The Genomics and Personalized Medicine Act Returns to Congress

by Guest Contributor and Dan Vorhaus

Meggan Bushee is a student at the Wake Forest University School of Law.

This past May, Congressman Patrick Kennedy (D-RI) and Congresswoman Anna Eshoo (D-CA) re-introduced a personalized medicine bill to the U.S. House of Representatives. The bill was originally introduced in 2006 by then-Senator from Illinois Barack Obama. While HR 5440, also known as the Genomics and Personalized Medicine Act of 2010 (GPMA 2010), has retained the name of the bill originally introduced by Senator Obama, its approach to the regulation of personalized medicine has taken a new direction.

GPMA 2010 is the fourth version of the GPMA since the original bill of 2006, and includes the most ambitious initiatives of all of its predecessors. Why has the GPMA re-surfaced after three prior versions failed to make it out of committee? According to Representative Kennedy, the bill has been re-introduced in response to increased public awareness and use of genomic tests. At present, GPMA 2010 is before the House Committee on Energy and Commerce. This is the same committee that recently conducted high-profile hearings to review the current state of the direct-to-consumer (DTC) genetic testing registry.

As the tools of personalized medicine, including genetic testing, have become both less expensive and more powerful, calls for expanded oversight of the field have intensified, particularly in the area of DTC genetic testing. While there is a pressing need for appropriate regulation to protect the consumers and patients targeted by personalized medicine, there is an equally pressing need to avoid crafting a system of oversight that will be an obstacle to continued growth and innovation. The current version of the GPMA aims to strike a balance between consumer protection and flexibility to allow for innovation.

This post outlines the material provisions of GPMA 2010 and examines the transformation the bill has undergone since it was first introduced in 2006.

The GPMA Defines Itself. The stated aim of the Genomics and Personalized Medicine Act is:

To secure the promise of personalized medicine for all Americans by expanding and accelerating genomics research and initiatives to improve the accuracy of disease diagnosis, increase the safety of drugs, and identify novel treatments, and for other purposes.

Interestingly, GPMA 2010 is the first iteration of the GPMA to formally define the term “personalized medicine.” However, the bill limits its definition of “personalized medicine” to:

any clinical practice model that emphasizes the systematic use of preventive, diagnostic, and therapeutic interventions that use genome and family history information to improve health outcomes.

It’s a broad definition, but is it broad enough? Conspicuously absent from the definition is any mention of environmental information, a category that is increasingly recognized as critical to the understanding and management of complex and common traits and diseases.

Despite its narrow definition of personalized medicine, GPMA 2010 includes several expansive initiatives. GPMA 2010 would appropriate $150 million for fiscal year 2011 to accomplish these initiatives, including the creation of an Office of Personalized Healthcare and several committees to address translational challenges of personalized medicine, the standardization of the collection of human biological samples, the funding of further research and education on personalized medicine, and the creation of a national biobank.

In order for those initiatives to bear fruit, the GPMA, should it proceed, is likely to find itself in need of a similarly expansive definition of personalized medicine.

The OPH: Coordinating Personalized Medicine. GPMA 2010 would create an Office of Personalized Healthcare (OPH) within the Department of Health and Human Services (HHS). The OPH would have two main roles: (1) to oversee the implementation of GPMA 2010’s initiatives, and (2) to coordinate the activities of various federal agencies and private and public entities. To fulfill these roles GPMA 2010 would appropriate $5,000,000 for fiscal year 2011, and “such sums as may be necessary” for later years.

The OPH is a new addition to the GPMA since its previous version in 2008. Prior to GPMA 2010, the GPMA provided for the establishment of an Intergency Working Group (IWG), an initiative that was first introduced in the 2006 bill. The IWG had goals similar to those of the OPH, but had few specific responsibilities. The IWG was mainly responsible for meeting twice a year and submitting a report every two years on IWG activities. The OPH, on the other hand, would be more directly involved in directing the expansion and acceleration of research, and signifies a large departure from all prior GPMA bills.

