

Re: 21st Century Cures on Closing the Gap to Access Treatment for PKU  
CC: Chairman Upton, E&C Committee *Subcommittee on Health*

Dear United States House of Representatives,

11 June 2014

I am writing to express my concerns about the current gap in our health care system regarding access to treatment for PKU. I am a federal employee for the Department of Veterans Affairs, and it is federal policy to inappropriately discriminate against adults with rare, inherited metabolic disorders like phenylketonuria (PKU) under the Federal Employees Health Benefits Program (FEHBP). My specific concerns surround the access and reimbursement for the specially formulated amino acid mixtures and modified proteins of natural origin (known as medical foods) that I depend upon for health and survival. The federal government fails to comply with 1995 Maryland House Bill 509 and the good and acceptable treatment practices as cited by the NIH consensus report, many medical specialty societies, and the American College of Medical Genetics and Genomics treatment guidelines that confirm the necessity of medical foods treatment for PKU - for **life**. There is a serious gap between discovery and delivery of 21<sup>st</sup> century innovations and research milestones within the nutritional management of PKU – efforts that have been federally funded by NIH and the FDA, and are commercially available but remain inaccessible. Please help close the gap and bring a voice for inclusion of PKU to the 21<sup>st</sup> Century Cures Initiative which offers opportunity to give momentum to HR 3665, the Medical Foods Equity Act as introduced to the House by Congressman Delaney of Maryland's 6<sup>th</sup> District.

We must ensure that everyone with PKU has access to the treatment they need for this rare genetic disorder and back up our resolve with action to assure access to safe, quality, affordable treatment and health care is afforded to all Americans. Last year commemorated the 50<sup>th</sup> Anniversary of newborn screening, one of the most successful preventative public health policies ever – because it saves lives. Every baby born in the United States is screened for the early identification of PKU to prevent severe disability. The treatment for PKU, which requires the daily use of medical foods that must be continued for life, is out-of-reach for most patients; especially the PKU adults. As per Genetics in Medicine, more than 70% PKU adults in the US are not being treated in a metabolic clinic. This is 100% preventable and unjustifiable, and results in unnecessary suffering and disease progression.

Provision of coverage for medical foods for the treatment of PKU (which is already afforded to children under the FEHBP) is medically supported, cost-effective, and promotes a safe and just culture. I am writing to ask you to advocate on my behalf, and for the PKU community, to address these concerns in the comprehensive look at 21<sup>st</sup> century innovations and help close the gap within the 3-Ds: Discovery, Development and Delivery. Please also draw your attention to the inappropriate and unsafe treatment of federal employees who serve and live with PKU. Passage of HR 3665 is a forwarding step towards much warranted change surrounding the medically essential category of medical food, which fails to be addressed by the Affordable Care Act. Failure to act is not an option and continues to put lives at risk and leaves

the patients medically disenfranchised. Please assist with implementation of responsible measures to change and solicit input from the agencies under HHS. They need to fulfill their mission – which is shared by all federal employees - when it comes to provision of safe, quality health care and access to treatment. The 21<sup>st</sup> Century Cures Initiative is especially pertinent to medical food, a designated orphan therapy, which saved my life and my children's lives and spared me a lifetime of institutional care, but continues to be denied.

Sincerely,

Jennifer [REDACTED]

[REDACTED]  
[REDACTED]  
[REDACTED]

1972

### Medical Food

- Medically essential
- Medically necessary
- Engineered for me



1977

NABSP Board of Directors recommended: "amino acid based foods be considered an exception under the prescription drug program, as is the case with insulin."

1978

"Lofenalac is the same product no matter under which plan one has, and the same rules and regulations should apply." PKU parent, to US Civil Service Commission

1979

Walter Sondheim, Jr., Chairman, MD Commission on Hereditary Disorders: "Although new products continue to be developed, the reclassification has left a trail of policy changes by insurance companies, such as yours, refusing coverage for a "food."

2013

A legacy of social injustice and discrimination remains - one that has spanned both generations of Congress and families touched by rare, genetic diseases like PKU as identified by newborn screening.

## A PKU Success Story....



PKU Victims	Outcomes
Child - untreated	mental retardation, autism, seizures
Child - treatment stopped as adult	brain disease, mental health problems, paresis
PKU females of child bearing age - untreated	microcephaly, developmental disability, life threatening heart malformations of the baby
child and adult - early and continuous treatment	normal IQ, normal offspring of properly treated PKU mother

### Senator Paul S. Sarbanes 1982:

"I share your strong commitment to the continuation of effective health care programs for the citizens of our nation, and want to assure you that I will continue to do all I can in the Senate in this most important area."

