

Report on NICE Citizens Council meeting

Health Innovation and Value

May 28-30, 2009

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What we were asked to do

- 1) To acquire an insight into the public's view of innovation, and to explore which features of innovation should be considered when assessing its value, specifically in the context of health care.

- 2) To provide a report to be fed into a study on the value of innovation in health care being carried out on behalf of NICE by Sir Ian Kennedy.

What we concluded

In identifying the features of a healthcare innovation that make it valuable, each of our members chose three features from the list below. The final vote was as follows.

Question: What makes an innovation valuable to you?

1. It increases quality of life	26
2. Other innovations may be developed from it in the future	11
3. A large number of people will benefit from it	10
4. It saves your life	9
5. It increases life expectancy	9
6. It meets a previously unmet need	6
7. It prevents a condition	5
8. It cures a condition	5
9. There are few other treatment options	1
10. It reduces risk to the patient	0
11. It has a one-off cost rather than ongoing costs	0

We were also asked to tackle two other questions. First, if the innovation is more expensive than NICE's current cost per QALY gained threshold and does not demonstrate benefits at the moment but could well do so in the future, what should NICE do?

Fifteen of us (a majority of two) responded that we should say "no" for the present, and ask the developers to fund and carry out more research themselves. The remaining 13 of our members also wanted us to say "no" for the present, but preferred that further research be co-funded for the developers on condition that the public would get a return proportionate to any investment.

For a clearer insight into these conclusions we were asked more specifically who should bear the costs and risks of research and development. We had five choices:

- The developer
- The NHS
- The taxpayer
- Charities
- Joint sharing between taxpayer and developer

Seventeen of us felt that the developer of the technology should bear the full cost. The rest, 11 in total, opted for joint sharing between the taxpayer and the developer.

We also offered NICE a raft of suggestions (listed in the body of the report) on how it might improve its communication with the public about innovation.

How we worked

Twenty eight of the members of our Council were able to attend the meeting, held from May 28 to 30 at the NICE headquarters in London. The format adopted was rather different from that of our previous meetings. In line with requests that we ourselves had made there were fewer presentations from invited speakers, and more time was given over to small group discussions.

On the morning of the second day we learned how NICE appraisal committees work – and were then granted the opportunity to “be NICE for a day”. This meant trying to reach our own decisions on the value of innovation. The afternoon was given over to a pair of presentations on the manner in which the media report medical innovations, and a subsequent discussion of ways in which NICE might improve its communications with the public around innovation.

Our final morning was devoted to refining a number of questions on which to vote, and so determining the main conclusions of our report.

At the beginning and the end of the meeting we completed tracker questionnaires. These were intended to assess our views on NICE, our thinking about innovation in healthcare, and the extent to which these had changed during the course of the meeting.

What we heard, and what we did

Introduction and background

Professor Peter Littlejohns of NICE introduced the issues we were being asked to address. Our meeting was part of a wider consideration of the benefits of innovation - a topic to which NICE has sometimes been

accused of not paying sufficient attention - and whether the current appraisal arrangements give them the weight they deserve. Formally expressed, the issue is as follows:

“Advances in science have radically improved the health of people around the world. We now have a vaccine for cervical cancer, we can insert stents to open blocked blood vessels, and advances in genomics mean we can now identify patients who will respond to treatments. But does new always mean better? When is something an “innovation”? What price should be paid for innovations? Who picks up the bill for them? And with limited NHS resources should innovative treatments have priority, or some special status above existing treatments?”

The wider consideration of these issues is being undertaken by Sir Ian Kennedy, to whom our conclusions will be forwarded for consideration along with evidence from other individuals and organisations.

Our task, Professor Littlejohns explained, was to explore how the public perceives innovative healthcare technology (whether positively or negatively), to consider whether the type of technology (drugs as opposed to devices, for example) affects this perception, whether the likely use of a technology (in acute illness or chronic disease or cancer) makes a difference, and whether the health outcome produced by the innovation (life-saving or life-prolonging as opposed to improving its quality) also matters. We were also asked to think what innovation-related factors might make people prioritise one technology over another, and explore what information might influence an individual’s views on whether or not a health care technology is innovative.

Innovation, he reminded us, comes in all sizes and may - as in the case of thalidomide - have unforeseen consequences. And although drug treatments are most often associated with NICE, the organisation is also concerned with the value of other health technologies including devices and also of public health issues.

At this point he handed over to Sir Ian Kennedy for his thoughts on the enquiry he is undertaking for NICE. Sir Ian described the Citizens Council as one of the ways in which he would try to plug into feelings from the wider world that extends beyond the laboratory and the clinic and comprises the people on the receiving end of whatever innovations are eventually adopted for routine use. If it’s important to encourage innovation, he asked, what do we mean by the word? Just new? Or new with the potential for substantial further development?

Furthermore, should we actively encourage innovatory developments by supporting them? If so, who should bear the cost of such support? Or do we just thank the innovator and ask him or her to return once the innovation is fully fledged?

Important innovation can be quite modest – as with the introduction of a pre-operative check list for surgeons. (The idea has been imported from aviation where it has been routine for decades.) And what of the balance between life-saving innovations in acute conditions as opposed to less dramatic innovations in chronic conditions? It has to be remembered, he concluded, that with a finite pot of money there will be losers as well as winners when choices are made.

Brainstorming on innovation in general

For our first discussion we divided into smaller groups to consider the meaning of the words “invention” and “innovation”, and to try identifying the difference between them. We also reflected on the value of innovation in general, not only in medicine in but in the rest of our lives. In the following plenary discussion it soon became clear that we had differing and contradictory ideas about the relationship between invention and innovation. Some of us felt that the latter encompassed the former; others believed the opposite. The discussion was abandoned when a Council member pointed out that the word used by NICE and by Sir Ian was “innovation”, and that it was unclear whether the niceties of distinction, whatever they are, would make little practical difference.

One group suggested that innovation is usually the product of bringing together different technologies and ideas. We could all offer plenty of examples of everyday innovation (from the internet to the widget designed to put a head on the contents of a can of beer), but we rate their value in many different ways. Economic benefit, and usefulness by comparison with what’s already available were just two such offerings. But it was also suggested that individuals’ opinions will differ depending on their economic situation and their personal values, and these may change as you go through life.

We were also aware that innovation can have negative as well as positive effects - and the negative features may not be discovered until the object or idea or whatever it is has been around for some time.

Brainstorming on health innovations in particular

Once again we divided into three groups to take a more focussed look at innovations in healthcare. The first group to report back had concentrated on a number of examples including anaesthetics, antibiotics, stem cells and keyhole surgery. The positive aspects of such developments were easy to identify; leaving hospital sooner after an operation in the case of keyhole surgery, for example. Their negative consequences were sometimes less obvious - and often, the group concluded, a consequence not of some inherent weakness in the innovation concerned, but of its inappropriate application. They offered the example of people who had become reliant on the use of drugs for depression, with its long term cost to the individual, the family and the NHS. The first group commented that looking at values and benefits raised many moral issues, but felt that valuable innovations had to be cost-effective and offer the greatest good for the greatest number.

Having also listed a number of innovative technologies, the second group concluded that those worth having were those that saved life, prolonged life, improved its quality, and were cost-effective. They too chose anaesthetics as a good example of past innovations with a long and positive track record. And they too were aware of the negatives as well as the positives of innovation. Under this heading they pointed out how antibiotics, for all their obvious benefits, are not a panacea. Overuse had led to the emergence of resistance, and doctors now had to be more careful in prescribing them.

The second group also offered two other important cautions. First, different people have different priorities, and these are not necessarily fixed. Second, a distinction has to be made between wants and needs. What we want may not be what we need, and vice-versa. Under this heading they mentioned IVF.

