

## **Briefing paper: Who decides the price and availability of NHS medicines?**



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The Centre for Health and the Public Interest (CHPI) is an independent think tank committed to health and social care policies based on accountability and the public interest.

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## Introduction

1. Outrage and dismay over the prices charged for new medicines are becoming an increasingly regular occurrence in England. Most recently it was over a life-extending drug for cystic fibrosis, Orkambi, with a hefty price tag of £105,000 per patient per year – far higher than NHS England's counter offer of £500m over 5 years across all patients. Unfortunately, the number of pricing disputes will increase in future but, as this briefing will explain, the price and availability of medicines is not simply a matter of recouping the costs of development but also reflects the tension between pharmaceutical companies, purchasers (e.g. the NHS), and patients' representative groups.
2. Once a new medicine has been identified and trialled it needs to pass two hurdles before being sold. Firstly, it needs to be shown to be safe, therapeutic, and meet certain quality standards. Most pharmaceutical companies opt to have this done centrally by the European Medicines Agency, where if approved the medicine can then be marketed across Europe. Secondly, the medicine needs to be accepted for reimbursement by those who purchase it, sometimes patients directly but usually insurance companies or public purchasers such as the NHS. Unlike with the approval for safety and quality, the decision over whether and how much to pay for a medicine is decided by national governments or insurance companies in negotiation with pharmaceutical companies. So when going into negotiations what are the incentives of the pharmaceutical companies?

## Pharmaceutical companies

3. As with all for-profit businesses the companies will want to maximise their profits for the cost of producing the medicine. In addition, they will want to recoup the development costs of any medicines which never made it through testing (i.e. were found to be unsafe, ineffective, or of low quality) which can be as many as seven out of every eight medicines chosen for testing.<sup>1</sup>
4. In order to encourage research into new medicines, companies are granted patents, usually for seventeen to twenty years, which gives them the sole right to sell a medicine and thus charge monopoly prices to recoup their costs and make profits. Usually the first nine to eleven years of the patent are spent trialling the medicine and getting it ready for sale. With the remaining patent years, the pharmaceutical company will want to maximise its profits through setting a low enough price to attract purchasers but high enough to make substantial profits on each purchase made.

<sup>1</sup> DiMasi, J.A., Grabowski, G., and Hansen, R.W. (2016). 'Innovation in the pharmaceutical industry: New estimates of R&D costs', *Journal of Health Economics*, (47), pp.20-33. Doi: 10.1016/j.jhealeco.2016.01.012

5. However, prices are not necessarily driven by costs. Prices used to be negotiated on a cost-based approach with a mark-up over the manufacturing costs, but the costs of a medicine are not easily isolated and identified, especially any research costs which were not medicine specific. Instead the price (and associated profits) that the pharmaceutical company thinks that purchasers are willing to pay will determine whether it will invest in medicines with higher development costs, so the anticipated price often determines costs (or the investment in the drug), and not vice versa.<sup>2</sup>
6. Given the lack of clarity over costs, price setting is therefore a negotiation between pharmaceutical companies and national governments or insurance companies. As such there is often no one universal headline ('list') price for each medicine around the world. The price set will vary depending on factors such as the size of the market and how much the patient contributes to the cost of their own medicines (i.e. co-payments).
7. Countries with smaller markets or with high co-payments are likely to buy less of the medicine and so are in a weaker negotiating position compared to a larger country/market which can negotiate lower prices in return for more sales. To help protect themselves against price discrimination of this type, many countries use 'international reference pricing' which typically involves setting the maximum price that they are willing to pay for a medicine based upon the (average, lowest, or same) prices of similar (or comparable) medicines in other 'reference countries'.
8. Generally the reference countries chosen have larger markets and so greater negotiating power. For EU countries, France, the UK, and Germany are the most commonly included reference countries due to their size.<sup>3</sup> Whilst the use of reference countries does allow smaller markets to cap their prices it also has the effect of encouraging pharmaceutical companies to delay launching medicines in reference countries where any drop in prices will have an impact on the price it can charge in others. This can explain why some medicines are launched later in the UK than in other EU countries and why pharmaceutical companies are loathe to drop their list prices for medicines sold here, given a concession in the UK will have a domino effect on the prices charged overseas.
9. To avoid this bind the NHS negotiates discounts without dropping the list price used a voluntary Pharmaceutical Price Regulation Scheme (PPRS).<sup>4</sup> This applies to all branded medicines in the UK and sets a cap on the level of NHS spending on branded medicines. When this cap is exceeded the manufacturers refund ('rebate') some of the income received from the NHS.

