Genetic Alliance UK
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Developing a method to assist investment decisions in specialised commissioning: next steps

NHS England consultation on a prioritisation process

Response by Genetic Alliance UK, 11 May 2016

Introduction

1. Genetic Alliance UK is the national charity working to improve the lives of patients and families affected by all types of genetic conditions. We are an alliance of over 180 patient organisations. Our aim is to ensure that high quality services, information and support are provided to all who need them. We actively support research and innovation across the field of genetic medicine.

2. In October 2014, Genetic Alliance UK published a Patient Charter that made a series of practical recommendations for improving how NHS England evaluates and commissions specialised treatments. This included proposals to improve transparency, streamline the evaluation and commissioning processes, improve patient engagement and provide additional support to Clinical Reference Groups. The Charter was directly informed by patient workshops and was subsequently endorsed by 88 patient groups (Patient perspectives and priorities on NHS England's commissioning of medicines for rare diseases. Genetic Alliance UK, published October 2014, available at: www.geneticalliance.org.uk/docs/nhsenglandpatientcharter.pdf).

3. Rare Disease UK (RDUK) is a multi-stakeholder campaign run by Genetic Alliance UK, working towards the delivery and implementation of the *UK Strategy for Rare Diseases*[1], signed by all four health departments in the UK and published by the Department of Health in November 2013.

4. As NHS England needs to evaluate those rare diseases medicines that NICE does not select for one of its appraisal routes, it is by default the primary appraisal body for rare disease medicines. For this reason it is of vital importance to patients with genetic and rare conditions that the process for prioritising commissioning policies for the limited funding is transparent and fair.

Q1. 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. Do you agree / disagree / don't know

5. While we cannot speak to whether there are existing methods for relative prioritisation that could be directly applied to the process of prioritising proposed investments in specialised services, this cost vs. effectiveness matrix seems an appropriate method. Our concerns relate to the lack of clarity and accountability on how decisions are made on where on the matrix to position each policy, how those terms are defined and the application of the principles, rather than the functioning of the matrix itself.

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[1] UK Strategy for Rare Diseases. Department of Health, published November 2013, available at: www.gov.uk/government/uploads/system/uploads/attachment_data/file/260562/UK_Strategy_for_Rare_Diseases.pdf

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Q1b. If you disagree, please provide details of alternative method(s):

6. We have no comment to make on this question.

Q2. Do you agree that the method proposed by NHS England:

2a. is transparent;

7. No. The full pathway for decision making is not transparent. We are very concerned that some of the most important decision-making stages are neither predictable nor accountable.

8. In general, the earlier stages of the process are reasonably clear. The functioning of the process and qualifying principles, and what happens to a proposed policy that does not meet these principles, is fairly straight forward. However, we do question to what extent NHS England examines those treatments or interventions which are already routinely commissioned, in order to determine whether a new policy offers equal or greater benefit. We are aware that many legacy commissioning policies have never been properly evaluated for clinical and cost effectiveness, due to differing processes in place at the time of initial commissioning. Decommissioning is outside the scope of the current consultation, however, and the principle of prioritising treatments offering greater benefits is a valid one, as long as it is applied fairly across the board and with an understanding of the challenges of demonstrating benefit in rare diseases.

9. The process described in paragraphs 24 to 26 of the consultation guide is far from transparent. Though a number of aspects are listed which might be considered to constitute patient benefit, no explanation or guidance about how the Clinical Priorities Advisory Group (CPAG) will weigh these metrics to come to a spread across the categories of prioritisation has been offered. From the information provided there is very little indication of how the CPAG will reach their decisions on which policies will fall into each category, other than that it will be through debate.

10. This lack of transparency is exacerbated by the reluctance of senior NHS England staff to reassure stakeholders that detailed minutes of CPAG's deliberations would be published. We welcome the fact that the proposed process includes a narrative provided by CPAG to NHS England explaining the reasoning behind any proposed adjustment to baseline rankings. However, in order to meet the minimum standards of process principle one (transparency of process) not only must this be made publicly available, not only to other decision-makers further along in the process, but this must not be the only part of CPAG's discussion to receive this illumination.

