

Personalized Medicine: Planning for the Future
American Association for the Advancement of Science (AAAS)
Food and Drug Law Institute (FDLI)

June 1-2, 2009

WHAT IS PERSONALIZED MEDICINE?

Predictors of the future course of medical treatment and policies generally agree that personalized medicine has the potential to transform health care. Personalized medicine, of course, has always been at the heart of the doctor-patient relationship, with doctors considering such factors as family history and life style when recommending treatment. Now there is hope that a more sophisticated version of personalized medicine will emerge from recent findings in genetics and molecular biology and from advances in imaging that are opening the door to new knowledge of the causes of disease and new treatment strategies. But before these challenges can be realized, several challenges must be overcome.

Decoding the human genome is allowing scientists to better understand the meaning of individual differences in genetic profiles. These profiles, along with differences in proteins or details seen through more refined imaging technologies, are leading to new ways to understand diseases, along with the ability to identify previously unknown subgroups of, say, cancer or diabetes. In some cases, imaging studies may help identify whether a treatment is having its desired effect.

Additionally, future clinicians may be able to assess susceptibility to disease more accurately and prescribe drugs to address that added risk and offer recommendations regarding life style changes.

For example, discovering differences in the genetic profile of cancers is enabling scientists to develop drugs that target these specific disease subtypes, replacing the medical system's usual trial-and-error method of finding an effective medicine. It may also be possible to determine whether patients might have a toxic, allergic or other harmful reaction to a particular drug, allowing for more precise dosing, as is the case now for the anti-blood clotting agent warfarin. In short, the goal is to give the right patient the right drug at the proper dose, without wreaking havoc on the rest of the body.

In addition to better outcomes, many observers predict that personalized medicine will lower health costs, especially if the pharmaceutical and biotechnology industries are able to devise a targeted drug discovery process. Further, drugs that once were deemed failures may be "rescued" if they are found to be effective with specific subtypes of patients. For example, the drug Vioxx was pulled from the market because some patients experienced life-threatening cardiac problems. In the future, clinicians might be able to use personalized medicine methods to predict which patients are able to take the drug safely and identify which patients should not receive the drug. Fewer adverse drug events also have the potential to lower health care costs.

The promises of personalized medicine will remain unfulfilled promises unless scientists, clinicians, policy makers, and industry leaders can overcome challenges that include:

- Educating doctors and patients about the goals and limitations of personalized medicine,
- Reorienting the pharmaceutical industry to develop and market drugs for relatively small populations,
- Designing clinical trials to maximize the possibilities offered by personalized medicine,
- Developing diagnostic tests with sufficient sensitivity and specificity and adopting appropriate regulatory mechanisms to ensure a test's clinical validity,
- Protecting the privacy of individuals when genetic tests indicate an elevated risk for future disease,
- Addressing health care disparities, and
- Encouraging health insurance companies to cover new treatments based on personalized medicine.

