

## Partnership for Personalized Medicine FACT SHEET

### *The Urgent Need*

By several measures, health care spending continues to rise at the fastest rate in our history. In 2005, total national health expenditures in the United States rose 6.9 percent—roughly two times the rate of inflation—with total annual spending reaching \$2 trillion, or 16 percent of the US Gross Domestic Product (GDP). By 2015, health care spending is expected to reach \$4 trillion, or 20 percent of GDP.

Given the significantly greater costs of advanced disease, there is a critical need for diagnostics that will enable the timely and effective implementation of treatment and prevention strategies, and thus contain the health care burden at an individual country and global scale.

Early detection is the most egalitarian form of health intervention, since it supports better health for all citizens at the earliest stage and the lowest cost, unlike expensive therapeutic regimens or interventional technologies that are unaffordable and hence inaccessible to poorer patients.

In addition, a limitation of many current therapies lies in the inability to match patients with appropriate treatments. This results in the broad application of very expensive technologies that benefit only a subset of patients. Significant cost savings could be realized if treatments were administered only to those patients most likely to respond and least likely to experience adverse reactions. Diagnostic tests that reveal those at higher risk for disease permit the selective application of prevention strategies only to those who will benefit, reducing costs and the number of adverse events.

### *The Promise of Diagnostics*

If persons at highest risk for diseases such as heart disease or diabetes can be identified for high intensity prevention, or if diseases such as cancer can be detected at an early highly curable stage, society will realize the benefits of a much healthier population.

The ability to detect and measure biomarkers in blood or tissue samples provides a powerful way to diagnose diseases. Some biomarkers of molecular or physiological processes have already been identified and are currently used in disease management. *The vast majority of such biomarkers remain to be discovered.*

Diagnostics can enable the selection of more cost-effective therapies through the identification disease and/or the prediction of treatment response. For example, testing of creatine kinase, myoglobin and troponin for rapid detection of heart attack in the emergency room yields a 30 percent savings in hospital costs.

However, proteins will be much more informative than DNA or RNA as the basis for diagnostic tests and can be applied to a broader spectrum of diseases.

The greater utility of proteins arises from several key features, including:

- Proteins change in response to variations in physiological conditions since they are the agents that mediate physiology. Proteins therefore can reveal the consequences of life-style and environmental exposures for disease risk, in contrast to DNA, which reveals only hereditary disposition.

- A single gene can produce a family of 10 to 100 variant proteins. This variation adds to the amount of information revealed by the spectrum of proteins present in a disease state.
- Proteins from diseased tissue are found in the bloodstream, whereas DNA and RNA molecules must be obtained by biopsy of the diseased tissue itself. The availability of proteins in blood samples allows clinicians to measure specific protein biomarkers in human blood, thereby sampling diseased processes throughout the body by a method that is much less invasive than tissue biopsy.

*Although blood contains an estimated 100,000 different proteins, very few have been validated for disease diagnosis and management.*

The most definitive diagnostic tests will remain those based on visualizing the disease within the anatomy of the body. The discovery of new protein diagnostics will empower developments in imaging technologies, including X-rays, nuclear magnetic resonance imaging (MRI), ultrasound and positron emission tomography (PET).

### ***A New Initiative***

A new model that employs a collaborative approach to the development and validation of new diagnostics is desperately needed.

The Partnership for Personalized Medicine is a major health care research initiative that unites contributions from two leading Arizona-based philanthropic organizations: The Virginia G. Piper Charitable Trust (\$35 million) and the Flinn Foundation (\$10 million) with leadership and research capabilities from Fred Hutchinson Cancer Research Center, the Translational Genomics Research Institute, and the Biodesign Institute at Arizona State University.

The Partnership will initially unite the efforts and capabilities of the Biodesign Institute at ASU and TGen, without replicating existing resources but rather leveraging them to generate the greatest impact per dollar of initial investment. The Partnership also will link in other Arizona institutions and initiatives, including the Arizona Proteomics Alliance (AzPA) and Arizona's Clinical Translational Science Award (CTSA).