Among other responsibilities, the OPH would be tasked with the development of a long-term plan to accelerate the research and development of personalized medicine products. Each year the OPH would issue a report discussing not only progress within personalized medicine research, but also the challenges that the OPH has identified and is currently addressing. This provides a case in point for how the narrow definition of “personalized medicine” in the bill might affect the implementation of the GPMA. To use our example, if the role of environmental factors is not included in the definition, the OPH’s long-term plan might not take adequate account of the need to utilize environmental data in developing effective personalized medicine products.

Importantly, as presently drafted, the OPH would also be responsible for recommending which personalized medicine products should be regulated, and what roles and responsibilities should be assigned to the Food and Drug Administration (FDA) as opposed to the Centers for
Medicare & Medicaid Services (CMS). Presumably this would include weighing in on areas where those two agencies’ regulatory authority appears to overlap, including the regulation of laboratory developed tests. Here again, the act’s definition of “personalized medicine” makes a difference.

GPMA 2010 recognizes the need for greater cross-agency coordination and for a centralized task force to direct the implementation of GPMA initiatives. One ongoing concern is that the development of personalized medicine and its translation to clinical practice will be hampered by redundant and inconsistent oversight at the hands of multiple, overlapping regulatory bodies. The OPH would address this concern, at least in theory, by assigning regulatory authority for personalized medicine products, clarifying and simplifying existing regulations, and providing a clear delineation between the roles and responsibilities of the FDA, CMS and other regulatory agencies. The key question, however, is whether adding a new agency (OPH) to the personalized medicine mix would bring much-needed coordination and strategic vision to the field, or whether it would simply add another layer of confusion and bureaucracy.

A National Biobank. Similar to the biobank initiatives in all three previous versions of the GPMA, GPMA 2010 would create a national biobank to collect and integrate human biological specimens and biobank data. As defined by GPMA 2010, “biobank data” includes health information, demographic genotype, molecular profile data, and (despite being excluded from the definition of “personalized medicine”) environmental data.

If implemented, GPMA 2010’s national biobank would not be the first of its kind in this world. Several countries, including the United Kingdom, Japan, Sweden, Finland, and Iceland have already undertaken similar biobanking initiatives. While the United States has many smaller public (at both the state and federal level) and private biobanks, the GPMA would authorize NIH to coordinate the first truly national biobank. Depending on how swiftly the biobank was created, and whether it incorporated samples from previously existing public or private biobanks, it might quickly become one of the largest repositories of biological specimens and data in the world.

While the implementation of the biobank would be left to the Director of the NIH (currently Francis Collins), working in coordination with the Centers for Disease Control and Prevention (CDC), GPMA 2010 does provide a basic framework. The Director of NIH would be responsible for coordinating the activities of the national biobank with the other public and private biobanks and genomic databases in the United States and developing guidelines to “safeguard[] the privacy of…biobank data.” The Director would also be tasked with addressing ownership and patient access issues and investigating new models of informed consent that balance privacy, risk disclosure and the need for long-term and open-ended research, a task that has recently been shown to be particularly challenging.

One inevitable challenge in implementing a truly national biobank populated with broadly characterized specimens will be funding. To establish the national biobank and fund a related grant program, GPMA 2010 would appropriate $150,000,000 for fiscal year 2011, and “such sums as may be necessary” for later years. While the biobank’s data and specimens would be made available to both government and non-governmental entities, it is unclear whether non-governmental entities would bear some portion of the cost of the biobank.

Is $150 million and the vague promise of more to come sufficient for a biobank of such ambition? By way of comparison, while the initial appropriation, as currently drafted, would be larger than the amount used to catalyze the UK’s national biobank in 2006, which collected £62 million from a variety of funding sources, including the Wellcome Trust, the UK’s largest non-governmental source of biomedical funding. For the GPMA’s national biobank to succeed, similar private funding commitments might well be a prerequisite.

The various incarnations of the GPMA have fluctuated in their treatment of race. The 2006 GPMA had an entire section dedicated solely to “Race and Genomics,” and included several initiatives aimed at including minority populations in genomics research and in improving minority populations’ access to genetic services. The 2010 bill lacks the separate section, but does instruct the Director of the NIH to develop guidelines to “ensure the inclusion of underrepresented populations with health disparities in the activities of the national biobank.” That is itself a departure from the 2008 version of the GPMA, which did not specifically mention minority or underrepresented populations at any point. The role of minority or underrepresented populations in genomic research, and the appropriateness of personalized medicine tools and products for minority or underrepresented populations, was an issue that came up several times at last month’s Congressional hearing on DTC genetic tests, and it is one that would be likely to play a central role in any future Congressional discussion of the GPMA and a national biobank.

