

As PCTs respond to popular local demand, the government must ensure illnesses that affect relatively few people are cared for too

Who will stand up for rare conditions?

A recent documentary on Channel 4, *A Boy Called Alex*, told the extraordinary story of a young man's success in rising above his daily battle with cystic fibrosis to conduct an orchestra through one of classical music's most challenging pieces, Bach's *Magnificat*.

Although the disease Alex has is rare, the cumulative number of people suffering from all rare conditions is considerable. No official figures exist, but we are talking about hundreds of thousands of people. That is why specialist medicine is central to a functioning health service.

As the prime minister said earlier this year, "as the cost of ever more effective technology interventions rises and there is little advance knowledge of upon whom the costs will fall, it is more important than ever to pool the risk and share the cost of those interventions fairly across our whole population".

This is what the NHS is all about: sharing risk fairly across the population and ensuring no one is excluded from treatment because the disease they suffer from is rare.

Nevertheless, there remains a faction in health service management – often in primary care trusts – which goes beyond the sensible step of devolving the majority of the NHS budget to local control, to suggest that the interests



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of local people and specialist medicine are in opposition.

Let's say it would be possible to dispense with the N in the NHS and move to a local model, locally funded. Let's suppose that each local authority area had local health services. Money for this might be raised or topped up through the introduction of a local income tax – as mooted by the Liberal Democrats. Although participation in local elections is typically half that in national elections, one could imagine such hefty tax-raising powers galvanising voter interest. But how would an LHS work in practice – and would its effects be altogether benign?

Certainly health spending would respond better to local demand. An LHS in a part of the country with, say, a high level of diabetes might invest accordingly in better prevention and earlier treatment. Another might prioritise treatment of arthritis or chronic obstructive pulmonary disease. Patient numbers, not least as a proxy for votes, would



count, as would an area's ability to pay. The local taxpayer would also be likely to take a much closer interest in the finances of an LHS. Residents on one side of the road might end up questioning why they were paying considerably more or less for the service than those across the street. It is hard to predict what the impact would be on sentiment towards spending, but LHS managers would surely find themselves subject to rigorous scrutiny.

Unpopular conditions?

So far, so good. But what if more votes were to be had in trimming access to services, maybe because local taxpayers favoured topping up alternative treatments? Might we also find, as with PCTs, that LHSs were too small to do their job well, possibly leading to mergers? And what about patients with rarer, expensive conditions who some LHSs might refuse to fund –

leading others to struggle, as patients migrated to areas that would treat them?

The fact is that, much as we crave them, simple solutions in healthcare seldom exist. We seek utility to maximise the health return on the money we invest. Equally, we are keen that, as far as possible, patients in different parts of the country should enjoy similar access to services and treatments and that, *in extremis*, world class care will be available to those in greatest need.

Although under constant challenge, the consensus remains that the NHS is the best way to maintain some gravitational pull on these centrifugal forces. This is especially important in specialised services and treatments.

The recognition that society has an obligation to people with rare diseases has found expression in the rare diseases work programme of the European Commission and the

'Working for foundation status is like growing up – you learn from personal experience'

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European Union's policy on so-called "orphan" drugs. In essence, policy-makers recognised that the high costs of drug development made the pursuit of treatments for serious conditions with an incidence of less than five in 10,000 people uneconomic: the conditions had orphan status with no one willing to take on the development of orphan drugs. So incentives were introduced to encourage investment in them.

This public policy initiative has delivered new hope for many people, with more than 450 applications for orphan drug status between 2000 and 2005.

Of course, the costs associated with such treatments can be considerable and are multiplied for the very rarest, or ultra-orphan, conditions that affect fewer than one in 50,000 people. The application of health economics rules reaches its limits where these high-cost treatments are concerned. The remit

of the National Institute for Health and Clinical Excellence seeks to address orphan drugs, applying the same cost per quality adjusted life year threshold as elsewhere, with a small degree of latitude for equity.

