



**Developing a method to
assist investment
decisions in specialised
commissioning: NHS
England's response to
consultation**

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Developing a method to assist investment decisions in specialised commissioning: NHS England's response to consultation

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1 Introduction

1. In 2015 NHS England held a public consultation called *Investing in Specialised Services*. As an outcome of that consultation NHS England agreed a number of principles to inform how it would prioritise new investments in specialised services. In our response to the consultation (June 2015) we described further work that we would undertake in 2015/16 to develop a method that could be used by the Clinical Priorities Advisory Group (CPAG) when forming recommendations on the relative prioritisation of new investments.
2. NHS England's public consultation *Developing a Method to Assist Investment Decisions in Specialised Commissioning* was held between 11 April and 11 May 2016. The consultation guide described a detailed method that it proposed would be applied by CPAG in the 2016/17 commissioning round. A number of questions were asked of respondents.
3. Pre-consultation engagement took place where possible so that stakeholders were fully informed. This included a presentation (and question and answer session) with industry representatives. To support the process of consultation NHS England hosted two webinars which were well attended and we held a stakeholder event attended by 60 people from a range of interests. In total, NHS England received 117 responses through the consultation hub and a further 5 responses through email and post. In terms of "sunshine" / conflict of interest disclosures, one third of respondents had received funding from the pharmaceutical sector, as had two-thirds of the patient charities / voluntary groups who responded
4. The report of an analysis of consultation responses by the Chartered Institute for Public Finance and Administration is included as an Appendix.
5. Responses to the consultation addressed both the immediate process of making relative prioritisation decisions for the 2016/17 year, and also addressed process issues that will be relevant to our decision making for 2017/18 onwards. NHS England wishes to thank everyone who took the time to respond to consultation, which has resulted in a rich source of views and ideas.
6. This document provides NHS England's response to the feedback received on consultation questions as described in the consultation analysis report.

2 Question: NHS England has concluded that there is no existing method for relative prioritisation that could be directly applied to the process of prioritising proposed investments in specialised services, and has described in this document the process for developing the proposed method. Do you agree with the proposed method?

7. The analysis of consultation feedback concludes that responses to NHS England's proposed method for relative prioritisation were divided between those who agreed and disagreed. However, many of the respondents who chose the "disagree" option explained that they were not proposing to delay the process of prioritisation for 2016/17. Rather, they called upon NHS England to adopt the method for 2016/17 on an interim basis and to further develop the method for future years, informed by the learning of this year's commissioning round. The need for a process to be operational for 2016/17 by June 2016 was acknowledged by respondents so that patients are able to benefit from new interventions as rapidly as possible.

8. The independent analysis of consultation responses provides an overview of positive comments about the proposed method:

- The intent to establish a more pragmatic decision-making framework
- The desire to improve the consistency and reduce the subjectivity of the approach
- The acknowledgement that it should help provide evidence to support specialised commissioning of services
- The recognition of the need for a clear and transparent method for relative prioritisation of treatments
- The facilitation of discussions about value for money

9. **For 2016/17 we will adopt the proposed method, but recognise the need to develop iteratively the method, including learning from the application of the method for 2017/18.**

10. Many helpful and detailed submissions were made about how to develop the method in the future, including calls for clarification on key aspects of the process. In this document we describe how we will further explore the issues that were raised during consultation.

3 Question: If you answered "disagree" to the above question, please provide details of alternative method(s).

11. Some respondents argued that alternative methods for relative prioritisation are available. Some also suggested that the length of the consultation has been too

short to enable alternative methods of prioritisation to be developed for submission to NHS England.

12. The purpose of this consultation was to seek views on the specific method developed by NHS England over the past year. However, reflecting NHS England's acknowledgement that development of the method will be iterative, we are keen to work with stakeholders, including a future consideration of alternative methods or elements of a method that stakeholders identify.

4 Question: Do you agree that the method proposed by NHS England:

4.1 Is transparent?

13. NHS England's commitment to a transparent process was welcomed, and respondents were interested in more information on how this will be achieved.

14. Over the past year the transparency of the process for developing clinical commissioning policy propositions has been significantly increased through more focused stakeholder engagement and consultation on the documentation that it is presented to CPAG to support each policy proposition. **For 2016/17, once NHS England has made a final decision on which proposals will be funded, we will publish the recommendations that were made by CPAG to NHS England with a summary description of the reasoning for CPAG's recommendations.**

15. In regard to 2017/18, detailed proposals were made by some respondents about how the transparency of the process overall may be improved, from development of the clinical commissioning propositions through to final decision by NHS England, and we will carefully consider these proposals further.

16. Respondents asked for clarity on whether an "appeal" process will be adopted if evidence has been inappropriately graded or overlooked. The current process already allows for this. All policy propositions benefit from an initial period of stakeholder testing to "sense check" the proposal, followed by a process of public consultation on each policy proposition before submission to CPAG. The testing and consultation provide manufacturers, clinicians, academics and other stakeholders with the opportunity to confirm or challenge the accuracy and interpretation of the evidence review. Stakeholders who are unhappy with the outcome of the prioritisation process are entitled to raise a formal complaint via NHS England's existing complaints process. As such, we do not consider that an additional formal appeals process is necessary.

17. Respondents have also asked for an explanation of how proposals that are not funded for 2016/17 may re-enter the process of relative prioritisation in future years. We need to balance the need for a process that is flexible when this is appropriate, with one that enables NHS England to make decisions that are final when this is appropriate. **We will give this further thought for future years, but for proposed investments that are not funded by NHS England in 2016/17 on the grounds of affordability we will ask the relevant National Programme of**

Care to consider whether the proposed service development should be included in the 2016/17 work programme alongside proposals for new clinical commissioning policies.

4.2 Will facilitate rational and consistent decision making?

18. Views were mixed on this question. Although some respondents were in agreement, some suggested that the proposed method may lead to unpredictable results year to year because of the relative nature of prioritisation and the fact that the budget available for new investments will vary each year. Our view is that the proposed method will increase consistency and predictability in decision making through its focus on sound principles around evidence of effectiveness and value for money. The fact that this is indeed a relative process, and the fact that the available budget may vary each year, do not in themselves support the suggestion of inconsistency and unpredictability, but this is something that we will review over time. Precisely because it is about choosing possible investments within a fixed budget constraint it inevitably operates differently to National Institute for Health and Care Excellence (NICE)-type appraisal processes.

19. In response to comments that our process of relative prioritisation needs to be aligned with broader strategic objectives, we will consider the report of the Accelerated Access Review (AAR) once it is published. The AAR aims to speed up access to innovative drugs, devices and diagnostics for patients.

4.3 Has, at its foundation, the core principles of demonstrating an evaluation of cost effectiveness in the decision making?

20. We received some very detailed and helpful comments on the proposed method. As many of the proposals relate to earlier stages of the process for developing clinical commissioning propositions, we will carefully consider all of the suggestions put to us by respondents and where appropriate we will engage further with stakeholders to help us learn from the 2016/17 commissioning round and refine the method for future years. In this document we have addressed the key themes raised by the consultation, but in terms of cost effectiveness, we will expect to consider:

- The potential use of the concept of minimum clinically important difference (MCID) as a ‘unit of health gain’, and test whether gains could be measured in multiples of MCID relevant to each particular proposition
- The basis on which the “cost” of a proposal is calculated, including the extent to which a consideration of opportunity costs can be introduced for future years alongside the method that we will adopt for 2016/17; we will also seek greater consultation feedback to the impact reports
- Whether we can introduce a “low value” threshold below which proposals would be excluded from prioritisation
- Whether the range of metrics to describe patient benefits should be re-visited, including a consideration of existing methods for measuring societal factors, and patient-reported outcome / experience measures.

