



Genetic Alliance UK
Supporting. Campaigning. Uniting.

Consultation Response

Department of Health

A new value-based approach to the pricing of branded medicines

Response by Genetic Alliance UK

1. Genetic Alliance UK (formerly Genetic Interest Group) is the national charity supporting all those affected by genetic conditions. Genetic Alliance UK aims to improve the lives of people affected by genetic conditions by ensuring that high quality services and information are available to all who need them. Our membership represents more than 150 voluntary organisations working for a wide range of conditions, many of which pose complex health and social care needs.
2. A baby with a genetic condition is born every half an hour in the UK; of these only 4 in 10 will have their condition cured or ameliorated, the rest will die or live with a lifelong chronic condition. Our member charities support patients who may require a broad range of pharmaceuticals.
3. We are grateful for the opportunity to comment on this consultation.

Question 1: Are the objectives for the pricing of medicines set out in Section 3 of this document – better patient outcomes, greater innovation, a broader and more transparent assessment and better value for money for the NHS – the right ones?

4. Genetic Alliance UK agrees with the principles of greater transparency, broader criteria for assessment, assessment based on patient outcomes, and adopting innovation.

Question 2: Should value-based pricing apply to any medicines that are already on the UK market before 1 January 2014? If yes, should this be determined on an individual basis, or are there particular groups of drugs which might be considered?

5. We do not take a view on this point.

Question 3: Are there types or groups of medicines, for example, those that treat very rare conditions, which would be better dealt with through separate arrangements outside value-based pricing?

6. We do not believe any groups of medicines should be excluded from the scope of this system.
7. A pricing system with separate arrangements for groups of medicines, such as orphan products, would necessarily create boundaries between the remits of each set of arrangements and possibly separate budgets for each system. Wherever boundaries or separate budgets exist, issues arise around criteria for deciding which side of the boundary a product falls or from which budget a product should be paid for.

8. In designing a new system for assessing the value and price of products, we should aim for a universal system that is capable of assessing the value of, and assigning a price to, any product which comes before it.
9. Special arrangements should be made to allow for the inclusion of orphan products in the value-based pricing appraisal process. Procedures that take account of the likely evidence gaps which arise due to the small target populations of orphan medicines should be designed. These should be capable of evaluating a product and producing an interim price to allow patients access to the treatment and for evidence to accumulate for future review.

Question 4: Do you agree that we should be willing to pay more for medicines in therapeutic areas with the highest unmet needs, and so pay less for medicines which treat diseases that are less severe and / or where other treatments are already available?

10. We should certainly be willing to pay more for medicines that bring greater value to patients and to the healthcare system.
11. This consultation does not address the question: “what should be valued in a value-based pricing system?” Or “what is value?” Genetic Alliance UK believes that unmet health need and disease severity should be a component of any measure of value. By measuring value in such a way, the system should be able to price treatments that address severe unmet health need appropriately.

Question 5: How should we approach the issue of a single drug which delivers significantly different benefits in different indications?

12. Medicines should be made available to all those who stand to benefit from them at an appropriate price. Scenarios which create a disincentive to bring a product through regulatory procedures should be avoided.
13. Genetic Alliance UK believes medicines with more than one indication should have a single weighted price. This should reflect the greater number of treatment options and the product’s wider market access, but should also provide sufficient incentive for companies to properly investigate wider usage options and follow the appropriate regulatory procedures.
14. Any approach taken should aim to limit regular off-label usage of licensed medicines.

Question 6: What steps could be taken to address the practical issues associated with operating more than one price for a drug, if we took such an approach?

15. We do not take a view on this point.

Question 7: Do you agree that – compared to the current situation – we should be willing to pay an extra premium to incentivise the development of innovative medicines that deliver step changes in benefits to patients but pay less for less innovative drugs?

16. Genetic Alliance UK believes we should certainly be willing to pay more for treatments that deliver extra benefits to patients.
17. The definition of innovation is a difficult area. Innovation *per se* is not necessarily a good thing; we do not believe it should be valued for its own sake. Innovation should be valued in these cases:
 - where it addresses a societally valued outcome;
 - where there is potential to build on the innovation to bring benefits in broader indications than its initial target;
 - where a technology can bring benefits across a wider range of disease areas.

