POLICY

Examining patient benefit

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ABSTRACT

Healthcare policy, clinical practice and clinical research all declare patient benefit as their avowed aim. Yet, the conceptual question of what exactly constitutes patient benefit has received much less attention than the practical means of realising it. Currently, three key areas of conceptual unclarity make the achieved, real-world impact hard to quantify and disconnect it from the magnitude of the practical endeavour: (1) the distinction between objective and subjective benefit, (2) the relation between individual and population measures of benefit, and (3) the optimal measurement of benefit in research studies. A philosophical understanding of wellbeing is required to clarify these problems. Adopting a rigorous philosophical framework makes apparent that the differing goals of clinicians, researchers and research funders may make differing conceptions of patient benefit appropriate. A framework is proposed for developing rigour in methods for specifying and measuring patient benefit, and for matching benefit measures to different contexts.

KEYWORDS: patient benefit, translational medicine, bioethics prioritisation

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Healthcare policy, clinical practice and clinical research all declare patient benefit as their avowed aim. Yet the *conceptual* question of what exactly constitutes patient benefit has received much less attention than the *practical* means of realising it. Three key areas of conceptual unclarity – the distinction between objective and subjective benefit, the relation between individual and population measures of benefit, and the optimal measurement of benefit in research studies – make the achieved, real-world impact hard to quantify and disconnect it from the magnitude of the practical endeavour.

The problem is well illustrated by the investigational 'test' so common in clinical medicine: a single or aggregate scalar value indexing some physiological parameter such as forced vital capacity. Does its numerical grounding give it a stronger claim to 'objectivity' than the patient's report? Is a statistically significant reduction of the mean, averaged over a large and heterogeneous population, of any significance – in the real-world sense of the word – to the individual that the population mean so often poorly describes? And does the use of such metrics in the research studies that precede the deployment of any therapy distort what the therapy could conceivably achieve?

It may be that these questions have no general answers, and must be dealt with case-by-case. No one could prefer symptoms to the CD4 count in the management of patients with HIV; conversely, no 'biomarker' of pain could illicitly over-ride a patient's sincere avowal of experiencing severe pain. Sometimes the correct emphasis depends on the timing of consequences projected deeper into the future than any contemporaneous symptom could forebode. And sometimes patient awareness of a clinical test result makes a difference to progress of a disease, relieving or exacerbating symptoms that would have followed a different course in ignorance of it.

It may also be that the patient's health conflicts with a broader notion of wellbeing. One might either risk one's health through sun-bathing or skydiving, for example, or tolerate its degradation through smoking or drinking alcohol for the enjoyment or other value such activities may bring to the individual. Patients with long-term conditions often adhere to treatment only selectively, to allow them better to pursue the projects they care about, or to reduce side effects, or spend the costs of medication on something that they perceive may better improve their wellbeing – believing that this will improve their lives overall even if it is worse for the management of their condition. While some hold more paternalistic views, most clinicians accept that the conception of benefit (and hence of beneficence) applicable to clinical medicine is one that must always respect patient autonomy: the primary goal should be to intervene in a way that is beneficial by the patient's standards, not the clinician's.[1] This avoidance of paternalism is already implicit in approaches such as shared decision making, whereby the patient and clinician work together to make decisions about care. However, shared decision making does not relieve the clinician of the duty to form a view about what is best for the individual patient, with all the complexity that comes with this task.[2]

Patient benefit can be construed and operationalised in many ways. But the notion of making a patient's life better is constitutively dependent on an underlying notion of wellbeing, 'a good life' or best interests, from which any departure can be judged and towards which any improvement can be measured.

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It is natural to seek an objective formulation of these aspects, one that can be grounded in science, and on which reproducible, justifiable decisions can confidently rest. But the real-life fidelity of any assessment need not be indifferent to the chosen approach: it may be that in sacrificing subjectivity we may lose more than we gain in objectivity, and the right compromise must be chosen.

