NMPA National Registration Gaps and FDA-to-NMPA Approval Lag Among FDA-Approved Rare Disease Therapies: An Indication-Level Analysis of China's 207-Condition Rare Disease Catalog, 2001–2024

HPR7
SESSION 1

Dilei Yan[1], **Ningying Mao**[1]

[1] School of International Pharmaceutical Business, China Pharmaceutical University, Nanjing, China

BACKGROUND

Rare disease therapies may remain without national registration in China after FDA approval. Registration counts alone cannot show how long approval takes or when the observed waiting time accumulates.

OBJECTIVE

To quantify national registration status, time from FDA approval to an exact same-indication NMPA approval date, differences between FDA-approval cohorts, and the components of approval lag among therapies approved by the FDA in 2001–2024 and mapped to China's 207-condition rare-disease catalogs. Registration status and exact approval-date availability were assessed separately.

METHODS

Data sources: Drugs@FDA, FDA orphan designations, NMPA registration records, CDE acceptance listings.

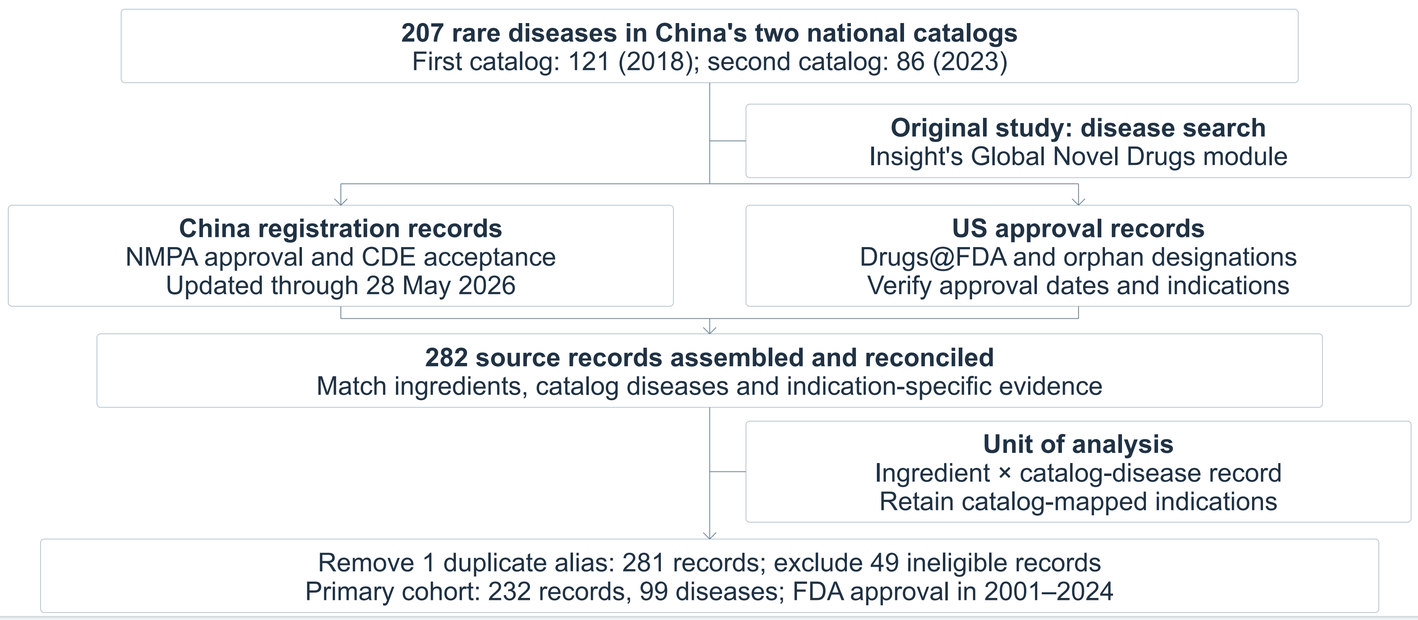
Unit: ingredient × catalog-disease record. **Time zero:** FDA approval. **Event:** an exact same-indication NMPA approval day. **Cutoff:** 28 May 2026, administrative censoring.

Overall analysis: 1−KM on 229 FDA-first records, excluding 3 China-first records. Pointwise 95% CIs used Greenwood log–log estimates. Ten known registrations with unresolved dates were censored.

Exploratory cohort comparison: FDA approval in 2001–2015 versus 2016–2024, restricted to five years. We compared cumulative registration and restricted mean time without a dated registration event.

Lag decomposition: 97 records with FDA submission/approval and CDE acceptance/NMPA approval dates. Means satisfy total lag = submission milestone difference + approval-interval difference. 95% CIs used 2,000 ingredient-cluster bootstrap samples (89 clusters).
Negative differences were retained.

