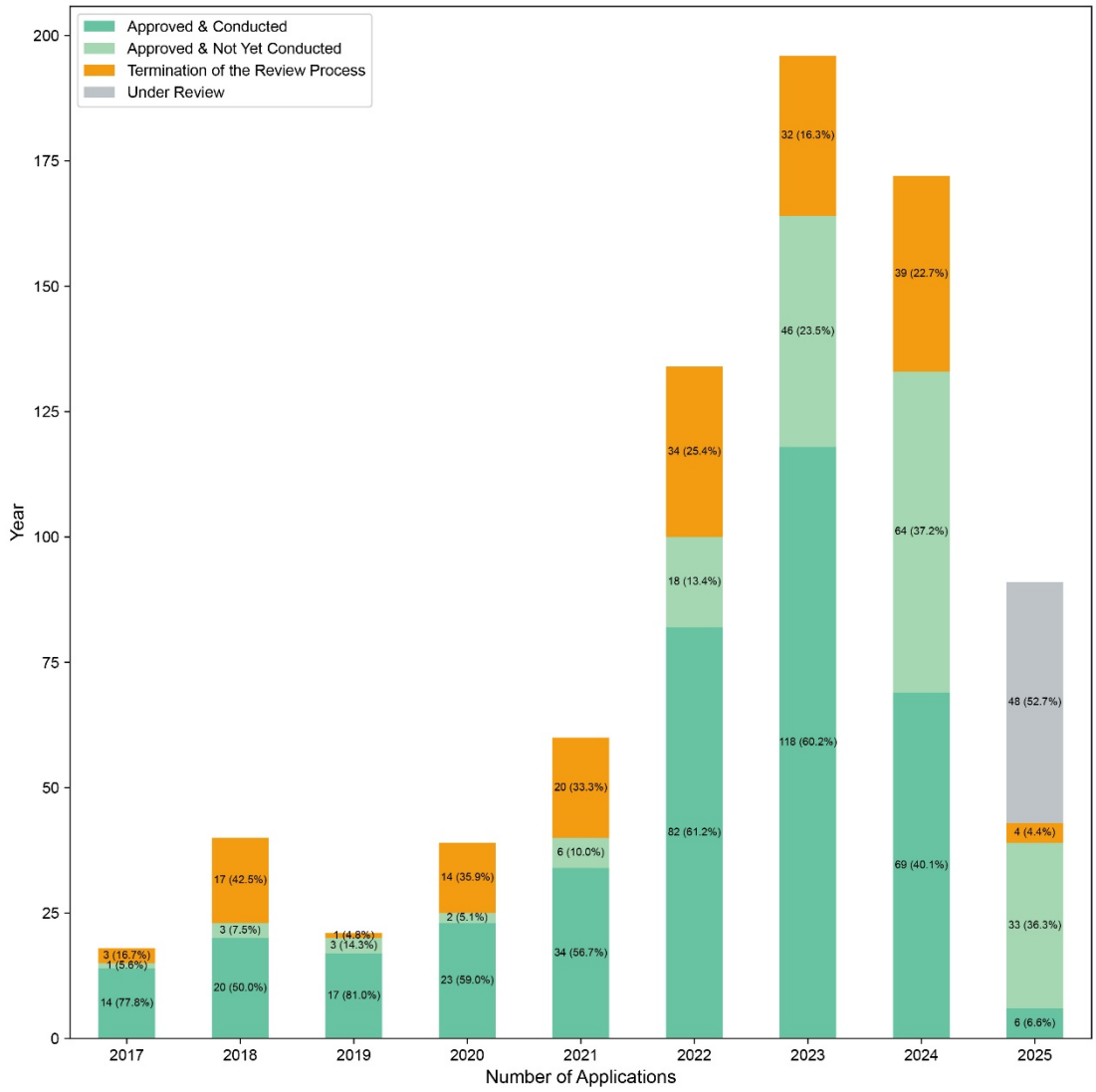
Product	Type	Date	Indication	Marketing authorization holder	Conditional approval	Efficacy result		
						Pivotal trial	Number of subjects in the efficacy data analysis set	Primary endpoint (%) (95% CI)
Axicabtagene ciloleucel	CD19 CAR-T	2021/6/22	Adult patients with relapsed or refractory large B-cell lymphoma after two or more lines of systemic therapy, including diffuse large B-cell lymphoma (DLBCL) not otherwise specified, primary mediastinal large B-cell lymphoma, high grade B-cell lymphoma, and DLBCL arising from follicular lymphoma.	Fosunkairos	No	KC876-2018-001 (bridging study)[#]	24	bORR [a] in 3 month 79 (58, 93) 2y OS 54.2%
		2023/6/21	Adult patients with large B-cell lymphoma that is refractory to first-line chemoimmunotherapy or that relapses within 12 months of first-line chemoimmunotherapy.		Yes	Accepted foreign clinical studies[#]		
Relmacabtagene autoleucel	CD19 CAR-T	2021/9/1	Adult patients with relapsed or refractory large B-cell lymphoma (LBCL) after two or more lines of systemic therapy, including diffuse large B-cell lymphoma (DLBCL) not otherwise specified, DLBCL arising from follicular lymphoma, follicular lymphoma grade 3B, primary mediastinal large B-cell lymphoma, high-grade B-cell lymphoma with MYC and BCL2 with or without BCL6 rearrangement.	JW Therapeutics	Yes	JWCAR029-002 A cohort	59	ORR at 3 month 60.3 (46.6, 73.0), ORR at 6 month 43.1 (30.2, 56.8) 2y OS 69.3%
		2022/9/30	Adult patients with relapsed or refractory follicular lymphoma (FL) after two or more lines of systemic therapy, including histologically grade1, 2, 3A.		Yes	JWCAR029-002 B cohort	27	CRR at 3 month 85.19 (66.27, 95.81) , CRR at 6 month 77.78 (57.74, 91.38) mOS NA
		2024/8/20	Adult patients with relapsed or refractory mantle cell lymphoma, including a Bruton tyrosine kinase (BTK) inhibitor		No	JWCAR029-005	59	ORR at 3 month 71.19 (57.92, 82.24) ORR at 6 month 55.93 (42.40, 68.84) mOS 19.5 (12.32, NA)
Equecabtagene autoleucel	BCMA CAR-T	2023/6/30	Adults with relapsed or refractory multiple myeloma (RRMM) after three or more prior lines of therapy and progressed, including a proteasome inhibitor and an immunomodulatory agent.	Iasobio	Yes	XL-LCYJ-0007 phase II	62	ORR at 3 months 87.1(76.1,94.3) ORR at 6 months 79.0 (66.8,88.3) mOS NA
Inaticabtagene autoleucel	CD19 CAR-T	2023/11/7	Adult patients with relapsed or refractory (r/r) B-cell precursor acute lymphoblastic leukemia (B-ALL)	Juventas	Yes	HY001201	38	ORR at 3 months 65.8 (48.65, 80.37) mOS NA
Zevorcabtagene autoleucel	BCMA CAR-T	2024/2/23	Adults with relapsed or refractory multiple myeloma (RRMM) after three or more prior lines	Carsge	Yes	CT053- MM-01	60	ORR at 3 month 88.3（ 77.43， 95.18 ）

