



Proposed changes to the statutory scheme to control the costs of branded health service medicines

Consultation response

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Executive summary

The voluntary and the statutory schemes for pharmaceutical pricing limit the growth in costs of branded health service medicines. This is done to safeguard the financial position of the NHS, while taking into account the need for medicinal products to be available for the health service on reasonable terms, the costs of research and development, and impacts on the UK life sciences industry, wider economy, and patients. The Government's objective is to deliver this in a way consistent with supporting both the life sciences sector and broader economy. The current voluntary scheme, agreed with the Association of the British Pharmaceutical Industry (ABPI) is the 2014 Pharmaceutical Price Regulation Scheme (PPRS). This will expire on 31 December 2018 and the Government is currently working on a successor scheme. Companies have a choice as to whether to be part of the voluntary scheme, and the statutory scheme applies to those companies that choose not to.

Earlier this year, the Government consulted on changes to the statutory scheme, which operates under the Branded Health Service Medicines (Costs) Regulations 2018 ("the 2018 Regulations"). In response to requests to set out additional details on calculations underlying part of the proposals, the Government also issued a clarification note and allowed further comment upon this. This document analyses the 48 initial consultation responses as well as the 11 further comments submitted during the clarification period, and sets out the Government's intentions.

In summary, the Government will amend the 2018 Regulations to set a payment percentage that we have calculated to be required to limit the expected growth of net branded health service medicines sales to 1.1% p.a. nominal growth from the 2018 baseline. We will adopt the policy described in Chapter 4 on sales under contracts with a contracting authority based on a framework agreement, or under public contracts (both referred to in the remainder of this document as "Agreements") set out in the original consultation, and will also amend the definition of a relevant medicine so that biological medicinal products marketed under the combination of INN and a company name are subject to the payment mechanism, price control and information provisions. There will be an annual review of the Regulations no later than April 2019 to consider, based on the available data, whether the changes introduced in April 2018 and January 2019 are delivering the Government's objectives for the statutory scheme.

Chapter 2 sets out the Government's consideration of responses received on the proposed payment percentages and the methodology of calculating them. Following consideration of the responses made to the consultation, the Government has determined that a 1.1% nominal p.a. growth rate from the 2018 baseline best balances the Government's objectives for the statutory pricing scheme. As a result, we will set payment percentages of 9.9%, 14.7% and 20.5% in 2019, 2020 and 2021 respectively. This is a downward revision

of the payment percentages for 2020 and 2021 compared to those stated in the consultation, as new data on medicines expenditure has become available since the consultation which has been used to update our estimates of future expenditure growth. Changes are described in more detail in chapter 2.

Chapter 3 sets out the Government's consideration of responses received on our proposals to bring all biological medicinal products, including biosimilars, into the scope of the payment mechanism, pricing controls and information provisions, irrespective of the naming convention applied to them. Following consideration of the responses received as part of the consultation, the Government intends to proceed with the implementation of the proposals as outlined in the consultation.

Chapter 4 sets out the Government's consideration of the responses received on our proposals for the treatment of sales of items of presentation under Agreements. Following consideration of the responses, the Government intends to proceed with the implementation of the proposals as outlined in the consultation.

Chapter 5 sets out the Government's consideration of responses received on our proposed approach to forecasting future medicines expenditure. Following consideration of responses received during the consultation, we remain of the view that the approach proposed represents the most appropriate way to forecast future expenditure.

In chapter 6, views expressed on our assessment of the impact of our proposals and implications for statutory duties of the Secretary of State for Health are considered. The Government has made some changes to the impact assessment both as a result of the responses received, and new data having become available (as discussed in Chapter 2), and these are detailed in this chapter. Consideration of the relevant statutory duties in relation to the final decisions made about the statutory scheme is presented at Annex A, and the final impact assessment is published separately.

The changes to the 2018 Regulations will be set out in Branded Health Service Medicines (Costs) (Amendment) Regulations 2018 ("the Amendment Regulation"), and will come into force on 1 January 2019. A copy of the Amendment Regulations is published alongside this document. Operational guidance, which has been published in draft following the coming into force of the 2018 Regulations will be updated to support companies in the implementation of the changes to regulations.

1. Introduction

- 1.1 The voluntary and the statutory schemes for pharmaceutical pricing limit the growth in costs of branded health service medicines. This is done to safeguard the financial position of the NHS, while taking into account the need for medicinal products to be available for the health service on reasonable terms, the costs of research and development, and impacts on the UK life sciences industry, wider economy, and patients. The statutory scheme is part of a broader set of measures, with which the Government seeks to create an environment where clinically- and cost-effective medicines are supplied at an affordable cost, in a way consistent with supporting both the life sciences sector (including research and development) and the broader economy. On 7 August 2018, the Government published a [consultation on proposed changes to the statutory scheme to control the costs of branded health service medicines](#), followed by a [clarification note](#) on 5 October 2018. The purpose of the proposals was to ensure that the Government's objective of safeguarding the financial position of the NHS can be met in light of the expiry of the voluntary Pharmaceutical Price Regulation Scheme 2014 (PPRS) on 31 December 2018, as it is this voluntary scheme that the level of the payment mechanism in the statutory scheme was previously aligned to. It was the Government's intention to provide clarity to companies subject to the statutory scheme as well as other pharmaceutical companies in the UK as to the Government's proposals for future payment arrangements under the statutory scheme.
- 1.2 The consultation document set out three proposed changes to the statutory scheme:
- Setting payment percentages for the years 2019 to 2021 in the 2018 Regulations;
 - Including all biological medicinal products within the scope of health service medicines captured by the payment mechanism, price controls and information requirements. As the definition of relevant medicines already includes almost all biosimilars, the practical change would be to include any biological medicinal product marketed under a combination of INN and company name; and
 - Changing the application of the payment system for sales of medicines supplied under a contract with a contracting authority based on a framework agreement or under a public contract (both referred to in the remainder of this document as "Agreements".)

- 1.3 The consultation on these proposals closed on 18 September 2018, with an additional opportunity for respondents to provide comments following publication of the clarification note up to 19 October 2018. Initially, the Department of Health and Social Care (DHSC) received 48 responses, of which three were from health bodies, 41 from pharmaceutical companies, trade bodies and groupings and industry consultants, two from patient organisations, and two from individual respondents. The Department received responses to the clarification note from 11 respondents. This included two respondents who had made entirely new submissions (one individual and one pharmaceutical company). Of those who submitted further comments, all were pharmaceutical companies, trade bodies or industry consultants. None substantively revised their positions from their original views provided, so for the purpose of the statistics in this document are counted as one response. However, they did offer further thoughts on the proposals which the Department has considered in developing policy and are addressed in this consultation response.

2. Responses on the payment percentages and methodology

- 2.1 After review of the consultation responses, the Government has decided to implement a 1.1% nominal p.a. growth rate from the expected 2018 baseline of relevant sales, as this best balances Government objectives for the statutory scheme. Payment percentages will be 9.9%, 14.7% and 20.5% in 2019, 2020 and 2021 respectively. Each year the 2018 Regulations will be reviewed. If there is evidence that the payment percentages are no longer appropriate to deliver the objectives of the scheme, the Department will be able to consult on revisions to these payment percentages.

Q1 Do you have any comments on the proposed payment percentages or the methodology used in determining the payment percentages?

Outline of consultation proposals

- 2.2 The consultation document proposed payment percentages for the years 2019 to 2021, alongside a methodology for deriving them.
- 2.3 The payment percentages are aimed at recovering the difference between a forecast level of relevant sales and an allowed level of relevant sales. The forecast level of relevant sales is derived from the DHSC forecasting model, and the allowed level of relevant sales is set on the basis of an expected 2018 baseline for relevant sales increased by the allowed annual growth rate in each year. Relevant sales for the calculation include sales of all branded health service medicines of companies in the 2014 PPRS, sales of all branded health service medicines of companies in the statutory scheme, and parallel import sales. The details of the forecast methodology were set out in Chapter 8 of the original consultation. We proposed to set an allowable growth rate on relevant sales for the period 2019-2021 that is consistent with the average annual growth rate agreed for the duration of the 2014 voluntary scheme, which equates to 1.1% nominal growth per annum from the 2018 baseline of allowed relevant sales

Summary of responses

- 2.4 A majority of respondents disagreed with the proposal, with 43 respondents expressing concerns, and four respondents agreeing.
- 2.5 Respondents agreeing with the proposals argued that increased savings delivered by a national cost control scheme are necessary to align with and support their local efforts to allocate resources to frontline services effectively and improve patient health.
- 2.6 Those respondents disagreeing with the proposals made the following overarching comments on the growth rate and payment percentages:
- Setting the allowed growth rate at the average level of allowed growth in the 2014 PPRS was undesirable as they believed the PPRS growth rate was a one-off agreement made against the backdrop of public sector austerity and significant financial pressures on the NHS;
 - The allowed growth rate should be in line with the agreed long-term NHS budget growth to avoid an industry perception of disinvestment in medicines;
 - The payment percentages, particularly in 2020 and 2021 are too high, and threaten UK profitability, therefore generating risks for the supply of medicines, the timing for UK launches of new products, the desirability of the UK as a destination for life sciences investment with an associated wider economic impact, and ultimately patient health outcomes due to the reduction in the availability of medicines; and
 - An additional financial burden for the life sciences sector when there is significant cost uncertainty concerning the post-Brexit regulatory environment may threaten the viability of UK operations.
- 2.7 A number of respondents made arguments for changes to the products that are required to make a payment under the scheme, with the proposals raised being that:
- Parallel importers should be subject to the payment mechanism, since parallel import sales are included in the overall branded medicines growth calculation;
 - The proposals would lead to price increases specifically for blood plasma protein therapies (with evidence submitted to support this), branded generics, and rare disease drugs; and

