Quackery or quality: the ethicolegal basis for a legislative framework for medical innovation

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ABSTRACT

Innovative therapy is a matter of recent public interest, particularly following Lord Saatchi’s Medical Innovation Bill. The purpose of the Bill is to encourage responsible innovation in medical treatment. We argue for the need to achieve a balance between the risks of medical innovation and patient safety considerations. We make the case for statutory regulation of medical innovation on the basis of responsible innovation, choice and patient-centred care. At the heart of regulation of medical innovation is care delivered by a process which is accountable, transparent and allows full consideration of all relevant matters. This paper proposes a two-stage test (to assess applicability of medical innovation as well as suitability for the choice of intervention to be undertaken). It is suggested that this model would provide safeguards for patients as well as define limits for doctors in the context of innovative therapy. Implementation and application of such therapy must be underpinned by due process and governance oversight, which could be provided through context-specific professional peer review. A combination of these ethicolegal principles would permit responsible medical innovation and maximise benefit in terms of therapy and patient-centred care.

INTRODUCTION

Innovative treatment is a matter high on the public agenda in England,1 as well as elsewhere.2 The vociferous debate over the recent Medical Innovation Bill introduced in the UK by Lord Saatchi (‘the Bill’) is testament to its continuing relevance for contemporary healthcare provision. The Bill has received trenchant criticism from several sectors of society, with suggestions that the proposed legislation is unnecessary and, at its most subversive, might even promote quackery.3 However, this criticism is perhaps too harsh, since innovation has often been the impetus for a range of diagnostic and therapeutic developments in medical science.

The laudable purpose of the Bill, as stated, is to encourage responsible innovation in medical treatment.4 Good clinical care, however, requires that any treatment (including innovative therapy) sustains public trust in that the treatment is clinically justified and subject to appropriate safeguards. There is relatively little ethicolegal discourse (in English jurisdiction) that is relevant to this area.

We believe that statutory regulation would provide a robust basis for medical innovation to be conducted responsibly by practitioners while maintaining the public confidence that patients are not being used for experimentation. We use the Bill to illustrate our vision of the key ethical and legal principles that should underpin a statutory framework, and present this with a focus on responsible innovation and patient-centred care. Legislation would permit doctors to deploy medical innovation optimally for the benefit of patients and within a legitimate assurance framework.

In this paper we proceed as follows: we begin by considering the inadequacy of post hoc scrutiny, which is currently the process typically used for evaluation of innovation; we propose that responsible innovation should be based on a two-stage prospective test followed by ongoing governance assurance through context-specific peer review; we emphasise that consent and compassionate care are central to patient-centred welfare; and finally, we offer our concluding thoughts.

INADEQUACY OF POST HOC SCRUTINY

As yet there is no formally established quality assurance framework for proactive evaluation of innovative therapy in respect of its applicability, or suitability, prior to delivery. The existing pathways tend to be post hoc review through litigation, regulatory or disciplinary processes, or local procedures such as root cause analysis, serious incident review, or clinical audit. These ‘after the event’ mechanisms are unsatisfactory, since they are usually predicated on some form of adverse outcome.

The Bill seeks to encourage ‘responsible innovation’4 on the basis that doctors will not be prima facie negligent for deciding to depart from the existing range of accepted treatment if their decisions are transparent and accountable and all relevant matters have been considered fully.5 However, in addition to potential liability in negligence, a doctor might also be exposed to local disciplinary and professional regulatory action, and ultimately criminal processes. The Bill, in its latest form, requires the innovator to ‘obtain the views of one or more appropriately qualified doctors in relation to the proposed treatment’.6 Doctors must consider patients’ opinions and requests as well as any other reasonably necessary matters such as clinical risk assessment. The requirement for valid consent remains,7 and treatment must be given only in the best interests of individual patients. The appropriateness of the decision to innovate is to be determined by the Bolam test.8

For an action in negligence, the question for the court will be whether the doctor has breached the standard of care expected in law. This is based on the Bolam test6 with the Bolitho proviso that the decision withstands ‘logical analysis’. A ‘reasonableness test’ was postulated in Hunter v Hanley (1955),9 a Scottish case (which pre-dated Bolam) where a doctor undertook a deep intramuscular
injection using a fine bore hypodermic needle (instead of a stronger wider bore one that would have been used normally). Negligence was alleged when the needle broke and was retained in situ. In Hunter, Lord Clyde asserted that mere deviation from normal practice would not necessarily be evidence of negligence and that even substantial deviation might be justified in particular circumstances. He stated further that, in order to establish a doctor’s liability, it would need to be proved that the course adopted by the doctor was one that ‘no professional man of ordinary skill would have taken if he had been acting with ordinary care. If [that] is the test, then it matters nothing how far or how little he deviates from ordinary practice. For the extent of the deviation is not the test’.11

