DUCHENNE ALLIANCE
68 EVERGREEN ST SUITE 2, KINGSTON, MA 02364

July 7, 2014

Dear Chairman Upton and Representative DeGette:

On behalf of the *International Duchenne Alliance*, consisting of 42 global Duchenne Muscular Dystrophy organizations representing nearly 300,000 Duchenne patients and families worldwide, we thank you for taking a special interest in all rare disease and beginning an initiative to identify faster pathways to patient access to therapies.

Duchenne is the most common lethal genetic disorder of children worldwide. It is a progressive neuromuscular disorder affect mostly boys that causes the loss of muscle function and independence. Those affected by Duchenne lose the ability to walk between the ages of 8 and 12, require respiratory support by their late teens and survive only into their twenties. While there are promising therapies in various stages of clinical trials, without FDA approval of new treatments, all will die.

The *Duchenne Alliance* is a rapidly growing group of independent Duchenne organizations dedicated to advancing each organization's mission to improve quality of life, care, and treatment of those affected by Duchenne. The *Duchenne Alliance* works to educate and provide support worldwide to those who are affected by the disease. The members of the Alliance share resources and help speed progress in the areas of research, treatment and care. The Alliance continues to seek out new research through the *Duchenne Dashboard*. To date, the *Dashboard* has funded over 30 research projects and provided grants totaling about $8 million.

Over the past several years, members of the *Duchenne Alliance* – parent advocates and supporters – have had the unique experience of dealing first-hand with the FDA as it works to implement the mandates set forth in the FDA Safety and Innovation Act (FDASIA) as they relate to new treatments to stop the advance of Duchenne Muscular Dystrophy. We believe our perspective may be useful to the Committee as you consider how best to ensure soonest possible access to safe and effective therapies for

the treatment of disease. We would be happy to discuss our thoughts and our experience further at your convenience.

Encouraging Flexibility and Innovation at the FDA

Duchenne is a rare and deadly childhood disease for which there is currently no FDA-approved treatment that will effectively halt or even slow its progress. All Duchenne patients share one common element: a genetic mutation that prevents the body from producing dystrophin, the protein necessary to sustain muscle function. The cause of this mutation is a faulty or missing exon, a segment of the DNA or RNA molecule that contains the necessary information coding for production of dystrophin.

However, not all Duchenne patients have same broken or missing exon. In fact, the single largest segment of boys have flaws in exon 51… but that group represents about 13% of the Duchenne population. There are multiple flawed exons that result in Duchenne, each with a smaller and smaller impacted population.

The FDA is currently evaluating a drug called eteplirsen which in over two years of trials has shown that it effectively causes the body to "skip" exon 51 and therefore produce sufficient dystrophin in the treated population to halt the progress of the disease. Importantly, the drug has proven to be safe, with no negative side-effects and no reported adverse events.

The FDA has finally provided guidance to the drug's manufacturer for moving forward with this drug.

However, at the same time, the FDA is suggesting that it may require placebo controlled trials for succeeding drugs that will "skip" other exons. It is our position that the FDA should not require placebo controlled trials where the safety and efficacy of a drug to treat a deadly disease has been demonstrated in earlier trials and where there is no alternative effective FDA approved treatment.

- It is unethical to require dying boys to forego effective treatment when we have sufficient natural history data that demonstrates what happens to boys when they are not treated: their bodies do not manufacture dystrophin and, eventually, the boys die.
- It is unnecessary to require placebo controlled trials even in successor drugs when the mechanism of action is the same for all… the drug simply "skips" a different exon.

- It is unreasonable to expect patients and their families to undergo painful, dangerous and repeated muscle biopsies when being "treated" only with a placebo.

Wednesday, July 9, 2014 marks the second anniversary of the signing of The FDA Safety and Innovation Act (FDASIA). The law was passed to, among other purposes, provide the FDA with the flexibility it needs in order to allow faster approval for new treatments for rare disease in order to ensure earliest possible patient access. However, FDA is, by design and culture, a risk-averse organization, slow to adopt new procedures and approaches to drug approval.

It is vital that FDA leadership – specifically Dr. Hamburg and Dr. Woodcock – know that legislators support and expect the FDA to adopt innovative approaches to evaluating data, that Congress expects FDA to be creative in seeking ways to help as many patients as soon as possible, and that members of congress will “have their back” as FDA works to implement FDASIA’s charge to be open to new approaches to trials and evaluation of data, especially when dealing with diseases like Duchenne.

Accelerated Approval Should Be Default for Treatments for Rare Disease

When evaluating a potential compound, the first question asked should be “Is this drug treating a rare and unmet medical need in a population that will otherwise suffer and/or die without treatment?" If the answer is yes, then the FDA must look for ways to move quickly. The FDA has a specific pathway -- accelerated approval -- to quickly evaluate and make new treatments available to patients even as additional data is collected and evaluated.

Since 1992, only nineteen drugs have been approved under accelerated approval in the rare disease space. The vast majority of treatments approved have been for treatment of cancers and HIV. Yet FDASIA was passed and signed into law specifically to give FDA the tools and flexibility necessary to develop pathways for accelerated approval of treatments for rare disease.

Where a new drug meets the criteria set forth below, FDA should work with the sponsor to enable primary consideration under the accelerated approval pathway:

- Section 901 of the FDA Modernization Act of 1997 (FDAMA) as amended by the FDA Safety and Innovation Act (FDASIA) creates a clear pathway and sets criteria for treatments for rare diseases to receive accelerated approval;
- The second criteria, after demonstration that the disease is rare and that there is no FDA approved effective treatment, is that a “surrogate endpoint” needs to be

identified that is reasonably likely to predict clinical benefits to prospective patients;
- The law states that safety data must be carefully weighed on a risk vs. benefit analysis, taking into account the needs and viewpoints of patients;
- Further, Section 902 of the law directs that clinical trials for these drugs be both small and efficient by "minimizing the number of patients exposed to a potentially less efficacious treatment." Clearly, FDASIA contemplates clinical trials of relatively small sizes and assumes the FDA will be flexible in considering new approaches to measuring efficacy.

We believe it is vital that all treatments for rare disease -- especially those impacting children where there is no existing effective FDA approved treatment -- are measured first against the accelerated approval pathway.

Increased Transparency and Communication across All Stakeholders

Too often, throughout the evaluation and approval process at FDA, key stakeholder organizations are put off, stopped in their tracks when they are told that communications between FDA and sponsoring organizations are confidential. Patient groups, families, physicians, researchers -- even legislators and regulators -- must rely on communications from the sponsoring company in order to decipher what is happening. These company communications are governed by securities industry regulations and must meet very specific requirements designed with the investor in mind.

The Securities and Exchange Commission should <u>not</u> be defining how, when and what FDA communicates to all stakeholders. It is essential that the FDA be given increased latitude to communicate with all stakeholders regarding its evaluation of specific drugs or classes. While recognizing that there is a duty to ensure proprietary information is not divulged, under the current system, the FDA is denied the potential benefit of knowledge and experience from multiple stakeholders. As it stands now, those with the largest personal stake in the outcome – patients and families – are left out of substantive discussions.

