Evidence-based medicine, clinical guidelines, and the role of patient preferences

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Two major developments when it comes to guiding decision-making in medicine are (i) an increased emphasis on the importance of autonomy and shared medical decision-making and (ii) the rise of the ideal of evidence-based medicine. The former has been building since the 1970s and the latter since the 1990s, so these are no temporary fads. But to what extent are these developments in alignment with each other?

On at least one of the early canonical accounts of evidence-based medicine, developed by epidemiologists at McMaster University, it is an ideal of how medicine is practiced that places good medical practice in the intersection between three domains: research evidence, clinical expertise, and patient preferences (Sackett et al. 1997). An updated version of this model included the patient's clinical state, the clinical setting, and clinical circumstances as a fourth component and broadened the patient-oriented

domain to also include patients' actions, including the extent to which patients will actually follow physician recommendations (Haynes et al. 2002). In either version, the McMaster conception of evidence-based medicine is not, at its heart, a conception of how research evidence should be compiled and weighted, but rather a conception of how research evidence should be integrated into clinical decision-making.

While the physician can bring clinical experience and knowledge of relevant research evidence to the process of shared decision-making, the patient is the main authority on his or her preferences. Although it should certainly be recognized that preferences are often formed when actually having to make a decision, and thus partly shaped by the exact nature of those circumstances (Epstein & Peters 2009), the practice of evidence-based medicine should still, on this kind of conception, facilitate shared decision-making. At the end of the day, it would, however, be unrealistic to expect of every physician to keep up to date with the research, even in his or her own specific field of expertise, and this means that for evidence-based medicine to function in actual practice there is a need for intermediates on which physicians can rely. Here clinical guidelines can play a crucial role. And while clinical guidelines are not inherently tied to evidence-basing, they are by now almost invariably at least advertised as being evidence-based (Guyatt et al. 2008). Accordingly, clinical guidelines will often be an

important intermediary through which evidence-basing potentially enters into the clinical situation. But the guidelines also involve a move from summarizing research to making recommendations – a move that cannot be made without relying, not just on evidence, but also on values. To what extent could this circumscribe the influence of individual patient preferences? In order to discuss this question, we will first have to say something about the structure of decision-making in general and medical decision-making in particular.

Two kinds of decision-making

While the focus in much of what is written about medical decision-making tends to lie on the patient-physician encounter, one important fact about contemporary medicine is that it is overwhelmingly practiced in an institutional context. This is not just about the steady decline of physicians in private practice in favor of employment at larger healthcare units, but also about the way in which health-insurance systems function, the role of government regulations, how questions about responsibility are handled by the legal system, how medical research and compilations of meta-analyses are conducted, and how the medical technology and pharmaceutical industries operate. These factors (and others as well) all come in degrees in terms of the extent to which they shape which possibilities are live options in the patient-physician encounter and which are

not. And while the exact shape many of these factors will take might vary from country to country, the overall trend seems to be clear: towards an increasing institutionalization and division of labor which ensures that individual physicians will, when meeting their individual patients, proceed to an increasing degree on the basis of a vast number of decisions that have always already been taken by others.

In any real-life decision we can distinguish between two main phases in the decision-making process: deciding on the menu and deciding from the menu. Deliberation takes time and effort, and so we need to limit the number of options we consider; we need a limited menu to choose from. What characterizes the items that are on the menu is precisely that they are the alternatives to which we give closer thought and ultimately decide between. There are two things to note here. One is that in everyday life menu-setting is largely unconscious. At any moment there are countless options that are in principle open to us, but we tend only to notice a very limited number of them. The other is that we can go back and forth between these two phases: on closer inspection we might find that there is no good alternative on the menu and then we can try to think critically about the menu again, and consider which items could possibly be added to it. In one-person scenarios this movement back and forth is fairly straightforward, but in multi-person scenarios it might very well be the case that there is a division of labor, and certain people do the main job of

deciding on the menu, while others do the main job of deciding from the menu. It might still be possible for the latter to add options to the menu, but the opportunities to do so will tend to be significantly more limited than in one-person scenarios.