5 Question: Please comment on whether the following four principles are applied at the appropriate point in the proposed method of relative prioritisation:

5.1 NHS England will normally only accord priority to treatments or interventions where there is adequate and clinically reliable evidence to demonstrate clinical effectiveness

21. Although the method's focus on evidence of effectiveness was broadly welcomed, some respondents suggested that our definition of "clinically reliable evidence" is overly stringent and excludes sources of evidence that may be relevant and useful. We will review for future years our definition in the context of the sources of information that are analysed by the evidence reviews for policy propositions. We will also review the process for quality assuring the summary reports that prepared for CPAG.

22. Some respondents suggested that the proposed methods for grading evidence are biased against medical devices and technologies as they favour interventions for which randomised controlled trials and meta-analysis are possible. We describe below that we will develop a principle that the level of evidence required should be proportionate to the patient group and the technology. We are working with NICE and industry groups to develop a horizon scanning tool for medical devices that, if adopted, would identify priority topics for evaluation at a much earlier stage enabling earlier adoption.

23. Some respondents queried why the review of potential methods undertaken by the University of Sheffield excluded a consideration of 'incremental cost-effectiveness ratios' (ICER) or 'quality adjusted life years' (QALY), and suggested that "tried and tested" methods such as cost utility analysis may be appropriate. NHS England's objective has been to develop a method that would enable a consistent evaluation of the broad range of patient benefits offered across the diverse portfolio of specialised treatments (drugs, medical devices, and acute and psychological interventions). We concluded that our work to develop a method of relative prioritisation should not be expanded to cover the complex field of health economics in ICER evaluation, and we concluded that unfortunately in practice the current NICE methodology would not deliver this.

24. Some respondents suggested that we should consider a quantified assessment of benefit against cost. We have explored this for 2016/17 and do not believe that there is a methodology that can be applied. We accept that the NICE method of assessment is superior in using quantification, but that this is not transferable to NHS England's process of relative prioritisation, and we will where possible, in future, ask NICE to increase their evaluation capacity so that where the method can be applied NICE may appraise the proposals.

25. Although outside the scope of this consultation, some respondents requested clarity on the future role of NICE. NHS England and NICE are in discussions about

aspects of the Highly Specialised Technologies Programme to create better alignment in future years.

5.2 NHS England may agree to fund interventions for rare conditions where there is limited published evidence on clinical effectiveness

26. Although there was significant support for the proposed application of the principle that treatments for rare conditions may be prioritised notwithstanding the limited evidence that may be available, several respondents expressed concern that the proposed method may disadvantage some interventions for rarer conditions.

27. For the 2016/17 commissioning round we will not adopt a separate process for prioritising treatments for rarer conditions:

- On the issue of cost and incremental benefit, it is right the process of relative prioritisation has a focus on benefit measured against cost, reflecting the key theme of financial sustainability in the [NHS Five Year Forward View](#). Some respondents alluded to the willingness of manufacturers to negotiate on price and we will look at this in the context of the commitment made in [NHS England's Business Plan for 2016/17](#) to enhance our commercial approach in specialised commissioning, particularly towards drugs and devices, to ensure we maximise what can be provided for the budget available
- We agree that it is appropriate for NHS England, as budget holder for specialised services, on receipt of CPAG's recommendations to consider the extent to which the overall budget impact of a proposed clinical commissioning policy may influence the relative prioritisation of a proposed intervention
- The process for 2016/17 includes an opportunity for proposals to be "elevated" to a higher prioritisation level based on a consideration of the four principles that assist NHS England in meeting its broader strategic objectives. One of these principles asks CPAG to consider whether the intervention would significantly offer the benefit of stimulating valuable innovation which, as respondents have observed, is perhaps more likely offered by treatments for rarer conditions.

28. Notwithstanding our approach for 2016/17 we will continue to engage with stakeholders on further work to ensure that treatments for rare conditions receive fair consideration, and we will review if and how a premium can be developed for rarer diseases. We agree with respondents who said that one of the biggest challenges with regards to developing treatments for rarer conditions is related to gathering adequate levels of data to support evidence requirements. Whilst we are clear that as a general principle commissioning decisions must be evidence-based, for the purpose of prioritising investment decisions in specialised services we will work with stakeholders to define with clarity a general principle that NHS England will look at each individual proposal to consider what would be the most appropriate level of evidence, being mindful of the need for the evidence to be proportionate to the patient group, and of the need for consistency with the approach of regulatory bodies so far as is this is possible. As the application of this principle would require a

consideration of each case, this may suggest that we should not be more prescriptive in defining a “rare” condition (as respondents have asked us to do) noting that the definition of “rare” is complex and differs in health systems.

29. Over 2016/17 we will continue to develop a strategic partnership between specialised commissioning and the National Institute for Health Research to help focus research funding to clinical priority areas.

5.3 NHS England will normally only accord priority to treatments or interventions where there is measurable benefit to patients

30. Whilst a focus on patient benefit was broadly welcomed by respondents, some detailed submissions were made with suggestions as to how the process may be refined in the future. As noted above, we will consider these ideas in detail, including through stakeholder engagement where appropriate.

5.4 The treatment or intervention should demonstrate value for money

31. There were some detailed comments about how the process could be refined in future years. We have described above the themes that were raised by respondents and the resulting further work that we will undertake, many of which also relate to the concept of “value for money”.

6 Question: Do you have any comment on how NHS England's Clinical Priorities Advisory Group should interpret and consider 'patient benefit', including the list of excluded factors?

32. As described above, in response to submissions made by respondents, we will explore for future years whether the range of metrics to describe patient benefits should be re-visited, including a consideration of societal factors and patient-reported outcome / experience measures.

7 Question: Do you agree that a proposed treatment or intervention may have a higher relative prioritisation if it meets one of the following principles?

33. The independent analysis of consultation responses concludes that *“there was considerable support for the four principles, providing the methodology used to consider the treatments against the principles is credible and transparent”*.

7.1 Does the treatment or intervention significantly benefit the wider health and care system?

34. Although there was broad support, some respondents queried whether treatments for rare conditions would be prejudiced on the ground that treatments for more common conditions have a greater potential to demonstrate benefits to the wider health and care system based on patient volume. In response to this concern we will clarify for CPAG that a consideration of the “significance” of a potential benefit should be relative to the size of the patient group and the cost of treating the condition.

7.2 Does the treatment or intervention significantly advance parity between mental and physical health?

35. While the advancement of parity was generally welcomed by respondents, questions were asked as to how this principle could be objectively measured and applied. We will review its application in 2016/17 for use in future years.

7.3 Does the treatment or intervention significantly offer the benefit of stimulating innovation?

36. There was broad support for this principle, but respondents offered a range of ideas about how it should be interpreted and applied. We do not agree with those respondents who have suggested that all proposals for new specialised treatments are, by their nature, “innovative”. For the purpose of the 2016/17 commissioning round we will clarify for CPAG that the term “innovation” refers to proposed treatments that would facilitate the emergence of high-impact new and ground-breaking areas of medicine. We will then review the interpretation and application of this principle for future years based on the consultation responses.

7.4 Does the treatment or intervention significantly reduce health inequalities?

37. There was support for the application of this principle, though many respondents asked for clarity on how health inequalities will be defined and measured, and they raised the potential impact of the proposed method to particular groups. These comments are addressed below.