# Awareness Knowledge Power

### Diagnosis 1973



## Partners in PKU

10/18/1982

### HONORABLE PAUL S. SARBANES:

"A consistent and vigorous supporter in Congress of measures designed to ensure that adequate and affordable health care is available to all Americans."

I would like to extend a thank you to the Honorable Paul S. Sarbanes for answering the call to action and assisting my mother, Mrs. Carole Weiland, to secure medically necessary treatment for me as a child, diagnosis 1973 classical phenylketonuria (PKU). This rare metabolic disorder can be considered one of the most significant breakthroughs in the history of biochemical genetics because the disease manifestations can be virtually eliminated by treatment with a restricted, lifelong, special diet.

- Jennifer Payne

**The Lofenalac generation is grown now. Can the FEDERAL government AGAIN set the LEAD for insurers to follow suit?**



**A 2013 Federal Plan:**

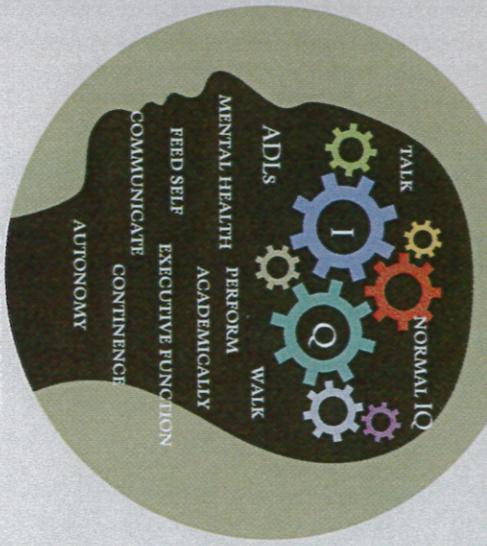
- denies access based upon age and pre-existing condition
- directly contradicts treatment guidelines as cited in the NIH Consensus report
- fails to be in compliance with the acceptable standards of the medical practice as modeled by the state in which I live

**Bring the Action!**

"PREVENTING ILLNESSES BEFORE THEY BECOME SERIOUS AND MORE COSTLY TO TREAT HELPS AMERICANS OF ALL AGES STAY HEALTHIER." SECRETARY SEBELIUS, MARCH 18, 2013

<b>ECONOMIC BURDEN TO SOCIETY FOR UNTREATED PKU AND VICTIMS OF IEM</b>
LONG TERM INSTITUTIONAL CARE
INCREASED HOSPITALIZATIONS
UNEMPLOYMENT
HIGH PHARMACEUTICAL COSTS, POLYPHARMACY W/PSYCHOTROPIC MEDICATIONS FOR PKU, INCREASED RISK FOR TREATMENT OF ADRS

**Cost to an untreated PKU victim?**



"LIFE, LIBERTY, AND THE PURSUIT OF HAPPINESS." - US DECLARATION OF INDEPENDENCE JULY 4, 1776

**MYTH:**  
"NO LONGER DO AMERICANS HAVE TO CHOOSE BETWEEN PAYING FOR PREVENTIVE CARE AND GROCERIES." SECRETARY SEBELIUS, 3/18/2013

**FACT:**  
THE TOTAL RETAIL COST FOR A GIVEN 1 MONTH SUPPLY OF MEDICAL FOOD FOR AN ADULT PKU PATIENT HAS BEEN IN EXCESS OF \$2900.00.

**FACT:**  
MEDICAL FOOD IS NOT AFFORDABLE WITHOUT ADEQUATE INSURANCE COVERAGE, A SIGNIFICANT FACTOR PREVENTING ">70% OF ADULT PKU PATIENTS IN THE US FROM ACCESSING TREATMENT." GENETICS IN MEDICINE, MARCH 2013

**ACCESS. EQUITY.**  
A Federal commitment & dedication to treatment for the American PKU patient, regardless of AGE, SEX, STATE, or EMPLOYER.

More information - Jennifer Payne please visit [www.pkupioneer.com](http://www.pkupioneer.com) or email: [pkupioneer@gmail.com](mailto:pkupioneer@gmail.com)



National  
**PKU**  
Alliance

PO Box 501, Tomahawk, WI 54487  
T: 715-437-0477 | F: 715-453-7670 | [www.npkua.org](http://www.npkua.org)



# The Medical Foods Equity Act H.R. 3665

The United States has screened newborns for PKU since 1963. Although law requires that every state screen newborns, there is not adequate coverage for the required treatment. The *Medical Foods Equity Act* would ensure that federal health programs cover the medical foods required to prevent severe disabilities and death so that children and adults with PKU can become healthy and productive citizens.

