

BY MARGARET A. ANDERSON

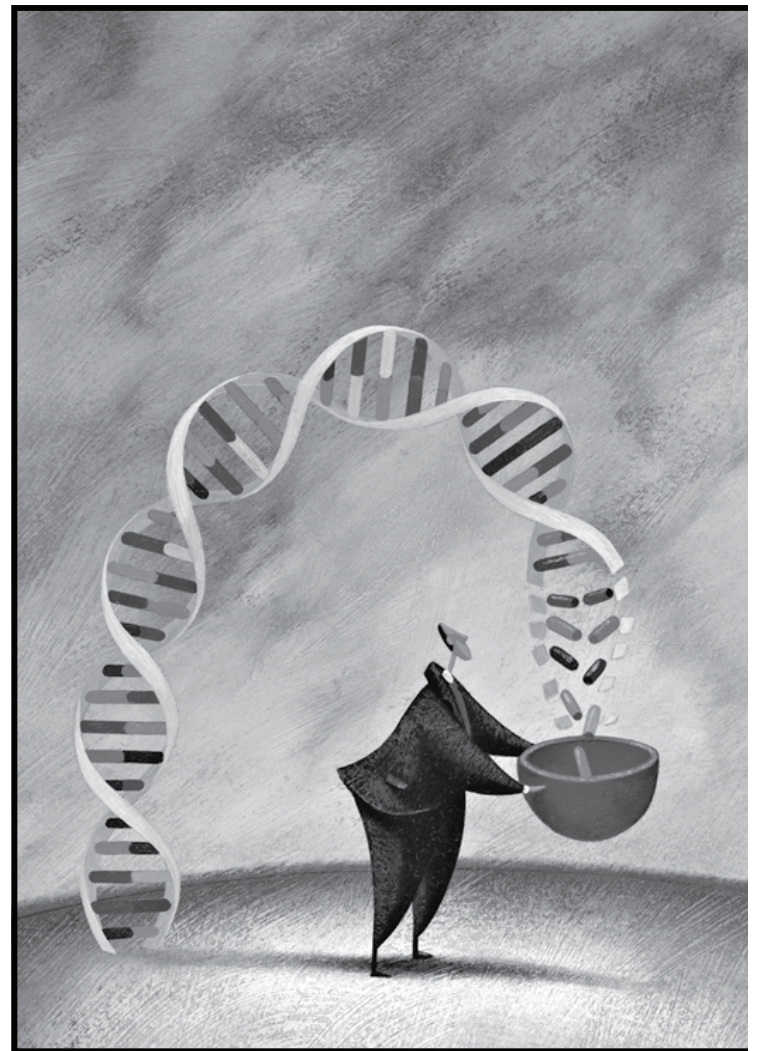
**With the** Obama administration and Congress now negotiating reform, the American health care system as we know it may be about to undergo the most significant makeover since the introduction of Medicare. Meanwhile, the unprecedented public engagement on the fundamental issues faced by policymakers offers a window into systemic transformation in action.

But as Americans focus on how best to deliver and pay for health care, we believe it is equally important not to lose sight of what I would call our “health cure” system. For unless we do a better job of translating promising scientific research into new means of preventing and treating disease, we will lack the wherewithal to contain health care costs – or, for that matter, productivity losses and human suffering.

**A PERFECT STORM**

The combination of genes, environment and lifestyle creates a complex stew of disease determinants that stubbornly resist analysis. Nonetheless, the past few decades have brought enormous breakthroughs in the fundamental knowledge needed to understand many pathologies – advances including the sequencing of the human genome, the application of RNA interference to inhibit gene expression, and the ability to use stem cells to treat disease.

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## INSTITUTE VIEW

Medical science thus seems poised for a perfect storm of discovery, one that offers a real hope of conquering many of the diseases that reduce longevity and generate immeasurable suffering. Now, the challenge is to sustain this effort and – equally important – to funnel a wealth of scientific insight into a renewed focus on finding cures.

### MANAGING THE BEHEMOTH

The success of taking a scientific discovery from clever idea to curative option turns on the joint efforts of federal agencies, academic institutions, non-profit foundations and the

biopharmaceutical industry. All have critical roles to play in this most important of enterprises, but the pace of innovation and development will heavily depend on their ability to advance solutions together.

