

Consultation Response

NICE Citizen's Council meeting report

“Departing from the threshold”



Response by the Genetic Interest Group

The Genetic Interest Group (GIG) welcomes the opportunity to contribute to the NICE Citizen's Council's discussions on “Departing from the threshold”. As the UK Alliance of charities and support groups for patients and families affected by or at risk from all forms of genetic disorders (ranging from very rare conditions resulting from an adverse mutation in a single gene to common complex disorders arising as a consequence of an intervention between genetic, environmental and lifestyle factors). GIG speaks from the perspective of approximately 140 organisations promoting awareness of the needs of millions of UK patients living with unmet medical needs arising from intractable and incurable severe, chronic and life limiting disease.

GIG responded to the call for evidence in advance of the NICE Report on Valuing Innovation, and believes that some of that evidence submitted is of value in this debate, we have therefore re-visited some of that evidence, and present the relevant points below.

For those affected or at risk from many of the rarer conditions GIG supports, high quality bio-medical research offers the prospect (albeit distant in most cases) of prevention, care and relief from symptoms and their consequences. Despite unprecedented progress in our understanding of our fundamental biology in recent years, most diseases arising wholly or partly from our genes remain intractable. As evidence of their commitment to the demand that this will not remain the case indefinitely many of GIG's members commit substantial efforts to raise funds to support bio-medical research into “their” condition. If this investment is successful and if new knowledge is to create opportunities to alter the natural history of intractable diseases then the outcomes of research must be systematically translated into products and services that are available to those who need them systematically, equitably and affordably in a timely and user friendly manner.

Arguably more resources should be committed to the provision of health care by the NHS, but even if this were to be the case, the total budget is finite, and once a line has been drawn then decisions need to be made about resource allocation that are fair, national, open to scrutiny and which command sustained public support for the application of taxpayers' and citizens' resources.

The arguments in support of the concept of health technology assessment (HTA) are unassailable. The NHS absolutely needs to know what works, for whom, under which circumstances, how well and at what cost. Having undertaken such an assessment it then needs to determine whether or not the intervention is to be purchased, how much of it is needed and under what circumstances it is to be deployed.

The “problem” is not with the logic underpinning HTA, but with the methods used to put the theory into practice. To patients and families these can be crude and unsophisticated omitting issues for consideration that are important to those affected by life limiting conditions and those who care for them.

Doubts about the validity of processes used to assess healthcare technologies create the potential for conflict and a risk that the equity essential for sustaining the NHS will be undermined by noisy advocacy by well resourced vested interests to secure personal gain. Of course, in a democratic society citizens have the right to challenge decisions made on their

behalf by others using all lawful means. And of course, at the level of the individual or group, if a process of evaluation results in a decision that is other than then one hoped for then that citizen or group will not be happy and may choose to challenge it. But if the process undertaken by those making the decision is acknowledged to be fair, relevant and robust then those on the receiving end of a “no” are more likely to be content that they have had a fair opportunity to make their case, even if they are unhappy with the result.

A framework for assessing healthcare technologies must have certain general principles fundamental to evidence based decision making. To be fair, GIG believes their application in practice needs to be determined on a case by case basis. Thus there needs to be clarity about what constitutes “evidence” and how much of it you need in order to reach a decision in a timely manner. Given that “evidence” will be generated by different stakeholders and from a range of sources it cannot be judged a being of equal value irrespective of provenance, so a clear framework for “weighting” evidence must be established and placed in the public domain together with the underpinning rationale. It should not, for example, be assumed that a Randomised Controlled Trial (RCT) is automatically the gold standard, trumping evidence from other sources, if the RCT in question measures changes of little importance to patients or families (unless these changes are strongly predictive of, say, important safety issues). Anecdotes in isolation may be relatively unimportant, but they may be indicative of a significant trend that needs to be factored into the process.

Whilst figures such as cost/QALY can provide a useful yardstick they are crude and omit many factors that are significant for patients and families. For example, families living with a member affected by a chronic complex condition often struggle to create a network of interdisciplinary inputs from different professionals employed by health, social care, education and voluntary sector bodies. Once in place they are loathe to disrupt it, creating lost opportunity costs arising from the need to stay put. If the condition affecting their family member becomes treatable than these may be reduced or removed but the financial, psychological and other benefits that result are not taken account of in valuing the health technology under assessment.