- The low cost exemption threshold of £2 should be raised to £5 to account for inflation since the threshold was set, and the forthcoming Falsified Medicines Directive which disproportionately affects low cost presentations.
- 2.8 In addition, the following technical comments were made on the calculation:
- The expected 2018 baseline for allowed relevant sales is partially derived from estimated figures (sales of voluntary scheme companies, statutory scheme companies, parallel imports, as well as the 2018 payment received as part of the 2014 PPRS) which introduces unacceptable uncertainty into the calculation of payment percentages;
 - The payments received as part of the 2014 PPRS in 2018 should not be taken into consideration for calculation of the baseline allowed relevant sales;
 - For the purposes of calculating UK impact, a scaling factor of 1.25 for the conversion of England-only to UK expenditure is incorrect.

Government response

Responses on the level of the allowed growth rate

- 2.9 As set out in the consultation document, in setting the allowable growth rate, the Department seeks to balance the interest of patients, the NHS, taxpayers, and the pharmaceutical industry.
- 2.10 The Government has made a significant commitment to increase NHS funding over the coming years, which is higher than the proposed allowable growth rate, and respondents suggested this should be the benchmark for medicines. It is the Government's responsibility to support the NHS in providing a high quality, comprehensive health services to patients, whilst delivering more health gain per pound in the round. There is no reason to assume that the share of medicines expenditure from the NHS budget should automatically be held constant to support this. There are many high priority areas for the NHS that the budget settlement is intended to support. As explained in the Impact Assessment, there will be significant overall economic benefits from the substantial savings being reinvested in the NHS under a 1.1% growth rate, which could, for example, help reduce waiting times, improve mental health services and deliver earlier cancer diagnosis.
- 2.11 There was no commitment that the 1.1% nominal growth rate in the 2014 PPRS would be a one-off agreement. Having considered that the voluntary scheme's members were able to continue to make significant investment, launch innovative new medicines and retain profitability under the 1.1% per annum targeted growth

rate from the 2014 PPRS, and the most likely effect across the balance of objectives considered in the impact assessment, the Department believes the optimum improvements overall are still delivered by using this as the basis for growth. We did not find that the evidence submitted by respondents contradicted our argument that we would expect some supply chain efficiencies and other cost reductions, and that companies will be able to maintain sufficient profit margins to support a commercial decision to supply medicines to the health service in the UK.

- 2.12 We do acknowledge that the growth rate is being determined at a point where the UK's exit from the EU creates uncertainty for industry. The Government's policy is to minimise this uncertainty through its negotiations with the EU and the technical notices published on a 'no deal' scenario – this includes, for example, confirmation that the UK would unilaterally accept batch testing from the EMA. The uncertainty around Brexit is comparable to wider economic uncertainties.
- 2.13 Therefore, it is not clear how any potential impacts of uncertainty could be appropriately factored into the determination of an allowed growth rate. In any case the proposed payment percentages largely apply to the time period after the UK's departure from the EU will have concluded. The annual review mechanism will allow the Department to consider the ongoing appropriateness of allowable growth rate and payment percentages.

Response on possible impacts on research & development

- 2.14 The Department has acknowledged in the Impact Assessment that there may be a small reduction in research and development investment. This will flow through from slightly lower levels of revenue for statutory scheme companies as a result of higher levels of payment percentage. The consequent estimated reduction of R&D investment in the UK is valued at £5.5m in 2021. However total UK pharmaceutical R&D investment was around £4.1 billion in 2016, according to the OLS Life Sciences Competitiveness Indicators, demonstrating the overall levels of UK R&D investment will continue to be healthy, even allowing for the changes. This is without accounting for the impact of any future voluntary scheme.
- 2.15 A range of published evidence and independent studies argue that the distribution of this investment across countries is not significantly influenced by the sales regime in a given local market. These reports include those produced by the [Office for Fair Trading](#), [NERA Consulting](#), [OECD](#) and [PwC](#). We recognise that many pharmaceutical companies responded to say that negative boardroom sentiment towards the UK market, which would result from greater payments under the amended scheme, should be incorporated into the Impact Assessment to reduce expected inward investment. Nonetheless we maintain the key determinants of pharmaceutical R&D investment across countries are supply side factors such as the availability of skilled labour, which are not altered by the statutory scheme. In

addition, the Government and NHS are collaborating closely with the industry to create a favourable environment for the life sciences sector, through taking forward proposals made in the Life Sciences Industrial Strategy, such as promoting uptake and usage of medicines and devices through the Accelerated Access Collaborative.

Response on possible impacts on the supply of medicines to the UK market

- 2.16 The consultation document acknowledges that too low a level of allowable growth rate could theoretically induce negative supply effects. The Department would of course view shortages, or deferred launches of medicines, as undesirable in cases where they have been deemed to be cost-effective and clinically-effective, given the potential impact on patient health overall. The Department is also mindful of avoiding such restrictions for conditions predominantly affecting patients with protected characteristics. However, the 1.1% level of allowable growth was chosen precisely because this was agreed to be appropriate for the five-year lifespan of the 2014 PPRS and we have seen no evidence that this level of growth led to material negative supply effects.
- 2.17 We also considered evidence around the impact on the UK's place in the international sequence for the launch of products, considering whether, if the UK pricing structure was less commercially attractive, it may lead to later product launches. Commercial attractiveness however reflects a multitude of factors where the UK performs strongly, such as the international value of world-class scientific and economic NICE assessments, the market value for international reference pricing and the co-location with clinical trials. We have therefore received no firm evidence which supports the contention that companies would – in the round – be commercially incentivised not to proceed promptly with UK launches.
- 2.18 The 2018 Regulations also include a price increase provision. Where a company applies for a price increase the Department can take into consideration a number of factors including a company's margins and costs. These are factors that allow us to grant an increase to the statutory prices.

Response on the calculation methodology

Estimation of the expected 2018 baseline

- 2.19 We agree that there are uncertainties in the value of the components of the payment percentage calculations, as they are based on estimates of sales and payments. This is however an unavoidable feature of an allowable growth rate mechanism – which the overwhelming majority of respondents supported. As payment percentages have to be set in advance, it is inevitable that they will be

set on provisional and/or forecast data. Given the variation in accounting reference periods across companies, it can take up to 11 months after the end of a given financial year until audited sales data is available from all companies.

- 2.20 Calculations of the payment percentages have therefore been undertaken on the most recent full-year data available and the resulting payment percentages for the years 2019 to 2021 have been updated accordingly from those set out in the consultation document. As new routinely refreshed data has been released, this means that although the same methodology has been used, the inputs are now different and more accurately reflect the latest known picture of growth in the UK branded medicines market. These updates directly address some of the requests made by respondents – sales by companies in voluntary and statutory scheme, as well as parallel import sales, are now based on data up to and including Q4 2017. Information on sales under frameworks, which is used in the Impact Assessment, has been updated with data for the 12 months up to 1 September 2018.
- 2.21 It is not possible to compel companies to submit data to the Department for the first quarter of 2018. However, as laid out above, payment percentages of an allowable growth rate scheme will always have to be set with respect to estimates of spend, and the expected 2018 baseline includes information from audited sales reports in earlier years. The Department will obtain audited sales reports for quarters two to four 2018 as part of the statutory scheme, and will use this information to estimate full-year sales in 2018.

England/UK scaling factor

- 2.22 We acknowledge that there are different possible approaches to deriving an England/UK scaling factor – historically, 1.25 has been used in the PPRS to distribute payments across devolved administrations. The estimate is based on PCA data, which provides the only comparable data source covering the whole UK, albeit only comprising primary care expenditure. Using population data for over-65s would point to a scaling factor closer to 1.2, while the industry has presented estimates that were around 1.22. That is based though on volume data combined with list prices, and therefore do not reflect the true underlying NHS expenditure (at net prices after any discounts).
- 2.23 As the scaling factor is uniformly applied to all numbers in the calculation of the payment percentage (allowed sales, forecast sales, and the 2018 payments received under the voluntary and statutory schemes), applying a different factor would, however, not alter the results of the calculation – payment percentages ultimately remain unaffected by the scaling factor chosen, as set out in the clarification note.