Accountability and Oversight

It has been two years since President Obama signed the FDA Safety and Innovation Act. Among the stated purposes of the law is to improve access to the Accelerated Approval pathway for rare diseases. However, as referenced above, there have been very few drugs outside of cancer and HIV that have been approved via this pathway. While many have speculated as to the reasons for this, it seems clear that Congress must require additional accountability from FDA and must provide enhanced oversight

in order to ensure FDA is complying with the mandates set forth in FDASIA. We suggest the FDA be required to:

- **Create a Rare Disease Patient Advocate** -- FDA scientists and researchers are among the most accomplished in the world. But even they would admit that there is much they do not know, especially in the area of rare diseases. Further, as researchers, they are missing the regular input and advice of the most important stakeholder group: patients. We recommend that the law be amended to require the appointment of a Rare Disease Patient Advocate within the FDA, an individual -- similar to an ombudsman -- who will be empowered to represent the voice of patients and families dealing with specific rare diseases, who can bring insights and perspective from patient experience that may not be otherwise available to FDA, who serves as a bridge between the FDA and patients.

- **Form a Rare Disease Advisory Committee** – To ensure FDA has access to the latest thinking in the field, create an Advisory Committee focused specifically on rare disease. This committee can help FDA set priorities for review based on severity of disease, and risk/benefit. Such a committee might include experts in several disease areas that could help create models that are acceptable to all that move drugs through approvals faster. The committee should also involve patient and advocacy groups and have an experienced project manager(s)/liaison at the helm.

  The committee and the project manager should be charged with evaluating every part of the process (ethics, protocols, contracts, IRB's), from the sponsor, the FDA, the CRO and the patients - to determine areas that can be streamlined and more efficient. Presently, this comes in the form of "consultants," who may be previous FDA officials, CMO's or CEO's or industry experts and in the latest example, from community advocates. The committee and project manager/liaison would facilitate all the necessary communication and examination of evidence that would ensure the most timely and proactive action, becoming a true liaison between the major stakeholders.

The Duchenne Alliance, its member organizations, it supporters and those for whom we advocate - patients and families struggling with horror that is Duchenne Muscular Dystrophy -- are very grateful to you and the bi-partisan membership of the Committee for taking on this vital issue. The FDA has an enormously challenging task as it seeks to fulfill its mission to ensure the safety and efficacy of drugs and devices. We are convinced that congressional leadership, oversight and encouragement has been and

will continue to be essential to ensure the FDA adapts to a rapidly changing research, development and treatment landscape, especially in the area of rare disease and unmet medical need.

Thank you for your consideration of our recommendations.

Respectfully Submitted,

The Duchenne Alliance

Christine McSherry	Steve Dreher	David Schultz
The Jett Foundation	*Hope for Gus*	*Ryan's Quest*

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July 9, 2014

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Energy & Commerce Committee
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Washington, DC

Diana DeGette, Ranking Member
Energy & Commerce Committee
2322A RHOB
Washington, DC

RE: 21st Century Cures Initiative – A Patient Advocacy Perspective

Dear Chairman Upton, Representative DeGette and Committee,

Thank you for presenting us the opportunity to submit comments on the 21st Century Cures initiative. We look forward to working with you and your staff to develop a better understanding of the needs of the Lyme Community, with a special emphasis on the health and welfare of the patients we serve.

Founded in 1991, the national Lyme Disease Association (LDA) is the only national volunteer run 501-C (3) dedicated to finding a cure through scientific research, with 34 peer-reviewed journal publications to date acknowledging LDA, while educating the public and physicians (15 continuing medical education conferences to date), and improving the quality of life for those affected by Lyme disease. To help achieve our goals, the LDA partners with key government, industry and academic stakeholders across the country as well as with other tick-borne diseases groups.

In the spirit of collaboration, we continue to work toward the day when we will have conquered the challenges presented by Lyme and tick borne diseases by: a.) assisting with the funding for research and development of accurate tests for all stages of disease, b.) developing safe and effective preventive measures designed to reduce cases of tick- borne diseases world-wide c.) making available to all affected the necessary and much desired gold-standard treatment protocols.

Lyme disease is the most common of all vector-borne infections, with the CDC recently estimating 300,000 new cases occurring per year in the United States and acknowledging sudden deaths due to Lyme carditis as a serious problem requiring more attention. With the increase in Lyme cases, problems due to poor diagnostics and ineffective treatments for Lyme disease have become overwhelming- affecting larger numbers of people over longer periods of time.

Many patients are suffering and understandably angry because progress in addressing Lyme disease has been impeded over the past several decades by entrenched bias and a lack of accountability in the science of tick-borne diseases. It is critical that we identify these biases and impediments that are constraining the science and open up the dialogue to honest and transparent debate.

The scientists who have long been marginalized, the treating physicians who have been intimidated and threatened, and most importantly, the sick patients and their families need and depend on our help.

We agree with the Committee that the goal of accelerating the cycle of discovery, development, and the delivery of promising new treatments and cures is shared by many, but most importantly by the patients and their families. Therefore, we submit the following on behalf of patients and the dedicated volunteers assisting them across the entire United States of America.

Portions of the following are excerpted from "Patient Perspectives" published in the Congressional Record—Extension of Remarks, September 29, 2010, as submitted by Congressman Christopher H. Smith (NJ) on behalf of the national Lyme Disease Association, Time for Lyme (now LRA), and the California Lyme Disease Association (Now LDo). Additional material has been added and updates made to specifically address the submission requirements for the ***21st Century Cures Initiative – A Patient Advocacy Perspective****.*

PATIENT PERSPECTIVES ON RESEARCH GAPS IN TICK BORNE DISEASES

In Lyme disease, there are two distinct disease paradigms, with advocates of each paradigm providing science to support their claims.

One paradigm views the disease as ``hard to catch and easy to cure" and denies the existence of chronic Lyme disease─persistent infection with *Borrelia burgdorferi*, the spirochete that causes the disease. Under this paradigm, the state of the science for patients with chronic or post-treatment Lyme disease is closed.

Under that first paradigm, any treatment is considered too risky because practitioners are unable to determine the cause or extent of patient symptoms, or they view the symptoms as insignificant and write off the patients' complaints as psychiatric in nature. This leaves seriously ill patients without any viable therapeutic avenues. It also shuts the door on future research necessary to get patients to a state of wellness.

The alternative paradigm says that the science is too unsettled to be definitive, and there can be one or more causes of persistent symptoms after initial treatment in an individual who has been infected with the agent of Lyme disease. These causes include the possibility of persistent infection, or a post-infectious process, or a combination of both, with the Lyme bacterium itself driving the autoimmune process. This paradigm allows doctors the ability to exercise their clinical judgment and provide therapies that are helping their patients.

Patients with Lyme disease need a research agenda that reflects outcomes that matter to patients, namely effective diagnostic tools and effective treatments that restore them to health. The reason there are two disease paradigms in Lyme disease is because central pieces of the puzzle are missing or are inadequate.

The first area of concern involves testing. There are no reliable biomarkers of the disease.\1\ Current diagnostic tests commonly used do not detect the spirochete that causes Lyme disease, rather, they detect only whether the patient has developed antibodies to the pathogen. Antibody production, if it registers on the tests at all, takes weeks to appear, thus rendering the current tests ineffective in the earlier and more easily addressed stage.

Additionally, the Lyme antibody has been shown to form a ``complex" with the bacterium itself--and tests cannot detect ``complex" antibodies. Once triggered, antibody reactions may remain long after an infection has been treated, also clouding the diagnostic and treatment picture.

The two-tier testing system endorsed by the Centers for Disease Control and Prevention (CDC) is very specific for Lyme disease (99%), so it gives few false positives. But the tests have a uniformly low sensitivity (56%)─missing 88 of every 200 patients with Lyme disease. By comparison, AIDS tests have a sensitivity of 99.5%─missing only one of every 200 infected patients.\2\ Sensitive AIDS tests were developed less than 10 years into the disease, while archaic Lyme tests remain unreliable 35 years later.