Data flow



RESULTS

Figure 1. FDA-approved rare disease indications without an exact NMPA approval date, 2001–2026

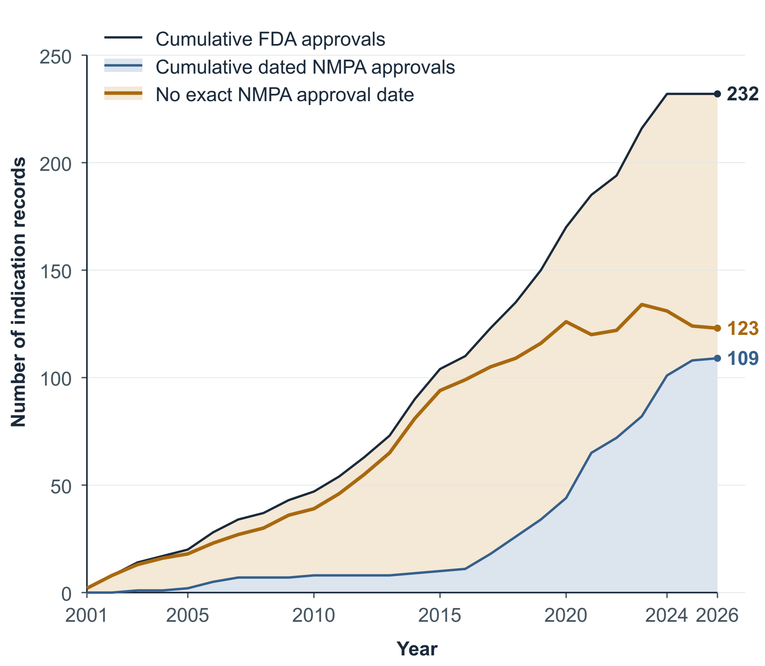
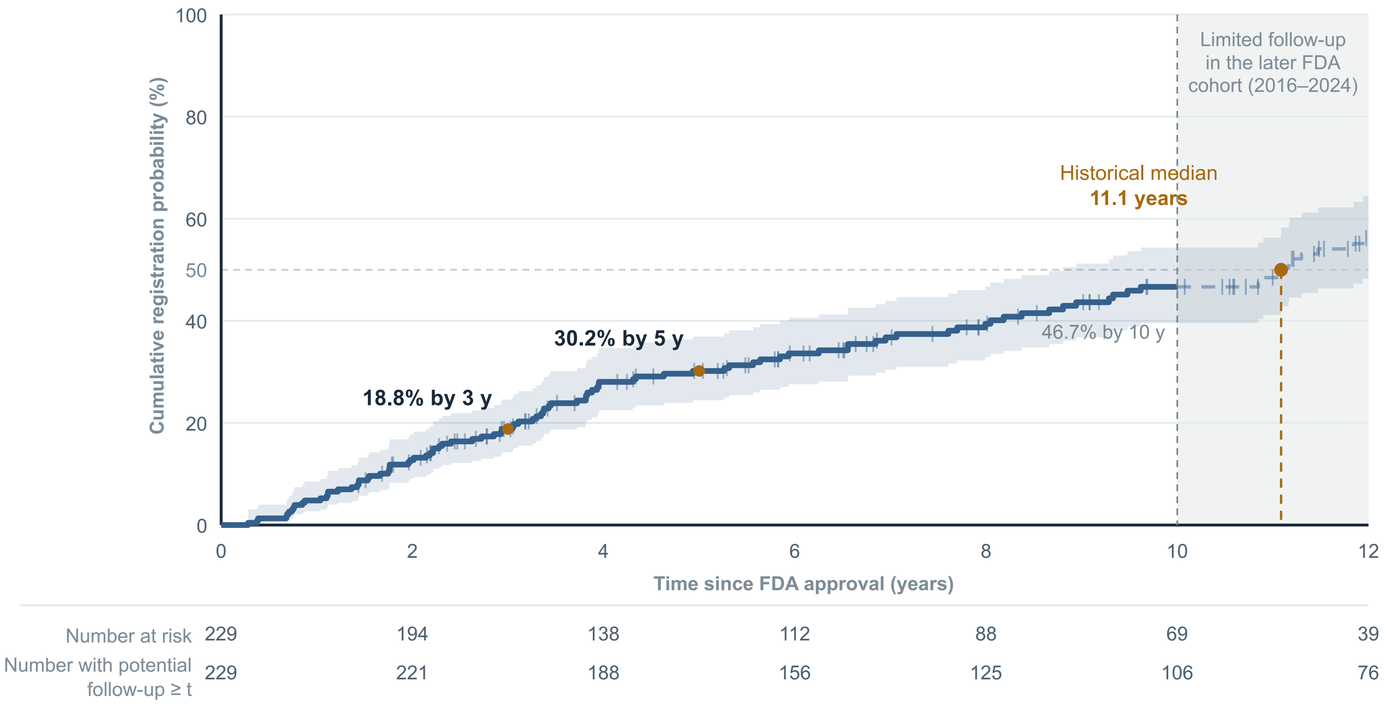
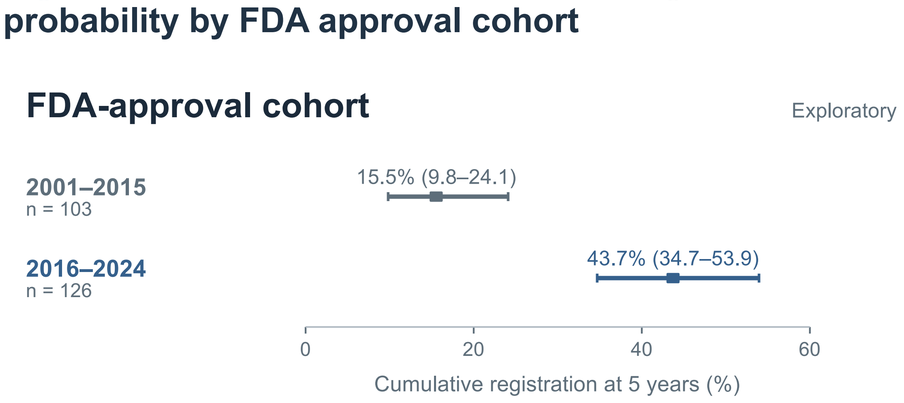