The third group said that they had begun by categorising innovations according to their use in diagnosis, treatment or prevention. Certain innovations, they commented, are especially influential because they underpin or pave the way for many later developments. Antibiotics are an obvious example. Most innovations, they added, could have a negative effect if misused. They stressed the importance of trials in assessing innovation, even if the results of such experiments proved to be negative.

The third group also noted that financial incentives, while a clear spur to innovation, can have negative effects. Although encouraging research they can also foster greed, leading to incidents of the thalidomide variety.

Rating the relative importance of different innovations is difficult; everyone is tempted to think that the most important is the one most likely to be of direct benefit to him or her. More appropriate is to consider factors such as the greatest good to society, and overall cost-effectiveness.

In the general discussion following these group reports the topics ranged from the importance of hand-washing in preventing the spread of infection to the value of fluoridation in preventing dental decay. Although preventative measures may not be among the first thoughts that come to mind when considering innovation, their value is something to be emphasised.

We also returned to the distinction between needs and wants. It was fundamental, said one of our members. Another illustrated the point with reference to plastic surgery. Many of these procedures could hardly be described as fulfilling a need as opposed to a want. But when applied to someone like Simon Weston, terribly disfigured in the Falklands War, the same technology assumes a wholly different significance.

We also touched on the importance of evidence. Self-evidently it must be relevant – but what of its integrity? Could we trust the integrity of the data put forward to NICE by companies and others seeking to demonstrate the value of a drug or procedure? Who is responsible, and must all evidence be disclosed particularly relating to suspected adverse effects? Sarah Garner of NICE was able to reassure us; The reporting of adverse effects is governed by more than mere gentlemen's agreements. Data submitted to NICE is also checked for integrity.

Healthcare innovations: the variety

The afternoon began with the first of two presentations on the range and variety of healthcare innovation. Steve Morgan, an electrical engineer at the University of Nottingham, designs and builds medical devices such as a microscope for examining the shape of blood cells in blood vessels beneath the tongue. It's intended for use in sickle cell anaemia...but what is its value to the NHS, he asked. When resources are finite, which technology to buy? Medical innovations can be great or small, they can arrive in tiny incremental steps or more dramatically and disruptively (he offered the examples of angioplasty replacing heart by-pass surgery, and personal glucose meters avoiding the need for central lab tests), and they can take many different forms – which makes all comparison difficult.

Innovations may also be resisted. As a consequence of the advent of a new and easy to use device, trained staff may find certain tasks being done by others with lesser skills. They may not be persuaded by the argument that this will free them to concentrate on patients with more difficult problems. And commercial companies may not like the added competition that comes with innovation by other firms. Purchasers too may prove risk averse when faced with a new product.

In assessing innovation, NICE's cost per QALY approach is good so far as it goes. But Steve Morgan doesn't think it goes far enough. Some of the value of an innovation will be manifested in advantages that NICE can only partially (if at all) take into account under the current system. He offered a couple of examples: returning people to work; and reducing the consequent burden on those who would otherwise have to care of them.

When it comes to major, disruptive technologies, we could have had no better example than the subject covered in our next presentation. Surgeon Professor Roger Kirby described how robotic surgery originally developed with money from the US Pentagon had radically changed operations for cancer of the prostate. With the aid of a television link the surgeon can operate the robot using the controls on a console to which it is electronically linked. The surgeon could, in theory, be in another clinic, another country or on another continent. But the real advantage lies in the precision with which the system allows the surgeon to operate. When working on the prostate, great care needs to be taken to avoid excessive bleeding and also damage to the local nerves (which could cause incontinence or loss of sexual function). The robot's instruments are smaller than a surgeon's hands – the movements of which can also be de-magnified by a factor of 10, making the operator's manipulations far more delicate.

There are now some 500 of these machines in the US, but only around ten in the UK. Each costs in the order of £1.5 million. Professor Kirby foresees many other applications for the robot.

In subsequent questioning we learned that demand for the procedure often now comes from patients themselves. Some surgeons have resisted their introduction, aware that their hard-earned skills will be made redundant, and also that using the robot requires them to learn a set of totally new skills. This too adds to the cost. One of our members wondered if there wasn't the danger of a new generation of surgeons becoming dependent on the machine. Professor Kirby conceded that although failures of the instrument were infrequent, without the correct basic training there could

be the danger that a new generation of surgeons would, by definition, have difficulty in taking over and finishing off a procedure in the conventional manner.

Healthcare innovations: more perspectives

Two more presentations offered us two further perspectives: that of an NHS manager and, before her, a representative of the drug industry. Alison Clough works for the Association of the British Pharmaceutical Industry (ABPI), the drug industry's trade body. She began by drawing our attention to the importance of the industry to the UK economy (67,000 people employed; one in five of the world's top medicines discovered and developed here), the extraordinary cost of taking a medicine from discovery to launch (some £550 million), and the very slim chance of a new agent making its way through the entire process, never mind turning out to be commercial money spinner. She pointed out that innovative drugs have transformed people's lives in conditions ranging from schizophrenia to leukaemia.

Innovation, she explained, could occur in a single dramatic step, or incrementally over time. Drugs for asthma illustrated both categories with inhaled steroids falling into the first category and long acting bronchodilators into the second. But the invention of completely new drugs is only one of several targets for would-be innovators. Others include increasing the duration of benefit of existing treatments, and improving their convenience to patients.

A future appraisal system should continue to rely on the QALY system, but make additions to it. Appraisal committees, especially when facing difficult decisions, can already turn to NICE's social value judgement document for guidance. The ABPI would like to see a more formal assessment of a new product's innovatory attributes: one that took account of, for example, the age of the drug with which it was being compared. She also made a case for more consideration of the severity of the disease in question, and of the extent to which the new treatment would relieve the burden on carers. Making these changes, she concluded, would benefit patients, carers, health care professionals, the NHS, academia and the UK economy as well as the drug companies.

Our fourth presentation of the afternoon was given by Anush Hinton, deputy chief pharmacist at the Central and North West London NHS Foundation Trust. Her organisation, she said, was committed to innovation, seeing it as a way of achieving a series of goals from the improvement of patient services to the creation of new ways in which

the trust could boost its income. Toward this end the Trust has set up an innovations scheme through which individuals or teams working for it can submit their ideas for judgement by an innovations panel. Besides an improvement in services to patients, the criteria used to judge ideas include evidence of a sound business case, and of long term sustainability.

In the last round of the competition there were 16 submissions. Six of these were shortlisted, and four received the funding required to put them into practice. These four were also submitted to NHS Innovations London, a body which - if it too approves them - can encourage their adoption beyond their home trust. To illustrate the kind of innovation she has in mind, Anush Hinton quoted the example of a heart rate monitor for children with behavioural problems of a kind that might lead to them being excluded from school. When the heart rate exceeds a predetermined level, the monitor triggers an alarm that alerts teachers to the problem, and allows them to intervene before things get out of hand.

During the question and answer session that followed the presentations Anush Hinton assured us that, in assessing ideas, benefit to patients takes priority over financial savings to the Trust. Alison Clough said that much the same applied to the drug industry; patient benefit was at the core of the enterprise. One member expressed concern that too little effort might be directed to understanding the sources of disease rather than trying to fix problem when they had arisen.

Other issues raised included the 'payment' of volunteers in drug trials, whether the individuals who make drug discoveries stand to gain personally, the apparently increasing prevalence of asthma, and who initiates research in a particular area of medicine.

The remainder of the afternoon was given over to further small group discussions on valuing innovation, and a final reporting back session. During this it became clear that there was a great variety of opinion among Council members. Some found the issue of opportunity cost particularly troubling; indeed, no-one can be comfortable with the reality that one person's gain is another's loss.

There was disagreement on who should bear the cost of any further development required for an innovation. The taxpayer, the NHS, a charity, or the company developing the innovation were some of the options considered. Such limited consensus as there was seemed to

favour the company bearing the cost– which would, after all, stand to gain if the innovation was successful.

