Department of Health consultation:
'Legislation to encourage medical innovation'

Response from the Academy of Medical Sciences, the Medical Research Council and the Wellcome Trust

30 April 2014

Summary

- We support the broad aim of the Bill in ensuring that patients have access to innovative, safe and effective treatments in a timely manner, but we have significant concerns about its detail and implications.
- We believe there is a need for better understanding of the extent to which practitioners are currently being deterred from medical innovation due to fear of litigation, which is the main premise of the Bill, as well as the best way to address this. We believe that there are other, possibly more relevant, barriers at the structural and organisational level that also need to be addressed to encourage innovation.
- Even with the safeguards provided in the Bill, there may be unintended consequences for patients who could be at risk of receiving treatments for which the evidence base is not well established, including treatments which could prove ineffective or even harmful.
- The lack of provisions for any form of follow-up or data collection to consider the effects of innovative treatments provided under the circumstances laid out in the Bill is of concern.
- There is a degree of disconnect between the Bill and the delivery of healthcare in practice and we question the practicality of implementation if it becomes law, as well as the appropriateness of legislation as a means to encourage innovation.
- We believe the best way to assess the efficacy and safety of innovative treatments is through full and robust research studies, and would prefer to see novel or experimental treatments - especially unlicensed drugs - prescribed in such settings where there are proper arrangements for clinical monitoring and ongoing data collection.
- This initiative may discourage patients and their clinicians from participating in clinical trials if they are aware that treatments can be provided without the necessity to do so.

Introduction

The Academy of Medical Sciences, the Medical Research Council and the Wellcome Trust are pleased to respond to this consultation, and welcome the opportunity it provides to discuss the wider issue of adoption and diffusion of innovation across the healthcare system. As organisations that support research and innovation to improve health, we welcome initiatives to encourage medical innovation. Patients should have access to the best possible treatments, which should be informed by the most innovative and high-quality medical research. Doctors should, in turn, be able to draw on the most effective and safe treatments and to innovate responsibly in the best interests of their patients without fear of litigation. We consider that the current barriers to this

merit careful consideration to ensure that changes to the statutory framework support this end without unintended adverse consequences.

However, we have concerns about the detail within the Bill and its implications. Our response predominantly concentrates on the implications of the Bill for research.

Question 1: Do you have experience or evidence to suggest that the possibility of litigation sometimes deters doctors from innovation?

No substantial recorded evidence has been raised with us to suggest that doctors are being deterred from the use of innovative treatments or procedures owing to fear of litigation, though we are aware that there are anecdotal cases. Doctors can, and do, prescribe unlicensed medicinal products now using the 'named patient' provisions of Section 9 of the Medicines Act 1968[1] with the consent of the patient and with some evidence that the treatment has a rationale. We acknowledge the rising number of litigation claims, but the consultation document does not provide evidence about the number of claims relating to 'standard' negligence rather than use of non-standard or innovative treatment. This distinction would help to indicate whether the risk of litigation against doctors providing non-standard treatment is real or perceived. If few such claims relating to non-standard treatment exist, it would be helpful to publicise this to allay concerns that clinicians, as well as NHS Trusts, might have about litigation threats. We believe that there is a need to develop a better understanding and evidence base regarding the extent to which practitioners are being deterred from medical innovation due to fear of litigation, which is the main premise of the Bill. We recognise that medical opinion has a role in driving innovation, and that there may be scope for clarifying the current case law on what constitutes medical negligence - but we believe that a clearer and more substantial evidence base is required to determine how best to do so.

We believe that some of the biggest barriers to innovation are at the structural and organisational level, rather than at the level of the individual. Such barriers include the cost of innovative products and budgetary constraints, bureaucracy, and the lack of financial incentives, clinical engagement and training for the development, adoption and diffusion of innovative approaches and treatments. The above all need to be addressed if we wish to support innovation and we welcome ongoing efforts by NHS England and the National Institute for Health Research in this area.

We have concerns that the Bill may shift the emphasis from the importance of prescribing treatments based on scientific evidence to those based only on medical opinion. As such the Bill has the potential to undermine the work, for instance, of Academic Health Science Centres in generating evidence to support innovative treatments, and that of Academic Health Science Networks and Collaborations for Leadership in Applied Health Research and Care in applying this evidence to support the adoption of new treatments, technologies and practices that have been shown to be safe and efficacious. The Bill may also conflict with the National Institute for Health and Care Excellence's technology appraisal system, which recommends the use of new and existing medicines, medical devices, diagnostic techniques, surgical procedures, and health promotion activities in the NHS.

[1] http://www.legislation.gov.uk/ukpga/1968/67

Question 5: Do you have any comments on the process set out in clause 1(6)-(7)? Are there any provisions that should be removed, changed or added – and if so, why?