Orphan and ultra-orphan medicines, however, stretch NICE's model to and beyond breaking point, both because clinical studies can be difficult to conduct for small patient numbers and because costs per quality adjusted life year can be well above NICE's normal maximum. Some argue that the high costs per quality adjusted life year of some certain rare treatments do not reflect a problem with NICE's threshold but merely a failure of nerve in applying it.

It is perverse for one branch of public policy to encourage the development of treatments giving hope to patients only for another to deny patients access to the resulting drugs.

Economics vs politics

At this point, bureaucratic principles come up against what are essentially political judgements about the kind of society in which we wish to live. Little research has been done in the field. As a result, the citizen's council, which NICE ran in November 2004, remains particularly valuable. It brought out some of the complexity of the issues, with 20 out of 27 members concluding that the NHS should vary its assessment of cost-effectiveness to allow expenditure on ultra-orphan drugs where necessary.

The council was also interesting for its near-unanimous view that in the long term we learn wider lessons from treating rare diseases, even if the immediate cost is high and the benefits restricted to a smaller number of people. In particular, the group was reportedly swayed by the way in which gene research concerning cystic fibrosis in the 1980s had not yielded the desired results but had underpinned the development of therapies for heart disease and cancer.

This theme features in Sir David Cooksey's 2006 report on medical research, which recognised the role

of specialised services in realising the early benefits of innovation. Nor is such innovation restricted to pharmaceuticals as it can also embrace surgery, gene therapy and mental health.

In many ways, support for the NHS and criticism of political interference in its affairs are contradictory positions. A taxpayer-funded national system cannot and should not avoid political guidance

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and scrutiny. This does not prevent considerable, thoroughly desirable, devolution, but it acknowledges that an irreducible core service remains for Parliament to determine. Doing this surely includes making social value judgements of the kind thrown up by rare diseases and high-cost treatments.

The key requirement, then, is a structure within the NHS which provides coherent decision making from local to national level. Ministers and Department of Health officials have responded commendably, with the 2006 Carter report on specialised commissioning and the world class commissioning initiative.

The Carter recommendations propose arrangements which promise to be robust, with specialised commissioning groups taking responsibility for the majority of services but with the national commissioning group standing ready to plan and fund those conditions which are rarest and most expensive to treat.

A survey conducted by the Specialised Healthcare Alliance last autumn showed that specialised commissioning groups had made an encouraging start, implementing

Carter recommendations, though much remains to be done. Further development requires first-class commissioning tools, with an updated national definition set complemented by designation procedures for specialist providers which encompass service specifications and standards of care.

Alliance members recognise that whatever the moral impetus to provide treatment for sufferers of rare conditions, it is important to identify the best configuration of providers to do this at the best quality, safety and cost. The DH would seem to have a common interest with the NHS in providing resources to expedite revision of the national definition set and designation criteria.

The Audit Commission at local level also needs to develop an understanding that risk sharing across a range of specialised conditions is in the interests of local people who will otherwise risk being denied access to treatment when the need arises.

The Specialised Healthcare Alliance is keen to explore the tension surrounding funding of high-cost treatments, which may become more prominent as NHS spending growth slows from April.

It seems improbable that absolute criteria can be developed for funding, but the NICE citizens' panel provides a starting point: looking at the severity of a disease and whether treatment will provide health gain, rather than just stabilisation of the condition.

Above all, the phoney distinction between the priorities of local people and specialised medicine needs to be challenged, and we will need to draw on the strengths that only a national health service can deliver. ●

John Murray is director of the Specialised Healthcare Alliance.

Find out more

A Review of UK Health Research Funding, Sir David Cooksey, HM Treasury, 2006
www.hm-treasury.gov.uk/independent_reviews

Review of Commissioning Arrangements for Specialised Services, Sir David Carter, Department of Health, 2006
www.dh.gov.uk