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FDA and NIH Announce New Funding Opportunities and Collaboration Designed to Help FDA Fast-track Innovations to the Public

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On February 24, 2010, the U.S. Food and Drug Administration (FDA) and the National Institutes of Health (NIH) announced a collaborative "initiative designed to accelerate the process from scientific breakthrough to the availability of new, innovative medical therapies for patients."[i] In the short-term, this initiative has created funding opportunities to third parties for research designed to provide "new methods, models or technologies that will inform the scientific and regulatory community about better approaches to evaluating safety and efficacy in medical product[s]."[ii] The goal of this joint initiative is to provide improved regulatory pathways that will deliver new medical products faster and safer. Prospective applicants must submit final applications by April 27, 2010.

What is the new initiative?

The two integral pieces of the initiative that will help the Agencies achieve their goals are the formation of the Joint Leadership Council and of the Funding Opportunity Announcement (FOA) entitled, "Advancing Regulatory Science through Novel Research and Science-Based Technologies." The Joint Leadership Council will be comprised of 14 members, including the NIH director, the FDA commissioner and six members each from the NIH and the FDA, selected from the Agencies' Directors and Senior Staff. The Leadership Council will "ensure that regulatory considerations form an integral component of biomedical research planning, and that the latest science is integrated into the regulatory review process."[iii]

In addition to the creation of the Joint Leadership Council, the FDA and the NIH are contributing funds for third parties to "study the applicability of novel technologies and approaches towards the development and regulatory review of medical products"[iv] through the FOA. Prospective applicants are encouraged to submit a letter of intent by March 27, 2010,[v] and must submit final applications by April 27, 2010.[vi] Eligible

prospective applicants include for-profit and not-for-profit organizations, public and private institutes of higher education, and federal, state and local governments.

As part of reaching the goal, the FDA seeks to fill in knowledge gaps with respect to regulation of innovative medical products. Dr. Margaret Hamburg, FDA Commissioner, understands that, while bench scientists develop new approaches to diseases and clinicians may be able to show that they work, "[FDA] regulatory scientists must have the knowledge and tools to help translate discovery, innovation and promise into real world products for those who need them."[vii] Dr. Hamburg also recognizes that the FDA does not have these tools in place with respect to new innovative fields such as personalized medicine.[viii] Without a clear regulatory pathway in place, entities are less likely to make investments in these fields.[ix] Dr. Hamburg is confident that the coordination and collaboration with NIH will enhance both Agencies' efforts towards "improving health, reducing disease and saving lives."[x]

What are the short-term impacts of this collaboration?

Initially, one of the biggest opportunities created by this new initiative is the funding opportunity for third parties designed to provide "new methods, models or technologies that will inform the scientific and regulatory community about better approaches to evaluating safety and efficacy in medical product[s]."[xi] Entities that are capable of this research not only have an opportunity for funding but also have the opportunity to play an integral role in shaping the development of these new FDA regulatory paradigms.

In addition to the funding opportunity, the FDA and the NIH announced they will be holding public meetings in the Spring, the purpose of which will be to solicit input on how the Agencies can better work together. This public hearing should provide opportunities for entities to potentially submit written testimony as part of the docket or give oral input at the hearing itself. Those entities that anticipate participating in the regulatory pathways established by the FDA to regulate these new innovations should consider participating in these meetings, as early input will likely play a large role in how the FDA establishes these new regulatory pathways.

What are the possible long-term impacts of this collaboration?

This collaboration is designed to provide improved regulatory pathways that will deliver new medical products faster and more safely by helping the FDA develop a consistent, integrated and comprehensive approach to the evaluation and regulation of innovative therapies, including personalized medicine. Over the long-term, this comprehensive approach is likely to impact the size and scope of clinical trials, speed of regulatory review, product labeling and product reimbursement.

This collaboration may reduce the size and costs of clinical trials, by providing the FDA the scientific knowledge necessary to allow it to validate new surrogate endpoints. Currently, the FDA faces challenges in regulating clinical trials for treatments of rare diseases because of limited sample sizes. This is also true with clinical trials for

personalized medicine. This collaboration will hopefully provide the FDA with sufficient knowledge to allow the FDA to analyze and feel comfortable with the results of smaller sample size clinical trials. This may allow entities to increase funding orphan drug discovery and personalized medicine with less risk of not finding sufficient numbers of individuals to participate in clinical trials. The decreased size and length of clinical trials will likely result in less data supplied to the FDA for review and will likely reduce approval timelines.

