

As the mother of a child suffering from PKD, a disease for which there is no cure, I urge you to seek a path to a treatment. At present, she has kidney failure , dialysis and kidney transplant as her only hope for survival. It is imperative that research be done to discover a viable alternative to transplantation.

Thank you

Gail [REDACTED]

In order to encourage innovation and discovery we need for the Legislature to enact laws that would assure that innovation and discovery proposals will be welcomed by the Health and Human Services Department and its subsidiaries, especially The National Institute of Health and the FDA. The NIH should always be required by laws to maintain an open invitation for suggestions that may improve their guidelines and standards for illness treatments and improvements of healthcare. If the present policies and operating procedures of the NIH and FDA are allowed to become dogma or law it will lead people to believe that improvement is not possible. In order for a person to be encouraged to look for possible improvements they must have faith that improvement is possible.

For example the NIH has been encouraging the passage of a bill by the House and the Senate that would essentially make the 2008 Guidelines for Physical Activities for Americans a law that would require all federal agencies to promote these guidelines in relation to all healthcare matters incorporating physical activities. It would also make it possible for the Sec. of HHS to perpetuate these guidelines as if they were law perpetually. That would tend to discourage any future possibility of scientific discovery in this matter. An example of this bill is H.R. 2179 113th Congress. Scientific exploration of possibilities of improving these guidelines in the future would disappear from consideration.

Strong evidence exists that verifies we could probably cure or greatly alleviate the majority of chronic noncontagious metabolic diseases by implementing the aerobic physical activity program that 2008 Physical Activity Guideline Committee predicted would produce the maximum benefit at the least risk.

Thousands of allegations of fraud and waste have been lodged by the members of Congress and the media. But the laws relating to fraud and waste and scientific misconduct committed by the government employees do not adequately address the penalties that must be applied to the employees of the government. The new law should unequivocally state that the government employees that are found guilty of fraud, waste, or scientific misconduct will be debarred and the seriousness of the offense thoroughly investigated to determine if their actions were intentional and therefore criminal in nature. If the evidence indicates a person or persons have been seriously injured as a direct or indirect result of the illegal activity the criminal penalties should be defined in the law commensurate with the harm done. The seriousness of the injuries should be based on the severity of each of the consequences of the injuries. For example did the injuries lead to amputation of a limb or thousands of people suffering premature death (possible manslaughter).

The mission of the NIH is to seek fundamental knowledge about the nature and behavior of living systems and the application of that knowledge to enhance health, lengthen life, and reduce the burdens of illness and disability.

The goals of the agency are:

To exemplify and promote the highest level of scientific integrity, public accountability, and social responsibility in the conduct of science;

to foster fundamental creative discoveries, innovative research strategies, and their applications as a basis for ultimately protecting and improving health;

to develop, maintain, and renew scientific human and physical resources that will ensure the Nation's capability to prevent disease; and

To expand the knowledge base in medical and associated sciences in order to enhance the Nation's economic well-being and ensure a continued high return on the public investment in research. Any employee that is found unwilling or unable to comply with the mission and or the goals of the NIH will be debarred when the evidence is presented and verified.

In order to encourage innovation and discovery the FDA should be forbidden to charge fees for an application to approve an old drug for a new use if the drug has been demonstrated to be safe while in

use for the past 20 years or more. The FDA has been demanding approximately \$1 million or more to be deposited with each application for approval of a new or old drug. They claim that a law requires them to do so. Apparently the law allows them to demand such fees but it doesn't require them to do so especially if the medication is primarily used to effectively treat diseases that lead to imminent disabilities and premature death. It has been demonstrated that there is a drug that is available throughout the world that can have a major beneficial impact on prevention and cure of chronic noncontagious metabolic diseases if it is utilized in conjunction with a proper aerobic exercise program. Such an exercise program was recognized by the committee that developed the 2008 Physical Activity for Americans to be most likely to provide the maximum benefit with the least risk. A great deal of evidence indicates the procedure and medications are safe and effective.

## Introduction

George [REDACTED]

Dear House Energy and Commerce Committee,

As I understand, The House Energy and Commerce Committee has launched the 21<sup>st</sup> Century Cures initiative to draw attention to and close the glaring gap between the number of diseases and the number of treatments available. We would like to share our experience with you concerning Polycystic kidney disease. Our son, who is the light of our life, was adopted at the age of 4 mos. At age 23, he was diagnosed with PKD. We have been devastated ever since. Our faith is what keeps us going. We pray daily for a cure.

As you may already know, Polycystic kidney disease (PKD) is a genetic disorder characterized by the growth of numerous cysts in the kidneys. The kidneys are two organs, each about the size of a fist, located in the upper part of a person's abdomen, toward the back. The kidneys filter wastes and extra fluid from the blood to form urine. They also regulate amounts of certain vital substances in the body. When cysts form in the kidneys, they are filled with fluid. PKD cysts can profoundly enlarge the kidneys while replacing much of the normal structure, resulting in reduced kidney function and leading to kidney failure. There is currently no treatment to slow or stop the growth of the kidney cysts that plague generations of families suffering from polycystic kidney disease (PKD). PKD patients only remedies are dialysis and transplantation once their kidneys fail.

**We implore you to help Congress move the ball forward and to give my son, and us, back our lives! We would be eternally grateful!**

Sincerely,

Gerald and Helen [REDACTED]

We need a cure or even a treatment for Polycystic Kidney Disease. (PKD).

I was diagnosed with PKD in June 1998. I was placed on a kidney transplant list in March 2011. I started dialysis in January 2013. I am 78 years old.

The cure is too late for me but I have three sons and one daughter with ages in their early 50s. **All have polycystic kidney disease. They need the cure.**

Dialysis keeps me alive but greatly limits travel and other activities. Dialysis and kidney transplant are costly primarily to the government. A cure or even treatments that slow disease progression would save a great deal of taxpayer and patient money. Patients could continue 100% productivity.

We need a cure for Polycystic Kidney Disease. (PKD).

Gilbert [REDACTED]  
[REDACTED]  
[REDACTED]

To Whom it May Concern,

I write to you to strongly advocate for more research to find a cure and/or treatment to preserve kidney failure for individuals with Polycystic Kidney Disease (PKD). As the most common form of genetic disease, the devastation to families to families is heart breaking and the cost to the American public is enormous--both because of providing care for those that do not have health insurance and through Medicaid and Medicare. As the primary provider of care for individuals with end stage renal disease on dialysis and post transplant, the treatment costs and drug costs, especially for immunosuppressant and Epo, are enormous. It makes much more economical sense, as well as humanitarian sense, to concentrate efforts on research to preserve kidney function, which will also reduce costs of diabetes and heart disease.

I know the devastating impact on families first hand, as well as the government and personal financial costs. Having been diagnosed with PKD at age 19, I have suffered with high blood pressure, growing pain, weight due to cyst growth, and related heart and circulatory complications until age 49 when liver and kidney transplant were my only options. While blessed to receive both a liver and kidney on March 17, 2014 and doing well in my recovery, others should not have to suffer this same journey. As example, at the time of removal, my liver weighed 18 pounds and each kidney weighed over 10 pounds. You can just imagine the pain and problems that occur with carrying an additional 40 pounds of organ due to enlarged cysts. This should not have to be. Further, if there was a cure or treatment for PKD, the organ list would be significantly shorter and numerous lives of individuals with other impairments or conditions would be saved.

With a mother, sister, brother, nephew, and possibly two children of my own still with PKD, I plead that more resources are dedicated to finding a cure and treatment for this devastating disease. If you would like to hear more about the impact of this disease and the potential financial and live savings that could result from a concentrated effort, please contact me at [REDACTED]

With appreciation for your consideration and action,

Gina [REDACTED]

Dear **House Energy and Commerce Committee**,

I want to express to you how valuable and important searching for a cure for PKD is. I am 54 and living with ADPKD. When I was 8 1/2 years old, my mother died suddenly of an cerebral aneurism because of PKD. This disease is deadly. Her father and his sister both died of kidney failure due to PKD. Finding a cure to stop or slow the growth of the cysts is such important work and would save or prolong the life of so many individuals. PKD is a genetic disease which we so desperately need a cure for. Please continue to fund research for PKD.

Warmly,

Gina [REDACTED]

[REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]

Dear Drs. Hamburg, Woodcock, Maynard and Koh:

After reading the information listed below, I am writing to you to kindly request your attention to ME/CFS.