There is a critical need for research exploring newer technologies such as polymerase chain reaction (PCR), which is used with many other diseases, and cutting-edge proteomics. Strain variations and co-infections with other organisms, often transmitted by the same tick bite, obscure the diagnostic picture further.

A vast number of strains of *Borrelia burgdorferi* have been identified and new ones continue to be discovered. Variation in strains may cause differing symptoms or severity of symptoms as well as determine the appropriate antibiotics and duration of treatment needed to clear the infection.\3\

Different strains may also express different proteins. Preliminary research shows that proteins need to be examined to find the ones most often expressed, then using microarray technology, doctors may be able to diagnose patients using a chip which contains the proteins.

Research is needed concerning the role of mutation on persistence. Some research indicates that bacteria can exchange genetic material, probably contributing to its ability to invade different systems in the body—some may have a proclivity for the heart muscle, others for the brain, and some for muscles and joints. By exchanging genetic material, bacteria may be able to form a symbiotic relationship to avoid detection by the immune response or to further invade the body.

To date, every NIH-funded treatment research study has been designed using the inadequate diagnostic test results as part of the entry criteria. The entry criterion in these studies excluded the vast majority of Lyme patients and created sample sizes too small (less than 220 patients to date) to detect clinically important treatment effects or generalize to the clinical population.

The IDSA treatment guidelines, which are central to Lyme disease controversies, are significantly based on four randomized controlled clinical trials on post-treatment Lyme disease. The published guidelines state that "antibiotic therapy has not proven to be useful," "American trials have demonstrated that additional prolonged antimicrobial treatment is ineffective in Post Lyme Disease Syndrome;" and "studies of prolonged antimicrobial treatments of patients with Post Lyme Syndrome have not shown sustained benefit."

Many have analyzed the four clinical trials and found them to be underpowered and to lack generalizability outside of the actual patients in the trials. A biostatistical review of the four published clinical trials evaluating antibiotic retreatment was conducted, focusing on trial design, analysis, and conclusions. The biostatistical analysis found that design assumptions in two trials and in two outcomes of a third trial were unrealistic and the trials were likely underpowered to detect clinically meaningful treatment effects. In addition, claims by IDSA, CDC, and NIH of no benefit have been adamantly rejected by several individuals who have analyzed the data for a primary outcome of one trial and for the results of another trial. The primary investigator of the most recent trial also has updated his findings, finding even more strongly that there has been significant benefit.

The IDSA, NIH, and CDC have repeatedly referred to the clinical trials as showing that longer-term treatments have no benefit, while omitting any reference to the design problems of two of the trials and some of the outcomes of a third trial. In an editorial last year, a CDC official simply dismissed concerns of Lyme "advocates" about the design and interpretation of the trials, saying that all trials have problems – implying inaccurately that these may be only minor glitches that do not affect the validity of the trials and the conclusions. CDC and NIH have both referred to the trials in presentation to staff of the Energy and Commerce Committee without any recognition that there are concerns about the design and conclusions of the trials. IDSA has referenced the trials as pivotal evidence in several letters to Congress, including letters to the Energy and Commerce Committee.

It is critically important to recognize that the IDSA and NIH and CDC have used these clinical trials to apply a level of certainty on the science that far exceeds the design limitations of the studies, including the small sample sizes.

Another problem is that Lyme has not attracted industry funding for treatment approaches, which places the disease at a considerable research disadvantage. To detect clinically relevant treatment effects requires much larger treatment trials, the types of trials often requiring industry funding, with sample populations that reflect those seen in clinical practice.\4\

One thing that past research has demonstrated is that patients with Lyme are a heterogeneous population. Hence, the course of illness and responsiveness to treatment may vary depending on the duration from onset of the disease to its diagnosis and treatment, the presence of co-infections, comorbid factors, other genetic characteristics of the patients, and the virulence of the strain(s) with which the patient is infected. Research sample populations must reflect those seen in clinical practice to yield clinically relevant results.

Research on the pathophysiology of Lyme disease is necessary. Research projects need to be designed which determine the course of the disease from inception, and which utilize treatments that effectively interfere with the mechanisms that allow the infection to persist. Little government sponsored science has been dedicated to the effects on persistence of the different forms of the Lyme bacterium (cyst vs. flagellar), the role, if any, of biofilms, sequestration of the organism from the immune system, the exchange and mutation of genetic material of the spirochete, and the role that components of the bacterial genome may play in protecting it from eradication by the immune system or antibiotics.

Understanding the pathology of the organism can greatly enhance targeted diagnostics and treatment modalities. Patients also need studies that explore a range of treatment options. The ideal antibiotics, route of administration, and duration of treatment for any stage of Lyme disease are not established. No single antibiotic or combination of antibiotics appears to be capable of completely eradicating the infection in all patients, and treatment failures or relapses are reported with all current regimens, although they are less common with early aggressive treatment.\5\

Treatment failure rates suggest the need to re-examine the effectiveness of the currently recommended monotherapy as a treatment approach. Studies need to explore combination treatments and longer term treatment regimens, which have been critical to the successful treatment of AIDS and tuberculosis. Patients need the type of outcomes research advocated by the Institute of Medicine to examine how well treatments are working in actual clinical practice.\6\

As presented during the 2010 Lyme conference at the Institute of Medicine, many Lyme researchers state that we desperately need additional clinical trials of many types, including long-term treatments – which have clearly been under-investigated. Although there have been only a small number of clinical trials on treatment of Lyme disease, and those clinical trials, as noted, have serious limitations and have been misrepresented, prominent individuals at HHS have recommended against doing further clinical trials of long-term treatments on the flawed premise that the trials and the science have already been done.

In a 2008 published article, an NIH Lyme disease researcher states that "At this point, the overwhelming evidence shows that prolonged antibiotic therapy, as tested in the clinical trials, does not offer lasting or substantive benefit…. Therefore, it is time to move forward to test other approaches that may help patients."

Setting aside the fact that the claim of no benefit in the clinical trials is inaccurate, using a small number of under powered clinical trials with relatively short treatment periods, with some serious design flaws, and lack of generalizability to recommend no further study is almost incomprehensible. Furthermore, it is hard to imagine why a clinical researcher would recommend abandoning a major avenue of treatment when there are numerous, major and relevant aspects of the bacteria's known physiology which have not been adequately accounted for in clinical trials conducted to date and when there are many unknowns in that pathophysiology.

While not all patients with chronic/persistent Lyme disease have returned to a state of wellness, many have, and we need to find out how and why. This information can then be applied to other patients and used to establish a research agenda for treatment that has a likelihood of success, rather than abandoning patients based on limited treatment trials.

With few exceptions, Federal agency processes over the past two decades have not allowed these research ideas to be heard in an unbiased and transparent fashion with balanced divergent viewpoints. The primary Federal health agencies with Lyme disease activities have been closely aligned with the Infectious Diseases Society of America (IDSA), a medical society that has a known bias against post-treatment persistent or chronic Lyme disease diagnosis and treatment. Major stakeholders in some fashion (including patients and treating physicians) have not been brought into the process and much deliberation has been and continues to be conducted behind closed doors.

From a research perspective, strongly held paradigms can create a closed loop, and experiments may be designed, implemented and interpreted to support a particular viewpoint.\7\ The antidote to bias is to balance scientific perspectives and to ensure that all scientific viewpoints are being heard and explored.

Given the extraordinary stream of federal funding granted to researchers who support the closed paradigm which was created and is supported by the IDSA and their vested interest in maintaining the status quo, it is not reasonable to expect this group of researchers to serve as neutral arbiters of scientific debates over competing scientific paradigms.