## The Medical Foods Equity Act would:

- Require federal health programs, including Children's Health Insurance Program, Tricare, Medicaid, Medicare, and Federal Employee Health Benefit Plans to cover the cost of medical foods for all inborn errors of metabolism.
- This bill is based on the recommendations of the HHS Secretary's Advisory Committee on Heritable Disorders in Newborns and Children.
- The Secretary of HHS would determine the yearly coverage amounts.
- Medical Foods are not addressed by the Affordable Care Act.

## What Is Phenylketonuria (PKU)?

- PKU is a metabolic disease in which the body cannot process a part of protein called phenylalanine. <sup>(1)</sup>
- If left untreated, PKU can lead to severe progressive intellectual disabilities. <sup>(1)</sup>
- In order to remain healthy, individuals with PKU are limited to a highly restrictive low-protein diet and must consume a liquid medical formula every day for life. <sup>(2)</sup>
- The specially manufactured low-protein foods and medical formula are extremely expensive. The average family cannot afford them without assistance. The medically necessary formula and foods are often not paid for by the state or insurance companies. <sup>(3 & 4)</sup>
- Infants of mothers with untreated PKU exhibit intellectual disabilities, heart defects, and growth retardation. <sup>(5)</sup>

## The Impact of PKU

- An estimated 15,000 in the United States have PKU, while 5,000 have another type of inborn error of metabolism.
- 5,000 adults with PKU are currently off the recommended treatment. <sup>(6)</sup>
- Studies show that individuals who discontinue diet before age 10 will experience an average 12 point drop in their IQ by adulthood.
- Children and adults who are off-diet also experience many other medical issues, including depression, impulse control disorder, phobias, epilepsy, tremors, and pareses. <sup>(7 & 8)</sup>
- The average annual cost of medically necessary formula and food for PKU is approximately \$7,000 per year. <sup>(9)</sup>
- Care for an untreated PKU patient in residential treatment is at least \$60,000 per year (or approx. \$200,000/year for an inpatient mental health facility). <sup>(10)</sup>
- Treating PKU is the right thing to do and represents a substantial cost savings.

1 [http://www.knowledgene.com/public/glossary\\_content.php3](http://www.knowledgene.com/public/glossary_content.php3)

2 <http://www.pku.com/AboutPKU/TheScienceOfPKU.aspx>

3 <http://www.mayoclinic.com/health/phenylketonuria/DS00514>

4 <http://www.matt denn.com/category/news/>

5 [http://depts.washington.edu/pku/pro\\_info/mgmtGuide.html](http://depts.washington.edu/pku/pro_info/mgmtGuide.html)

6 BioMarin

7 Koch et al. "Phenylketonuria in adulthood: A collaborative study." *Journal of Inherited Metabolic Diseases* 25 (2002): 338.

8 <http://www.pku news.org/adults/livesin.htm>

9 *Top Clin Nutr* 24 (4):289-306, 2009

10 California Study, Richard Koch, MD

**The Medical Foods Equity Act will save and improve lives!**

Dear Chairman Upton,

Please find attached a position statement from Peter Saltonstall, President and CEO of NORD, in support of HR 3665, The Medical Foods Equity Act. This historic piece of legislation offers opportunity for the federal government to take a forwarding step in bridging the gap between discovery and delivery of 21st century innovations within the category of medical food, for which I depend upon for health and survival as a patient living with phenylketonuria (PKU). Please be aware it is federal policy to discriminate against adults under the FEHBP, which limits coverage to children and puts my health and safety in jeopardy, not to mention the failure of the federal government to comply with the good and acceptable treatment practices exigent in the state of [REDACTED] where I live and work.

Sincerely,

Jennifer [REDACTED]  
[REDACTED]



April 30, 2014

The Honorable John Delaney  
1632 Longworth House Office Building  
Washington, D.C. 20515

Dear Mr. Delaney

On behalf of the 30 million men, women, and children affected by one of the 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks you for your continuing support of the rare disease community and your leadership regarding access to life-saving medical foods for inborn errors of metabolism and other rare conditions. We are writing to you today to emphasize the importance of accessing medical foods within these patient communities, and to thank you for sponsoring the Medical Foods Equity Act.