8 Question: Would adoption of the proposed method unfairly discriminate against any group of protected characteristics?

38. Promoting equality and addressing health inequalities are at the heart of NHS England’s values. Throughout the development of the method we have given due regard to the need to eliminate discrimination, harassment and victimisation, to advance equality of opportunity, and to foster good relations between people who share a relevant protected characteristic (as cited under the Equality Act 2010) and

those who do not share it; and given regard to the need to reduce inequalities between patients in access to, and outcomes from healthcare services and to ensure services are provided in an integrated way where this might reduce health inequalities.

39. The consultation guide described NHS England's legal duties around the promotion of equality and the reduction of health inequalities. It considered the impact of the proposed method to specific groups with protected characteristics (people with rare conditions; people with mental health problems; children and young people; transgender people; and non-binary people). The impact assessment concluded that the proposed method would not unfairly discriminate against people with protected characteristics, and respondents to consultation were specifically asked for their views in this regard.

40. The independent analysis of consultation responses concludes that "a few more respondents" were concerned that the proposed method would discriminate against particular groups.

41. Many of the submissions referred to people with **rare conditions**. We have addressed this issue above.

42. Many respondents referred to interventions for **children and young people**, particularly **infants and children under five years**, given that the availability of evidence for treatment of children's diseases is generally not as good as for adult diseases. We will address the concern around the availability of evidence through the proposed work described above, which aims to describe a principle that the level of evidence required should be proportionate to the patient group. Some respondents also noted that there will also be instances where NICE has approved the use of a drug for adults but not for children, providing strong clinical evidence but only for the treatment of adults. In the context of licensed medicines, we plan to consult in 2016 on a proposed generic commissioning policy that will support the use of medicines licensed in adults for children if they meet the criteria described in the relevant NICE Technology Appraisal or NHS England clinical commissioning policy.

43. We will also consider whether in future patient benefit should be measured over a period longer than five years, in response to respondents who suggested that this is a short timescale in relation to treatments which are typically designed for younger cohorts of patients. We will also explore the argument that for a patient cohort which is largely children and not homogenous across age groups, the cost over the first five years may be higher than the costs over subsequent years.

44. Some consultation responses raised the issue of **people with mental health problems**, as the benefits of psychological therapies can sometimes be more difficult to measure. CPAG will not be considering any clinical commissioning propositions for psychological therapies in June 2016 but we will address this issue as we further test our approach for measuring benefit, and our definition of clinically reliable evidence for future years (as previously described).

45. There were no significant concerns expressed about the impact of the proposed method to **transgender people** and **non-binary people**. One respondent

organisation, which agreed with the proposed method, suggested that the process would discriminate against **gender variant children**, based on dissatisfaction with the specific process of developing a clinical commissioning proposition for the prescribing of cross sex hormones to young people. The relevant National Programme of Care will consider the respondent's concerns within the established process for considering the outcome of public consultation on specific policy propositions.

46. It was suggested during consultation that the overall approach to specialised commissioning decision making could disadvantage **people living with HIV**, based on a concern that as HIV treatments are not covered by NICE technology appraisals, nor is HIV the subject of any specific NHS Constitutional commitments, HIV treatments could never constitute a mandatory priority outside the process of relative prioritisation of discretionary investments, regardless of evidence of benefit or cost-effectiveness. The issue raised is outside the scope of the current consultation.

47. One respondent suggested that methods of assessing patient benefit for ophthalmic treatments may fail adequately to take into account vision loss. The list of patient benefit metrics that we have described includes mobility and "other health metrics determined by the independent evidence review", and we have described our further work to consider whether the range of metrics to describe patient benefits should be re-visited.

9 Question: Would adoption of the proposed method assist NHS England in promoting equality and in reducing health inequalities?

48. Overall, around a third of respondents concluded that the proposed method would not help to promote equality or reduce health inequalities (around 40% of respondents were unsure or did not answer the question). Some respondents explained that "this is not what it was designed to do". Other respondents were concerned about the impact on rarer conditions and/or treatments for children. As one of the principles deployed in the process of prioritisation is a consideration as to whether the proposed intervention would reduce health inequalities, we will have a good opportunity to review the learning from the 2016/17 commissioning round in this regard.

49. In summary however, with the caveats and further actions described above, the majority of the evidence submitted during consultation does not lead to the conclusion that the proposed method should not be adopted for use in 2016/17 based on a consideration of equality and inequality principles.

50. The assessment of the impact on promoting equality and reducing health inequalities will be revised and updated as the method is used and learning derived from it, and as NHS England further develops the method for use in future years.

10 Conclusion

51. NHS England is grateful for all of the detailed submissions made during this consultation. We will use the proposed method when CPAG meets in June 2016 for the purpose of forming recommendations for 2016/17, and we will review the learning from this process as we take forward the development of the method for use in future years, taking into account a consideration of the issues raised by respondents in this consultation. This is likely to involve further stakeholder engagement.

11 Appendix: CIPFA report on the feedback received during the public consultation

Developing a Method to Assist in Investment Decisions for Specialised Services

A Content Analysis of the Responses to NHS England

May 2016

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1. Introduction/Background

In early 2015, NHS England carried out a 90-day consultation on a set of principles and the characteristics of a potential process, to be used by its Clinical Priorities Advisory Group (CPAG) when forming recommendations regarding investment in specialised services.

In its response to consultation NHS England confirmed that it had adopted the principles, and it gave a commitment to further develop a detailed method or tool that could be used by the CPAG when it met to form recommendations for the 2016/17 year. As an outcome of this work NHS England launched a consultation exercise on 11 April 2016 on a proposed method. To encourage response it was publicised in a number of ways, including through the NHS England news page, bulletins/newsletters, Twitter and emails to stakeholders/groups. Alongside this, NHS England conducted a number of face-to-face consultation events and webinars.

NHS England has commissioned CIPFA to undertake a detailed content analysis of the 117 online and 5 offline responses.

2. Management Summary

- Responses to the consultation emanated from a broad range of respondents, including the public, and commercial and charitable sectors as well as those within the medical profession¹.
- Agreement with the proposed method by NHS England in relation to relative prioritisation was, overall, evenly split between those who agreed and those who disagreed. However, those respondents within the medical sector were more inclined to agree, whilst those from industry/commerce were more inclined to disagree.
- As to whether the proposed method offered transparency, would facilitate national consistent decision making and demonstrate an evaluation of cost effectiveness in decision making, we found that those within the medical profession broadly agreed and those outside disagreed.
- The majority of respondents agreed that the four principles² were being applied at the appropriate point.
- A number of respondents recommended the inclusion of societal benefit, as part of patient benefit, within the proposed methodology.
- The notion of according a higher relative prioritisation when the intervention significantly: benefits the wider health and care system; advances parity between mental and physical health; offers the benefit of stimulating innovation; and reduces health inequalities, was given considerable support, provided that the methodology used is credible, transparent and utilises innovation in order to deliver patient benefit.
- Some respondents were concerned about the adoption of the proposed method for those with rare or ultra-rare diseases.

¹ Overall a third of respondents declared a Sunshine provision/conflict of interest, as had 80% of Clinical Reference Group/Clinical Advisory Group respondents and 68% of Charity or voluntary sector respondents.

² **A:** NHS England will normally only accord priority to treatments or interventions where there is adequate and clinically reliable evidence to demonstrate clinical effectiveness; **B:** NHS England may agree to fund interventions for rare conditions where there is limited published evidence on clinical effectiveness; **C:** NHS England will normally only accord priority to treatments or interventions where there is measureable benefit to patients; **D:** the treatment or intervention should demonstrate value for money.