I have been taking care of a patient with ME/CFS since 2007. In 2012, she moved to Nevada to get Ampligen. I will not explain all the challenges of the move from Boston, Massachusetts to Lake Tahoe, Nevada but that is what ME/CFS patients do when there is an opportunity to improve. It was a physical, emotional and financial endeavor.

Before Ampligen:

-Cognitively, she was in a fog. She could not concentrate on any task for too long. Making decisions was exhausting. There was very limited "awake time". Everything was a mental effort to the point that conversing was also very limited. I remember the comment: "I cannot talk today". The prolonged silences at home were very difficult; we basically lived in silence.

-Physically, she did not have any energy. Spent hours and hours sleeping and would wake up more tired and wanting to sleep more. She gained weight and her appetite was very erratic. She could not walk or do anything outside or inside the house. She was totally homebound not being able to sit at the dinner table or share in any of the household activities/chores like cooking, laundry, food shopping, etc.

-Emotionally, it was very, very distressing. Since she could not participate/share in any family/friends activities, the isolation increased and the depression too. This was "a speed of light" "person who was now confined to a bed for hours and hours. She could not watch TV or watch a movie. It seemed like the switch was gone off on her. It was very sad to watch and be around from my perspective.

After Ampligen:

-Cognitively: I visited her seven times during the first year of Ampligen in Nevada. Ampligen was and is the right medication for her. The progressive change was amazing to watch. Every time that I saw her it was like a dim light growing brighter and brighter. She reported having the fog decreased. I was able to carry a conversation and she was able to do some shopping for herself and expressed not being as tired mentally as before. She could make brief outings and was not mentally exhausted. Her memory was better and improved more progressively and the awake time was more productive: she could watch TV, listen to music and sleep less during the day. This continues to be the case today. Even though she needs down time, she does not require total silence and/or sleep all the time.

-Physically she started to manage her weight, eat healthier and go for walks. It really was a person coming back from a cave and progressively being able to enjoy the sun and being outside. She was doing the dishes and laundry and starting to come to life. This also is the case today. She has to pick between things to do or not but at least she has a choice. This past Thanksgiving she attended the family gathering and it was great to watch her participating while being cautious about saving her energy.

-Emotionally she is doing great. We disagree; we discuss topics, exchange opinions and even though she asks for quiet times, those have a different flavor. She smiles and laughs and

sometimes feels that she can again “go at the speed of light.” Quality of life has improved, for both of us.

I know Ampligen is not for everybody but is the only drug that has provided real, visible relief to a number of people. If it were to be more affordable by being approved by the FDA (insurances would come to the table) more patients could benefit from it. As patients and caregivers, we need Ampligen. I recognized that Ampligen is not for every patient but, as you so well know, there are no drugs approved by the FDA to treat this very complicated illness. We need for more patients to regain some quality of life and to believe that there is light at the end of the tunnel. Also, having Ampligen approved will bring other pharmaceutical companies to the table to develop other drugs that could be used for those that cannot benefit from Ampligen.

Thank you for taking into consideration my concerns and request. Please let me know if I can assist in any way.

Dr. Gisela [REDACTED]  
[REDACTED]  
[REDACTED]

NEWS Flash... Aduro BioTech, Inc. has received a “breakthrough designation” after positive clinical evidence in the treatment of pancreatic cancer. A breakthrough designation is reserved for drugs that would treat a serious or life threatening condition and preliminary clinical evidence shows great potential for improvement over available therapies, the FDA states. The San Francisco Times reported that the FDA's action could result in drugs being approved in as soon as 60-days, but it does not guarantee approval of the therapy.

Thanks for tackling this important issue. My son is 20 and was diagnosed with Friedrichs Ataxia when he was 13. He is actually doing really well, comparatively to others his age, but did start using a wheelchair last fall, when he went back to college. When we received his diagnosis, our inept pedi-neurologist started the meeting with “I haven’t been looking forward to this” and gave us a copy of a brief article from a medical book on what FA was...He didn’t give us any hope and little information. We still do not have an FDA approved treatment and certainly no cure for this progressive disease, that most profoundly effects the peripheral nerve cells resulting in loss of proprioception, motor control, and speech impacts, as well as the heart muscle, resulting in cardiac hypertrophy – complications of which are what typically kill or kids. We are blessed to be part of the Friedrichs Ataxia Research Alliance (FARA) [www.curefa.org](http://www.curefa.org) which was started by a few parents. This unique organization’s mission is to bring together families, researchers and clinicians, in a collaborative manner, who are in one manner or another, working one a piece, or pieces of the FA puzzle, and to raise and funnel money to researchers that are working on aspects of this disease that may lead to a treatment or cure. I’ve been privileged to attend three symposiums hosted by the Children’s Hospital of Philadelphia (CHOP) and Dr. [REDACTED] one of the world’s leading clinicians in the FA field. Since attending my first conference 6 years ago, to the most recent conference in Oct. 2013, there have been amazing strides, many new drugs in the development pipeline and real hope that we may find something in time to impact my son’s life. As you well know, the path through the FDA for approval for a new drug is long and very expensive. The government can do to incentivize and support

- basic research and sharing of information, that crosses boundaries for many diseases (i.e. FA is a disease of the mitochondria of the cell)
- Ease the path for clinical trials for orphan diseases, to advance them to usable therapies. (orphan diseases don’t have big pools of subjects to test on...)
- Lower the “benefit/risk” quotient for determining treatment efficacy and allowing clinical use of new treatments for devastating diseases with little or no approved treatment options. Let the patient / family make that risk judgment.
- Hold “Charitable” organizations accountable for the money donated in good will, to the proper use of those precious funds. I see so much activity and \$\$\$ towards things like “breast cancer awareness” and “America Heart Association”, “American Cancer Society” collected by large bureaucratic organizations, but never see information on what positive advances are made with this money. Both Cancer and heart disease have had billions donated to their causes and are very treatable and some cases preventable by lifestyle changes.

Thanks for the opportunity to provide input.

Glen [REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

Dear Congress,

Unless you know about PKD, you will not understand the pain in our American families. You will not exercise the vote given too you and the duties we all should strive and cure.

To Whom It May Concern:

While encouraging innovation and the use of new technology to develop new cures for people suffering from a wide variety of diseases please do not neglect the potential of the widespread use of drugs for "off label" or non-FDA-approved purposes. Please consider legislation that would simplify and accelerate the procedure for FDA approval of drugs for currently unapproved (but not disapproved) conditions. My own experiences with FDA have been quite exasperating in this regards.

Sincerely

Gordon [REDACTED]

[REDACTED]  
[REDACTED]

My wife is currently being treated at [REDACTED] for stage IV melanoma cancer. We desperately waited for over a year for her to obtain access to the best investigational drug treatment option available for her condition (anti-PD-1) while her health & quality of life declined significantly. She is currently in her 6th different treatment (5 were clinical trial drugs). During this ordeal I have become very passionate about the compassionate use of investigational drugs. I understand that Congressman Griffith introduced HR-4475 on 4/10/14 to deal with this issue, but that the bill has no chance for passage during this legislative session or probably in any future sessions.

Prior to my knowledge of any pending legislation, I drafted the attached non-codified language to address this mammoth issue. HR-4475 leaves it up to the drug companies whether or not they wish to furnish these drugs to these terminal patients. I believe that absent a requirement for drug companies to furnish drugs on a reasonable basis, terminal patients & their families would have an additional huge & unnecessary impediment/stress factor to deal with after the FDA is out of the way. My language also has more qualified physician involvement in the process of dispensing & administering these drugs. My goal is to craft a bill that will be strongly supported by Congress for passage in the next session.

Greg [REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

For any investigational drug ("new drug") that: 1) the IND (Investigational New Drug) application has been approved by the FDA, and 2) an IRB (Institutional Review Board) has established clinical trial protocols, the drug company that produces the new drug ("drug co.") must, in a reasonably diligent and expeditious manner, make available the new drug and the IRB clinical trial protocols to any patient if: 1) such patient's attending physician certifies under written oath that in his opinion such patient has a terminal disease (no currently approved treatment that will extend the life of such patient for more than 12 months) and that use of the new drug is the best current treatment option for such patient, 2) such patient is not currently eligible for any new drug clinical trial within 500 miles of patient's current permanent residence, and 3) such patient executes a written consent document accepting all risks for the new drug treatment and waives all claims, present or future, against the drug co. The drug co. shall have the right to charge such patient for all actual costs associated with providing the new drug (exclusive of cost for research, development, etc.). If the drug co. elects not to charge all such patients for this non-clinical trial use, and if the new drug is subsequently approved by the FDA, the FDA shall grant the drug co. an additional year of patent exclusivity for the new drug. In order for patient's attending physician to certify on patient's behalf as stated above, he must be a licensed medical doctor in good standing who has extensive training and experience in treating the type of terminal disease currently inflicting patient. Use of the new drug must be administered through patient's attending physician and such physician shall administer the new drug for the exclusive use of such patient.