Furthermore, Lyme related panels dominated by IDSA have time and time again excluded opposing viewpoints from participating or controlled the review process to ensure outcomes that reinforce the IDSA closed paradigm.

Further, they claim that the state of the science is sufficient to determine with certainty that chronic Lyme disease does not exist, is not treatable with antibiotics, and that no further research on this topic is needed. Sample size affects the strength of the conclusions that may be drawn from them: ``Providing definitive answers in the face of

low event rates and small-to-moderate treatment effects necessitates sample sizes in the thousands or tens of thousands…. Funding for such mega-trials is very limited, and is often restricted to industry sources." \8\

For that reason, the Connecticut Attorney General antitrust investigation into the development process of IDSA Lyme guidelines found exclusionary practices and suppression of divergent viewpoints on the part of IDSA panels that crafted IDSA 2000 and the 2006 Lyme disease guidelines. Although IDSA settled the investigation with the Attorney General by agreeing to review its guidelines with a panel without conflicts of interest, the control of the process was in the hands of IDSA, which again selected a panel consisting almost exclusively of IDSA members and excluding treating physicians who held divergent viewpoints.

Patients need processes to occur in a transparent manner, without bias, and with the participation of all stakeholders. Patients want research which will restore their health. Their voice and the voice of the clinicians must be given the necessary weight to legitimize the research agenda and the research process.

Truth in science can be achieved through open debate in an independent process free from bias and conflicts of interest. The scientific process fails when one side of a debate controls the arena and sets the rules to ensure that its viewpoint prevails.

Thank you for your time.

Sincerely,

[redacted]

Patricia V. Smith

President, Lyme Disease Association,Inc.

ENDNOTES from Patient Perspectives published in Congressional Record 9-29-10 written by: Lorraine Johnson, JD, MBA, Chief Executive Officer, California Lyme Disease Association. Patricia V. Smith, President, Lyme Disease Association, Inc. Diane Blanchard/Deb Siciliano, Co-Presidents, Time for Lyme, Inc.

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\8\ Guyatt GH, Mills EJ, Elbourne D. In the era of systematic reviews, does the size of an individual trial still matter. PLoS Med, 2008; 5:e4.

The Honorable Fred Upton
Chairman

The Honorable Diana DeGette
Ranking Member

Committee on Energy and Commerce
2125 Rayburn House Office Building
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21st Century Cures Initiative – Incorporating the Patient Perspective

Dear Representatives DeGette and Upton, the Committee and Staff,

The volunteer members, patient advocates and supporters of the American Lyme and Tick Borne Disease Foundation, Lyme Disease Education and Support Groups of Maryland, Maryland Lyme, Virginia Lyme, Florida Lyme, Children with Lyme Support Network and our Treat The Bite educational program wish to thank you for the opportunity to submit comments for the 21st Century Cures- Patient Perspectives initiative.

I'm sure you will be pleased to know, unlike some disease oriented groups with sincere problems facing them, we are NOT asking for more money. None! In fact, if you can find it in your heart to listen, do your research and correct some of the shameful, wasteful, ongoing situations you may find funding that can help additional patient groups in need.

Brief History- Since 1986 our groups and individual volunteers have dedicated themselves and their personal resources to educating the public and health care professionals concerning the prevention, diagnosis and treatment of Lyme and tick borne disease. Many of them have been negatively affected and/or have family members who have been suffering from improperly diagnosed or inadequately treated Lyme infections. Their motives are simple. They do not want others to suffer as they have done, and hope by educating people this can be avoided.

As a true all "grass roots" effort, originally initiated by the mother of a multiply infected tick borne disease patient, we receive no federal, state or local funding to carry out our mission. We work daily to educate and improve a Lyme patients quality of life, while also assisting Lyme-related groups across the country with various Lyme-related projects.

For the decades of hard work we've invested in this cause, our people have been publicly referred to by a National Institutes of Health (NIH) Lyme Program official as:

> "Disorganized, antagonistic, vindictive, back-biting, fratricidal groups, conspiracy nuts, and any number of certified mental patients acting as self-appointed Movement spokespersons. Now they just may have provided the medical community with a legitimate reason for considering "chronic" Lyme disease as a psychiatric manifestation." [NIH Lyme program- McSweegan]

Efforts to educate the public concerning Lyme disease prevention and updating citizens on a regular basis concerning the science as it evolves has also been hampered over the years by federal agency employees.

> "It's been a busy week in LymeLand. There must have been some kind of nuthouse furlough recently because three Lymee wackjobs have just dumped a load of nonsense into the Internet, which in all fairness is basically what the 90% Internet is—a digital landfill for the mentally ill, the conspiracy-minded, the juvenile, and the criminal." [NIH Lyme program- McSweegan]

Health care professionals who have dedicated their lives to treating the sickest of the Lyme and tick borne disease (TBD) patients, many true life-savers by all accounts, have also been targeted, publicly disparaged and reported to medical boards for using advanced treatment protocols based on science, after the standard protocols failed their chronically ill Lyme patients, to prevent worsening illness, disability and death.

Some people supporting the federal agencies Lyme programs and recommendations and receiving research funding are so anxious to crush the competition they have resorted to volunteering their services to places like the CT Department of Health, who they hope will assist them in destroying others. Dr. Lawrence Zemel in Connecticut, for example, in a letter dated 9/14/93, is offering advise on how to ruin a Lyme treating doctor's reputation and shut down their practice. This would cost the unsuspecting doctor tens of thousands to millions of dollars in legal fees, with a possible additional loss of his/her license.

To do something so despicable would require sneaking in another doctor's office and faking an illness to determine if the doctor is following the federally supported Lyme disease guidelines. In the spirit of true cooperation, he advises the CT Department of Health staff to:

> "Have one of your staff investigators pose as a patient, complete with vague symptoms and negative Lyme results but insisting she have Lyme Disease. I would be happy to rehearse that investigator."

> "Examine insurance records of some of the major carriers in the state to see if he is a consistent outlier in terms of duration of home intravenous therapy."
>
> "Examine records of patients treated over the past few years for Lyme Disease to see if they truly fulfill established criteria [federally sanctioned] for Lyme Disease." [Lawrence Zemel, CT]

Ironically, for all their efforts, just last month the antibiotics promoted for use by the NIH, CDC and other federal and state agencies (who have been under pressure by the CDC and its partners to tow the federal line) were confirmed by a Johns Hopkins study to be less effective for Lyme persisters (biofilms, cyst and L-forms, particles and spirochetes) than the standard insurance-friendly antibiotics typically prescribed- Doxycycline and Amoxicillin. The study, confirming what many in the clinical setting realized years ago and what has been used by experienced Lyme-treating doctors, described 165 different FDA-approved antibiotics that are more effective for Lyme than the federal agency supported protocols.

The inability of federal agencies to control or interfere (as much) with patient-funded studies has allowed the science to advance much further and faster, and has been more definitive and useful in actual clinical patient care than the government controlled studies costing millions more dollars. Still, to retain control of its assets and keep government money flowing in the friends of federal agencies direction, NIH and other federal employees and their associates have been behind a two decades old smear campaign, as demonstrated by NIH employees comments.