We would like further clarification of who the 'Responsible Officer' is, particularly in relation to the private sector. Furthermore, the role of Responsible Officers is currently focussed on revalidation, and it is not clear whether they are equipped for the proposed new role under the Bill. Further thought will be required on the most appropriate individuals, teams or organisations to offer advice to doctors seeking to invoke the provisions in the proposed Bill. We are not aware of existing institutions, including for instance the Academy of Medical Sciences, that are appropriately constituted and resourced to offer such support.

Clause 1(7) acknowledges the importance of discussions between doctors and patients about the best course of treatment and the basis for decisions. The importance of patients' participation in clinical research, which is crucial to building a robust evidence base for new treatments, should also be highlighted during these discussions, where opportunities for participation in relevant studies are available.

Question 7: To reinforce the Bill, are there other things that need to happen to encourage responsible innovation?

The Bill is concerned with medical innovation yet does not itself deal with the conduct of research. We feel that innovation and research are intimately linked since innovation requires an evidence base if it is to be put into practice, which is unachievable using data obtained from a single patient (or a small number of patients).

While we recognise that more can be done to foster a culture of innovation in the NHS, with appropriate recognition, incentives and training for the rapid adoption and diffusion of innovation, we would like to acknowledge again the work already carried out by the National Institute of Health Research and NHS England in this regard. We fully support their ongoing efforts, which we consider necessary to drive innovation within the NHS.

We believe the best way to assess the efficacy and safety of treatments is through full and robust research studies with appropriate collection of data and other evidence, on a rigorous statistical basis and with appropriate ethical approval(s). We also support measures to increase access to innovative medicines where these are based on robust regulatory mechanisms and appropriate evidence review; we welcome the Medicines and Healthcare products Regulatory Agency's recent announcement of the Early Access to Medicines Scheme[2] to provide a rapid approval mechanism for innovative medicines when there is a clear unmet medical need and before phase III trials, as well as the European Medicines Agency's decision to provide 'adaptive licensing' through its pilot project[3]. These two recent initiatives, together with widespread information on the provisions of the 'named patient' prescribing system, should provide a stronger basis for innovation.

[2] http://www.mhra.gov.uk/Howweregulate/Innovation/EarlyaccesstomedicinesschemeEAMS/index.htm
[3] http://www.ema.europa.eu/docs/en_GB/document_library/Press_release/2014/03/WC500163410.pdf

We also support work aimed at streamlining the governance of clinical trials. We welcome the new Health Research Authority Assessment and Approval process[4], which should reduce the delays and duplications in obtaining research permissions and consolidate the NHS research governance pathway.

Question 8: Do you have any comments and suggestions for inclusion in the draft impact assessment and equality analysis?

We are concerned that the Bill does not make provisions for follow-up or data collection. This could lead to some practitioners continuing to provide untested and ineffective (or potentially harmful) treatments to numerous patients. The lack of data collection is also counter to the concept of innovation, which must be based on robust evidence. Furthermore there is no provision for the testing of novel treatments in comparison with a control, as is standard in formal research studies. Without appropriate collection and sharing of results - locally and centrally - it would be impossible for the clinical community to learn from existing and new evidence.

It should be noted that even if a database of results is established, it will be difficult to analyse the information contained within it to inform decision-making. Success of treatment in one patient will not, and should not, provide an evidence base for service improvement for patient benefit as it cannot be generalised.

There also needs to be further consideration of the implication of the Bill on use of innovative devices and procedures. For devices, there is currently little evidence provided on clinical utility and the Bill could make it even harder to obtain this (although the proposed European Regulation on Medical Devices is seeking stronger evidentiary requirements). In surgery, randomised controlled trials are difficult to conduct[5] and innovation through iterative processes is the norm. The Bill, however, may undermine the importance of fostering a culture where outcomes are recorded, and practices are examined and compared continuously.

We have concerns that, in order to ensure compliance with the safeguards included in the Bill, NHS Trusts will introduce a 'tick-box' approach where all the conditions have to be satisfied for everything related to innovation, including research. This may add an additional hurdle to the already lengthy task of setting up and carrying out clinical trials and other studies, and dissuade practitioners from performing such studies. Added to that, we are concerned that the Bill, if it becomes law, might discourage patients from participating in clinical trials if they are aware that treatments can be provided without the necessity to do so. This may unintentionally authorise uncontrolled experimentation and mean that an appropriately informed research evidence base can never be accumulated on novel interventions. This would make decisions about approval and adoption across the NHS very difficult, if not impossible.

We also think it would be important to establish how commissioners would allocate resources for activities outlined in the Bill and how the process described in the Bill takes account of weighing up the value or opportunity cost of treatments.

[4] http://www.hra.nhs.uk/documents/2014/03/press-release-single-health-research-approval-31-3-14-final.pdf
[5] See eg: www.mrc.ac.uk/Utilities/Documentrecord/index.htm?d=MRC002417

Finally, we would like to raise concerns about the possibility of undue pressures placed on individual doctors to test unproven treatments by manufacturers. We believe it would be important to raise awareness of the safeguards that already exist to ensure that this does not take place.

This response was prepared jointly by the Academy of Medical Sciences, the Medical Research Council and the Wellcome Trust.

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