3. Respondent Information

Respondents to the consultation were requested to provide:

- their name
- the capacity in which they were responding
- the name of their organisation, if applicable
- their email address.

Information, such as their name and email address, was removed from the dataset to ensure anonymity. However, below we illustrate the responses to the question regarding the capacity in which they were responding.

In what capacity are you primarily responding?

Capacity (self-classified):	Percentage	Cases
Individual patient or member of the public	18%	22
Industry/commercial partner	18%	22
Charity or voluntary sector	16%	20
Individual clinician	15%	18
Royal college/medical representative committee	10%	12
Other health body	8%	10
Clinical reference group/clinical advisory group	5%	6
NHS commissioner	5%	6
NHS provider organisation	2%	3
Bodies with informed representation bodies	2%	3
Total	100%	122

Sorted in descending order by cases/percentage

The above table includes both online (117) and offline (5) responses.

To aid analysis or highlight differences within the responses, we have aggregated together a number of the above classifications as follows:

- **group A:** individual patients or members of the public; charity or voluntary sector; and bodies with informed representation bodies
- **group B:** individual clinicians; royal college/medical representative committee; and clinical reference group/clinical advisory group
- **group C:** industry/commercial partner

- **group D:** NHS regulator; NHS commissioner; NHS provider organisation; and other health body.

'Sunshine' provision/conflict of interest disclosures: have you or your organisation received any payments, grants or other funding from the pharmaceutical industry in the last three years?

Proportions, by capacity (group), declaring Sunshine provision/conflict of interest

Capacity [group]:	Percentage	Cases
Individual patient or member of the public [A]	14%	22
Charity or voluntary sector [A]	65%	20
Individual clinician [B]	39%	18
NHS regulator [D]	0%	0
Royal college/medical representative committee [B]	17%	12
NHS commissioner [D]	33%	6
NHS provider organisation [D]	33%	3
Industry/commercial partner [C]	18%	22
Other health body [D]	30%	10
Clinical reference group/clinical advisory group [B]	67%	6
Bodies with informed representation bodies [A]	33%	3
Total	33%	122

Aggregated proportions declaring Sunshine provision/conflict of interest

Capacity (group):	Percentage	Cases
Group A	38%	45
Group B	36%	36
Group C	18%	22
Group D	32%	19
Total	33%	122

Whilst the overall picture is that a minority of respondents (33%) declared a Sunshine provision/conflict of interest, for two specific subgroups the opposite was the case. For those who self-classified as responding in the capacity of a clinical reference group/clinical advisory group the figure was 80% and for the charity or voluntary sector it was 68%.

4. Content Analysis

The purpose of this analysis is to analyse key themes emerging from the consultation Developing a Method to Assist Investment Decisions in Specialised Commissioning. More specifically the topics on which respondents commented were:

- CPAG, process and qualifying principles
- the proposed method of prioritisation
- consideration of equality and health inequalities.

The following sections, 5.1 to 5.8 inclusive, illustrate the key themes by the order in which they arose. Where pertinent, quotes have been used to highlight the issues raised as well as suggestions for alternative methods. It should be noted that the identification of a key theme is reflective of when a significant number of respondents have highlighted a common issue, and the figures cited in each table are based on online replies.

The source of a quote is identified by the capacity in which they are primarily responding and, if pertinent, whether they had a "Sunshine provision/conflict of interest disclosure" to make. Within the appendices can be found a list of the organisations who responded, online or offline.

4.1 Question 6

NHS England has concluded that there is no existing method for relative prioritisation that could be directly applied to the process of prioritising proposed investments in specialised services, and has described in this document the process for developing the proposed method. Do you agree with the proposed method?

Response groups	Agree		Disagree	
	Count	%	Count	%
Group A	16	44%	20	56%
Group B	20	59%	14	41%
Group C	1	6%	16	94%
Group D	13	72%	5	28%
Totals	50	48%	55	52%

Response groups	Commented	
	Count	%
Group A	33	37%
Group B	27	30%
Group C	17	19%
Group D	13	14%
Totals	90	100%

Response groups

The table above demonstrates that a slightly higher percentage of respondents stated that they disagreed with the proposed methods compared to those who agreed. The table also shows the following:

- More respondents agreed than disagreed in the following response groups:
 - clinicians, royal colleges and clinical reference groups
 - NHS/other background
- More respondents disagreed than agreed in the following response groups:
 - public, charities, informed bodies
 - industry

In addition, 60% of those who agreed with the proposed method provided a comment, whereas 95% of those that disagreed provided a comment. Consequently, the comments are not necessarily representative of the overall response group. Many of those that agreed and provided a comment included caveats in their responses.

Overview

Positive statements concerning the proposed method included:

- the intent to establish a more pragmatic decision-making framework
- the desire to improve the consistency and reduce the subjectivity of the approach
- the acknowledgement that it should help provide evidence to support specialised commissioning of services
- the recognition of the need for a clear and transparent method for relative prioritisation of treatments
- the facilitation of discussions about value for money
- the decision to consult with stakeholders about the proposed method.

Rare/ultra-rare diseases

Several respondents were pleased with Section 29 of the consultation guide, which accounts for rarer diseases “where there is limited published evidence on clinical effectiveness”. However, it was suggested that rarer diseases are also likely to affect smaller numbers of patients compared to more common diseases, and it is possible that treatments for rarer diseases will have higher costs per patient.

It was also felt that the reduced evidence available for rare diseases, eg specialist surgical areas, might make it challenging to categorise submissions. Therefore, it was proposed that there should be some emphasis on the benefits to individual patients, so that treatments for rare diseases with high benefit will be given more parity with treatments for more common diseases. Some respondents sought more detail on how this parity would be provided. Additionally, some respondents asked for the definition of ‘rarity’ to be clarified.

Some respondents agreed that rarer diseases and their treatments, which concern small patient groups, should be fairly assessed under the proposed methodology, particularly where there are weaker evidence bases and higher initial costs per patient.

We believe this framework represents a unique opportunity to revisit how treatments for rare diseases are assessed for reimbursement on the NHS to establish a system which is more pragmatic and efficient, improves outcomes for patients and stimulates investment in new treatments. (Industry/commercial partner; Sunshine provision/conflict of interest disclosures: yes)

Products for rare diseases have less evidence available, which makes it very difficult to determine the difference between low, medium and high incremental benefit. (Industry/commercial partner; Sunshine provision/conflict of interest disclosures: yes)

One-size-fits-all solution

It was noted that it is challenging to find a suitable method for the diverse range of diseases and treatments. There was some concern about attempts to produce a one-size fits-all solution, due to the range of services covered by specialised commissioning. For example, it was suggested that there are considerable differences between the treatments used for common and rare diseases, and the impact of treatments on such diseases can also vary. It was argued that some treatments or techniques may take several years to demonstrate patient benefits, while others may vary in their level of benefit year on year.

There was also a desire to find out how incremental benefits will be categorised where the range of effects and treatments vary, eg how this will apply to treatments for children. It was hoped that submissions are not 'forced' into prioritisation categories, as the CPAG may be required to agree an equal number of proposals as either high, medium, or low benefit. Furthermore, it was suggested that prioritisation decisions might be made on the basis of competition as opposed to purely on merit, which could create a chance element.