6/9/14

I am writing in support of the 21st Century Cures initiative. Polycystic Kidney Disease seems ripe for this program as there is no cure and roughly 600,000 in the US alone suffer from this disease.

Please note that I am afflicted with Polycystic Kidney Disease (PKD). There is no cure for this inherited disease.

At around age 45 to 50 one will either need a Kidney transplant - which necessitates lifelong use of expensive Immuno suppressive drugs - or lifelong kidney dialysis.

Very little PKD research is done at the current time. End stage renal disease could be prevented through finding a cure. It would be much more cost effective to find a cure than to pay for all medical expenses. Thank you.

Hans [REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

June 5th, 2014

Energy and Commerce Committee  
United States House of Representatives  
Chairman Fred Upton

Dear Chairman Upton and Rep. DeGette:

I am writing to express my concern about the current gap in our health care system to access treatment for PKU. I am a mother of a child with PKU. PKU has been successfully treated in the United States for more than 50 years, yet many children and adults cannot access the treatment needed to manage the disorder. We must ensure that everyone with PKU has access to the treatment they need for this rare genetic disorder.

Every baby born in the United States is screened for the early identification of PKU as a public health activity to prevent severe disability. The treatment for PKU includes the daily use of medical foods and foods modified to be low in protein that must be continued for life. However, this treatment is out-of-reach for most patients with PKU because of a lack of insurance coverage. Providing coverage for medical foods for the treatment of PKU is medically supported, cost-effective, and the right thing to do. I am writing to ask you to pass H.R. 3665, the Medical Foods Equity Act, so that federal health programs provide medical foods coverage for the treatment of Phenylketonuria (PKU). This will be a significant step forward in closing the gap in coverage.

- Medical evidence has demonstrated the safety and efficacy of medical foods as treatment for PKU for more than 50 years. Just recently, the American College of Medical Genetics and Genomics issued the first-ever treatment guidelines for PKU that confirms the necessity of medical foods treatment for PKU for life.
- The impact of this lack of coverage on patients with PKU is disastrous and expensive. The average family cannot afford to pay for medical foods without insurance coverage. We are dealing with this personally and it has been a nightmare, we are having to FIGHT our insurance company, spending hours of our work days dealing with this and we can't afford \$500 out of pocket a month for her formula that essential to her health.
- The long-term costs to the government for the care of untreated children and adults with PKU far exceed the cost of providing this essential treatment.

Decades ago, before the implementation of newborn screening and treatment with medical foods, children with PKU were doomed to a life of intellectual disability and costly institutionalization. Now, because of mandatory newborn screening and the proven treatment with medical foods, children and adults with PKU can lead normal and healthy lives. Don't put these lives at risk.

**Please ensure that medical foods for the treatment of PKU are provided by the federal health programs and pass H.R. 3665, the Medical Foods Equity Act, so that everyone with PKU can grow up and become healthy and productive citizens of this country.**

Sincerely,

Heather [REDACTED]

[REDACTED]

[REDACTED]

June 13, 2014

Energy and Commerce Committee  
United States House of Representatives  
Chairman Fred Upton

Re: 21<sup>st</sup> Century Cures: The Gap in Access to Treatment for Phenylketonuria

Dear Chairman Upton and Rep. DeGette:

I am writing to express my concern about the current gap in our health care system to access treatment for Phenylketonuria (PKU). I am a mother to a child with PKU. For over fifty years PKU has been successfully treated here in the United States, yet many children and adults cannot access treatment needed for the disorder secondary to financial strain. We must ensure that **Everyone** with PKU be afforded access to treatment for this rare genetic disorder.

Providing coverage for medical foods for the treatment of PKU is medically supported, cost-effective and the right thing to do. I am writing to ask you to pass H.R. 3665, the Medical Foods Equity Act, so that federal health programs provide medical food coverage for the treatment of PKU.

Failure to include coverage for medical foods for all patients with PKU in the federal health programs is not in accordance with the accepted standard of care. Guidelines for treatment for life provided by the American College of Genetics and Genomics can be found at  
[https://www.acmg.net/docs/Phenylalanine\\_Hydrosylase\\_Deficiency\\_Practice\\_Guideline\\_AOP\\_Jan\\_2013.pdf](https://www.acmg.net/docs/Phenylalanine_Hydrosylase_Deficiency_Practice_Guideline_AOP_Jan_2013.pdf)

Decades ago, before the implementation of newborn screening and treatment with medical foods, children affected with PKU were doomed to a life of intellectual disability and costly institutionalization. Now because of mandatory newborn screening and the proven treatment with medical foods children and adults with PKU can lead normal and healthy lives. Don't put these lives at risk.

Please ensure that medical foods for the treatment of PKU are provided by the federal health programs and pass H.R. 3665, the Medical Foods Equity Act, so that everyone with PKU can grow up and become healthy and productive members of society.

Sincerely,

Heather [REDACTED]  
[REDACTED]  
[REDACTED]

May 22, 2014

Energy and Commerce Committee  
United States House of Representatives  
Chairman Fred Upton  
Re: 21<sup>st</sup> Century Cures: The Gap in Access to Treatment for Phenylketonuria

Dear Chairman Upton and Rep. DeGette:

I am writing to express my concern about the current gap in our health care system to access treatment for PKU. I am a mother to a bright and happy little six year old boy with PKU who is currently being successfully treated for this condition. PKU has been successfully treated in the United States for more than 50 years, yet many children and adults cannot access the treatment needed to manage the disorder. We must ensure that everyone with PKU has access to the treatment they need for this rare genetic disorder.

Every baby born in the United States is screened for the early identification of PKU as a public health activity to prevent severe disability. The treatment for PKU includes the daily use of medical foods and foods modified to be low in protein that must be continued for life. However, this treatment is out-of-reach for most patients with PKU because of a lack of insurance coverage. Providing coverage for medical foods for the treatment of PKU is medically supported, cost-effective, and the right thing to do. I am writing to ask you to pass H.R. 3665, the Medical Foods Equity Act, so that federal health programs provide medical foods coverage for the treatment of Phenylketonuria (PKU). This will be a significant step forward in closing the gap in coverage.

- Medical evidence has demonstrated the safety and efficacy of medical foods as treatment for PKU for more than 50 years. Just recently, the American College of Medical Genetics and Genomics issued the first-ever treatment guidelines for PKU that confirms the necessity of medical foods treatment for PKU for life.
- Treatment for PKU is currently covered in 39 states through a state insurance mandate or state program. However, this coverage only benefits a small percentage of PKU patients.
- Failure to include coverage for medical foods for all patients with PKU in the federal health programs is not in accordance with the accepted standard of medical care.
- The impact of this lack of coverage on patients with PKU is disastrous and expensive. The average family cannot afford to pay for medical foods without insurance coverage.
- The long-term costs to the government for the care of untreated children and adults with PKU far exceed the cost of providing this essential treatment.

Decades ago, before the implementation of newborn screening and treatment with medical foods, children with PKU were doomed to a life of intellectual disability and costly institutionalization. Now, because of mandatory newborn screening and the proven treatment with medical foods, children and adults with PKU can lead normal and healthy lives. Don't put these lives at risk.

Please ensure that medical foods for the treatment of PKU are provided by the federal health programs and pass H.R. 3665, the Medical Foods Equity Act, so that everyone with PKU can grow up and become healthy and productive citizens of this country.

Sincerely,

Heidi [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]

Thank you for this opportunity to help speed the approval of critically needed drugs for suffering children and adults everywhere.

My son [REDACTED] was diagnosed at age 7 in December 2009 with Friedreich's ataxia (FA) a debilitating, life-shortening, degenerative neuro-muscular disorder. [REDACTED] has gone from an active happy little boy playing baseball on the All-star Team to a pre-teen who can not get around without assistance such as a wheelchair. Many nights he asks why can't he walk anymore and how unfair it is that he could only walk 10 years. [REDACTED] s very bright which sometimes makes this more difficult. Friedreich's Ataxia does not affect victims cognitively. Learn more about Friedreich's Ataxia here <http://www.curefa.org/whatis.html>

We are lucky because we do have some wonderful people working toward a treatment and hopefully a cure one day although it can not come soon enough for [REDACTED] I would definitely talk with FARA, Friedreich's Ataxia Research Alliance, for advance and how you could better help [REDACTED] and others overcome this devastating disease. I have tried to answer some of your questions below.