Figure 2. Cumulative probability of NMPA registration following FDA approval



1 − Kaplan–Meier survival estimate for 229 FDA-first records. Events: same-indication NMPA national registration with an exact approval date (n = 106). Censored at 28 May 2026: 123 records, including 10 confirmed registrations with unresolved dates. Shading: pointwise 95% CI (Greenwood, log–log).

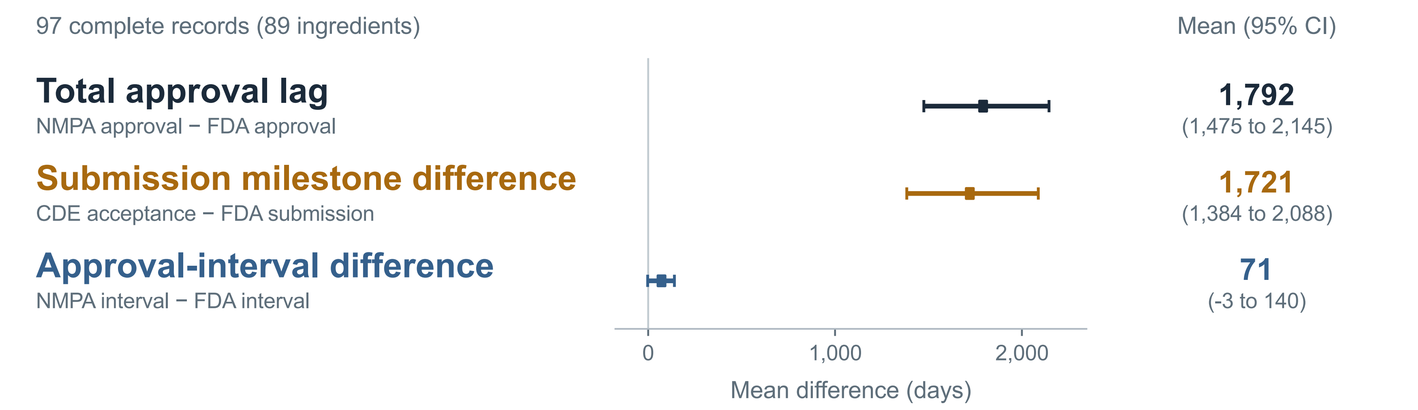
Figure 3. Five-year cumulative NMPA registration probability by FDA approval cohort



Restricted mean time without a dated registration event was **1,725 vs 1,377 days** (earlier vs later cohort). Difference: **348 days** (95% CI 226–464).

Points: 1−KM at five years. Bars: pointwise 95% CIs. Potential follow-up ≥5 years: 103/103 vs 71/126.

Figure 4. Decomposition of mean FDA-to-NMPA approval lag



1,792 = 1,721 + 71 days. The submission component represented **96.0%** of the mean total (95% CI 91.7–100.1%).

Means and 95% ingredient-cluster bootstrap CIs among 97/232 records with all four dates. Approval intervals run from CDE acceptance to NMPA approval and from FDA submission to approval.

CONCLUSIONS

1. **National registration:** 113/232 records (48.7%) had no confirmed registration. Another 10 had confirmed registration with unresolved approval dates.
2. **FDA-approval cohort:** the later cohort had higher estimated five-year registration (43.7% vs 15.5%). This exploratory comparison does not identify a policy effect.
3. **Complete records:** submission milestone differences accounted for most of mean approval lag. Generalization beyond the 97 complete records requires caution.

LIMITATIONS & DISCLOSURES

Missing CDE acceptance records do not prove no filing. Incomplete dates and later-cohort follow-up limit interpretation. Withdrawals and terminations were not tracked.

Source reconciliation and revised definitions after abstract acceptance changed the estimates. The cohort comparison is exploratory.

AI assisted code, and language under human oversight.

Contact: Dilei Yan, 3125044308@stu.cpu.edu.cn