Commentaire

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The modern scientific physician: 5. The useful property of an intervention

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he modern scientific physician is well familiar with 'the scripture' on the putatively meaningful 'properties' of diagnostics, with medical textbooks, for example Harrison's,¹ among the sources. Critically reflective as (s)he inherently is, (s)he may nevertheless have come to the succedaneous view that a diagnostic actually has just one useful property: marginal informativeness — critical to consider in deciding whether to invoke the diagnostic, but irrelevant in interpreting the result it produces. This useful property of a diagnostic contrasts with *effectiveness*, the useful property of an intervention.

That diagnostic versus intervention duality, resulting in the informativeness versus effectiveness duality in the useful properties of medical procedures, is denied in the corresponding monistic idea that a quarter-century ago was adopted and subsequently was propagated — until its recent dismantling — by the Office of Technology Assessment of the US Congress. In effect denying the diagnosis versus prognosis duality that has prevailed in medicine ever since Hippocrates, the Office of Technology Assessment quite cavalierly adopted the view that diagnostics, too, are invoked to change the course of health (for the better); and that therefore they, too, are interventions, with effectiveness their useful property.² In the Office of Technology Assessment's realm of medical "technology assessment" this idea brought diagnostics to the scope of its intervention-centred interests in medical "outcomes research," which the Office of Technology Assessment itself recently recast as "effectiveness research."3 The leadership of American radiology embraced these aberrations from central and ingrained concepts of medicine,4,5 and even the US National Cancer Institute went along with this in quite a spectacular way.⁶

Nevertheless, the modern scientific physician still holds that Roentgen did not provide for inventing an effective intervention for pulmonary tuberculosis; that the advent of its foundation truly was marked by the discovery of streptomycin, by Waksman and his co-workers much later. The use of a diagnostic, (s)he understands, is invoked to enhance knowing about the present, diagnosis — in part for the purpose of thereby knowing about the future, prognosis. An intervention, by contrast, is not invoked for the purpose of any type of knowing but to change the future for the better, through the intended effect of the intervention. Etymologically, intervention is coming in-between — a feature not of diagnostics but of preventives, therapeutics and rehabilitation. (In medicine, an intervention is intended to come between stages of the 'natural history' of health or illness.)

The Office of Technology Assessment also introduced the now quite widely held notion that an important distinction is to be made between effectiveness and *efficacy*. As it most recently again expressed this distinction, effectiveness putatively is a matter of whether an intervention improves health "under ordinary circumstances, in ordinary settings," while efficacy putatively is the counterpart of this "under ideal circumstances."⁷ This distinction-making is not altogether surprising when it is realized that "medical technology" to the Office of Technology Assessment consisted of entities such as "drugs"⁸ instead of suitably defined techniques/algorithms of intervention (involving drugs or whatever agents of intended change) on suitably defined indications. (In scholarly terms, neither drugs nor drug uses are technologies; drug development is.⁹)

I once asked an exceptionally scientific surgeon (John Kirklin) how he thinks about surgeons' variable skills as a determinant of outcome in surgical research toward knowledge to guide practice (under whatever circumstances). His retort was that the so-called "good surgeon" to him is one who uses a good technique, and the so-called "bad surgeon" correspondingly one who uses a bad technique; that what matters is the scientifically necessary specificity on all relevant particulars of the surgical technique used (e.g., pump time in coronary bypass surgery); and that conditionally on these specifics, the surgeon's skill is scientifically a nonissue (and merely a determinant of what variant of the generic technique gets to be used). The same imperative of specificity he naturally would have extended to the intervention's indication. As for effectiveness versus efficacy, there thus was no such distinction in the mind of this genuinely scientific physician.

Science is characterized not only by its generalizations (particularistic to abstract) but also by the distinctions that it makes in these. In particular, any *quantitative* idea about effectiveness must involve the requisite degree of specificity to *subtypes* of both the intervention (degree of adherence to the plan included) and the indication (recipient of the intervention). In these terms the effectiveness versus efficacy distinction indeed does vanish. Consonant with this, the terms "effectiveness" and "efficacy" are synonyms in general English; and so they also were in the mind of the original and highly esteemed advocate of directly practice-relevant effectiveness research on health-care interventions, conducted under the ideal circumstances of randomized trials.¹⁰

Integral to the concept of a medical intervention's effectiveness is, naturally, the course or outcome that the intervention is to change; and to this, too, the Office of Technology Assessment's "outcomes research" interest brought an innovation: the notion that measures of "*health-related quality of life*" need to be included.¹¹ The modern scientific physician, however, likely still prefers the traditional idea that the intended effects of medical interventions are somatic, inclusive of their patient-relevant in-vivo manifestations (in reduced discomfort, deformity and/or dysfunctionality); and that welcome changes in quality of life — psychologic¹² — are secondary to these, matters not really of medicine per se but of the utility — subjective — of the somatic effects to the person at issue. Further, (s)he inclines to take the view that insofar as these quality of life implications of genuinely medical states/events call for, and are subject to, research, they are to be studied as attributes of health states/events themselves, irrespective of the role that any intervention may have had in bringing these about.

Central to the Office of Technology Assessment's concerns with medical interventions was "cost-effectiveness analysis" of these¹³; and central to this, in turn, is taken to be an intervention's effectiveness in terms of its resultant gain in "*quality-adjusted life years*."¹⁴ For this measure, health-related quality of life is defined on a quantitative scale in which "1 corresponds to perfect health and … 0 corresponds to a health state judged equivalent to death."¹⁵ The scientific physician's intellect faces a serious challenge in attempting to apprehend the concept of health-related quality of life that is "equivalent to death," and equally if its zero value actually is taken to characterize the health aspects of life post-mortem!

Puzzling, too, is the related 'cost-effectiveness analysis' idea that an intervention's 'expected' - average - effect on the duration (quality-adjusted) of life — and thus effect on 'life expectancy' (quality-adjusted) — is appropriate to use as the measure of its effectiveness. For, with or without intervention, a person faces a chance duration of life; and only one of the possibilities will become the reality, not their probability-weighted average. If an intervention indeed prolonged the person's actual duration of life by the 'expected' amount, it generally would have minimal utility to him/her; but the chance utility is different, generally much higher. Buying a lottery ticket, or insurance for that matter, is characterized by a negative 'expected' effect on a person's finances; but rational people nevertheless see such choices as financially justifiable for their individual purposes, mindful of what *might* turn out to be their particular luck or lot. If a young scientific physician has an acute illness characterized by 1% case-fatality rate in the absence of an available, fully curative (but perhaps very expensive) intervention, (s)he and the society just the same - attaches a much higher utility to curing that illness than to avoiding a 1% shortening of his/her actual lifespan (conditional on surviving that illness).

The concepts of intervention and its effectiveness, and the terms in which to quantify the latter, may appear to be obvious to a physician, and indeed they should be. But the modern scientific physician actually has a lot to think about in this context, now that the effectiveness of medical interventions has become a concern also to professionals other than physicians. As with ideas intrinsic to medicine itself, (s)he does not simply believe the exogenous ideas, taking them for granted; in the Baconian spirit¹⁶ (s)he weighs and considers them — and even in this now-multidisciplinary context ultimately alone. Dr. Miettinen is with the Department of Epidemiology and Biostatistics and the Department of Medicine, Faculty of Medicine, McGill University, Montreal, Que., and also with the Department of Medicine, Weill Medical College, Cornell University, New York, NY.

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