Sincerely,

Hope [REDACTED]  
Mom to [REDACTED] with Friedreich's Ataxia (FA) age 12

**What is the state of discovery of cures and treatments for your disease? Are there cures and treatments now or on the horizon?**

Currently there are no FDA (nor other country drug safety organization) approved treatments for Friedreich's Ataxia. There several drugs in clinical trial right now but none are approved. Gene therapy seems to be the most promising toward a cure however that is probably 5 to 10 years away. Time is passing and our children are dying, so your interest in speeding the approval process is of the utmost interest to us. See the status of FA research here. <http://www.curefa.org/pipeline.html>

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

FARA has created a patient registry which helps recruit patients for drug trials. [REDACTED] actually participated in a trial this year. Unfortunately we did not see any benefits. About one in 50,000

people in the United States have Friedreich's Ataxia. Because this disease is rare it is not well known or understood, we travel each year to Philadelphia to see an expert in this disease. The FA-family has a parent group (through e-mail) set up to help support each other as the disease progresses.

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

Have the NIH pay for it since many drug companies won't be interested until translational research shows promise. For Rare disorders the subject of who to fund can be difficult.

Congress could stop cutting the budget of the FDA and the NIH!! You do not "incentivize" nor "accelerate" by taking away their money. The FDA is being mandated to expand their various scopes of responsibility and at the same time their budget is constantly at risk and does not increase easily.

Perks for orphan designation and fast track was a step in the right direction.

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

Join the collaboration between patient organizations, drug companies and researchers to identify the technologies and how to integrate them into the testing and review processes. This has to be funded.

**How do you coordinate your research and outreach with other patients?**

Through communication in the FAPG email group, FA Facebook groups, FARA FA Registry notifications and the FARA news distribution list.

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

Because this disease is rare it is not well known or understood, we travel each year to Philadelphia to see an expert in this disease. Each year Dr [REDACTED] tells us about the progress that is being made toward finding a cure. The FA-family has a parent group (through e-mail) set up to help support each other as the disease progresses.

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

That collaboration and teamwork do work. Adversarial relationships do not work as well or as fast. Drug development process is frustrating because ultimately it is about money and whether or not a drug will be profitable. To many this is the difference between life or death.

Patient and their families should be financially supported to participate in drug trials. For the drug trial we participated in we traveled 8 times across country in a four month period. Thankfully our local community helped with this financial burden along with a local business.

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

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

Many current drugs being used now have a huge list of possible side effects and risks. Seems drugs under development are held to a higher standard in my opinion. It feels like the process is moving at a snails pace.

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

For me public funding should be used when private funding is not forthcoming or inadequate.

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

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

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

The financial and mental burden varies as FA presents itself in each individual. Many patients receive therapy such as speech, physical and occupational therapy trying to delay or maintain current abilities. Different assistive devices will be used as the disease progresses.

Many/most FA'ers never work so they are on SSI and/or on SSDI (retired parent) getting \$600-ish to \$1,000-ish a month to live on. If they live with someone SSI removes \$300-ish for room and board. If they live independently it is a big financial struggle just to live. Parents help to the limit of their own budgets and the limits set down by SSI.

Copays, PT/dental/acupuncture not covered, supplements thought to perhaps help, exercise equipment, ramps, bathroom adaptations, wheelchair maintenance, etc are areas of extended cost.

Caregiving is needed for many adult FA'ers but even the hours that are given (often none) are not adequate. Parents wear out, get old and get injured/sick. Many FA'ers desiring to live independently cannot because they cannot get/afford caregivers.

**How can Congress help?**

More money to help support NIH and FDA and companies to working toward treatments/cures for rare disease. Commitment to finding a cure!

It takes a diagnosis to develop a cure. Living with and without one I hope my opinion and experience as a patient and entrepreneur can be of value.

Over the past 26 years I have traveled to visit the most awarded doctors at the best hospitals around America. At the age of three I began collecting diagnosis starting with Crohn's disease. In kindergarten I was diagnosed with CVID / hypogammaglobulinemia. They kept coming – Rheumatoid arthritis, SVT, Chronic Fatigue Syndrome, POTS, Chiari malformation, connective tissue disease....

By taking my medication I was able to push through symptoms. What kept me going was a bright future and outlook on things – medicine would continue to progress and I would be alright. I graduated from college in 2011 with three majors – Biology, Finance, and liberal arts.

I underwent brain surgery after graduation and my group of symptoms escalated. More abnormal test results were found and the doctors were still no closer to figuring out a diagnosis. Physicians are good at running tests and ruling out possible diagnoses. It was easy to tell me what I didn't have: Scleroderma, Stiff Skin Syndrome, or Buschke-Ollendorff syndrome. Everyone agreed I had something seriously wrong but no one established or could come to a conclusion what it was. By the age of 20 I had gone through an unimaginable amount of insurance. I was on the high risk pool in my state; my mother had to plead my case and lobbied for an increased maximum lifetime.

I am now at a stumbling block, candidate gene defects have been identified, through exome sequencing of my family and my DNA. My team of physicians, at Johns Hopkins, are stumped. I have been referred to the Undiagnosed Diseases Program at the NIH. Being at my last hope and hearing nothing back for months, it is a grueling wait.

There are many in need of services to try and get a diagnosis and then a possible treatment. Treatment needs to come before it is too late and the disease has caused too much damage enjoy life, or to be able to use the skills one had worked so hard for in college and planned on expanding in graduate school. The undiagnosed disease program needs to be expanded so wait times are not so long and it does not become too late for help. The NIH cannot be shut down for sequestration or government closure as many patients rely on this as their last only hope for life.

It is imperative to have library access, such as those available to students at medical institutions. Patients going through a rare disease need access to academic journals beyond what has been made open access. As a patient with a rare or undiagnosed disease it requires you to become your own advocate, learn about connected symptoms, and read primary literature. I have been fortunate to have access to libraries, as my sister has been in graduate school. I make time to do research. Doctors are now being forced to see more and more patients each day. A majority don't have the time to spend hours searching through literature and reading for one abnormal patient. I have often found relevant information that doctors were not aware of and many times they are very open to suggestions. Reading papers on similar diseases and symptoms has led me to doctors that had expertise in the area that I needed. Everyone needs access to this knowledge.

The estimated ninety-eight million dollars each year that are paid out in Medicare and Medicaid fraud would provide a great deal of funding for untreated diseases. A concentrated effort needs to be made to cut fraud and reallocate these funds to better uses.

There must be legislation so full electronic medical records are made available to patients, including the relevant and pertinent information in the clinical notes. The way meaningful use standards were designed, no assistance is given to the future of medicine – linking phenotypic and genotypic data together. The Blue Button Initiative does not provide enough information to patients or other doctors when patients are trying to transfer their health records from place to place. E-prescribing exchanges, such as SureScripts, must be opened up so patients can get direct access to information regarding all medications and medication reconciliation can be eased. Fully open and free movement of data is needed for an Undiagnosed Disease Network Repository where genotypic and phenotypic information can be linked and new discoveries can be made as the study translational bioinformatics matures.

In regards to re-purposing drugs - If a new pathway for exclusivity is generated to create economic incentive for re-purposing drugs the process must be designed around the patient and the physician as there are already enough complexities when working to get specialty pharmaceuticals approved. Many fail to understand that there are often no tests to diagnose rare diseases as there is often not a treatment. Therefore, trying a drug and seeing if it works is often a diagnostic measure and if the drug works it is a tool for diagnosis. As it stands now insurance companies will not approve a drug for experimental uses. With a rare disease doctors need to treat symptoms and treating the symptoms may confirm a diagnoses if the medication works.

The FDA should use more compassion and discretion in using experimental drugs. If there is a patient suffering from a disease that will become disfigure, disabled or is going to be terminal the FDA should allow the use of an experimental drug that shows compelling promise in the ability to help the patient. The FDA should not protect someone to death.

Having a diagnosis is a luxury; having a new disease does not have that luxury. If this country wants to have the best health care system in the world, patients must be able to rest at ease knowing that research is going on for their condition, entrepreneurs have incentive to bring their future treatment to market, and there is an FDA that shows much compassion.

Dear Chairman Upton and Representative DeGette,

You have asked for feedback on your whitepaper “21st Century Cures: An Update on the President’s Council of Advisors on Science and Technology 2012 Report on Propelling Innovation.”