Treatment of 2018 payment in calculation of allowed relevant sales

- 2.24 The overarching objective of the statutory scheme is to limit the growth of net expenditure on branded medicines expenditure to allowable levels, bearing in mind the need for medicinal products to be available for the health service on reasonable terms (i.e. representing value for money given the constraints on the NHS budget) and the costs of research and development. Respondent's proposals to either disregard the current payments, or to undertake some other form of rebasing of the level of allowed sales periodically, would run directly contrary to this objective.
- 2.25 If the 2018 baseline for allowed relevant sales was set without taking account of payments received in 2018, the 2019 allowed level of relevant sales would effectively be set on gross expenditure on branded medicines in 2018. This is not the relevant measure of medicines expenditure that the scheme intends to control, and would have the same effect as permitting greater annual growth than 1.1% - we estimate that growth of net branded medicine expenditure would grow by 7.3% from 2018 to 2019 if the 2018 payment would be disregarded in the calculation of allowed relevant sales.
- 2.26 We acknowledge the potential for payment percentages to increase over time, if the mechanism of allowable growth were applied on an ongoing basis and if the growth rate of branded health service medicines also continually outstripped that allowed growth. The annual review mechanism will allow us to consider whether the payment percentages remain reasonable, and where appropriate to consult on any changes.

Response on medicines that should be required to make a payment

Inclusion of parallel imports in relevant sales

- 2.27 The proposals set out in the consultation do not require companies to pay for sales of parallel imports, and Government would treat the payments due from these sales as foregone income. This is because parallel imports are "relevant sales" and therefore included in the calculation of the payment percentage, but we would not be imposing the payment percentage on sales of parallel imports and are not proposing to adjust upwards the payment percentage applied to other products to make up for the resulting gap.
- 2.28 Including parallel imports in the expected 2018 baseline of both allowed relevant sales and measured relevant sales lowers payment percentages compared to a counterfactual calculation that excludes them.
- 2.29 There is a possibility of statutory scheme companies facing higher payments where parallel imports grow faster than statutory and voluntary scheme sales. This

is however not unique to parallel imports, as excess growth in any of the three components of the calculation (voluntary scheme sales, statutory scheme sales, parallel import sales) would increase the (implicit) payments for the other two components compared to a situation where payments would be calculated on a single-component basis. Similarly, even a calculation of payment percentages segmented by scheme (i.e. in the way the 2014 PPRS operates) would lead to slow growing companies implicitly subsidising faster growing companies. The rationale for a payment percentage that tracks growth in the entire branded market, including PIs, and payments based upon these is sound – by tracking whole market growth better, it is more likely that the statutory scheme's growth objective (and the attached benefits estimated in the impact assessment) are delivered.

- 2.30 Sales of parallel imports are partly driven by currency fluctuations, as well as by supply side factors in other European markets. The central forecast used currently by the Department sees parallel imports growing slower than the overall branded medicines forecast. This does not however impact the payment percentage calculations, as the overall market growth rate applied in the calculation is estimated on the basis of all sales including parallel import sales.

Exclusion of parallel importers from the payment mechanism

- 2.31 The Government has recognised that there are valid arguments both for, and against the exclusion of parallel importers from the payment mechanism in its response to the consultation on the 2018 Regulations. However, parallel imports provide the only competition to patented drugs (apart from wider competition within therapeutic classes), and thereby help to keep prices at lower levels for the NHS. Work undertaken during the development of the 2018 Regulations has shown that parallel importers operate on low margins, such that the application of the payment percentage to their sales is likely to endanger the business model of parallel importers and thereby jeopardise this additional form of supply to the UK market.
- 2.32 Further, parallel importers have limited ability to influence their own margins. Their costs are largely determined by the prices of medicines in other European markets, which depend on regulatory arrangements in those markets, and the Sterling/Euro exchange rate. Their prices are bound by the net prices of medicines in the UK market, and suppliers of parallel imports have fewer mechanisms for mitigating the risk of changes to such economic factors compared to a Marketing Authorisation holder. In particular, they cannot agree a price increase nor are they likely to be able to negotiate a better price with the supplier.
- 2.33 The Department has undertaken further analysis to establish whether the evidence used as part of the decision making on the 2018 Regulation remains valid, and concluded that this is the case.

Exemption of low cost presentations

2.34 We considered the case for increasing the low-cost exemption threshold. In line with GDP deflator, the standard measure of inflation in this area, the increase would be to £2.47, rather than £5. Our judgement is that there is not sufficient evidence that the current threshold has led to material negative supply impacts in the market for low cost presentations, or that there would be sufficient benefit from increasing the threshold to justify the resulting reduction in savings.

Exemption of blood plasma protein therapies

2.35 Blood plasma protein therapies, and their interaction with the payment percentage, are discussed in full in Chapter 4. This is because the relevant products to the discussion are currently supplied under framework agreements with the Commercial Medicines Unit (CMU) at NHS England, which were entered into prior to the 1 April 2018, and there is a relationship with the policy discussed in Chapter 4 in that regard.

Q2 Do you agree with the overarching aim of maintaining broad commercial equivalence of the statutory scheme to the voluntary scheme?

Outline of consultation proposals

- 2.36 The 2018 Regulations introduced a payment system in the statutory scheme. The payment percentage applied in the statutory scheme was set at 7.8%, aligned with the payment percentage operational in the final year of the 2014 PPRS – the calendar year 2018. This re-established a level of broad commercial equivalence with the PPRS that had been lost when the 2014 PPRS agreement adopted a payment percentage mechanism.
- 2.37 The consultation document set out our proposal to take into account any final agreement reached in the ongoing negotiations around a successor voluntary scheme to the 2014 PPRS with a principle of broad commercial equivalence between the statutory scheme and the voluntary scheme.

Summary of responses

- 2.38 Responses on the proposal were split, with 13 respondents agreeing and 18 disagreeing.

- 2.39 Respondents agreeing with the proposal argued that continued alignment of the payment percentage was necessary to ensure companies are being offered a genuine choice between schemes. Some respondents argued that this alignment should go beyond the methodology for setting payment percentages and also should result in incorporating the same exclusions and exemptions, such as the treatment of new active substances.
- 2.40 Respondents disagreeing with the proposal argued that there would be no reason for the continued administration of two schemes if there was insufficient differentiation between them. They noted that there are distinct commercial strategies and product portfolios amongst the diverse range of manufacturers supplying to the UK, and that the two schemes should be distinct so as to allow each individual firm to choose to be a member of the Scheme most suited to their particular needs. Other respondents argued that the priority for Government should be pursuing cost control of branded in a consensual manner, prioritising the commercial attractiveness of the voluntary scheme so that this covered the majority of the market. The implication of the argument from these respondents was that a statutory scheme must be less favourable than the voluntary scheme for almost all companies.
- 2.41 Some respondents stated that they saw broad commercial equivalence as effectively binding the Government to delivering the provisions set out in the statutory scheme in the voluntary scheme negotiations.

Government response

- 2.42 At the point of considering the responses to the consultation, a successor voluntary scheme to operate from January 2019 had not been finalised. Even though the Heads of Agreement to the voluntary scheme was agreed in mid-November, a proper assessment of whether the proposals under the statutory scheme achieved broad commercial equivalence with a final agreed voluntary scheme was not possible. As set out in the consultation document, with the 2014 PPRS expiring on 31 December 2018, changes to the statutory scheme have to be made by 1 January 2019 to ensure that the Government's objective of safeguarding the financial position of the NHS can be met.
- 2.43 However, an annual review of the statutory scheme regulations will take place no later than April 2019, and we therefore propose to consider the principle of broad commercial equivalence of the statutory scheme to any agreed successor voluntary scheme as part of that annual review.
- 2.44 The Government is clear on the benefit of negotiating a voluntary scheme to run alongside the statutory scheme, and of the value that is delivered by collaborating

with the pharmaceutical industry to ensure branded medicines expenditure continues to grow at a reasonable rate and patients get timely benefit from the best new medicines. It does therefore follow that we hope that many manufacturers and suppliers would see the benefits of any negotiated scheme and wish to be party to it.

- 2.45 We do not agree that broad commercial equivalence between the voluntary and statutory schemes should be an impediment to this. The successful co-existence of the current statutory scheme (as revised in April 2018) and the 2014 PPRS demonstrates this. We consider these to be broadly commercial equivalent in terms of the financial impact on their members, to the extent that they have the same payment percentage as a deliberate result of alignment. Yet the majority of pharmaceutical companies have chosen to remain in the voluntary agreement, as the other features of the 2014 PPRS are sufficient points of differentiation.
- 2.46 Broad commercial equivalence does not require the voluntary and statutory scheme to be completely the same or indeed for the payment percentages in the voluntary and statutory scheme to be the same. A design principle of broad commercial equivalence is entirely compatible with a limited number of differences in the application of the payment percentage to particular types of products, to the overall payment percentage or growth rate or in wider aspects of pricing and access that are within the remit of the voluntary but not the statutory scheme.
- 2.47 As respondents note, companies supplying branded medicines to the UK are highly diverse, in terms of size and turnover, product mix and age and commercial strategies. The statutory scheme is intended to be a scheme which is appropriate for all UK pharmaceutical companies and suppliers. In parallel to that, a voluntary scheme – where agreed – is intended to provide an additional option for companies.
- 2.48 We do not agree with responses suggesting that the statutory scheme should be designed as a deliberately punitive backstop. The two schemes may have differences in structure and exemptions, but should always work in a cohesive, complementary fashion. We believe that broad commercial equivalence is the best way of ensuring that there is a viable choice for companies at the same time as delivering the Government's overall objectives.
- 2.49 The proposals set out in the consultation did not limit options discussed in the voluntary negotiations. Broad commercial equivalence does not necessitate adoption of identical growth rates, exclusions or the other aspects of the deal that are outside the scope of the statutory scheme. The successful relationship between the current voluntary and statutory scheme demonstrates this. As a result, the Department was conducting voluntary negotiations freely and in good

faith and considered the rationale for these statutory scheme changes on their own merits.

