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Congressman Fred Upton
Mark Ratner, Legislative Director
2183 Rayburn Bldg.
Washington, DC 20515
mark.ratner@mail.house.gov

Congresswoman Diana DeGette
Ms. Rachel Stauffer, Health Policy Director
2368 Rayburn House Office Bldg.
Washington, DC  20515
Rachel.Stauffer@mail.house.gov

Dear Mr. Upton and Ms. DeGette:

Re: Comments on the 21st Century Cures Initiative

This comment on the 21st Century Cures Initiative is being submitted on behalf of LymeDisease.org (LDo), founded in 1989. We apologize for the lateness of response, but we only became aware of the effort this morning and we were encouraged to reply notwithstanding the fact that the June 13 deadline has passed. We appreciate your willingness to accept our comments as well as the opportunity to share our views.

LDo is a non-profit organization that represents the interests of patients with Lyme disease nationwide, and seeks to increase patient participation in all aspects of healthcare policy-making by promoting meaningful direct involvement of patients. LDo has the broadest reach of any Lyme disease organization in the nation, with state-based internet groups in every state and the most extensive communications network for Lyme patients through its website, blogs, and The Lyme Times, which is the only national print publication dedicated to Lyme disease.

LDo fosters patient engagement in research issues. Toward this end, since 2004 it has conducted surveys of the Lyme disease population to better characterize this population by soliciting information from the patients themselves. A recent survey on the topic of access to care drew over 5,000 responses from patients with Lyme disease and was published in the journal Health Policy. Our most recent survey was published in PeerJ and used the CDC HRQoL measures to assess patients’ quality of life.

The Executive Director of LDo is the Co-Chair of Consumers United for Evidence-Based Healthcare (CUE), a national coalition of over 40 patient and consumer advocates committed to evidence-based medicine. She also serves as a patient representative for the Patient Centered Outcomes Research Institute (PCORI); sits on the Steering and Executive Committees of the National Patient-Centered Clinical Research Network (PCORnet) and heads their patient council, which provides a heightened examination of big data issues concerning patients such as privacy, consent and autonomy. She has published over 40 articles in peer-reviewed journals addressing the patient perspective on Lyme disease and has played a pivotal role in conducting LDo large-scale patient surveys.

The remainder of this comment will address your questions to the patient community, one-by-one.
What is the state of discovery of cures and treatments for your disease? Are there cures and treatments now or on the horizon?

The state of discovery and treatment in Lyme disease is just emerging. We know that our diagnostic tests for Lyme disease are flawed and miss roughly 50% of people with the disease. These tests are indirect tests that measure antibody reaction to the Lyme bacteria. There is no generally accepted diagnostic test that can determine whether a patient in fact has active disease or whether treatment has been effective.\(^1\)

Early diagnosis and treatment can be very effective. However, 25% of patients are not cured by short term antibiotics and 50% or more of those patients who are diagnosed late and develop chronic Lyme disease are not cured by short term treatments.\(^1\) We also know that patients who remain ill are profoundly disabled—roughly 45% of patients have been forced to quit work and approximately 25% of patients are on disability at some point in their illness.\(^2\),\(^3\) The optimal treatment of late or chronic Lyme disease has not been determined. We do not know the best antibiotic or combination of antibiotics or the optimal duration of treatment.

Accordingly, the treatment of chronic Lyme disease is an unmet medical need.

What programs or policies have you utilized to support and foster research, such as patient registries, public-private partnerships, and venture philanthropy?

We have funded academic research of Lyme disease and other groups have been involved in venture philanthropy. Neither NIH nor CDC funding of research includes patient input, and research funding from the government has gone toward a small group of researchers who, for the most part, have not addressed patient needs nor improved their quality of life. We have also conducted and published in peer-reviewed journals large scale surveys (more than 5,000 respondents) to assess the quality of life implications of chronic Lyme disease and the barriers impeding access to care.

How can Congress incentivize, coordinate, and accelerate basic research for diseases we know relatively little about?