Currently, a beneficial treatment may be withdrawn from the market or denied access altogether if it poses even a relatively minimal risk of causing a serious adverse event. Dr. Hamburg recognizes, however, that if there is a reliable way to identify at-risk patients and exclude them from the treatment population, complete product withdrawal from the market may not be necessary, and it is possible that some of these beneficial treatments could continue to potentially save lives. This new collaboration may provide the FDA with the scientific knowledge necessary to identify at-risk patients and allow these beneficial products to remain on, or have access to, the market so long as the label carries an indication preventing at-risk patients from being exposed to the treatment. It also may potentially open up new reimbursement routes for new and existing products.

The knowledge gained through this collaboration also may impact reimbursement for innovative treatments in several ways. First, this knowledge may provide pathways to approval for new innovative treatment areas. At the same time, product labeling is known to impact reimbursement decisions, and any labeling changes may affect how and if an entity's product is reimbursed by payers. For instance, if the label contains a warning that either certain individuals are known to be at-risk for serious adverse events if exposed to a treatment or that the treatment is known not to be beneficial to a certain segment of the population, these treatments may not be reimbursed if provided to these excluded populations. Additionally, new doors to reimbursement may open for those entities that can develop such testing protocols and capabilities, since testing would likely be necessary to determine if a patient is part of the excluded population.

The goal of this collaboration between the FDA and the NIH is to develop improved regulatory pathways at the FDA that will deliver new medical products faster and more safely by developing a framework for evaluating and regulating the determinations discussed above. While these new regulatory pathways may impose additional hurdles that entities must overcome before an FDA-regulated product is placed on the market, they may provide entities with new pathways for products that would otherwise be denied access to the marketplace because of the risks associated with the products. This will hopefully allow more products to move faster from microscope to marketplace.

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This Client Alert was authored by Lynn Shapiro Snyder, Dan Gottlieb and Lee Rosebush. For additional information about the issues discussed in this Client Alert, please contact one of the authors or contributors or the EpsteinBeckerGreen attorney who regularly handles your legal matters.

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ENDNOTES:

[i] News Release, U.S. Food and Drug Administration and National Institute of Health, NIH and FDA Announce Collaborative Initiative to Fast-track Innovations to the Public (Feb. 24, 2010), http://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm201706.htm
[ii] *Id.*
[iii] FDA-NIH Joint Leadership Council Charter, http://www.fda.gov/ScienceResearch/SpecialTopics/RegulatoryScience/ucm201654.htm
[iv] Request for Applications, Department of Health and Human Services, RFA-RM-10-006, Advancing Regulatory Science through Novel Research and Science-based Technologies, (Feb. 24, 2010).
[v] "Although a letter of intent is not required, is not binding, and does not enter into the review of a subsequent application, the information that it contains allows IC staff to estimate the potential review workload and plan the review." *Id.*
[vi] *Id.*
[vii] Dr. Margaret Hamburg, Commissioner, U.S. Food and Drug Administration, Remarks at Announcement of FDA/NIH Collaboration (Feb. 24, 2010).
[viii] "The term 'personalized medicine' can mean many different things, . . . [f]or the FDA, however, the term has a very distinct meaning: it is the application of genomics in the development of medical products that are safer and more effective for the public." Dr. Margaret Hamburg, Commissioner, U.S. Food and Drug Administration, Remarks at AAAS – The Future of Personalized Medicine (Oct. 26, 2009).
[ix] *Id.*
[x] Dr. Margaret Hamburg, Commissioner, U.S. Food and Drug Administration, Remarks at Announcement of FDA/NIH Collaboration (Feb. 24, 2010).
[xi] Dr. Margaret Hamburg, Commissioner, U.S. Food and Drug Administration, Remarks at AAAS – The Future of Personalized Medicine (Oct. 26, 2009).