

Comparative Medicine

Should the concept be introduced into US Medical law?

I am indebted to Kate Spall of the Pamela Northcott Fund for suggesting I might like to make this journey.

I would like to thank the Kidney Cancer Association for inviting me to speak to this conference and Bill Bro and others for the foresight in addressing this topical issue.

Changes to legislation on healthcare can have a massive effect. This is both immediate and perhaps many years later on in the process when the original changes have gathered around them a momentum which carries the original decision further and in directions it never thought it might go. If you change a system, I think you need to get it right at the outset. This is an "Acorn" moment in the life of what might be termed "Socialized medicine". The adoption of the wrong legislative role model for developing systems of healthcare can end up hindering rather than helping patients in certain circumstances.

I am from the UK. I was born in 1959 when the NHS had been running for over 10 years. I have benefitted from it enormously. My family members have benefitted from it. We would not have had the means to provide healthcare for ourselves in the ways in which we have benefitted. However, it is also true, that we have family members who have suffered under it as it is now operated. Our family is not alone in having these problems.

This needless suffering comes about because of the way the NHS is poorly administered and the "woolly thinking" of those at the top in charge of governing and administering the NHS. It is poorly managed. It is an organisation which directly employs over 1.3 million people and indirectly around 400000 more. It is the single largest civilian employer in the world. It should have top level management with clear goals and clear structures as to how to deliver what is needed.

This weekend in January 2009 a pensioner kidney cancer patient in Swansea Wales was taken into hospital as she awaited the outcome of a judicial High Court review of her Local Health Boards decision to refuse Sutent in her case on the basis that it was not "cost effective". She is currently undergoing a complete blood transfusion. The money she had has now dwindled to nothing as she became so desperate she has paid privately for Sutent.

Last Christmas 2008, Mrs. Elke Williams of Oxfordshire a kidney cancer patient, died whilst trying for 6 months to overturn the decision of her Primary Care Trust ("PCT") which refused her Sutent, despite her being unable to take Interferon. The wait proved too long. The PCT relied for the basis of its refusal on Interim NICE guidance in September 2008 which was to not recommend Sutent as it was deemed not cost effective.

Mr. Robert Derrick was refused Sutent 6 months ago but took Sutent privately and showed it caused tumour reduction. He won his appeal this Christmas 2008 – but only after 6 months of fighting Gloucestershire PCT and threatening judicial review. You have his appeal papers and can see how he was forced to deal with a weight of bureaucratic papers and processes. He had at one point three consultants involved in pleading his case.

Pamela Northcott sought Nexovar and her daughter Kate Spall had to fight the refusal to grant that drug by invading the NHS meeting which was going to refuse her once again. Kate Spall has continued to fight for other patients and other conditions after the untimely death of her mother.

A lot of people are not happy with the nonsensical system which has been put in place to make poor administrative decisions.

Unbelievably, in the UK when a clinician recommends the best treatment in a particular patient, no matter how senior a figure, the decision on funding at a local level is taken out of the clinicians hands and put in the hands of people who are often not qualified to make the decision.

The local decision makers are guided by the recommendations of a body which advises the government known as the National Institute for Health and Clinical Excellence ("NICE").

When NICE says "no", the local decision makers have to follow that guidance. This is known as NICE "negative" guidance.

Exceptional patients.

Because of NICE negative guidance, many patients have begun applying since 2006 to be treated as exceptional cases.

Between 3000 and 7000 per year – no one really knows the true figure – patients with orphan or ultra orphan cancers apply for treatments to be given to them in "exceptional circumstances".

There is between 50000 and 70000 patients more who do not apply. They are often too ill, too ignorant of their rights, too tired, frightened and demoralised to apply.

Patients question the need to have the "double whammy" of the bad news on their condition and then a refusal of the treatment they need. They resent having to apply to be considered "exceptional". After all, are not we all "exceptional"?

Aims of the NHS.

The stated aim of the NHS (now under the 2006 Act) has been to provide a comprehensive health service to improve the health of patients and that by the improvement of the prevention, diagnosis and treatment of patients.

Unfortunately we seem to be going backward on treatments due to bureaucratic rules and mismanagement.

Is it the money?

In theory, under the National Insurance ("N.I.") scheme in the UK, every working person in the UK has paid into the NHS. Since 1948 9% of income, up to a capped limit, has been taken from wages to pay for a scheme which truly does offer treatment free at the point

of need. In the fourth largest economy in the World, rivalled only by California, surely MONEY cannot be the problem?