As indicated there is no current correct 'one size fits all' approach. But the range of services covered by specialised commissioning is simply too great to even attempt this. The services range from treatments for very rare diseases to very common and widespread procedures. (Clinical reference group/clinical advisory group; Sunshine provision/conflict of interest disclosures: yes)

In some clinical areas such as spinal cord injury the numbers are small and changes may not be measurable in similar ways to other patient groups, however benefits can be achieved for this group, if delivered in a timely fashion and in an appropriate setting. (Individual clinician)

Non-clinical benefits

It was acknowledged that non-clinical benefits can be difficult to measure, however some respondents felt that these should be taken into consideration. There are also wider societal benefits/benefits for young people that could be considered under the proposed methodology, in order to help prevent discrimination against children and young people, eg:

- social care
- education attendance
- reduction in the needs for other treatments
- prevalence of diseases.

Assessment of benefit should be wider than just clinical effectiveness and look at social care/wider wellbeing gains. (Royal college/medical representative committee)

Research in young children is challenging, and the measurement of benefit by objective measurements is difficult, eg lung function, so I am pleased that you have allowed some extrapolation of the results from young people to younger children, but there will still be a risk that younger children could be excluded from receiving disease-modifying treatments, if high quality research evidence is always required. (Individual clinician)

Transparency and accountability

Some comments referred to the provision of more transparency and accountability to the decision-making process, including publication of decisions or adjustments made by the CPAG. There was also a desire for clarity around the methodology used to produce the summary report and how it will be applied. There were questions relating to appeals processes, resubmissions and independent reviews for any decisions made. In addition, transparency was sought about the quantification of patient benefits. Further suggestions relating to transparency included details of:

- the role of the Rare Disease Advisory Group, including how advice will be interpreted
- the credentials of organisations assessing the evidence
- the evaluation of technology and the range of evidence types to be used.

It is vital that all decisions made by CPAG are seen as legitimate, and as such must be fully understood by stakeholders. We therefore urge NHS England to publish the details of CPAG decisions, and to implement a process for challenging errors. (Charity or voluntary sector; Sunshine provision/conflict of interest disclosures: yes)

Further clarification

Several respondents sought clarification about a number of areas, which included the following:

- how CPAG will “be able to undertake equitable prioritisation of highly specialised services against more common specialised services” and “compare the relative incremental benefits to be categorised”
- whether there will be regional adaptations of specialist services based on local clinical needs
- the nature of financial impact assessments
- whether treatments will be classified in relation to each other rather than on individual assessments
- the different terminology to describe the categorisation of high, medium and low benefits.

Further comments

Some comments were outside the scope of the question, and included suggestions about how the proposed method could be adapted. These included:

- development of multi-criteria decision analysis
- utilisation of established methodologies, eg QALY and NICE’s highly specialised technologies programme (HST)
- weighting/quantifying the importance of the benefits to each patient

- providing specialised commissioning at the local/regional level.

4.2 Question 7

If you answered 'disagree' to the above question, please provide details of alternative method(s).

Several respondents took the opportunity to provide general comments on NHS issues. These comments will be passed on to NHS England but are not outlined in this report. Other respondents suggested alternative methods or amendments to the proposed methodology, of which several are highlighted below:

- Compare benefits and costs of new treatments with well-established, existing treatments from the same field.
- Use a broad range of evidence.
- Use quantitative methods to assess the metrics (cost and benefits) as opposed to 'value judgments' in order to improve transparency, eg:
 - multi-criteria decision analysis with defined weighting and scoring systems
 - a points-based system.
- Utilise NICE and/or their methodologies, including use of QALY, HST evaluation and medical technologies guidance.
- Use subcommittees/reference groups to evaluate new procedures, monitor best practice and/or assess the evidence used for decision making.
- Use of 'public scoping workshops' to include clinicians and members of the public.
- Use historical models, eg methods used in other countries.
- Incorporate procedures for reviews and decision appeals.
- Provide greater transparency around CPAG decision making.

Respondents also suggested that further clarity should be included in the methodology, including on:

- how the "incremental cost" of each proposal will be calculated
- the timescale during which the measured benefits will occur, noting that some treatments take several years to demonstrate health benefits.

4.3 Question 8

Do you agree that the method proposed by NHS England:

- A. is transparent**
- B. will facilitate national and consistent decision making**
- C. has, at its foundation, the core principles of demonstrating an evaluation of cost effectiveness in the decision making?**

The method proposed by NHS England:	Agree % [cases]	Disagree % [cases]
A. is transparent	50% [55]	50% [56]
B. will facilitate rational and consistent decision making	37% [41]	63% [69]
C. has, at its foundation, the core principles of demonstrating an evaluation of cost effectiveness in the decision making	66% [71]	34% [36]
Total	51% [167]	49% [161]

Proportionally we find that the levels of agreement and disagreement more or less match, ie 51% agree whilst 49% disagree. From a statistical perspective the difference of 2% falls within the margin of error for a survey of this size, estimated at $\pm 5\%$, hence this conclusion.

We then cross-tabulated responses to the 'conflicts of interest' and 'capacity in which responding' questions against the overall levels of agreement for the above three statements. In respect of the provision/conflict of interest responses, we found that of those who said 'yes', some 45% were in agreement overall, whilst of those who said 'no', the level of agreement increased to 55%.

In regards to the capacity in which they responded to the consultation, the levels of agreement varied as follows:

- group B: 67%
- group D: 62%
- group A: 48%
- group C: 16%.

The method proposed by NHS England:	Level of agreement (%)			
	Group A	Group B	Group C	Group D
A. is transparent	45%	74%	0%	63%
B. will facilitate rational and consistent decision making	40%	45%	0%	50%
C. has, at its foundation, the core principles of demonstrating an evaluation of cost effectiveness in the decision making	59%	81%	50%	72%

As can be seen in the table above, levels of agreement with statement A vary from as low as 0% (group C) to as high as 74% (group B). For statement B the level of variation is from 0% (group C) to a maximum of 50% (group D). Whilst for statement C the range is from 50% (group C) to 81% (group B). This would appear to suggest a clear split in opinion between medical professionals (groups B and D) and industry (group C), with medical professionals seen to be far more supportive.

Statement A

In response to the question on transparency we find that where agreement has been expressed, respondents have generally gone on to caveat their support. A number of respondents provided ideas on how to ensure transparency:

Publication of the prioritisation method will increase the transparency of the process. To further increase this transparency, [the respondent] strongly recommends that NHS England make public not only the documents which have been presented to CPAG to inform their relative prioritisation but also those which CPAG go on to submit to the Specialised Commissioning Committee: including both their recommendations and any qualitative commentary on, for example, any adjustments made to baseline recommendations.

A truly transparent approach to specialised commissioning also requires significant improvement in public communication from NHS England. At a minimum, the specialised commissioning sections of the NHS England website need to be kept up to date with contact details of specialised commissioning hubs, responsible commissioners, service specialists, CRG members, working group members, papers which have been made public to registered stakeholders and dates of key meetings, as well as all CRG products. (Charity or voluntary sector; Sunshine provision/conflict of interest disclosures: yes)

Interestingly, where disagreement was expressed we find that respondents are more vocal in their opinions. There were a number of points around which feedback converged:

- Rare and ultra-rare conditions:

We are also concerned that the second principle indicates that NHS England may agree to fund interventions for rare conditions whereas if the methodology is followed as described and there are a large number of proposals in any year, it is unlikely that treatments for rare conditions would receive sufficient priority to be funded under this method. (NHS provider organisation)

- The '5 year time horizon':

The 5 year time horizon may be appropriate for some interventions but others would normally take a life time horizon approach, which would not make such costs comparable. (Industry/commercial partner)

- The necessity to publish:

As with the system adopted by NICE, [the respondent] believes that NHS England should allow members of the public to attend meetings of the Clinical Priorities Advisory Group and to have the opportunity to contribute to the meeting and to the wider prioritisation process. (Charity or voluntary sector; Sunshine provision/conflict of interest disclosures: yes)

- Application of the low, medium and high categories:

There are no clear criteria for categorising interventions into high, medium and low in terms of benefits and costs [...] It is unclear how equal numbers of treatments will be assigned into each category or, importantly, why. (Other health body)

Statement B

Overall the level of agreement with statement B ("...will facilitate rational and consistent decision making") was markedly lower than that for statement A ("...is transparent") and statement C ("...has, at its foundation, the core principles of demonstrating an evaluation of cost effectiveness in the decision making").

