



President and CEO Judy Mohraz

**The Partnership for Personalized Medicine and
Virginia G. Piper Center for Personalized Diagnostics:
FREQUENTLY ASKED QUESTIONS**

Why does the Partnership for Personalized Medicine have the potential to change the practice of medicine?

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 insurance companies. 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. This will mean better health for all patients and long-term savings in the cost of health care, the fastest growing sector of many countries' Gross Domestic Product.

What is different about the Partnership for Personalized Medicine's approach compared to many other cutting-edge efforts in personalized medicine? What is unique?

Many of the recent breakthroughs in medical treatment focus on developing and applying personalized *therapies* and emphasize *treatment*. The Partnership seeks to develop personalized *diagnostics* that place a greater emphasis on *prevention*. The current emphasis on treatment entails the use of costly drugs that have contributed to escalating health care costs. This emphasis has been encouraged by insurers' reimbursement policies that provide much greater coverage for treatment rather than prevention. The Partnership's emphasis on prevention, while requiring changes in reimbursement policies, holds greater promise to reduce the overall cost of health care.

There are a lot of unique aspects to the Partnership. It is a completely untested concept. It is more applied than would occur in most academic settings, larger in scale and stronger in quality control. It also is unique in the aspect of partnering with international health care systems to achieve validation, implementation, approval and reimbursement all at the same time.

Why does the Partnership for Personalized Medicine focus on the study of proteins, or “proteomics,” rather than the study of genes, or “genomics”?

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 are likely to be more informative than genomes as the basis for diagnostic tests and are applicable to a broader spectrum of diseases. The greater utility of proteins arises from several key features:

- Proteins change in response to variations in physiological conditions. Proteins can therefore 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 10 to 100 variant proteins. This variation adds to more detailed information revealed by the range of proteins present in a disease state.
- Proteins from diseased tissue exist in the bloodstream and can be collected via a simple blood draw; DNA and RNA must be obtained via a biopsy of the diseased tissue itself.
- The remarkable genomic resources at both TGen and The Biodesign Institute at ASU offer real potential for leveraging the combined resources of proteomic and genomic markers.

How are protein biomarkers detected and validated for disease diagnosis and management?

Biomarkers are identified by comparing proteins found in disease-associated and healthy samples of blood or tissue. The presence or absence of a particular protein (or combination of proteins) may correlate to a particular disease state or response to treatment. To date, protein biomarker discovery has been limited because of the very low-levels of proteins in the blood. Estimates suggest that there are possibly 100,000 different proteins in the blood, but only a very few of these have been validated for disease diagnosis and management. Recent advances in a technology—called mass spectrometry—now make it possible to identify and analyze proteins at previously undetectable levels. This and other cutting-edge techniques will enable the validation of protein biomarkers for disease diagnosis and management.

Other breakthroughs in protein diagnostics will come from the field of medical imaging using tools such as X-rays, magnetic resonance imaging (MRI), ultrasound and positron emission tomography (PET). Combining protein biomarkers with imaging technology will enable the precise identification of disease activity within the body. However, such imaging tests are expensive. Therefore, using less costly blood-based protein diagnostics will be much less expensive than imaging and can also be used to identify which patients require more costly imaging tests also will contribute to the reduction in health care costs.

What are the key elements of the Partnership for Personalized Medicine?

The Partnership will employ a different scientific method to understand disease and will represent a radical new model in terms of its organizational structure. It will use a systems approach and bring together stakeholders from across the health care chain and beyond, including discovery laboratories, insurance carriers, health care providers, health care economists and regulatory agencies. Together, these stakeholders will design a better and more economical approach to healthcare.

The Partnership will create the Virginia G. Piper Center for Personalized Diagnostics (PCPD) which will conduct a series of demonstration projects. Each project will develop a specific diagnostic test for a specific condition and will be underwritten by an insurance payer or other funder. For example, countries with single-payer health care systems may be eager to support demonstration projects that address a disease that is a major cause of mortality in that nation. Large health care systems in the United States will be promising partners, as will disease-based and healthcare-focused philanthropic organizations. Each project will show a clear return on investment, measured in terms of both healthcare cost savings and increased productivity due to improved health.

