Rising Levels of Innovation in Expedited Designations Among FDA-Approved Drugs, 2012-2025

Authors

Beck Miller, Naushin Hooda, Ivar Jensen, Bob Nordyke

Objectives

- Drug affordability is a critical issue. Some observers point to rising launch prices for new drugs as a barrier to patient access.
- This concern may have validity if new drugs are equivalent to existing drugs and treat the same conditions and patients in similar ways.
- FDA grants certain drugs expedited designations to accelerate patient access to promising therapies that demonstrate potential to improve outcomes in serious, rare, or high unmet need conditions.
- This research aims to evaluate FDA's review and approval designations for drugs approved throughout 2012-2025 to assess trends in the innovativeness of new drugs.

Conclusions

- Trends in FDA review designations indicate that innovativeness measures of new drugs are stable or increasing.
- Innovation in small molecules is rising: Small molecule drugs account for over 62% of all CDER-approved drugs 2015-2025
- The US preference for innovative medicines is reflected in FDA approvals: Drugs first approved in the US account for 75% of all CDER-approved drugs that received ≥1 expedited approval and 75% of all CDER-approved with First-in-Class designation.
- Launch price assessments should account for the scientific advancements inherent in new products that reflect continuing innovation and meet clinical needs better than prior treatments.

BlueRidge LIFE SCIENCES

Methods

Data on initial approvals and review designations for FDA-approved drugs were abstracted from CDER's New Drug Therapy Approvals annual reports and CBER's Biological Approvals by Year website, with supplemental data from FDA press releases, 2012-2025. FDA designations for expedited review, including Accelerated Approval, Breakthrough Therapy, Fast Track, Orphan, Regenerative Medicine Advance Therapy, and Priority Review, were identified. First-in-Class (FIC) designations for drugs approved by CDER throughout 2015-2025 were also recorded. Blood products, diagnostic agents, and human tissue products were excluded.

Results

683 new approvals were included, 490 of which were approved by CDER throughout 2015-2025. Figure 1 shows slight upward trends in new approvals that received at least one expedited review designation and CDER approvals with FIC designation.

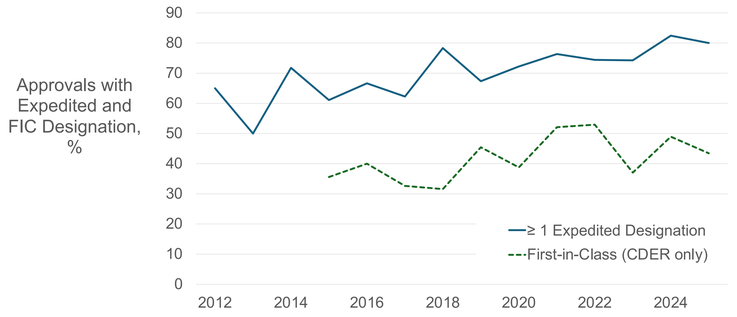

Figure 1. CDER and CBER approvals with ≥1 expedited review and First-in-Class designations

Figure 2 depicts approvals with ≥1 expedited designation by therapeutic area (TA). The mean for all approvals was 71%. Oncology (94%), rare (90%) and Infectious Diseases (77%) were above the overall mean, while Cardiovascular Disease (65%) followed. Other TAs had 35-40% of new approvals receive ≥1 designation.

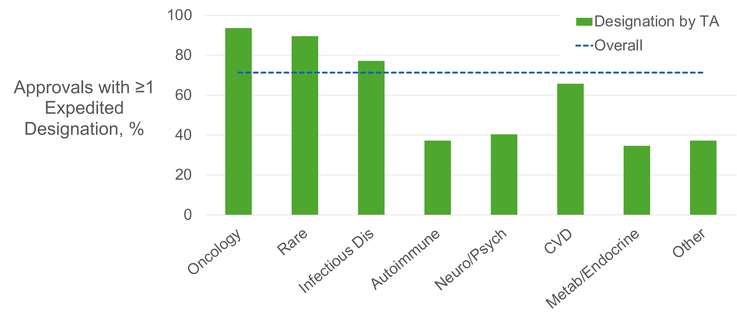

Figure 2. CDER and CBER approvals with ≥1 expedited designation

Accelerated approvals, and their confirmatory trials, are subject to recurring debate. Overall, 17% of included drugs received Accelerated Approval. Oncology approvals had the highest rate (44%); no other TA had more than an 8.8% (Infectious Diseases) rate of accelerated approvals.

CDER designated 41% of approvals FIC (Fig. 3). Drugs for Rare Diseases led (66%), followed by Metabolic/Endocrine (53%) and CVD (50%).

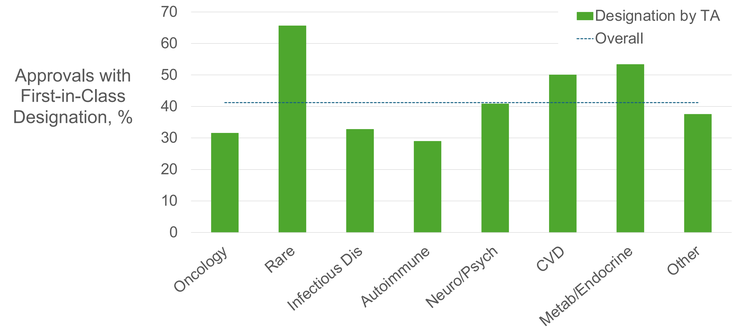

Figure 3. CDER approvals with First-in-Class designation

Fig. 4 shows that small molecules account for the greatest number of CDER-approvals with FIC designation, with substantial growth over the study period.

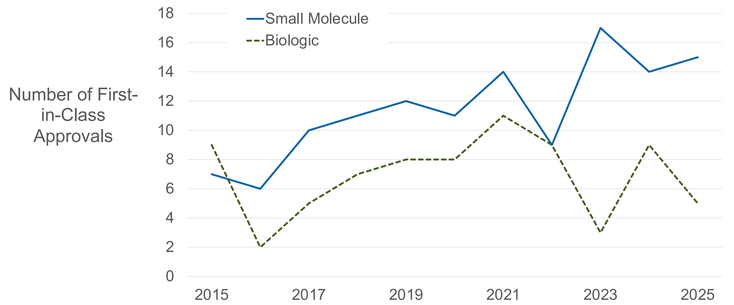

Figure 4. Number of CDER-approved drugs with FIC designation

Table 1 depicts expedited and FIC designations stratified by where the drug was first approved globally. Most drugs deemed innovative by the FDA were first approved in the US.

	First Approval, by Market	
	US	**Ex-US**
Total CDER Approvals	349	141
Any Expedited, N (%)	271 (77.7)	91 (64.5)
First-in-Class, N (%)	151 (43.3)	51 (36.2)

Table 1. Expedited and FIC designations by location of first global approval (CDER only)