# Speeding up access to new drugs

Plans to cut the time it takes to get innovative treatments into clinical practice in England are finally to be put out to consultation this month. **Nigel Hawkes** covers the key points

**The prime minister promised at the end of last year to give some patients quicker access to new drugs. Was this just an empty aspiration?**
No. A lot of thought has been given to a faster access scheme since David Cooksey recommended it in his review of UK health research funding in December 2006. A working group from industry and government produced a plan by November 2009. It sat on a shelf until resurrected two years later.

**How would it work?**
Medicines that have completed phase III trials (or in exceptional circumstances phase II) and that will treat or prevent life threatening, chronic, or seriously debilitating conditions that lack adequate existing treatments would qualify. Manufacturers would have to apply, and a decision would be promised in 75 days.

**How would that be any quicker than licensing, if phase III trials have already been completed?**
It usually takes a year or more to get licensing approval after a successful phase III trial. The new process is expected to get these medicines to patients a year earlier than otherwise would be the case.

**But doesn't it mean that proper risk assessment will be skimped?**
There's a danger of that. The burden of risk will be shifted towards the doctor and the patient and away from the manufacturer. Good information will be vital; patients will need to be fully informed and give active consent. Legally, the position will be the same as that for any unlicensed medicine, and the working group believes—but cannot guarantee—that primary care trusts or clinical commissioning groups will not be liable should anything go wrong. And it says that if the decision by a doctor to treat a patient was reasonable in all the circumstances and all relevant information was provided, a successful claim for negligence is unlikely.

**If unlicensed medicines can be marketed and sold, what's the point of licensing?**
The medicines under this scheme would be exceptional and few in number, perhaps only one or two a year. The NHS already uses unlicensed or off-label medicines in some


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cases, on the authority of the prescribing doctor (for example, bevacizumab for age related macular degeneration). Patients consulted by the working group were confident that they were competent to make a proper assessment of the risks. Doctors were not so sure.

**What happens when the medicine is licensed?**
The approval will last a year and can be renewed if necessary. When the medicine gets a licence it will become part of the normal process and the National Institute for Health and Clinical Excellence (NICE) will examine its cost effectiveness. Arrangements for continued funding of early access patients will need to be agreed for each medicine in advance.

**So these fast access medicines will bypass NICE?**
Yes. NICE deals with licensed medicines; these are unlicensed, so NICE does not have a role.

**So how can we tell if manufacturers are overcharging?**
We can't. They will set the price. It will be up to commissioning organisations, without NICE input, to decide if it's a price they want to pay.

**Where's the money coming from?**
An important question, the answer to which could yet put a spanner in the works. Since these medicines will not be approved by NICE, there will be no obligation for primary care trusts or clinical commissioning groups to pay for them. There will be no additional money available, unless the Department of Health or the drug companies provide it. Commissioning bodies will have to fund the drugs out of their normal allocations and in hard times may be reluctant to do so. This could lead to "postcode prescribing," as the working group conceded. But it hoped that if the process were applied only to medicines providing "significant clinical benefit in areas of current unmet need" the variation could be minimised.

**Has anybody else attempted a similar scheme?**
Yes, the French Temporary Authorisation for Use (ATU) scheme is quite similar and has operated for 15 years. To qualify for this, the medicine must have no satisfactory alternative and patients cannot access the scheme if they could become part of a clinical trial of the same medicine. The scheme is restricted to hospital specialists, and companies must agree to submit an application for a full licence, usually within a year, of the temporary authorisation being granted. Experience shows that companies usually set high prices, but if the price fixed after market authorisation is lower they can be asked to refund the difference.

**What about surveillance of side effects?**
Applications for faster access would have to include plans for drug surveillance. "Collecting safety data is essential to protecting patients receiving the medicine," the working group concluded and is also an important way of developing a better understanding of the medicine. But the demands should not be so burdensome as to discourage companies from applying; nor should data gathering be seen as a clinical trial.

**What's next?**
The Medicines and Healthcare Products Regulatory Agency plans to launch a consultation on the scheme at the end of March. It will allow 12 weeks for responses.

