

## **Response to the consultation on the Medical Innovation Bill**

### **About MND and the MND Association**

- i. Few conditions are as devastating as motor neurone disease (MND). It is rapidly progressive in the majority of cases, and is always fatal. People with MND will, in varying sequences and combinations, lose the ability to speak, swallow and use their limbs; the most common cause of death is respiratory failure. Most commonly the individual will remain mentally alert as they become trapped within a failing body, although some experience dementia or cognitive change. There are about 5,000 people living with MND in the UK. Half of people with the disease die within 14 months of diagnosis. There is no cure.
- ii. The MND Association is the only national organisation supporting people affected by MND in England, Wales and Northern Ireland, with approximately 90 volunteer led branches and 3,000 volunteers. The MND Association's vision is of a world free from MND. Until that time we will do everything we can to enable everyone with MND to receive the best care, achieve the highest quality of life possible and to die with dignity.
- iii. Funding and supporting research into MND is one of the core elements of the MND Association's mission. We spend over £2.5million on MND research each year; our current research portfolio consists of projects costing over £8million.

### **Introduction**

- i. As both a funder of research and a patient organisation, the MND Association is keen to see effective treatments for MND developed and made available to people with MND as quickly as possible. We therefore wholeheartedly support the motivating spirit behind these proposals, and it is only after very serious consideration that we have decided that we cannot support the Bill, and must recommend to the Government that it does not legislate as proposed.
- ii. Given the devastating nature of MND, it is entirely understandable that people who have been diagnosed with it are often keen to try new and unproven treatments. As an Association we neither endorse such treatments nor advise people with MND against using them; the decision must always rest with the individual. The Association provides information and support to assist people with MND to make an informed choice.
- iii. Unfortunately it is the case that some people who promote such treatments are misguided, mistaken about their efficacy, or motivated by the prospect of monetary gain without any regard for the safety or wellbeing of people with MND. While there are some potential new treatments for MND in development, there are also numerous unproven treatments that have been developed, and are

marketed, outside orthodox research and clinical channels. We note that the Bill does not specify that the treatments prescribed under its provisions must be in any way new or innovative; it is therefore the case that all of these unproven treatments could be made available via the provisions of the Bill, subject to passing the tests it sets out (which, as we will outline, many could potentially do with ease).

- iv. It is the bedrock of medical ethics that treatments must be both safe and efficacious, and these principles serve well to strike a balance that allows scientifically proven new treatments to be promoted without making it easy to exploit vulnerable people with a serious illness. We fear that the provisions of this Bill could undermine this position, and unintentionally open the door to the exploitation of people with MND.
- v. Even if our fears were to prove unfounded, we must also observe that the Bill would not remedy the problem it is aimed at, for such a problem does not exist: uncertainty around the law or fear of litigation do not, as far as we can see, dissuade doctors from trying new treatments. It is certainly the case that such factors are not at all the reason for the lack of curative treatments for MND.
- vi. There are undoubtedly numerous barriers to the promotion and uptake of innovation within the NHS: in too many parts of the health service, personnel lack research skills, leaders do not appreciate the value of research, and research is not recognised as a route to providing high quality care. Too many parts of the NHS see it as someone else's responsibility, and not theirs. We would welcome initiatives to address these problems, and feel that the Government might more usefully prioritise these than progress these proposals (although we are pleased to note the growing emphasis placed on research by NHS England).
- vii. This response considers the version of the Bill published for consultation. We are aware that significant amendments have been made to the draft Bill during the consultation period, but as they have not been formally published we cannot comment on them. With that caveat, we feel that despite its highly laudable intentions this Bill offers no prospect of benefit but poses a genuine risk of causing harm, and we advise the Government not to proceed with it.

