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Responses to Additional Questions and Member Requests for the Record for the June 11, 2014, 21st Century Cures Initiative Hearing, House Committee on Energy and Commerce

The Honorable Michael C. Burgess

Question: Would you comment on some of the barriers that Class III medical device manufacturers face when seeking coverage and payment from CMS for innovative, cutting edge technology that improves the lives of patients?

Advanced and innovative diagnostic tests, including companion diagnostics (class III medical devices), have the potential to dramatically increase the efficacy and safety of medicines by better predicting how patients will respond to a given therapy. These innovative, cutting-edge technologies improve the lives of patients by identifying which patients will benefit from a medicine, and therefore should receive that treatment, and which patients will not benefit or are more likely to suffer side effects of a medicine, and therefore should not receive that treatment. Unfortunately, manufacturers of diagnostic tests have increasingly struggled with the uncertainty surrounding coding and reimbursement of these tests. Open questions include whether these tests would be covered and reimbursed at all, and, if so, would they be reimbursed at a rate that allowed the manufacturer to recoup its investment. This uncertainty makes it difficult for manufacturers to obtain financing and increases the risk of developing these cutting edge technologies. Without increased certainty regarding a test's potential return on investment, some diagnostics may not be developed at all, never making it to patients to help guide their treatment and enhance their clinical outcomes.

Congress recently took a great step forward in addressing the problems with coding and reimbursement of these tests. On April 1 of this year, as part of the Protecting Access to Medicare Act of 2014 (H.R. 4302), Congress enacted provisions that will incentivize the development of innovative diagnostics. These provisions were originally included in H.R. 3116, the Modernizing Our Drug and Diagnostics Evaluation and Regulatory Network Cures Act of 2013, or the MODDERN Cures Act. The new law establishes a value-based payment system for diagnostic tests and a process for assignment of a temporary reimbursement code to a new test after it is approved by the Food and Drug Administration (FDA). I commend Congress for taking this step and believe that this new law will help alleviate some of the coding and reimbursement challenges faced by diagnostic manufacturers, bringing more certainty to the industry and more benefit to patients.

Longer term, the patient community is urging the diagnostics and medical device industries to work with us to develop a framework for integrating patient perspectives into the product development process. Such a framework should create a consensus-based definition for patient engagement, validate methods of engagements for use at each step of the development timeline, and remove unnecessary barriers that currently prevent companies from engaging with patients. We believe this will help alleviate some of the uncertainty faced by manufacturers for coverage and reimbursement of their products by supporting the development of diagnostics tests and medical devices that can demonstrate higher value. The National Health Council is working with other organizations such as the Medical Device Innovation Consortium to create an environment that makes patient engagement a core function of the research, development, and regulatory processes.

The Honorable Henry A. Waxman

Member Request: Would you please provide the committee with the data and information to show whether there are significant numbers of dormant therapies that are waiting to be developed? Would you also please provide the data that explains why 15 years of exclusivity and patent protection are necessary for these therapies?

As Senator Hatch explained in his recent foreword to the *William Mitchell Law Review* issue on the anniversary of the Hatch-Waxman Act: While “the foundation laid by the Hatch-Waxman Act thirty years ago will continue to be the mechanism by which the government incentivizes development of lifesaving drugs. . .we cannot rest on the laurels of this legislative achievement. . .[W]e have an obligation to periodically reevaluate how the balance can be adjusted to account for the sweeping changes in the broader health care sector.”¹

The time has come to reevaluate this balance as current incentives are no longer optimal to incentive new treatments for unmet medical needs. The National Health Council’s discussions with individual patient advocacy organizations and drug manufacturers have confirmed to us that manufacturers factor in questions of patent protection when deciding whether or not to continue the development of a drug, particularly for those disease areas in which the clinical development timeline can be long. In addition, I have been told by researchers funded by the National Institutes of Health that much of their most promising research cannot obtain sufficient patent protection to be picked up and developed by a manufacturer – one of the reasons that scientific discoveries fail to translate into clinical benefit for patients. In fact, one 2012 article estimated