3. Responses on the inclusion of all biological medicinal products

- 3.1 After review of the consultation responses, the Government has decided to amend the definition of "relevant medicines" in the 2018 Regulations to ensure that all biological medicinal products, as defined at regulations 8(1) of the [Human Medicines Regulations 2012](#), (including biosimilars) marketed under a combination of INN and company name come within the scope of the payment mechanism, price controls and information requirements in the 2018 Regulations.
- 3.2 The overwhelming majority of biosimilars are already in scope of the 2018 Regulations, so the immediate impact of this policy is negligible. However, it ensures the Scheme is guaranteed to capture all relevant products, whatever their naming convention, in future.
- 3.3 A summary of the issues raised in respect of this proposal is set out below, alongside the Government's response to these.

Q3 Do you agree with the proposal to bring biological medicinal products (including biosimilars) marketed under a combination of INN and company name within the scope of the payment mechanism, price controls and corresponding information requirements?

Outline of consultation proposals

- 3.4 The Medicines and Healthcare Products Regulatory Agency (MHRA) requires biosimilars to be marketed as branded medicines, which brings them within the scope of the payment system of the statutory scheme. Recently, however, the European Medicines Agency (EMA) has granted a marketing authorisation to a biosimilar medicine under a combination of International Non-Proprietary Name (INN) and company name.
- 3.5 To ensure all biosimilars continue to fall within the scope of the payment system irrespective of the naming convention, the consultation document proposed to amend the 2018 Regulations to bring all biological medicinal products, including biosimilar medicines and those marketed under a combination of INN and

company name, within the scope of the payment mechanism, price control mechanisms and information requirements in the statutory scheme.

Summary of responses

- 3.6 A majority of respondents were in favour of the proposed changes, with 24 respondents agreeing and 16 respondents disagreeing.
- 3.7 Respondents in favour of the proposal argued that there should be a level playing field for all biological medicinal products to allow for non-discriminatory competition. INN and company name should essentially be treated as a brand. Furthermore, respondents argued that due to the slow-moving nature of prescribing practices and the requirement to prescribe biological medicinal products by brand with no opportunity for pharmacy-level substitution, competition is unlikely to work effectively.
- 3.8 Respondents disagreeing with the specific proposal for inclusion of products marketed under a combination of INN and company name raised the following issues:
- The definition of branded medicines as currently in the 2018 regulations, should be an "inviolate principle";
 - Including INN-marketed products would set a worrying precedent regarding the inclusion of non-branded products in the scheme;
 - As biosimilars marketed under the INN and company name are currently not available to the NHS, the Department is addressing a non-existent problem;
 - The European approach to marketing authorisation for biosimilars is currently under review and being consulted on, any changes should therefore wait for the outcome of this review.
- 3.9 In addition, a number of respondents argued more generally that biosimilars should not fall within the scope of the payment mechanism. This was based on two principal reasons:
- Biosimilars are operating in a competitive market and already producing high levels of savings for the NHS; the Department's estimates of the reduction in expenditure upon loss of exclusivity were questioned as being too low, especially considering NHS England (NHSE) has a clear future policy statement that will encourage the use of biosimilars;

- The manufacturing process for biosimilars is complex and costly, such that similar drops in expenditure as those observed in small-molecule generic markets should not be expected in this market.

Government response

Response on the inclusion of medicines marketed under a combination of INN and company name

- 3.10 While a definition of branded medicines is set in the 2018 Regulations, it is reasonable for the Department to amend the scope of the statutory scheme following consultation where necessary to deliver the intended objectives of the scheme.
- 3.11 The Department has considered concerns that the policy sets a precedent for subjecting other medicines without a brand name into the scheme. However, the delineation between these products and other non-branded products is made and maintained clearly. This policy is founded on the specific rationale concerning the characteristics of biosimilars marketed through INN and company name, which are most appropriately treated as equivalent to all other biological medicinal products belonging to the broader category of ‘protected, originator biological medicinal products and biosimilars’. The provisions in the statutory scheme were designed to apply across this category to promote and secure non-discriminatory competition. We cannot see evidence for the assertion that an INN and company name product adopts behaviours as if it were “unbranded”, and therefore remain of the view that their inclusion in a cost control mechanism is most appropriate to align with the principles of the scheme and maintain its overall integrity
- 3.12 While it is correct that use of INN and company name to market a product is at present isolated to a single case, we do not accept this to be an argument against making the amendment to the 2018 Regulations. With the EMA having adopted this naming convention once previously, the Department needs to ensure any future biological medicinal product marketed under INN and company name would be covered as intended should the EMA do so again in the future.
- 3.13 The European Commission consultation referenced by respondents concerns a change to the assessment where a company marketing a product applies for a second licence for the same product. It would require these requests to be based on sound evidence and properly substantiated. If adopted it does not appear this would affect the EMA’s ability to grant marketing authorisations for biological medicinal products under a combination of INN and company name.

Response on the inclusion of all biosimilars

- 3.14 The Department considered the issue of competition provided by biosimilars in developing the 2018 Regulations, and set out its position in the [2017 consultation response](#). This considered information provided on the additional investment required for the development and production of biosimilars, as compared to small molecule, chemical generics, and the price pressures that already exist for this class of presentations. We accepted that research and development costs will be higher than for unbranded generics, however these costs would still be lower than for the originator medicine, and post-marketing costs should be similar between the originator and the biosimilar. In the round we did not consider that this justifies an exemption. Competitive conditions are not homogenous across different products, and will be affected both by the length of time a product has been on the market as well as the similarity between originator and biosimilar.
- 3.15 We have considered further evidence received during this consultation setting out the price discounts companies have provided for recent biosimilar medicinal products entering the market, as well as whether there might be further increases in future competition in the biologics market driven by NHS England's published commissioning framework for biosimilar products.
- 3.16 We acknowledge that some biosimilars, including some of those that were noted as examples in responses, offer a significant price reduction as compared to the originator medicine. As is clear through NHS England's commissioning policy, competition enabled by biosimilars does in aggregate offer an opportunity for savings upon patent expiry for biological products.
- 3.17 When developing our understanding of the behaviour of biosimilar products in the market place, the Department shared the assumption made in the consultation, regarding the level of expenditure decay across the molecule after patent expiry (i.e. the drop in total sales value over time of the originator and biosimilar together, accounting for both price and volume of the original and alternative products) with NHS England. We reviewed data on recent biosimilar introductions. The data shows market behaviours of biosimilars are as suggested in the original consultation. Significant pricing discounts (of up to 93%) reported in consultation responses do apply to individual products, but that is not the value of the average reduction in expenditure seen across the biosimilars market. It is the latter factor that is the relevant consideration for the Department in establishing whether it is appropriate to apply the payment mechanism.
- 3.18 Here there are - for good reasons set out by respondents - notable differences to the effect of true generic competition. These include enhanced pharmacovigilance requirements and the variability inherent in the production process for biological medicines which limit the interchangeability of biosimilars and biological

medicines. Since this results in competition working less effectively than in true generics markets, an exemption for biosimilars is not warranted.

- 3.19 We have also not seen evidence of instability in the biosimilar market as a result of including them in scope of the payment mechanism, nor that the products are unable to compete in the UK market, or finally that it is causing supply issues through an unwillingness to make early UK launches.
- 3.20 We do acknowledge there is a possibility that commissioning practices for biosimilars may change over time. In the annual review of the statutory scheme Regulations we will engage with NHS England and review additional data to ensure the inclusion of biosimilar products is still appropriate to the competitive conditions that are apparent in the market.

Q4 Do you have any evidence of further products marketed under a combination of INN and company name for which competition is limited and which could therefore be considered for inclusion in the statutory scheme?

- 3.21 No evidence was submitted by respondents in response to this question. The Government will therefore not make further changes to the definition of medicines in scope of the payment mechanism at this time.