### **Consultation questions**

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

and

**Question 2: Do you have experience or evidence to suggest that there is currently a lack of clarity and certainty about the circumstances in which a doctor can safely innovate without fear of litigation?**

- i. We have no evidence to suggest that that the possibility of litigation, or a lack of clarity and certainty about the circumstances in which a doctor may innovate without fear of litigation, has ever deterred a doctor from deploying an innovative treatment in respect of MND.
- ii. There are many barriers to developing effective treatments for MND and other age-related neurodegenerative diseases, but the most important of these relate to

the sheer complexity of the science involved: understanding ageing and degeneration processes within the body and finding ways to slow them, still less halt or even reverse them, remains a monumental challenge for biomedical research. It must also be understood that MND is not technically a single disease, but an umbrella term for numerous diseases that all involve the body's motor neurones dying; our understanding of these different forms of MND is still developing, which adds further to the challenge of identifying effective therapies. It is certainly not the case that genuinely promising and innovative potential treatments for MND currently exist that could be pressed into use if only the legal framework were clearer and doctors felt able to use them.

- iii. We are therefore unconvinced of the stated rationale for the Bill: barriers to innovation and developing new therapies certainly exist, but they are not related to a fear among doctors of litigation as far as we can ascertain.
- iv. Indeed, we believe that the current legal position offers an appropriate balance between latitude for doctors and safeguards for patients – if anything, it is already tilted somewhat in favour of the former. Currently a doctor may proceed with a treatment if a responsible body of medical opinion – even if that is not a majority body of opinion, and the proposed treatment represents a departure from generally accepted practice – would support it. At the same time, if a patient's death is caused by reckless treatment for which only scant support among colleagues can be found, the doctor responsible will be found negligent; this is an important safeguard for patients who need to know that their doctor is acting responsibly, and we would be concerned at its removal. The law currently offers ample latitude for doctors, over and above the separate regulatory regime for innovation (which could be improved, but is not at issue here), while providing appropriate safeguards for patients.
- v. Furthermore, the evidence presented for change in the consultation paper is unconvincing: for instance, it assumes that doctors are well versed in the case law and that the law itself presents a barrier; it does not investigate the possibility that there may be a cultural problem within the medical profession, where risk aversion arises from a lack of awareness of the significant protection offered by the Bolam and Bolitho tests. We are not convinced that this is in fact a significant barrier, but it is striking that the consultation paper does not consider it.
- vi. The evidence presented of a rise in clinical negligence claims is also susceptible of alternative interpretations. It is not made clear that the claims are related to innovative procedures; indeed, we understand that they relate overwhelmingly to established procedures. If there were a rise in claims relating to innovative procedures, that might be evidence that innovation within the NHS was increasing and would therefore be, on one level, positive news. The consultation paper's interpretation of these figures as evidence of a problem of risk aversion that stifles innovation is not clearly sustainable.

**Question 3: Do you agree with the circumstances in which the Bill applies, as outlined in clause 1(3)? If not, please identify any changes you suggest, and give your reasons for them.**

and

**Question 4: Do you have any comments on the matters listed in clause 1(4)-(5) on which the doctor's decision must be based for it to be responsible? Are there any that should be removed, or changed, or added, and if so why? For example, should the Bill explicitly indicate that the other treatments mentioned in 1(5) (a)-(c) include treatments offered as part of research studies?**