<sup>2</sup> As an example, it has been estimated that over 33% of the total R&D cost of a new medicine is due to the costs of capital, i.e. the time cost of investing, which grows the longer the medicine is in development. See here for further information: <https://www.ohe.org/publications/rd-cost-new-medicine>

<sup>3</sup> Rémuzat, C. *et al.* (2015). 'Overview of external reference pricing systems in Europe', *Journal of Market Access & Health Policy*, 3(1). Doi: 10.3402/jmahp.v3.27675

<sup>4</sup> From 1<sup>st</sup> January 2019, the PPRS has evolved into a similar arrangement called the 2019 Voluntary Scheme.

This allows a higher list price to be set for the NHS whilst the actual price paid overall is lower due to the rebates. However as with all schemes there are issues over the size of the cap, the way rebates are assigned, and the fact that the rebates are kept at the national level.

10. In summary, pharmaceutical companies will look to maximise profits through balancing prices and the number of sales but are wary of the impact of price cuts in reference countries. So how do purchasers decide whether they want to use a medicine and at what price?

## Purchasers (e.g. the NHS)

11. The goal of the NHS is to improve the overall health and longevity of the population. However, as with all health systems, the NHS has funding constraints and so cannot afford to purchase every new medicine approved for sale in Europe. Inevitably, the redirection of future or existing funding to new medicines means less for existing treatments, which could have a net positive, negative, or neutral impact on overall population health i.e. the health opportunity cost. Thus each purchaser is faced with answering two questions: should we use the medicine and what is the maximum price that we can afford to pay for it?
12. For England and Wales decisions over the cost-effectiveness of new medicines are made by the National Institute for Health and Care Excellence (NICE). If NICE recommends a new medicine, at a certain price, then the NHS in both England and Wales is legally obliged to make the treatment available in all regions as per the NHS Constitution. This prevents the previous situation of a 'postcode lottery' where the availability of new medicines depended upon the decisions of a local NHS authority.
13. When NICE assesses a new medicine (or technology) it reviews the clinical evidence on how well the medicine works and the economic evidence of its cost-effectiveness. A new medicine needs to show that it provides an economic advantage over the currently used next best treatment for the same condition, i.e. the additional benefits of the medicine must outweigh the additional net costs.
14. To measure the health benefits of a new medicine NICE estimates the quality-adjusted life years (QALYs) provided by using it. QALYs take into account any increases in lifespan and/or any improvements in the quality of life from a treatment, so a QALY gain of 1 could be a one year increase in life expectancy with the same quality of life, from treatment, or two additional years with a quality of life improvement of 0.5 (so  $2\text{yrs} * 0.5 = 1 \text{ QALY}$ ). The cost of the new medicine is then divided by the number of QALYs it provides to calculate the cost per QALY gained. For example, if a new medicine provides 4 QALYs and has net costs of £40,000 per patient treated then it works out costing £10,000 per QALY gained.



15. Once the cost per QALY is calculated it is compared to the cost per QALY of the next best current medicine. For example, the current treatment being used provides 2 QALYs for £10,000 per patient and the new medicine provides 2 extra QALYs ( $4-2=2$ ) for an additional cost of £30,000 (£40,000-£10,000 per patient difference). This means that using the new medicine costs £15,000 per additional QALY gained (£30,000 / 2 QALYs) which is called the incremental cost-effectiveness ratio, ICER.
16. NICE's current threshold for cost-effectiveness is set at £20,000 per QALY and any medicines that are below that will be considered cost effective, so the example above of £15,000 per QALY would be considered cost effective. In practice NICE rarely rejects new medicines with a cost effectiveness below £30,000, meaning that it approves 8 out of every 10 treatments assessed.<sup>5</sup> Fundamentally this threshold is there to help decide whether a new treatment is worth the NHS spending money on, at a certain price, as it allows a benchmark against which to measure the additional benefits of the new drug over existing treatments.
17. But the use of cost per QALY to measure cost effectiveness is disputed by some because it undervalues end of life treatments (where life may only be extended by a few months thus providing few QALYs), and medicines for rare conditions where the small number of patients treated makes the medicine cost per patient too high. For these scenarios the threshold is higher (around £50,000) and NICE will consider other factors even if the cost-effectiveness isn't below £30,000.<sup>6</sup> In the case of Orkambi, treatment is expected to bring an additional 3.49 QALYs per patient at a net additional lifetime cost of £748,794, resulting in an ICER of £214,838 per QALY gained (£748,794 / 3.49), far above the threshold of £30,000 per QALY.<sup>7</sup>
18. It's also important to note that the chosen threshold is at times controversial. It makes a decision based upon what it considered the best for overall population health outcomes and not how much an individual patient values a new treatment. NICE has to make decisions under the constraints of the funds allocated to the NHS by Parliament and not based upon an idealised NHS where funding is unlimited.