Both these tests could be problematic in the context of innovative practice. The Bolam test may not be practicable because innovation, by definition, may be such that it does not have the support of a responsible body of opinion. The test in Hunter has its weakness in that a ‘reasonable’ deviation from accepted practice based on opinion (albeit from an expert) could be seen as insufficient for patient protection, and allowing doctors unbridled latitude to innovate. There is an urgent need for the formulation of a standard that would permit responsible innovation while at the same time adequately protecting the interests of patients, especially those who might be particularly vulnerable. A standard for responsible innovation that is defined by legislation would carry the force of law, provide clarity for vulnerable. A standard for responsible innovation that is de

RESPONSIBLE INNOVATION
A two-stage test
We propose that the standard for responsible innovation should be based on a two-stage test that requires positive responses at both junctures. First, is innovative therapy applicable to the patient’s unique circumstances, and second, is the intervention or treatment proposed suitable for that particular case?

Applicability
Applicability relates to the decision to adopt innovative recourse. It is the starting point and of paramount importance to all subsequent steps. The level of support required for medical innovation was tested, to a degree, in Simms v Simms (2003) (an application for a declaration that innovative treatment would be in the best interests of a person with incurable neurodegenerative disease).12 Although the treatment proposed had never been tested in humans, Dame Elizabeth Butler-Sloss P was satisfied that the support of a single, albeit well-informed, expert opinion was sufficient to proceed. She said: ‘The Bolam test ought not to be allowed to inhibit medical progress... [If] one waited for the Bolam test to be complied with to its fullest extent, no innovative work... would ever be attempted.’13 In so doing, she recognised the circularity of the argument that the Bolam standard could only be satisfied once treatment was relatively established, yet if innovation was not permitted, it would be impossible to obtain that level of support.

Innovative therapeutic approaches may be considered for two categories of patients: (a) those for whom all standard therapies have been exhausted, either because they have failed or because they are contraindicated; and (b) those for whom options of some standard therapies remain.

(a) The exhaustion of all existing standard therapies would be a clear gateway for the applicability of innovation. In this situation, it is likely that there would be Bolam support that no further standard interventions exist. Under these circumstances the decision to undertake innovative treatment would rest with the (competent) patient and his/her treating physician subject to conditions, which would include risk assessment, consent and the physician’s duty of good medical practice.

(b) Applicability for innovation may be challenging or even problematic in situations where evidence-based therapies have been attempted (but not necessarily all therapies), or where a range of previously tried treatments have been ineffective (short of trying all known treatment). Other equivocal situations include proceeding to a (heavily) risk-laden procedure which may have some proven efficacy. In these situations, inefficacy of previous treatment should be a factor (but not the only factor) to be considered in deciding on innovative recourse. Inefficiency of previous treatment or potential risk of a proposed intervention would need to be given due weight as appropriate. In these circumstances, we would advocate the Bolam standard as the safeguard for legitimacy of innovation.

Suitability
The second stage of our proposed test pertains to the type of innovative therapy that is chosen. In Simms, the expert opinion supported both the need to innovate and the form of therapy proposed. For any new therapy, there might not be sufficient positive evidence to meet the Bolam standard. In our view, the standard for suitability should be a mandatory de minimis level of evidential support for the proposed intervention. The evidence base in medicine is hierarchical, ranging from the strongest (randomised controlled trials) at the top to less robust evidence at the farthest end of the spectrum. Thus, for innovative therapy, at least some form of evidence, albeit at the less definitive end of the scale, should be available—for example, observational studies, case reports or perhaps experiential evidence. The minimal level of evidential support may need to be set depending on the particular facts of each case. For example, if sole reliance is placed on non-human data, then more exacting evidence may be required compared with a situation where reliance is placed on data from human studies.

The concept of suitability has been addressed partly by Clause 1(3) of the Bill through the requirement to assess relevant factors that support the doctor’s decision. We suggest that this assessment could be enhanced further by explicit consideration of any relevant available evidential support for the effectiveness and safety of the treatment proposed, or if this is not forthcoming, then at least scientific support for its concept and rationale. The suitability stage, by extension, should cover the delivery of the chosen intervention.