> "... and these LLMDs and ILADS guys are charlatans and quacks, and are a general threat to the health and wealth of their patients. ... he's just as delusional as the chronic Lyme activists." [NIH Lyme program- McSweegan]
>
> "Below is a list of conference "faculty" for an upcoming meeting of the ILADS, a group of like-minded quacks preying on people who think they have a chronic, incurable bacteria infection otherwise know to the saner world as Lyme disease. If the roof of the conference room was to fall in, it would put an end to a large amount of quackery in the U.S., save many people from financial ruin, and lessen the workload of numerous state medical licensing boards." [NIH Lyme program- McSweegan]
>
> "And speaking of the current ILADS president, here's part of a letter he just fired off to the IOM about their ongoing study of Lyme disease. It's really an amazing compilation of lies. Maybe

> it's the result of treating so many people who think they have a chronic infection that can't be eradicated except by walletectomy." [NIH Lyme program- McSweegan]
>
> "So what do they talk about year after year? Bill padding? How to hide cash payments? Property values in the Caribbean? How to get your patients to pay your legal fees through Internet-solicited defense funds?" [NIH Lyme program- McSweegan]
>
> "More tautologic nonsense." "Again, more nonsense from a psychiatrist wanting to practice infectious diseases. … Doctors (and quacks) are in control….right up to the point where they kill, injure or rob someone." [NIH Lyme program- McSweegan]

Members of Congress who have reached out in the past to assist chronically ill Lyme patients have also been publicly targeted in this fashion, as can be seen in the NIH comments below. Although the federal agencies continue to bite the hand that feeds them, to the patients dismay Congress continues to steadily feed them, and feed them to the exclusion of others and with seemingly no over sight and no regrets.

> "Control of infectious disease research now passes from medical experts to a vast lumbering bureaucracy and an ignorant, but easily frightened and confused Congress." [NIH Lyme program- McSweegan]
>
> "No, I think Blum [Attorney General Richard Blumenthal, CT] has demonstrated that he's just another crooked pol … He's a media-addicted clown who will temporarily pursue any cause for a few minutes of television time. He's a bad politician and a bad lawyer." [NIH Lyme program- McSweegan]
>
> "She sounds a lot like the equally agitated, white, blue-collar, unemployed people who show up at tea party rallies to foam at the mouth…" [NIH Lyme program- McSweegan]
>
> "Grassley may have other motives; people in Washington usually do." [NIH Lyme program- McSweegan]
>
> "Boy, this … character is a real wackjob. She can't seem to get anything straight. She may be the Sarah Palin of LymeLand." [NIH Lyme program- McSweegan]
>
> "I doubt Dick Blumenthal has much more time for this nonsense; he's busy losing a Senate race in Ct. Still, the idea of calling, faxing or emailing him is tempting. Frankly, I'd like

to tell him—as Jon Stewart often tells Fox Noise—to go f*** himself. Though I suspect all I'd get for my trouble would be a long, citation-filled deposition stating why he cannot comply legally, morally, or anatomically." [censored by masking foul language ***- NIH Lyme program- McSweegan]

"Congressman Chris Smith (R-NJ) must have a lousy staff. He's hosting a forum on Lyme disease and other tick-borne infections for his constituents this week." [NIH Lyme program- McSweegan]

"So, yes, I think the new Administration, the new congressional committee chairs, the new OSTP staff, science advisors such as former NIH Director Harold Varmus and incoming FDA Administrator Peggy Hamburg are very aware of the "serious conflicts and scientific misstatements" characteristic of Lyme activists and their quack physicians." [NIH Lyme program- McSweegan]

"Secondly, the panel was selected by the IDSA and that sleazeball lawyer in Ct. now trying to become a sleazeball senator." [NIH Lyme program- McSweegan]

"Still, Dick Blum must be in the news....even if he has to make up the news. What next? The alleged Tampon shortage? Over-priced bagels in East Coast grocery chains?" [NIH Lyme program- McSweegan]

"Fortunately, they can't seem to keep their diabolical plots from leaking out, not to the NYT or a congressional committee, but to middle-aged mental patients who sit around on the Internet all day looking for fantasies to blame for their personal problems. ... Get off the Internet and get some therapy." [NIH Lyme program- McSweegan]

"Connecticut is a state endemic for Lyme disease, Lyme activists, Lyme quacks, and foolish local politicians willing to take up the banner of "chronic" Lyme disease on behalf of their deluded, but voting, constituents. It's a state with a near perfect mixture of bad medicine and bad politics." [NIH Lyme program- McSweegan]

Unfortunately, Senator Blumenthal, while still serving as the CT Attorney General, was targeted in a much more harsh fashion. On May 1, 2008, after conducting a lengthy investigation into the IDSA's Lyme disease guideline development practices, which were originally and are still

supported by the federal agencies, he determined and reported in part:

> "My office uncovered **undisclosed financial interests held by several of the most powerful IDSA panelists**. The IDSA's guideline panel improperly ignored or minimized consideration of alternative medical opinion and evidence regarding chronic Lyme disease, potentially raising serious questions about whether the recommendations reflected all relevant science."
>
> "The IDSA's 2006 Lyme disease guideline panel undercut its credibility by allowing individuals with financial interests -- **in drug companies, Lyme disease diagnostic tests, patents and consulting arrangements with insurance companies** -- to exclude divergent medical evidence and opinion."
>
> "The IDSA subsequently cited AAN's [American Academy of Neurology] supposed independent corroboration of its findings as part of its attempts to **defeat federal legislation to create a Lyme disease advisory committee and state legislation** supporting antibiotic therapy for chronic Lyme disease."
>
> "The IDSA failed to follow its own procedures for appointing the 2006 panel chairman and members, enabling the chairman, who held a bias regarding the existence of chronic Lyme, to **handpick a likeminded panel without scrutiny** by or formal approval of the IDSA's oversight committee…"
>
> "The IDSA's 2000 and 2006 Lyme disease panels **refused to accept or meaningfully consider information regarding the existence of chronic Lyme disease**, once removing a panelist from the 2000 panel who dissented from the group's position on chronic Lyme disease to achieve "consensus"…"
>
> "The IDSA **blocked appointment of scientists and physicians with divergent views on chronic Lyme** who sought to serve on the 2006 guidelines panel by informing them that the panel was fully staffed, even though it was later expanded…"
>
> "The **IDSA portrayed another medical association's Lyme disease guidelines [American Academy of Neurology] as corroborating its own when it knew that the two panels shared several authors,** including the chairmen of both groups, and were working on guidelines at the same time. In allowing its panelists to serve on both groups at the same time, IDSA violated its own conflicts of interest policy."

It seems no one is immune to repeated harassment or retaliation and anyone can become a target of federally funded tormenting. When NIH employees feel bold enough and compelled to go after people like the Deputy Director of the FBI with a law suit in federal court, a chronically ill grand mother running a local support group, or a volunteer patient advocate caring for her sick children don't stand much of a chance against such powerful forces. Due directly to the federal agencies employees harassment, stalking and threats, some very kind and helpful citizens have been forced out of public view and/or have stopped trying to educate and help others to prevent themselves and their families from being stalked and threatened.

NIH employees and their research, paid for with our collective tax dollars, have been put in charge of making life-altering decisions from leadership positions, and developing policies that directly and indirectly affect funding for Lyme disease research. When requesting funding and responding to Congress, it is doubtful these federally employed offenders and their associates display their true feelings as seen here, even while perched in their ivory towers and looking down on Congress.

It appears the NIH does not discriminate on the basis of race, color, age, ethnicity, religion, national origin, pregnancy, sexual orientation, gender identity, etc. In fact, when it comes to Lyme disease everyone can become a target at any given time.

