Case study 2:

Personalized medicine and health care:

re-imagining sickness and health

For all the marvels of medical advances, three big problems have emerged in recent decades. First, many patients don't respond treatment; second, the pharmaceutical industry is finding it harder and harder to develop effective new drugs; and third, the explosion of chronic diseases means that the costs of present-day health care systems have become unsustainable.

Watching countries around the world grapple with the complexities of tackling these challenges, it's clear there are no simple solutions. But the innovative insight embodied in the mantra of personalized medicine – the right treatment, for the right patient, at the right time – is starting to bring significant changes that are gradually seeping into clinical reality and suggesting powerful new ways to deal with the dilemmas of ensuring effective and affordable health care.

"The drivers are powerful, although the reality is slow to arrive," says Iain Miller, CEO of Healthcare Strategies Group, who has watched the tortured progress of personalized medicine since Herceptin, the first targeted therapy for a specific kind of breast and gastric cancer, was approved in 1998. The breakthrough with Herceptin was to identify a biomarker in the tumor, which, if present, indicated that the patient would not respond to ▶

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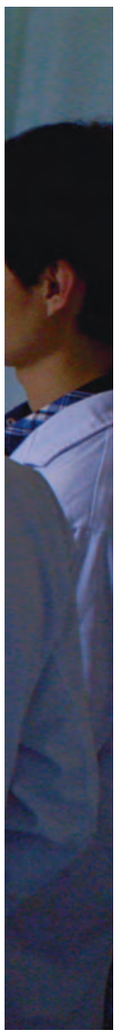
the drug – yielding a significant change in the effectiveness of treatment.

Major scientific advances in gene sequencing followed, as the human genome was decoded amid massive publicity, raising hopes of a revolution in medicine as more and more genetic biomarkers were identified. Parallel advances in data analytics and computational power enabled researchers to process the growing reams of genomic information. "But for years, we had just this one story to talk about," Dr. Miller recalls. "Now, though, we are getting potent evidence of impact and we are seeing significant changes in both health care and the way drugs are approved."

The greatest progress has been made in cancer treatment. Around half of all oncology drugs coming on to the market



are now based on the presence or absence of biomarkers. With the focus on specific and sometimes tiny sub-sets of patients, who would have been invisible in a normal trial, the clinical and approval phase has halved in the past two years from an average of 8.7 years for standard drugs to 4.5 years for those with a personalized plan. The impact on extension and quality of life, especially for patients with advanced cancer, has been significant – not to mention the ability to withhold treatment that is both expensive and harmful where patients would not respond.

These successes reflect a completely new understanding of what cancer is – and increasingly of neurological, auto-immune and other diseases too. Rather than identifying the disease by the organ affected (lung or breast cancer, for example) and treating all patients as if they were the same, research at the molecular level has revealed a startling heterogeneity of types of cancer. That transformation in the perception of disease brings the promise of dramatically more effective treatment. However, it raises equally dramatic challenges to the entire health care industry, from doctors to regulators, drug companies to labs, and policy-makers to insurers.

"Undoubtedly, the treatment of cancer has become more complicated," says Wolfgang Wein, former head of global oncology for the pharmaceutical company Merck. "Practising doctors and health insurers struggle to keep up with the flood of new cancer types and sub-types. The pharmaceutical industry also needs to react to new findings, which regulatory authorities will require of the industry."

The long delay between promise and reality in personalized medicine is partly to do with the need to transform perceptions of disease through rigorous research, and partly to do with coping with the fallout from this new understanding by optimizing regulations, logistics and new business models. Regulators, for example, are only just starting to comprehend the processes needed to combine medicine with diagnostic tests. Drug companies are still struggling with the need to

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forge close partnerships with diagnostics and device companies as they develop personalized treatments. And policy-makers are still trying to work out whether personalized medicine will add to costs or reduce them.

But as personalized medicine starts to transform medical care by re-imagining the nature of disease, so the innovative insight behind it – that care should be tailored to individual characteristics and needs – is leading to a re-imagining of the nature of health and the needs of health care.

"Historically, we've thought of health care as episodic. We associate it with doctors and hospitals," says David Shaywitz, co-author of Tech Tonics and an expert on the potential and pitfalls of digital health. "This is not how we experience life or health." Increasingly, as smartphones and wearable sensors provide constant data on everything from individuals' levels of glucose to their levels of sociability, health care can become something continuous and preventative, taking place outside the walls of a doctor's office or hospital.

For now, most people still see personalized, digital health as a collection of gadgets and gizmos for self-absorbed techies. It is still difficult to see how best to integrate this technology with clinical realities, how to ensure that the possibility of more continuous monitoring improves lives rather than causing new burdens, and how to assess what will have most impact and what is just noise and hype. "It's similar to the beginnings of human genomics and personalized medicine," says Dr. Shaywitz, referring to two Nobel Laureates who warned that a gene sequence is not automatically a drug. "It's important to realize that information is not a cure. We need to do the groundwork to develop the potential, working with front-line providers. We need to make the data actionable."