When it came to valuing an innovation, one group came up with five criteria: the success rate of the innovation; whether it was preventative; its cost-effectiveness; the possibility of it having multiple applications; and its safety. Other factors suggested by another group were whether the innovation would be a one-off or an on-going cost, whether it would make for greater efficiency in the system - the use of specialist centres for particular problems, for example - and whether there were any other means of tackling the problem.

In thinking about the nature of possible innovations, one group explored the value of taking procedures to patients rather than vice-versa, and of ensuring that clinics were open at times chosen to suit patients rather than staff. The group also wondered if the NHS should be more proactive in launching the development of innovations.

Other points that were made during the session included: the need to take the long view when judging the value of an innovation; the importance of pushing back boundaries; that devices and drugs should be treated similarly; and the relative merits of innovations that saved life as opposed to making it easier.

The reports and the subsequent conversation had gone hither and yon, with few clear themes emerging. While satisfied with the time given to us for discussion it was clear that, at the end of the first day, many of our ideas were still far from completely worked out.

How appraisal committees work

The first part of the morning of the second day of the meeting was given over to Helen Chung, one of NICE's technical advisers, to explain the basis on which NICE evaluates a new technology, and how its committees operate in practice. She covered the variety of technologies examined (not just drugs), how they are selected for review, and the principles underpinning committee decisions. These are robustness (sound evidence), inclusiveness (consultation of all stakeholders), transparency (as much information as possible is in the public domain), independence (the use of external advisory committees) and timeliness (including periodic reviews of previous decisions). Committees are charged with assessing both clinical effectiveness and cost effectiveness. The real difficulties for a committee begin when a treatment or a procedure is judged clinically effective, but is also very expensive to get

those health gains compared to what's already available. : Helen Chung illustrated the variation between people's decisions by showing a picture of some highly desirable shoes (for women), and then asking what we would be prepared to pay for them. £30? Or £300? Or £3000?

To assess cost-effectiveness in drugs, devices and procedures, NICE uses the "quality adjusted life year" or QALY. This is a way of measuring health gains that takes account of both length and quality of life, and allow comparisons to be made between different illnesses. At £20,000 per QALY gained, NICE generally regards a new drug or device as cost-effective. Between £20,000 and £30,000 it requires clear and firm evidence of cost-effectiveness. Above £30,000 the case for acceptance must be exceptionally pressing. In short, the higher the cost per QALY gained, the more compelling the argument in favour of a new technology has to be.

Various factors can tip the balance. They include the guidance contained in the social value judgement document issued to all committee members, and the extent to which the technology is innovative. The appraisals 'methods guide' which contains the advice on this second point refers to "the innovative nature of a technology, specifically if the innovation adds demonstrable and distinctive benefits of a substantial nature which may not have been adequately captured in the QALY measure". Helen Chung went on to quote examples where NICE committees had decide in favour of a technology even though it was above (and in a few cases well above) the upper threshold.

Being NICE for a day

At this point we had the opportunity to experience for ourselves what it's like to sit on an appraisal committee. We divided into two groups, each of which was given the details of two technologies to be appraised for their innovative qualities. The four examples (for details see Appendix 1) were loosely based on actual drugs or devices that had come before NICE. This allowed the decisions that we made to be compared with what had happened in reality. After an hour's deliberation the groups rejoined to report their conclusions.

Case study 1: Diabreathe

The group decided that Diabreathe, an insulin inhaler, was innovative in its field, but not innovative enough to justify the estimated £50,000 it would take to gain an extra QALY. They wondered if there were other and cheaper alternatives for getting insulin into needle-shy patients (tablets or suppositories, for example) that would be worth investigating.

The Diabreathe system also has various drawbacks ranging from dosing to concerns over adverse effects on the lungs.. They also feared that many people already using injections and not genuinely needle-phobic might wish to take advantage of this more benign but much more expensive arrangement, so increasing demand.

The actual NICE decision had gone in favour of the device on which this case study was loosely based, but only for people with a marked and significant phobia, or skin problems at the injection site. The cost per QALY for this sub-group came out at £25,000. In the event the manufacturer had discontinued production of the device shortly after the granting of this limited approval.

Case study 2: Neverreturn

Neverreturn is a hypothetical breast cancer drug used in addition to surgery, radiotherapy or chemotherapy to prevent reoccurrence caused by any remaining cancer cells. The group agreed that it was innovative and offered major benefits but, given the cost per QALY, was split on whether to recommend it. Those in favour pointed out that it was of low risk and reduced the need for further treatment, although adding the caveat that it should be monitored in further research. Those opposed felt that the evidence was too thin; it was not a complete cure and could have serious side effects on the heart. In the end the group could not reach a verdict.

In real life, and despite much uncertainty, NICE recommended the drug on which this case study was loosely based- but only with regular monitoring for heart problem. A primary care trust that would have to fund the recommended drug, appealed against the decision, but lost. In the end NICE's recommendation was allowed to stand, but with an earlier than normal review date to take account of any further evidence that might have been accumulated.

Case study 3: Coprolong

The group rated this hypothetical treatment for bowel cancer (which cuts off the blood supply to the tumour) as innovative and with great potential. But given its cost of £80,000 per QALY gained, it attracted little support – either for the short length of extra life it conferred (an average of four months) or for the quality of that life. It was though felt that the principle might be worth further investigation.

The NICE appraisal committee reached a similar decision about the real product – which was refused a recommendation. An appeal against the decision was unsuccessful.

Case study 4: Lukolife

A hypothetical treatment for a rare type of leukaemia, the group decided that Lukolife was innovative and looked as if it could hold the disease in check, though with some possibly severe side effects. But with an additional QALY costing no less than £35,000 and possibly as much £70,000, a majority of the group felt unable to recommend it.

NICE itself initially said no to the drug on which this case study was loosely based. But the manufacturer offered to refund the cost to the NHS of treating any patients who didn't respond. This reduced the cost per QALY gained and, with this proviso, NICE was prepared to change its mind and say yes.

In a brief subsequent discussion of what it was like to be “NICE for day”, no one seemed to relish the idea of undertaking this kind of duty. Not easy decisions and I'm glad I'm not one of the people having to make them, was one comment. Another member agreed; I wouldn't like to have to do it, she said.

Health innovation and the media

The afternoon was devoted to a different aspect of health innovation: the manner in which it is presented in the media. Following small group discussions on the issue we heard two presentations: one from journalist Nigel Hawkes, a sometime science and health editor of the *Times* and who now writes regularly for the *British Medical Journal*; the other from a doctor, Ben Goldacre, who writes a column on bad (mainly biomedical) science for the *Guardian*.

Nigel Hawkes described how he'd tried unsuccessfully over many years to ban the word “breakthrough” from the pages of the *Times*. The problem, he said, was that the word fitted all too well with the general tone of the media coverage of medicine – which could be summed up in the phrase “miracles about to be achieved”. Reporting on health care innovations had, like everything else, to be fitted into the “breakthrough mould” – and this had a powerful influence on what was selected for coverage. New drugs or vaccines or developments such as gene therapy fitted the breakthrough category; equally valuable procedural changes (better training and monitoring and the like) did not, and were consequently less likely to receive coverage. In short, while the media do

focus on innovation, they tend to report a very narrow selection of what's actually happening.

He didn't think this was the ideal way of reporting the world; but it was the model which, for good or ill, currently underpinned the choice of material and the manner in which it was handled.