11. Without detailed, publicly available narrative minutes, it will be impossible for stakeholders to tease out the precise reasons why each policy was given its relative position compared to each competing policy. While we understand that the processes are being proposed for 2015/2016 only, and will not necessarily be reused in subsequent years, in order for NHS England to learn from the experience external stakeholders must be able to understand how these decisions are being made and hold decision makers to account.

12. There is also a lack of transparency at the other key decision-making stages: the Specialised Commissioning Oversight Group and the Specialised Commissioning Committee. It is not clear based on the very limited information available to what extent the recommendations made by CPAG will be reconsidered by each of these bodies, and on what grounds they may be able to overrule those recommendations.

2b. will facilitate rational and consistent decision-making

13. No, for the reasons listed above, the lack of any clear guidelines to be given to CPAG on how they will make judgments about the relative levels of patient benefit leave us in great doubt as to whether those decisions will be made in a rational and consistent manner.

2c. has, at its foundation, the core principles of demonstrating an evaluation of cost effectiveness in the decision making.

14. Patients affected by genetic or rare conditions understand that there is a need to prioritise investment into specialised treatments and interventions on the grounds of clinical and cost

effectiveness, and that this should take place regardless of any external financial pressures. What is essential, however, is that the process for doing so is both transparent and fair, and takes account of the patient, public and governmental support that exists for ensuring that patients affected by rare conditions are able to access effective treatments.

15. Unfortunately, the proposed interpretation of cost and benefit is so pure and narrow, that this system will not allow any acknowledgement of either the financial factors that affect the pricing of treatments for rare diseases, or the societal consensus that treatments for rare diseases should be accessible through the NHS. This approach is divergent from all modern systems that have been designed to make decisions on access to treatments for populations affected by rare diseases in the UK, all of which make arrangements to account for the characteristics of rare diseases treatments.

16. Though there is the principle mentioned below, that "interventions for rare conditions where there is limited published evidence on clinical effectiveness", the system has been designed in such a way that the threshold for evidence is not the limiting factor for the rare disease population.

17. Rather, the arbitrary message that any treatment that is judged to be of high cost, cannot be cost-effective, whatever its health benefit, will disproportionately affect treatments for rare diseases. In the context of difficulties with evidence generation, a system that will not effectively gather evidence across the breadth of potential benefits gained from treatments for rare diseases, and budgetary constraints, this approach to decision making regarding high cost medicines is effectively a decision not to commission high cost treatments however cost effective they are.

18. We were surprised to read that NHS England believe that a "small number of proposals are likely to relate to treatments for rare conditions". Specialised commissioning is the main route in which treatments for rare conditions are commissioned. There are certainly procedures and treatments that are not for those with rare diseases, but proportionately speaking, it seems strange that the number of proposals that relate to rare conditions (defined by the EU as affecting fewer than 5 in 10,000 – more common than conditions within the scope of the Rare Disease Advisory Group) would be small.

Q3. Please comment on whether the following four principles are applied at the appropriate point in the proposed method of relative prioritisation:

3a. NHS England will normally only accord priority to treatments or interventions where there is adequate and clinically reliable evidence to demonstrate clinical effectiveness

3b. NHS England may agree to fund interventions for rare conditions where there is limited published evidence on clinical effectiveness

3c. NHS England will normally only accord priority to treatments or interventions where there is measureable benefit to patients

3d. The treatment or intervention should demonstrate value for money.

19. The prioritisation principles essentially propose the positioning of each policy proposal on a matrix of cost vs. benefit. As such, it is difficult to see why this question of measurability of benefit is being brought up again, given that all policy proposals which have reached this stage will have already passed the stages of process principles and qualifying principles, which address the demonstration of benefit.

20. The terminology "adequate and clinically reliable evidence to demonstrate clinical effectiveness" and "measureable benefit to patients" presents a potential barrier to patients with rare conditions being able to access new treatments and interventions on the NHS. For many rare conditions, there is considerable variation in how the condition presents, in terms of the range of symptoms, their severity and the rate or degree that the disease progresses. The ability to accurately measure and define benefit could be problematic due to the small number of affected individuals, even in cases where, on an individual basis, there are clear benefits.