			of therapy and progressed, including a proteasome inhibitor and an immunomodulatory agent.					ORR at 6 month 76.7（63.96，86.62） mOS NA
Ciltacabtagene autoleucel injection	BCMA CAR-T	2024/8/20	Adults with relapsed or refractory multiple myeloma (RRMM) after three or more prior lines of therapy and progressed, including a proteasome inhibitor and an immunomodulatory agent.	Legendbiotech	Yes	CARTIFAN-1	58	ORR at 3 month 86.2(74.6, 93.9) ORR at 6 month 82.8(70.6, 91.4) 2yOS 75.8%
Amimestrocel injection	Human mesenchymal stromal cells	2025/1/2	Steroid-refractory acute graft versus host disease (SR-aGvHD) with gastrointestinal tract involvement in 14 years of age and older	Platinumlife	Yes	BSSC2019092403002	54	ORR at D28 63.0 (48.74, 75.71)
Dalnacogene ponparvovec injection	Gene therapy	2025/4/8	Adults with moderate-to-severe hemophilia B (congenital Factor IX deficiency)	Beliefbiomed	No	BBM001-CLN100	26	Mean ABR in 52 weeks 0.60 bleeds/year (0.18-1.99)

[#]: A bridging clinical trial was conducted, based on the data of axicabtagene ciloleucel ZUMA-1 study and the transfer of manufacturing site to China, to evaluate the safety, efficacy, and pharmacokinetics/pharmacodynamics (PK/PD) of the China-produced product in Chinese patients. The study design was consistent with ZUMA-1. For the second-line indication, standard treatments are generally consistent between China and other countries, but long-term survival remains limited, indicating an unmet clinical need. The domestic study KC876-2018-001 showed no significant differences in safety or efficacy compared to ZUMA-1, with similar 2-year survival. Real-world data also support consistent effectiveness across populations. Approval was based on foreign clinical data (mainly ZUMA-7), which demonstrated clear efficacy and no new safety risks. Although the Asian subgroup in ZUMA-7 showed results consistent with the overall population, the sample size was limited. Based on regulatory review and expert consultation, conditional approval was granted with a requirement for post-marketing studies to further evaluate safety and efficacy in Chinese patients. The clinical technical requirements for drugs that are approved overseas but not yet marketed in China can refer to *the Technical Guidelines for Acceptance of Overseas Clinical Trial Data of Drugs* and *the Technical Requirements for Clinical Studies of Overseas Approved Drugs Not Yet Marketed Domestically*.



Supplementary Figure 1 | Trends in IND applications for CGT products in China (2017–2025 Q2). This figure presents the distribution of IND applications for CGT products received by the CDE from 2017 to 2025 Q2. The applications are categorized into five major therapy groups: somatic cell therapy (including CAR-T, CAR-NK, TCR-T, other genetically modified immune cell, non-genetically modified immune cell and non-immune cell); stem cell therapy (including iPSCs, other stem cell sources and drug-device combination products); oncolytic microorganism (including oncolytic viruses and oncolytic bacteria); gene therapy; therapeutic vaccine. The analysis here mapped 765 INDs and 6 supplemental applications, for a total of 771 applications. The 6 supplemental applications were for progression of clinical trials to subsequent phases (e.g., from phase II to phase III). Under previous regulatory requirements, separate submissions were required for such progressions.



Supplementary Figure 2 | Trends in IND review conclusions and clinical trial initiation (2017–2025 Q2). IND terminations may include voluntary withdrawals by applicants or withdrawals suggested by the CDE due to a refusal to approve. The analysis here mapped 765 INDs and 6 supplemental applications, for a total of 771 applications.