Pfizer response to questions received from Just Treatment

Drug discovery and development

At the heart of our business is our mission to help people around the world live healthy lives. This means applying our science, our passion and our global expertise to discover and develop new medicines and bring them to the people who need them.

Working in collaboration with both UK universities and the NHS, and similar institutions around the world, our scientists have achieved transformational outcomes for patients; from being the first company to mass produce penicillin back in the 1950's, to the discovery of new treatments in important areas of unmet medical need including heart disease, HIV and acute pain.[1]

Developing a medicine takes an enormous investment of time, money and knowledge. A single medicine can take 12 years,[2] 1,600 scientists,[3] half a million lab tests to create,[3] and recent research has found that on average its costs over £1.5 billion to develop and bring a medicine to market.[4,5] This process is long, complicated and risky, with no guarantee of success; 19 out of every 20 early discoveries fail.[6] Between 1998 and 2014 there were 75 unsuccessful attempts to develop a new treatment for brain cancer and only 3 new medicines were successful. This is the norm; our scientists have an incredibly difficult job and we are dependent on their resilience and determination to find the game-changing breakthroughs which do eventually make it to patients.[7,8]

Innovation starts in the research laboratory, often in partnership with academic or public institutions. Once a new molecule has been discovered, we run studies to ensure it has the desired effect at the biological level. We then conduct tests to verify how the compound acts in the body and run toxicology tests to determine its safety. This all happens before we initiate, often extensive, clinical trials programmes to examine the dosing, the safety and efficacy of the new potential medicine and importantly the risk benefit to patients. Only once we have collated enough evidence are we able to submit our data to regulatory bodies like the European Medicines Agency (EMA) or the Food and Drug Administration (FDA) for approval and if a medicine is granted marketing authorisation we then begin negotiations at a country level to bring the medicine to patients. In the case of palbociclib it took 20 years of research and development before this medicine was approved for use in the US and Europe. Through years of hard work, determination and often disappointing failures along the way, it is our ambition that people around the world can benefit from safe and effective medicines.

But the work doesn't stop there. We continue to invest in medicines like palbociclib to continually monitor efficacy, meet our commitments to pharmacovigilance and understand their potential use in other disease areas.

Medicine pricing and company return on investment

Nine in ten medicines are discovered and developed by the pharmaceutical industry.[6] It is an intensive and costly process with a vast amount of R&D costs incurred before the new medicine (if successful) can be prescribed to patients and a return made.

To continue our research and development in search of the next transformative treatment, and to remain competitive and innovative, we need to make a profit on the medicines we produce so that we can reinvest back into R&D. In 2016, Pfizer invested over $7.87 billion globally in R&D in the

search for new medicines.[9] External investment is also critical. As with other industries, we need to attract investors in order to fuel further research, and although there is no guaranteed return, they expect to invest in a healthy, sustainable business.

The patent system, and the introduction of similar competitor molecules, means we only have a relatively short period of time to recoup our investment on a successful medicine. By the time a medicine is approved, a large proportion of its patent life has lapsed and once the patent has expired, competitor companies can create a "generic" version of the medicine, selling it for much less without the discovery and development costs.

This is why the pricing of our medicines is so important. We need to recoup our investment on the medicine that has succeeded, and the compounds that didn't, whilst delivering enough profit to ensure our business and commitment to R&D remains viable. When determining the price of a medicine, we evaluate a host of different factors. We consider the impact the medicine will have on patients and their disease, relative to other available treatments; its value to the health service, including the potential to reduce other healthcare costs, such as hospital stays; and importantly affordability in relation to a country's economic situation and healthcare system.

We work closely with the healthcare community and payers to build an understanding of the value of the medicine. Recognising our responsibility to ensure medicines are affordable in light of today's budget pressures, we seek to negotiate a price in each country which enables patients to benefit from the medicine at a cost that allows continued investment into new treatments.

We are committed to ensuring medicines are affordable by balancing the long-term need for investment in innovation with short-term government and payer budget priorities. In the UK, the pharmaceutical industry entered into an unprecedented deal with the government in 2014 (known as the Pharmaceutical Price Regulation Scheme (PPRS)) to cap the amount the NHS spends on medicines. Through this deal, the pharmaceutical industry agrees to make repayments to the Department of Health on any medicines spend that exceeds the agreed cap, and since 2014, the pharmaceutical industry has repaid over £1 billion.[10] Including these PPRS repayments to the Department of Health, in 2015-16 the total spend on medicines was 12.4% of the total health budget.[11]

Our global financial results are presented quarterly and information relating to our revenues is publicly available on our global corporate website (www.pfizer.com).

Patient access to medicines

It is our mission to help people around the world live healthy lives and our goal is access to our medicines for patients who need them.

