

Karen Ignagni  
President &  
Chief Executive Officer



July 25, 2014

The Honorable Doris Matsui  
U.S. House of Representatives  
2434 Rayburn Building  
Washington, D.C. 20515

The Honorable Bill Johnson  
U.S. House of Representatives  
1710 Longworth Building  
Washington, D.C. 20515

Dear Reps. Matsui and Johnson:

On behalf of America's Health Insurance Plans (AHIP), I am writing to express our support for H.R. 3750, the "Telehealth Modernization Act," which would provide guidance to the states in establishing common standards for the delivery of health care services through telehealth.

We appreciate your leadership in addressing the needs of patients who face barriers to obtaining the health care they need. Your bill recognizes that new developments in information technology, coupled with advances in telehealth and medical science, have the potential to transform the delivery of care for consumers who otherwise must travel long distances to see a physician or, in some cases, forego needed health care services. By removing obstacles to telehealth, your legislation opens the door to improved health outcomes and a higher quality of life for these consumers.

Thank you again for introducing this bipartisan legislation. We look forward to continuing to work with you to improve access to care through innovative approaches to health care delivery.

Sincerely,

  
Karen Ignagni  
President and CEO

Karen Ignagni  
President &  
Chief Executive Officer



July 9, 2014

The Honorable Devin Nunes  
U.S. House of Representatives  
1013 Longworth Building  
Washington, D.C. 20515

The Honorable Frank Pallone  
U.S. House of Representatives  
237 Cannon Building  
Washington, D.C. 20515

Dear Congressmen Nunes and Pallone:

On behalf of America's Health Insurance Plans (AHIP), I am writing to express our support for H.R. 3077, the "TELE-MED Act," which seeks to improve seniors' access to health care by allowing licensed Medicare providers to treat patients across state lines through telemedicine.

We appreciate your leadership in proposing this important step toward improving patient care for Medicare beneficiaries, particularly those in rural and underserved areas. Your bill recognizes that new developments in information technology, coupled with advances in telemedicine and medical science, have the potential to transform the delivery of care for seniors who otherwise must travel long distances to see a physician or, in some cases, forego needed health care services. By removing obstacles to telemedicine in the Medicare program, your legislation opens the door to improved health outcomes and a higher quality of life for our nation's Medicare beneficiaries.

Thank you again for introducing this bipartisan legislation. We look forward to continuing to work with you to improve choices and access to care for Medicare beneficiaries.

Sincerely,

  
Karen Ignagni  
President and CEO



July 25, 2014

The Honorable Fred Upton, Chairman  
U.S. House of Representatives  
Committee on Energy & Commerce  
2125 Rayburn House Office Building  
Washington, D.C. 20515

Dear Chairman Upton:

These comments from the National Organization for Rare Disorders (NORD) are intended to supplement the record for the hearing entitled “21st Century Cures: Examining Barriers to Ongoing Evidence Development and Communication.”

In announcing the hearing, Chairman Pitts said: “Keeping the discovery, development, and delivery cycle of 21st Century Cures constantly improving means ensuring that each phase of that cycle can inform the next. We need to be sure that patients, providers, researchers, and drug and device companies are able to communicate and collaborate in the most productive and transparent manner possible. Patients, for example, know best the real world impact of certain treatments. Taking this information from the delivery cycle and translating it back to those working in the development phase will help ensure that the cycle of cures is constantly improving.”

We at NORD agree on the need for increased communications. There are innumerable communications issues that might be addressed, but in these comments we will focus on two:

1. The need for the patient voice to be heard in the drug development process, and
2. The need for there to be clarity regarding the communication of off-label information, since so many patients with rare diseases use products that are prescribed off-label.

NORD is a unique federation of more than 450 patient advocacy groups, members, and voluntary health organizations dedicated to helping all people with rare diseases. NORD provides resources, research, advocacy, education, community, and infrastructure support to the rare disease community that small individual organizations cannot. NORD’s support allows our member groups to focus on their primary mission, progress towards understanding, treating and curing their diseases.