As a veteran, I’m particularly concerned about antibiotic resistance. It’s a threat to all of us, but a particular danger to wounded warriors and veterans who are dying this very day from infectious disease superbugs that existing antibiotics cannot stop.

I would urge you to address the PCAST recommendations by advancing H.R. 3742 – the Antibiotic Development to Advance Patient Treatment (ADAPT) Act. ADAPT is an important piece of legislation which can help speed antibiotics to patients with serious unmet needs by establishing a new regulatory pathway for novel antibiotics that are intended to be used in limited populations of people with no other treatment options. The pathway established in ADAPT responds to Recommendation 4 of the 2012 PCAST report, targeted to antibiotics.

Since the wars in Afghanistan and Iraq began, our military health systems have advanced thanks to speedy evacuation and cutting-edge medical technology. Wounds that were once fatal can now be survived, and many injured soldiers go on to lead healthy and productive lives. But too often, grave wounds received in battle render soldiers vulnerable to drug-resistant infections.

Our service men and women and our veterans need cures fast, and I salute your efforts to ensure that innovative treatments get to market. I urge you to act on the 2012 PCAST recommendations and advance your innovation initiative by moving H.R. 3742.

Sincerely,

Rear Admiral [Ret.] James [REDACTED]

Rear Admiral [Ret.] James [REDACTED]  
[REDACTED]

To whom it may concern;

I'm writing to share and describe my personal experience with Polycystic Kidney Disease (PKD) and the passion I have to create attention in the U.S. and for government involvement to become the leader in medical/technical advancements for untreated diseases.

My personal experience with PKD involves my wonderful fiancée and her father. Since PKD is a genetic disease, it was hard for her to go through the process to determine whether or not she would be affected by it, but through general curiosity she went ahead and found out she had it and would travel a similar path as her father. She is 28, and should not have to worry about a lifelong disease -- especially handed down through genetics. It has since damaged her self esteem slightly and created stress. We have agreed not to live life acknowledging the disease, but due to the lack of awareness of the disease, we must make it a part of our lives. This is not to mention an ill father for whom we stand for as well. When we are looking for preventative care and advice, doctors would rather encourage the ancient treatment of dialysis rather give any advice on herbal, natural, or food remedies. Why not? The flawed medical system would rather get a customer for life than encourage a healthy lifestyle or study for a treatment. Why can't we change that?

Lastly, the U.S. is removed from leading areas of technological advancements, in most cases medical procedures and cures. Allocating money towards curing diseases that are long term, painful, and widespread can be an achievement if more attention were put towards it. It seems as if certain issues don't get a spotlight until someone in a power position is afflicted with it. *For example, rarely do politicians' children enlist in the military, though many politicians create false military values that are woven into their politics.* The United States should lead the pack in finding cures and treatments for diseases, starting with putting its citizens first.

My goal is to create attention, and I hope that with this brief letter I have shone a small light somewhere to accelerate the steps to cures. I love my fiancée with all my heart, and would love her no less due to any hardship. Her worries only inspire me to want to relieve any discomfort, suffering, and stress, when in fact its presence is unnecessary.

It's very strange to try to be an advocate for saving lives and curing diseases. It seems like we have put health on the back burner, since it is not as profitable as other issues.

Thanks for your time,

--

**James** [REDACTED]  
[REDACTED]  
[REDACTED]



According to the Energy commerce website, "If we want to save more lives and keep this country the leader in medical innovation, we have to make sure there's not a major gap between the science of cures and the way we regulate these therapies."

Fred Upton,

I read an online article about the meeting in Washington this week to encourage more funding for medical cures.

As a local Naturopathic doctor in your district in Michigan, I know that NIH is working with some recognized Functional Medicine doctors to understand the need to improve medical care for chronic diseases. Dr. [REDACTED] and Dr. [REDACTED] are two doctors who train other medical doctors and naturopathic doctors to understand why we have chronic symptoms. They take the time needed to understand the whole person and not just recommend a drug to cover up the current symptom.

This is where the problem with health care comes in today. We look for the magic pill to get rid of the symptoms we are having. But most medications alter the biochemistry of the body changing the function and causing deeper and more chronic symptoms. Dr. [REDACTED] has a brand new book out called Disease Delusion. I have read most of it and am recommending other doctors to read it.

When we take many medications we get chronic symptoms that are more difficult to reverse. This is especially true for the increasing incidence of Alzheimer's Disease. I know three very common medications that taken over a long period of time lead to dementia. Even the AARP magazine has had articles about these medications causing Alzheimer's. So this knowledge is available but not acted upon by many medical doctors.

Since health care is a huge business for our country, there are few incentives to truly heal patients from disease. I personally know 2 biologists that used to work for pharmaceutical companies where cancer "cures" were suppressed because they potentially could be too effective. I also know medical doctors who have recommended proven natural remedies with less cost only to be turned down by the insurance companies because they were within not the standard of care protocol. Cures are available, they just need to be recognized and funded.

I personally do not take insurance payments, so I am not part of the health care system. My clients come to be educated on how to live a healthier life by understanding their own patterns of stress, and taking action to reduce those stress reactions. Many of my clients have health improvement by using tested natural remedies that support their individual healing process.

Jane [REDACTED]  
[REDACTED]

One important thing that needs to be regulated is food labeling. Consumers have the right to know if their food has been genetically modified. Companies do not have the right to hide from us information that could be harmful. It seems so simple. How can we be denied information about our food?

Jane [REDACTED]

[REDACTED]

Attn: Members of 21<sup>st</sup> Century Cures

**FDA FAILS TO APPLY EQUAL STANDARDS LEAVING PATIENTS TO SUFFER**

*NEWS Flash... Aduro BioTech, Inc. has received a “breakthrough designation” after positive clinical evidence in the treatment of pancreatic cancer. A breakthrough designation is reserved for drugs that would treat a serious or life threatening condition and preliminary clinical evidence shows great potential for improvement over available therapies, the FDA states. The San Francisco Times reported that the FDA's action could result in drugs being approved in as soon as 60-days, but it does not guarantee approval of the therapy.*

**If I had cancer instead of a devastating Myalgic Encephalomyelitis/Chronic Fatigue Syndrome (ME/CFS), the FDA would be willing to let me have treatment.**

Drugs with risks of fatal autoimmune (Yervoy) and other extreme adverse events are perfectly justified if I might live a month longer – yet being sick for more than two decades and unable to participate in life from a disease that costs this nation more than \$22 billion a year **warrants nothing! There are no approved therapies for ME/CFS.**

Ampligen is the only treatment that has positively shown to help those with ME/CFS – and has been provided to patients via an open label trial for more than a decade – clinical trials covering 90,000 doses. It is **deemed safe for approval** by the top experts in the field and by the FDA advisory committee (Dec. 2012), yet the FDA continues to deny patients the opportunity for treatment.

Why? because they say they are unsure of its efficacy although they admitted after denying approval that they did not understand the disease.

**THERE IS NO JUSTIFICATION FOR FAILURE TO PROVIDE TREATMENT.**

FDA has the power to approve Ampligen with conditions. FDA is to protect public health not deny it. Give us the right to choose our care. We want our lives back. This isn't a game – it's the lives of more than 1 million Americans.

Janice [REDACTED]  
[REDACTED]  
[REDACTED]

I live in Jacksonville FL and have severe Rheumatoid Arthritis. I am 51 years old and was diagnosed when I was 25. My disease has progressively worsened even while taking medications. I have had 10 joint surgeries.

While I hope for a cure in my lifetime I'm realistic. Unfortunately I'm not a candidate for the Biologic drugs which are supposed to help prevent further joint damage. The newer medications that are coming out are intimidating because they tout possible frightening side effects and even death!

I'm blessed to have Medicare which covers my treatment but my current basic rheumatoid medication is expensive and it still doesn't relieve my pain completely. The FDA recently approved the generic form of Celebrex (Rx I take), but I'm still waiting for my pharmacy to receive it.

Please help bring Arthritis to the forefront of Congress so a cure may be found in my lifetime.

Sincerely,

Janine [REDACTED]

[REDACTED] Republican and proud registered voter since 1980



Hello,

I am writing in regards to the call for patient input in the 21s Century Cures initiative.

I am a primary care physician who treats many patients with Autosomal Dominant Polycystic Kidney Disease (ADPKD). I am a patient with this disease as well. This is an incredibly common disease that affects as many as 1 in 500 people in the United States. To put that in perspective, given the roughly 8 million people in the Chicagoland area, this disease would affect upwards of 16,000 individual patients (or over 600,000 affected patients in the entire United States). The disease uniformly leads to renal failure in all affected patients. Given it's autosomal dominant mode of transmission, affected patients have a 50% of passing the disease on to each child they have.