21. As such, we suggest that prioritisation principle 3 (NHS England may agree to fund interventions for rare conditions where there is limited published evidence on clinical effectiveness) be moved to the qualifying principles, and the other three dropped as redundant. Instead the prioritisation principles should consist of a clearer elaboration of the factors CPAG should be taking into consideration in grouping policies as to patient benefit. NHS England may find it useful to draw on the decision-making framework previously developed by the Advisory Group for National Specialised Services (AGNSS), which were widely supported.

Q4. Do you have any comments on how NHS England's Clinical Priorities Advisory Group (CPAG) should interpret and consider 'patient benefit', including the list of excluded factors?

22. We are concerned that the description of patient benefit used serves to de-prioritise treatments for rare disease patients relative to other patient groups. In particular, the refusal to take into consideration the severity of the condition, the extent of unmet need, or any non-clinical factors disadvantages treatments for rare conditions.

23. The Sheffield paper acknowledged the weaknesses of the ENEQ scale, particularly as they related to the availability of appropriate clinical effectiveness data, and recommended that the approach be tested further before being adopted. Instead NHS England appears to have ignored these caveats, and proposed a model heavily based on the Sheffield recommendations, but with a vague reference to "clinical benefit" (defined largely in the negative) replacing the minimum clinically importance difference (MCID) analysis.

24. Instead of the black box of clinical benefit, we urge NHS England to produce a more nuanced approach to benefit, including not only all forms of clinical benefit, but also quality of life issues, consideration of wider social values, burden of illness, unmet need, health inequalities etc. NHS England may find it useful to draw on the multi criteria decision making frameworks developed in the NICE HST programme or the Scottish Medicines Consortiums ultra-orphan process, for example.

25. The insistence on only considering evidence published in the form of peer reviewed journals conflicts with the well known difficulties associated with obtaining this type of evidence for rarer conditions. For a more complete assessment of patient benefit, patient testimonies and patient reported outcome measures should be incorporated into the evidence base. This would include information both about the impact that an intervention or treatment may have had on their health, as well as on their quality of life and the quality of life of their family and carers. For many rare conditions, the most significant benefit of a treatment from the patient perspective will be in enabling them to live more independently, relying less on social care and the care provided by those around them.

26. In addition, the use of a five year window for costs changes the perception of one-off costs versus repeated costs in a distorting manner.

Q5. Please comment on whether a proposed treatment or intervention should have a higher relative prioritisation if it meets one of the following principles:

5a. Does the treatment or intervention significantly benefit the wider health and care system?

27. We consider that this principle should be considered earlier in the process, as part of the cost/benefit determinations, not at this late point. A proper weighing of the cost of a treatment should consider all aspects of this cost to the NHS, and potentially also to other government departments, not simply the price of the medicine. A treatment may itself command a high price, but may bring significant clinical benefit to the patient and, therefore, may also save money for the NHS in the long-term if the patient no longer needs to access other treatments or services.

28. We would suggest that instead of being a prioritisation principle, considered only at the very last stage of the process, these aspects should be separated and considered at the more appropriate stage of the cost/benefit grouping. It would be appropriate for cost to the National Health

Service (NHS) and personal social services to be considered as part of cost, and, similarly, as part of benefits considerations whether there are significant benefits other than health and whether a substantial proportion of the cost (savings) or benefits are incurred outside of the NHS and personal and social services. This last aspect forms part of decision making in the NICE Highly Specialised Technologies programme, and is one way of bringing other aspects of value into consideration, short of formal value based assessment.

5b. Does the treatment or intervention significantly advance parity between mental and physical health?

29. We have no comment to make on this question.

5c. Does the treatment or intervention significantly offer the benefit of stimulating innovation?

30. Yes, it is appropriate that an innovative treatment or intervention receive a slightly higher relative prioritisation than its cost/benefit ratio may initially appear to warrant. The first of a particular modality which reaches the market is likely to be more expensive than those which follow. However, it is sometimes necessary to invest in a more costly first treatment in order to encourage further therapeutic innovations. We would like to emphasise that innovative products should not be valued simply because they are new, however, but should contribute something new clinically to patients and carers.

5d. Does the treatment or intervention significantly reduce health inequalities?