We find that generally the same concerns were raised in respect of consistent decision making as were in regard to transparency, ie regarding rare and ultra-rare conditions, and the application of low, medium and high categories. For example:

For the majority of proposals we feel there will be consistent decision making; however, we are concerned about the impact of the method on those proposals for patients with rare conditions, as it's still not entirely clear how they will not be disadvantaged (given the costs of treatment are likely to be high, and the patient population affected will be low). (NHS commissioner)

'Forced' Categorisation – Paragraph 24 of the document makes it clear that CPAG will be required to agree an equal number of proposals as either high/medium/low benefit so that there is a relative prioritisation process. However, this does pose a risk that in a year when a large number of very 'strong' proposals are put forward, some proposals may be ranked as lower

than they would have been in a year with a relatively 'weaker' set of proposals. (Industry/commercial partner)

A number of recommendations were made to address such concerns, although it should be noted that most of these were isolated:

Whilst we understand the rationale for a ranking system, it makes no sense to force equal numbers of interventions into categories, even when those categories are inappropriate. It would make much more sense to either have no quota for each category, or to have a scale from higher to lower relative benefit, split into top, middle and lower thirds [...]. (Charity or voluntary sector; Sunshine provision/conflict of interest disclosures: yes)

Given there are two processes (NICE led for medicines and CRG led for all other interventions and treatments) to develop the policy proposals for 2016/17 leading into the prioritisation process there is potential for inconsistency of input. As such, NHS England must ensure there is a robust feedback and oversight mechanism put in place to evaluate the consistency across the two processes [...]. (Industry/commercial partner)

Statement C

Statement C ("Has, at its foundation, the core principles of demonstrating an evaluation of cost effectiveness in the decision making") obtains, at the headline level, the most significant levels of agreement (66%). Across the capacity of response groups we find this varies from a relative low of 50% (group C – industry) to a high of 81% (group B – clinicians, royal colleges, CRGs). However, even amongst this heightened level of agreement what stood out was the number of detailed caveats cited. A proposed remedy, identically shared by two respondents, was:

It appears that NHS England has chosen the cost per patient benefit as a tool for cost effectiveness. However, cost benefit analysis needs to have clear defined thresholds on which NHS England has failed to provide clarity. As suggested previously, a simple MCDA approach with defined thresholds and weightings as well as burden/severity/impact of the disease [...] taken into account in a transparent manner would be a more suitable alternative approach to enable relative prioritisation across specialised conditions. (Industry/commercial partners)

Alongside this alternative view:

You should compare the proposed investments to a ranked table of existing similar investments – perhaps over last 10 or 20 years – comparing them all first by cost, then by agreed/likely benefits [...]. (Individual patient or member of the public)

4.4 Question 9

Please comment on whether the following four principles are applied at the appropriate point in the proposed method of relative prioritisation:

- A. NHS England will normally only accord priority to treatments or interventions where there is adequate and clinically reliable evidence to demonstrate clinical effectiveness.**
- B. NHS England may agree to fund interventions for rare conditions where there is limited published evidence on clinical effectiveness.**
- C. NHS England will normally only accord priority to treatments or interventions where there is measureable benefit to patients.**
- D. The treatment or intervention should demonstrate value for money.**

Principle A

In respect of principle A ("NHS England will normally only accord priority to treatments or interventions where there is adequate and clinically reliable evidence to demonstrate clinical effectiveness"), we found that some six in ten respondents agreed with this principle. Although, of this group, we found that slightly more than four in ten (43%) applied a caveat. Typical supportive comments included:

I agree with this method. (NHS commissioner)

I agree with the four principles but [...]. (Bodies with informed representation bodies)

The four principles are patient centred and therefore appropriate. (Individual clinician)

We agree with the above statements. (Industry/commercial partner)

We found that, regardless of whether the comment is one of qualified support or not, the following points were voiced most frequently:

- clarification on adequacy/sufficiency of clinically reliable evidence
- the appropriateness of the method in the case of rare and ultra-rare diseases.

A number of observations/proposals were made, a selection of which are presented here:

Several interventions in children do not yet have adequate and clinically reliable evidence due to various ethical challenges in doing research with children and also due to methodological challenges. For example, there are 27 PICUs [Psychiatric Intensive Care Units] in England, Wales and NI, compared to 205 adult ICUs. There are only 16,000 admissions to PICU whereas there are 131,000 admissions to adult ICUs. This is an example of challenges when doing research with children. Often multicentre, multicountry trials are required to achieve sufficient sample size in paediatric research. Whereas this is less often the case in adults. (Individual clinician)

The proposed process briefly acknowledges that gathering trial evidence that will score sufficiently high on the GRADE standards to reach medium/high weighting is difficult in children. This is due to a variety of challenges, including availability of research funding and ethical constraints. The guidance assumes that where there is an absence of evidence in children, an

appropriate adult proxy will be available. There are many conditions and circumstances, beyond rare diseases, where a paediatric drug/intervention or service will not have an adult proxy. These will be adversely affected by the global imposition of the GRADE standards. We propose that a separate evidence weighting score may be required in the consideration of childhood evidence, perhaps using a relative scale that acknowledges what the ceiling of research might be for a particular condition. [...] The guidance also does not recognise the risk of convenience and ascertainment biases, where certain conditions are more likely to have research funded, or present easier opportunities for research evaluation to be undertaken (for example, short term surgical outcomes versus long term quality of life outcomes). This introduces systematic bias towards certain disease groups and intervention types. (Individual clinician)

There does need to be evidence. For very rare conditions and interventions, and for some more common developmental disorders, this evidence may be at case report level, rather than higher quality evidence levels. This is because research on clinical intervention in disabled children with multiple co-morbidities is methodologically very difficult, due to multiple factors such as: definition of population (disabled children even with the same diagnosis, eg cerebral palsy, may have significant variation in presentation/needs due to severity and co-morbidities), interventions (these can be multiple, variable and with significant confounders) and outcome measures (limited validated outcome measures for many interventions due to confounders of child development; timing of outcome and potential) [...]. (Industry/commercial partner)

Principle B

The level of support for principle B ("NHS England may agree to fund interventions for rare conditions where there is limited published evidence on clinical effectiveness") was at a similar level to that for principle A, ie six in ten provided supportive statements, although in this case only three in ten statements carried any sort of caveat. Nonetheless, whether supportive or not, the most frequently cited concern was in respect of a definition or clarification of 'rarity':

Furthermore, NHS England needs to provide a robust definition of what constitutes rarity. (Other health body; Sunshine provision/conflict of interest disclosures: yes)

However, much greater clarity is required as to how these principles are going to be used and to what extent these modifiers could change the initial prioritisation level assigned by CPAG. For example NHS England must set out in more detail what constitutes clinically and reliable evidence. (Industry/commercial partner)

