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]

[redacted]

Dear House Energy and Commerce Committee,

As I understand, The House Energy and Commerce Committee has launched the 21st 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]

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 prolong 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 never 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 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]

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 Polycistic 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: 21st 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

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: 21st 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. Friedriech'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 Friedriech'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 delaying 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 libral 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 is 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]

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 fiance 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 fiance with all my heart, and would love her no less due to any hardship. Her worries only inspires 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]

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 21st 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

The House Energy and Commerce Committee

Delighted that you have launched the 21st Century Cures Initiative . Polycystic Kidney Disease (PKD) is a common disorder that leads to kidney failure. It is a heart-breaking disease because it is inherited from a parent, a gift no parent would want to bequest to a precious child. Scientists have made giant strides towards developing effective treatments awaiting testing in patients with PKD -- however, clinical trials are frightfully expensive and funds are simply not available to do the confirmatory studies that must be done before recommending a new treatment to patients.

I was co-founder of the PKD Foundation in 1982 and have watched as progress has grown to the point of clinical testing of candidate drugs. But we are stymied by lack of support from the Federal government and from industry. That is why your advocacy is so highly welcomed.

Best wishes,
Jared [redacted]

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]

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]

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 21st 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]