18. Many other aspects of innovation should be covered in other areas of the assessment of value, such as areas of unmet health need and disease severity.
19. The issue of whether the NHS drugs budget should pay for these measures of innovation should be examined further. The growth of the medical research sector in the UK has been identified as a strategic priority for the UK economy. Support for this initiative should not come from our budget for pharmaceuticals but from the relevant government department.
20. Environmentally friendly drugs and drug production means have been mooted as a form of innovation to be valued; again, Genetic Alliance UK does not believe this form of innovation should be paid for from our budget for pharmaceuticals. The NHS should not pay for innovation that does not contribute to health gain.

Question 8: In what ways can we distinguish between levels of innovation?

21. It should be recognised that “incremental” innovation can deliver just as much value to the patient, or to wider areas such as research and society, as “step-change” innovation. One “type” of innovation should not be favoured or promoted over another.
22. The first, and therefore “most innovative”, treatment to arrive for a particular indication may not be, and is unlikely to be, the best in the field. The first gene therapy treatments for X-linked severe combined immunodeficiency disease (X-SCID or the “bubble-boy disease”) was delivered by a relatively crude vector, which caused leukaemia in a significant number of treated patients.
23. The successors to this first product for X-SCID have become significantly more refined and significantly safer. There are two forms of innovation at play here: first the “step-change” innovation which delivers the concept, though faulty; and then the incremental innovation which eventually produces a safe product. Neither form of innovation is more important than the other in this case, they should both be valued and both be fostered.
24. Innovation should not be judged on what “type” it is, it should be judged on the value it brings to the health service and patient.

Question 9: How can we best derive the weights that will be attached to each element of the assessment? Are there particular elements we should put greater weight on?

25. This focus on weighting comes early in the design process. Genetic Alliance UK cannot conceive of a system that would be able to price any treatment that comes before it solely by utilising a system of metrics; particularly if such a system were to be universal. We believe there should be a reasoned examination of evidence available as part of the assessment process, and that this assessment should be carried out by a representative group of stakeholders, which includes patients, able to assess value to patients and value to the health service.
26. Simple systems, frequently implemented to create as transparent a process as possible, are poorly equipped to deal with complicated situations such as this.
27. Decisions regarding weightings should come after all aspects of value have been identified, and should be part of a flexible system, able to assess all that comes before it and able to accept and cover the gaps in the evidence base that so often appear in the evidence base for treatments for rare disease.

Question 10: What measure should we use to define the weightings? Options might include using the existing Quality Adjusted Life Years (QALY) measure, patient experience and expert opinions or some combination of these.

28. Genetic Alliance UK believes weightings in categories should only be a part of the process of valuation and pricing. We believe there should be a reasoned examination of evidence available as part of the assessment process, and that this assessment should be carried out by a representative group of stakeholders, which includes patients, able to assess value to patients and value to the health service.
29. It is important that measures used in assessments are meaningful. Hard metrics must have a practical, qualitative benefit. A treatment, for example, which brings the benefit of allowing a patient's elbow to bend to a greater number of degrees should not be judged on the distance of motion but on the subjective implications this has for the patient in their daily life.
30. Patient experience measures should be broad and aim to capture the full impact a treatment can have not only to the individual patient, but also to the healthcare system and to society as a whole. We believe further consultation, including patient workshops, will be necessary to capture all aspects of value that should be captured by a value-based pricing system.

Question 11: How can we best derive the different categories for burden of illness and therapeutic innovation and improvement?

31. Genetic Alliance UK believes weightings in categories should only be a part of the process of valuation and pricing. We believe there should be a reasoned examination of evidence available as part of the assessment process, and that this assessment should be carried out by a representative group of stakeholders, which includes patients, able to assess value to patients and value to the health service.
32. Any system built solely from weightings, measures and categories cannot hope to be able to deal with the combinations of advanced innovation and treatments for serious unmet health need that this system should be able to deal with.
33. We believe further consultation will be necessary to capture all aspects of value that should be captured by a value-based pricing system.

Question 12: What approach should be taken under value-based pricing where insufficient evidence is available to allow a full assessment of the value of a new medicine?

34. This issue would be best addressed by the addition of assessment of evidence by stakeholders, as part of the assessment process; and by vigilance, feedback and reassessment as evidence is gathered.
35. Genetic Alliance UK believes all decisions made by a value-based pricing system should be revisited on a timetabled basis (based on data need, disease severity and drug quality) to ensure the value of a treatment to patients or the healthcare system has not changed.

Question 13: Does the system set out above describe the best combination of rapid access to prices and affordability?