<sup>5</sup> National Institute for Health and Care Excellence (2019). *Technology appraisal data: appraisal recommendations* [online]. Available at: <https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/data/appraisal-recommendations> (Accessed: 26<sup>th</sup> February 2019).

<sup>6</sup> National Institute for Health and Care Excellence (2019). *6 The appraisal of the evidence and structured decision-making* [online]. Guide to the methods of technology appraisal 2013. Available at: <https://www.nice.org.uk/process/pmg9/chapter/the-appraisal-of-the-evidence-and-structured-decision-making#decision-making> (Accessed: 26<sup>th</sup> February 2019).

<sup>7</sup> National Institute for Health and Care Excellence (2019). *Memorandum to the Health and Social Care Committee of the House of Commons* [online]. Available at: <https://www.parliament.uk/documents/commons-committees/Health/Correspondence/2017-19/Letter-from-NICE-to-Chair-2018-11-29.pdf> (Accessed: 26<sup>th</sup> February 2019).

19. The greatest anguish over the threshold is usually reserved for cancer medicines which mostly deliver less than one additional QALY because they only extend life by weeks or months at a low level of quality. NICE currently approves six out of every ten cancer medicines but in response to public pressure, the Cancer Drugs Fund was set up in 2011 to fund cancer drugs which NICE deemed not to be cost-effective. This fund, which was intended to be an interim measure, has grown in budget from £50m in 2010/11 to £340m in 2018/19. However it has been criticised heavily for not providing a clear value for money and for not collecting sufficient data on how it improved patients' lives.<sup>8</sup>
20. Finally, recent research suggests that the NICE threshold should be set lower at £13,000 per QALY, instead of at £30,000, if it is to take into account the health opportunity costs of new treatments.<sup>9</sup> This research empirically estimated the actual cost effectiveness threshold across the NHS using historical data. The main goal of using a cost-effectiveness threshold is for the NHS to maximise the length and quality of the nation's lives (QALYs) by only investing in new treatments if the QALYs gained in the new treatment are greater than the QALYs lost from not providing treatment in existing areas of spending i.e. the health opportunity costs of diverting spending away from existing treatments.
21. Based upon the empirical estimate for the NHS, moving £13,000 of spending away from existing treatments leads to a loss of one QALY so the new treatment needs to provide at least one QALY for that amount of spend to make it worthwhile overall. However, cutting the threshold by more than half would drastically limit the number of new treatments available on the NHS if manufacturers did not cut their prices. NICE settling on £30,000 as a threshold reflects their desire to balance investment in new treatments with ensuring fair access to existing NHS treatments.<sup>10</sup>

## Patients' representative groups

22. The views of patients and their families are also included in the NICE appraisal process. This can be helpful in humanising the beneficiaries of a new medicine for decision makers. For many conditions, particularly chronic ones, there are patient-advocacy organizations which support and advise patients and raise public awareness.

<sup>8</sup> National Audit Office (2015). *Investigation into the Cancer Drugs Fund* [online]. London: National Audit Office. HC442, Session 2015-16.

<sup>9</sup> Claxton, K., *et al.* (2015). 'Methods for the Estimation of the NICE Cost Effectiveness Threshold', *Health Technol Assess*, 19(14). Doi: 10.3310/hta19140

<sup>10</sup> Dillon, A (2015). 'Carrying NICE over the threshold' [blog], *National Institute for Health and Care Excellence Blog*, 19<sup>th</sup> February 2015. Available at: <https://www.nice.org.uk/news/blog/carrying-nice-over-the-threshold> (Accessed: 26<sup>th</sup> February 2019).

23. However, there has been a long debate over the risks of conflicts of interest within patient-advocacy organizations. In the US 83% of the largest patient organisations receive funding from pharmaceutical companies.<sup>11</sup> Recent evidence from the UK found that 72% of patient organisations involved in the NICE appraisals for new medicines or treatments had accepted funding, in the same year, from the manufacturer of the medicine or a competitor. The NICE decision-making committees were only made aware of less than a quarter (21%) of these interests.<sup>12</sup>
24. There are concerns that a heavy reliance on pharmaceutical companies for funding can lead patient groups to overly advocate the (potentially less effective) treatments made by their donors at the expense of competitor or non-pharmaceutical treatments. It also could encourage them to stay silent on issues such as extortionately high medicines pricing. It's worth noting that pharmaceutical companies tend to donate to those patient-advocacy organisations who represent patients for whom the company has developed a treatment.
25. As such, when funding is taken away from one area of health spending and given to another, it can negatively impact those with conditions that aren't as well funded, defended, or publicised but where the marginal benefits of spending are higher, for example on mental health or learning disabilities. Even if the funding of new treatments comes from an increased NHS budget then this comes at the expense of spending in other public areas or from increased taxation, which is, however, supported by a majority of the British public.<sup>13</sup>

