Trade-offs in paying for new drugs in health systems

An evaluation of new drugs recommended by the National Institute for Health and Care Excellence (NICE) in England, 2000-2020

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Background

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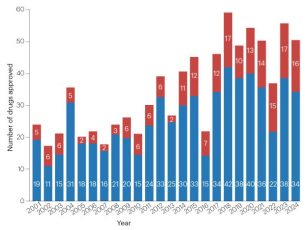
A growing number of new drugs are entering the market every year



The average number of new drug approvals per year now stands at an average of 45 to 50

Ref: Mullard A. Nature Reviews Drug Discovery 24, 75-82 (2025)

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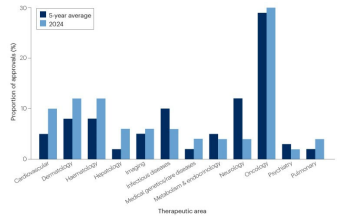
Cancer drugs stand out as the single largest category of new approvals



Cancer drugs now account for nearly one-third of new drug approvals.

Ref: Mullard A. Nature Reviews Drug Discovery 24, 75-82 (2025)

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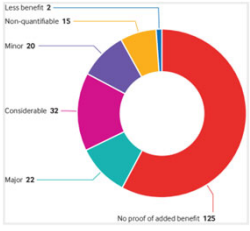
The growing number of new drug approvals has not been indicative of therapeutically beneficial innovation.



More than half of new drugs lack proof of added benefit over existing treatments.

Less than a quarter of new drugs have proof of considerable or major added therapeutic benefit over existing alternatives.

Ref: Wieseler B et al. *BMJ* 2019;366:l4340

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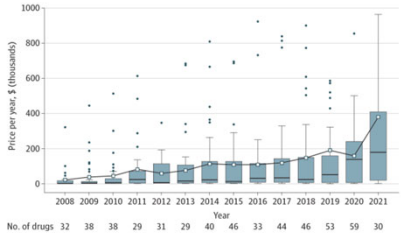
Launch prices for new drugs increased by 20% per year in last decade.



In 2020-2021, nearly half of new drugs were initially priced above $150 000 per year

Ref: Rome BN et al. *JAMA*. 2022;327(21):2145-2147

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A growing challenge for policymakers

- The combination of uncertain therapeutic benefits and extremely high prices presents policymakers with significant challenges for which there is little historical precedent.

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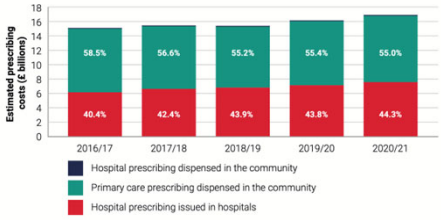
In the UK, the recent rate of growth (5.5%) in spending on pharmaceuticals far exceeded the rate of growth (1.4%) in total NHS budget.



Ref: NHS Digital

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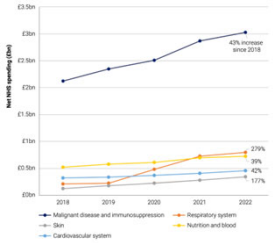
Five therapeutic areas drove the highest growth in hospital pharmaceutical spending during the 2018-2022 period.



Ref: Angelis A et al. Promoting population health through pharmaceutical policy. 2023. Available on the LSE website

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The National Institute for Health and Care Excellence (NICE)

- NICE was established in 1999 to address 'post-code lottery' in access to new drugs (and other health technologies) in England.
- NICE evaluates new drugs following regulatory marketing authorisation.
- NICE's remit is to make funding recommendations for the National Health Service (NHS) in England.
- Local decision makers in England are mandated to make funding available for treatments recommended by NICE within a specified timeframe.

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NICE and value for money

- NICE bases its recommendations on an assessment of population benefits and value for money.
- NICE explicitly mentions that its assessments consider the 'opportunity cost' of recommending one intervention instead of another.

Image credit: BMJ. 2004; 329(7459): 227–229.

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NICE and quality-adjusted life years (QALYs)

- NICE assessments compare the *clinical-effectiveness* and *cost-effectiveness* of new versus existing treatments.
- NICE measures health benefits (*clinical-effectiveness*) in terms of quality-adjusted life years (QALYs).
- QALYs are an overall measure of health that weigh the **life expectancy** of an individual with an estimate of their **health-related quality of life**.
- 1 QALY = 1 year spent in perfect health.