A number of suggestions were made that may help mitigate against the challenges presented:

NHS England should publish clear guidelines on what constitutes reliable evidence and how this will be assessed. It must ensure that its criteria do not disadvantage certain types of treatment, for example by acknowledging the different forms of evidence commonly used for drugs and devices. It is positive that NHS England recognises the more limited availability of evidence for certain interventions for rarer conditions. [...]. (Charity or voluntary sector; Sunshine provision/conflict of interest disclosures: yes)

We note the importance of principle B and believe that the use of Managed Access Agreements should be strongly considered at the earliest opportunity by NHS England. (Charity or voluntary sector; Sunshine provision/conflict of interest disclosures: yes)

There should be further clarification of the definition of rare disease, currently this is defined from a pharmaceutical perspective. (Industry/Commercial partner)

Principle C

In the case of principle C ("NHS England will normally only accord priority to treatments or interventions where there is measureable benefit to patients"), we find that the level of support is at or close to seven in ten respondents, with only three in ten providing any form of caveat. In the few cases where concern was expressed, whether from those who were supportive or not, the principle theme was in respect of weighting or calibration. The following quotes are from respondents who were supportive of principle C:

The consultation document is not sufficiently clear in terms of how CPAG will calibrate and standardise its assessment of benefits, particularly between high numbers of patients gaining a small benefit, and small numbers of patients gaining large benefit. (NHS provider organisation)

We welcome the intention to prioritise interventions where there is measurable patient benefit. However, [...] it is important that the types of measurable benefit – and how these will be measured – are agreed and further defined. (Industry/commercial partner)

Principle D

For principle D ("The treatment or intervention should demonstrate value for money"), we found that close to six in ten respondents agreed that the treatment or intervention should achieve value for money (VfM). But in a number of cases, respondents queried how it could be determined, assessed, defined or detailed. A handful of respondents expressed concerns around the short time-frame of five years within which to demonstrate VfM.

Potential remedies, in respect of clarification and the time-frame issue, included:

In relation to principle D, we would urge NHS England to consider the potential for savings accrued over a longer period of time, rather than in immediate financial years. (Charity or voluntary sector; Sunshine provision/conflict of interest disclosures: yes)

In agreement with the ABPI position, we recommend NHS England to view the 2016 process as interim and to continue to develop the methodology – both of cost and patient benefit – in conjunction with academic and industry experts during the rest of 2016 with a feedback mechanism in place to further evolve the process. This will ensure a more robust, cost effectiveness based approach can be put in place for the 2017 decision making process. (Industry/commercial partner; Sunshine provision/conflict of interest disclosures: yes)

4.5 Question 10

Do you have any comment on how NHS England's Clinical Priorities Advisory Group should interpret and consider 'patient benefit', including the list of excluded factors?

Feedback on this question varied. However, there were some common factors around which respondents concurred:

- use of patient-reported outcome/experience measures (PROM/PREM)
- use of quality-adjusted life year (QALY/QUALY), including EuroQol EQ-5D
- inclusion of societal benefit (within patient benefit), as per NHS England's Five Year Forward View (5YFV)
- inclusion of health and wellbeing.

Illustrative of these issues are the following extracts:

The patient benefit impact of disease/illness should be measured using PROMS or PREMS where possible, such as the National Paediatric Diabetes Audit PREM. (Royal college/medical representative committee)

The QUALY (quality-adjusted life year) concept needs to be further extended [...] The economic, societal and individual benefits should be included actively in the process. (Royal college/medical representative committee)

[...] However, currently not included is societal benefit, potential financial savings and the prevalence of the underlying condition/illness. The [respondent] strongly believes that medical technology can bring huge societal benefits which will help alleviate pressure on the NHS, improve the benefit to the patient and reduce the strain on the economy of the country as a whole. The [respondent] would recommend that societal benefits are included within the considerations around 'patient benefit'. (Industry/commercial partner)

A number of suggestions were made, reflective of the concerns expressed above:

The clinical priorities should not be constrained by the list of metrics. There should be consideration of cognitive and wellbeing factors especially for children in their life course, eg improved cognitive outcomes, educational attainment or educational resilience. (Royal college/medical representative committee)

In the absence of an algorithm that clearly defines the weighting of value given to differing health dimensions (as found in standardised instruments for measuring generic health status, such as the EQ-5D) it is important that the CPAG are well informed in terms of how the general population is likely to rank patient benefits. This analysis and subsequent training would reduce the risk that the CPAG may be led by their own value judgements on patient benefit when aiming to decipher which position a certain technology should take in their prioritisation matrix. (Industry/commercial partner)

4.6 Question 11

For each of the following principles, do you agree that a proposed treatment or intervention may have a higher relative prioritisation if the principle is met?

- A. The treatment or intervention significantly benefits the wider health and care system.**
- B. The treatment or intervention significantly advances parity between mental and physical health.**
- C. The treatment or intervention significantly offers the benefit of stimulating innovation.**
- D. The treatment or intervention significantly reduces health inequalities.**

Overview

There was considerable support for the four principles, providing the methodology used to consider treatments against the principles is credible and transparent.

We support the adoption of the above principles which we believe should be accorded equal priority. We welcome the inclusion and recognition of innovation's role in seeking to achieve health system goals. We would like to highlight the need for greater national leadership to drive implementation and adoption in a consistent way so as to afford the wider benefits that new technology can bring. (Industry/commercial partner)

[The organisation] is broadly supportive of the prioritisation adjustments outlined as part of NHS England's proposals. However, NHS England will need to demonstrate that these adjustments have been applied meaningfully, rather than as an afterthought. If eventual prioritisation decisions simply map to clinical vs cost effectiveness, there will be doubts over whether these further principles have been applied. (Other health body; Sunshine provision/conflict of interest disclosures: yes)

Principle A

In reference to principle A ("The treatment or intervention significantly benefits the wider health and care system"), there was a desire for more clarity about how benefits to the wider health and care system will be measured, how the criteria will be applied and what the relative weightings will be. It was noted that prevention is a significant benefit to the wider health and care system, but this has been excluded from the criteria.

[Principle A:] Yes, although for a rare disease any benefit will always be small because of the small number of patients involved. (Clinical reference group/clinical advisory group; Sunshine provision/conflict of interest disclosures: yes)

Principle B

In reference to principle B ("The treatment or intervention significantly advances parity between mental and physical health"), while advancing parity was generally welcomed, there was a desire for equality of outcomes for all groups. It was hoped that the principle does not create competition between treatments for mental and physical health, and

that decisions for higher relative prioritisation are based on merit and not solely on the desire for parity.

[Principle B:] Mental and physical health should be considered equally and the methodology should ensure both are considered on an equitable basis; it should not be about advancing one over the other. (NHS commissioner; Sunshine provision/conflict of interest disclosures: yes)

[Principle B:] No, it could equally be argued that interventions should be neutral to all affected groups (gender, geography, wealth, disability, deprivation etc.). (Individual patient or member of the public)

Principle C

In reference to principle C ("The treatment or intervention significantly offers the benefit of stimulating innovation"), it was agreed that NHS England should encourage/support innovative treatments and research. It was suggested that innovation should only be encouraged if it will help to improve health and patient benefits. It was hoped that principle C will be used to prevent discrimination against treatments for rare diseases that have high costs per patient. Furthermore, clarification was sought as to how "innovation" is defined and how "stimulating innovation" can be measured.

