PMC Personalized Medicine Coalition

Member Newsletter

JANUARY 2006

"As for the future, your task is not to foresee it, but to enable it." — *Antoine de Saint-Exupéry*

The Personalized Medicine Coalition (PMC), publicly launched at the end of 2004, was established with that exhortation in mind to address the myriad issues that affect adoption of a new paradigm in medicine. Although personalized medicine – defined as optimizing healthcare through molecular understanding of disease and treatment approaches – is already transforming drug development and clinical care in cancer, its true potential still lies more in the future than the present. Our mission is to hasten its fulfillment.

Science and technology, of course, move at a much faster pace than the public policies that support or impede them. As a result, the PMC is dedicated to shaping a political and business landscape that facilitates the development of personalized medicine. Clearly, this is a concept whose time has arrived: PMC membership more than tripled in 2005 and now encompasses more than 60 pharmaceutical, biotechnology, diagnostic, and information technology companies as well as research institutions, patient advocacy groups, payers, and government agencies. These members believe that prescribing the right drug for the right patient at the right dose at the right time is far superior to our present approach, which relies on trial-and-error more often than we wish to admit.

Our members recognize that the current regulatory, reimbursement, and privacy laws, as well as our prevailing business models and educational systems, are unequipped to deal with, let alone encourage, the changes that personalized medicine requires. For example, in the future, even more than today, safer and more effective drugs will be developed for and targeted at individuals based on particular biomarkers rather than based on a one-size-fits-all model. In addition, individuals may be treated before they actually develop clinical symptoms as we know them today because they will understand their own genetic profiles and disease susceptibilities. But such scenarios raise a host of legal, ethical, operational, financial, and policy questions that must be addressed before the promise of personalized medicine can be realized.

PMC members have joined together to ensure that our society puts in place a coherent, integrated approach to the legal, financial, social, and professional issues that will shape the development and adoption of personalized medicine.

2005 was a year of tremendous growth for the PMC. With an expanding base of leaders among our multiple constituencies, we were able to establish the PMC as a "go-to" source of credible information in the media from the *American Journal of PharmacoGenomics* to *The New York Times*. Our well-attended programs included luncheon seminars with Dr. Francis Collins of the National Human Genome Research Institute and Dr. Janet Woodcock of the U.S. Food and Drug Administration; a Capitol Hill briefing with Newt Gingrich; a seminar on public attitudes towards genetics; and inaugural receptions in Boston and San Francisco with Dr. Raju Kucherlapati of the Harvard-Partners Center for Genetics

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PMC Luncheon Address March 6, 2006 12:00p.m.-2:00p.m.

Andrew C. von Eschenbach, M.D.
Acting Commissioner
U.S. Food and Drug Administration

At the National Press Club
in Washington, D.C.
To register, click here.

Dr. Raju Kucherlapati Proposes Key Trial of Personalized Medicine Approach

Dr. Raju Kucherlapati, Scientific Director of the Harvard-Partners Center for Genetics and Genomics, gave the keynote speech at the PMC's West Coast Inaugural dinner in Menlo Park, CA on November 29th. A scientist and early proponent of targeted approaches to diagnosis and therapy, Dr. Kucherlapati noted that while the tools for determining the best therapies for patients are already available, they have not yet become the standard of care.

Although it is known that drug-associated adverse events are a major cause of mortality and morbidity in the United States, and it is also widely

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PMC Presents First Leadership in Personalized Medicine Award

In a presentation at the Harvard-Partners Center for Genetics and Genomics Personalized Medicine conference in November, the PMC presented its first Leadership in Personalized Medicine award to Dr. Janet Woodcock, the U.S. Food and Drug Administration's Deputy Commissioner for Operations. The PMC sought to recognize "a visionary and insightful individual" who has both instituted change in science, business, or policy and "has raised awareness for personalized medicine across a broad sector." The PMC presented Dr. Woodcock with the Leadership award, citing her efforts to ensure that the latest advances in molecular medicine are incorporated into the regulatory process at the FDA, as well as her encouragement of innovation on the part of industry. The PMC also recognized her for her role in the FDA's Critical Path Initiative, which seeks to ensure that drugs of the future are both more effective and safer than those of today.

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Letter from the Executive Director

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and Genomics. Partnering with Burrill & Company and *Scientific American*, the PMC also co-sponsored major symposia. And by providing a clearinghouse of information about personalized medicine, our website unites and defines the community.