Each year, approximately 2,550 children in our country are diagnosed with inborn errors of metabolism, requiring them to access life-saving treatments such as medical foods, foods to be modified as low protein, supplements and amino acids. These costly medically necessary foods and supplements are often not covered by insurance or public assistance, causing irreparable mental and physical damage. Families continue to struggle with the tremendous financial burden associated with these medically necessary foods.

Insurance coverage of medical foods is vital to children in order to access medically necessary foods and supplements; however, there is tremendous inequity in coverage amongst the states. Although there are currently 35 states that have legislation in this regard, the legislation is subject to interpretation and more often than not, families have to pay out-of-pocket to ensure their child's well-being and survival. As there is no current cure for these inborn errors of metabolism, these treatments are necessary during the entire lifespan of the individual.

While Congress may not be able to assure access to medical foods in private plans, as that is under state jurisdiction, the Medical Foods Equity Act assures access to medical foods under Federal Health Programs, specifically Medicare, Medicaid, CHIP, TRICARE, and the FEHBP. Congress can demonstrate its commitment to children with disorders of inborn errors of metabolism by passing this important legislation.

This legislation has NORD's support and we look forward to working with you to ensure passage of this important legislation.

Sincerely,



Peter L. Saltonstall  
President and CEO

Dear Chairman Upton,

Please find attached a position statement from Congressman Sarbanes, in support of medical foods equity and note the date, September 10, 2012. This long awaited historic piece of legislation offers opportunity for the federal government to take a forwarding step in bridging the gap between discovery and delivery of 21st century innovations within the category of medical food, for which I depend upon for health and survival as a patient living with phenylketonuria (PKU). Please be aware it is federal policy to discriminate against adults under the FEHBP, which limits coverage to children and puts my health and safety in jeopardy, not to mention the failure of the federal government to comply with the good and acceptable treatment practices exigent in the state of [REDACTED] where I live and work.

Sincerely,

Jennifer [REDACTED]  
[REDACTED]

JOHN P. SARBANES  
3RD DISTRICT, MARYLAND

COMMITTEE ON  
ENERGY AND COMMERCE

Congress of the United States  
House of Representatives  
Washington, DC 20515-2003

www.sarbanes.house.gov

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44 CALVERT STREET  
SUITE 349  
ANNAPOLIS, MD 21401  
(410) 295-1679  
FAX: (410) 295-1682

September 10, 2012

Ms. Jennifer [REDACTED]  
[REDACTED]  
[REDACTED]

Dear Ms. [REDACTED]:

Thank you for contacting me to share your concerns regarding the costs and insurance coverage of medical foods. I appreciate having the benefit of your views and I welcome the opportunity to respond.

You'll be pleased to know that I am a co-sponsor of H.R. 1311, the Medical Foods Equity Act of 2011, which would provide for the coverage of medically necessary food under Federal health programs and private health insurance. It would apply to food which is formulated to be consumed or administered under the supervision of a qualified medical provider and which is intended for the dietary management of a disease or condition with distinctive nutritional requirements. H.R. 1311 was referred to the House Committees on Energy and Commerce, Ways and Means, Education and Labor, and Armed Services. As I am fortunate to serve on the Committee on Energy and Commerce, you may be sure that I will keep your views in mind should this legislation be considered by the committee or the full House of Representatives.

Again, I appreciate hearing from you. Please do not hesitate to contact me if I may be of assistance in the future.

Sincerely,



John P. Sarbanes  
Member of Congress

JPS/ch

Dear Chairman Upton,

11 June 2014

We are pleased to present you with this petition affirming this statement:

**"Please close the gap between PKU and the readily available treatment known as the category of medical food, a designated orphan therapy. Patients deserve access to the latest innovations in the nutritional management of PKU, which stem from NIH research but continue to be denied and are often not reimbursed. "**

Background:

In the United States, over 70% of PKU adults are not being treated in a metabolic clinic. Among the untreated adults, there is an increase in mental illness, psychological disorders, neurological deterioration (epilepsy, tremor, paresis), and children born to pregnant women with untreated PKU have severe birth defects and mental retardation. This is 100% preventable and the barriers to access care are unjustifiable. Medical food saved my life and my children's lives and coverage is denied by my employer, the federal government because of my age. This discriminatory policy is not prohibited by the Affordable Care Act and poses a serious threat to the health and safety of the PKU victim. It is essential to include PKU in this initiative in keeping with good and acceptable treatment practices - "diet for life." Please help.