4. Responses on the treatment of sales under Agreements

- 4.1 Having reviewed responses regarding the future treatment of sales under Agreements, the Government has decided to implement the proposals outlined in the consultation document.
- 4.2 The Government noted particular concerns from eight respondents - who represented blood plasma protein therapy manufacturers and patients - that a withdrawal of the exemption relating to future Agreements would risk supply issues given global competition and low profit margins. This is because these medicines are currently supplied through framework Agreements entered into prior to 1 April 2018, and until this point sales of albumin and Immunoglobulin products have as a result not been subject to payments under the statutory scheme. Under the changes proposed for the amendment Regulations, this will no longer be the case once these frameworks came to an end and are replaced.
- 4.3 We recognise that these products face a specific set of market conditions due to the manufacturing process and the considerations for global companies in allocating restricted stock between competing international markets. We believe that appropriate use of the existing maximum price increase mechanism is a more specific tool to ensure these products receive sufficient incentive to supply to the UK than a generic, blanket exemption, as explained in the Government response below.

Q5 Do you agree with the proposed treatment of the Agreements within each of the cohorts?

Outline of consultation proposals

- 4.4 The consultation document proposed a differentiated approach to the application of the payment mechanism to sales under Agreements, depending on the date an Agreement is entered into:
 - For sales under Agreements entered into before 1 April 2018, retain the current exemption from application of the payment percentage for products supplied under that Agreement for the duration of that Agreement;

- For sales under Agreements entered into between 1 April 2018 and 31 December 2018, continue the application of a 7.8% payment percentage for products supplied under that Agreement for the duration of that Agreement;
- For sales under Agreements entered into on or after 1 January 2019, apply the payment percentages set out in Regulations in each year, including any future changes as a consequence of the annual review of the statutory scheme.

4.5 A majority of respondents disagreed with the proposal, with 15 respondents agreeing and 20 disagreeing.

Summary of responses

4.6 Respondents raised the following issues:

- Frameworks are already delivering competitive prices to the NHS, and any application of payment percentages would simply raise tender prices;
- These price increases might distort purchasing decisions as savings made through the payment mechanism are held centrally, with local commissioners having insufficient visibility of these savings;
- There is insufficient certainty on payment percentages given potential changes following annual reviews and the fact that only three years of payment percentages have been set out while some frameworks operate for up to four years; and
- There is an inequitable treatment of branded generics where they compete with generics on frameworks, with the payment percentage not being applied to the generic product.

4.7 Manufacturers of blood plasma protein therapies raised specific concerns around the removal of the exemption for sales under Agreements going forward, given that the vast majority of blood plasma protein therapies are supplied under Agreements. Respondents argued that blood plasma protein therapies should be exempt from the application of the payment percentage, given the specific circumstances of the plasma market.

Government response on the treatment of sales under Agreements

- 4.8 The proposed treatment of sales under Agreements from 1 January 2019 follows the application of the payment percentage to sales made under Agreements entered into on or after 1 April 2018 introduced in the 2018 Regulations, and it is our view that the rationale set out in the 2017 consultation response continues to apply in light of responses received during this consultation.
- 4.9 We acknowledge that many Agreements provide a level of competition that generate savings to the NHS as compared to supply at the list or original launch price. However, there are other Agreements which do not provide significant reductions in price, such as those for which there is only one supplier able to bid, or where there are some savings achieved for the NHS but competition has not reduced prices to the levels seen in the unbranded generics market. Having established this view in developing the 2018 Regulations, we have considered whether there is any new evidence suggesting consistent sustained competition across Agreements as a whole, or whether overall savings or supply for products on Agreements would be adversely disadvantaged through inclusion in a payment percentage. We have not found this to be the case, and it is still true that the application of payment percentages does not disadvantage any one company bidding in an individual tender process, as each company will be able to take account of the payments in their tender submissions. There is no additional step involved in the procurement submission to do this; companies could submit a proposed discounted price that factors in the expected payments across their portfolio in the same manner as other costs.
- 4.10 We do not agree that potential future revisions to the payment percentages for the years 2019 to 2021 as a result of annual reviews would create unacceptable uncertainty for companies. Companies are aware of the possibility that payment percentages might be revised, and are therefore able to price in this uncertainty in their tenders in the same way they are currently pricing in uncertainty around similarly variable input costs or exchange rates over the horizon of an Agreement. Companies in any case will have an indication of payment percentages, which will be reviewed as part of the annual reviews in light of the scheme objectives
- 4.11 In addition, Agreements let by the CMU include annual review provisions, which enable companies to apply for price variations where a significant change in external circumstances warrants this.
- 4.12 We do not agree that price increases on sales under Agreements as a result of the application of the payment percentage would lead to distortions of local purchasing decisions. Prescribing decisions at the local level are made on the basis of clinical

need. The expected value of payments under the payment mechanism is passed back to the health service in full in each financial year and there will be a decision on the appropriate allocation of these funds across different expenditure categories to achieve optimal patient outcomes.

- 4.13 The treatment of branded generics sold under Agreements is consistent with the general treatment of branded generics, the rationale for which was set out in the 2017 consultation response.
- 4.14 This included medicines required to have a brand name by the MHRA, which are not as interchangeable as unbranded generics. Therefore, competitive forces will act more slowly and less effectively, which means that decreases in actual selling prices are likely to be lower and price regulation is required.
- 4.15 A company may choose to apply a brand name to a presentation where there is no requirement to do so, and where that presentation has identical generic competitors. In these circumstances the company has made a commercial decision to market the presentation as a brand, and expects to generate greater revenue as a consequence. We therefore consider that no exemption should apply.

Government response on the treatment of plasma protein therapies

- 4.16 We accept that the blood plasma products, and the associated market, are unique, given the need for human donation, the lengthy and complex manufacturing processes, and the long-term consequent limitations in global supply. We acknowledge that blood plasma products are not easily substitutable, or necessarily interchangeable. To support patient access to effective medicines, supply of a range of comparator blood products does need to be secured.
- 4.17 We also acknowledge that there are challenges for blood plasma products in relation to the proposed removal of the exemption from the payment percentage for Agreements entered into after the new regulations come into force.
- 4.18 We do not consider that an exemption provision is necessary to deal with supply risks because the existing price increase mechanism in Regulations already allows the Department to consider the impact of various costs (including the payment mechanism) when determining the maximum price. As an exemption mechanism is only a blanket measure, we think it is likely to be an inadequate mitigation to the risks raised by consultees. What is needed is the flexibility the price increase mechanism grants to make a rounded assessment of the specific situation of the

particular product, the company and the market conditions over the relevant horizon, tailoring any price increase required accordingly.

- 4.19 We therefore consider that the price increase provision is a more appropriate way of considering all of the factors which may put supply at risk, focusing on the individual circumstances of a product.
- 4.20 We accept that the process for requesting and agreeing price increases has an associated lead in period in which the request is considered, so any desired change would not take place immediately. This has not had a material effect on the security of supply of the majority of products where a price increase is sought, not least as in exceptional circumstances an exemption from the maximum price can be applied, but we do recognise that respondents raised other factors that might mean that this is not effective for blood plasma products. These include extended manufacturing timescales, which would mean that pricing decisions would impact on supply 7 to 12 months hence, rather than immediately.
- 4.21 While we recognise the particular challenges around production lead-in times in the blood plasma markets, they do not preclude companies from applying for price increases sufficiently in advance to be able to secure a price increase where necessary in time for their internal sourcing and production processes. We acknowledge concerns raised about the limited historical use of the price increase provision. If companies are making allocation decisions on the basis of expected international market conditions during the planning horizon, the Department has the ability to review a price increase application with the relevant supporting data and can take future conditions into account, where anticipated market conditions and comparator prices are relevant to setting a price that considers the need for blood plasma products to be able to be supplied to UK patients on reasonable terms.
- 4.22 We therefore believe that price increases demonstrably can provide the necessary flexibility to respond to the unique circumstances of blood product manufacturers.

5. Responses on statutory duties

5.1 The consultation document included an assessment of the relevant statutory duties. We have made some changes to the Impact Assessment based on feedback from respondents, and accounted for this in re-considering the impact on these factors. Our analysis is presented below.

Q6 Do you agree with the analysis in the accompanying Impact Assessment on: (a) the impact of our proposals and (b) the effect on those areas where the NHS Act 2006 requires we consider and consult?

Q7 Do you have any evidence that would help inform, and improve the quality of, our analysis?