This disease poses a tremendous personal burden to the patients affected as well as a financial burden to the healthcare system as, currently, the only treatment available is dialysis and/or kidney transplant. These treatments both carry enormous financial strain both on the individual patient and the healthcare system as a whole.

Beyond the financial burdens, there is significant morbidity associated with both dialysis and transplant, including increased infection rates, increased severity of infections when they occur and significant decrease in quality of life associated with ongoing medical therapies.

It is extremely important that effective treatment is discovered that can postpone or even avoid end stage renal failure in these patients. Some promising research has been conducted, most recently involving tolvaptan, which was unfortunately not approved by the FDA. However, much more needs to be done to alleviate the suffering and burdens on the hundreds of thousands of Americans affected by this disease as well as the strain this places on US healthcare expenditures. At an estimated \$87,000/year expenditure per patient on dialysis, it is clear that preventing end stage renal disease requiring hemodialysis treatment should be a priority in the era of the Affordable Care Act and general efforts to reduce annual healthcare spending.

Thank you for your time and consideration of this very important issue.

Sincerely,

Jason [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]

Dear Committee Member:

I am a PKD patient , went through dialysis and kidney transplant. It was tremendous suffering and cost to went through this process. It is especially sad for the patients because this is a genetic disease. It passes on to our kids and how horrible to see them will go through the same suffering all over again!! With all the progress in medicine, there is still no medicine to slow down the progression of the cyst! It is imperative to have more research support for finding medicine to slow down or stop the growth of the cysts!

It will not only reduce the human suffering but also save the cost for government for dialysis and transplant. I sincerely hope you will support this endeavor .

If there is anything opportunity to do this research,I will be most happy to dedicate my life for it. Please kindly let me know.

Best regards,

Sincerely,

Jean [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]

Thank you for sponsoring legislation to support the development of cures for rare diseases. Two of my sons, now in their 40s, have Friedreich's Ataxia. They were diagnosed in their 20s, and we have watched this disease rob them of the ability to walk, to hear, to play the music they dearly loved. We are hopeful that treatments might be discovered that will halt the progress of Friedreich's Ataxia for them and that a cure might be on the horizon for the many young people who suffer from this condition, and whose stories you can see on the FARA Website (<http://www.curefa.org>).

\* What is the state of discovery of cures and treatments for your disease?  
Are there cures and treatments now or on the horizon?

*FARA is doing a great job of engaging medical professionals and pharmaceutical companies in research and development of treatments for FA. No treatments/cures yet available.*

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

*All of these are being used by FARA. Parents are working hard to raise money for research.*

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

*Learn about these diseases and the people who suffer because of them. Encourage appropriations to fund NIH and FDA.*

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

*As much as we want treatments/cures, we also want them to be safe for our children. We need to support testing in a timely way.*

\* How do you coordinate your research and outreach with other patients?

*We communicate by a wonderful listserve of parents whose children suffer from FA. One of our members regularly sends information about journal articles touching on this disease. We read the FARA newsletter and Generations, the newsletter from the National Ataxia Foundation.*

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

*In addition to the above answer, we attend an annual gathering sponsored by NAF to share the latest information about ataxias and to provide and offer support.*

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

*That it moves very slowly!*

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

*We depend on NIH and the FDA to advance breakthroughs. Fund them.*

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

*Question for the experts. We need safe treatments.*

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

*Both are important. We need to know that legislators care about us.*

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

*Best information will come from FARA.*

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

*Parents' support one another through the listserve and the annual meeting.*

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

*Financial considerations for durable medical equipment alone are high. When people become so disabled that work is not possible, we all suffer.*

\* How can Congress help?

*Funding and publicity.*

Many thanks,  
Jeanne [REDACTED]

To whom it may concern,

After reading the Breitbart piece entitled “Hope Comes to RHOB 2123—Fred Upton Leads a Cure Strategy for the 21<sup>st</sup> Century,” I’m writing to commend Rep. Upton & DeGette for their leadership on this issue as well as urge them to continue to press forward. As a 30 year old with a so-called “orphan disease” – Cystic Fibrosis – I can attest to both the need for greater urgency in pursuing cures and the results of that urgency as I have benefitted from the incredible work of the Cystic Fibrosis Foundation and their “venture philanthropy” model. I’m certain there are millions of other Americans that have a story similar to mine and appreciate the efforts of this bipartisan initiative. Keep going!

Jeff [REDACTED]

[REDACTED]  
[REDACTED]  
[REDACTED]

[REDACTED]  
[REDACTED]  
[REDACTED]

I read your update of six priorities for 21st Century Cures. I wish you could add one that is focused on expanding or accelerating access to advanced diagnostics. I believe the impact of molecular diagnostics is underappreciated by payers. The path from the research lab to the clinic runs directly through the clinical lab. All the great advancements in our knowledge in disease management will have zero impact on medical decision making without an efficient mechanism to commercialize the discovery. Better diagnostics will improve delivery of healthcare and save money for tax payers.

Joe [REDACTED]  
[REDACTED]

I'd like to submit to congress that the Palmetto GBA MolDx program is a nothing more than a mechanism to exclude CMS payment of advanced molecular diagnostics. The newsletter published on January 7 provides an updated exclusion list of genetic procedures that will be denied by CMS.

Honestly, it would be easier if CMS would just exclude the entire human genome from payment consideration. This is ridiculous.

[http://www.palmettogba.com/palmetto/MolDX.nsf/ls/MolDX~9E8NBK5152?opendocument&utm\\_source=MDX&utm\\_campaign=MDXs&utm\\_medium=email](http://www.palmettogba.com/palmetto/MolDX.nsf/ls/MolDX~9E8NBK5152?opendocument&utm_source=MDX&utm_campaign=MDXs&utm_medium=email)

Joe [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]

I support the efforts of the 21<sup>st</sup> Century Cures initiative. However, there are roadblocks being constructed that if not dealt with by congressional action, the path to curing difficult disease states will have a hard time taking off. Medicare, and in particular the MACs have made it a priority to minimize and deny the use of advanced molecular diagnostics. One particular MAC – Palmetto GBA – is using the MolDx program to set diagnostic advances back 10 years. Investment in this sector has virtually dried up since coding changes were enacted in January 2013, and the problem has only gotten worse.

I strongly encourage the members of 21<sup>st</sup> Century Cures to engage in the debate over coding changes in molecular diagnostic procedures. The path from “discovery” in government funded university labs to 21<sup>st</sup> Century Cures in the clinic runs directly through the clinical laboratory. Physicians need better tests to make better decisions! Congress needs to pass legislation that opens the use of molecular diagnostics to Medicare patients and their families. The use of early stage detection and screening for genetic predisposition to disease has the potential to return vast amounts of value to the system ... far more than what the projected spend on better diagnostics will ever be. Please add diagnostics to your agenda. This is a critical time in our progression to personalized medicine and CMS is a major roadblock to progress...and future savings will never be achieved if we don't take advantage of technological advances in molecular diagnostics. It is the only way to match a patient to better therapies and reduce end of life spending on people that can be cured.

Joe [REDACTED]  
[REDACTED]  
[REDACTED]

Dear Representatives Upton and DeGette:

I'm responding to the latest email from 21<sup>st</sup> Century Cures titled "How the US can Remain the World Leader in Medical Innovation". I applauded the work of this bipartisan effort and support your individual commitments to enable innovation in healthcare. At the same time, I remain concerned at the lack of serious discussion of clinical diagnostic procedures and how advances in basic research MUST flow through the clinical diagnostic lab before they can every reach patients.

The diagnostic industry is at a real crossroads. Insurance companies are hell bent on driving diagnostic costs down by claiming all new procedures are experimental regardless of the patient's circumstance or the doctor's need for better information. Investment in diagnostics will dry up without payers in the system that are willing to pay for test procedures. There is a desperate need to streamline the development of promising diagnostics and to provide a mechanism get paid for performing the tests.

Changes in Medicare reimbursement policy in January 2013 turned the molecular diagnostic industry upside down. In an effort to improve clarity into testing procedures, CMS decided to throw the baby out with the bathwater; all major insurers have followed suit. The result is significantly reduced access to molecular diagnostics for all citizens. Your letter today pointed out how much less it costs to map a patients genome compared to 10 years ago..."1/10,000<sup>th</sup> the cost". Ironically, although the cost has come down dramatically, there is almost no way to get this test without paying out of pocket, virtually the same as it was 10 years ago. I suspect that most genomic testing is currently performed at large medical centers that do the tests because they improve outcomes. In this setting, cost reimbursement is an afterthought. On the whole patient access to molecular diagnostics significantly favors the wealthy and/or highly educated segments of our society.

