FDA Approvals of Specialty Drugs, 2000-2024

Knox SE[1], Brennan CH[1], Enright DE[1], Neumann PJ[1], Chambers JD[1]

1. Center for the Evaluation of Value and Risk in Health, Tufts Medical Center, Boston, MA

HPR159

BACKGROUND & OBJECTIVE

- Specialty drugs account for less than 5% of prescriptions yet represent 54% of pharmaceutical spending (~$263 billion in 2024), up from 47% in 2019. [1]
- The median annual list price of drugs launched in 2024 exceeded $350,000, with oncology and rare disease therapies often surpassing $400,000 per patient, raising concerns about health care budget sustainability.
- We analyzed 25 years of FDA drug approvals to estimate the proportion classified as specialty drugs and characterize key attributes, including orphan status and therapeutic area.

METHODS

Data Source and Methods

- We identified novel drug and biologic approvals from 2000-2024 in the FDA's New Molecular Entity (NME) database, excluding drugs later withdrawn from the market.
- We assigned specialty status using lists (as of September 2025) from the three largest pharmacy benefit managers (PBMs): CVS Caremark, Express Scripts, and OptumRx. A drug was considered specialty if it appeared on at least one PBM list.
- Cell and gene therapies were classified as specialty due to their high costs and complex management requirements.
- For each specialty therapy, all initial and follow-on indications were categorized by orphan status, cancer indication, and FDA expedited review pathway.

Analyses

- We used Poisson regression to test temporal trends in annual specialty drug approvals, adjusting for total annual approvals using a log offset.

RESULTS

Figure 1. Specialty Drug-Indication Approvals 2000-2024 (n=947) by Orphan Status and Cancer Indication

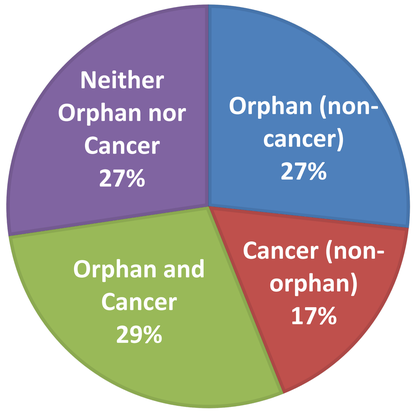
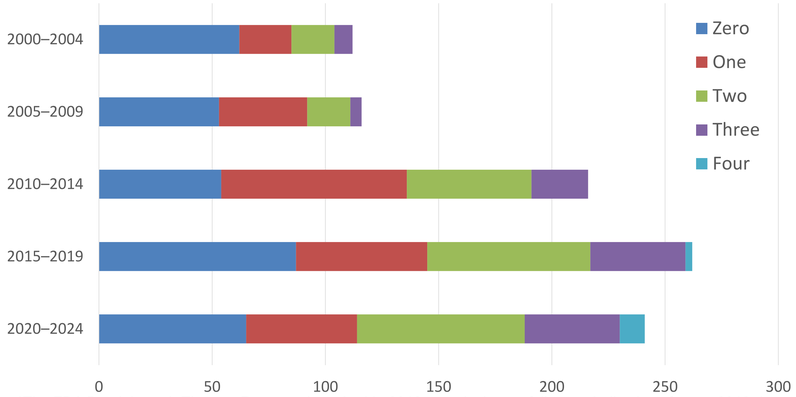
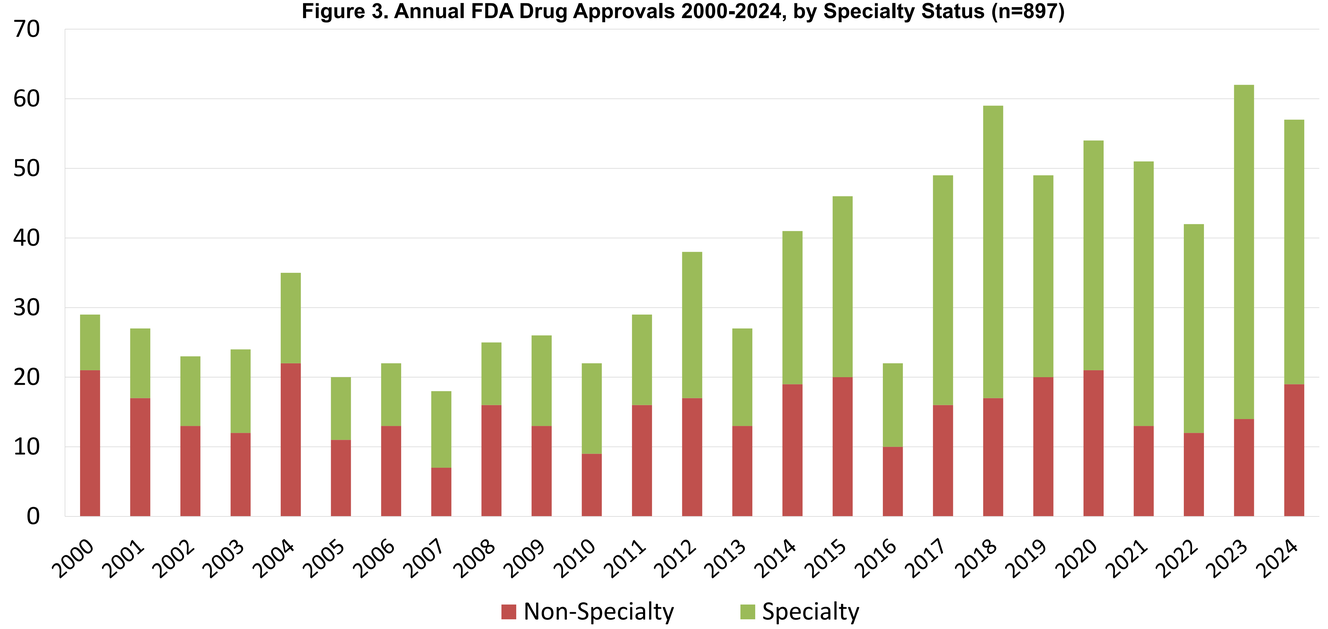


