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Trends in Health Gains, Costs, and Value from Pharmaceutical Innovation, 1999–2022

Pharmaceutical innovation is often measured by the number of new drugs approved, research and development activity, or spending on new medicines. These measures do not directly capture the incremental health gains, costs, or value associated with new therapies. We examined the magnitude, distribution, and evolution of patient-level health gains, incremental costs, and cost-effectiveness associated with drugs approved by the US Food and Drug Administration (FDA) from 1999 through 2022, and assessed how these outcomes varied across different types of pharmaceutical innovation.

We used the Tufts Medical Center Health Gains IQ Database, which identifies FDA-approved drugs and corresponding published cost-effectiveness and comparative-effectiveness studies reporting health benefits in quality-adjusted life-years (QALYs). The primary unit of analysis was the drug-indication pair. To approximate the incremental benefit and cost at the time of market entry, included studies compared newly approved drugs with the standard of care available at the time of approval, or with best supportive care when no active treatment was available. We summarized incremental QALY gains, costs, and incremental cost-effectiveness ratios (ICERs) overall and compared these outcomes between cancer and non-cancer indications. Planned analyses will compare these outcomes across additional drug and indication characteristics, including orphan status, molecule type, first versus follow-on indication, and first-in-class versus next-in-class therapies. We also examined longitudinal trends in incremental QALY gains, costs, and ICERs according to year of FDA approval. Planned multivariable analyses will assess which drug and indication characteristics are independently associated with health gains, costs, and cost-effectiveness.

Across 585 drug-indication pairs approved between 1999 and 2022, the median incremental health gain was 0.16 QALYs (interquartile range, 0.52). Overall, 23.6% of newly approved drug-indication pairs were associated with no incremental health gain or worse outcomes relative to the comparator, while 11.29% generated gains of at least 1 QALY.

Incremental health gains increased significantly over time, with median incremental QALYs increasing by 0.011 per approval year (β=0.0109; 95% CI, 0.0053–0.0165; P<0.001); this trend was also significant using Cuzick's nonparametric test (z=4.403; P<0.001). Incremental costs also increased over time, by a median of $1,972 per approval year (β=$1,971; 95% CI, $1,156–$2,788; P<0.001), with a significant trend also observed using Cuzick's test (z=6.163; P<0.001). Median ICERs increased by $7,129 per QALY per approval year in quantile regression (β=$7,129; 95% CI, $1,633–$12,626; P=0.011), although the trend was not statistically significant using Cuzick's nonparametric test (z=1.654; P=0.098).

Health gains, costs, and cost-effectiveness also differed between cancer and non-cancer indications. Median incremental QALY gains were higher for cancer than non-cancer indications (0.35 vs 0.04 QALYs; Mann-Whitney U test, P<0.0001), as were median incremental costs ($82,902 vs $4,128; P<0.0001). Median ICERs were also numerically higher for cancer indications ($232,140 vs $134,032 per QALY), although this difference was not statistically significant (P=0.1529). The overall distributions differed significantly for incremental QALYs, incremental costs, and ICERs (Kolmogorov-Smirnov test, all P<0.0001).

We will also conduct additional univariate analyses comparing health gains, costs, and cost-effectiveness across drug characteristics, including orphan versus non-orphan status, small molecules versus biologics, and first-in-class versus next-in-class therapies. In planned multivariable analyses, we will examine which characteristics are independently associated with larger health gains, higher costs, and more or less favorable cost-effectiveness after accounting for changes over time in the composition of newly approved drugs.

The health gains, costs, and value associated with newly approved medicines vary substantially. Nearly one-quarter of drug-indication pairs provided no incremental health gain or worse outcomes relative to existing treatment, while a smaller share produced large gains in patient health. Encouragingly, incremental QALY gains increased over time, but incremental costs also rose substantially. ICERs increased in the quantile regression analysis, although evidence of a longitudinal trend was less consistent across statistical tests. These findings suggest that newer drugs have delivered greater health gains, but at increasingly higher costs, and provide a broader view of the productivity and value of the pharmaceutical innovation system.

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