> "What is it about white, middle-class, college-educated, middle-aged women that compels them to use a common infectious disease as the vehicle for their emotional and psychological problems? Why the endless lies and absurd street theater? Why are the spokespersons and leaders of the Lyme Movement mostly former mental patients, former felons, and belligerent, paranoid egomaniacs? Barnes accuses me and others of trying to discredit Lyme patients, but it's clear people like her do a much more effective job of undermining any political and social support for Lyme disease." [NIH Lyme program- McSweegan]
>
> "Maybe this person is a recent immigrant with an imperfect command of written English. Or maybe he/she is just a nut. Probably the latter." [NIH Lyme program- McSweegan]
>
> "Wow. Marylanders must be the dumbest people in the U.S. So who's the audience for this pathetic propaganda?" [NIH Lyme program- McSweegan]
>
> "… do we cheer for the scum-bag personal injury lawyers or for

> the deluded patients? I had to go with the scum-bag lawyers—after all, it's not the fault of the lawyers. They're just acting out their nature like a shark or a mamba or some other dangerous predator." [NIH Lyme program- McSweegan]
>
> "Boy, that LymeNut discussion board gets a little bit wackier every day. Its managers must be following the lead of Iranian mullahs and Chinese Politburo members in defining how to control the media..." [NIH Lyme program- McSweegan]
>
> "The host of this intellectual catastrophe is Eva Sapi, (another Hungarian? Does anyone else miss the Berlin Wall?)" [NIH Lyme program- McSweegan]

Efforts to shut down Lyme disease patients, groups and organizations has been so focused that a "hit-list" was developed and regularly updated by NIH's McSweegan, with actions designed to discredit, frighten, torment and take out the most vocal of the volunteers in order to provide smooth sailing and sustain the status quo for NIH and other federal agencies and related entities.

Published and shared on the internet, the Wacky World of Lyme Disease documents some of the attacks made on various Lyme patient volunteers over the years. It was written by NIH Lyme program's McSweegan- in a clever enough fashion to be able to skirt the laws of the land and rules of decency in order to avoid additional punitive actions taken against him.

When people across the country began, unfortunately, contracting Lyme disease and additional diseases from a tick bite they could never have imagined the disease spectrum would include being relentlessly stalked, harassed, having children removed from their home due to false reports to child protective services, have their employers and insurers contacted by federal employees, and even be physically threatened.

> **Patient Report-** "Lastly, I admit that I did cry during the deposition. Yup. It was during a reading of material Ed [McSweegan] admitted writing, that mocked our dead son and pets. And, a number of such writings were done WHILE he was a public official working at NIH as LD Project Officer." [KF- patient and support group leader of LDF]
>
> **Patient Report-** "In my case there have also been anonymous death threats sent to me both publicly and privately, suggesting that "cars come up on the sidewalk", and similar themes. … Finally in late April the harassment culminated with a threat placed on an internet Lyme group directed at my

children. The same day I reported it to the police, a close friend in England had to leap out of the way to avoid an oncoming car, speeding the wrong way down a pedestrianised lane in her local shopping precinct.

On 8 May 2006, three police officers, two doctors, two social workers and a community psychiatric nurse arrived without warning at my door. They had a warrant for my arrest. ... Dr Ellis questioned me briefly about the hacking, and the threat to my children. Dr Maya Ranger, a consultant psychiatrist .. was placed in charge of my care. She immediately diagnosed me with "Delusional disorder". I asked her in a ward round what was the basis of her diagnosis. She explained that it was three things:

That I disagree with the views of Dr Susan O'Connell. O'Connell is the UK "expert" on Lyme Disease, who believes that Lyme is hard to catch, is easily ruled out by blood tests, and almost always curable with a simple 3-week antibiotic course. ...

She ignored detailed material sent to her about Dr McSweegan, whom I have reason to believe is one of the Steere camp people who has harassed and threatened me, whilst hiding behind false internet names, since 2003.

I am certain that if Dr Ranger had got her way, she would have fed me steadyingly increasing doses of Risperidone in her attempt to "cure" me of my beliefs about Lyme disease until she had turned me into a cabbage." [EC- patient]

Patient Report- "DeRose was warned by others McSweegan has stalked [and] to contact her [College] Dean immediately [after she received a written threat from Mcsweegan] and tell him to expect communications from McSweegan, which she did. The following day, true to habit, McSweegan called DeRose's PhD advisor to discredit her.

DeRose sent an email to patient (LB) concerning the anonymous complaint filed with child protective services shortly after McSweegan contacted DeRose's college. Several patients have had anonymous complaints filed against them in the same manner, and always after speaking up about a Lyme-related issue.

DeRose wrote: "It's a long complex story but the CPS

complaint made accused: my husband of sexual abuse and me of crackpot medical treatments for our daughter [I was sick when I was pregnant with her- hence a possible connection to the autism], so I'm pretty much cleared. My husband, however, was thrown out of our house with 20 minutes notice when the temperature was 7 degrees outside.

He ended up trying to sleep in his car but after about two weeks he gave up on trying to live with these false accusations. The CPS counts on your being so shamed and humiliated that you eventually just give in to whatever they say. He decided to kill himself instead.

My kids are 12 and 16-the oldest is just old enough that they can't take him away. It is my autistic 12 year old daughter that is at risk. She does not interview well because of her autistic mind and is bewildered by all this. In fact she never wants to be interviewed or have her body examined again now.

To quote: "All complaints are strictly confidential" but I now understand why he [referring to McSweegan] uses this tool. Once CPS gets involved all sense of due process is thrown out the window. Case workers can, after about half a day of interviews, make life altering decisions that can destroy a family for years to come. I can now fully understand the other women's plights that he stalked.

My husband, who is a PTSD Vietnam vet to start out, has ended up attempting suicide and is now in a mental institution with his previously manageable mental illness converted into permanent schizophrenia. Last paycheck: last Thursday- income for foreseeable future $0. My 16 year old is now under suicide watch also and our daughter is just about destroyed.

It seems that if you are accused and guilty you are guilty and if you are innocent you are also guilty." [MM Drymon- patient]

McSweegan's Hit List Quote- "What do you do about people like this? You can't sue them; they don't have enough money to make it worthwhile, and a defense of emotional or mental incompetence would probably be compelling. She thinks I exhibit "bizzare behavior" and "could be dangerous" so I just may have to content myself with stopping in Centreville to punch her in the face the next time I drive over to Rehoboth. Would that be considered bizarre behavior or just proof of being dangerous? Maybe I could just show up at the next

> meeting of her support group, "Eastern Shore Hicks with Ticks." Do they serve coffee and donuts?" [NIH- McSweegan]

The decades long attacks on patients, health care professionals and politicians didn't stop when McSweegan was finally removed from the NIH's Lyme disease program for his public condemnation of Lyme patients, their doctors, support groups and specialty labs. He, while still employed by the NIH, published in journals everything he could, with his coauthors who had been receiving federal grants for research right beside him. Using federally funded research to attack others is not acceptable, however it is a regular practice.

> "As with other antiscience groups, some Lyme disease activists have created a parallel universe of pseudoscientific practitioners, research, publications, and meetings, arranged public protests and made accusations of corruption and conspiracy, used harassment and occasional death threats, and advocated legislative efforts to subvert evidence-based medicine and peer-reviewed science."
>
> "National Institutes of Health (NIH)-sponsored, double-blind, randomised, placebo-controlled treatment trials have been done to examine whether persistent (for ≥6 months) subjective symptoms were improved by retreatment with antibiotics after standard courses of oral or intravenous treatment for Lyme disease."
>
> "The accusations eventually drew the attention of the US Congress. During a 1993 Senate hearing on Lyme disease, one LLMD accused "a core group of university-based Lyme disease researchers and physicians…of act[ing] unscientifically and unethically. They work with government agencies to bias the agenda of consensus meetings, and have worked to exclude…those with alternate opinions."
>
> "In 2000, activists persuaded a few congressmen to investigate the federal Lyme disease research programmes of the Centers for Disease Control and Prevention (CDC) and the NIH."
>
> "In December, 2006, a New Jersey congressman complained that it was "inappropriate for CDC to highlight IDSA's findings—to the exclusion of others."