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NICE and cost-effectiveness analysis

- NICE estimates the new treatment's costs and how much benefit it produces compared with the next best alternative:

$$\textit{Incremental cost-effectiveness ratio (ICER)} = \frac{Cost_{new\ treatment} - Cost_{alternative\ treatment}}{Benefit_{new\ treatment} - Benefit_{alternative\ treatment}}$$

- Expressed as cost (in £) per additional QALY gained.
- NICE considers drugs with an incremental cost-effectiveness ratio between £20,000 and £30,000 per QALY gained as cost-effective.
- There is no empirical evidence to support NICE's current funding threshold.

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Motivation for the study

- Econometric analyses examining the relationship between NHS expenditures and health outcomes have provided empirical estimates of the health system's marginal productivity.
- NHS in England spends approximately £15,000 to generate one additional QALY.[1]
- **Paying more than £15,000 per QALY would represent poor value for money and do more harm than good to population health.**
- Our aim was to quantify the net health benefits of NICE-recommended new drugs, considering both the value for money of new drugs and the opportunity cost of resources required to pay for them.

1. Claxton K et al. Health Technol Assess. 2015 Feb;19(14):1-503.

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Study objectives

- To document the value for money of new drugs recommended between 2000 and 2020.
- To characterise the health benefits of new drugs over time and by therapy area.
- To estimate the population health impact of NICE-recommended new drugs by therapy area.

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Methods (in brief)

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Estimating the net health benefits of new drugs

The amount of NHS spending for a new drug is determined by NICE's funding threshold for recommending that drug.

£30,000 per QALY gained

1000 patients receive the drug
1000£30,000 = £30,000,000*

1000 QALYs

Estimated health benefits gained from using the new drug in the NHS

1 additional QALY

—

2000 QALYs

Estimated health benefits that can theoretically be gained from reallocating that same money to other services in the NHS

2 additional QALYs

Net health effect
the difference between what is gained with new drug vs what could be gained by reallocating the funding to other services

Negative net benefit
(-1 QALY)
(-1000 QALYs)

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Data and approach

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New drugs and indications appraised by NICE	**Value for money (ICER)** **Incremental health benefits of new drugs** **Incremental costs of new drugs**	**Numbers of patients receiving new drugs**
Publicly available information from: ➢ EMA website ➢ NICE documents	Publicly available information from: ➢ NICE documents ➢ Other HTA organizations ➢ Peer-reviewed literature	Proprietary data from IQVIA + Publicly available data from peer-reviewed literature

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Results

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Final analysis sample

We included **183** new drugs → approved in **287** indications → with **339** appraisals

- Appraisals for cancer drugs accounted for nearly half of all appraisals (46%).
- More than half of appraisals were published between 2015-2020.

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Health benefits of new drugs over time

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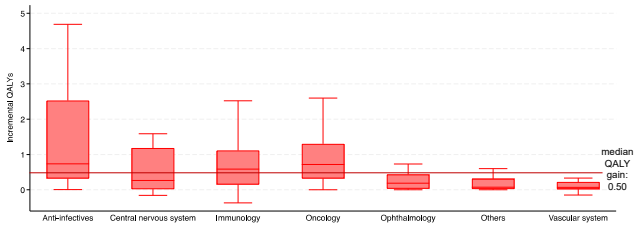
Health benefits of new drugs by therapy area



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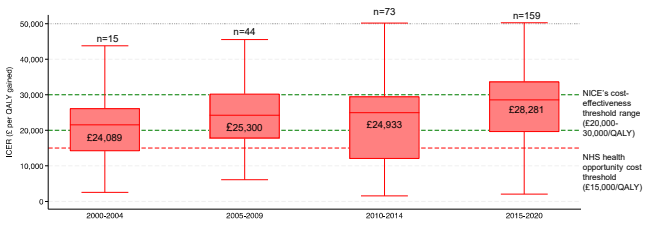
Value for money over time