36. In our view the system set out lacks patient input. We believe patients should be considered to be an equal stakeholder in the decision making process and that value should be judged both from the perspective of the healthcare system and from the perspective of patients. We outline further thoughts on patient involvement in our answer to question 20.

Question 14: In what circumstances should a value-based pricing assessment be subject to review?

37. Genetic Alliance UK believes all decisions should be open to appeal and that any stakeholder should be able to appeal. Appeals should be possible for an adjustment in either direction. The threshold should be set in such a way to deter frivolous appeals.

38. We do not take a view on any appeals process.

Question 15: What arrangements could be put in place within the new medicines pricing system to facilitate access for patients who may benefit from drugs previously funded through the Cancer Drugs Fund, at a cost that represents value to the NHS?

39. Continuity of treatment provision should be ensured for individual patients as the new system is implemented. This may prove to be a difficult undertaking due to the regional mechanism by which the cancer drugs fund was implemented.

40. The Cancer Drugs Fund has led and will lead to inequity in the provision of high cost treatments in the NHS, creating a skew towards cancer drugs. Genetic Alliance UK hopes that the value-based pricing system will not have any similar inequity.

Question 16: Will the approach outlined in this document achieve the proposed objectives of better patient outcomes, greater innovation, a broader and more transparent assessment and better value for money for the NHS?

41. The principles behind this initiative are sound. Patient involvement as equal stakeholders should be added to these.

42. Greater clarity on the definition of value and the means by which value will be judged is necessary before Genetic Alliance UK can support this system.

Question 17: Are there other factors not mentioned in this document which the new system should take into account?

43. NICE's appraisal system provided a benefit absent from these proposals: the binding nature of a positive opinion from NICE. This made purchase of these treatments mandatory for healthcare providers, ensured that the most cost-effective treatments were being delivered to patients, and drove the uptake of innovation by healthcare providers.

44. Once a product has negotiated the value-based pricing procedure, the price should not be subject to further negotiation at local level.

45. Attention should be given to ensure that the value-based pricing system can ensure uptake of assessed products, to ensure patients can access new treatments when they become available.

Question 18: Are there any risks which might arise as a result of adopting the value-based pricing model as outlined above? If so, how might we try to reduce them?

46. The key risk is that this system is implemented poorly and that it does not take into account of all relevant elements of value or takes account of non-health care related terms.

Question 19: What steps could be taken to ensure that value-based pricing has a positive impact in terms of promoting equalities?

47. A key inequality in health is the disparity between healthcare experiences felt by those with rare diseases when compared to the experiences of those with better understood common conditions.

48. This system can therefore contribute to the reduction of healthcare inequalities by valuing treatments for those with rare diseases appropriately.

Question 20: Are there any other comments or information you wish to share?

49. Patient involvement is notably absent from this consultation document. We believe patients should be involved in two ways in this system.

As participants in the value-based pricing appraisal process

50. Patients should be given the duty and responsibility first to be included first in the design of this system, in the definition of value and in the design of the appraisal process; and second to be involved in the appraisal process itself, as disinterested experts with expertise in patient experience. From these two positions patients can contribute to the appraisal process as equal stakeholders and ensure that value to patients is assessed on an equal level to value to the health service.

As expert witnesses in individual appraisal processes

51. Patients with expertise in individual disease areas should be given the opportunity to contribute to the assessment of the value of a treatment during the appraisal process. NICE currently provides this opportunity during its appraisal processes. We hope that the inclusion of patients in the appraisal process will ensure that patient testimonies are taken into account properly in the decision making process.

52. In the past questions have been raised as to the ability of patient organisations to act at this level in the UK. Whilst we certainly do not believe all patient organisations have the capacity and expertise to contribute to appraisals in both of the ways we propose here, many do.

53. We point to the example of the involvement of patients in the decision making at the European Medicines Agency (EMA) as evidence of the level at which patients can contribute. At those scientific committees on which patients sit, they have equal rights to any member state member, and frequently add value and change the perspective of arguments. Patients also contribute as expert witnesses at the EMA. At this level of involvement, single testimonies have been known to reverse a decision by an expert committee. We hope patients can contribute to the value-based pricing process at the same level as they are allowed to at the EMA.

54. There is a vast spectrum of capacity of patient organisations in the UK. Many patient organisations for rare diseases lack the capacity to submit sophisticated papers, this should not preclude their involvement, but neither should it preclude the involvement of patient organisations with greater capacity at a higher level. Outreach and support should be available to ensure patient organisations can contribute at the level they wish to.