Investment dollars are fleeing start-ups trying to bring molecular diagnostics to market. I think it would be helpful if 21<sup>st</sup> Century Cures made specific mention of the need for advanced molecular diagnostics in its vision of the future of medicine. Without a testing platform by which to identify the need for personalized medicine (precision medicine), the concept of faster cures become a little nebulous. Targeted therapeutics only work when a physician sees the target. I welcome you both to my lab in Charlotte NC to witness how we are trying to enable personalized medicine.

I believe payers will eventually be forced to embrace genomic information to improve treatment. I thank you for your efforts to advance this vision. I urge you to include diagnostics in your game plan. As there is a placeholder in the proposed legislation, I'm providing three suggestions for strategic focus of this section of your bill:

1. Mandate CMS to actively manage the review and reimbursement approval of molecular diagnostics with the explicit goal of improving disease state management. A management mandate should place limits on how much of this process can be delegated to the MACs, whose mission is often times diametrically opposed to expanding access to new technologies as they are heavily focused on costs to the system in the near term.

2. Mandate the development of a diagnostics review framework within CMS that encourages step by step review of the reimbursement decision. This would be similar to the PMA process in the FDA. Currently, a company that wants to get reimbursement for a new diagnostic at CMS is required to go through the entire process of demonstrating clinical utility with little input from CMS with regard to the medical need, the indicated use, and the disease state management goals for the test. This is a multi-million dollar risk to the developer and there is no way to control the expenditures by gauging CMS along the way (or to capture CMS input that could improve the test). There needs to be a feedback mechanism to guide developers in their efforts to commercialize a new test by gaining a favorable reimbursement decision.
3. Ensure streamlined regulatory oversight of diagnostic procedures. As the FDA moves to regulate laboratory developed tests (LDTs), we are skating on thin ice with regard to a split regulatory environment involving the CDC (CLIA), CMS, and the FDA. That is not in the best interest of a streamlined framework for the diagnostic procedures that will be required to enable the 21<sup>st</sup> Century Cures initiative.

Thank you.

Joe [REDACTED]  
[REDACTED]  
[REDACTED]

Mr. Upton, Ms DeGette, I see two problems with your initiative. 1) Unless the tax on medical devices (actually the entire ACA) is repealed I wouldn't anticipate a rush, by manufacturers, to research and/or produce new devices. 2) The decision about what therapy a patient wants, not needs, MUST be taken out of the hands of the government. As long as these two mechanisms remain in place the advancements you both seek won't take place. As long as researchers and doctors know that the federal government can, at any time with any patient, make the patient wait for periods of time that are unreasonable relative to the issue the patient is having, the motivation to develop advancements just won't occur.

In my opinion the first thing that needs to be addressed are all the road blocks via the ACA need to be removed. Doing this first, I believe will give doctors and researchers the confidence of knowing they can move forward with treatments/research projects without fear of government intervention. Again, just my opinion.

Jeff [REDACTED]

[REDACTED]

The "FDA cleared fully validated tests" pushed by the CDC rely upon decades old technology and miss approximately 1/2 of actual cases pursuant to numerous peer reviewed studies. In early infection, when treatment is most effective, CDC recommended tests miss up to 70% of actual cases. Without a sufficiently sensitive and reliable test for Lyme Disease, it is impossible to determine with certainty who has an active infection verses past exposure. Notwithstanding the unreliability of CDC approved tests, the CDC and FDA have recently taken steps to limit access to more sensitive testing, further compounding the problem. Tick-borne co-infections are also frequently missed using currently available laboratory tests further diminishing the chances of a successful treatment regimen.

While the CDC estimates that up to 20% of patients will suffer persistent symptoms after standard Lyme treatment, in a study by researchers at Johns Hopkins University, the treatment failure rate for early Lyme disease was estimated to be as high as 36%. Other studies have shown that with late Lyme disease, treatment failure rates may exceed 50%.

Chronic symptoms following Lyme disease may be long lasting, may significantly impair patient quality of life, and may be costly to patients, employers, healthcare systems, and society. In a survey of more than 5,000 patients with chronic Lyme, half report that they have been ill for more than 10 years. These patients suffer a worse quality of life than those with most other chronic illnesses, including congestive heart failure, diabetes, MS and arthritis. Over 43% report that they had to stop working and 25% report that they have been on disability at some point in their illness. They are five times more likely to visit healthcare providers and twice as likely to be seen in emergency rooms compared to the general population.

In August 2013, the CDC dramatically revised its estimate of the annual incidence of Lyme disease from roughly 30,000 cases per year to over 300,000 cases in the United States, a ten-fold increase. To put this in perspective, the annual incidence of Lyme disease is now 1.5 times more than the estimated number of cases of breast cancer and six times higher than the annual incidence of HIV/AIDS. However, federal funding of Lyme disease has been meager. For example, while Lyme disease occurs six times more often annually than HIV/AIDS, it receives less than 1% of the funding allotted to HIV/AIDS by the National Institutes of Health.

It is time to put aside the agendas, dogma, and presumptions about Lyme Disease. We need fresh research to explore divergent strains, testing to detect such strains, the impact of co-infections, to what extent other ticks spread Lyme disease to humans (including the Lone Star tick as discovered by Dr. Kerry Clark and other researchers), geographic reach of the disease, and more effective treatment options beyond short term mono-therapy that has failure rates of up to 50%.

My life and my struggle matter; I'm sick of having to fight for an effective standard of care that will allow me to work and live my life, and I'm sick of spending the majority of my income on treatment because government agencies allow or even encourage insurance companies to renege on their responsibility to treat me. There are too many people sick from Lyme—you can't keep implying that we are all delusional and relegating us to disability and suffering. The U.S. can't AFFORD to continue on this path, when 20% of Americans are on some sort of disability. Are you going to ignore this epidemic until the country is 50% disabled? Help us become productive again. We don't want to have Lyme. We want to be cured.

Thank you,  
Joyce 

Hello,

My mom was overdosed (killed) by her chemotherapy treatment - 5FU.

Linda [REDACTED] was a 73 year old woman, otherwise healthy, diagnosed with poorly differentiated squamous cell cancer of the rectum. Her cancer treatment was to be two 96 hour 5FU treatments at week one and week 7 with radiation Monday through Friday for the duration.

Her 96 hour drip started on a Monday and by Thursday she was vomiting and having diarrhea. By Saturday am, having been an RN in her former career, she knew she was dehydrated from the vomiting/diarrhea/lethargy. She had her husband drive her to the ER. They hydrated her and sent her home. Only in hindsight do we now know that we should have asked them about 5FU toxicity. The ER knew she was having this chemo treatment, however, they lacked the training to spot 5FU toxicity. If they had been trained - they may have been successful in administering the antidote and saving a vibrant 73 year old. Unfortunately for her family, they did not do this. She was released from the ER. She suffered at home where we couldn't get any food or liquid other than ice chips in her. When we called her oncologist Monday - they had her husband come in for a mouth rinse to deaden the mouth pain. We kick ourselves now for not bringing her in that day (Monday- one week since chemo began). It was Wednesday morning about 4 am when the paramedics took her to the hospital from which she would not leave alive.

Linda [REDACTED] family wants to help make pretesting for 5FU toxicity a standard of care. We do not want any other family to suffer this tragic loss of a healthy active wife, mother, grandmother, great grandmother, sister, friend and neighbor.

Our question to you is how can we help push this pretesting for 5FU toxicity? In personalized patient care, testing should be standard.

Sincerely,

Karen [REDACTED]

Dear Sir,

I was very pleased to read of this important legislation. I am a clinical trial designer and oftentimes when giving lectures point out the extraordinary irony that we as biostatisticians are tasked with identifying whether the newest, most technologically advanced medicines and medical devices are safe and effective, but typically required by the U.S. government to use 50 to 100-year-old statistical methods to do so.

In addition to working with academic investigators and medical device, biotech and pharmaceutical companies, I teach a weeklong class each summer at the [REDACTED]. Adaptive designs are clinical trials designs that learn as they go and seek the correct scientific answer. Treatments in these trials are usually blinded to patients and caregivers and scientifically sound.

The device group within the FDA has been very amenable to innovative designs, however the Center for Drug Evaluation and Research -- where I think the most is to be gained -- has been amazingly hesitant to incorporate these advanced methods.

