

**Testimony of Marc Boutin, JD, Executive Vice President and Chief Operating Officer of
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21st Century Cures Initiative Hearing - June 11, 2014

House Committee on Energy and Commerce

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Summary

More than 133 million Americans – over 40% of the U.S. population – live with a long-term disease or disability.¹ For many of them there are no treatments, and for a large percentage of people whose conditions have treatments, the current treatments do not work.² To address these unmet medical needs, we need to ensure that economic incentives and policies encourage the development of the most promising therapies.

Two major barriers currently prevent promising medicines from being developed or reaching patients: (1) a complete lack of patent protection, and (2) the lack of a predictable post-approval period of patent protection. The primary determinant of whether a product is developed should be its social utility, not the strength of its patent protection. Unfortunately, this is not currently the case, and if a medicine fails this patent protection assessment, it is routinely abandoned and left dormant.

H.R. 3116, the Modernizing Our Drug & Diagnostics Evaluation and Regulatory Network Cures Act of 2013, or MODDERN Cures Act, was drafted to remove these barriers and align economic incentives with the needs of patients by setting a term of regulatory exclusivity for the development of new drugs intended to treat unmet medical needs. For a promising product with no or uncertain patent protection, the MODDERN Cures Act would protect the medicine from generic competition for a specific period of time after FDA approval, thereby eliminating the question of whether a medicine will have sufficient patent protection from the development equation. In addition, the MODDERN Cures Act promotes increased data transparency.

¹ CDC Website, available at: <http://www.cdc.gov/chronicdisease/overview/index.htm> (last accessed June 5, 2014).

² Spear et al. Clinical application of pharmacogenetics, *Trends Mol Med* (2001) 7(5):201-4.

Introduction

The National Health Council (NHC) welcomes the opportunity to submit the following testimony to the House Committee on Energy and Commerce to explain the need to remove existing barriers to the development of promising treatments for unmet medical needs. The NHC is the only organization that brings together all segments of the health community to provide a united voice for the more than 133 million people with chronic diseases and disabilities as well as their family caregivers. Made up of more than 100 national health-related organizations and businesses, its core membership includes the nation's leading patient advocacy groups, which control its governance. Other members include professional societies and membership associations, nonprofit organizations with an interest in health, and major pharmaceutical, medical device, biotechnology, and insurance companies.

The magnitude of patient need is great. More than 133 million Americans – over 40% of the U.S. population – live with a chronic disease or disability.³ But for many people there are no treatments, and existing treatments work for only 50-75% of the patients who currently use them.⁴ There are limited treatment options for too many diseases and disabilities, including mental health ailments, neurological, autoimmune and many rare diseases, or for the prevention of various diseases and disabilities. Millions of patients struggle daily with conditions such as Alpha-1, ALS, Alzheimer's, epilepsy, lupus, mesothelioma, and multiple sclerosis. Many are waiting for a single treatment, while others wait for new and better medicines.

I am honored to mention one patient with a chronic condition – Michael Gollin. Mr. Gollin is a patent attorney. He also lives with ALS, commonly known as Lou Gehring's Disease.

³ CDC Website, available at: <http://www.cdc.gov/chronicdisease/overview/index.htm> (last accessed June 5, 2014).

⁴ Spear et al. Clinical application of pharmacogenetics, *Trends Mol Med* (2001) 7(5):201-4.

Patients like Mr. Gollin should not lose out on potentially life-saving treatments because our current system fails to address barriers to the development of new treatments.

Current policies have not kept pace with the evolution of science in the U.S. 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.”⁵

Insufficient Patent Protection Prevents Promising Medicines from Reaching Patients

Two situations currently prevent some promising medicines from being developed and making it to the market and patients: (1) a complete lack of patent protection, and (2) the lack of a predictable and sufficient period of patent protection once the medicine enters the market.

First, the best new medicines do not automatically qualify for a patent, and without any patent protection, manufacturers will not continue developing the treatments, despite their potential to treat unmet medical needs and benefit patients. In order for any invention to secure a patent, it must be deemed as novel and nonobvious. While these thresholds to receiving a patent are designed to encourage ingenuity, they have also created barriers to innovation in the drug development process.⁶

As for novelty, “a drug cannot be patented if it was previously disclosed to the public; no exception is made for when the disclosed drug has not yet been tested in clinical trials and thus

⁵ Hatch, O. William Mitchell Law Review. Accessible at: http://www.wmitchell.edu/lawreview/Volume40/40_IV.html. (last accessed June 7, 2014)

⁶ 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>

has not been approved by the FDA.”. Current law allows seemingly insignificant disclosures to undermine the novelty of drugs, which makes it easy for researchers to unwittingly disclose their discoveries. Companies [file] overly broad patent applications to establish priority over large numbers of potential new drugs. As their research advances, the companies typically disclaim most of those compounds from their applications, leaving only the prior disclosure of the drugs. Practices such as these have created a significant body of potentially valuable drugs that cannot be patented.”

Nonobviousness is defined as “A new drug with beneficial therapeutic properties is therefore considered obvious if those properties would have been reasonably expected at the time it was invented.” Obvious drugs are defined as ones 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.” The [nonobviousness] standard withholds patented protection from drugs based on the scientific advances that allowed researchers to identify them as ones that are likely to be effective.” Without MODDERN, the scope of the problem caused by the nonobviousness standard is likely to expand as scientific progress increases the likelihood of success for many products, rendering more and more of them “obvious.”