[http://petitions.moveon.org/sign/chairman-upton-include?source=c.em.cp&r\\_by=10572179](http://petitions.moveon.org/sign/chairman-upton-include?source=c.em.cp&r_by=10572179)

Sincerely,

Jennifer [REDACTED]

[REDACTED]  
[REDACTED]

[REDACTED]  
[REDACTED]

Dear The United States House of Representatives,

We are pleased to present you with this petition affirming this statement:

**"Please close the gap between PKU and the readily available treatment known as the category of medical food, a designated orphan therapy. Patients deserve access to the latest innovations in the nutritional management of PKU, which stem from NIH research but continue to be denied and are often not reimbursed. "**

Attached is a list of individuals who have added their names to this petition, as well as additional comments written by the petition signers themselves.

Sincerely,  
Jennifer [REDACTED]

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My son-in-law has PKU. The whole family suffers from the effects if environmental changes necessitate that his doctor adjust his PKU diet. More research could really help. The special drink that keeps him alive without brain damage has begun to severely irritate his stomach, but he has no known viable alternative.

Deanna [REDACTED]  
[REDACTED]

Jun 9, 2014

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Please help save life and enrich or countries greatest resource.

Jamie [REDACTED]  
[REDACTED]

Jun 7, 2014

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G. Thomas [REDACTED]  
[REDACTED]

Jun 7, 2014

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andrew [REDACTED]  
[REDACTED]

Jun 7, 2014

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Cindy [REDACTED]  
[REDACTED]

Jun 5, 2014

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Please pass this!!!

Sarah [REDACTED]  
[REDACTED]

Jun 5, 2014

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Danny [REDACTED]  
[REDACTED]

Jun 4, 2014

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x

Jay [REDACTED]  
[REDACTED]

Jun 4, 2014

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Tobin [REDACTED]  
[REDACTED]

Jun 4, 2014

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Krystal [REDACTED]  
[REDACTED]

Jun 3, 2014

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Monica [REDACTED]  
[REDACTED]

Jun 3, 2014

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Dave [REDACTED]  
[REDACTED]

Jun 3, 2014

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Megan [REDACTED]  
[REDACTED]

Jun 3, 2014

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I am an adult with PKU, and not on diet for this exact reason. Please consider making the treatment more excessible, affordable and available.

Jennifer [REDACTED]  
[REDACTED]

Jun 3, 2014

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Mandi [REDACTED]  
[REDACTED]

Jun 3, 2014

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Joanna [REDACTED]  
[REDACTED]

Jun 3, 2014

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Include HCU and other medical food patients.

Martha [REDACTED]  
[REDACTED]

Jun 3, 2014

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This bill has been needed for many years. We tried to get a similar bill passed in Illinois, but Illinois being what it is, we were unsuccessful.

James [REDACTED]  
[REDACTED]

Jun 3, 2014

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Erin [REDACTED]  
[REDACTED]

Jun 3, 2014

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Kelly [REDACTED]  
[REDACTED]

Jun 3, 2014

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Pamela

[REDACTED]  
Jun 3, 2014

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Ben

[REDACTED]  
Jun 3, 2014

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Lauren

[REDACTED]  
Jun 2, 2014

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Ann

[REDACTED]  
Jun 2, 2014

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Ama

[REDACTED]  
Jun 2, 2014

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Gregory

[REDACTED]  
Jun 2, 2014

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Please help people with PKU,(including myself), find ways to improve their lives and find a cure!!

Kelly

[REDACTED]  
Jun 2, 2014

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marianna

[REDACTED]  
Jun 2, 2014

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cara

[REDACTED]  
Jun 2, 2014

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Allison

[REDACTED]  
Jun 2, 2014

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Tina

[REDACTED]  
Jun 2, 2014

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Heather

[REDACTED]

Jun 2, 2014

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Michelle [REDACTED]  
[REDACTED]

Jun 1, 2014

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Kirsten [REDACTED]  
[REDACTED]

Jun 1, 2014

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sara [REDACTED]  
[REDACTED]

Jun 1, 2014

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Heather [REDACTED]  
[REDACTED]

Jun 1, 2014

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Jeron [REDACTED]  
[REDACTED]

May 31, 2014

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Jennifer [REDACTED]  
[REDACTED]

May 29, 2014

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Suzanne [REDACTED]  
[REDACTED]

May 29, 2014

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Melanie [REDACTED]  
[REDACTED]

May 29, 2014

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Shinobu [REDACTED]  
[REDACTED]

May 28, 2014

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Christina [REDACTED]  
[REDACTED]