However, as R&D becomes more challenging and healthcare systems around the world become more financially stretched, we are facing fresh challenges in bringing new medicines to patients. This is particularly evident in the UK. We share the concerns of patient groups and academics that our NHS and the systems supporting it are struggling to keep up with advances in medical science while caring for more patients. Cancer medicines, for example, are becoming more targeted, advanced and complex as we delve deeper into the biology of cancers, and yet our systems for evaluating these new therapies have not evolved for nearly two decades.[12] Continued collaboration between industry, Government and the health and the life sciences community is needed to enable constructive discussions and ensure the system is able to keep pace.

There is also an onus on industry to drive efficiencies in certain R&D processes and on regulators to consider how the licencing processes can be better streamlined. Underpinning all of this is the continued collaboration between industry and academia. No medicine is ever made in isolation and this partnership is crucial to success.

We are committed to doing what we can to help patients access the medicines they need and we combine our commercial strategies with philanthropic approaches for those most in need. The free-of-charge palbociclib Patient Programme is an example of how we endeavour to ensure patients can access our medicines. Through this programme, more than 800 women across the UK accessed palbociclib free-of-charge whilst we continued negotiations with the National Institute for Health and Care Excellence (NICE) and the Scottish Medicines Consortium (SMC). These women will continue to receive the medicine free-of-charge for the full duration of their treatment, even now that the programme has closed and the medicine is available through the NHS. Elsewhere, we operate compassionate use programmes through which healthcare professionals can apply for access to a medicine on behalf of their patients.

Globally, our access initiatives also include:

- In partnership with the Edna McConnell Clark Foundation, we founded the International Trachoma Initiative (ITI) which aims to meet the World Health Organization's (WHO) call to eliminate trachoma, the world's leading cause of blindness, by 2020. Since this partnership was formed we have donated more than 600 million doses of an antibiotic which treats trachoma.
- In June 2017, the Clinton Health Access Initiative (CHAI) and the American Cancer Society (ACS) announced a new collaboration with Pfizer to supply six countries in sub-Saharan Africa with access to 11 essential oncology medicines via government tender and procurement mechanisms. This collaboration underscores our longstanding efforts to address unmet medical needs of patients in Africa and significantly improve cancer care by increasing access to lifesaving cancer treatments.

These are just some examples of how we continue to work with different parties on novel solutions and new models which improve the quality of cancer care, patient access, and the efficiency of healthcare delivery. More details on these programmes can be found in our Annual Report which is publicly available on our global corporate website.

The approval process for palbociclib

We are delighted for women across the UK that both NICE and the SMC have approved palbociclib for first-line use in combination with an aromatase inhibitor for the treatment of hormone receptor positive (HR+), human epidermal growth factor receptor 2 negative (HER2-) locally advanced or metastatic breast cancer. Following approval by NICE and the SMC, palbociclib is now routinely available through the NHS to eligible patients in the first line setting in England, Scotland, Wales and Northern Ireland. The medicine remains available privately in accordance with its licensed indication, granted by the EMA in 2016.

The appraisal process for medicines like palbociclib are complex and involve significant discussion around the need for the medicine, the potential benefit to patients and society, and the anticipated costs and cost savings of the medicine and its administration. Through these discussions, the manufacturer seeks to reach an agreement with NICE and the SMC on the value of the medicine and its cost. In the case of palbociclib, we worked closely with NICE and the SMC to provide additional data from clinical trials and submit a revised economic case (with confidential discounts) and we are pleased that both NICE and the SMC have deemed the medicine to be cost-effective and good value

for money. The discounts agreed remain confidential but we are pleased that NICE and the SMC have recognised that palbociclib demonstrates excellent value for the NHS.

Now that we have secured NICE and SMC approval of palbociclib in combination with an aromatase inhibitor for the first-line treatment of HR+, HER2- metastatic breast cancer, we are in discussions with both reimbursement bodies to explore its approval for use in later lines of therapy. As with many of our medicines, we also continue to study palbociclib in other tumour types so as to explore the medicine's full potential to help patients.

Our ways of working

Finally, at Pfizer, operating with integrity is one of our core values and it reflects who we are as a company and as individuals. Our Code of Conduct, known as "The Blue Book", governs our behaviours, policies and the decisions we make, forming the basis of our everyday business activities from interactions with healthcare professionals, patients and society, to how we price our medicines. We demand of ourselves and others the highest ethical standards as we strive to improve the lives of people around the world. We also have a number of global policy documents available on our global corporate website.

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[7] Patel JD et al, Clinical cancer advances 2013: annual report on progress against can from the American Society of Clinical Oncology, J Clin Oncol. 2014;32(2):129-160

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[11] According to a response to a written parliamentary question by Nicola Blackwood MP, Parliamentary Under Secretary of State for Public Health and Innovation, regarding the total spend on drugs in 2015-16. (Hansard, 25th October 2016). Available at: http://www.parliament.uk/business/publications/written-questions-answers-statements/written-question/Commons/2016-10-17/49032/ [Last accessed January 2018]

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