What, exactly, is the Virginia G. Piper Center for Personalized Diagnostics?

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 Phoenix, Arizona and will integrate resources within Arizona and beyond. The PCPD will oversee many diverse activities, including the recruitment and coordination of participants and funders for the demonstration projects; the design of these projects; the execution and management of each project, including biomarker discovery, diagnostics creation, and data management and analysis; and the development and commercialization of resulting intellectual property, among many others.

Because the development of the diagnostics hinges on identifying and validating protein biomarkers, the Piper Center will feature a world-class proteomics production laboratory facility. This production facility will operate within the Translational Genomics Research Institute (TGen) but will draw upon a wide range of resources within the state, particularly at the Biodesign Institute at Arizona State University. The PCPD will build its high performance computing and informatics capabilities around the current, cutting-edge resources shared by ASU and TGen. The PCPD also will incorporate health care economics capabilities, both by supporting faculty recruitment at ASU and by contracting with subject matter experts as needed. Finally, yet importantly, the PCPD will have capabilities in the areas of nanotechnology and imaging, which will be critical to the development and commercialization of diagnostics tools.

How is the Partnership for Personalized Medicine different from the Virginia G. Piper Center for Personalized Diagnostics?

The Partnership is a broad-based effort to develop new, protein-based diagnostic tools to improve human health and reduce health care costs. The Piper Center is only one component, albeit a critical one, in the execution of the Partnership. The Partnership may develop other components in the future.

What is the Flinn Fund for Arizona Proteomics Research?

This is another component of the Partnership for Personalized Medicine, established by the Flinn Foundation, will emphasize and enable research collaborations among other Arizona-based institutions, including the state's research universities, health care providers, research institutes, and industry partners. Examples include emerging statewide research consortia such as the Arizona Proteomics Alliance, the Advanced Research Institute for Biomedical Imaging at the University of Arizona, and the Arizona Biospecimen Alliance.

How will the Virginia G. Piper Center for Personalized Diagnostics structure its leadership?

A three-person Founding Executive Council (Council) will provide leadership for the Piper Center and maintain financial and management oversight over all PCPD activities. The inaugural members of the Council will be Lee Hartwell, PhD, chair, president and director of the Fred Hutchinson Cancer Research Center and 2001 Nobel laureate; Jeffrey Trent, PhD, president and scientific director of TGen; and George Poste, DVM, PhD, DSc, director of the Biodesign Institute at ASU. The Council will also create an External Advisory Board to advise on scientific and business matters. Each member has agreed to commit 10% of his time to Piper Center activities.

What role will Dr. Hartwell play as Chair of the Council?

In his role as Chair, Dr. Hartwell expects to play an active role in all Piper Center activities, including directing the design, launch and execution of the demonstration projects, and identifying and recruiting research partners and financial supporters for those projects.

What is the succession plan for the Council?

Upon vacancy, the seats currently occupied by Dr. Trent and Dr. Poste will be filled by whomever succeeds them as the President of TGen and the Director of the Biodesign Institute at ASU, respectively. Upon vacancy, the other two Council members, in close consultation with representatives of the founding donors, will appoint a third member to fill the seat currently occupied by Dr. Hartwell.

Does the Piper Center for Personalized Diagnostics change the Piper Trust's personalized medicine initiative?

The goals of the personalized medicine initiative that the Trust established 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 original design of 10 chairs sought to achieve these goals. What Piper Trust could not anticipate in January 2006 was the opportunity to link the Trust's initiative with Dr. Lee Hartwell's revolutionary vision of advancing the field through focused work in personalized diagnostics.

The Piper Center for Personalized Diagnostics will enable the Trust to realize all of the above goals, and at a level even greater than that originally anticipated with the filling of the 10 chairs. Furthermore, personalized medicine initiative funds remain to continue to populate the community with stellar candidates proposed by the research hospitals.