The GPMA and DTC Genetic Testing. GPMA 2010 directs the FDA to collaborate with the FTC to “identify and terminate...advertising campaigns that make false, misleading, deceptive, or unfair claims about the benefits or risks of products used for personalized medicine.” While similar consumer protection provisions existed in prior versions of the GPMA, the scope has been expanded in the current version of the bill to apply to advertising and marketing of any personalized medicine product (previous versions focused solely on genetic tests).

Events may have overtaken this proposal, however. Last month’s Congressional hearing and GAO report (pdf) highlighted “misleading test results” and “deceptive marketing and other questionable practices” on the part of DTC genetic testing companies. The report was forwarded to the attention of both the FDA and the FTC and, in its aftermath, it seems unlikely that it will take the passage of new legislation for those two agencies to begin working together to more aggressively police the personalized medicine marketplace.

Interestingly, a separate provision of GPMA 2010 would instruct the CDC, the FDA and the FTC to work together to “conduct an analysis of the public health impact” of “products used for personalized medicine (including genetic and genomic tests) for which consumers have direct access” and to do so “to the extent possible from available data sources.” The joint agency initiative would also “analyze the validity of claims made in [DTC] marketing” and “make recommendations...regarding necessary interventions to protect the public from potential harms” of DTC marketing and access to personalized medicine products. While such an undertaking might appear redundant with the GAO’s recently-concluded investigation, the GAO’s report was an admittedly unscientific snapshot of the field (“GAO did not conduct a scientific study but instead documented observations that could be made by any consumer.”), for which it has been frequently criticized. While a more comprehensive and data-driven analysis of the field would be welcome, recent events suggest that agencies such as the FDA are likely to proceed with additional DTC regulatory oversight on the basis of the data (or lack thereof) currently at hand.
Expanding the Role of Companion Diagnostics and Pharmacogenomics at the FDA. Another provision targeted at the FDA would permit the agency, under certain circumstances, to “require the sponsor of a drug or biological product” (emphasis added) to develop a companion diagnostic test in connection with regulatory filings for a new drug. This provision was originally included in the 2006 bill, but was removed in the 2007 and 2008 versions. Those versions merely permitted the FDA to recommend companion diagnostic development to drug and products sponsors.

The 2010 GPMA also instructs the FDA to “clarify and issue guidance” that explains when companion diagnostics will be included in labeling – including appropriate “standards of evidence…such as with respect to the analytical validity, clinical validity, clinical utility, dosing, adverse events, and drug selection….” – and when such tests will be either recommended or required.

In many respects these provisions of the GPMA seem to reflect the increasing reliance on genomic and genetic data in selecting and administering therapeutics, including the use of companion diagnostic tests.

Where Will the GPMA Go From Here? While GPMA 2010 itself represents a significant departure from the bill originally introduced by Senator Obama in 2006, it is exceedingly unlikely to become law in its current form. Among other considerations, the recent (and ongoing) developments in the areas of laboratory developed tests (LDTs) and DTC genetic testing – two important components of personalized medicine – suggest that substantial revisions would be required to reflect an ever-changing technological, commercial and regulatory environment.

At least for the moment, passage of the GPMA in any form does not appear to be imminent. Perhaps it will never become law – at least in anything like its current form – and either existing legislation or other contenders, such as Senator Hatch’s proposal to create a new regulatory category for “advanced personalized diagnostics” – will be used to fill gaps in the oversight of personalized medicine products. Then again, recall that crafting legislation to respond to the successes of modern science and technology can be a painfully slow process. For instance, the only piece of federal legislation specifically directed at genetic technologies and information, the Genetic Information Nondiscrimination Act (GINA), took thirteen years from the date it was first proposed to its signing into law in 2008. After a mere five years, the GPMA likely has a long way to go.