¹ Hatch, O. *William Mitchell Law Review*. Accessible at: http://www.wmitchell.edu/lawreview/Volume40/40_IV.html. (last accessed June 7, 2014)

that roughly 30,000 drugs were abandoned by the pharmaceutical industry over the past thirty years.²

The patentability standards of novel and nonobvious explain many situations in which a promising medicine lacks patent protection.³ An invention is only eligible for a patent if it is new (novel) and it would not have been obvious to make the invention based on the body of knowledge that was already known at the time of its creation (nonobvious). Often, patents protecting potential new drugs will address a family of related drugs, sometimes hundreds of drugs, but only protect a few of the described potential drugs. The fact that the issued patent (which is public) describes the potential drugs renders those drugs unpatentable because they are no longer “new.” In addition, other public disclosures (inadvertent or not) can have the same effect, preventing the manufacturer from obtaining a patent protecting a potential drug. Other times the issued patent does protect all the potential drugs described, but the patent expired while the manufacturer was developing one or a few of these drugs, leaving no patent protection for the drugs that are developed later. In addition, in the case of drugs, “obvious” ones are those “that would have been reasonably expected to succeed at the time of their invention . . . drugs that initially look most likely to be effective are often the least likely to be patentable.”⁴

We can do better to incentivize and bring treatments to patients suffering from unmet medical needs. For a promising product with no or uncertain patent protection, sufficient protection from generic competition for a specific period of time after FDA approval creates certainty for manufacturers. This would allow them to pursue medicines that have the greatest

²Wadman, Meredith, New Cures Sought from Old Drugs, *Nature* 490, 15, October 4, 2012.

³ See in general, Roin, Benjamin N., Unpatentable Drugs and the Standards of Patentability (February 2009). *Texas Law Review*, Vol. 87, pp. 503-570, 2009. Available at SSRN: <http://ssrn.com/abstract=1127742>

⁴ Roin, Benjamin N., Unpatentable Drugs and the Standards of Patentability (February 2009). *Texas Law Review*, Vol. 87, pp. 503-570, 2009. Available at SSRN: <http://ssrn.com/abstract=1127742>

potential to meet an unmet medical need, even if the treatment has insufficient patent protection. As I mentioned in my written testimony submitted to the Committee on June 11, 2014, the uncertainty created by the reliance on patents discourages companies from pursuing medicines with long development timelines – those intended to prevent disease or treat early stage or chronic diseases – in favor of those with shorter development timelines – those intended to treat later-stage diseases and acute conditions.⁵

In cancer, for example, this leads to more research and development of drugs intended to treat later-stage cancers, reducing the development of promising drugs intended to prevent cancer or treat early-stage disease.⁶ Research and development in the later cancer stages is encouraged at the expense of the enormous public health benefit of studying drugs to treat early-stage patients or to prevent cancer. Longer development timelines are also likely for an innovative drug that could treat a disease that has never had any treatments, a drug with a new mechanism of action, or a drug to prevent, cure, or slow the progression of a disease or disability.

The MODDERN Cures Act aligns incentives with the needs of patients by setting a term of regulatory exclusivity for these medicines. We defer to Congress in determining the appropriate length of the exclusivity period, as Congress is uniquely positioned to weigh competing interests and decide on an appropriate balance that reflects the current patent, regulatory, and commercialization realities for manufacturers of new medicines to treat unmet medical needs. We anticipate that passage of the MODDERN Cures Act, with a certain regulatory exclusivity protection period, will result in increased research and development into

⁵ Budish et al. National Bureau of Economic Research. Do fixed patent terms distort innovation? Evidence from cancer clinical trials. September 5, 2013. Available at: <http://www.nber.org/papers/w19430.pdf>. (last accessed June 9, 2014)

⁶ Budish et al. National Bureau of Economic Research. Do fixed patent terms distort innovation? Evidence from cancer clinical trials. September 5, 2013. Available at: <http://www.nber.org/papers/w19430.pdf>. (last accessed June 9, 2014)

medicines with the potential to prevent disease or disability, treat early-stage conditions, and address chronic conditions with long development timelines, such as Alzheimer's disease or other progressive conditions.