In tough times, personalized medicine needs specific partners

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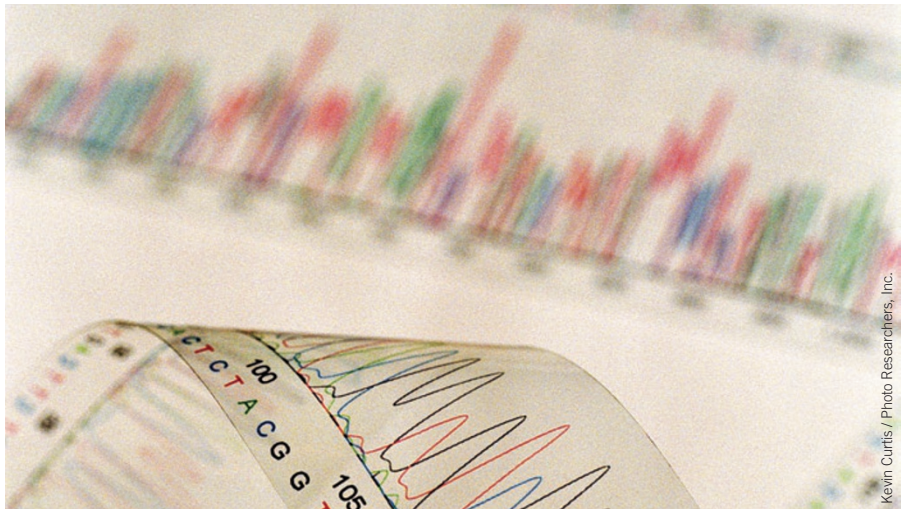
Experts estimate that within a decade average citizens will be able to afford to have their entire genomes decoded. Each person's genetic blueprint will be stored in his or her medical file, and doctors will tailor treatments on the basis of this information. Major progress has been made toward this dream of personalized medicine. Recent studies on genetic variation across ethnic groups demonstrate that the process of sequencing entire genomes is becoming faster and more accurate (*Nature* **456**, 53–59; 2008; *Nature* **456**, 60–65; 2008).

But turning science into personalized healthcare will require smart plans for research funding. The Kansas City, Missouri–based Kauffman Foundation, a leading nonprofit that champions entrepreneurship, has proposed an idea to accelerate the translation of research from lab bench to doctor's office: encourage biotechnology companies and disease advocacy groups to walk hand-in-hand through the 'Valley of Death'.

The Valley of Death refers to the funding gap that arises after scientists make a promising discovery but before investors are willing to take a gamble on it. "It is the space in which nobody wants to fund outcomes of research, because the commercial potential is still unknown," says Lesa Mitchell, co-author of the Kauffman Foundation's recent report on personalized medicine. This gap widens during tough economic times, such as the current global financial slump.

With venture capital becoming increasingly scarce, medical research foundations are in an excellent position to help companies navigate through this difficult terrain in personalized medicine, Mitchell explains. Traditionally, these disease-focused nonprofits have primarily provided information and patient support, but a new generation of advocacy is emerging, one that is intensely devoted to searching for treatments and cures, she says.

The Kauffman report points to the Connecticut-based Multiple Myeloma Research Foundation (MMRF) as one example of this new type of disease-focused nonprofit. In its ten years of existence, the MMRF has raised over $100 million to fund more than 90 laboratories studying myeloma, an incurable cancer of immune cells. The foundation supports academic research but also facilitates industry-run clinical trials. "We provide venture funding to biotechs trying to bring promising discoveries to market," says Louise Perkins, the MMRF's chief scientific officer.



A tailored approach: Researchers should work with foundations focused on specific diseases

Beyond blockbusters

Nonprofits focused on specific diseases might provide key support for those aiming to develop personalized therapies. For decades, the pharmaceutical industry has relied on a 'blockbuster' business model, which is based on producing drugs for large, heterogeneous patient populations. But this one-size-fits-all approach is not conducive to creating therapies designed for specific genetic profiles, which is one of the goals of personalized medicine. Companies must blaze alternative paths toward developing personalized diagnostics and treatments, and disease research foundations can help by linking them to the specific patient populations the companies aim to help, Mitchell explains. She points to the successful partnership between the MMRF and the South San Francisco–based company Proteolix.

Two years ago, just after Proteolix had completed phase 1 trials for its myeloma drug carfilzomib, the MMRF offered to help the company get its phase 2 trials off the ground. By connecting Proteolix to an extensive network of individuals with myeloma and researchers, the MMRF saved the company much of the time and money associated with recruiting subjects and finding principal investigators.

Crucially, MMRF also gave the company access to the Multiple Myeloma Research Consortium tissue bank containing tumor samples from 1,550 individuals. Some 250 of these specimens are being analyzed by scientists at the Broad Institute of the Massachusetts Institute of Technology and Harvard in Cambridge and the Translational Genomics Research Institute in Phoenix. With the MMRF's support, the scientists aim to uncover the genetic idiosyncrasies behind different tumor types, helping companies develop more personalized therapies. "The ultimate goal is to link drug response to genetic profile," says Lori Kunkel, Proteolix's chief medical officer.

Despite such success stories, some experts point out that disease groups have limited financial might. "Their pools of capital aren't as deep as you'd like," says Steven Burrill, who heads the San Francisco investment firm Burrill & Company.

Mitchell agrees but hopes that the Kauffman report, which was submitted to the US Department of Health and Human Services (HHS) in October, will prompt federal agencies to begin offering research grants to industry-nonprofit partnerships.

On 14 November, HHS released a larger personalized medicine report: *Personalized Health Care: Pioneers, Partnerships, Progress*, which draws on perspectives put forth in the Kauffman report and other papers from leaders in the field. Although nonprofits might have a key role in bolstering personalized medicine, the report points out that venture capital is likely to remain the primary funding source for healthcare technologies, as investing in such projects tends to be risky. By publishing this compendium of papers, HHS has made one thing very clear: personalized medicine should be a top priority for the US. "Government, and HHS in particular, has a primary role in supporting scientific discovery," writes HHS Secretary Mike Leavitt in the report's prologue. "We need to work closely with the medical community to improve the translation of proven techniques 'from bench to bedside'."

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