- i. We are very concerned at the proposed clause 1(3), which appears to apply when a doctor wishes to disregard all expert medical opinion (that is, when the doctor knows that his proposed course of action would fail the Bolam test – potentially for entirely sound reasons). We are also concerned that under clause 1(4) the doctor may proceed simply on the basis that the rationale for a proposed treatment might appear plausible.
- ii. MND is a complex disease in which relatively few clinicians in the UK are highly expert. Faced with superficially plausible claims by the promoter of an unproven or experimental treatment, a doctor inexperienced in MND could easily err and come to the view that the treatment might be beneficial, particularly if encouraged by a patient who is understandably anxious to explore every last option, however slight its chances may be, to overcome the devastating effects of MND (as in Vignette A, which presents the scenario of a patient encouraging a doctor to consider what can only be described as a long-shot treatment).
- iii. These clauses together appear to give a signal to doctors that 'anything goes' when faced with a terminal and profoundly disabling illness such as MND. Under 1(5)(b), (c) and (d), doctor and patient together could quite reasonably come to the conclusion that the results of current treatments will be death, at best only slightly delayed, and that there is little to be lost by trying an experimental or unproven therapy, even if its effects also culminate in the patient's death. The doctor may seek the opinion of non-expert colleagues (indeed, according to the footnote in the consultation paper, the opinion of colleagues who are not even doctors), and as already noted may proceed even when there is essentially no feasible expectation of efficacy (on the basis that the outcome would be death either way). While the clause as drafted nominally includes an obligation (1(5)(c)) on the clinician to consider the possibility of adverse effects or undesirable side effects, by definition these will often not be apparent in relatively undeveloped treatments: more phase II/III trials of potential new MND drugs have been halted because of adverse effects or serious side effects than have ever succeeded in proving a new drug to be efficacious, despite the presence in all cases of "plausible reasons why the treatment might be effective."
- iv. In short, these clauses appear to create a charter for irresponsible treatment and experimentation, and the exploitation of potentially vulnerable patients faced with a devastating diagnosis and feeling – rationally or not – that they have nothing to lose (considerations around approaches to risk among people with MND are explored under 'Further Considerations' below). We reiterate that the ultimate decision about whether or not to proceed with an unproven treatment must rest with the individual, but this Bill would reduce both the quality of medical advice potentially available to them and the redress available in the event of their suffering harm as a result of the treatment.

**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?**

- i. These clauses do not appear to offer any safeguard against the inappropriate or exploitative use of unproven treatments. We would expect the great majority of doctors to be able to exercise sound judgement over whether or not an innovative potential treatment might be appropriate. But, as noted above, the involvement of other doctors and the discussions between doctor and patient could easily be undertaken to the satisfaction of the terms of the Bill but without involving any meaningful expertise in the treatment of MND, and this creates significant scope for poor decision-making. Under these provisions, it may be possible for a determined but possibly badly-advised patient to gain access to an unproven treatment simply by touring from doctor to doctor until he or she finds one unwise enough to prescribe the treatment.
- ii. It must be acknowledged that in practice, the ‘perfect storm’ of elements that could lead to an unsafe and unproven treatment being dispensed to a person with MND under the terms of this Bill – an unproven treatment with a superficially plausible rationale, a patient determined to access the treatment and to ‘shop around’ for a doctor to facilitate this, and a doctor who takes reasonable steps under the Bill’s terms but fails to consult any colleague with meaningful expertise on MND – may occur only rarely, but such a scenario certainly appears to be possible. We can, however, foresee other potential problems arising from this Bill, which we explore below.
- iii. We have not attempted to suggest modifications to the Bill to avoid the scenario outlined above: the problems identified stem from the central purpose and structure of the Bill. While it may be possible to amend the Bill to include thorough safeguards, it seems likely that such a regulatory regime would produce so much bureaucracy and scope for regulatory uncertainty as to create new disincentives to innovation and wholly undermine the stated purpose of the legislation. Fundamentally this Bill is about removing safeguards (which its sponsors perceive to be barriers), so we have not attempted to suggest new ones to add to it.

**Further Considerations**

**a) The Bill is likely to have unintended consequences**

- i. As we have set out, there is a clear danger that the Bill could lead to the exploitation of vulnerable people.
- ii. It may also lead to an increase in litigation: if a patient approaches their doctor wishing to be prescribed an unproven treatment and the doctor refuses, could the patient take legal action to attempt to reverse this decision?
- iii. The Bill may also add an extra layer of bureaucracy, or create confusion about the legal situation in the minds of doctors who may already be hazy on the case law, in situations where experimental treatments may legitimately be deployed.

**b) People with MND and attitudes to risk**

- i. It is right to acknowledge that attitudes toward risk may be different among people with terminal and life-shortening illnesses, compared to the general population. It

is important to remember, however, that this is not a homogeneous body of opinion, and that attitudes toward risk will vary significantly among terminally ill people. A more relaxed attitude toward risk does not automatically flow from a diagnosis of a terminal illness.