5.2 Respondents felt that there were inaccuracies in the methodology the Government had used to set out the impact on the life sciences sector, the UK economy and NHS patients. They also stated that some of the assumptions made were not correct and suggested alternatives. The main points raised were:

- The impact of the increased payments made by pharmaceutical companies on shareholder profits are understated because of the methodology used - they should be deducted in full;
- An unfavourable commercial environment, which these proposals would result in, would generate negative sentiment in global boardrooms, and this has a direct relationship to inward investment decisions;
- The impact assessment should incorporate other costs that the industry will also face concerning commercial uncertainties and contingency preparations associated with Brexit, worsening exchange rates and cost-recovery for NICE appraisals;
- Using an assumed £15K/QALY figure for opportunity costs in the NHS to monetise the impacts (from a University of York study) is invalid and has been contested by other economists. This should undergo an independent review if it is to be applied;

- The treatment of wider social benefit (which is considered here) is inconsistent with NICE appraisals that determine whether a medicine is funded;
- The assumption regarding average company profits of 30% is inaccurate and in any case, does not account for the fact that companies growing slower than the market rate may be adversely affected and be commercially unfeasible

Government response

- 5.3 We have considered the above points and revised a number of assumptions in the associated Impact Assessment to reflect the evidence or challenges raised by respondents. The Impact Assessment is published alongside this document and sets out in full consideration of the points made by respondents regarding the methodology for considering R&D implications and wider economic uncertainties, which can be found in paragraphs 127 to 134. We also responded to these points in Chapter 2 (2.12 - 2.15)
- 5.4 Regarding the figure used for opportunity costs in the NHS to translate increases in NHS funding into Quality Adjusted Life Years (QALYs), this is the standard figure used across DHSC Impact Assessments, because it is supported by independent peer reviewed literature. We therefore consider use of the figure to be appropriate.
- 5.5 The Impact Assessment has been amended to take into account explicitly the possibility raised by respondents that over the short term, companies might be unable to take mitigating actions to limit the impact of reduced revenues on profits, and that therefore reductions in revenue translate directly into lower profits for UK shareholders. Importantly, this change does not alter the qualitative result of the Impact Assessment, which continues to show a significant net benefit to the UK economy.

Q8 We welcome any comments, including any evidence, on our assessment of proposals in relation to the public sector equality duty and Secretary of State duties under the NHS Act 2006

- 5.6 Most respondents disagreed on the basis of fundamental disagreement with the construction of the IA. Where specific additional concerns were raised in relation to the Public Sector Equality Duty, the general Secretary of State duties under the NHS Act 2006 or the consultation factors and specific duties related to medicines pricing under sections 263 and 266 of the NHS Act, respondents noted:

- The duty to promote a “comprehensive health service” designed to secure improvement in the treatment of illness has been inaccurately judged as the proposals will significantly delay the launch of some medicines in the UK;
- The duty to promote research and the use of evidence obtained from research in the health service will be negatively affected because the impact assessment acknowledges there will be a decrease in R&D spending as a result of the proposals;
- The duty related to "reducing health inequalities" has not been properly considered as for individuals with ultra-rare diseases, the range of treatment options would be limited in the event of supply problems or UK launch delays;
- The Department needs to also consider the Family Test because the impact on products such as plasma protein therapies, and other genetic conditions, reduces the likelihood for patients with inherited, chronic conditions to receive medicines.
- the duty to secure education and training is not considered, even though the significant services industry deliver in the NHS in this regard will be reduced due to the constraints the size of the payment would impose on budgets.

5.7 In relation to the specific duties to consult upon and consider in making proposals, the factors set out at section 263 of the NHS Act 2006 regard the:

- economic consequences for the life sciences industry in the UK;
- consequences for the economy of the UK; and
- the consequences for patient access to medicines.

As we noted above in considering the comments on the Impact Assessment, some respondents noted clear disagreement, specifically related to these duties with the conclusion that “company revenues from the NHS should [not] affect the attractiveness of the UK as a location for R&D”. They argued the two are intrinsically linked and the proposals would have a considerable role in the continued downgrading of the UK in the receipt of life sciences investment.

Government response

Responses on general duties under the NHS Act 2006

Duty to promote a comprehensive health service (Section 1 NHS Act 2006)

- 5.8 We do not accept respondent's contention that the proposals will negatively impact the supply and launches of medicines in the UK market. The commercial attractiveness of a market is determined by a range of factors, many of which strongly favour the UK, including the international value of a NICE assessment, a UK list price and the co-location for clinical trials. These factors will not be affected by the current proposals.
- 5.9 Where individual products might not be commercially viable in the UK under the current proposals, the Department has provided a clearly set out route to securing supply with the price increase provision set out in the 2018 Regulations. The price increase provision provides a mechanism to consider the ability of the company to supply a product, the clinical need for that product, and its cost.
- 5.10 We also do not accept that the proposals would disadvantage patients with rare diseases or no available low cost generic treatment options. The NHS will continue to fund medicines which have received a positive NICE appraisal, irrespective of whether these medicines are low cost generics or branded health service medicines.
- 5.11 We do not agree with respondents who argue that they would be unable to take account of payment percentages in tender submission. Companies currently take account of overheads – such as taxes or R&D costs – in making commercial decisions on product prices, and we consider the payment percentage to be a similar business cost. While it is true that payment percentages might change over the lifetime of a framework agreement, companies would be aware of this possibility in advance and could price in any uncertainty accordingly, in the same way uncertainties around other input costs are priced into tender submissions.
- 5.12 Moreover, we remain of the view that the additional savings generated through the proposed changes will be reinvested into the health service, thereby increasing resources available to provide a comprehensive health service.

Duty as to reducing inequalities (section 1C NHS Act 2006)

- 5.13 Respondents argued that smaller companies disproportionately produce medicines for rare conditions, or conditions that disproportionately affect some communities. As set out in the consultation document, we do not propose any changes to the small companies exemption currently operating in the statutory

scheme, which means that any company with sales of branded health service medicines of less than £5 million per annum will not have to make payments. Furthermore, where medium sized companies feel that supply of a product becomes economically unviable, they would be able to apply for a price increase using the existing price increase provision. The price increase provision provides a mechanism to consider the ability of the company to supply a product, the clinical need for that product, and its cost. In considering price increase applications, the Department would consider the impact of its decisions on relevant patient groups.

- 5.14 Similarly, we do not accept that the proposals would disadvantage patients with high levels of unmet need, for two reasons. Firstly, the uptake of new medicines (which respondents contend would be negatively impacted by our proposals) is determined by a range of factors, only one of which is the overall medicines budget. The Department is working actively with NHS England and the ABPI to improve access and uptake across the board, and our current proposals need to be seen in the context of these efforts and the wider medicines regulation landscape in the UK. Secondly, we believe that our proposals have the potential to improve services for patients with unmet need. This is because they will increase resources available to the NHS, which explicitly takes into account unmet need as a factor in allocating expenditure across local areas (see [Unmet need health inequalities adjustment](#)).

Duty to promote research (section 1E NHS Act 2006)

- 5.15 As set out in Section 2 above, we maintain that decisions on the distribution of international pharmaceutical R&D investment are largely driven by supply side factors which the UK will continue to perform favourably on and which are not affected by our proposals.
- 5.16 While respondents have noted a reduction of UK pharmaceutical investment between 2011 and 2016 documented in the Office for Life Sciences Life Sciences Competitiveness Indicators 2018, considering a wider range of indicators - e.g. academic citations, enrolment in clinical trials, foreign inward investment - in the round does not support the hypothesis that the 2014 PPRS, which operated under a 1.1% average annual growth rate, has led to material negative impacts on the attractiveness of the UK pharmaceutical market.
- 5.17 Considering the responses to the consultation and wider evidence in the round, we therefore do not believe that the current proposals negatively impact on the duty related to research.

Duty as to education and training (section 1F NHS Act 2006)

- 5.18 It was not clear from responses what form of education and training was referred to. While we acknowledge that the companies provide informational resources and events to medical professionals around their specific products, we would regard this type of activity as part of companies' marketing efforts, which are not covered by this duty.
- 5.19 The ability of Health Education England to carry out its function as the main Arm's Length Body overseeing education and training for medical professionals in the NHS remains unaffected by the current proposals.
- 5.20 In summary, we therefore do not agree with respondents who argue that there is a risk to education and training.

Responses on duties specific to the statutory scheme

Consultation factors under section 263(1A)

- 5.21 We have addressed points around supply and launch of medicines in the UK, including those relating to smaller companies and rare diseases, in the section discussing duties under section 1 of the NHS Act 2006 above.
- 5.22 The impact assessment accompanying the consultation uses HMT Green Book principles to estimate impacts on the UK economy of the proposals. It is consistent with these principles to only account for spill over effects of R&D, as the resources utilised in pharmaceutical R&D would be used otherwise in the absence of this R&D activity.
- 5.23 We acknowledge that the Impact Assessment does not attempt to quantify any benefits on patient health from reductions in R&D activity. However, these impacts are unlikely to be material, given the magnitudes involved – the impact assessment estimates a reduction in global R&D spend of c. £58 million in 2021 (10%, or £5.8m, of which is estimated to accrue to the UK), which compares to c. £148 billion in global pharmaceutical R&D expenditure as sourced from the [ABPI](#), with the 2016 figure extrapolated to 2021 at the 2006-2016 compound annual growth rate of 3.8%, and converted into GBP at 0.78GBP/USD. This is 0.04% of global expenditure.
- 5.24 The Department has also acknowledged the specific issues faced by the blood plasma industry. Following careful consideration of the evidence, including engagement with the Plasma Protein Therapeutics Association (PPTA) and NHS England's Commercial Medicines Unit (CMU), we continue to believe that the existing price increase provision provides an appropriate mechanism to address these concerns, as it would allow companies to increase their sales price following

a decision of the Department's pricing committee, which will consider evidence around threats to supply in deciding on price increase applications. Furthermore, we propose no changes to the existing temporary exemption provision in the Regulations, which allows the Secretary of State to exempt any presentation from the price control mechanism where necessary to ensure adequate supplies.

