

A case for a science-informed perspective on health care reform

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Personal perspective

As a physician-scientist elected to represent the American Society for Clinical Investigation, I hope to provide a voice for at least some of the physician-scientists who tirelessly devote their energies to translating their findings from the laboratory to improve patient care. We labor because we believe elucidating the molecular underpinnings of disease will translate into more effective treatments that ease the burden of disease suffering and improve the lives of millions of Americans (and people across the globe). That is what drives us. Many of us also believe that molecular medicine, by directing more specific, less toxic treatments to subsets of patients most likely to benefit from them, can also reduce medical costs and improve care. Thus, we are deeply invested in the outcome of the health care debate. As an individual, I hope for health care reform that is humane and pervasive, universal in scope, and science-informed in its details — in short, the kind of health care reform our country must have to be the civilized beacon of hope it deserves to be. Through application of powerful technologies to study genes, proteins, and metabolites in human patients, we have learned that a variety of different molecular alterations can underlie phenotypically similar diseases. An entity originally considered as one homogeneous disease more likely represents multiple pathogenic routes toward a [...]

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Through application of powerful technologies to study genes, proteins, and metabolites in human patients, we have learned that a variety of different molecular alterations can underlie phenotypically similar diseases. An entity originally considered as one homogeneous disease more likely represents multiple pathogenic routes toward a common disease phenotype. This distinction is more than academic when drugs that target specific genetic or biochemical altera-

tions are used as treatments. The average American does not need to understand molecular biology to recognize that their health care could be greatly affected by this. Take cancer as an example. Virtually every American will be challenged by cancer, either their own or in a loved one. We are all likely to become familiar at some point in our lives with the pain and disappointment of traditional anticancer treatments, including cytotoxic chemotherapy and radiation, that are toxic and only modestly improve the lifespan for most patients whose cancers are detected at an advanced stage. Molecular medicine promises to change this.

Studying the underpinnings of cancer has begun to yield new treatments that target specific molecular lesions needed by the cancer cells, providing the potential for much more effective, less toxic treatments. These targetable lesions are usually only present in a subset of patients with that cancer type. In fact, most patients with that type of cancer won't benefit. Traditional metrics would put these new targeted treatments into the "doesn't work" category, yet for patients with the specific targetable lesion, these treatments may be life saving. One can imagine a future in which each patient's cancer is analyzed for targetable lesions and then a personalized treatment plan is tailored to best treat their disease. Thus, all Americans have a stake in the outcome. A path toward realizing this future will require forward-thinking, science-informed health care reform that develops, tests, and incorporates biomarkers

into the determination of efficacy of new treatments in well-designed clinical trials so that we can better answer the question "What works and what doesn't?" by asking, "What works for whom?" I believe that if most Americans were to recognize this fact, they would gladly invest in a process to personalize their care.

I am not an economist. I claim no expertise in understanding the full scope of budgetary issues shaping this challenge. However, I believe that this is a debate about more than just cutting costs and improving access to care. It is also an opportunity to develop a framework for evaluating new tests and treatments that are being dramatically transformed by advances in molecular medicine. As our elected leaders craft policy that will determine whether America will be a model for the seamless integration of cutting-edge science into a health care delivery system that is effective and sustainable, I hope they will: (a) recognize the impact of disease heterogeneity on evaluation of new diagnostics and treatment strategies; (b) encourage development of biomarkers to guide treatment toward patients most likely to benefit; and (c) provide support for well-designed clinical trials to evaluate their efficacy.

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Creating meaningful health care reform

In the heated debate about health care reform, there has been little serious discussion about how to fix the extraordinarily expensive and inefficient delivery system that makes meaningful expansion of coverage difficult. The public debate has degenerated into polemics, while the real issue of providing better health care is lost in the fray. The know-how and capability to create a far more rational and

cost-effective system is within our grasp, and there has never been a more important time for the medical profession to take the lead in advocating for reform. Academic physicians are highly respected by the public and politicians; our views are valued, and we must engage more actively to support better approaches to health care. To improve care, I believe that practice must shift from a reactive, sporadic,

disease event-oriented approach to one that promotes health, prevents disease, and intervenes early and effectively when it occurs. To do this, we must combine three key elements: (a) a personalized strategic approach to care with meaningful patient engagement, (b) a delivery system designed to support and coordinate care over time, and (c) a rational reimbursement system.