Second, the unfortunate reality is that manufacturers stop developing a drug when they believe that its patent protection will not extend long enough after the drug enters the market to allow the company to re-coup their investment. Because drug manufacturers must apply for patents very early in the research and development process, there can be little or no patent life left when the drug finally enters the market, even with patent term extensions granted through Hatch-Waxman. The longer the drug development process goes on, the shorter the patent term once the drug is approved.

This uncertainty discourages companies from pursuing medicines with long development timelines in favor of those with shorter development timelines. In cancer, for example, this leads to more research and development of drugs intended to treat later-stage cancers, which often have shorter development timelines.⁷ Conversely, the development of promising drugs intended to prevent cancer or treat early-stage disease with longer development timelines is reduced because of shorter periods of patent protection once the drugs are approved. This leads to increased research and development in the later cancer stages at the expense of the enormous public health benefit of studying drugs to treat early-stage patients or to prevent cancer. Longer development times are also likely for innovative drugs 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.

How Congress Can Help – the MODDERN Cures Act

I would like to commend Ranking Member Waxman for his courageous efforts to overhaul the pharmaceutical industry with both the Hatch-Waxman Act and the Orphan Drug Act. These laws have made a huge difference in the lives of patients. It is once again time for courageous action. We need to re-align our economic incentives and policies to encourage the development of treatments for people with unmet medical needs.

Patient advocacy organizations have already begun to address the patent protection barriers to developing new treatments by crafting a bill titled H.R. 3116, the Modernizing Our Drug & Diagnostics Evaluation and Regulatory Network Cures Act of 2013, or the MODDERN Cures Act. The bill was introduced in the House in September 2013 by Representative Leonard Lance and is currently cosponsored by 54 other Members of Congress. I want to recognize and

⁷ 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)

commend Representative Lance on his leadership and work in moving this bill forward. The MODDERN Cures Act aligns incentives with the needs of patients by setting a term of regulatory exclusivity for medicines intended to treat unmet medical needs. In addition, it encourages the development of innovative diagnostics that help identify which patients will benefit from a specific therapy. The MODDERN Cures Act aims to ensure that the most promising therapies for unmet medical needs are not shelved due to uncertain patent protection.

Specifically, the MODDERN Cures Act provides for a drug or biologic to be designated as a “dormant therapy” if it is a new medicine being studied to treat an unmet medical need. A designated dormant therapy can receive regulatory exclusivity, which protects the drug from generic competition for a certain amount of time after FDA approval. This allows manufacturers to pursue medicines that have the greatest potential to meet an unmet medical need, even if the treatment has no patent protection.

The MODDERN Cures Act also addresses the problem of uncertainty created by long, unpredictable development and review times for treatments that address unmet medical needs.⁸ The Act's provisions give manufacturers the certainty that the medicine will be protected from generic competition for a specific period of time once approved, freeing up manufacturers to decide a medicine's fate not by whether enough patent protection may exist at an unknown date in the future, but by the drug's potential to benefit patients and enhance the public's health. We anticipate that this will result in increased research and development into medicines with the potential to prevent disease or disability or treat early-stage conditions.

Finally, the MODDERN Cures Act contains the additional benefit of consistency with the increasing demand that clinical studies data be made public, i.e., a policy of enhanced data

⁸ Designation as a dormant therapy is optional. If a product has sufficiently strong patent protection, manufacturers will still be able to use the existing provisions in the Hatch-Waxman Act.

transparency. Many anticipate that increased data transparency will benefit patients by helping to eliminate unnecessary and costly duplication of clinical studies, allowing others to confirm or challenge study conclusions, facilitate learning about existing medicines, and help to inform patient decisions on treatment and physician prescribing – all accelerating research and enhancing patient outcomes. Under the MODDERN Cures Act, dormant therapies receive a set term of regulatory exclusivity, which decreases industry reliance on the use of trade secrets to protect their products. Additionally, the MODDERN Cures Act requires that manufacturers waive patent enforcement beyond the period of regulatory exclusivity, thereby creating a predictable timeline for generic manufacturers to bring their products to market. This bill contains the strongest “anti-evergreening” protections ever included in legislation.

Conclusion

The MODDERN Cures Act removes barriers to the development of products that treat unmet medical needs of people with devastating diseases that have few or no current treatments. This can benefit a great number of patients who suffer from a multitude of diseases – from mental health ailments to neurological, autoimmune, and rare diseases.

Congress has recently demonstrated its willingness to legislate needed fixes by enacting certain bill provisions incentivizing innovative diagnostics. These provisions were originally included in the MODDERN Cures Act and were enacted on April 1 of this year as part of the Protecting Access to Medicare Act of 2014. 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. I commend Congress for taking this step and strongly urge the Committee’s support of the remaining provisions of the MODDERN Cures Act.

All patients, including Mr. Gollin, who continue to wait for new treatments for their unmet medical needs, deserve a modernized regulatory system that incentivizes innovation and helps to bring life-saving therapies to the people who need them. Passing the MODDERN Cures Act of 2013 is a much-needed step to attain the goals of the 21st Century Cures Initiative.

Thank you very much.