CLOSING THE CLINICAL TRIAL & PATIENT ACCESS GAP

Fast-tracking Approval for Innovative Rare Disease Therapies (FAIR) Act

FAIR ACT

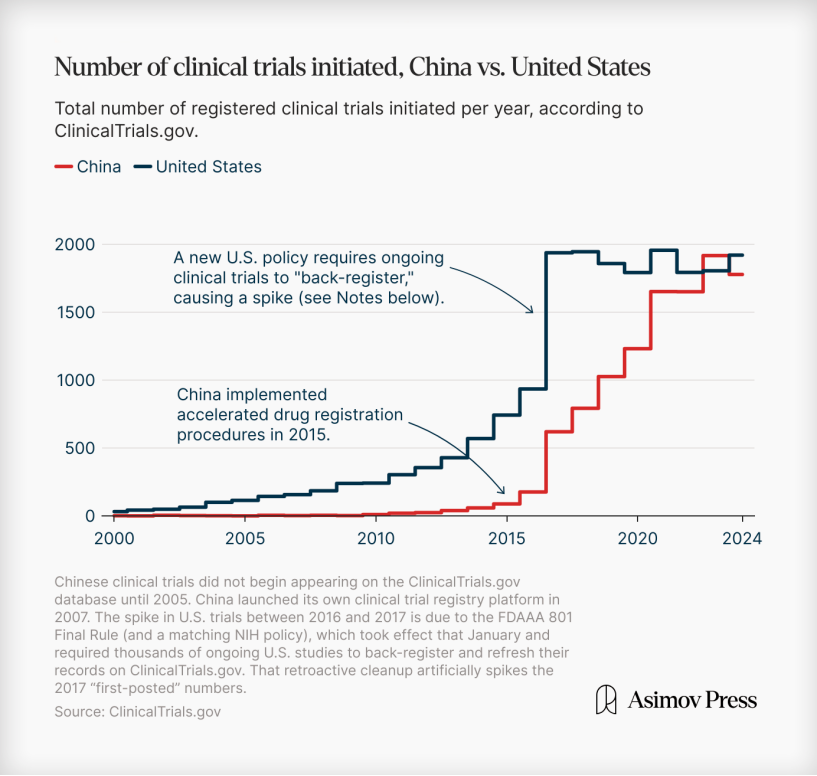
THE FAIR ACT

The biopharma industry is routinely opting to launch clinical trials outside of the United States[1] to avoid what can be a lengthy review and approval process at the U.S. Food and Drug Administration (FDA). The result is a growing imbalance in the number of clinical trials between the U.S. and the rest of the world. Already, China is dramatically ramping up the number of registered clinical trials[2] and has stated its intention to dominate the race for global biopharmaceutical innovation.[3] Simultaneously, Chinese companies are leveraging publicly available patent and press release data from U.S. biopharma companies to create copycat or fast-follow drugs.[4] As a result, China is poised to leapfrog the U.S. in medical innovation. There have been several historical examples of U.S. drug approvals lagging behind trusted nations and regulatory authorities, resulting in delays in patient access to innovative medicines, including life-threatening diseases with inadequate therapeutic options.[5]

Several biotech executives, investors, and consultants report that companies are now considering launching trials in markets such as the European Union and Australia, while engaging with those regions' regulators earlier in the drug development process.

"We know that across our companies, the discussions include whether to go ex-U.S. because of recent FDA uncertainty," said Peter Kolchinsky, managing partner at RA Capital Management, which manages approximately $9 billion in assets focused on biotech investments.

This sentiment is echoed by Sabrina Martucci Johnson, chief executive officer of Daré Bioscience, a women's health biotech company. "What's happening has forced all of us to discuss other approaches," Johnson stated. "We are definitely looking at Europe first for certain products where the need is great and the U.S. regulatory path has become more uncertain or slower."[6]



1. https://endpoints.news/us-drugmakers-seek-clinical-trials-abroad-as-confidence-in-fda-wavers/
2. https://www.fiercebiotech.com/biotech/chinas-share-trials-jumps-along-enrollment-times-iqvia-report
3. https://carnegieendowment.org/research/2025/01/biopharmaceuticals-rising-chinas-strategic-pivot-to-southeast-asia-amid-great-power-tech-competition?lang=en&center=china
4. https://www.statnews.com/2025/05/06/how-to-stop-the-shift-of-drug-discovery-from-the-u-s-to-china/
5. https://www.gene.com/media/press-releases/14893/2021-01-20/fda-grants-priority-review-to-genentechs
6. https://trial.medpath.com/news/bd2682d332f5c08f/us-biotech-companies-shift-clinical-trials-abroad-amid-fda-uncertainty

CLINICAL TRIAL GAPS

UniQure for Huntington's Disease

UniQure, a gene therapy company with multiple promising therapies in development, has released exciting and never-before-seen data for patients with Huntington's Disease, a rare, universally fatal neurological disorder. The company has been grappling with a public dispute with the FDA around use of control cohorts in clinical trials with an unclear path forward in the U.S. Meanwhile, in the UK, UniQure is planning to submit a Marketing Authorization Application (MAA) for their Huntington's therapy based on their current three-year data, which will likely lead to a substantial approval gap between Europe and the U.S.[7]