May 28, 2014

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jennifer [REDACTED]  
[REDACTED]

May 28, 2014

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Bour [REDACTED]  
[REDACTED]

May 28, 2014

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Michelle [REDACTED]  
[REDACTED]  
May 28, 2014

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Tammy [REDACTED]  
[REDACTED]  
May 28, 2014

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Jenny [REDACTED]  
[REDACTED]  
May 28, 2014

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Chelsea [REDACTED]  
[REDACTED]  
May 27, 2014

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Elijah [REDACTED]  
[REDACTED]  
May 27, 2014

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chelsea [REDACTED]  
[REDACTED]  
May 27, 2014

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Jason [REDACTED]  
[REDACTED]  
May 27, 2014

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Mercedes [REDACTED]  
[REDACTED]  
May 27, 2014

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Food is their only medicine

Julie [REDACTED]  
[REDACTED]  
May 26, 2014

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This is essential! There is no other way to protect these innocent little ones. As a society, we have the responsibility to give every child the best start possible.

Denice [REDACTED]  
[REDACTED]  
May 26, 2014

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Brett [REDACTED]  
[REDACTED]  
May 26, 2014

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My friends little brother has pku and I have seen the struggle they have gone through for his dietary needs

laura [REDACTED]

May 26, 2014

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MaryJo [REDACTED]

May 25, 2014

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Heidi [REDACTED]

May 25, 2014

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Very important to have to treatment available, even mild untreated PKU have ramifications and causes long term problems that people dont understand

Alison [REDACTED]

May 25, 2014

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Tiffany [REDACTED]

May 25, 2014

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Vicki [REDACTED]

May 25, 2014

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My brother has PKU and he is now taking on the burden of paying for all of his medically necessary foods and drugs because he is an adult and insurance doesn't cover them. Other people that are medically in need or disabled get things covered so now it's time to include PKU adults.

Karen [REDACTED]

May 25, 2014

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kate [REDACTED]

May 25, 2014

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I have PKU, and I know the struggle of not being able to pay for your medabological diet.

Chelsie [REDACTED]

May 25, 2014

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Olympia

[REDACTED]  
May 25, 2014

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John

[REDACTED]  
May 25, 2014

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My son was covered by our insurance. He is very intelligent. That intelligence is threatened now.

Laurette

[REDACTED]  
May 25, 2014

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catherine

[REDACTED]  
May 25, 2014

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My little brother has PKU. My parents had 5 children and worked multiple jobs to pay for his medically necessary diet, which insurance did not cover. Now he is an adult working at McDonalds and going to school. He can't afford his food on his salary. Please help.

Beth

[REDACTED]  
May 24, 2014

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Joseph

[REDACTED]  
May 24, 2014

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Christeen

[REDACTED]  
May 24, 2014

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Heather

[REDACTED]  
May 24, 2014

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Lark

[REDACTED]  
May 24, 2014

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Jerome

[REDACTED]  
May 24, 2014

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Lisa

[REDACTED]

May 23, 2014

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kaleigh [REDACTED]  
[REDACTED]

May 23, 2014

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My son has PKU and we really did this Thank you!

Bonnie [REDACTED]  
[REDACTED]

May 23, 2014

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It is not just to feel better, it is to survive. It should be essential, not an option.

Elizabeth [REDACTED]  
[REDACTED]

May 23, 2014

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Jami [REDACTED]  
[REDACTED]

May 23, 2014

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Ted [REDACTED]  
[REDACTED]

May 23, 2014

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Virginia [REDACTED]  
[REDACTED]

May 23, 2014

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Kristen [REDACTED]  
[REDACTED]

May 23, 2014

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Leah [REDACTED]  
[REDACTED]

May 23, 2014

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Penny [REDACTED]  
[REDACTED]

May 23, 2014

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Tamera [REDACTED]  
[REDACTED]

May 23, 2014

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P. v. [REDACTED]  
[REDACTED]

May 23, 2014

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Kathleen [REDACTED]

May 23, 2014

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Jacqueline [REDACTED]

May 23, 2014

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Amy [REDACTED]

May 22, 2014

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Janice [REDACTED]

May 22, 2014

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darla [REDACTED]

May 22, 2014

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Timothy [REDACTED]

May 22, 2014

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Misty [REDACTED]

May 22, 2014

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Frank [REDACTED]

May 22, 2014

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Connie [REDACTED]

May 22, 2014

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Holly [REDACTED]

May 22, 2014

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Amanda [REDACTED]