Ben Goldacre - who takes a more jaundiced view of the media than Nigel Hawkes - began by commenting that the media were vulnerable to PR and like simple stories, human stories, stories about pills. An academic had calculated that as much as 80 per cent of the content of newspapers was derived from PR material or press agencies – an inevitable consequence of the radical reduction in reporting staff that virtually all newspapers had suffered in recent decades. For obvious reasons, press releases - even from reputable bodies like universities - do not draw attention to the weaknesses or limitations of the work described; they're issued to give it a positive gloss. These factors conspire to generate an unbalanced view of what is happening in medicine and medical research. He agreed with Nigel Hawkes that minor but often incremental and so ultimately important procedural rather than technical advances were the least likely to receive attention.

He offered one particularly telling example of the selectivity of the media: a research paper published in the *British Medical Journal* on an effective intervention to boost the performance and behaviour of schoolchildren. It was accompanied by an analysis giving evidence of its cost-effectiveness. But the research was ignored because the intervention relied on a change in parenting: not a topic that generally arouses media enthusiasm. This was in sharp contrast to the handling, around the same time, of another story about childhood performance and behaviour. This featured the use of pills containing fish oil. Although there was little or no hard evidence to back the claims, they were widely reported. This story had fitted neatly into the media mould; the other had not.

In the question period that followed the presentations we began by asking how to overcome media misinformation. It appeared there was no simple remedy beyond knowing how newspapers work, developing an appropriate degree of scepticism and, in the case of the internet, seeking out trusted sources. Asked to advise NICE on its PR, Nigel Hawkes pointed out that one of the organisation's problems is that it's not always saying things that people want to hear, and there's not much that can be done about that. He thought that most of the time NICE was doing quite a good job. Ben Goldacre added that it was often easier to criticise NICE

than to criticise the drug companies; the latter usually had a simpler case to make than NICE.

Journalists as a group were criticised by one of our members for not being more supportive of NICE. Nigel Hawkes admitted that the media tended to take the side of the patients, and that this was not always entirely fair. Ben Goldacre commented that news media are not really the ideal place to cover evidence about issues concerning good and bad health because the truth usually takes time to emerge, and only does so following the accumulation and analysis of much data. This isn't the stuff of newspapers which are generally more concerned with the here and now. In this respect he found internet sources more valuable.

Other topics discussed included the different perspectives of journalists as opposed to their readers, the confusion that people feel on reading what appear to be contradictory advice on matters such as diet, how they should respond (or perhaps not respond) to such reports, the way that companies sometimes try to use media stories to boost their share price, the pros and cons of press regulation, the integrity of journalists and the conflict between this and boosting circulation, and standards in journalism generally.

Communication: suggestions to NICE

The final session of our second day began with small group discussions of what we'd heard, and the formulation of some suggestions to NICE on the future of its attempts to communicate with the public on the subject of innovation. At the end of the afternoon we met up to pool our thoughts.

We had a variety of ideas. Some of them appear in the following list, arranged in (approximately) ascending order of extravagance.

- NICE needs to be as transparent as possible.
- It should emphasise good news rather than bad.
- Make the website more user-friendly and interactive, with surveys and pictures, and arrange more links to it. Use Facebook.
- Publicise summaries of NICE decisions more widely.
- Ensure that information on particular topics gets to the relevant clinics or charities.
- Have NICE represented in schools, universities or even at major public events such as music festivals.
- Get the public more involved in the decision-making process rather than presenting them with it at the end.

- Use NHS facilities - blood transfusion centres, GP waiting rooms, A&E etc - to publicise the existence of NICE and its activities.
- Use road shows to publicise NICE and its work.

The final group to report back went further still. NICE, its members suggested, needs to raise the level of peoples' awareness of its existence. This might involve market research to establish exactly what was known to the public of its work, and then a rebranding exercise to encourage a view of its character as innovative, impartial, effective, valuable, warm, friendly, positive and "on our side". At present too many people - if they know anything of NICE - probably just think of it as a "grumpy bunch of people who stop your relatives having the medicines they want". As long as care is taken to ensure that the public don't feel money is being wasted, good PR and even advertising could alter such perceptions.

Mindful that our meeting was taking place around the time that the actress Joanna Lumley had emerged as the champion of the Ghurkhas in their struggle with the Government, one of our members made the ultimate (if not entirely practicable) suggestion: recruit Lumley for NICE!

Final discussion and conclusions

Before the close of the second day of the meeting we had discussed how best to express our views on innovation in a way that would be most helpful to Sir Ian and to NICE. It had been decided that, overnight, our facilitators would draw up a list of the characteristics of health care innovations that might be of value to the NHS. The list itself was to be based on all the characteristics that had been mentioned during the first two days of the meeting. We had decided that we would then proceed by voting to establish an order of priority.

On the morning of the third day, when we saw the final list, we ourselves added one more characteristic, making 26 in all. We also discussed and subsequently voted on two other issues that had arisen out of Sir Ian's opening address on the first day. One of these concerned the appropriate response to an innovative intervention priced above NICE's current cost per QALY gained threshold. The other was the question of who should bear the cost and risks of further R&D that might be required in the development of what appeared to be an expensive but potentially beneficial innovation.

We began with the 26 characteristics of innovation. Each was printed on a sheet of paper attached, in a line, to one of the walls of the meeting room. We had ample time to meander along the line, reflect on the

alternatives, discuss them, and decide for ourselves which five we felt were most important. We indicated our choices by placing a cross on each of the five relevant sheets. The sheets were collected and the crosses counted. The list of characteristics, and the crosses received by each, were as follows.

Question: What makes an innovation valuable to you?

1. A large number of people will benefit from it	20
2. It increases quality of life	17
3. It meets a previously unmet need	15
4. Other innovations may be developed from it in the future	14
5. It increases life expectancy	13
6. It prevents a condition	12
7. It saves your life	9
8. There are few other treatment options	3
9. It cures a condition	3
10. It reduces risk to the patient	3
11. It has one-off cost rather than ongoing costs	3
12. It saves the NHS money	2
13. It has fewer side-effects	1
14. The only other treatments are old	1
15. It allows you to go to work	1
16. It alleviates the burden on a carer	1
17. It is for a condition that is a national priority	1
18. It treats a very severe condition	1
19. It stabilises a condition	1
20. It allows people to be treated in the community	1
21. It avoids people taking medicines	1
22. It avoids waste	1
23. It brings benefits to the UK	1
24. It is more convenient for the patient	0
25. It treats a rare condition	0
26. It is for a small number of people	0

It is apparent from the voting that while there were clear majorities who felt that certain characteristics were more important than others, there was a broad range of views – just as there would be among members of an appraisal committee.

As is often the case in votes of this kind, some of us were slightly surprised by our own decisions! Why, someone wondered, had “It cures a

condition” not attracted more votes? Others pointed out that “It saves a life” had garnered far more support, and that cures aren’t necessarily life saving. A member who had supported the characteristic emphasising that “Other innovations may spring from it in the future” admitted that this was not one of the more heart-tugging items on the list, but said it reflected the importance of investing in the future. Someone else raised the issue of innovations that would benefit only a small number of people. This prompted an (inconclusive) discussion of the nature of fairness, and the justification for allocating disproportionate resources to a small group of people simply because there are not many of them and, in absolute terms, their demands would therefore be limited. Our member responsible for the sole vote for “It gets them back to work” explained that her motivation was more about the individual’s personal dignity than the national interest in boosting the workforce.

To narrow the field we next eliminated all characteristics that had received fewer than three votes, and re-voted (three votes per person) to prioritise the remaining 11.

Question: What makes an innovation valuable to you?

1. It increases quality of life	26
2. Other innovations may be developed from it in the future	11
3. A large number of people will benefit from it	10
4. It saves your life	9
5. It increases life expectancy	9
6. It meets a previously unmet need	6
7. It prevents a condition	5
8. It cures a condition	5
9. There are few other treatment options	1
10. It reduces risk to the patient	0
11. It has a one-off cost rather than ongoing costs	0

Quality of life emerged as the clear favourite, with all but two members granting it one of their votes. To this extent our feeling about innovation is in line with the broader aims of NICE – which explores the extent to which a treatment produces not just more life but better life. We feel that judgements on innovation should follow this same principle.