Figure 2. Number of Expedited Review Programs Used for Specialty Drug-Indication Approvals 2000–2024 (n=947)



*The FDA Breakthrough Therapy Program, launched in 2012, can designate follow-on indications for pre-2012 drugs. This happened twice for drugs approved in 2000-2004 and three times for those approved in 2005-2009.

Figure 3. Annual FDA Drug Approvals 2000-2024, by Specialty Status (n=897)



- Between 2000 and 2024, the FDA approved 897 novel drugs, of which 516 (57%) were classified as specialty. Specialty drug approvals increased at approximately 3% per year (β = 0.0295, P <0.001).
- Including follow-on approvals, the FDA approved 947 specialty drug-indication pairs. Of these, 188 therapies (36%) received at least one follow-on indication (mean 1.7 indications per therapy).
- Among specialty drug-indication pairs, 55% (525) had an orphan designation and 46% (435) were for cancer.
- The FDA granted expedited reviews to 515 pairs (54%) through Priority Review, 198 (21%) through Fast Track, 193 (20%) Accelerated Approval, and 245 (26%) through Breakthrough Therapy.

CONCLUSION

- The FDA has approved significantly more specialty therapies over time, both in absolute numbers and as a share of all approvals, reflecting a shift to complex, high-cost drug development.
- FDA approvals in 2025 suggest continuity with these longer-term patterns: 74% of new drugs were approved via expedited pathways and 52% receiving orphan designation.

REFERENCES

- IQVIA Institute for Human Data Science. Understanding the Use of Medicines in the U.S. 2025: Evolving Standards of Care, Patient Access, and Spending. IQVIA; 2025.

FUNDING

This research had no outside funding.

CONTACT

For more information about the SPEC Database,
please contact James Chambers
at james.chambers@tuftsmedicine.org.