For instance, currently in Phase 3 trials (the final stage before a drug approval) companies must identify precisely the right dose and precisely the right patient population then run a very expensive (20-100+ million dollar) clinical trial. If the dose is not optimal, or they're enrolling a type of patient who may not benefit from this new therapy, then the expensive trial is bound to fail -- a fact they may not realize until after spending enormous time, money, and other resources.

Whereas with a adaptive trials, using prospective rules, we can update the dose and/or identify the optimal group of patients in whom the drug is effective during the ongoing trial. Currently, the trial is left to fail, then if the company has any remaining resources, they have to run another trial from the start. With adaptive designs, the trial can learn in real-time the right dose or patient population.

I oftentimes make the analogy that most clinical trials are like ballistic missiles: without knowing if a drug or device works, a company or research has to declare how much it works (to define a sampel size), declare in whom is works, etc, then run the trial. If he or she is slightly off, then the trial misses its mark. Whereas adaptive trials are like new 'smart bombs'. All the same prior calculations are performed, but after 'launch' the trial continues to monitor its own progress. If minor adjustments are necessary, they occur automatically using predefined rules. They are far more likely to identify useful therapies, and even identify in whom these therapies are most beneficial. And like smart bombs vs. ballistic missiles, there is much less collateral damage.

I and my colleagues are some of the world leaders in adaptive trial design and would be very happy to discuss the benefits and fairly discuss the concerns with such designs. They aren't always the answer, but most often they are more efficient than standard clinical trials which are slow, expensive, and certainly drive up drug costs while not working toward our 21st century goal of personalize medicine.

But most importantly we are thrilled that legislators are exploring the benefits of these techniques and the benefits they may bring to patients.

Sincerely,  
Jason [REDACTED]

[REDACTED]

please ,keep working together that is what is needed in this country

Sent from my iPad jack

Dear Congressman Upton - your plan sounds good. And I hope by now you have stopped voting to repeal the ACA which has helped 11.4 million citizens get health insurance and Heath care. If more Republican controlled states would accept Medicaid extension like Michigan has done, millions more would get medical care.

I urge you to send a message to the Supreme Court asking them to uphold the federal subsidies in the ACA for all states.

Thank you for reading my message.

Joseph

[REDACTED]

[REDACTED]

To The Honorable Fred Upton,

I'm encouraged to see that you are leading the way when it comes to patients receiving timely cures. As a longtime supporter and resident of Southwest Michigan I've witnessed your passion at championing what's best for Southwest Michigan.

I am a [REDACTED] working for [REDACTED] (A leader in women's health). Hologic introduced the Thinprep T-5000 (diagnostic device for Gyn and Non-gyn testing) over 5 years ago. The T-5000 is used extensively in Europe and Asia, but not in the U.S.. This is due to the device waiting on FDA approval for Gyn testing. If there is anything you can do as a part of the 21st Century Cures Initiative to accelerate the approval of the T-5000 for Gyn testing it would be greatly appreciated.

Thank you,

Glenn

[REDACTED]

Good info, glad to see cooperation here.

But "cures" are only good if they are accessible to those who need them. The ACA has given millions of people access to affordable health care, yet you voted to destroy it many times. Even though it was modeled after Romney's program, using Romney experts, you voted to keep people from getting health care.

Maybe you have reformed with this new initiative, or maybe you are 2 faced - time will tell.

Dr. Jack [REDACTED]

Representative Upton,

I realize that your initiative focuses on new products and there is definitely a need for that. I am, however, very concerned about the cost that will be charged for those as well as currently available meds and devices.

The costs of generic medicines are continually rising, most increase are for the simple reason of making more profit. Another example of the exorbitant costs of medicine is the relatively new Hep C med. A 12 week course of this med costs \$84,000. The costs for the production of the med, the research that went into it and marketing is at most \$8,000. No one really deserves profits at that level.

Is there anyway that your initiative can put some of its' focus into the costs of health care.

Thank you,

Jim

[Redacted signature]

[Redacted contact information]

To whom it may concern at [Cures@mail.hous.gov](mailto:Cures@mail.hous.gov).

I am a concerned citizen responding to your one pager describing the **21<sup>ST</sup> CENTURY CURES DISCUSSION DOCUMENT**.

I have worked in the clinical trial “space” for both Academia and the pharmaceutical industry for over 25 years and I am currently a statistical consultant to the pharmaceutical industry. I am following these activities because they will influence the way clinical trials are conducted in the future as well as the ability to obtain data on clinical trials run in the past. The bill written covers many topics of interest to medical research workers like myself and the emphasis of the bill indicates under the first title:

- **PUTTING PATIENTS FIRST BY INCORPORATING THEIR PERSPECTIVES INTO THE REGULATORY PROCESS AND ADDRESSING UNMET MEDICAL NEEDS**

Conceptually, I am looking forward to new and improved ways to take into account the patient perspective as displayed in the title above. However, I have some pause for concern and I want to make sure that the decision makers for continuing to advocate for this important bill do not overlook the following which to me is buried in the bill **and does not put patients first**.

For your convenience I have included a screen shot of verbiage found on page 172 which I believe is under section 2085. Expanding Availability of Medicare Data.

Specifically, it reads as illustrated below. I have underlined the verbiage indicating that implies that information on patients may contain information that individually identifies a patient of such a provider or supplier.

**In the 25+ years of doing clinical trials research I have never come across the need in any way for any perceived reason, scientific or logistic, to disclose clinical data that individually identifies a patient of a provider or a supplier. Respectfully, this does not put patients first by incorporating their perspectives. I feel that if the verbiage is not removed then it needs to be vetted honestly and not buried in the section below.**

1 or (2) shall not contain information that  
2 individually identifies a patient.

3 (B) INFORMATION ON PATIENTS OF  
4 PROVIDER OF SERVICES OR SUPPLIER.—  
5 extent consistent with applicable inform  
6 privacy, security, and disclosure laws, an  
7 ysis that is or data that are provided or s  
8 a provider of services or supplier under  
9 graph (1) or (2) may contain informatio  
10 individually identifies a patient of such pr  
11 or supplier, including with respect to item  
12 services furnished to the patient by othe  
13 viders of services or suppliers.

As a follow-up to the verbiage describe above, other verbiage I find particularly noteworthy can be found within sections 2221 and sections 2241. It is section 13442 which is not in the table of contents:

**“SEC. 13442.  
TREATING DISCLOSURES OF PROTECTED  
HEALTH INFORMATION FOR RESEARCH SIMILARLY TO DISCLOSURES OF SUCH  
INFORMATION FOR PUBLIC HEALTH PURPOSES.**

Specifically,

- Beginning on page 209 there are issues that pertain to patient privacy that I believe need to be given the proper visibility and examination by the Congress.
- As an aside, I am also an expert on adaptive clinical trials and Bayesian methodology. Truly as a biostatistical consultant this will be a gold mine for me because of all the new data that I will have access to for analysis. You should know that It is not in my interest to point the following out because it could make data that I need to analyze to inform projections, etc more difficult to obtain. **However, the madness needs to stop! The inherent dignity of being able to preserve our privacy when it comes to our medical conditions must be protected!!**
- I am wanting to reach out to you because from a societal perspective, I believe increasingly our ownership and sense of privacy is being destroyed and that the sections I would like you to pay attention to are at the very essence of why the barrier of trust continues to widen between government and the private citizen.
- As I read the bill, those of us who don't understand who will be given permission to see the most intimate data pertaining to our health history will eventually come out in a news blurb only later if we don't bring up the issue front and center during these congressional discussions and let the public know what those discussions entail.

I want you to know that I will continue to follow the evolution of this bill and the progress being made. You should also know that I will be attempting to influence what occurs by writing others and participate in public forums as applicable. I have already written my congressional representative on this topic, Congresswoman Clark from the 5<sup>th</sup> congressional district of Ma.

There are many vested stakeholders that will be coaxing this along. The point is if we don't pay attention to this, the law will be decided by people who have no earthly idea what is going on (meaning you!!!! And please, I mean to say this respectfully in order to get your attention!) pushed by interests that are not in the interests of a free society when it comes to divulging identification of individuals for whom clinical data are being used to determine effectiveness outcomes of medical treatments.

I look forward to your continued efforts to pass a bill that truly will put patients first in the 21<sup>st</sup> century regulatory process when coming up with best practices to quickly expedite new treatments for treating unmet medical needs. Going forward, I urge you to remove any allowance of being able to individually identify patients providing clinical data that will benefit us all.

I look forward to your thoughts on my note: especially, if I got it wrong!

Best Regards,

John