The argument here is that the growing institutionalization of medicine has increasingly separated these two phases of decision-making – deciding on the menu and deciding from the menu. The proliferation of clinical guidelines is an example of menu-setting. Good menu-setting (in any context) reduces complexity and correctly identifies the best options available. Arguably, the value of reducing complexity can even, at least up to a point, justify the options on the menu simply being *good enough* rather than absolutely the best (although it should also be recognized that where the bar of being good enough is set will depend on the context). Menu-setting cannot, however, be accomplished in a reasoned way without guidance from certain values. In the institutional medical context two such values or concerns stand out. Foremost is *cost-effectiveness*. For instance, in the UK, the National Institute for Health and Care Excellence (NICE) manual for developing clinical guidelines, while focusing primarily on procedures for reviewing research evidence, strongly emphasizes the importance of analyzing cost-effectiveness (2014, Chapter 7). The other value is what might be called *stakeholder approval*. From a purely ethical perspective, this may seem to be of little direct importance,

but from an institutional perspective having stakeholder approval, which can be a matter of engaging both with representatives of different medical professions and specializations and with various patient groups, is very important, and it will be difficult to achieve stakeholder approval without involving stakeholders in the process of formulating the guidelines.

What this means, however, is that it will be hard to formulate clinical guidelines without at least partly preempting the role that individual patient preferences and circumstances could potentially have played: certain value-based assessments will already have been made. Up to a point, this is quite reasonable, since especially cost-effectiveness is not just an intrapersonal issue for the individual patient, but an interpersonal one: to the extent that health care is cost-effective, we will be able to provide more health care for more patients. But in potentially moving towards what is starting to look like a utilitarian cost-benefit calculus there is also a risk that patient autonomy will suffer, so a balance needs to be struck here.

Minimizing preemptive paternalism

Preemptive paternalism is here understood as the act of imposing judgments about what are to count as good health outcomes in setting the menu of choices that will then form the starting-point for discussions between individual patients and the physician(s) with whom they interact

in making decisions about which treatment options to pursue. The relevant judgments can be imposed in several different ways, but formulating clinical guidelines on the basis of cost-effectiveness assessments is clearly one of them. If we value patient choice, and if we believe that individual patient preferences are highly relevant in determining what will count as a good, or at least acceptable, health outcome in the individual case, we shall have reason to seek to minimize this kind of preemptive paternalism.

We can distinguish between two kinds of cost-effectiveness assessment. To begin with we have what might be called *fine-grained* analyses, where every treatment option can be precisely ranked in terms of a common metric and where the standard candidate in a healthcare context (and the one embraced in the NICE manual, although not as something that should be applied mechanically) is *cost per QUALY*, i.e., the mean cost for the treatment option divided by the mean number of quality-adjusted life years that it will buy us – a figure that can then be compared with other possible treatment options. But it is also possible to use a *coarse-grained* approach instead, where rankings of health outcomes are constructed in terms of broader categories – e.g., whether two treatment options typically have roughly the same types of health outcome (in which case, if one is more expensive, it probably should not be on the menu) or whether one treatment option is clearly superior to another (it has significant effects that for most patients are likely to

count as good health outcomes, while the other has marginal effects that are unlikely to count as good health outcomes for most patients). This kind of analysis will focus primarily on removing ineffective treatment options from the menu, as compared with the best option(s) and should therefore typically result in a bigger menu, as compared with what tends to come out of fine-grained assessment, and, hence, a potentially larger role for the preferences of the individual patient to play.

Two things should be noted here. One is that the application of coarse-grained cost-effectiveness assessments will not completely remove the element of preemptive paternalism in the making of recommendations; rather, the point here is that opting for such assessments should allow us to minimize the extent to which they are preemptively paternalistic. The other point is that it should be recognized that using fine-grained assessments, and presumably relying on a QUALY framework, does not necessitate a strong narrowing down of choice menus; however fine-grained the analysis, we might still just use it for making more coarse-grained decisions. At an institutional level, it does, however, seem likely that a fine-grained analysis will exert a certain gravitational pull on our decision-making processes. And in the case of formulating clinical guidelines, this would then mean a tendency to narrow down the number of choices that are live options for physicians and patients, and hence the role that can be played by the individual patient's prefer-

ences in determining which treatment option that is the most suitable one. If we value the latter, it would accordingly seem reasonable to use mainly coarse-grained cost-effectiveness assessments in developing clinical guidelines.

References

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