Delivery of innovative treatment, as with orthodox treatment, necessarily requires all due care and attention. If a new drug or intervention was to be administered without due care or attention, a doctor should not expect to escape liability on the basis of its novelty. Yet judging ‘due care’ could be problematic in this context. This is not simply a Bolam requirement, because new standards will emerge and evolve as innovation is implemented. Heightened vigilance will be required of clinicians to demonstrate a reasoned basis at each stage of the decision-making process in the way that particular therapy is deployed. There should always be a logical and defensible pathway that can be articulated from beginning to end detailing the ongoing effects in the provision of innovative therapy. This would be of practical value in keeping innovation grounded in the welfare of the patient, as well as for developing a repository of information regarding the intervention that has been deployed.
The Bill requires the innovator to take steps to secure that the decision is made in a way that is accountable and transparent. In a previous iteration, attempts to regulate this process was by requiring the doctor’s responsible officer (RO) to be given advance notification. The rationale behind this provision was unclear, since the RO’s role is principally that of revalidation, albeit with some clinical governance function. If notification of the RO was to provide for some form of regulatory absolution, then this would have been both unrealistic and inappropriate. The function of the regulator (the General Medical Council) is to examine whether doctors are ‘fit to practise’, and it is empowered to do so by statute. Legislation for medical innovation cannot, and should not, grant regulatory immunity for clinicians. It is a positive amendment that the revised Bill has removed the notification requirement. However, it leaves open the process required to maintain accountability and transparency. We suggest that this could be best achieved through governance oversight.

Governance oversight
Governance oversight could be by way of protocols or peer review. Therapeutic protocols are based on what is already established regarding specific treatment regimens; research protocols are based on sponsorship assurances. The mismatch between treatment or research and innovation means that neither of these protocols can adequately apply to innovation.

Consequently, peer review is likely to be the optimal approach in terms of governance arrangements for innovation.

A range of models for professional peer review exist. In New Zealand, for example, the operational standard for ethics committees provides guidance on ethical standards for committees. The recommendations require that innovative practice is subject to the same systematic evaluation. In the UK, the Final Report of the Bristol Royal Infirmary Inquiry recommended that clinicians who undertake untried and innovative invasive clinical procedures should first satisfy local research ethics committees, which should then maintain some level of oversight. Various hospitals in the USA, such as the University of Pittsburgh Medical Centre and Boston Children’s Hospital, have created specific review processes to systematically evaluate innovative practice.

The extent to which review processes such as these could provide genuine safeguards is uncertain. Innovative therapy, in similar vein to standard treatment options, will be accompanied by risk and uncertainty for patients’ unique circumstances and in the context of systems available to manage medical risk. If mechanisms for peer review are intended to genuinely enhance efficacy and patient safety, rather than operate as subversive attempts to discourage, peer review must focus on individual patient situations rather than operate as an extended remit of a research ethics committee. The paradigm for oversight of innovation involves experience, evolving knowledge and interaction between the proposed intervention and the disease process, all within the framework of the individual’s unique and specific needs.

Appropriate peer review can provide added assurance about safeguards for innovation provided that the threshold is set at an appropriate level. A threshold that requires intensive and possibly multilayered oversight might negatively impede innovation. One that is set too low might be ineffective. In setting the threshold, the level of risk involved must be considered together with the degree of deviation from standard practice. A calculus such as this is unlikely to be amenable to a standard algorithm, and evaluation will require case by case consideration buttressed by consultation with relevant experts in the field.

Doctors working in public healthcare organisations or as part of team-based practices may already seek collegiate, or professional, peer review prior to innovating. Peer review incorporates a blend of experience and standards of those who might be accustomed to dealing with new interventions, rather than just peer review focused on current acceptability. A tertiary care centre dealing with ‘last chance’ situations (eg, terminal haematological cancer) may bring a unique perspective of risk awareness and appropriate controls for patient benefit. What is required is that this best practice is incorporated within a structure that meets the needs for monitoring new techniques. We suggest that an optimal model might be one of ‘context-specific peer review’. Considering the many contexts of innovation that are subject to different levels of regulatory control, this governance function might be deployed more responsively by professional guidance. This would provide a measure for the safe application of innovation as well as monitoring for the overall ‘suitability’ of the new intervention.

PATIENT-CENTRED CARE

Consent
Valid consent is a necessary precursor for therapeutic treatment of competent patients and raises particular challenges in the context of innovation. The human instinct to cling to hope together with a dearth of alternative options might mean that duress of circumstances can interfere with the validity of apparent consent. The potential vulnerability of those who seemingly have ‘nothing to lose’ is recognised in international guidance for research participants with incurable and life-limiting conditions. Nevertheless, while circumstances such as these may well affect freedom of choice, it is wrong to conclude that this inevitably compromises autonomous choice, and the possibility of well-intentioned beneficent paternalism needs to be guarded against.