31. We agree that a proposed treatment or intervention should have a higher relative prioritisation if it significantly reduces health inequalities. Patients affected by rare or genetic conditions experience a number of health inequalities, such as relating to time to diagnosis and access to treatment. There are several aspects to health inequality experienced by rare disease patients: between people living with the same rare condition; between people living with one rare condition and those with a different rare condition; between people living in communities with a high prevalence of rare conditions, and those in communities with a lower prevalence; and between people living with rare conditions and those living with common conditions.

32. The UK government published the UK Strategy for Rare Diseases in 2013 as a first step to addressing the unmet healthcare needs of the millions of people living with a rare disease in the UK, and who currently struggle to get access to integrated care and support from the NHS. Genetic Alliance UK believes that an effectively implemented plan for rare diseases has the opportunity to address many of the inequalities currently experienced by patients affected by rare diseases and their families.

33. While we welcome the acknowledgment of the health inequalities faced by rare diseases patients discussed in the equality impact assessment which formed part of the consultation guide, these issues are only considered as part of the proposed method at the final stage of possible adjustment to ranking. This is too little too late. Coming at the end of a process of prioritisation which repeatedly and consistently de-prioritises treatments for rare conditions, it is not possible for a simple adjustment to rankings to undo this disadvantage.

34. This is particularly the case as senior NHS England staff have said that they do not consider it likely for a policy to receive an upward adjustment of more than one priority level. It is not clear how this consideration of health inequalities can be more than tokenistic, unless the adjustment is more substantial than is being suggested.

Q6. Would adoption of the proposed method unfairly discriminate against any group with protected characteristics?

35. As discussed elsewhere, the proposed method unfairly discriminates against patients with rare conditions.

Q7. Would adoption of the proposed method assist NHS England in promoting equality and in reducing health inequalities?

36. As described above, the proposed method de-prioritises treatments for rare conditions, an aspect which has been acknowledged by senior staff at NHS England. This represents a step backwards from the previous recognition of the patient, public and governmental support that exists for ensuring that patients affected by rare conditions are able to access effective treatments.[i,ii,iii,iv,v,vi], particularly when the medicine is required by children[vii,viii].

37. We are particularly concerned about some comments which were made by Dr James Palmer, in the company of Dr Jonathan Fielden, at NHS England's public workshop on this consultation, Wednesday 4 May. Palmer recognised the likelihood that most rare condition medicines would receive a maximum priority ranking of three, and so be unlikely to be funded, stating that "high cost medicines must deliver high benefit". This conflicts with the special challenges of demonstrating benefits in rare conditions, acknowledged by NHS England in the third prioritisation principle.

38. For the vast majority of rare conditions, generating clinical evidence of a standard that can support commissioning decisions is challenging. The small number of affected individuals, the often heterogeneous nature of the condition and a lack of interest in clinical research for treatments in unlicensed indications, despite there being a good scientific basis for their efficacy, are often limiting factors for generating data on the clinical (and cost effectiveness) of a treatment for a rare disease.

39. Palmer also expressed the opinion that the current system cannot give priority to rare diseases and that this 'would only change if there was a separate budget for rare disease ... but then we'd be arguing about the definition of rare'. There is much that is concerning about this statement.

40. First of all, the method proposed here, with a few small changes and slight shifts in focus, is entirely capable of dealing with the special challenges of rare disease medicines, as discussed above.

41. Second, a ring-fenced fund for rare diseases is definitely not the answer here. Genetic Alliance UK does not support the use of ring-fenced funding to maintain access to medicines and services for rare diseases, or for any group of patients. We believe that the best means to ensure that all patients are able to access effective medicines and services on the NHS is by establishing a system for evaluating and commissioning them that is timely, transparent and fair to all.

42. Ring-fenced funds make it more difficult to adapt resource allocation to patient need. Once a ring-fenced budget is put in place, it becomes much harder to justify either increasing or decreasing the amount of money allocated for that purpose. This means that is possible for either too little or too much of the limited NHS budget to be spent on one group of patients or type of condition. As a result, those patients whose medicines or services are financed by the fund may not receive the care or medication they require, or alternatively, they may receive disproportionately more while other patients, whose medicines or services are not paid for by the fund, lose out.

