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**Discussion Paper: Towards effective community engagement with personalised medicine**

*Introduction*

This brief discussion paper introduces a new research project on the challenges of and opportunities for public engagement on the development of personalized medicine. Genetically targeted pharmaceuticals (pharmacogenomics) could be extraordinarily beneficial to patients. However, the systemic transition from mass medicine to personalized medicine raises important policy questions about cost and access, prioritization of medical conditions, social divisions, and the comparative influence of stakeholder groups. I am interested in comparing public engagement in the sphere of personalized medicine and multiple sclerosis to previous experiences with two early prototypes, BiDil and Herceptin, in order to map the issues and to improve pathways for moving technological breakthroughs from the lab to the patient in sustainable and effective ways. I argue that the P6 Medicine model (to be explained below) needs to evolve to P7 in recognition of the promissory aspect of personalized medicine and its effects on public expectations and engagement.

*What is Personalized Medicine?*

Pharmacogenomics is the study of how genes affect the way an individual responds to medicine. Personalized medicine—often described by advocates as “the right drug at the right dose to the right person at the right time”—is supposed to improve health care, increase the accuracy of prescribed doses and produce medicines targeted specifically (and

beneficially) to genetic cohorts rather than to a mass and undifferentiated population. If the era of personalized medicine does happen as its advocates imagine it, the future of medicine will be simultaneously more efficient and more patient-centered.

Pharmacogenomics would also represent a tangible social and economic pay-off justifying the billions of taxpayer dollars spent on genetic research in the last 50 years.

As S. Hall notes “the defining characteristic of every form of personalized medicine is its biomarker, a kind of biological fingerprint that distinguishes a subset of the patient population” (2003: 66). Advocates of personalized medicine see so-called rational drug design (based on biomarkers and other genomic techniques) as an innovation that will make the current blockbuster pharmaceutical model as outdated as the Model T-Ford (which Henry Ford famously promised could be bought in any color as long as that color was black). Eric Lander, Director of the Human Genome Center at the Whitehead Institute (MIT), for example, states “people looking back 50 years from now will consider medicine a barbaric, random process. If the promise of genomics is fulfilled, it will transform the lives of everyone” (Fischer 2001). A report from Price Waterhouse Coopers (PWC) equates pharmacogenomics to previous advances such as antibiotics and vaccines and contends that the new approach “promises to usher in an era of individualized patient care or personalized medicine” (PWC 2005: 1). Personalized medicine discourse thus links public and private incentives by ostensibly putting the individual patient at the center of this new healthcare model, while simultaneously encouraging investment in lucrative new/segmented drug markets and promising major improvements in medical system efficiency. G. Levey (Board Member of the Center for Accelerating Medical Solutions and Dean of the UCLA School of Medicine), for instance, foresees “the beginning of the genetic medicine era which presents

extraordinary opportunities, not only for our personal health, but also for the entire healthcare system" (2003).

New stakeholder groups and policy networks are rapidly emerging to capitalize, literally and figuratively, on the increasing value of genetic information. The Personalized Medicine Coalition (<http://www.personalizedmedicinecoalition.org/>) —launched in 2004— defines personalized medicine as “the use of new methods of molecular analysis to better manage a patient’s disease or predisposition towards a disease” (PMC 2006). It sponsors public forums on genomics and medical applications and actively seeks to influence federal legislation on genetic non-discrimination and other related priorities. Similarly, Faster Cures/The Center for Accelerating Medical Solutions (<http://www.fastercures.org/>), an initiative of the Milken Institute, is "committed to accelerating the medical research process to find new treatments for deadly and debilitating diseases" (Faster Cures 2006).

#### *Brief Political History of BiDil and Herceptin*

Herceptin (Roche Pharmaceuticals) and BiDil (Arbor Pharmaceuticals) are two early prototypes of personalized medicine. While neither is pharmacogenomic in the strictest sense, these two case studies demonstrate that the introduction of personalized medicine on a large scale will have significant political and social implications.