Although there is a sizable gap between this and the next most favoured characteristic, our second choice reflects a different aspiration. It looks to the future potential of the innovation. This will no doubt be of interest to stakeholders who view NICE appraisals not solely in terms of their

immediate benefit to patients, but also in terms of future benefits – whether to coming generations of patients, or to the future viability of the research and development enterprises that have brought the new drug or device this far.

We next moved on to answer two further questions. The first featured decisions on what to do about innovations that seem to have future potential but are priced above NICE’s current threshold. Following much discussion of the precise wording of the question on which we should vote, we arrived at the following formulation.

Question: If the innovation is more expensive than the cost per QALY gained threshold, and does NOT demonstrate benefits at the moment, but there is a case for believing that it may do so in the future, should NICE:

- (a) Say NO for the present, and ask the developer to fund and carry out more research.
- (b) Say NO for the present, but publicly fund some of the research for the developer on condition that the public gets a return proportionate to its investment.
- (c) Say YES, but only on condition that there is a risk-sharing arrangement, e.g. the NHS only pays in cases where the innovation works
- (d) Say YES - but at how much above NICE’s cost per QALY gained threshold: £30K; £35K; £40K; £45K; £50K; £55K; £60K?

When the vote was taken, opinion divided between the first two options. Fifteen of us voted for option (a) and 13 for (b). There was no backing for either of the other alternatives.

The final question sought more detail on the source of any funds for the further development referred to in the previous question. This too prompted much discussion, principally of the precise identify of the alternative sources. The question as eventually formulated was as follows.

Question: Who should bear the costs and risks of research and development?

- The developer
- The NHS
- The taxpayer
- Charities
- Joint sharing between taxpayer and developer

When we voted, 17 of us felt that the developer of the technology should bear the full cost. The rest, 11 in all, opted for joint sharing between the taxpayer and the developer. As with the previous question, it appears that there was a sharp division between those who believe that the private sector should take full responsibility, and those who would prefer the risks and benefits to be divided.

With the completion of the second tracking questionnaire, our meeting on innovation drew to a close.

Tracker questionnaires

We completed the first questionnaire at the beginning of the meeting, and the second at the end. The full questionnaire and the results can be found in Appendix 2.

Question 1 It comes as no surprise to see that most of us are sympathetic to NICE and its objectives. Rather harder to explain is why, by the end of the meeting, more of us had adopted this view. One plausible explanation is that our new members, having got grips with what NICE is trying to do, had decided that they approved of it! But this must remain a matter of speculation.

Question 2 Did we realise over the course of the meeting that we knew more about science and technology in general terms than we had realised? Or was the meeting itself an education? Again, we can only speculate....

Question 3 Although the question asked for examples drawn from science and technology “in general”, the findings make it clear that most of us were already thinking in biomedical terms. Not surprisingly, this was even more pronounced by the end of the meeting:

Question 4 The meeting seems to have brought about some changes in our thinking on the nature of innovation. Our definitions are just as various, but more nuanced and, in some cases, more exact.

Question 5 If the answers to this question reflect future intentions as well as past experience, our members will be paying less attention to the written and broadcast media, and more to internet searches.

Question 6 The two sets of responses reveal a slight lessening of certainty that some health innovations are more important than others.

But this was nonetheless our predominant view at the beginning of the meeting and at the end.

Questions 7a/b/c There was very little movement in our opinions on the importance of the nature of the illness, the health outcome, or the age at which treatment is given as factors for NICE to consider when making value judgements about health innovations.

Questions 7d/e In the case of two further factors there was some sign of change: By the end of the meeting, more of us accepted that there was a case that NICE should take note of the number of people affected by an innovation when making value judgements, and there was an even more pronounced move in favour of the need to take account of cost.

Appendix 1

Case Study 1: Diabreathe

- There are over two million people with diabetes in the UK.
- Diabetes is a condition where the body cannot properly control the amount of sugar in the blood. It is often because the body is not producing enough of a hormone called insulin.
- Having too much or too little sugar in the blood can make people feel very unwell and can be fatal. In the long term, the lack of proper control of blood sugar can cause damage to the kidneys, eyes and nerves.
- Many people with diabetes need treatment with insulin to control their blood sugar. Insulin used to have to be given by injection because it was broken down by stomach acid.
- Most patients don't have difficulties injecting insulin, but a few people are so averse to injections that they would rather suffer the consequences of diabetes than inject the treatment.



- Diabreathe is a new formulation of insulin that can be inhaled rather than injected.
- Clinical evidence shows that Diabreathe works as well as injected insulin, but that it is more difficult to adjust the dose to get the best possible control of blood sugar.
- The clinical trials were, however,

only a few weeks long and it is not known what the long-term effects are, including whether it could cause damage to the lungs.

- Diabreathe is more expensive than injected insulin. It is estimated that to get an extra QALY Diabreathe would cost £50,000 if given instead of injected insulin (in other words it is £50,000 per additional QALY gained).

Questions for discussion:

1. Is Diabreathe an innovation?
2. In what ways is Diabreathe valuable, and to whom?
3. The NHS budget is fixed, so the more the NHS pays for a new medicine, the less money there is left to provide other medicines and healthcare services. NICE usually considers technologies that cost less than £20,000 per additional QALY gained to be cost effective use of NHS resources. Diabreathe would cost more than twice as much as that.

With this in mind, do you think Diabreathe is worth paying more for than the standard £20,000 per additional QALY? If so, how much more do you think Diabreathe is worth paying for to get an extra QALY compared with injected insulin? 50% more? Twice as much?

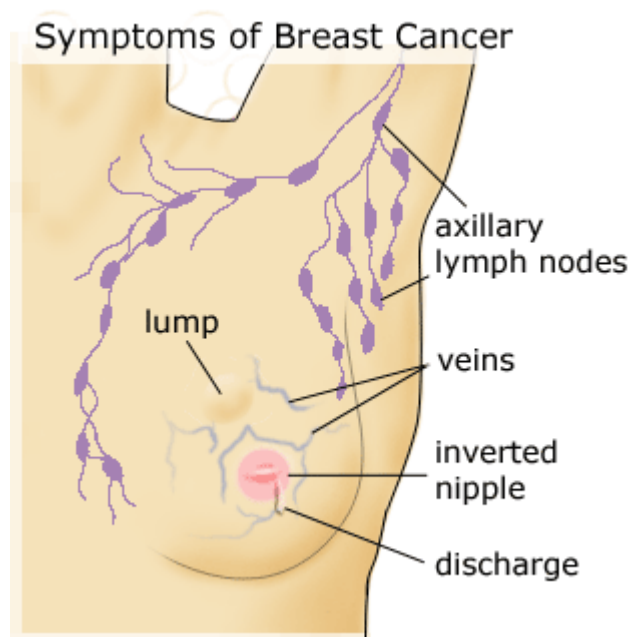
4. When NICE recommends a technology that costs more than £20,000 per QALY gained, it has to give reasons to justify drawing funds away from other medicines and healthcare services. In particular the factors it considers are: how uncertain the evidence is, whether health benefits have been fully captured, and the innovative nature of the technology.

With this in mind, what reasons are there to pay more than £20,000 per QALY gained for Diabreathe?

What are the reasons not to?