We would support adoption of the above principles. Innovation is critical to achieving many of the health system's goals and needs to be encouraged both specifically and culturally. Innovations in medical devices rely on and support changes to healthcare service delivery processes. These changes can have a wider benefit outside of a delivery of a single service or intervention. A definition of innovation would help transparency of the process and we would recommend that the form proposed within Innovation Health and Wealth is adopted. (Industry/commercial partner)

[The organisation] agrees with NHS England's inclusion of innovation principle, as specialised commissioning is an important route for innovation to enter the NHS. Specialised services often operate at the forefront of medical science. As such, prioritising treatments with an innovative potential can deliver benefits over time to the wider health system. (Other health body; Sunshine provision/conflict of interest disclosures: yes)

Principle D

In reference to principle D ("The treatment or intervention significantly reduces health inequalities"), there was a desire for more clarification on how "health inequalities" will be defined and measured.

[...] The inclusion of the innovation principle as specialised commissioning is an important route for innovation to enter the NHS. Specialised services often operate at the forefront of medical science. As such, prioritising treatments with an innovative potential can deliver benefits over time to the wider health system. Furthermore, the outcomes from research into rare diseases may offer insights into treatments for more common disease thus benefiting the wider health and care system. This is another reason for considering a higher relative position for these treatments and policy proposals. (Charity or voluntary sector; Sunshine provision/conflict of interest disclosures: yes)

4.7 Question 12

Would adoption of the proposed method unfairly discriminate against any group of protected characteristics?

Generally respondents did not think that the proposed method will unfairly discriminate against any group of protected characteristics.

One third of individuals from group A was not sure about the implications of the proposed method on any group and would need more information before judging whether the method would unfairly discriminate against a certain group. One third believed that the proposed method will not unfairly discriminate against any group, whereas the final third was concerned that the method would unfairly discriminate against certain patient groups, with patients with rare/ultra-rare diseases most likely to be affected:

Only a small number of participants in group B were not sure about the effect of the proposed method on certain groups. However, slightly more participants in group B didn't think that the method will unfairly discriminate against any group or protected characteristics. Those who believed that the method could discriminate against any group mention rare diseases as well as children.

Groups C and D on the other hand showed that the majority of participants were concerned about the proposed method for rare/ultra-rare diseases. Compared to group B, only a small number of participants in groups C and D mentioned small children as an affected group.

4.8 Question 13

Would adoption of the proposed method assist NHS England in promoting equality and reducing health inequalities?

	Agree	Disagree	Unsure	Unanswered	Cases
Group A	22%	27%	24%	27%	45
Group B	28%	31%	11%	31%	36
Group C	23%	36%	14%	27%	22
Group D	32%	32%	21%	16%	19
Total	25%	30%	18%	26%	122

The overall response to question 13 indicates that participants are uncertain whether the proposed method will assist NHS England in promoting equality and reducing health inequalities. Respondents who expressed doubts regarding the goals for the proposed method were more likely to show concern for either patients with rare/ultra-rare diseases or children.

Overall we feel this method will promote equality and reduce health inequalities, but some clarity is required regarding what is meant by 'adequate' evidence and how judgement is applied. A rationale for decision-making would ensure the method is truly transparent. (NHS Commissioner)

Responses from group A are almost evenly divided, with close to quarter claiming that the proposed method will assist NHS England in promoting equality and in reducing health inequalities. However, respondents stated that this will only be achieved if certain conditions are met. Unfortunately no common theme occurred for those conditions. The other half believed that the proposed method will not assist in promoting equality and reducing health inequalities.

For group B, among those that answered definitively, there is an almost even split apparent between participants who believed that the method will assist NHS England in promoting equality and reducing health inequalities and those who expressed doubts. A small number of those who disagreed with the method's proposed aims suggested that the method may have the opposite effect.

Similar to group B, among those that answered definitively, an even split in group D can be observed, whereas in group C there were more respondents disagreeing than agreeing.

5. Appendices

The list below is presented as entered by the respondent, ie not all respondents were necessarily responding officially on behalf of an organisation.

Charity or voluntary sector

Anthony Nolan
Breast Cancer Now
British Kidney Patient Association
British Liver Trust
Gender Identity Research and Education Society
Genetic Alliance UK
Healthwatch
Leber's Hereditary Optic Neuropathy Society
MPGN/DDD Support Group
MPGN/DDD Support Group
Muscular Dystrophy UK
National AIDS Trust (NAT)
Neurological Alliance
Niemann-Pick UK
Prostate Cancer UK
Royal College of Surgeons
Society for Mucopolysaccharide Diseases
Teenage Cancer Trust
The British Pain Society
UK Gauchers Association Ltd

Individual clinician

Birmingham Children's Hospital
Birmingham Children's Hospital
British Association of Plastic, Reconstructive and Aesthetic Surgeons (BAPRAS)
Central Manchester Healthcare NHST
Great North Children's Hospital
Great Ormond Street Hospital
Honorary Contract with Central Manchester Foundation Trust
Leeds Teaching Hospitals NHS Trust
London Spinal Cord Injury Centre
NW Coast Genomic Medicine Centre
Queen Elizabeth Hospital Birmingham
Queen Elizabeth Hospital UHB NHS Foundation Trust Birmingham UK
Queen Victoria Hospital
Royal Wolverhampton Hospitals NHS Trust
Small Heath Practice

Royal college/medical representative committee

Association of Paediatric Anaesthetists of Great Britain and Ireland (APAGBI)
British Association of Plastic Reconstructive and Aesthetic Surgeons
British Nuclear Medicine Society
British Orthopaedic Association
British Paediatric Neurology Association
British Society for Rheumatology
Central and North West London NHS Foundation Trust (CNWL)
Faculty of Pain Medicine
Royal College of Nursing
Royal College of Paediatrics and Child Health

Royal College of Psychiatrists
UK Neurointerventional Group

NHS commissioner

East and North Hertfordshire CCG
Leeds West CCG
NHS Blood and Transplant
NHS East Riding of Yorkshire CCG
NHS Sheffield CCG
St Helens CCG

NHS provider organisation

Birmingham Children's Hospital
Great Ormond Street Hospital for Children NHS Foundation Trust
Shelford Group

Industry/commercial partner

AbbVie
Aegerion Pharmaceuticals Limited
Association of British Healthcare Industries (ABHI)
Association of the British Pharmaceutical Industry (ABPI)
AstraZeneca UK Ltd
Baxalta
Cook Medical
Edwards Lifesciences
Ethical Medicines Industry Group (EMIG)
European Medicines Group (EMG)
Gilead Sciences Ltd
Intuitive Surgical Sarl
Janssen
MAP BioPharma Limited
Medtronic
Novartis Pharmaceuticals
Pfizer Ltd
PTC Therapeutics, Limited
Sanofi
Shire Pharmaceuticals Limited
Sykes Anaesthetics Ltd
The Medical Technology Group

Other health body

British Academy of Childhood Disability
British HIV Association (BHIVA)
Cell and Gene Therapy Catapult
Cochrane UK
National Institute for Health and Care Excellence (NICE)
Neonatal and Paediatric Pharmacists Group (NPPG)
Regional Genetics Lab, Nottingham
Specialised Healthcare Alliance (SHCA)
UK Genetic Testing Network

Clinical reference group/clinical advisory group

Blood and Marrow Transplant Clinical Reference Group (BMT CRG)
King's College Hospital NHS Foundation Trust

Metabolic Disorders CRG
NHS-E PET-CT CRG
Royal Bournemouth Hospital
Specialised Urology CRG

Bodies with informed representation bodies

APPG for Sickle Cell and Thalassaemia
Institute of Physics and Engineering in Medicine (IPEM)
University of Manchester

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