

Health Advocacy Organizations and Evidence-Based Medicine

Sheila M. Rothman, PhD

STRONG AND INDEPENDENT NOT-FOR-PROFIT ADVOCACY organizations are vital to a democratic society. At their best, they stand apart from the interests of the marketplace and the government, helping to promote diverse public concerns. The scope of their activities is extensive. Some not-for-profit advocacy organizations spearhead campaigns to eliminate discriminatory legislation and improve the life chances of vulnerable groups. Others challenge economic interests to better protect the environment. Still others work to advance civil and human rights.

Health advocacy organizations are part of this sector. They speak for targeted populations affected by a variety of diseases such as epilepsy, breast cancer, mental illness, and autism. They effectively communicate their priorities to legislators, regulators, private and public funders, and the media. Their initiatives often highlight needs that might otherwise be overlooked. However, some health advocacy organizations, as shall be illustrated, are so committed to securing diagnostic and treatment interventions for their targeted populations that they minimize the value of evidence-based medicine (EBM) and comparative effectiveness research. They also ignore the potential effects of their advocacy on health care costs.

Many health advocacy organizations responded to a recent Institute of Medicine survey, the goal of which was to identify criteria for determining the essential health benefits package.¹ They contended that EBM should serve merely as an aid in medical decision making, not as the basis for it. Outcome data, they insist, should not limit patient choice or restrict available services. The Epilepsy Foundation of America, for example, opposed the use of EBM "to broadly establish coverage rules that trump an individual's circumstances and specific needs."² It should be "a tool for practitioners, patients and caregivers not applied across the patient population as a final decision on coverage."² So too, the National Alliance on Mental Illness states that EBM should be "a tool for practitioners, patients and caregivers, not a bright-line decision applied across the board."² The National Coalition of Cancer Survivorship also supports "an individualized approach to

medical necessity, relying on evidence-driven decisions made by patients and physicians."²

Some health advocacy organizations are so committed to promoting patient choice that they insist on unfettered access to branded drugs, regardless of the availability of generic products, and access to still unproven interventions. They oppose policies such as Fail First, which require a trial on less expensive medications before moving to brand name and other costly products; and they resist prior authorization requirements and the establishment of closed formularies on the grounds that they limit physician prerogatives and patient preferences. Mental Health America, for example, opposes these stipulations, stating they "fail to acknowledge that physicians and consumers should make individualized treatment decisions, recognizing the unique and non-interchangeable nature of human beings and psychotropic medications."³

Moreover, some health advocacy organizations urge unlimited access to screening technologies regardless of evidence of efficacy or cost. They frame advocacy for early detection in more universal terms by emphasizing how many individuals are at risk for the disease. Mammography screening is a case in point. The American Cancer Society maintains that since 1 in 8 women have a lifetime risk of developing cancer of the breast, annual mammograms for all women older than 40 years are essential.⁴ This calculation, however, blurs the lines between the harms and benefits of screening a population with a low prevalence of the disease (women aged 40 to 49 years) and screening and surveillance of those with a higher risk (women older than 50 years).

This approach to screening led some health advocacy organizations to oppose the recommendations of United States Preventive Services Task Force (USPSTF) when it recommended against "routine screening mammography in women aged 40 to 49 years." The USPSTF stated that "although false-positive test results, overdiagnosis, and unnecessary earlier treatment are problems for all age groups, false-positive results are more common for women aged 40 to 49 years."⁵

Author Affiliation: Department of Sociomedical Sciences, Mailman School of Public Health, Columbia University, New York, New York.

Corresponding Author: Sheila M. Rothman, PhD, Center for the Study of Society and Medicine, 630 W 168th St, New York, NY 10032 (smr4@columbia.edu).

A number of health advocacy organizations took a markedly different stance. From their perspective, the problem was not too much but too little screening. Their focus was on the potential benefits not the potential risks. For example, the Susan G. Komen for the Cure organization acknowledged that although “mammography is not perfect,” its goal is “to eliminate any impediments to regular mammography screening for women age 40 and older. . . . One-third of the women who qualify for screening under today’s guidelines are not being screened due to lack of access, education or awareness. . . . If we can make progress with screening in vulnerable populations, we could make more progress in the fight against breast cancer.”⁶

Some health advocacy organizations exhibit a disregard for evidence and cost in their work on behalf of an array of medical services. As stakeholders in the health care system, health advocacy organizations undoubtedly know that the cost of treatment for chronic conditions accounts for more than 75% of all health care costs.⁷ But their concern is less with the overall health care budget than with how cutbacks in reimbursement might affect patients with their targeted disease.

Autism Speaks estimates that 1 out of every 110 children in the United States will be diagnosed with autism.⁸ It then contends, in its response to the Institute of Medicine survey, that a full array of habilitative services must be included in the essential health benefits package.² Accordingly, Autism Speaks criticized insurance companies that refused to pay \$300 000 for 4 years of applied behavior analysis therapy for toddlers, despite a lack of evidence of effectiveness. “No doubt applied behavior analysis and other autism therapies have their limitations,” the organization maintained, “but who would deny treatment to a cancer patient just because the prospects for survival are no better than 50 percent? And who would deny an accident victim intensive therapies just because he or she may never recover fully?”⁹ Their priority is not medical practice based on outcome data, but the need for “addressing the huge unmet needs of the more than 1 million Americans already afflicted.”⁹ So, however weak or absent the data on the efficacy of a diagnostic or treatment intervention, the family should be the ultimate decision maker, not the regulator, the insurer, or a team of experts.

These positions notwithstanding, deference to health advocacy organizations’ advocacy is widespread. Some

observers are convinced that because health advocacy organizations represent vulnerable groups, their proposals should be respected and implemented. Others defer to them because they can be counted on to support expansive government insurance programs. But the result of this deference is that when health advocacy organizations are indifferent to evidence-based data, espouse unproven therapies, or resist accepted cost-saving strategies, they may contribute to a public view that equates EBM with rationing.

However valuable independent advocacy organizations are for a democratic society and however important their services provided to targeted populations, their advocacy positions and the related underlying assumptions must be scrutinized with the same diligence as those of other stakeholders. There should be no automatic assumption that all health advocacy organizations deserve special standing or represent the common good.

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