This year in a series of seminars we will explore how personalized medicine is transforming the treatment of cancer, cardiovascular disease, and mental illness, focusing in particular on the scientific, business, and public policy nexus that will determine the scope and pace of change in the treatment of these major illnesses. In addition, we will take a hard look at the business models for personalized medicine, notably whether or not public policies should be introduced to encourage its advancement.

In addition to public education programs on the general benefits of personalized medicine, we look forward in 2006 to having an increased impact in the public arena, and have identified a number of pivotal areas. Because there are so many uncoordinated initiatives on personalized medicine within the federal government and in the private sector, the PMC will undertake a comprehensive survey of the healthcare environment as it relates to personalized medicine. Using these data as a platform, the PMC will be able to assist decision makers in both the public and private sectors to bring personalized medicine products and concepts to patients in the future.

The PMC will also focus its attention on engaging payers, including CMS, based on an emerging set of principles developed by our Public Policy Committee. We will continue to advocate increasing reimbursement for molecular diagnostic testing—which we view as an essential building block of personalized medicine—as well as our effort to remove barriers to integrating such testing into medical practice due to public concerns regarding genetic discrimination. In addition, task forces have been created to examine intellectual property issues and healthcare electronic records as they may affect personalized medicine, and the possible need for additional public incentives to encourage the co-development of diagnostic and therapeutic remedies.

All this comprises an ambitious program. We will not complete it in 12 months, and we will surely not achieve it without your continuing and growing support of our presence in the marketplace of ideas. But we have no reason to believe that, as the science and technology proceed apace, in the not-too-distant future we will no longer be referring to "personalized" medicine but simply to medicine as it is practiced in our own time.

—Edward Abrahams

PMC Presents First Leadership in Personalized Medicine Award

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Dr. Janet Woodcock accepts the first Leadership in Personalized Medicine Award. From left to right, Edward Abrahams (PMC), Brian Munroe (PMC), Janet Woodcock (FDA), Raju Kucherlapati (HPCGG)

Dr. Woodcock accepted the award, stating her belief that a better understanding of the molecular basis of disease and treatment response variability will be one of the most significant factors in improving healthcare. She reiterated her dedication to using pharmacogenomics to develop safer and more effective drugs in the future, and thanked the PMC for its support of the FDA's efforts to advance that goal. ■

"I believe that better understanding of the basis for disease and treatment response variability will be one of the most significant factors in improving the quality of healthcare and improving the outcomes of healthcare for the people of this country."

- Janet Woodcock, M.D., U.S. Food and Drug Administration

"We are not there yet, but when personalized medicine becomes standard operating medical practice in what we hope will be the not-too-distant future, we will look back and see that the first tracks leading to that destination were laid by Dr. Woodcock in 2005."

- Edward Abrahams, Ph.D., Personalized Medicine Coalition

Dr. Raju Kucherlapati Proposes Key Trial of Personalized Medicine Approach

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believed that the use of genetic and genomic information would significantly reduce these adverse events, he contended that the genetic and genomic tests for diagnosis, prognosis, and treatment of many common illnesses are not as widely employed as they should be.

Dr. Kucherlapati pointed to the example of testing for epidermal growth factor receptor (EGFR) mutations in lung cancer tissue and the response of patients to a class of drugs called tyrosine kinase inhibitors (TKIs). It has been shown, he noted, that patients whose lung cancers have a somatic mutation in the EGFR gene have a significantly higher probability of responding to TKIs. Therefore, incorporation of such a test could influence the decision-making process by oncologists and may save or extend the lives of patients.

"Several companies and academic laboratories," he said, "offer a large number of such tests but it is apparent that these tests are not used as extensively as appropriate, and many of them have not become a part of the clinical decision-making process." According to Dr. Kucherlapati, lack of content, lack of support by drug makers, lack of clear-cut evidence that the testing would change outcomes, and lack of awareness that the myriad tests in the medical or patient communities, all contribute to the underutilization of molecular diagnostic testing. "Perhaps the most important factor," he suggested, "is the lack of evidence to support the notion that genetic and genomic testing would change the outcomes."

Dr. Kucherlapati challenged the entire industry to prove once and for all that a targeted approach is superior.

"I therefore propose," he said, "that we undertake large-scale, definitive sets of prospective clinical trials in which we examine the effectiveness of incorporating genetic and genomic testing as part of the clinical decision-making process. The design of the trials should be rigorous, with very well-defined endpoints, comparing personalized and state-of-the-art care. The trials should be large enough to obtain a definitive answer and they should also shed light on the cost/benefit ratio of incorporating molecular diagnostics into the particular areas of clinical medicine."