May 22, 2014

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David [REDACTED]

May 22, 2014

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maureen [REDACTED]  
May 22, 2014

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Rubina [REDACTED]  
May 22, 2014

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Margvelashvili [REDACTED]  
May 22, 2014

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Tamsen [REDACTED]  
May 22, 2014

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Stephanie [REDACTED]  
May 22, 2014

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Carrie [REDACTED]  
May 22, 2014

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Esther [REDACTED]  
May 22, 2014

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Jillian Doey [REDACTED]  
May 22, 2014

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NICOLE [REDACTED]  
May 22, 2014

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For my friend Marti and her family

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Jodi [REDACTED]  
May 22, 2014

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Kristen [REDACTED]  
May 22, 2014

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For my nephew.

Jennifer [REDACTED]  
[REDACTED]

May 22, 2014

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Donna [REDACTED]  
[REDACTED]

May 22, 2014

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Becca [REDACTED]  
[REDACTED]

May 22, 2014

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Danielle [REDACTED]  
[REDACTED]

May 22, 2014

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Marla [REDACTED]  
[REDACTED]

May 22, 2014

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James [REDACTED]  
[REDACTED]

May 22, 2014

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Sue [REDACTED]  
[REDACTED]

May 22, 2014

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Pat [REDACTED]  
[REDACTED]

May 22, 2014

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Lali [REDACTED]  
[REDACTED]

May 22, 2014

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Albert [REDACTED]  
[REDACTED]

May 22, 2014

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Jenelle  
[REDACTED]

May 21, 2014

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Beth [REDACTED]  
[REDACTED]

May 21, 2014

---

Please include PKU! I have a son with it and it is so extremely important for him to be on treatment and diet and it would be wonderful if a cure could be found.

Amy [REDACTED]  
[REDACTED]

May 21, 2014

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Mary [REDACTED]  
[REDACTED]

May 21, 2014

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Derrick  
[REDACTED]

May 21, 2014

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Daniel  
[REDACTED]

May 21, 2014

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Dana [REDACTED]  
[REDACTED]

May 21, 2014

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Sharon [REDACTED]  
[REDACTED]

May 21, 2014

---

Let's make this happen!!!!

[REDACTED]  
[REDACTED]

Kelsie  
[REDACTED]

May 21, 2014

---

My cousin's 1 year old grandson has PKU. Until I read this petition I didn't know that it can be treated with nutrition. It makes no sense to deny this to people suffering from PKU including my cousin's little grandson.

Arthur [REDACTED]  
[REDACTED]

May 21, 2014

---

kay  
[REDACTED]

May 21, 2014

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Laurn [REDACTED]  
[REDACTED]

May 21, 2014

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Marti [REDACTED]  
[REDACTED]

May 21, 2014

---

My grandson has PKU and we need your support. Please support all measures that give access to research, medications and the free foods for this amazing group of people in the PKU community of rare metabolic disorders.

Margie [REDACTED]  
[REDACTED]

May 21, 2014

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Thank you. This must happen

Candace [REDACTED]  
[REDACTED]

May 21, 2014

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thomas [REDACTED]  
[REDACTED]

May 21, 2014

---

Suzy [REDACTED]  
[REDACTED]

May 21, 2014

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Joyce [REDACTED]  
[REDACTED]

May 21, 2014

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rhonda [REDACTED]  
[REDACTED]

May 21, 2014

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Derrick [REDACTED]  
[REDACTED]

May 21, 2014

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Kasey [REDACTED]  
[REDACTED]

May 21, 2014

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Tim [REDACTED]  
[REDACTED]

May 21, 2014

---

My daughter [REDACTED] has PKU please help so I can keep her healthy. Thank you.

Jacqueline [REDACTED]

May 21, 2014

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Courtney [REDACTED]

May 21, 2014

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Breanna [REDACTED]

May 21, 2014

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For Abby [REDACTED]

Elizabeth [REDACTED]

May 21, 2014

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Tim [REDACTED]

May 21, 2014

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Staci [REDACTED]

May 21, 2014

---

Just do it!!!

Kevin [REDACTED]

May 21, 2014

---

Kristin [REDACTED]

May 21, 2014

---

Tiffany [REDACTED]

May 21, 2014

---

My son has PKU

Anne [REDACTED]

May 21, 2014

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John [REDACTED]  
[REDACTED]  
May 21, 2014

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Melody [REDACTED]  
[REDACTED]  
May 21, 2014

---

Stacie [REDACTED]  
[REDACTED]  
May 21, 2014

---

Please support insurance coverage for medical food.