In the U.S., there are more than 7,000 diseases defined as “rare,” affecting more than 30 million Americans, as well as their families and caregivers. Much progress has been made in public understanding of rare diseases but much more still needs to be accomplished. The vast majority of rare diseases are lacking an approved treatment.

The first issue is how to strengthen the patient's voice in the drug development and approval process. The Food and Drug Administration Safety and Innovation Act (FDASIA) of 2012 made groundbreaking strides in encouraging that patients play a greater role during the drug approval process. The FDA has implemented many of these changes admirably but there are various other measures contained within FDASIA that are not being implemented to the fullest extent, or not at all. We support efforts to implement existing law concerning patient involvement in the drug development process.

For example, the FDA must include a patient or patient representative on the drug review committee as mandated by section 903 of FDASIA. While the FDA has increased patient involvement in other aspects of the drug approval process, such as in advisory committee meetings, the FDA has yet to include patients on a review panel. NORD requests that FDA be required to fulfill this mandate. Rare diseases are an excellent place to start.

While the FDA has conducted several patient-focused drug development meetings, it has yet to demonstrate how it intends to use the information to inform the drug review process. While NORD appreciates the FDA's efforts in implementing the patient-focused drug development initiative, we are particularly eager for the findings from these meetings to be incorporated within the drug review process.

NORD requests that the FDA develop a guidance advising patient organizations on how they can administer their own patient-focused drug development meetings and provide data that will be useful to the drug approval process. The FDA is holding 20 patient-focused drug development meetings. The information derived from these meetings can be broadened substantially if FDA provides guidance on how patient organizations can independently conduct their own patient-focused drug development meetings in a manner that would enable the FDA to use the findings of these meetings to enhance the drug review process.

We request that patients be regarded and treated as **partners** with the FDA in the drug review process. At present, despite progress, patients are regarded as outside participants who are asked to occasionally consult on drug efficacy and effectiveness, usually under the auspices of the drug companies. Specifically, we ask FDA to standardize patient input within the drug review process. Currently, the level of patient involvement varies among review divisions. Patient contribution at regular and predictable times must be built into the process.

The second issue has to do with off-label uses of approved drugs. Many rare disease patients use drugs outside of FDA-approved uses, based on the judgment of their physicians that the drugs will benefit them and will not be harmful.

Recently, patients with rare diseases have been denied reimbursement when they fill a prescription for a drug not specifically approved for their rare disease, but which is approved for other diseases, and from which their doctors believe they will benefit. The insurers are increasingly telling patients with rare diseases that they will not be reimbursed for drugs that their physicians have prescribed for them, if the FDA has not approved the drug for that specific purpose.

The reality is that many drugs are not approved to treat specific rare diseases, even though they are safe and effective for rare disease patients, simply because it does not pay for the

manufacturer to submit an application to the FDA, or to undertake expensive studies. However, many of these drugs are generally accepted as being medically beneficial to rare disease patients, and they should be reimbursed.

At the same time, the government severely restricts what drug companies can say about new research and about off-label uses, thus cutting off information from the most knowledgeable sources. The Congress should seek new policies that permit drug companies to share appropriate information without fear of enforcement action. This will enhance patient and healthcare professional understanding of how approved drugs can be used in the best interests of patients.

We at NORD look forward to continuing to work with the Committee on this important 21<sup>st</sup> Century initiative.

For questions regarding NORD or the above comments, please contact Diane Dorman, Vice President of Public Policy, at [REDACTED] or (202) 588-5700 [REDACTED]

Sincerely,

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Peter L. Saltonstall  
NORD President and CEO



July 24, 2014

The Honorable Fred Upton, Chairman  
Energy and Commerce Committee  
United States House of Representatives  
Washington, DC 20515

The Honorable Henry Waxman, Ranking Member  
Energy and Commerce Committee  
United States House of Representatives  
Washington, DC 20515

The Honorable Joe Pitts, Chairman  
Energy and Commerce Committee  
Health Subcommittee  
United States House of Representatives  
Washington, DC 20515