Similarly, it is often asserted that a “QALY is a QALY is a QALY”. For families and for patients this is clearly not the case. The value assigned to additional or improved life will be dependent on circumstances associated with the patient’s situation, not just his or her disease. Thus a gain of x months may be valued differently by a father waiting the birth of a child, a grandmother approaching a grandchild’s wedding or a person experiencing suffering who feels tired and that they have had enough. Any scheme to value treatments appropriately needs to take account of subjective elements (both positive and negative) if it is truly fair and equitable and commands public legitimisation.

Many patients find it difficult to comprehend why health care systems at apparently similar levels of development can reach significantly different conclusions about the value of an intervention. The notion of “therapeutic added value”, i.e. change from the expected course of the condition brought about by a given intervention ought to be reasonably constant in comparable health care systems such as those in Western Europe. Reaching a consensus that involves all key stakeholder groups, including patients, and across national boundaries as to the therapeutic added value of a given intervention ought to be a goal of systems established to value health technologies. This is not to confuse therapeutic added value with cost/resource allocation decisions which and will remain a national or regional responsibility for Member States, not something that can be determined at European level. Separation of these two elements would help allay suspicions that decisions about clinical effectiveness are often unduly influenced by financial constraints, but the responsibility of making them is shifted unfairly from the political to the clinical arena.

Clearly, any process of assessing healthcare technologies will depend on balancing objective and subjective criteria derived from research, social values, clinical opportunities and available resources (material, financial and human). As such it will be an imperfect process, but it should be capable of working equally effectively across classes of interventions, making possible fair comparative evaluation between, say a surgical procedure, a novel pharmaceutical and a device; with similar standards, rigour and judgement of relevant end points applied.

Key parameters for such a system might include:

- The seriousness of the condition - with research commissioned to establish a consensus about the relative weights to be attributed to, for example, conditions which might result in early death for children, significantly reduced life expectancy for adults, impaired quality but not necessarily quality of life etc.
- The impact of the proposed intervention - does it prevent, cure, treat, palliate symptoms (and if so how important are these for those affected and their carers - e.g. preservation of daily living skills compared with cognitive functioning in Alzheimers' patients).
- Existing interventions for this indicator, and how satisfactorily they work in comparison with the novel one. In general a first opportunity to intervene in a serious condition ought to be valued more highly than a marginal improvement in conditions where therapeutic possibilities exist.
- Innovation *per se* is not useful as a determinant for resource allocation by the NHS, though it may be of high value in other sectors and knowledge has intrinsic value. Innovations need to be valued with reference to the health gain they deliver, but this can be direct or indirect in its effect, e.g. by the improvement in circumstances of the patient and for his/her carers and family that result from their adoption (see above).
- Care must be taken to avoid simplistic expectations that may result in unfair discrimination. Whilst it may be a bonus that the use of intervention 'x' allows citizen 'y' to resume employment and become a taxpayer again, the fact that interventions may benefit people not of working age, or who may not be able to seek employment for other reasons should not be used as an adverse weighting factor. Similarly, expensive interventions should not be penalised by reference to an anticipated financial payback needed (but unlikely to be achieved) to provide a return on investment in a narrow financial sense. A financial payback is not automatically better (or worse) than a physical, a psychological or a societal one.

Finally, the NHS has an obligation to develop mechanisms (such as the National Commissioning Group) that operate at the right level in the system to take account of the impact and benefit to the system as well as to patients of apparently expensive interventions. For example, high cost drugs for very small numbers of patients can have a disproportionate impact when judged at the level of the PCT or Foundation Trust hospital (or similar bodies in the other home nations) but may become reasonable and manageable when considered at regional or national level. Equally greater use should be made of risk and benefit sharing methodologies when considering potential value added in cases where the evidence base is judged to be lacking in key respects.

We would be happy to expand on the above in person or in writing if this was felt to be helpful.



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