Case Study 2: Neverreturn

- The earlier breast cancer is diagnosed, the smaller the tumour is likely to be and the lower the chance that it has spread.
- Women who have early breast cancer can often have surgery which can remove the tumour completely and hopefully cure it.
- They are also given chemotherapy and sometimes radiotherapy to help remove any remaining tumour cells and prevent it returning. Unfortunately, the cancer can come back, sometimes quickly, sometimes after many years.
- Standard chemotherapy cannot be given for a long time because the drugs affect normal cells as well as cancer cells. This causes side effects that make people feel very unwell, such as sore mouth, diarrhoea, hair loss, anaemia and inability to fight infections.
- Neverreturn is a new treatment for preventing the return of breast cancer in women who have been previously treated for early breast cancer. Neverreturn can be taken for long periods of time or until the breast cancer comes back.
- It is given after the usual care of chemotherapy (and sometimes radiotherapy) has been completed, that is, when there would not usually be any further treatment.



- Neverreturn is a new type of drug that targets the cancer cells; because it doesn't affect normal cells it doesn't cause the side effects that chemotherapy does. However, it does seem to cause a rare but serious side effect on the heart. The manufacturers advise that it should not be given to people with heart conditions. However it also could cause this side effect in people with healthy hearts too.
- The evidence about Neverreturn is over a short period of less than 2



years. A study has shown that one year after surgery, fewer women had their cancer come back when they took

Neverreturn (87%), compared with 92% of women who didn't take Neverreturn. In the study, more of the women (6 out of 1000) who took Neverreturn had the rare but serious side effect on their heart than those who didn't have Neverreturn (2 out of 1000).

- It is estimated that using Neverreturn after usual care has been completed would cost £20,000 per QALY gained compared with no further treatment . However it's very unclear whether this number is correct and it could be much higher, perhaps as much as £100,000 per QALY gained. This is because it is not known how much benefit Neverreturn will have in the long run, for example, how long it will delay return of cancer, whether it helps people to survive for a longer time and what long-term side effects it might have.

Question for discussion:

5. Is Neverreturn an innovation?
6. In what ways is Neverreturn valuable, and to whom?

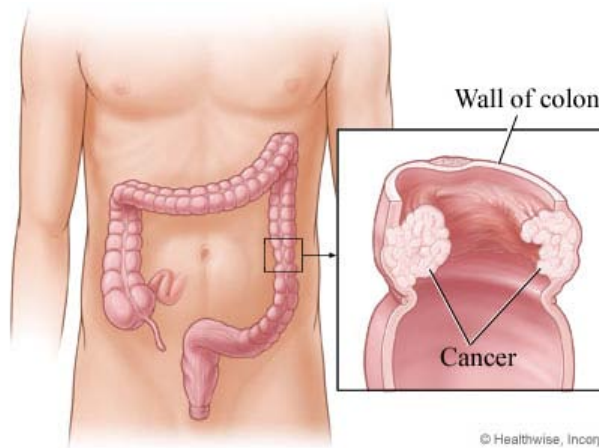
7. The NHS budget is fixed, so the more the NHS pays for a new medicine, the less money there is left to provide other medicines and healthcare services. NICE usually considers technologies that cost less than £20,000 per additional QALY gained to be cost effective use of NHS resources. Neverturn could be within that range, however the evidence is very uncertain and in truth Neverturn could cost as much as £100,000 per QALY gained. When the evidence is uncertain there is more chance that recommending it would draw funds away from medicines and health services that provide more health benefit for the same money. Other factors that NICE considers in situations like this are: whether health benefits have been fully captured, and the innovative nature of the technology.

With this in mind, do you think Neverturn should be recommended by NICE? Why / why not?

Case Study 3: Coprolong

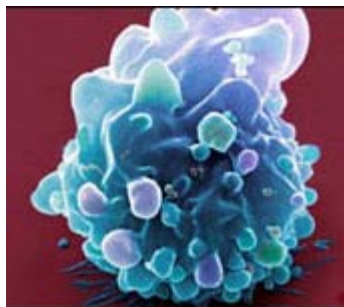
- Coprolong is a new treatment for bowel cancer that has spread to the liver and other parts of the body.

- Bowel cancer is common, with over 30,000 people being diagnosed every year in the UK. The cancer starts in the bowel but it can spread.



- Once it has spread to the liver and other parts of the body it is very difficult to treat – most people who are not treated with chemotherapy live for only 6 months. If they are treated with chemotherapy, this can be increased to 16 months.

- Coprolong works in a new way to slow the spread of cancer that is different to chemotherapy– it cuts off the blood supply to the cancer so it is starved and won't be able to grow, rather than attacking cancer cells themselves.



- Chemotherapy is only given for a few months, but Coprolong is continued for a longer time, until the cancer show signs of getting worse.
- There have been several studies of Coprolong used in addition to chemotherapy. In clinical trials, people who were treated with Coprolong had slower cancer growth and lived longer than those who only had chemotherapy. On average Coprolong extends survival by 4 months longer than chemotherapy alone.

- People receiving Coprolong tend to get more side effects (such as wound-healing problems and high blood pressure) than with chemotherapy alone but doctors and patients say that this is not too much of a problem because the side effects can be managed.
- Adding Coprolong to chemotherapy means there are the additional costs, mainly due to the cost of Coprolong, which costs £2,000 per month. It is estimated that adding Coprolong to chemotherapy would cost between £80,000 and £100,000 per additional QALY gained compared with chemotherapy alone.

Question for discussion:

8. Is Coprolong an innovation?
9. In what ways is Coprolong valuable and to whom?
10. The NHS budget is fixed, so the more the NHS pays for a new medicine, the less money there is left to provide other medicines and healthcare services. NICE usually considers technologies that cost less than £20,000 per additional QALY gained to be cost effective use of NHS resources. Coprolong would cost more than four times as much as that.

With this in mind, how much more do you think Coprolong is worth paying for to get an extra QALY compared with chemotherapy alone? Four times as much? Five times as much?

11. When NICE recommends a technology that costs more than £20,000 per QALY gained, it has to give reasons to justify drawing funds away from other medicines and healthcare services. In particular the factors it considers are: how uncertain the evidence is, whether health benefits have been fully captured, and the innovative nature of the technology.

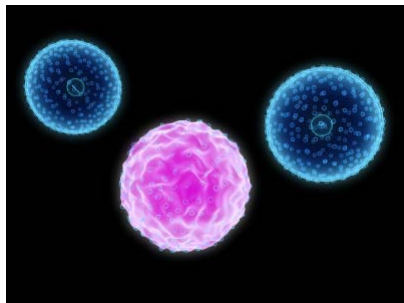
With this in mind, what reasons are there to pay more than £20,000 per QALY gained for Coprolong?

Case Study 4: Lukolife

- Leukaemia is a cancer of the bone marrow and white blood cells which fight infection.
- Because the white blood cells aren't properly formed they're less effective at fighting bacteria and viruses, making the body more vulnerable to infection.



- Lukolife is a new treatment for a fairly rare type of leukaemia which 4,000 people in the UK have. People with this form of leukaemia are very tired, they bleed very easily and for longer periods of time, are at a greater risk of infection and bone fractures, and are expected only to live about 4 years.
- Existing treatments can stop the disease getting worse, which improves quality of life, but no treatments enable people to live longer, and no new treatment options have been developed in the last 10 years.



- Clinical evidence about Lukolife is mainly based on one study in which the disease was held in check for 6 months (on average), while standard treatment held the disease in check for only 3 months (on average). The study was finished early because the study investigators thought the results were so good that the people in the trial not already receiving Lukolife should be offered it.
- The study also showed that Lukolife is likely to extend life, but because it was a short trial it did not show for how long. People who were given Lukolife had more side effects than with standard treatment, one of which caused severe pain in the fingers and toes, and another was high fever with increased risk of very serious infection.

- Lukolife is a lot more expensive than standard treatment – it costs £800 per month rather than £80 per month. It is estimated that Lukolife would cost £35,000 per additional QALY gained if given instead of standard treatment, but this figure is very uncertain and could be as high as £70,000 per QALY gained. This uncertainty is due to the lack of evidence about the long-term effects of Lukolife.