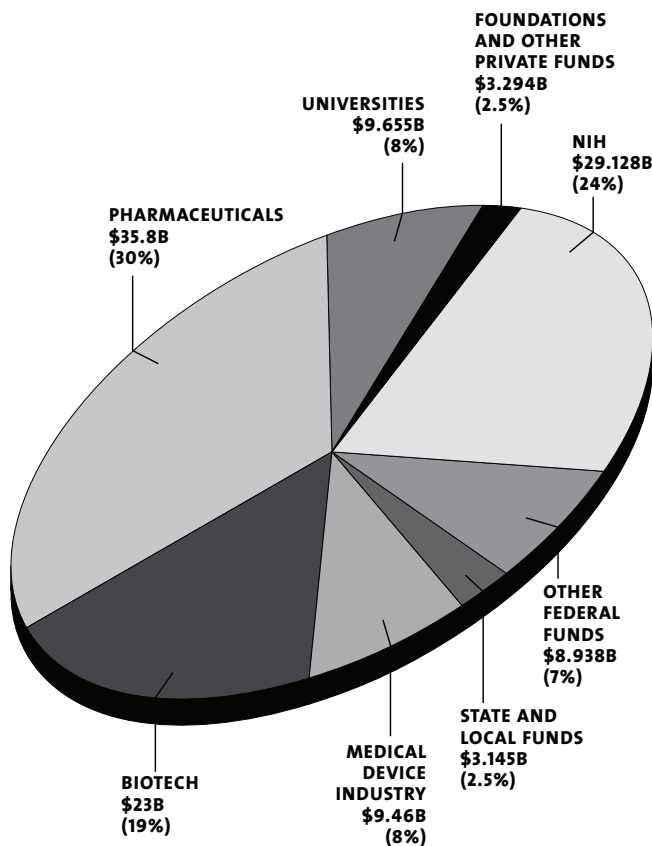
Coaxing the needed synergies from the medical R&D establishment is no small problem. A variety of factors can get in the way of clinical discovery and application, and often these barriers prevent key actors in the medical research enterprise from achieving their shared goal of creating much-needed treatments.

The National Institutes of Health is the single largest public sponsor of biomedical research in the world. And it's about to get bigger, thanks to a massive infusion of funds from the American Recovery and Reinvestment Act (aka the economic stimulus act). NIH's formal mission is both to promote science in pursuit of fundamental knowledge, and to apply that knowledge to extend life, and reduce disease and disability. That is a tall order, but one that we believe is within reach.

