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Supplementary information

Breakthrough therapy designations in China and the United States

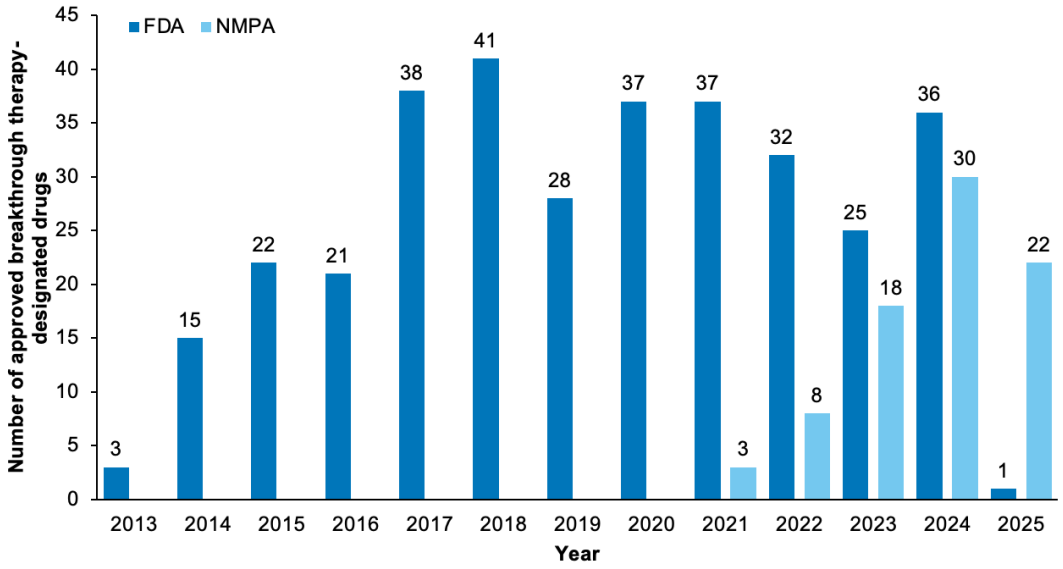
In the format provided by the authors and unedited

Data sources and analysis

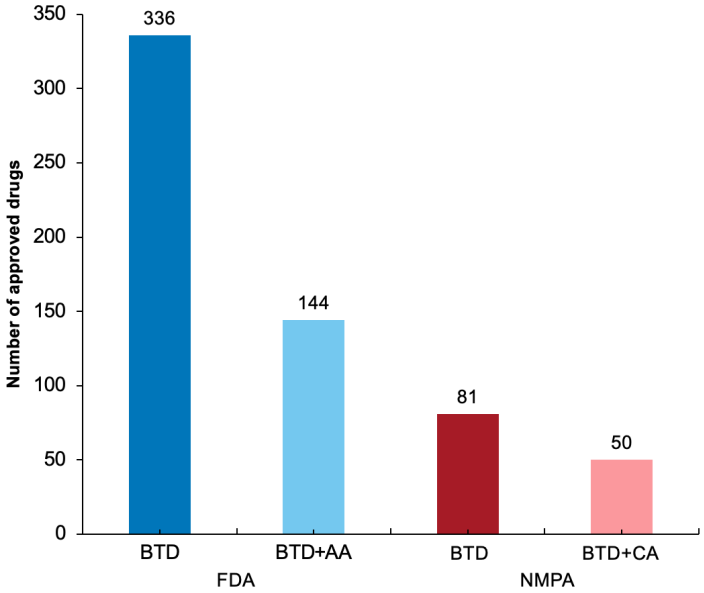
China. China's Breakthrough Therapy Designation (BTD) program was launched in 1 July 2020 and applies to new drugs intended for the prevention or treatment of life-threatening or seriously debilitating conditions for which there are no effective therapies, or where there is sufficient evidence demonstrating significant clinical advantages over existing treatments. Data on BTD designations granted in China through 1 July 2025 were obtained from the Center for Drug Evaluation (CDE) under the National Medical Products Administration (NMPA). Information on BTD-designated drugs that subsequently received marketing authorization was primarily extracted from the annual drug review reports published by the NMPA from 2020 to 2024. For approvals not explicitly mentioned in these reports, we traced each BTD-designated product individually to determine whether the designated indication had been granted approval. This process was supplemented by cross-referencing official press releases, drug-specific review reports, and publicly disclosed information from pharmaceutical companies. Each BTD designation was counted at the level of drug–indication pair, meaning that multiple indications for a single drug were treated as separate BTD designations.

US. In the US, the Breakthrough Therapy program was established under the Food and Drug Administration Safety and Innovation Act (FDASIA) on 9 July 2012. Data on the total number of BTD designations and approvals were retrieved from the FDA's Drugs@FDA database and include information from both the Center for Drug Evaluation and Research (CDER) and the Center for Biologics Evaluation and Research (CBER). For designation data, the fiscal year (1 October of the previous year through 30 September of the current year) was used as the reporting period. For approval data, the calendar year (1 January to 31 December) was applied. Due to differences in database update timelines, the CBER cohort includes designations from 9 July 2012 through 31 March 2025, while the CDER cohort spans 9 July 2012 through 31 December 2024. All data were independently extracted and cross-validated by two authors to ensure accuracy and consistency.

Identification of non-BTD drugs. All new drug approvals by the US FDA and China's NMPA between January 2021 and December 2023 were identified using the FDA's Drugs@FDA database and China's Center for Drug Evaluation Annual Drug Evaluation Reports. Key regulatory information was extracted from review reports, including the use of expedited regulatory pathways associated with each approval. For FDA approvals, expedited programs included accelerated approval, breakthrough therapy designation and priority review; for NMPA approvals, we included breakthrough therapy designation, conditional approval and priority review. Based on this information, we calculated the proportion of non-BTD drugs approved through the accelerated approval program in the US or the conditional approval pathway in China.



Supplementary Fig. 1 | Annual trends in the number of approved breakthrough therapy-designated drugs by the FDA and NMPA.



Supplementary Fig. 2 | FDA and NMPA-approved breakthrough therapy-designated drugs and numbers receiving accelerated approval (AA, FDA) or conditional approval (CA, NMPA).