McSweegan was allowed to remain at the NIH and to his own admission was given too much free-time at our expense. When sanctions should have been imposed, instead Congress stepped up to protect NIH's

McSweegan.

> "I doubt the Congress has any interest in investigating what I do in my house after work. What I do at work has been investigated. Check with Senator Grassley's office if you're curious." [NIH Lyme program- McSweegan]

Eventually, Mr. Phillip Baker was placed in charge of the Lyme disease programs and grants at NIH. His attempts after retirement to follow in McSweegan's foot-steps provides us all with an overview of the state of the science while grants were under his direction.

> "Obviously, the credibility of guidelines proposed by ILADS, a pseudoscientific organization with an undistinguished membership of about 300, as well as the similar views of those often referred to as Lyme Literate Physicians (LLMDs), should no longer be given credence and serious consideration." [NIH Lyme program- Baker]

Attempts to censor other viewpoints regarding Lyme disease continued with Baker at the helm of the American Lyme Disease Foundation (ALDF). Rabid and rapid responses to news articles that did not follow the federal agencies party line were fired off at every possible chance. Working behind the scenes to change or kill Lyme related bills was a popular past-time for former NIH Lyme disease employees and their partners, and continues today.

Funding for Lyme programs once under NIH's McSweegan were later directed by NIH's Baker, and funds were funneled to the same small group of like-minded researchers, such as Raymond Dattwyler, an obviously well-funded friend of the federal agencies who published 67 studies in 22 years and shared like-minded opinions with McSweegan, Baker, et al.

> "I am indeed proud to have assisted Dr. Dattwyler, as well as many other NIH grantees, in getting support for the outstanding work that they are doing." [NIH Lyme program- Baker]

> "The doctor at Southampton Hospital didn't evaluate you properly," he began, then stopped, correcting himself: "Nah, the doctor was a jerk." [NY Medical College- Dattwyler]

Exposure of wrong-doings often prompted NIH's employees mentioned above to either stand up publicly for the repeat offenders using their credentials in the process, or it led to more public and private retaliatory

attacks on suffering patients, volunteers and their doctors.

> "To characterize such individuals as "loonies" might be too kind of a description." [NIH Lyme program- Baker]

> "As a result of such close interactions, many of us have become better acquainted not only with each other, but also with scientists who actually do the research that is funded by grants from the NIH and other government agencies. As Program Officer for NIAID's Lyme Disease Basic Research Program, I managed the grants and therefore had personal contact and direct interactions with almost every well-known and accomplished scientist doing research on Lyme disease…" [NIH Lyme program- Baker]

> "During my long scientific career, I have had the privilege of knowing many outstanding and dedicated scientists who do excellent work and really care about the public health. I am extremely proud to have been associated with all of them. Your biased article does them and all that they have accomplished a great disservice." [NIH Lyme program- Baker]

The 21st Century Cures Initiative will hopefully address some if not all of the serious problems created when Congress first mandated federal agencies representatives to work together **to the exclusion of outside stake holders**, which allowed the continuation and promotion of biased and conflicted science, and in our specific case permitted attacks on and dismissals of patients and others.

> "About 2-3 years before I became Program Officer for the National Institute of Allergy and Infectious Diseases's (NIAID) Lyme Disease Basic Research Program, the **U.S. Congress mandated** that NIH establish an NIH Lyme Disease Advisory Panel to facilitate the exchange of information and the development of co-operative interactions between those institutes of the NIH that support clinical studies and basic research on Lyme disease; representatives from the CDC and the FDA also were invited to serve on this panel which is required to meet at least once per year and more often if needed." [NIH Lyme program- Baker]

> "Therefore, it should not be surprising to discover that the **NIH, CDC, and FDA work closely together on Lyme disease**; not only have they been **encouraged by the Congress to do so** in this and other areas of scientific research… This hardly constitutes collusion or a conspiracy as some naïve individuals

believe to be the case." [NIH Lyme program- Baker]

The Committee for the 21st Century Cures initiative will hopefully ensure federal agencies (CDC, NIH, FDA, HHS) and their partners (IDSA, ALDF) who are controlling Lyme research and directing the majority of funding for science, often for their own purposes, while miserably failing the patients, will be overhauled and the bad apples from all agencies will be eliminated so we can start on a progressive and positive course that will actually benefit patients and those truly interested in assisting them on the road to finding a cure.

Federal agency employees and others report about their attacks on Lyme patients, advocates, doctors and groups:

> "Millions of dollars have been spent refuting their [patient, etc.] claims, and thousands of hours have been spent responding to false allegations, legal threats, congressional queries, and other harassments." [NIH Lyme program- McSweegan- and IDSA guideline authors]

The solution is obvious and quite simple, with the added bonus of saving millions of dollars. Get rid of those who continue to misuse federal funding and road block the science to exclude diverging points of view.

Changes in personnel and overhauling of the Lyme program within federal agencies should include requiring all federal agencies to be transparent and beholding to the public they serve. The changes should include appointing representatives from the patient community, group leaders, outside researchers and health care professionals with differing view points to not only be included and have "a seat" at the table for the first time, but to have their numbers be in the majority when it comes to the decision making process regarding future policy decisions and directing the available research funding.

Due to their pitifully poor record over the past three decades and the destruction caused to countless lives by the run-amuck federal non-transparent, not accountable Lyme related system currently in place, we additionally request the agencies be investigated and be held accountable both now and in the future.

We request this be carried out to a greater extent than typically required in other circumstances. This type of change, if prompted and supported by Congress, will save millions of dollars and help reduce the budget while still advancing the science.

How wrong can the federally funded studies be?

The federal agencies funding for research produced all and more of the following conclusions, findings and comments, and all of the statements have since been disproven. They do not have a good track record and they do not care.

1. Lyme was originally reported to be caused by a virus.

WRONG! It is caused by a form of bacteria.

2. Lyme should not be treated with antibiotics because Lyme is a virus.

WRONG! Lyme is caused by a bacterial infection that, like other bacterial infections, is susceptible to antibiotic therapy.

3. If a short course of antibiotics doesn't work to cure Lyme, no more antibiotics are needed.

WRONG! Short courses of antibiotics have been found to be extremely unreliable. Re-treatment has shown to improve the patient's condition.

4. The blood tests the IDSA and its partners developed (and patented for profit) are accurate.

WRONG! The have been proven to miss up to 75% of people who are infected.

5. There are a lot of false-positive test results.

WRONG! There are an extraordinary amount of false-negative test results.

6. Lyme disease is easy to diagnose and cure.

WRONG! Lyme disease can mimic countless medical conditions and a cure has never been developed.

7. Reporting practices are sufficient and give us a good picture of the spread of Lyme disease.

WRONG! The actual numbers of Lyme cases is over 10 times what is currently reported.

8. After treatment people do not have Lyme, just the "aches and pains" of daily living.

WRONG! Countless people have become chronically ill, disabled and many have died.

9. There is no evidence chronic Lyme exists.

WRONG! There are over 1,000 scientific studies proving otherwise.