**Nigel Hawkes** freelance journalist, London, UK
**nigel.hawkes1@btinternet.com**
Competing interests: None declared.
Provenance and peer review: Commissioned; not externally peer reviewed.
Cite this as: *BMJ* 2012;344:e999

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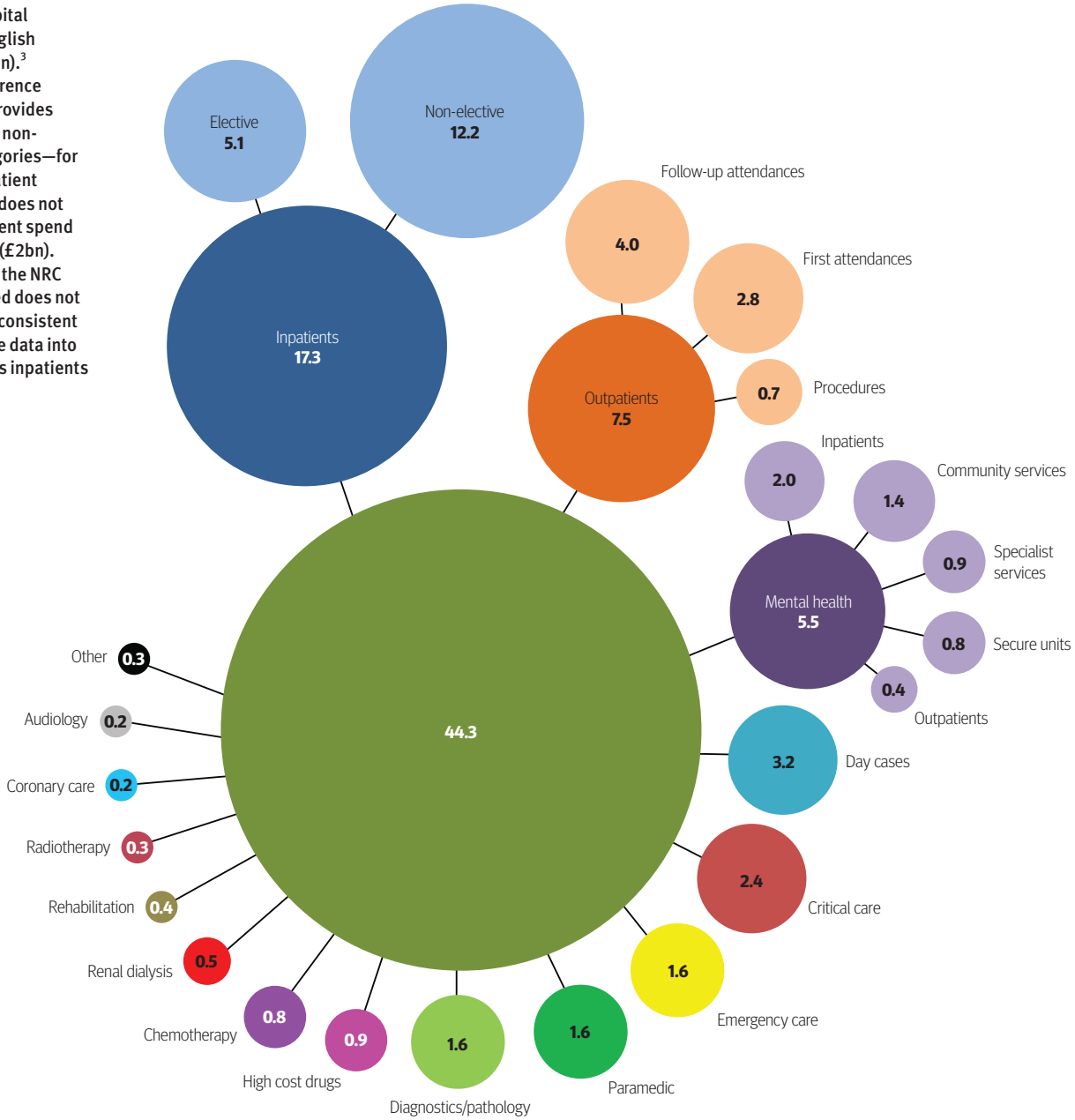
# HOSPITALS: WHAT DO THEY DO AND HOW MUCH DOES IT COST?

**John Appleby** takes a look at where the NHS budget on English hospital services goes

**Spending on hospital services in the English NHS, 2009-10 (£bn).[3] The National Reference Costs database provides spending data for non-overlapping categories—for example, the inpatient bubble (£17.3bn) does not include the inpatient spend for mental health (£2bn). However, the way the NRC data is constructed does not allow completely consistent aggregation of the data into categories such as inpatients and outpatients**



The death of the hospital has been widely predicted—indeed advocated—since (probably) moments after Rahere (courtier, jester, and clergyman) flung open the doors of St Bartholomew's in 1123. But hospitals are survivors: in Barts' case, of the Great Fire of London and the dissolution of the monasteries.[1] And there are good practical reasons for concentrating some types of healthcare service in one place: it's generally a more efficient use of expensive resources, produces better health outcomes, and acts as a physical focus for research.

To survive, hospitals have also had to change. Advances in clinical techniques and better community and home care have led to shorter hospital stays and consequently fewer beds. For example, the number of general and acute beds in England fell by nearly a quarter between 2000-1 and 2011-12, from just under 136 000 to around 105 000 [2]; and, partly as a result of mergers, there are fewer hospital sites and organisational entities.