The Partnership is a broad-based effort to develop new, protein-based diagnostic tools to improve human health and reduce health care costs. The Partnership aims to develop, test and validate personalized diagnostic tools for a wide range of diseases and then obtain approval for clinical use of these tests which would be reimbursed by health care systems. The result of this effort will be an entirely new approach to medicine that offers more accurate assessments of disease risk; better predictions of responses to treatment; and safer, more effective treatments.

The Virginia G. Piper Center for Personalized Diagnostics (PCPD) is the scientific discovery and development engine for the Partnership. The PCPD will be located in the Greater Phoenix metropolitan area and will integrate resources within Arizona and beyond. Because the development of the diagnostics hinges on identifying and validating protein biomarkers, the PCPD will feature world-class proteomics production laboratory facilities.

Another component of the Partnership, the Flinn Fund for Arizona Proteomics Research, will emphasize research collaborations among Arizona's research universities, health care providers, research institutes and industry partners. It will support the collection and storage of biospecimens and drive Arizona-centric demonstration projects.

Apart from an emphasis on diagnostics instead of therapies, the Partnership also aims to focus on proteins rather than DNA or RNA as biomarkers of disease. Proteins will be much more informative than genomes as the basis for diagnostic tests and are applicable to a broader spectrum of diseases.

Nobel Laureate Dr. Lee Hartwell, president of Fred Hutchinson Cancer Research Center, will be the executive committee chair of the newly created Partnership for Personalized Medicine, with leadership also including Dr. Jeff Trent, president and scientific director of TGen, and Dr. George Poste, director of the Biodesign Institute at ASU.

The goals of the personalized medicine initiative established by the Trust are several:

1. To make Phoenix an intellectual hotbed of distinctive work in the field of personalized medicine so that Phoenix will attract world-class talent and international recognition.
2. To develop new medical strategies that will advance the translation of cutting edge research into tangible changes in patient care based on individualized diagnosis, treatment and prevention.
3. To leverage the Trust's dollars so that more investments will be made in the Valley in advancing the field of personalized medicine.
4. To forge strategic partnerships among institutions in the Valley that will produce greater impact and less duplication.

The benefits expected to be generated by the Partnership and the Piper Center are improved patient outcomes, reduced long-term health care costs, and avoidance of the costs associated with decreased productivity due to illness and disease.

For example, improved diagnostic tools will enable earlier assessment of disease risk and application of preventative measures that will improve human health and avoid much of the suffering and cost associated with disease and disease treatment. Such tools also will improve the sensitivity and specificity of diagnoses, allowing for earlier, more accurate detection of disease. In doing so, these tools will enable patients to avoid the negative health impact and costs that result when diseases go undetected and untreated.

By combining these far-reaching benefits, the Partnership and Piper Center aim at nothing less than a revolution in health care that will usher in a new era of individualized therapies that are safer, more effective and more cost-efficient.

The Piper Center will serve as a hub for intellectual activities and collaborative endeavors related to personalized diagnostics and as a convening point for all stakeholders in this effort. The PCPD will bring together scientific, research and policy expertise in the fields of proteomics, imaging, nanotechnology, computing, informatics and health economics to:

- Pursue the discovery of biomarkers (primarily but not exclusively in the form of proteins identified in blood samples),
- Translate these biomarkers into molecular diagnostic tools,
- Conduct multiple demonstration projects to illustrate and document the value of adoption of these tools into common clinical practice, and

- Drive the development and commercialization of these tools and other related intellectual property.

With an emphasis on collaborative research, the Piper Center will tap into the resources of and interact with stakeholders from around the world.

The Piper Center will be established in roughly 12 months, including recruiting personnel, purchasing equipment and outfitting space. During this period, the Partnership also will work to design specific demonstration projects and secure partners. The Partnership will launch its first demonstration project at the end of this 12-month period. Each demonstration project will take roughly three years to complete, and the Partnership expects to launch an average of one new project every year.