10. The federally supported IDSA Lyme Disease Guidelines represent the best of the science.

WRONG! They were found to be developed by a handful of people with conflicts of interest and do not work to produce a cure for most.

11. Lyme disease can not be passed from mother to fetus.

WRONG! The literature indicates simple complication to still births and death are possible.

12. There is no Lyme here in -fill in the blank with your State's name.

WRONG! Absence of evidence isn't proof of anything.

13. We have no conflicts of interest.

WRONG! May 1, 2008- The CT Attorney General's investigation proved otherwise.

14. A tick must be attached for 48 hours to transmit Lyme disease.

WRONG! Studies prove otherwise, and transmission can occur in less than a few hours after a person is bitten.

15. Two pills of Doxycycline taken within 3 days of a tick bite will prevent/cure Lyme disease.

WRONG! Lyme disease can disseminate throughout the body and studies have proven this is not true.

16. We care about patients.

WRONG! You just need to ask anyone who was denied treatment and developed the late chronic stages of Lyme disease what they think.

17. The standard, federally supported 2-3 weeks of Doxycycline protocol cures most cases of Lyme disease.

WRONG! Thousands of studies, along with thousands of chronically ill patients prove otherwise.

18. You can not have more than one tick borne disease at a time.

WRONG! People can be multiply infected with a number of various organisms.

19. The new vaccine is safe and effective.

WRONG! It was pulled from the market after reports of injury began climbing and law suits were filed by those who were injured.

20. Lyme disease can not be sexually transmitted.

WRONG! Multiple studies found evidence of the spirochete in secretions from both men and women.

21. Only certain ticks carry certain diseases.

WRONG! Each year more new discoveries are made proving that theory wrong.

22. Steroids are a viable treatment for those with Lyme disease.

WRONG! Steroids are contraindicated in all but the most severe complications for those who have Lyme disease.

23. Some exercise, visits to psychiatrists and Advil are all that are needed if symptoms remain or become worse after treatment.

WRONG! Coinfections and other sources for the symptoms need to be explored.

24. Prevention efforts are working.

WRONG! Federally funded studies prove otherwise.

25. Blood donations can be safely made by those who were treated for Lyme with the minimal federally approved antibiotic protocol.

WRONG! The Red Cross and others have proven the spirochetes can remain active even through blood storage conditions.

Proposed Solutions

We request the total elimination of those presently working on Lyme related activities for federal agencies in any capacity due to the horrendous and unthinkable way Lyme disease research, funding, education and patient care has been handled to date. If the NIH can send one offender (McSweegan) to Russia, pay him over $100,000 a year to do nothing (his own admission), then lend him out to vaccine promoters for an undetermined amount of time, and retire another federal employee (Baker) and set him free to immediately join hands with those he funded over the years to continue in his attempts in suppressing the science and discredit those with differing opinions, they can certainly ship the others somewhere. As the saying goes, if they can send one man to the moon, why can't they send them all?

We request members of the Committee, either Republican, Independent or Democrat- no matter- provide strict and close (to the point of smothering) oversight to federal agencies responsible for policy making and research funding for science related to Lyme and tick borne diseases to ensure the CDC, NIH, FDA and other federal agencies will no longer abuse the system. This requests excludes any oversight services suggested by or provided for by Congressman Frank Pallone (NJ) due to his Lyme related conflicts of interest.

We request Congress be mindful that agencies they are funding use our monies and their authority appropriately. With the requirement to have open communications and full transparency there should be a sincere reduction of situations where the fox is guarding the hen house and the conflicted are reporting directly back to Congress with their biased, self-serving reports.

We request the decades of wasteful spending, self-promotion, retaliation and conflicts of interest linked to the federal agencies be addressed in a serious fashion. We request the Committee provide all stakeholders an opportunity to participate in developing their futures. After all, to many of us this is a matter of life and death for ourselves and our children.

We request, until more quality research is forthcoming, Congress demand diagnostic and treatment options utilized by health care professionals and doctors while caring for Lyme patients remain flexible and patient oriented. New treatment guidelines developed by Lyme treating doctors and researchers should soon be forthcoming, barring the continued blocking by the friends of the federal agencies who serve as editors on various scientific journals. The guidelines, developed by the International Lyme and Associated Diseases Society (ILADS), will be offered to help guide clinicians world wide in the diagnosis and treatment of complex cases of Lyme and tick borne diseases.

We request the FDA be ordered to back off the independent Lyme disease labs in their quest to shut down the competition, which will benefit the federally supported financial stakeholders. To note- the IDSA Lyme Disease Guideline editor and spokesperson, who also served on the IDSA Board and was involved in the conflicts of interest scandal- Paul Auwaerter (Hopkins)- was recently appointed to the FDA panel. Shortly thereafter, it was announced that for some reason Democratic Senators were pushing for a bill to support the FDA actions.

We request public meetings and workshops be held across the country by a newly developed committee, with committee members to be designated by Congressman Chris Smith (NJ), and only Congressman Chris Smith. The Congressman has spent years studying this situation and has a deeper understanding than most concerning the actual problems patients and their doctors are facing. The committee should be encouraging patient, advocate and group input, and equal time and weight should be provided for each respondent. Funding for this project and other measures mentioned here can be covered by the elimination of certain federal employees in the overhaul process.

We request clinical trials be real-life clinical trials using real patients in various stages of disease who are provided reasonable treatments by

doctors with a long track record of successfully treating chronically ill Lyme patients. Funding has been grossly lacking in this area for decades.

We respectfully request our elected representatives make a focused effort to safeguard the public and put an end to the political games that are causing the citizens of the United States to become chronically ill, disabled and/or are allowing them to die while waiting for help for Lyme disease from, and directed by, the federal agencies.

We thank you all for taking time to read, listen and respond to these concerns. Please contact me if you have any questions or require more documentation.

This submission has been brought to you by, as the NIH puts it-

> "Speaking of nuts, this local newspaper article reads like it was dictated by Lucy Barnes… Her juvenile online comments about scientists and academic physicians, and her online lectures about Lyme disease suggest she's a horribly ignorant and ill-mannered 13-year-old hillbilly, or maybe just a liar. Is it possible to be both?"

Lucy Barnes, Director
Lyme Disease Education and Support Groups of Maryland
631 Railroad Avenue
Centreville, MD 21617
AfterTheBite@gmail.com

Sources for quotes and other documentation are presented (highlighted and linked) throughout the original text. More documentation is available upon request.

Additional Information

1.) NIH- McSweegan, Edward- Partial List of Lyme Disease Related Quotes (2009-2010)
2.) LLMD definition- Lyme patients term for a doctor who has educated themselves on the diagnosis and treatment of tick borne diseases and is willing to risk everything to see their patient's receive appropriate medical care. **L**yme **L**iterate **M**edical **D**octor.
3.) ILADS definition- a group of health care professionals working together to educate others concerning the diagnosis and treatment of tick borne diseases. **I**nternational **L**yme and **A**ssociated **D**iseases **S**ociety.

lymedisease.org
Advocacy, Education and Research

July 10, 2014

Congressman Fred Upton
Mark Ratner, Legislative Director
2183 Rayburn Bldg.
Washington, DC 20515
[redacted]

Congresswoman Diana DeGette
Ms. Rachel Stauffer, Health Policy Director
2368 Rayburn House Office Bldg.
Washington, DC 20515
[redacted]

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.[5] 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 researcher- rather than patient-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.[6] 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.[4]

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.[5] 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 achiness.[5]

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.[7] 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.[3] 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.[3] 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.[3] 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.[3] 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,

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