Question for discussion:

12. Is Lukolife an innovation?
13. In what ways is Lukolife valuable?
14. The NHS budget is fixed, so the more the NHS pays for a new medicine, the less money there is left to provide other medicines and healthcare services. NICE usually considers technologies that cost less than £20,000 per additional QALY gained to be cost effective use of NHS resources. Lukolife would cost nearly twice as much as that, and possibly over 3 times as much.

With this in mind, do you think Lukolife is worth paying more for? If so, how much more do you think Lukolife is worth paying for to get an extra QALY compared with standard treatment? Twice as much? Three times as much?

15. When NICE recommends a technology that costs more than £20,000 per QALY gained, it has to give reasons to justify drawing funds away from other medicines and healthcare services. In particular the factors it considers are: how uncertain the evidence is, whether health benefits have been fully captured, and the innovative nature of the technology.

With this in mind, what reasons are there to pay more than £20,000 per QALY gained for Lukolife?

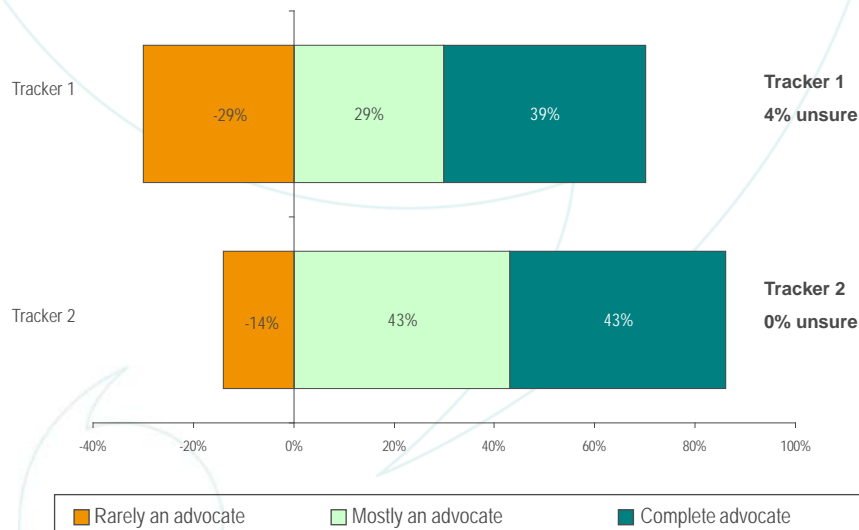
Appendix 2

NICE Council tracker questionnaire

May 2009

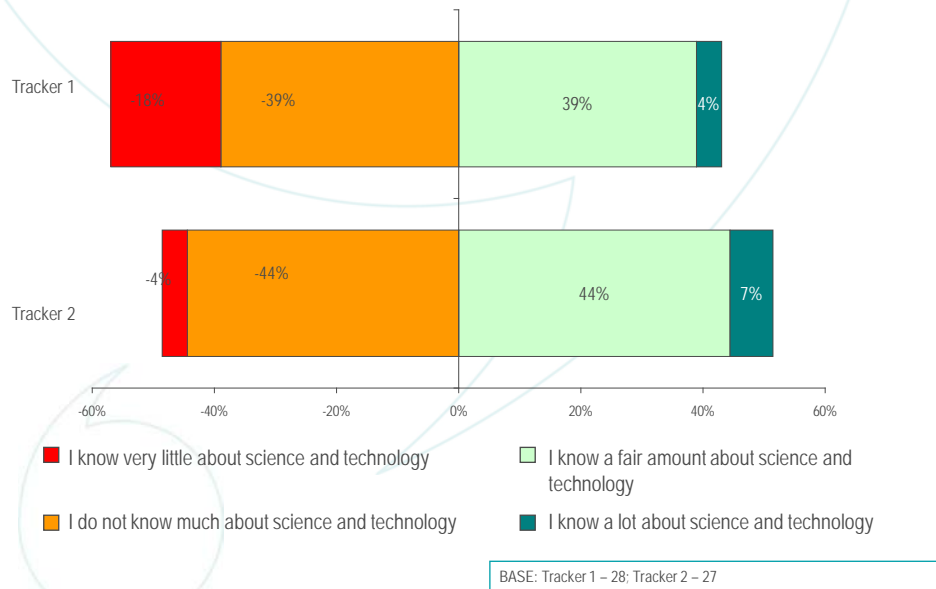
opinion leader

1: Thinking about what you know about NICE's work in general, to what extent would you consider yourself an advocate?

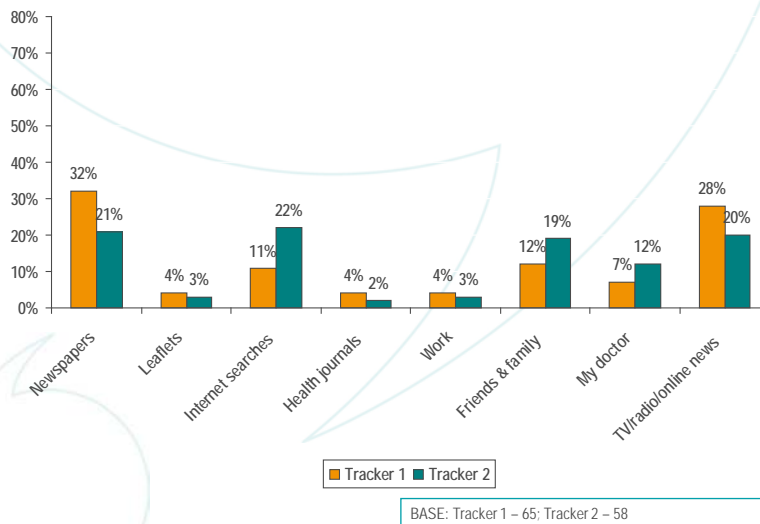


BASE: Tracker 1 – 28; Tracker 2 – 28

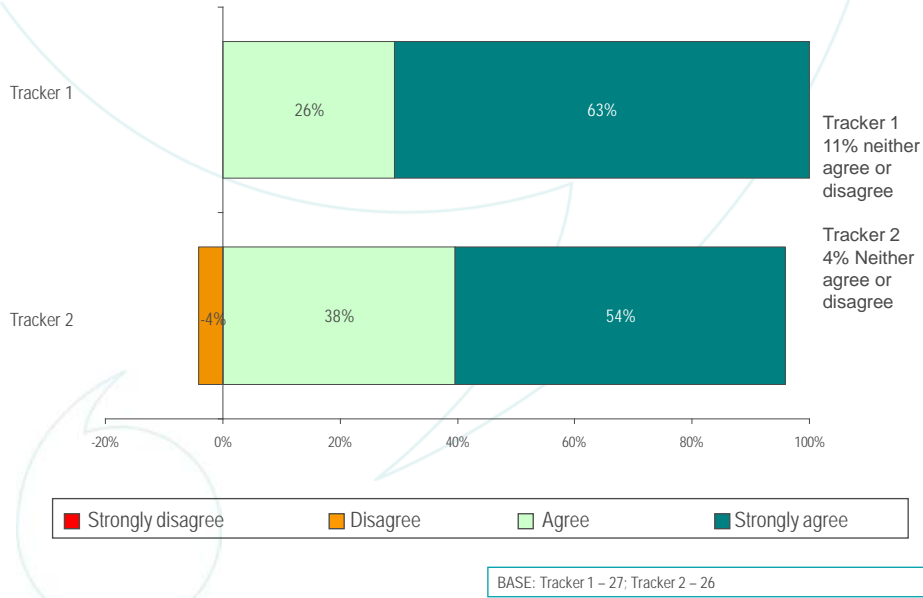
2: How much would you say you know about science and technology in general?