Now, with tight budgets and efforts to improve productivity, pressure is mounting on hospitals to again rethink their purpose and scope. In the current financial climate hospitals can be increasingly seen as expensive bits of estate doing expensive things to patients that could be better done somewhere else (and more cheaply). But the question is not (and never has been) whether hospitals are needed but, rather, what types of hospital, how big, where located, doing what to whom, and how often?

### But what do hospitals do now?

The activities and costs of hospitals could be described in many ways. One view is provided for English NHS hospitals by detailed costs of activities supplied by the National Reference Costs database (NRC).[3] The database covers activities at a very detailed level—down to around 2500 separate procedures and activities, from hip operations to audiology tests. In 2009-10, around £44.3bn (€53bn; $70bn) of spending (just over 40% of the total NHS budget) was recorded by the NRC database (figure).

Perhaps as expected, most spending on hospital services is devoted to inpatient and outpatient services. Add in day case activities and these services account for two thirds of the NHS spend on hospitals. The highest spending area is emergency care—a total of nearly £14bn for emergency treatment and non-elective admissions.

At a more detailed level, the figures from the NRC database make it clear that, although hospitals are doing more varied things, it's often a relatively small group of procedures and interventions that account for large amounts of spending. The top 10 elective inpatient procedures (out of 1235) by spending account for nearly 20% of total spend in this area. Knee and hip replacements top the list for elective work, but the most commonly recorded "intervention" is "planned procedure not carried out"—a snip at an average cost of £729.

John Appleby is chief economist, King's Fund, London W1G 0AN, UK
j.appleby@kingsfund.org.uk

**Competing interests**: The author has completed the ICJME unified disclosure form at www.icmje.org/coi_disclosure.pdf (available on request from the corresponding author) and declares no support from any organisation for the submitted work; no financial relationships with any organisation that might have an interest in the submitted work in the previous three years; and no other relationships or activities that could appear to have influenced the submitted work.

**Provenance and peer review**: Commissioned; not externally peer reviewed.

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**Cite this as:** *BMJ* 2012;344:e1759

BMJ.COM BLOGS **Deborah Cohen**

## Access to NICE approved drugs

Amid a heavy schedule defending his controversial NHS reforms, Andrew Lansley, health secretary for England, has found the time to form a new expert panel that will contribute to a government report on the NHS constitution.

The constitution will set out in one place what patients can expect from the NHS, including the rights to be treated with respect and humanity, to have access to NICE approved drugs, and to make choices about their NHS care.

There's little to argue with in the requirements that people be treated decently and their choices about their care respected. What's interesting is the specification that patients should have access to NICE approved drugs. It seems logical at first sight—a way around the so-called postcode lottery.

But it's exactly this right that representatives of the drug industry have been lobbying for. Such a stance might, at first, suggest that companies are softening on a previously hostile relationship with NICE, an organisation they see as preventing the rapid uptake of new drugs.

At a meeting of the Association of the British Pharmaceutical Industry (ABPI) last year Ramona Sequeira, managing director of Lilly UK, said patients needed better access to NICE approved drugs.

There is an industry view that UK doctors are conservative in their prescribing patterns and medicines are not prescribed once NICE have given them the green light. Commissioners tend to go for generic drugs. As Sequeira said, the UK spends only 0.9% of its gross domestic product on medicines, compared with 1.2% in the rest of the European Union.

"The NHS is very good at the uptake of old and very cheap technology. The uptake of innovation is a problem in the UK," said Paul Catchpole, value and access director of the ABPI.

NICE approves lots of drugs that aren't always the best or most cost-effective option for certain conditions. In many cases the "new," more expensive version might be the best of the lot—but in many cases the benefit is marginal and not worth the cost.

For example, NICE has given the nod to both analogue and human insulin. However, analogue insulin is far more expensive than human insulin and NICE guidance, produced alongside drug appraisals, says that the analogue should be used only in defined circumstances for patients with type 2 diabetes.

A paper in *BMJ Open* (http://bit.ly/AgEWsz) concluded that given the high marginal cost of analogue insulin, adherence to prescribing guidelines recommending the preferential use of human insulin would have resulted in considerable financial savings. It estimated that if all patients using insulin analogues between 2000 and 2009 had received human insulin instead, the NHS would have saved £625m.

So where would the proposed right to approved drugs leave doctors who follow NICE guidance? They would be vulnerable to concerted campaigns by some industry funded patients groups that argue for the latest treatment, even if the evidence base does not support its preferential use. If patients wanted the more expensive drug—with no obvious clinical benefit—would that render a doctor's prudent decision redundant? These issues need to be clarified before such an ill defined right is committed to legislation.