"We have a lot of work to do," he concluded, adding that he was nevertheless optimistic that through education and advocacy we would overcome the barriers that block the implementation of personalized approaches to medicine that hold so much promise for patients. ■

PMC in the Press

When the *Oakland Tribune* covered the topic of the promise of personalized medicine in October, Edward Abrahams, PMC's Executive Director, was asked to add his insight. Abrahams noted, "It is very exciting, because a lot of patients who have gone through the trial-an-error process of being prescribed medications know how painful it can be. Personalized medicine promises patients much better results."

In December, the *San Francisco Business Times* article, "Personalized medicine could be antidote to soaring care costs," discussed the political, regulatory, and economic barriers that must be addressed for the promise of personalized medicine to be realized. In response to how the PMC is helping to address these barriers, Edward Abrahams was quoted as saying, "We're asking the questions that need to be answered. We don't necessarily have the answers. We are setting the table where interested parties can come and work out those answers."

For links to theses and other PMC-related articles, including *USA Today*'s, "Personalized drugs draw biotech dollars," *Genetic Engineering News*', "Americans Want Genetic Information in Healthcare," and *Oncology Times*', "'Personalized Medicine': Survey Finds Public Generally Wary of Genomics in Medicine," visit the Communications section of our website at: *www.PersonalizedMedicineCoalition.org/communications/overview.php*.

Genetic Information Non-Discrimination Act of 2005 Advances in the House of Representatives

The Genetic Information Non-Discrimination Act (GINA) will be on the docket for the U.S. House of Representatives this year when lawmakers return to Washington, D.C. in January to kick off the second session of the 109th Congress. The bill, H.R. 1277, has gained significant momentum on Capitol Hill following lobbying efforts led by the Coalition for Genetic Fairness, of which the PMC is a member. Since passing the Senate by unanimous vote last February with the endorsement of President Bush, the list of co-sponsors in the House of Representatives has expanded to more than 160 members, but the House Leadership has not yet scheduled a vote on the measure.

At a time when large-scale population studies and widespread use of genetic tests are critical for the discovery of safer, more effective treatments, most observers believe that public anxiety regarding the safety and security of genetic information represents a significant barrier to better healthcare. Proponents of the legislation contend that it is essential for the patients and clinical trial volunteers to believe that their genetic information, gathered for medical care and research, will be treated with the utmost discretion and not be used for discrimination in health insurance coverage or employment. The proposed legislation would seek to do that. "More than half the states have already passed laws to protect the privacy of this information," observed Robert Wells, Vice President for Government Relations and Public Policy at Affymetrix, Inc. and PMC Board member. "It's time Congress sets a standard that protects the entire country." ■

PMC Welcomes the Following New Members in 2006:

Aureon Laboratories, Inc. >>
GenVault, Corp. >>
HistoRx >>
Johnson & Johnson >>
Karolinska Institutet >>
Nixon Peabody LLP >>
Quest Diagnostics>>
RAND Corporation >>
Sarcoma Foundation of America >>
TAP Pharmaceutical Products Inc. >>

Sign up

To receive the PMC Newsletter and other announcements from PMC, sign up at our website: *www.PersonalizedMedicineCoalition.org.*

Upcoming Events

Board of Directors Meetings:

To be held at:
1401 H Street NW, Suite 200
Washington, D.C.

March 30 | 2:00 p.m. - 5:00 p.m.
June 28 | 2:00 p.m. - 5:00 p.m.
September 27 | 2:00 p.m. - 5:00 p.m.
December 11 | 2:00 p.m. - 5:00 p.m.

Committee Meetings:

To be held at:
1401 H Street, NW, Suite 200
Washington, D.C.

Public Policy Committee
February 2 | 12:00 p.m. - 2:00 p.m.
March 30 | 10:00 a.m. - 12:00 p.m.
June 28 | 10:00 a.m. - 12:00 p.m.
September 27 | 10:00 a.m. - 12:00 p.m.
December 11 | 10:00 a.m. - 12:00 p.m.

Communications/Program Committee
March 30 | 12:00 p.m. - 2:00 p.m.
June 28 | 12:00 p.m. - 2:00 p.m.
September 27 | 12:00 p.m. - 2:00 p.m.
December 11 | 12:00 p.m. - 2:00 p.m.

Clinical Science Committee
March 30 | 10:00 a.m. - 12:00 p.m.
June 28 | 10:00 a.m. - 12:00 p.m.
September 27 | 10:00 a.m. - 12:00 p.m.
December 11 | 10:00 a.m. - 12:00 p.m.