Guidance from the General Medical Council and the Department of Health states that patients must be informed if treatment is experimental before seeking their consent, along with information regarding any standard alternatives. The British Medical Association suggests that patients are informed about why innovative therapy is proposed in their case, the evidence to support its use, and known areas of uncertainty. The doctor’s experience, any alternatives, as well as measures for safety monitoring and support should also be disclosed.

Following the Court of Appeal decision in Pearce v United Bristol Healthcare NHS Trust (1998), it is likely that failure to inform a patient with capacity of the fact that a proposed treatment is innovative would undoubtedly be considered a material risk for the purposes of consent. Innovative therapy is likely to be offered in extreme situations where there is no realistic evidence-based alternative, or perhaps where that alternative has failed. Therefore, in sum, it will be imperative that the doctor engages with the patient and his/her values at a level that accords with his/her personal goals.

Compassionate care
Innovative therapies may be considered for patients who are critically, or terminally, ill and where evidence-based standard therapy has failed to provide a benefit. To what extent should such patients have a ‘right to try’ innovative therapy? In English jurisdiction, on the basis of R (Burke) v General Medical Council [2005], the law is well settled in that no individual can demand and expect to receive medical treatment that is not clinically indicated. This presents an additional challenge in that...
innovative therapy, by its very nature, is an ‘experiment’ and therefore cannot be clinically indicated.

Colorado (in the USA) has recently approved the Right to Try Act, 2014, and similar legislation is likely to be approved in Arizona, Louisiana and Missouri. These statutes are intended to give patients with life-threatening illnesses that have no effective treatment the right to access medications before they have been approved and without enrolling in clinical trials or seeking approval of the US Food and Drug Administration. The legislation provides that patients with terminal illnesses have fundamental rights to attempt to pursue the preservation of their own lives by accessing available investigational drugs, biological products and devices. The Colorado Right to Try Act 2014 seeks to offer patients greater access to investigational pharmaceutical and other products that are (already) controlled by statute. By comparison, the Bill does not address this interface, which could be conceived as a potential weakness. However, both the Bill and the ‘right to try’ legislation share a common theme in that they seek to facilitate intervention on compassionate grounds.

‘Compassionate use’ programmes permit developmental medicinal products to be made available to patients with life-threatening or severe disease in the event that no reasonable alternative exists. The European Clinical Research Infrastructures Network (ECRIN) conducted a comprehensive survey on regulations on compassionate use programmes. A wide disparity was found across different European Member States. For example, in Germany compassionate use programmes for medicinal products were introduced into legislation, whereas in Hungary there is neither regulation nor implementation of such programmes. In the UK, there is some provision (through the Medicines and Healthcare products Regulatory Agency) for ‘expanded access’ of unlicensed medicinal products to meet the needs of some patients. Nevertheless, the requisite ethics committee review prior to its use is not uniformly available nationwide. In our view, an opportunity exists to develop the Bill in order to address this lacuna for the use of unlicensed products on a special needs basis by means of legislation. This would be a positive step in the advancement of compassionate care.

CONCLUSION
Innovative therapy falls between two highly regulated areas: standard medical treatment and clinical research. The regulatory mechanism of post hoc review is largely unsatisfactory. In the presence of terminal or incurable illness, patients may be vulnerable to a greater or lesser degree and might not be best placed to assess the risks and benefits of potential innovations adequately, particularly if they believe they have nothing left to lose. It has been argued that since the decision-making process is based typically on an individual doctor–patient axis, a clear framework is needed for appropriate regulation.

Innovative therapy includes departures from standard medical therapy, which may lack an evidence base or a demonstrable safety profile. The primary purpose of innovation must be to benefit the individual patient. We hold that legislation of medical innovation would be beneficial. Legislation would provide a legal standard for responsible innovation, define the boundaries of acceptable medical conduct in this area, and provide the basis for legal protection of patients (many of whom could be highly vulnerable) against wrongful exposure to inappropriate therapies (at best) or unwarranted experimentation (at worst). The Bill as it stands, however, requires strengthening. We propose that this should be through developing a two-stage test for responsible innovation, creating mandatory requirements for ongoing oversight accountability, and placing patient-centred care at the heart of the statute with an emphasis on consent and compassion. There is need for fine-tuning between empathetic care, innovation and regulation in order to get the balance right.

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