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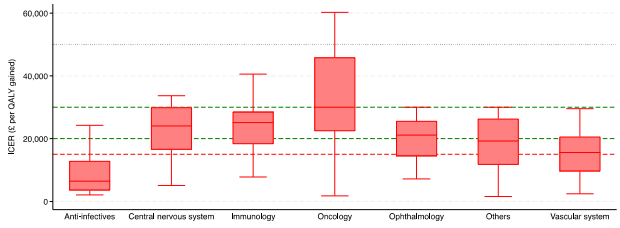
Value for money by therapy area



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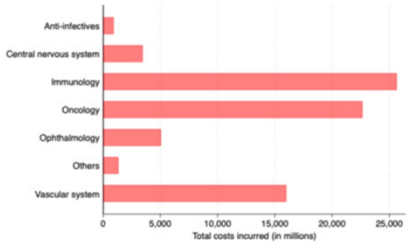
Total additional costs of new drugs



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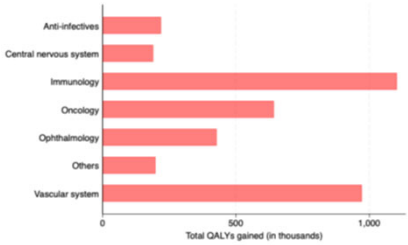
Total additional benefits of new drugs



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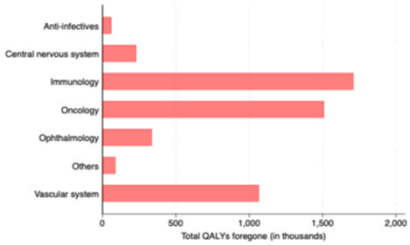
Total foregone health benefits



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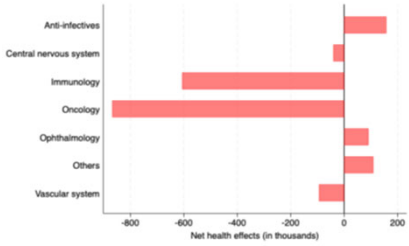
Net health effects of new drugs



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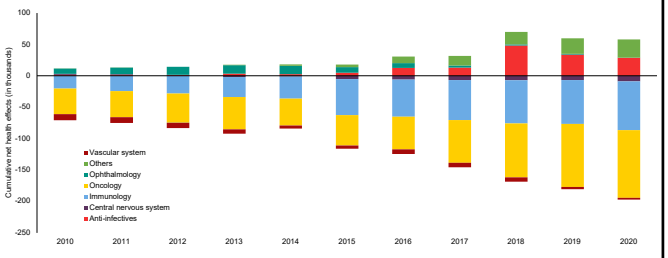
Population health impact of new drugs over time and by therapy area



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Discussion

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Discussion (I)

- During 2000–20, NHS coverage of new drugs displaced more population health than it generated.
- Results highlight the trade-offs and implicit prioritisation of people who directly benefit from new drugs at the expense of people who do not.
- NICE frequently recommends drugs with ICERs that exceed its regular cost-effectiveness threshold.
- NICE prioritises patients who could benefit from new drugs, valuing their health gains more than those of patients whose needs can be met elsewhere in the NHS.

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Discussion (II)

- There is a discrepancy between NICE's overt commitment to the principle of opportunity cost and population benefits and the NHS opportunity cost.
- One policy option is lowering NICE's funding threshold.
- Presenting committee recommendations relative to the health-opportunity cost threshold would convey the trade-off between individuals who directly benefit and other NHS users.
- Example:
  - NICE recommended trastuzumab for treatment of people with metastatic gastric cancer in 2010, at £43 206 per QALY.
  - This can be expressed as 2.88 QALYs forgone per QALY gained.

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Discussion (III)

- English NHS operates under a limited budget and intense resource constraints.
- The most critical performance indicators of the NHS are no longer met.
- Paying high prices for new drugs can adversely affect population health in this environment.
- The concept of opportunity cost is relevant for other health systems as well.

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Conclusions

- A growing proportion of NICE appraisals recommended funding of new drugs at levels considerably above the health system's opportunity cost threshold.
- Overall, the cumulative net benefit of NICE-recommended new drugs on population health in England appears to be negative.

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Articles

Population-health impact of new drugs recommended by the National Institute for Health and Care Excellence in England during 2000–20: a retrospective analysis

Huseyin Naci, Peter Murphy, Beth Woods, James Lomas, Jinru Wei, Irene Papanicolas

Lancet 2025; 405: 50-60

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