- ii. In the face of legislation which could open the way for, and perhaps even encourage, people with terminal illnesses to take significant risks with their health, it should be remembered that people who live with such illnesses can achieve a quality of life sufficient to make life worth living. We commissioned research to explore these issues, and the following two quotations from people with MND who took part in the study illustrate the point:

*“The quality of my life now I wouldn’t have accepted twenty years ago, but I do now, so as it gets worse you sort of come to terms with things and you think, “Ah well, I don’t have to go out,” or, “I don’t have to do this,” life’s not too bad.”*

*“From wanting to die because my outlook did appear hopeless, I’m so grateful to be alive. If I had chosen to die I would have missed the best years of my life. I know from experience that life with a terminal illness can be managed with the right care and support.”<sup>1</sup>*

- iii. None of this is to diminish or understate the devastating nature of MND. But it does demonstrate that the quality of life that a person with MND can achieve with appropriate care and support should not be gambled with lightly.
- iv. A fully informed person with MND contemplating an innovative therapy will also be aware that the process of degeneration in their motor neurones began long before they first exhibited symptoms, and that the remaining neurones were able to compensate for the damage for some time; the damage will therefore always be significantly advanced by the time a person is diagnosed and potentially looking at innovative treatment options.
- v. Even bearing all of these things in mind, some people with MND, perhaps many, may feel that it is still worth taking on a substantial amount of risk, and we support their right to make such a decision. But the choice will be a difficult one. The Bill and the consultation paper at times seem to assume that there are many patients willing to take such substantial risks, if only their doctors were allowed to help them; at the very least, it should be kept in mind that this may often not be the case. With fewer safeguards available, in terms both of obligations on doctors to act responsibly and of redress available to patients whose treatment harms them, the framework within which people with MND make these difficult decisions would be substantially degraded by this Bill.

### **c) The Bill poses a risk to legitimate research**

- i. People living with MND often take an active interest in research: indeed, it is consistently identified in surveys of our membership as the thing that our supporters wish us to prioritise above all else, including the provision of care and support. Unsurprisingly therefore, many people with MND closely monitor reports

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<sup>1</sup> [Choices and control when you have a life-shortening illness](#), April 2012

of new developments in the world of MND research, and will be aware, for instance, of successful phase II trials of promising new drugs.

- ii. Currently, many people with MND seek out opportunities to participate in clinical trials and other research, understanding that the benefit of this work will flow to future generations and not, in all likelihood, to themselves. This Bill could create a situation, however, in which people with MND can effectively demand new treatments (or potential treatments, effective or not) for themselves; in this situation, it would arguably not be rational for them to volunteer for a double-blinded, placebo-controlled phase II/III trial, with its attendant 50% chance of being given the placebo rather than the new intervention.
- iii. The significance of the problem must not be understated: for a low prevalence disease whose effects are so disabling, at worst it could eliminate entirely the body of patients available to take part in phase II/III trials. It must also be noted that only one drug to slow the progression of MND has ever been found to be efficacious in a phase III trial, although several dozen more have appeared promising at phase II; so the most likely outcome of patients demanding a new intervention and deserting trials is that no benefit will flow to them as individuals, while the benefit of identifying that an intervention does not work, or is even harmful, (and therefore not wasting further resources on it) will not be realised.

**d) The Bill would be bad law**

- i. At best, this Bill might have zero effect. It may be that our concerns about the exploitation of vulnerable patients and other unintended consequences are not borne out in reality (though if so, this would be more by good fortune than by judgement). Even in this scenario however, the Bill would not remedy the supposed problem it seeks to, because in truth the law is not an obstacle to developing and deploying new treatments in the way described in the consultation paper.
- ii. For this reason alone, the Bill has no place on the statute book. Under the auspices of its 'Red Tape Challenge' the Government has recently conducted an exercise to remove redundant legislation from the statute book; even if clear policy reasons for its removal, such as the above unintended consequences, do not emerge, we would expect any similar future exercise to find the Act (as it would then be) to be redundant and to instigate its repeal. To remain consistent with its stated aversion to unnecessary legislation, the Government should not proceed with the Bill.

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