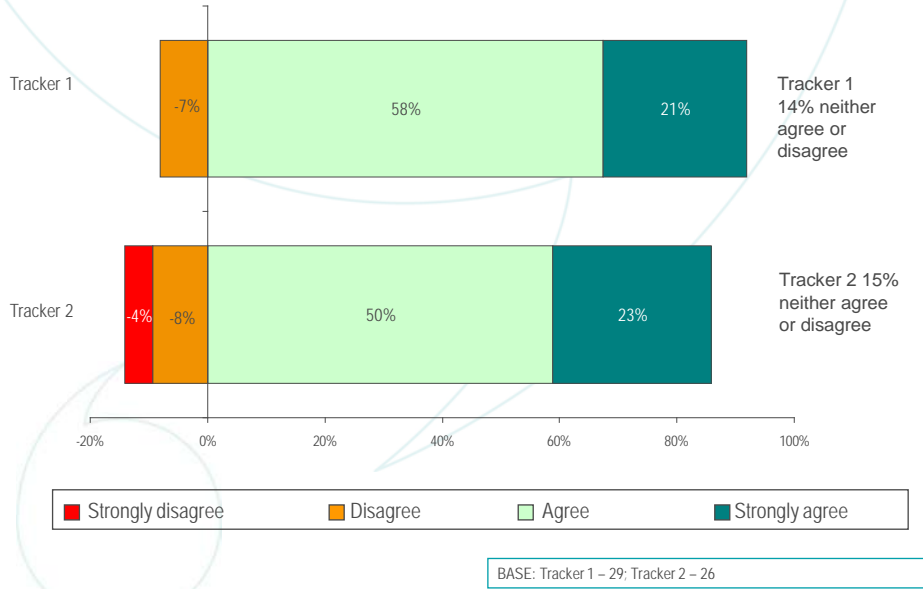
5: How do you hear about innovations in health?



6: Some health innovations are more important/valuable than others.

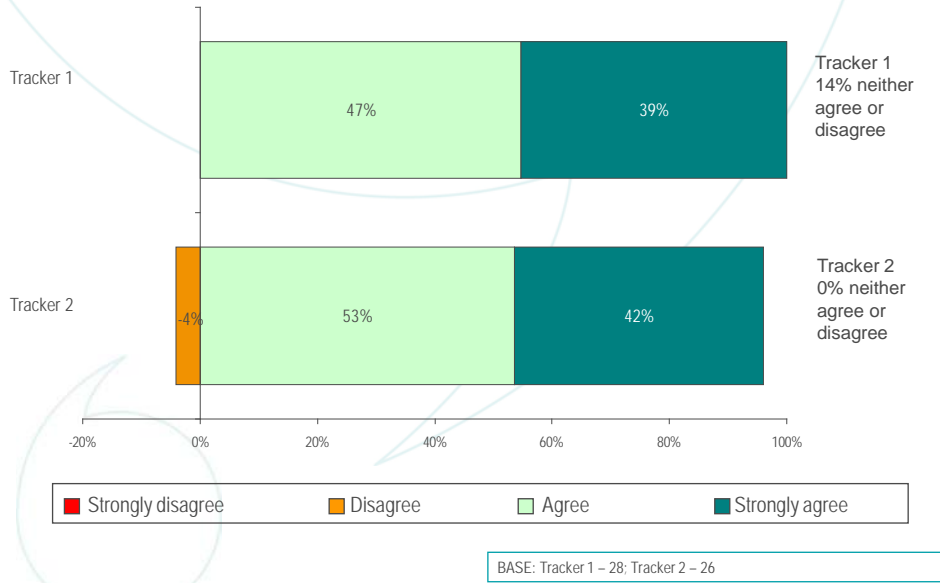


**7a: The factors for NICE to consider when making value judgments about health innovations are:
The nature of the illness e.g. whether acute cancer or long-term asthma**



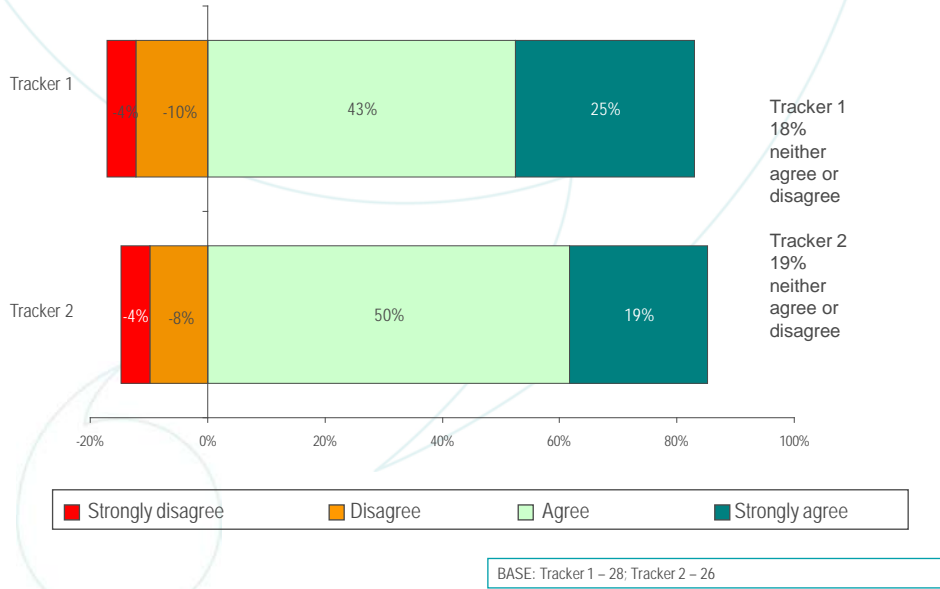
7b: The factors for NICE to consider when making value judgments about health innovations are:

The health outcome e.g. life-saving, life-prolonging, improving quality of life.

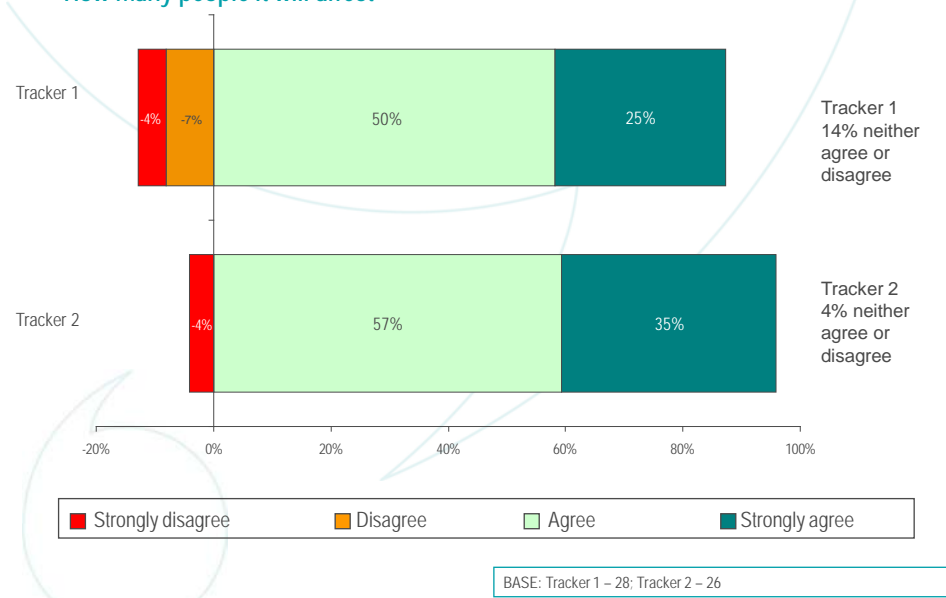


7c: The factors for NICE to consider when making value judgments about health innovations are:

The stage of treatment e.g. for young people, at the end of life.



7d: The factors for NICE to consider when making value judgments about health innovations are:
How many people it will affect



7e: The factors for NICE to consider when making value judgments about health innovations are:
How much it costs