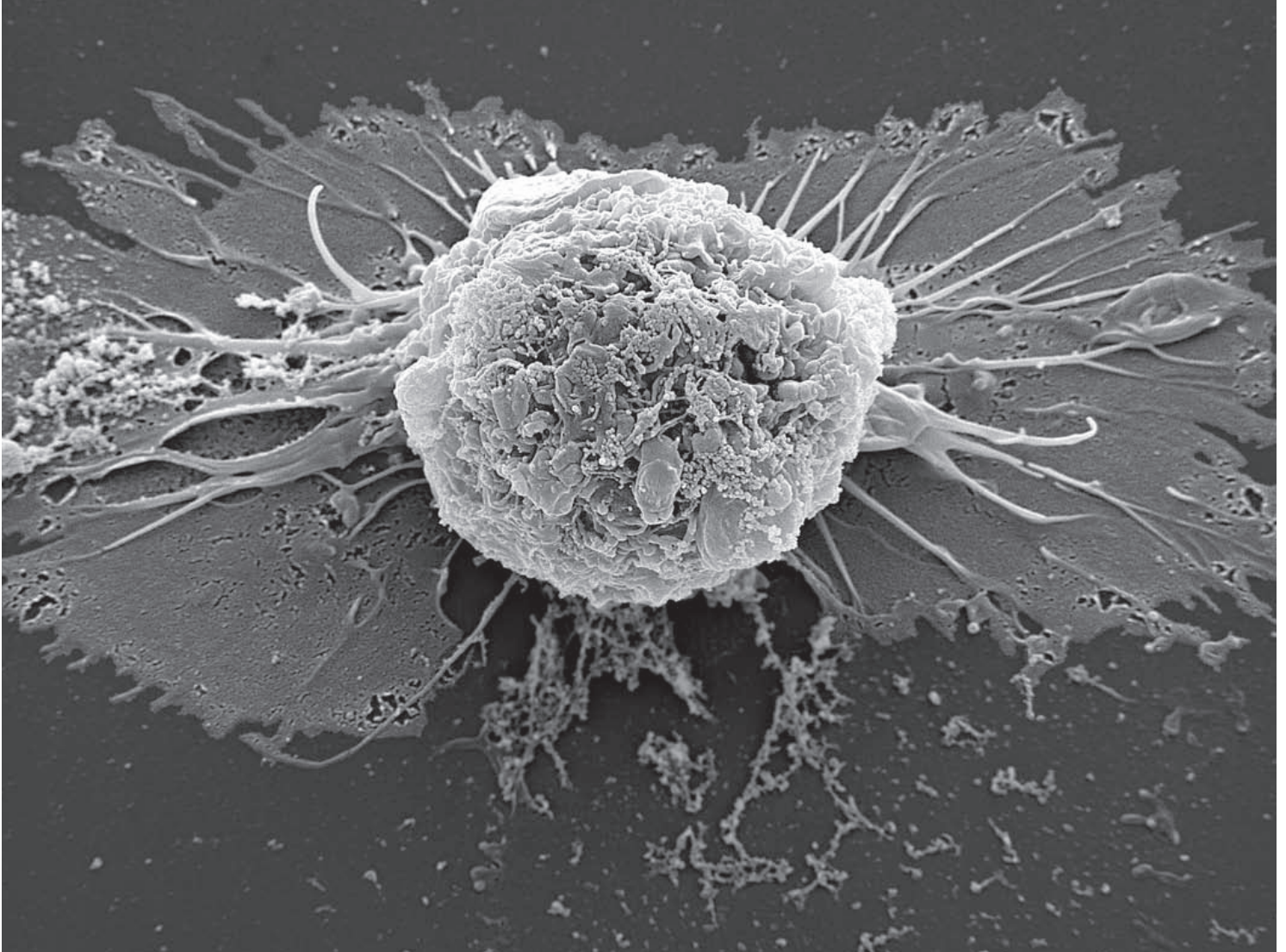
So, what are the most effective and efficient ways to harness science toward treatments? At academic institutions, to which the majority of NIH funding goes, efforts are focused primarily on revealing the underlying mysteries of biology – that is, basic research. This is necessary, but unfortunately, not sufficient to develop patient therapies.

Non-profit disease research organizations funded by philanthropy can play an important role here. Because of their close relationships with the patient communities and tools such as trial registries and tissue banks, their ability to move quickly to address emerging translational and clinical opportunities, and their capacity to leverage public investment, these organizations can catalyze innovation. However, as important as they are in the process, private contributions to medical research remain quite a small piece of the funding

**ESTIMATED HEALTH RESEARCH EXPENDITURE IN THE U.S. BY SOURCE, 2007**  
US\$, BILLIONS (PERCENT OF TOTAL)



SOURCE: Amit Mistry, PhD, Emily Connelly and Stacie Propst, PhD, Research! America, November 2008



stream and cannot match those of the federal government or industry. Nor can they match the basic research activities of academia.

Biopharmaceutical companies focus on developing new therapies as well as funding the expensive clinical research required to commercialize new products. However, industry is restrained by bottom-line considerations that often leave promising research unexplored or discarded. The small size of the potential markets for many therapies makes them unlikely to be profitable, or else the scientific challenges appear too great to risk stockholders' money.

#### **LEADERSHIP IS VITAL**

Science is hard and unpredictable. But science applied effectively will ensure our nation's global competitive edge and, more im-

portant, save lives. We need to ask more of our research enterprise. At the same time, we need to honor the enterprise and the scientists themselves, and to acknowledge the struggles to get from here to there.

NIH can lead this fight against disease by applying the considerable weight of its influence (not to mention its \$30+ billion budget) to removing barriers to progress. The selection of Dr. Francis Collins as director is an enormously positive step. During his tenure as head of the Human Genome Research Institute, Collins led the effort to crack the human DNA sequence and facilitated the translation of discoveries toward treatments. Now Collins will have the resources and the bully pulpit to push NIH – and for that matter, the broader research infrastructure – to focus institutional eyes on the prize. **M**