There have always been budget limits and constraints. The way they were managed previously was to rely on trained doctors to decide to treat patients until the system every now and then ran out of money. Then there would be a "crisis". Then more money would flow. Clinicians would choose whom to treat. They were the experts.

Then someone suggested we should use money "more wisely". We could not leave it to the Clinicians.

Change for changes sake.

In 1999 the aim was simple – to establish a single national resource to promote what was clinically effective. They called it NICE.

S.I. 1999 No. 220

Functions of the Institute

3. Subject to and in accordance with such directions as the Secretary of State may give(b), the Institute shall perform such functions in connection with the promotion of clinical excellence in the health service as the Secretary of State may direct.

Until NICE came about in 1999 (amended 2002 and 2005), budget constraints were not managed nationally or systematically in the UK.

The then Health Secretary Frank Dobson said he did not know if NICE would work but it was "worth a bloody good go".

I do not think NICE has been properly thought out.

More change.

By 2005 the job description given to NICE changed so that NICE would incorporate Health Development.

The UK government wanted to introduce a system which provided a "clear, consistent, transparent and fair" (Select Committee on Health evidence) way of allocating resources to healthcare.

Instead of that, rationing was introduced through NICE.

The Chairman of NICE in June 2008 in Parliament stated that the regime which he applied was "the right treatment at the right time to the right patient AT THE RIGHT PRICE". He denied to Parliament in the Select Committee meetings that it is "rationing". It is for him a question of allocation of resources.

S.I. 2005 No. 497

Amendment of Article 3 of the principal Order

3. *For Article 3 of the principal Order (functions of the Institute) substitute the following—*

"3. *Subject to and in accordance with such directions as the Secretary of State may give, the Institute shall perform—*

(a) such functions in connection with the promotion of clinical excellence, and the effective use of available resources in the health service,

(b) such functions in connection with the promotion of excellence in public health

provision and promotion and in that connection the effective use of resources

available in the health service and other available public funds,

(c) such other functions as the Secretary of State may direct".

This was the first time the words “available resources” and “effective use” and “health service” had been put together in one sentence.

As a first attempt it was not bad but it allowed too much latitude to those implementing it. It was not precise enough or clear enough or transparent or indeed fair.

NICE introduce “cost effectiveness”.

NICE now operate a system of deciding whether to recommend a treatment or not on the basis of something they term “cost effectiveness”. They try to compare one treatment with another where possible. Many times, direct comparisons are not possible as it would be unethical to have a test with a control arm of no treatment when there is at least some treatment.

They – and not the UK Government – have come up with their own formulary whereby by means of apparent calculation and math, a person life can be evaluated. They say that they can introduce an element of objectivity into this and it is only by adhering to their method that they can evaluate whether someone lives, or someone dies.

They are essentially utilitarian in approach. However, they have not made it plain if they are “Act” utilitarian (each case on its own merits) or “Rule” utilitarian (the same set of rules applied to each situation). In fact, when one analyses their decisions, they appear to switch from one form of utilitarian to the other with ease.

I am unaware of the statutory authority for the methodology they use; “cost effectiveness” or “comparative cost effectiveness”. Perhaps it exists. Perhaps by implication.

They have rejected specifically something known in medical ethics as “rule of rescue”. Ironically on the basis that it is too vague.

They do not adhere to the value of human life and justice approach.

Chosen Mechanics of NICE

NICE have divided their decision making into the money side [Incremental Cost Effectiveness Ratio ("ICER") and Quality Added Life Year ("QALY")] and the social value side. How much of each element is taken into account by NICE is ...

...Up to NICE. Again, it is entirely in their discretion. They, and only they, can move the goal posts. So you can have different committees bringing different approaches to different conditions. The only constant for them is the threshold for ICER.

The main criticism of them is that the money side always takes precedence over the social value side. For new technologies, they set a limit of £30000 in 1997. That should be £42000 now if medical inflation was applied. However Parliament in 2007 instructed NICE to make it £20000 and only £30000 in "exceptional cases".

They use health economists to provide economic models of the comparative costs involved, including the cost essentially in people living longer.

They invite people to submit better alternatives to these mechanics – yet do not say why they do not prefer say the Swedish, Dutch or New Zealand models.

Human Rights have no place in their decision making.

NICE therefore assess certain new treatments to see if they fit their criteria of what is "cost effective" before they approve them. What constitutes "Cost effective" is a matter only for them.

We are travelling in Lilliput and Brobding in our adventures with Gulliver.

Some timely criticism.

NICE publish their decisions. But only "Eventually". They have been criticised by government for taking too long in bringing out decisions. NICE might fairly counter that it is government that sets the resources for NICE.