43. The eligibility criteria for a fund will by necessity rule out some treatments or conditions and it is possible that some patients will find themselves falling between the gaps of all the funding pots and unable to access any medicines or services. There is also the risk that a medicine or service won't be commissioned and paid by one ring-fenced fund on the assumption that it will be captured by an alternative fund.

44. If medicines and services are paid for by a number of distinct funding pots it is likely that each will have their own criteria regarding cost-effectiveness. This can mean that potentially unfair and inconsistent decisions are made if one ring-fenced fund considers a medicine to be cost-effective when consideration through another's process would not have found it to be. As a result patients may be unable to access an effective medicine for their condition on the grounds of cost while a less effective medicine for a different condition is made available.

45. Third, a rare disease is defined by the European Union as one that affects less than 5 in 10,000 of the general population. This definition is found in European legislation, for example EU Regulation on Orphan Medicinal Products (1999), is widely accepted in Europe and the UK, and applied by the European Medicines Agency. Additionally, many conditions, both rare and common, can be further stratified based on clinical presentation, genetic or biochemical differences. Increasing knowledge of the genetic and molecular basis of disease has revealed that what used to be considered a single disease is in fact a collection of related but fundamentally distinct conditions. Moreover, this improved understanding has led to greater recognition of the fact that different subgroups within a condition may respond differently to medical interventions.

46. This makes identifying these subgroups and differentiating between them in a clinical setting of critical importance. However, contrary to Palmer's implication, it is entirely possible to have a clear and unambiguous set of criteria to define what constitutes a rare condition, or a rare presentation of a more common condition, which reflect the realities of rare diseases as they present in the clinic.

47. We also wish to rebut Palmer's suggestion, made at the same workshop, that medicines for rare conditions are more appropriate for evaluative rather than routine commissioning routes. To imply that a medicine for a rare condition will never attain the level of evidence required to gain routine commissioning is entirely contrary to the purpose of having the third prioritisation principle.

48. In our experience patient representatives are positive about the application of CtE to medicines for rare diseases. They agreed that pilot schemes enabled the development of a higher quality evidence base than might otherwise be available for a rare disease medicine, where randomised controlled trial evidence is less prevalent. They also felt that it had the benefit of considering a treatment in a 'real-world' scenario, allowing patients to access medicines in a controlled way while avoiding the strict and unrealistic patient eligibility requirements associated with clinical trials.

49. However, following a process of CtE, should a medicine for a rare condition ever be selected for, it, we understand that the medicine would then need to go through the standard specialised commissioning prioritisation process in order to access routine commissioning. For the Clinical Director of Specialised Commissioning for NHS England to be of the opinion that even after going through CtE, a medicine for a rare condition would still be unable to meet the required effectiveness and evidence standards, suggest that the decks are truly stacked against rare disease patients.

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[i] Social Value Judgements: Principles for the Development of NICE's Guidance. NICE, 2005, available at: www.nice.org.uk/media/c18/30/svj2publication2008.pdf

[ii] M. Rawlins et al., Pharmacoeconomics: NICE's approach to decision-making. British Journal of Clinical Pharmacology, 2010, available at: www.ncbi.nlm.nih.gov/pmc/articles/PMC2949905/

[iii] Citizens Council Report, Rule of Rescue. NICE, January 2006, available at: www.nice.org.uk/niceMedia/pdf/Rule_of_rescue_report_final_0606.pdf

[iv] Citizens Council Report, Inequalities in Health. NICE, June 2006, available at: www.nice.org.uk/niceMedia/pdf/CCreportonHealthInequalities.pdf

[v] Governmental willingness for 'appropriate priority': House of Lords (2013) Hansard, 28 February, Column 1161 [Online], available at: www.publications.parliament.uk/pa/ld201213/ldhansrd/text/130228-0001.htm

[vi] Rare Diseases: House of Commons (2013) Hansard, 17 December, Column 177WH [Online], available at: www.publications.parliament.uk/pa/cm201314/cmhansrd/cm131217/halltext/131217h0001.htm

[vii] Social Value Judgements: Principles for the Development of NICE's Guidance. NICE, 2005, available at: www.nice.org.uk/media/c18/30/svj2publication2008.pdf

[viii] M. Rawlins et al., Pharmacoeconomics: NICE's approach to decision-making. British Journal of Clinical Pharmacology, 2010