GH Research for Treatment-Resistant Depression, Bipolar II Disorder, & Postpartum Depression

GH Research has an ongoing investigational clinical trial for an inhaled product. To date, the product, GH001, has shown ultra-rapid antidepressant effect with a 57.5% remission rate.[8] Currently, the study, which is recruiting in the UK, is not being allowed to proceed in the U.S. due to FDA toxicology requirements.[9]

Candid Therapeutics for Rheumatoid Arthritis

Candid is a San Diego-based biotechnology company seeking to develop multiple product candidates for rheumatoid arthritis and other autoimmune and autoantibody diseases.[10] Due to challenges with the FDA, Candid has launched their clinical trial at multiple sites in Europe and New Zealand but provides no options for U.S. sites.[11]

Anonymous Biotech for Oncology Clinical Trials

One biotech CEO, speaking anonymously, revealed plans to seek European Medicines Agency (EMA) approval for early-stage oncology trials across three European countries—in addition to an ongoing U.S. trial. This expanded European strategy will cost approximately $1 million in additional filings, consultants, and research organization support, plus several million more to run the trials. "We cannot just hope that things will turn around and that the cuts at the FDA will not have any impact on our business," the executive explained. "The irony of this is it goes against the grain of 'America First', because we are offshoring away from the U.S. over to Europe."[12]

7. https://www.uniqure.com/investors-media/press-releases
8. https://investor.ghres.com/news-releases/news-release-details/gh-research-announces-primary-endpoint-met-phase-2b-trial-gh001
9. https://www.globenewswire.com/news-release/2025/07/23/3120164/0/en/GH-Research-Announces-Global-Pivotal-Program-Plans-and-Further-Development-Updates.html
10. https://www.candidrx.com/pipeline/pipeline-overview/
11. https://clinicaltrials.gov/study/NCT07052032?spons=Candid%20Therapeutics&rank=3
12. https://trial.medpath.com/news/bd2682d332f5c08f/us-biotech-companies-shift-clinical-trials-abroad-amid-fda-uncertainty

APPROVED PRODUCT GAPS

Sydnexis for Pediatric Myopia

Sydnexis was granted marketing authorization approval in June 2025 by the EMA for a novel pediatric medicine called *Ryjunea*.[13] In October 2025, the FDA sent Sydnexis a Complete Response Letter (CRL), questioning the product's efficacy, in spite of the fact that the drug reached its primary and secondary endpoints.[14]

Egetis Therapeutics and Emcitate for MCT8 Deficiency

MCT8 deficiency is a highly debilitating ultra-rare disease. Egetis developed *Emcitate*, a novel medicine that received marketing authorization from the EMA in December 2024. The FDA is requiring Egetis to conduct additional studies in 16 patients.[15]

Amryt Pharma (Chiesi) for Epidermolysis Bullosa (EB)

EB is an ultra-rare and fatal disease with few treatments. In June 2022, the EMA provided marketing authorization for *Filsuvez*, the first approved treatment for junctional and dystrophic EB.[16] The same product was eventually approved for marketing in the U.S., 1.5 years later in December 2023.[17]

Amicus Therapeutics for Late-onset Pompe Disease

Pompe disease is a rare and fatal disease with high unmet medical need. In March 2023, the EMA approved *Pombiliti*, an orally administered enzyme replacement therapy.[18] The same product was approved nearly seven months later by the FDA in late September 2023.[19]

InterMune/Roche/Genentech for Idiopathic Pulmonary Fibrosis (IPF)

IPF is a rare and universally fatal lung condition. Pirfenidone was first approved by the EMA in 2011, and not yet approved by the FDA until 2014, a three-year delay. The FDA required the sponsor to conduct an additional Phase 3 clinical trial, which caused the yearslong delay.

Janssen for Soft Tissue Sarcomas

In 2007, Europe approved trabectedin[20] for patients who had failed standard therapies. The FDA declined to approve the drug in 2009, but it was later approved in the U.S. in 2015, an eight-year delay.[21]

13. https://www.ema.europa.eu/en/medicines/human/EPAR/ryjunea
14. https://www.ophthalmologytimes.com/view/fda-issues-complete-response-letter-to-sydnexis-for-syd-101-in-pediatric-myopia
15. https://www.egetis.com/pipeline/
16. https://www.ema.europa.eu/en/medicines/human/EPAR/filsuvez#authorisation-details
17. https://chiesirarediseases.com/media/fda-approval-for-filsuvez-topical-gel
18. https://www.ema.europa.eu/en/medicines/human/EPAR/pombiliti#authorisation-details
19. https://www.nasdaq.com/press-release/amicus-therapeutics-announces-fda-approval-and-launch-of-new-treatment-for-pompe
20. https://www.ema.europa.eu/en/medicines/human/EPAR/yondelis
21. https://www.jnj.com/media-center/press-releases/us-fda-approves-yondelis-trabectedin-for-the-treatment-of-patients-with-unresectable-or-metastatic-liposarcoma-or-leiomyosarcoma-two-common-subtypes-of-soft-tissue-sarcoma

REV JUNE 2026