Duties under section 266 of the NHS Act 2006

- 5.25 The areas for consideration under section 266 of the NHS Act 2006, require the Secretary of State to exercise his powers in a way which would be reasonable in all the circumstances and to bear in mind in particular the need for medicinal products to be available for the health service on reasonable terms, and the costs of research and development. These duties are closely related to the general duties to promote a comprehensive health service and to promote research discussed above. Our assessment is therefore broadly similar to the discussion in the preceding section.
- 5.26 We have taken both supply issues and impacts on research in development into account in the development of our proposals and re-evaluated our assessment in light of the responses received during the consultation. The relevant issues are discussed in the sections above discussing duties under section 1 of the 2006 Act as well as section 1E of the 2006 Act.
- 5.27 The proposal in the consultation that would affect pricing controls as referred to in section 266 are the changes related to biological medicines marketed under a combination of INN & company name, which we propose will come within the scope of the price controls. Respondents did not comment specifically on this duty. However, in light of the responses received, we do not believe the proposals would have negative effects on the need for medicinal products to be available to the health service on reasonable terms or the costs of research and development as they are a consistent extension of the current policy around biological medicinal products and biosimilars. A discussion on the effect of inclusion of these medicines is set out at paragraphs 3.14 to 3.20 above.

Responses on the Public Sector Equalities Duty

- 5.28 As set out above, our proposals include a continuation of the existing SME exemption, thereby providing specific support for smaller companies. Similarly, as discussed above we do not accept respondent's contention that the proposals would negatively affect the availability of medicines which are clinically necessary for patients. Where individual products would become unviable to supply under our proposals, companies would have the ability to submit a price increase application to the Department.

5.29 Some respondents to the consultation have argued that our proposals disproportionately affect blood plasma protein therapies, and thereby disadvantage patients requiring these treatments. We have assessed these arguments in the policy making process and concluded that in light of the responses received during the consultation the existing price increase provision facility remains the most appropriate way to effectively mitigate any potential risks to supply from the application of the payment percentage. This is because the price increase provision allows the Department to consider the circumstances of each company and product individually and come to a view on the adequate price to secure supply, and is therefore preferable to a general exemption provision which was asked for by some respondents.

Family Test

- 5.30 One respondent argued that an inability of patients with inherited, chronic conditions to receive their therapies will have an impact on the family test.
- 5.31 It is not clear from the response which specific family question the respondent believed would be affected. The premise of the response however seems to be that patients will lose access to certain medicines as a result of our proposal. As set out above, there are mechanisms in place to prevent this.
- 5.32 We therefore do not believe that any areas of the Family Test are impacted by the proposals.

6. Responses on the forecasting model

6.1 The consultation document explained the methodology used to forecast branded medicines expenditure for the years 2019 to 2021. In summary, the approach uses past data on medicines expenditure to estimate parameters of a representative product life cycle for four categories of medicines (primary/secondary care and biological/non-biological medicines). In addition, a cohort growth rate, capturing the degree to which spend on newer medicines is higher than spend on older medicines at equivalent points in their lifecycle, was estimated. Aggregate forecast expenditure on medicines at any given point in time is therefore given by the sum of forecasts for individual medicines and cohorts of medicines derived from the estimated lifecycles.

Q9 Do you have any comments on our use of a data-driven approach to forecasting based on product lifecycles?

6.2 Health service respondents agreed with the methodology used to construct the model. However, the majority of respondents believed that the forecast annual rates of growth over the period were too high. Those who disagreed stated that they had the following issues with the overall methodology deployed:

- Forecasts based on existing market trends are only appropriate for short run periods, while the impact of future launches should be estimated using horizon scanning, market insights and expert interviews;
- Market dynamics are going to change significantly over the next 2 to 3 years with several large brands losing exclusivity, alongside changes to NHS procurement, potential changes to the supply environment and the introduction of Regional Medicines Optimisation Committees (RMOCs) and these are not fully considered;
- The model is not sufficiently sensitive to reflect expenditure on medicines launched between 2015 and 2018;
- The DHSC approach does not consider fully the effects of competition prior to and after loss of exclusivity nor adequately take into account the impacts of therapeutic tendering; and

- Basing the forecast on four broad categories of medicines is not a granular enough approach to capture differences between therapy areas, and results in assumptions that all types of branded medicines will fit to similar lifecycles (failing to account, for example, for branded medicines that are required to have a brand name).

6.3 In addition, respondents highlighted issues with the parameter used to establish the reduction in expenditure for a molecule once a patent has expired:

- The derivation of the six-month gap between loss of exclusivity and drop in expenditure is unclearly explained, and the estimated gap is too long as savings after patent expiry are realised much earlier; and
- The drop upon loss of exclusivity seems to be underestimated, which could be caused by either looking at expenditure before and after patent expiry (rather than competitor entry) or by calculating the drop at molecule level (where some formulations might still be patent protected). Some respondents argued that for biological medicines, the price drop should be in the region of 65% or higher, rather than 45%.

Government response

Response on the overall methodology

6.4 We acknowledge that there are different approaches to forecasting medicine expenditure, and that any forecast will have uncertainty attached to it. The Department has conducted a range of sensitivity analyses to understand the sensitivity of expenditure forecasts to individual parameter estimates, and will continue to keep the assumptions used in the model and the accuracy of the overall model under review, refreshing it with new data as it becomes available.

6.5 While many industry respondents have highlighted the importance of expert judgement in producing forecasts, the Department has not seen evidence that expert views produce more reliable forecasts than quantitative techniques based on observed data. Studies from the US market (Cha et al. (2013): "Pharmaceutical forecasting: throwing darts?", *Nature Reviews Drug Discovery*) show that a majority of analyst forecasts result in error for peak sales of new medicines by 40% or more. It is noted that the Department's aim of constructing a whole-market forecast of aggregate medicine expenditure differs from the individual product-level forecasts that are produced by pharmaceutical companies.

6.6 In response to a specific concern that modelling undertaken discards data for medicines launched since 2015, the Department wishes to clarify that this is not

the case. It is simply that with the more limited time series of data available for these more recently launched medicines, it is more appropriate to forecast such products not individually, but rather at the aggregate level, as annual cohorts.

- 6.7 We do not believe that arguments related to recent or future market changes (e.g. therapeutic tendering, changes in the Cancer Drugs Fund, RMOCs, patent expiry of high expenditure products in near future) invalidate the approach taken. This is because such trends have generally been affecting the medicines market for some time, and therefore will be reflected in recent growth rates, while the impact of any future changes is uncertain by definition. Our assessment is the issues raised are unlikely to actually exert downward pressure on expenditure that is not already baked into our forecast through taking a view of historic trends, with the exception of biosimilar competition, where - as reflected - we did increase the expected effect to account for future NHS England policy.
- 6.8 For example, reforms to the Cancer Drugs Fund in recent years will have been reflected in the data used for the forecast. Similarly, patent expiry dates are included in the model, and so significant rates of expenditure reduction, consistent with past major patent expiries, but based on the level of expenditure for the relevant molecule are clearly seen in our forecast. No evidence was provided to suggest we should expect a step-change where the rate of expenditure decay will in future be higher than those observed in historic cases.
- 6.9 The Department has noted that there are a number of trends, such as an increasing rate of NICE approvals that may contribute to increased medicines expenditure, but has determined it is more appropriate to utilise the data-driven model rather than applying a subjective overlay to inflate the forecast where there may be uncertainty. Should any future policy change have significant inflationary or deflationary impacts compared to the predicted growth rate, the annual review mechanism would be the appropriate way to consider how to handle this.
- 6.10 While the forecast model assigns life cycle parameters on the basis of the four categories of medicines for which parameters were estimated, for those products launched before 2015 the available expenditure trends (based on data extracted up to 2017) in the uptake period are taken into consideration and a forecast is generated at the product level.
- 6.11 Generally, it is likely that there are individual products for which certain parameters will differ from the estimated aggregate parameters. As the goal of the model is to predict the growth rate of aggregate medicine expenditure though, these outliers do not pose a problem unless error can be shown to be structurally biased in one direction.

- 6.12 In particular, there would have to be a subset of the market which is currently being included within the broader categories for which the behaviour of expenditure behaves significantly differently from the category average, and which would also grow significantly in future such that their behaviour has a measurable impact on the overall market.
- 6.13 Increasing the granularity of the model also decreases the precision with which parameters can be estimated. This is because with a larger number of groups of medicines, parameters for each group will necessarily have to be estimated on the basis of a smaller set of products within each group.
- 6.14 Some respondents suggested the Department should engage with the ABPI, in order to use a separate forecast model methodology that they have developed. The Department has considered a range of different forecasting methodologies during the development of its model, including those used by IQVIA and EvaluatePharma, which use individual product forecasts or therapy area forecasts based on expert knowledge. The Department's model is predominantly data-driven and statistical, whereas the alternative would be to accept overlays whereby individual product forecasts, or therapy area forecasts, are adjusted manually based on expert knowledge. The Department accepts there are some benefits to using such a methodology, but we are concerned it is subject to unconscious bias and is difficult to quality assure due to its subjective nature.
- 6.15 On balance, the Department understands how the ABPI model could be considered as an alternative approach, but is not convinced it offers any substantial advantage over the Department's model as a basis for forecasting and establishing payment percentages. Furthermore, the approach would be incompatible with a data-driven annual review process.