The Honorable Frank Pallone, Jr., Ranking Member  
Energy and Commerce Committee  
Health Subcommittee  
United States House of Representatives  
Washington, DC 20515

The Honorable Diana DeGette  
United States House of Representatives  
Washington, DC 20515

Dear Chairman Upton, Chairman Pitts, Ranking Member Waxman, Ranking Member Pallone, Representative DeGette, and Members of the Energy and Commerce Committee:

As members of the Patient, Consumer, and Public Health Coalition, we are writing to express our views as consumers, physicians, scientists, and public health experts regarding the 21st Century Cures Initiative. We want to be supportive of your efforts to ensure that Americans have the best possible medical treatments.

Our coalition and others are concerned that the 21<sup>st</sup> Century Cures Initiative has focused on speeding approvals of medical products while paying very little attention to making sure that FDA's essential safeguards are protected for men, women, children, and regarding prenatal exposures. Proposals to substantially revise the well-established FDA standards for approval without adequate research to evaluate the likely impact will put patients' safety at risk and also presents substantial risks to small companies.

At the hearings and roundtable discussions, witnesses that brought up safety and efficacy concerns were clearly in the minority. At the July 23, 2014 roundtable discussion, Dr. Laura Koontz from the Ovarian Cancer National Alliance brought up safety concerns regarding the OvaSure test, which did not work and resulted in patients being harmed. The FDA had to halt sales of the OvaSure test because the company was “offering a high risk test that has not received adequate clinical validation, and may harm the public health.”<sup>1</sup>

At the July 9, 2014 hearing, Dr. Aaron Kesselheim voiced concerns about safety issues such as using surrogate endpoints and post-approval studies to expedite the approval of medical products. He noted that medical products approved without rigorous tests put patients at risk and lead to more products being withdrawn from the market because of safety problems or lack of effectiveness. We share these concerns and know from our experience that these safety issues regarding FDA approval deserve much greater attention than they have received at all the 21<sup>st</sup> Century Cures events thus far.

Also at the July 9 hearing, Congresswoman Lois Capps noted that current clinical trials have not been representative of the population at large with women and ethnic minorities being excluded. If clinical trials are made smaller (as is already occurring for FDA’s expedited pathways)<sup>2</sup> in an effort to expedite trials, there will be even less clinical data available to analyze the effect of medical products on significant population groups, such as women, men, and specific racial groups.

A major goal of the 21<sup>st</sup> Century Cures initiative is “to accelerate the discovery, development, and delivery cycle to get promising new treatments and cures to patients more quickly” by embracing new technologies to achieve this goal. However, new technologies are not always better. Metal-on-metal hips are the poster child for how new technology can cause more harm than good, costing Medicare and patients billions of dollars. In addition, basic research and comparative effectiveness research needs to be federally funded through NIH, AHRQ, and other agencies to make meaningful progress in health care. We should not focus on FDA alone if we want to accomplish comprehensive change.

We thank you for your efforts to ensure that patients receive the best medical care. However, we strongly urge that future hearings and roundtable discussions have a better balance of witnesses who will address how accelerated approval strategies will affect safety and efficacy of new medical products. Members of our coalition are well-qualified to provide that perspective. We look forward to working with you more closely as these discussions move forward.

National Center for Health Research  
Annie Appleseed Project  
American Medical Student Association  
Woody Matters  
Center for Science and Democracy, Union of Concerned Scientists  
The TMJ Association  
Jacobs Institute of Women’s Health

*The Patient, Consumer, and Public Health Coalition can be reached through Paul Brown at (202) 223-4000 or at [pb@center4research.org](mailto:pb@center4research.org)*

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<sup>1</sup> Food and Drug Administration (August 7, 2008). OvaSure Manufacturer Letter.

<http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/IVDRegulatoryAssistance/ucm125130.htm>

<sup>2</sup> Food and Drug Administration (May 2014). Guidance for Industry: Expedited Programs for Serious Conditions—Drugs and Biologics.

<http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM358301.pdf>