In cancer care and treatment, by the time some decisions come out from NICE, the real treatment world of best practice or real "Clinical excellence" has moved on.

We all know of course that taking time to take a decision can be an effective rationing process in itself.

For example Bowel cancer. Cetuximab. Last major evidence received August 2005. Final Appraisal Document ("FAD") negative guidance "Cetuximab not cost effective" January 2007. Between August 2005 and January 2007 the results of two major studies came out and if taken into account, made it cost effective. Since then of course, June 2008 ASCO K-RAS testing has come into being to make it even more likely to be cost effective. Merck sought a review by NICE of their own negative guidance. January 2009, NICE are still prevaricating.

So the decisions are far from timely and as a result are not up to date. This undermines their claim to be "excellent" at date of publication. Aiming for excellence "at date of admission of last evidence" is as good as they can get.

Threshold at £20000 to £30000.

NICE openly state that they view the problem as lying with the Pharmaceutical pricing policies.

Some in the Pharma industry see it as government and NICE as playing politics with peoples lives to bring down prices. Already, the UK has obtained the lowest pricing per drug in Europe. Even the Chairman of NICE admits that this is so and that other European countries will quickly catch on to what is happening and adopt their own NICE.

I asked a government accountant representative in August 2008 how much had been saved by NICE using patient lives to put pressure on Pharmaceutical companies pricing. A bit of a loaded question you might think.

"3%" was the answer I straightaway received.

Now there is no way of knowing if that figure is correct, or even said as a joke, but the government this Christmas announced a NICE present to their surrogate decision taker. They increased the NICE role and budget by many tens of millions of £.

They are not a physical Institute. They have some leased and very plush offices in Holborn, London but their meetings and decisions can be held and taken anywhere.

Are they independent?

Not really. Only independent until it embarrasses the government.

For example – Herceptin. The government countermanded NICE negative guidance on this when NICE did not subdivide the patients groups by reason of a genetic test. It took a High Court review to bring it to the attention of the government.

For example – wet macular degeneration when it was more cost effective apparently for people to be allowed to go blind in one eye before deeming them suitable to receive Lucentis.

Shopping around.

The government do not look only to NICE for advice. They also, when it suits them, go to other individuals and bodies. They sometimes have taken individual cases which have gone against the government in court as a basis for telling NICE to change their advice. They have also gone to Government appointed experts such as Sir Mike Richards to provide them with a way to get out of inevitable NICE refusal in conditions where the QALY will exceed the £30000 threshold.

This must be so annoying to those in NICE.

Government is effectively susceptible to lobbying on some conditions and some circumstances.

Lobbying has been used to effect in the pressure brought to bear on how NICE appraise kidney cancer. Effectively, for a certain

section of kidney cancer patients the rules were rewritten part way through the appraisal process. For those terminally ill, less than 2 years expected life, increase in life expectancy by treatment greater than 3 months, where the condition affects less than 7000 in population of UK, the threshold was effectively moved to £80000.

Finally, rare disorders.

NICE do not consider ultra orphan conditions.

Thus Soliris for PNH national commissioning did not go near NICE.

Some orphan conditions are referred to NICE by government.

Enforcement

NICE is not "National" in the sense of the UK. They directly apply only to England for both MTA and Single Technology Assessments ("STA"). There are complicated rules on how and when they apply to Scotland and Wales. Scotland has its own advisory body which takes precedence on single technology assessments. Wales does not support NICE. Northern Ireland is a law unto itself.

NICE decisions feed down to PCT and LHB.

But these local areas are independent and do not always follow NICE guidance.

NICE positive recommendations. Should be followed. But only after 3 months. No direct route for patients to enforce this quickly and simply.

NICE absence of decision – should not be a reason for refusal – but too often is by PCT and LHB.

To sum up.

A national health system can work but can also become top heavy in administration.

Time limits or "sunset clauses" in its operation and management should be considered to prevent ossification and bureaucracy.

A lot of thought, care and time should be taken as to how it will work in practice before embarking upon it. "Less haste more speed".

Those in charge at the top ought to be drawn from upper levels of private healthcare management. They should have a proven track record of delivery. "Poachers turned gamekeepers".

A "one size fits all" NICE model for working out what is cost or comparatively "clinically" effective plainly does not work all of the time for all situations.

If a NICE body is to be set up, the parameters by which it operates should be accountable, be set by government and elected representatives and the decision making models should not be delegated to the body itself.

Local administrative bodies should be strictly limited to implement government policy.