Response on the parameter concerning expenditure decay at loss of exclusivity

- 6.16 The estimated time gap between loss of exclusivity and decreases in expenditure of 6 months is an average across molecules and is used to approximate a gradual decline in expenditure through a vertical drop in expenditure. The anecdotal evidence provided by respondents that this period is shorter for some molecules does not invalidate our conclusion which is based taking into consideration the behaviour of all products.
- 6.17 The estimate was based on the following patterns observed in the data:
- The duration of the drop from beginning to end was: 6-32 months in primary care; 5-54 months for Secondary Non-Biologicals; 4-43 months for Secondary Biologicals;

- The median drop length was 9 months in primary care and 11 months for both Secondary Biologicals and Secondary Non-Biologicals.

Given these results we approximated a gradual drop over the period of one year with a vertical drop after six months

- 6.18 The opinion submitted by one respondent that the reduction in prices upon biosimilar entry should be 65%, as opposed to the 45% drop in expenditure estimated by DHSC. These two figures are not inconsistent. Given that for biological medicines, originator medicines often retain significant market share after entry of a competitor due to limited substitutability of products for existing patients, a 65% drop in the price of a biosimilar would be consistent with a 45% drop in expenditure on a product if the originator retained 30% of the market share. These assumptions were agreed with NHS England as generally representative of the biosimilar market.
- 6.19 A recent example of the impact of NHS England policy on biological medicine expenditure is the [guidance on the usage of Adalimumab](#) issued to Trusts and CCGs. The expected savings from increased use of biosimilars in this area are in the region of £150 million per year, or slightly over a third of the total annual expenditure on this medicine of over £400 million - a total drop in expenditure roughly comparable with our parameter choices.

Q10 Do you agree with our approach to modelling the plateau gradient in the lifecycle?

- 6.20 On the approach to modelling the plateau gradient (Q10), a majority of respondents disagreed. Respondents raised the following concerns:
- The approach is too simplistic, and unable to capture the wide range of dynamic elements that influence the future medicines bill. The effect of competition on growth is not adequately captured, as effects of competition are ‘averaged’ across the four categories of medicines considered; and
 - The plateau gradient assumes that the amount of money spent on a medicine remains flat during the plateau period, which is at odds with the experience for some products (Hepatitis C, breast cancer, multiple sclerosis and rare bone disease are provided by respondents as examples).

Government response

- 6.21 We have not seen evidence in response to the consultation that there are particularly competitive submarkets (which would have lower plateau gradients) which are currently being included in one of the four categories which will significantly grow in future, thereby depressing overall plateau gradients. By definition, expenditure will be lower on highly competitive products, such that they will make up less of total medicines spend and thereby have a lower impact on aggregate forecasts.
- 6.22 More broadly, the positive plateau gradients in three out of the four product categories considered reflects historic trends in market penetration of branded medicines, with sales volume continuing to grow up until patent expiry due to a combination of demographics and approval for additional indications.
- 6.23 The plateau gradient is non-zero for all four categories of medicines, ranging between -1% p.a. on non-biological primary care medicines to 8% p.a. for biological secondary care medicines. The estimates are based on considering all relevant products in each category, so it is natural that certain products within each category will exhibit plateau gradients which are higher or lower than the central estimate. The evidence provided does not support the hypothesis that the central estimate is biased.
- 6.24 Following consideration of the responses received, we remain of the view that our approach to modelling the plateau gradient is an appropriate way of capturing the behaviour of product expenditure following full uptake.

Q11 Do you agree with our approach to modelling cohort growth rates?

- 6.25 A majority of respondents who commented disagreed with the approach to modelling cohort growth rates. Concerns raised were:
- 2016 and 2017 cohorts of medicines were excluded from the cohort growth analysis;
 - The consultation states that expert opinion was sought in the cohort growth rate modelling, but not whether industry views were incorporated;
 - Anchoring the analysis in historic trends does not take into account impacts on medicines already available, expansion of the market, or recent changes in NHS procurement practice; and

- Expenditure on more recent cohorts of medicines is higher due to faster rates of uptake, rather than total expenditure of these medicines being higher once full uptake is reached.

Government response

- 6.26 Products launched in 2016 and 2017 had to be excluded from the cohort growth analysis as at the time of the analysis as only one annual data point was available and therefore no growth rates could be calculated. The analysis was therefore carried out on the best available data, and will be refreshed as additional information becomes available. While this is a limitation of any approach that uses historically observed trends to forecast future expenditure, we believe that the approach taken remains the most appropriate in light of the advantages it offers over less data driven approaches such as those described in 6.16-6.18.
- 6.27 Expert views were sought from NHS England Specialised Commissioning as well as from other Arm's Length Bodies. The Department commissioned external consultancy support in constructing the forecasting model and these individuals held significant experience working in the life sciences industry and are familiar with industry forecasting models and commercial drivers.
- 6.28 With regard to the relevance of historic data, a number of changes referenced by respondents have been affecting the market for some time and would therefore be visible in recent data, while the impacts of any potential future changes in policy or market conditions are unknown at this time and could be considered as part of annual reviews, were they to have a significant effect on aggregate outcomes. In any forecast there will be uncertainty not only due to limitations of data and assumptions, but also due to future events or as yet unknown policy changes. The annual review mechanism gives the department the opportunity to react to such changes, where they have a material impact on the forecast of expenditure.
- 6.29 The Department has undertaken sensitivity analysis around the possibility that observed cohort growth is driven by faster uptake rather than by higher levels of spend on more recent cohorts. This analysis shows that over a three-year horizon, the impact of this would be limited, with overall UK medicines expenditure growing by c.£120m less compared to the central estimate.
- 6.30 However, for this mechanism to work, uptake periods would have to be extremely short for newer cohorts (see Figure 1 below). The implication is that later cohorts (e.g. products launched in 2023) would need to end their uptake period between one and two years after launch in order for the chosen cohort growth parameter to simply result in faster uptake, rather than higher levels of expenditure. We do not

consider the short uptake periods that would be required in the model to match observed cohort growth to be plausible.

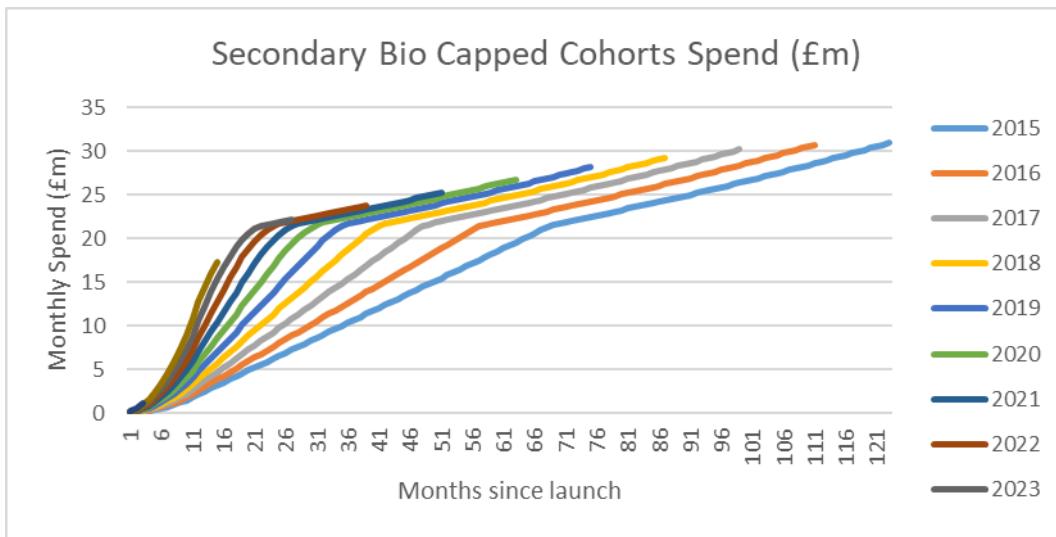


Figure 1: Implied uptake period for new cohorts if cohort growth is assumed to be driven by faster uptake rather than higher total expenditure

- 6.31 The approach of using expert prediction discussed at paragraph 6.14-6.15 can be adopted for products yet to be launched, as much as for existing products. However, analyst predictions as to the value of the future medicines pipeline generally hold constant (or trend flat) simply because these launches are unavoidably harder to confirm and value at this point of time. A flat trend is out of line with external literature on expectations regarding the future development of the pharmaceutical pipeline and associated costs for payers.
- 6.32 We remain of the view that cohort growth rates represent an important feature of product lifecycles, and that our approach to modelling them is appropriate for determining expected future growth.

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