In 2005, the Food and Drug Administration (FDA) approved BiDil (<http://bidil.com/>) in the United States for treatment of heart failure in self-identified black patients. This decision is the first time that a medicine received approval to be marketed to a specific cohort defined by race. For the FDA, the BiDil decision represented an important progress toward the promise of personalized medicine. Given that the African-American population has historically been underserved in the medical system and even—as in the case of the infamous Tuskegee syphilis

experiments in Alabama (1932-1972)—blatantly discriminated against, advocates of the decision often framed the BiDil approval as both medically and socially progressive. Dr. Keith C. Ferdinand, speaking for the Association of Black Cardiologists—an organization that strongly supported the FDA decision—emphasized that “we all know race is an imperfect proxy for any kind of biological status, and hopefully clinicians will interpret this new approval to suggest that any patient regardless of race or ethnicity that fits the profile of the patients in the trial would benefit from this medication.” Yet for critics of the decision, the campaign to market BiDil primarily to African Americans threatened to reify race as a biological category and reintroduce dangerous notions of racial difference. As Dorothy F. Roberts argued, “no one is complaining that BiDil is available to people who will benefit from it. The problem is that BiDil was made available on the basis of race (2011, 1).” Critics also emphasized that the probable real motive for BiDil development was money, noting that the statistical findings on differences in efficacy based on alleles only arose because Arbor Pharmaceuticals wanted to secure thirteen additional years of patent protection by recombining existing drugs.

Herceptin (<http://www.herceptin.com/>) is a monoclonal antibody used in the treatment for HER2 Positive Metastatic Breast Cancer and HER2 Positive Gastric Cancer. Twenty-four OECD countries authorize the public health system to subsidize a twelve-month treatment regimen for breast cancer patients. In New Zealand, however, PHARMAC chose—based on its mandate to consider costs and benefits and the published scientific evidence—to approve subsidy of a nine-week course of Herceptin. Against the backdrop of the 2008 election, Herceptin became an unusually visible public health issue. Advocates of a 12-month regimen noted both the gender-specific aspects of the disease and the OECD norm in arguing that PHARMAC’s decision was both discriminatory and biased. Ultimately PHARMAC was ordered to revisit the decision and to engage in much wider public engagement. When PHARMAC, upon appeal, did not reverse its

decision, the National Government (winning the 2008 election) stepped in to force (via statute and increased budget authority) the subsidization of the 12 month course of treatment. As in the BiDil case, the controversy around Herceptin generated complex political and ethical fault lines. Virtually all parties to the dispute recognized the severity—financial, emotional, and physical—exacted by breast cancer. However, the Labour Party—in particular—argued that undermining PHARMAC’s statutory authority in this case, no matter the good intentions, now opened the door to a wholesale politicization of the decision-making process for advanced and expensive treatments. Whether the political process or the health budget could withstand the precedent set by the Herceptin case remains to be seen.

Both the BiDil and Herceptin cases merit substantive analytical attention on their own merits. For the purposes of this discussion, each demonstrates that the promise of personalized medicine, even if it can be realized scientifically, will create significant regulatory challenges. Genetically-targeted medicines, by definition, segment populations and arguably undermine the communitarian ethos that underpins universal health care systems. In both public and private health systems, pharmacogenomics also forces us to consider how differential political power and stakeholder access can affect which medicines receive regulatory approval and which are attractive candidates for private investment and research. These problems exist already, of course, but are likely to be exacerbated in the move from the mass market/blockbuster model to highly differentiated genetic and financial cohorts. The potential paradox is that at the same time we call for more public engagement with medicine and health system issues—underpinned by the goal of reaching socially relevant and democratic decisions—personalized medicine by definition (and due to its likely cost) pits different patient groups against each other and elevates the role of consumer over that of citizen.

### *Personalized Medicine and Multiple Sclerosis*

Multiple sclerosis is a complex disease that affects the central nervous system. Essentially, the myelin sheath that surrounds the nerves deteriorates progressively, causing gaps that affect the transmission of brain signals to the body. M.S. affects both men and women, though is diagnosed much more frequently in women. Its symptoms manifest in a variety of ways, ranging from mild to severe, and the disease can take several unpredictable courses (such as relapse-remitting). It also tends to strike in the patient's early 40s, thus having a profound effect on earnings and on families and communities. There is no cure for M.S., though there is significant research and investment being made in genetically-based treatments ranging from stem cells to personalized medicine.

In this new space—but drawing upon the lessons learned with BiDil and Herceptin—I propose a case study to map the politics and actor networks of multiple sclerosis and to determine both how public engagement is understood by the stakeholders involved and how the promise of personalized medicine intersects with the ensuing dialogue. My central question is: how do patients engage with scientists, research institutes and corporations when so much of the debate revolves around cures that exist in an elusive future, some 10, 20 or 30 years down the road? My hypothesis is that the P6 model—personalized, predictive, preventive, participatory, psycho-cognitive and population based (Bragazzi 2013)—is evolving into the P7 model in order to encompass the promissory dimension of pharmacogenomics.

I look forward to discussion of these issues and welcome input on this research project.

## REFERENCES

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