Congress needs to ensure that all public funding for specific disease research is driven by patient interests and that patients are involved in prioritizing the research, determining the research question, selecting the patient population included in the sample, determining the intervention to be tested, and participating in the analysis and dissemination of research findings. This approach is reflected in the patient engagement rubric developed by the Patient Engagement Advisory Panel of the Patient Centered Outcomes Research Institute (PCORI), and I had the privilege of sitting on that panel.\(^4\) Until the PCORI approach is adopted, research will have all of the carrots and sticks aligned with commercial and career interests that frequently are not aligned with addressing patient needs.

How can we work together to better translate advances in science into safe and effective new therapies for patients?

We need to get patients pulling in the direction of research. Recruitment of patients in traditional research is low; RCT's take time, are expensive and don't reflect the patient population seen in clinical practice. Explaining the patient value proposition of research to the public is important generally. Also, it is important to ensure that privacy, consent and autonomy interests of patients are adequately protected. I am currently heading the Patient Council of PCORNet, which is devoted to protecting the interests of the patient in autonomy, privacy and consent in big data trials.
How do you coordinate your research and outreach with other patients?

We have a remarkable reach in the community. Our patient surveys draw over 5,000 patients in a very short time frame. We have been engaging the Lyme disease community in patient research for over ten years. We have published two large scale patient surveys in peer reviewed journals.

How do you learn about new treatments and cures? How do you communicate with other patients regarding treatments and cures?

We read all breaking research and keep abreast of the latest science in Lyme disease as well as other infectious diseases that are analogous. We also fund research.

What can we learn from your experiences with clinical trials and the drug development process?

Lyme disease is not a rare disease. The Centers for Disease Control estimates that there are over 300,000 cases per year. However, it is a research-disadvantaged disease. This is true because all of the treatments for Lyme disease consist of generic antibiotics. The antibiotic market generally has suffered significant vacuums in the drug pipeline because the market is less profitable than so-called annuity drugs, like statins, that are taken by a very broad population over the course of the rest of their lives. We need to incentivize “cures” as opposed to management of chronic diseases over a lifetime. Presently, pharmaceutical companies are not incentivized to cure disease; they are incentivized to manage it over a lifetime. They are also not incentivized to research medications for diseases, like Lyme disease, that are capable of cure when the market does not provide sufficient profit margins.

What is the role of government in your work, including any barriers to achieving your goals and advancing breakthroughs?

The NIH and the CDC use peer-review by researchers to select grant awardees. This peer review is dominated by a small group of researchers who have received the lion’s share of research grants over the years, but have not conducted research that has improved the quality of patient care over this period of time. This is a research-centric model of grant determination. Research that does not address the needs of patients or improve patient quality of care should not be funded. Researcher testimony before Congress last year indicated that the peer review system for Lyme disease is broken and that researchers who don’t subscribe to a narrow research paradigm are unable to obtain funding. The solution to this problem is to engage patients directly in making research funding determinations, using transparent open processes, and ensuring accountability of those conducting research. PCORI has developed a patient engagement rubric for research that should be used as a hallmark of patient engagement quality.

How should regulators evaluate benefit-risk? How do you work with regulators regarding benefit-risk? Can this process be improved?

Regulators need to consider the needs of the patient population of interest. Risk-benefit determinations should be adjusted to reflect the severity of the condition. How acceptable is the status quo for the patient? For example, safety considerations should dominate the conversation when an illness is mild, such as the common cold. For more serious diseases, like Parkinson’s, the question is whether the need for treatment options outweighs the risks associated with treatment given the level of quality-of-life impairment. Are other treatment options available generally? Are they available for this patient? How impaired is the patient population—how
impaired is this patient’s quality of life? Are they able to work, engage in meaningful family and social activities? The burden of proof for evidence is much lower when the patient quality of life is impaired.