How is such a compromise to be found? Specifically, what intellectual tools must be brought into play? Clearly this is an area where neither medicine nor science alone could provide the answers: we need a philosophical understanding of wellbeing to help us. The consensus emerging in that philosophical literature is that neither extreme of objectivity and subjectivity could conceivably work, and that there is a plurality of valid measures of well-being that may all be useful in some circumstances.[3,4]

The focus of clinical medicine is the individual patient, in response to whose individual complaint the physician is invited to provide a specific answer. But some medical decisions, especially those whose solutions ramify beyond the individual – the management of contagion, for a topical example – operate at the population level, introducing another level of complexity and the possibility of conflict.[5] For example, the more rational use of antibiotics, to reduce spread of antimicrobial resistance, will sometimes place patients at risk of harm.[6] And where the effect of an intervention is not easily discernible at the individual level – as in the prevention of common diseases of complex and individually uncertain causation, such as coronary artery disease – population medicine becomes the norm, for which little guiding intelligence can be extracted from the individual patient alone. In such circumstances the population level tools of epidemiology must find a comparably de-individuated counterpart in ethics: not an easy task.[7]

The usual answer – health economic tools such as quality adjusted life years (QALYs) – mask, rather than resolve, these points. That an intervention provides a benefit averaged across a trial population naturally does not imply a benefit for all, and may conceal a wide heterogeneity of individual responses, including harm. Evidence-based medicine tends to assume that the most rigorous approach to medicine is to base clinical interventions on randomised controlled trial evidence that invariably excludes many subgroups and ignores subgroup variation in those that are included. However, it is becoming increasingly obvious that it is better therapeutically to strive to capture this heterogeneity, and adapt to its inherent structure, targeting interventions not to homogenised populations but to clusters or 'families' of similar patients grouped by a diversity of individuating features.[8] As artificial intelligence increasingly makes such an approach feasible, we need philosophical thinking to provide a commensurately granular presentation of the ethical aspects.

Of course, since all clinical practice is derived from innovation, even if often informal or lost in the mists of time, an examination of patient benefit needs to consider the research from which interventions arise. Here the complexity is amplified by all benefit being definitionally counterfactual, a promised land that might never be reached.

What fundamental principles could we draw from in so complex a conceptual landscape? Here we offer a tentative sketch.

First, we need to acknowledge that there is not, and will not be a single, general notion of patient benefit. Legitimate pluralism does not imply that anything goes; rather, what is required is a more contextual inquiry into where and when given measures of patient benefit are appropriate. This will produce a degree of unity amongst diversity by explaining why paediatrics, palliative care, and public health may justifiably diverge in their conceptions of benefit and how to measure it, and what it would take for these diverse measures to each be rigorous in context.

Second, we need a robust conceptual framework within which the best index of benefit –objective, subjective or both, and which dimensions of wellbeing should be focused on – can be determined for different contexts. Crucially, such a framework must enable a principled comparison of multiple competing models, so that any determination can be justified across the space of available possibilities. Philosophy must suggest the way forward, as there are some conceptual limits to characterising patient benefit, beyond which neither empirical nor conceptual inquiry could conceivably deliver a coherent instrument for indexing benefit. For example, the pursuit of 'physiological' measures of pain, to be used in preference to symptomatic report, misconceives the nature of pain, indeed of psychology in general, and can yield no coherent index. However, the broader project is everyone's: healthcare institutions, patients and citizens more broadly must co-produce, by determining how to specify and measure benefit for the different contexts.[4]

Third, we need a mechanism for quantifying the natural uncertainty of any measure, and the meaningful granularity of its range, so that the response to any given value can be appropriately calibrated. It is not statistical uncertainty we are concerned with here –though, where any measure is quantitative, formal probabilistic models should always be preferred – but the constitutional indeterminacy that is so commonly a feature of the landscape of human experience. To derive a measure is not to conclude the task of its interpretation and contextualisation, but to begin it.

Fourth, co-production of standards for specifying and measuring benefit that are refined over time should itself be a priority for health systems and for translational research. While the NHS does already rigorously operationalise health technology assessment via NICE, it has long been recognised that measures of health-related quality of life such as the EQ-5D (on which NICE bases its QALY measurements) perform poorly for conditions such as dementia or hearing impairments.[9] In response, NICE itself has recently been involved in the development of a new measure of health and wellbeing (EQ-HWB).[10] This is overdue, but should not be a one-off: the NHS, supervised not just by government, but by community and professional organisations across health and social care should have a task of monitoring and implementing agreed practices in conceptualising and measuring patient benefit.

This paper has provided an examination of patient benefit as conceived in healthcare policy, clinical practice and clinical research, highlighting the need for a unified approach, but also a multiplicity of rigorous yet contextually grounded ways of conceptualising and measuring benefit. What patient benefit should mean is not something for clinicians or funders of research alone to decide, but one that should be governed by both conceptual and practical reflection. ■

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