Deborah Cohen is investigations editor, *BMJ*

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BMJ Group Improving Health Awards In association with MDDUS
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# The unsung heroes of champions

**Zosia Kmietowicz** introduces the sports and exercise medical teams shortlisted for the 2012 BMJ Group award

How difficult can it be to keep a muscle clad gymnast or sprinter motivated and injury free, you might ask. Professional sportspeople are well known for their commitment, hard work, and sheer grit. More difficult perhaps is the task of reassuring and encouraging someone who has survived a heart attack to adopt an exercise programme. But working with sports professionals and nervous patients requires similar qualities—patience, perseverance, and innovative thinking. To be outstanding takes meticulous data collection, a dogged approach to auditing, and a high degree of responsiveness.

The entries for the BMJ Group award for sport and exercise, a category launched to mark the Olympic year, had those qualities in abundance. What distinguished those who made it to the shortlist was their multidisciplinary and reflective approach.

### Leicester Kidney Exercise Team

The eureka moment for the first team shortlisted came when the members realised that although patients having dialysis needed reasonable comfort, they did not need to be stationary—a cycle seat would do just as well as an armchair. Backed with a Cochrane review on the benefits of exercise in people with renal insufficiency, the Leicester Kidney Exercise Team began to carve out a brilliantly simple example of translational medicine in action.

Since September 2011 the team has recruited 30 dialysis patients and 38 pre-dialysis patients into studies to monitor the effect of the exercise programme on markers of cardiovascular risk, appetite regulating hormones, kidney symptoms, muscle wasting, quality of life, and mental wellbeing. Although some patients have been nervous about their physical ability as well as the safety of exercising, the sight of dialysis patients on a specially adapted training bike in the middle of the unit was inspiring. Signing up patients is no longer a problem, and the research ideas just keep coming.

### Cricket Board Science and Medicine Team

The mission of the next shortlisted team is to keep England's four national and 18 county cricketing teams in peak condition. Based at the University of Loughborough the 28 member England and Wales Cricket Board Science and Medicine Team was established in 2007 in response to the high rate of injuries among English cricketers.

An online system of injury surveillance has helped to identify the effect of injury on performance and trends—for example, a rise in facial injuries led to identification of a deficiency in helmet design.

**The travelling medical team (cricket board) who are away more than 250 days per year**

The results of the team's efforts speak for themselves. England's senior men and women are now ranked number one in the international test rankings and hold the Twenty20 world cups. The success is partly down to fewer injuries helping to improve team consistency. Data show that in 2011 an Australian or Indian cricketer was more than twice as likely to miss a game through injury, and that injury would keep them out for 2.5 times longer, than an English player.

Nick Peirce, the board's chief medical officer, says the key to the team's success is rigour. "Aim-plan-do-review every aspect of your work and don't tolerate any precious professional boundaries. Don't be afraid to challenge tradition whether it be medical, coaching, or otherwise. Every sport and environment has unique demands and therefore different solutions," he said.

### Paralympics GB Health Care Team

Unlike most athletes, paralympians have a medical condition that often requires a multidisciplinary approach even before they enter the sports arena. The ParalympicsGB Health Care Team's mission is "to send the best prepared team to London 2012." To do this it aims to install expert medical practitioners into the teams that support paralympic athletes while boosting interest and experience in the discipline.

There were some obstacles to overcome. In previous games practitioners have worked within single sports. For example, a goal ball physiotherapist may have worked solely with the visually impaired athletes who play this sport. Medical record keeping, communication, and team work were also poor. To overcome these problems the team has held a series of information and education days, established forums for exchanging ideas, held team building days, and developed a unified electronic medical records systems across sports. The value of these and other efforts will only become evident after the games.

### British Horseracing Authority

The final shortlisted team, the British Horseracing Authority, is already responsible for the largest longitudinal study on sports related concussion in the world. A year and a half ago it set about replacing a 25 year old paper based system used to record jockeys' injuries. Collaboration with several partners and an investment of £75 000 (€89 000; $118 000) resulted in the launch in January of a nationwide injury management system for Britain's 2000 licensed jockeys that can be used online by 238 doctors at 61 racecourses.

According to Michael Turner, chief medical adviser to the authority, the prospect of unlimited research opportunities was a driving factor of the project. His advice to others? "Sort out a realistic budget early on, recruit a dynamic and enthusiastic project group, keep plugging away until you get the go ahead, never lose sight of the ultimate goal, use every opportunity to tell the decision makers that 'if only we had the new system in place, I could have answered your question instantly.'"

**Zosia Kmietowicz** freelance journalist, London, UK

Cite this as: *BMJ* 2012;344:e1787

**The Sports and Exercise Team of the Year Award is sponsored by Technogym**

For more about the BMJ Group Improving Health Awards 2012 go to http://groupawards.bmj.com