Also, is this a group decision covering all patients with a particular disease or an individual determination that takes into consideration the risk/tolerance and acceptability of the current quality of life for this patient? Individualized risk-benefit assessments respect patient autonomy better. Another factor is the need for innovation in the particular illness. Is it appropriate to centralize and standardize medical decision-making in expert bodies or is point-of-care determination necessary to promote innovation? Historically, evidence-based medicine has been a bottom-up rather than top-down process. It is far more practical to have point-of-care innovation by thousands of physicians in concert with their individual patients as a first step to determine whether it makes sense to incur the cost of a randomized control trial.

What is the role of public and private funding in the research and development of cures and treatments?

Both are necessary. Public funding should not be wasted on research that does not improve patient quality of life or address their concerns. Public funding by Big Pharma should be incentivized toward cures; otherwise, the incentive is to prolong palliative care to increase profits. Of course, cure is not always attainable. Patient organizations are incentivized to serve their patient population and improve care. However, patient organizations lack the depth of funding necessary to support the cost of research. Providing federal funding through patient advocacy would completely change the research paradigm as we know it. It would also align the public interest with those of researchers. Researchers granted funds by advocacy organizations address the concerns of the patient community. Researchers funded directly may instead pursue their own curiosity and/or pet theory.

Are there success stories the committee can highlight and best practices we can leverage in other areas?

The AIDS movement with activist patients who prompted innovation and changed research policies to address the needs of their patient population is a good example of how patients and Big Pharma can work together to find solutions to complex problems.

How have you worked with other patients to support one another?

Patient groups work together when their needs are aligned. Patient groups want cures and improved quality of life for the patients they represent. This is an aligned goal. Our organization, for example, has co-funded research with other Lyme disease organizations.

What is the financial burden of your disease? How would better treatments and cures help save money for your family and the federal government?

Lyme disease is the most common vector-borne disease in the United States. It is caused by the spirochete Borrelia burgdorferi and transmitted via tick bite. The CDC estimates that roughly 300,000 people (approximately 1% of the U.S. population) are diagnosed with Lyme Disease each year. This figure is 1½ times higher than the number of women diagnosed with breast cancer each year in the USA (approximately 200,000), and 6 times higher than the number diagnosed with HIV/AIDS each year in the USA (50,000). In its early, or acute, form, the disease may cause a hallmark erythema migrans (EM) rash and/or flu-like symptoms such as fever, malaise, fatigue, and generalized aches.

A proportion of patients with Lyme disease develop debilitating symptoms that persist in the absence of initial treatment or following short-course antibiotic therapy. This condition is commonly referred to as post-
treatment Lyme disease (PTLD) or chronic Lyme disease (CLD). It is estimated that as many as 36% of those diagnosed and treated early for Lyme disease remain ill after treatment. This means more than 100,000 more Chronically ill people each year are added to the prevalence figures. We believe the prevalence of chronic Lyme disease is currently more than one million people.

Lyme disease is a costly illness. Currently many insurers are denying care to patients based upon the treatment recommendations of the Infectious Diseases Society of America. This means that patients must go out of pocket to afford healthcare that they need. Our survey of over 5,000 patients with chronic Lyme disease found that patients incurred high out-of-pocket expenses compared with other diseases. The percentage of chronic Lyme disease patients spending in excess of $5,000 in out-of-pocket costs was 46% compared to 5% in the general population. Compared to the general public, on a yearly basis, chronic Lyme patients have a) five times more physician visits, b) two times more trips to the ER and overnight hospital stays, and c) 6 times as many home care visits. When insurers deny coverage of these costs, they are borne by the individual, their families, and ultimately, society. Approximately 45% of patients have had to quit work and 25% have been disabled at some point in their illness. These costs are reflected in reduced societal productivity and loss of tax revenue from lost wages.

How can Congress help?

Congress can and should require that federal funds expended for research address the needs of patients and that true patient engagement in research is a necessity for federally funded grants. We have appreciated this opportunity to provide comments on this important topic. If you have any questions or comments, please contact me.

Very truly yours,

Lorraine Johnson, JD, MBA
Executive Director

LymeDisease.org
PO Box 1352
Chico, CA 95927