## Conclusions

26. The pricing and availability of medicines is a contentious area where conflicts between the interests of pharmaceutical companies, patients, and wider health budgets will regularly arise. The number of these conflicts is likely to worsen in future for multiple reasons. First, the level and growth of healthcare spending is failing to keep up with the increasing costs of new technology and changes in population needs. The pressure for the rapid adoption of new, potentially life-saving, medicines can involve approving them before the full benefits (in terms of QALYs) and costs are known. In addition, more personalised treatments aimed at smaller groups of patients are often cost-

<sup>11</sup> McCoy, M.S., *et al.* (2017). 'Conflicts of Interest for Patient-Advocacy Organizations', *N Eng J Med*, 376(9), pp.880-885. Doi: 10.1056/NEJMSr1610625

<sup>12</sup> Mandeville, K., *et al.* (2019). 'Financial interests of patient organisations contributing to technology assessment at England's National Institute for Health and Care Excellence: policy review', *BMJ*, 364. Doi: 10.1136/bmj.k5300

<sup>13</sup> Evans, H. (2018). 'Does the public see tax rises as the answer to NHS funding pressures?', *The Kings Fund* [online], 12<sup>th</sup> April 2018. Available at: <https://www.kingsfund.org.uk/publications/does-public-see-tax-rises-answer-nhs-funding-pressure#how-does-the-public-want-to-pay-for-the-nhs> (Accessed 26<sup>th</sup> February 2019).



ineffective when measured against other broader treatments, even if they are more helpful for those targeted.

27. New proposals on pricing have been put forward which seek to reimburse pharmaceutical companies for medicines based upon the 'outcomes' or the 'value' delivered to the NHS, patients, and wider society.<sup>14 15</sup> These are attempts to better link the price paid to the value provided by the medicine. They are designed to reward companies which produce new effective treatments and not just those who charge a lot for little to no new benefits or those who make small changes to extend their patents (ever-greening).<sup>16</sup> Whichever pricing mechanism is used there needs to be consistency over which medicines are approved, or not, so that companies do not waste resources developing low value medicines of little benefit.
28. More fundamentally, the relative importance of investing in new medicines versus other areas of health spending needs to be reassessed. Increasing amounts of resources are required to find effective treatments for more complex diseases and personalised therapies. Society pays for these, and all the failed attempts, through the public funding of basic scientific research and ultimately the price of new medicines. With increasing pressure on health budgets, better value for money may be found in non-pharmaceutical health investments, such as tackling the systematic drivers of chronic conditions such as obesity (e.g. the food industry's incentives).<sup>17</sup> Whilst the pharmaceutical industry will undoubtedly continue to produce valuable new treatments, it may be more cost-effective to incentivise the production of certain categories of treatment which may be unprofitable but socially valuable (e.g. for niche genetic conditions or tackling antibiotic resistance). The remaining resources could then be focused on achieving larger health gains from say, population-wide lifestyle interventions or by improving the existing levels of care within the NHS.

<sup>14</sup> Cole, A. et al. (2019). *Making Outcome-Based Payment a Reality in the NHS: Executive Summary* [online]. London: Cancer Research UK. Available at: [https://www.cancerresearchuk.org/sites/default/files/obp\\_final\\_ex\\_sum\\_pdf.pdf](https://www.cancerresearchuk.org/sites/default/files/obp_final_ex_sum_pdf.pdf) (Accessed: 26<sup>th</sup> February 2019).

<sup>15</sup> Centre for Health Economics (2016). *Pharmaceutical Pricing: Early Access, The Cancer Drugs Fund and the Role of NICE* [online]. York: University of York. Available at: [https://www.york.ac.uk/media/che/documents/policybriefing/Drug\\_prices.pdf](https://www.york.ac.uk/media/che/documents/policybriefing/Drug_prices.pdf) (Accessed: 26<sup>th</sup> February 2019).

<sup>16</sup> Moir, H., and Gleeson, D. (2014). 'Explainer: evergreening and how big pharma keeps drug prices high', *The Conversation* [online], 5<sup>th</sup> November 2014. Available at: <https://theconversation.com/explainer-evergreening-and-how-big-pharma-keeps-drug-prices-high-33623> (Accessed: 26<sup>th</sup> February 2019).

<sup>17</sup> Wang, Y. et al. (2014). 'Applications of Complex Systems Science in Obesity and Noncommunicable Chronic Disease Research', *Advances in Nutrition*, 5(5), pp. 574-577. Doi: 10.3945/an.114.006650