---

Peter and Anna [REDACTED]  
[REDACTED]  
May 21, 2014

---

Jeannine [REDACTED]  
[REDACTED]  
May 21, 2014

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Janet [REDACTED]  
[REDACTED]  
May 21, 2014

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eugene [REDACTED]  
[REDACTED]  
May 21, 2014

---

This Is Where The Real Medical Focus Should Be. This Is Where Disciplined, Normal, Hard Working, Contribute To Society Type Families That Need Assistance.

---

Michael [REDACTED]  
[REDACTED]  
May 21, 2014

---

Beverly [REDACTED]

beverly [REDACTED]  
[REDACTED]  
May 21, 2014

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vilka [REDACTED]  
[REDACTED]  
May 21, 2014

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Lane [REDACTED]  
[REDACTED]

May 21, 2014

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Alisia [REDACTED]  
[REDACTED]

May 21, 2014

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Kerry [REDACTED]  
[REDACTED]

May 21, 2014

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sandra [REDACTED]  
[REDACTED]

May 21, 2014

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Dee [REDACTED]  
[REDACTED]

May 21, 2014

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Stuart [REDACTED]  
[REDACTED]

May 21, 2014

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carl [REDACTED]  
[REDACTED]

May 21, 2014

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Please close the gap

Evelyn [REDACTED]  
[REDACTED]

May 21, 2014

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maureen [REDACTED]  
[REDACTED]

May 21, 2014

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Rachel [REDACTED]  
[REDACTED]

May 21, 2014

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Pam [REDACTED]  
[REDACTED]

May 21, 2014

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SCOTT [REDACTED]  
[REDACTED]

May 21, 2014

---

hania

[REDACTED]

May 21, 2014

---

Lauren

[REDACTED]

May 21, 2014

---

I do this for those who can't, fight strong and win!!!!

tabitha

[REDACTED]

May 21, 2014

---

Tessa

[REDACTED]

May 21, 2014

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Jimmy

[REDACTED]

May 21, 2014

---

Dana G.

[REDACTED]

May 21, 2014

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Karissa

[REDACTED]

May 21, 2014

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Samantha

[REDACTED]

May 21, 2014

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Cathy

[REDACTED]

May 21, 2014

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Suehelie

[REDACTED]

May 21, 2014

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Jennifer

[REDACTED]

May 21, 2014

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Kelly [REDACTED]  
[REDACTED]  
May 21, 2014

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Greg [REDACTED]  
[REDACTED]  
May 21, 2014

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Amanda [REDACTED]  
[REDACTED]  
May 21, 2014

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Kari [REDACTED]  
[REDACTED]  
May 21, 2014

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alyssa [REDACTED]  
[REDACTED]  
May 21, 2014

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Korin [REDACTED]  
[REDACTED]  
May 21, 2014

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caroln [REDACTED]  
[REDACTED]  
May 21, 2014

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Becky [REDACTED]  
[REDACTED]  
May 21, 2014

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Jennifer [REDACTED]  
[REDACTED]  
May 20, 2014

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Karen [REDACTED]  
[REDACTED]  
May 20, 2014

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Linda [REDACTED]  
[REDACTED]  
May 20, 2014

---

Love my chunky monkey

April [REDACTED]  
[REDACTED]

May 20, 2014

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tonia [REDACTED]  
[REDACTED]

May 20, 2014

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Debbie [REDACTED]  
[REDACTED]

May 20, 2014

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Mary [REDACTED]  
[REDACTED]

May 20, 2014

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Renee [REDACTED]  
[REDACTED]

May 20, 2014

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Carole [REDACTED]  
[REDACTED]

May 20, 2014

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Thomas [REDACTED]  
[REDACTED]

May 20, 2014

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Ashley [REDACTED]  
[REDACTED]

May 20, 2014

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Shayla [REDACTED]  
[REDACTED]

May 20, 2014

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Eric [REDACTED]  
[REDACTED]

May 20, 2014

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Maxine [REDACTED]  
[REDACTED]

May 20, 2014

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Treena [REDACTED]  
[REDACTED]

May 20, 2014

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Eve [REDACTED]  
[REDACTED]

May 20, 2014

---

This is a worthy use of research to help citizens deal with a very real disease that affects both the individuals and their families. Thank you for considering a positive action.

nancy [REDACTED]  
[REDACTED]

May 20, 2014

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Kristie  
[REDACTED]

May 20, 2014

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Krista  
[REDACTED]

May 20, 2014

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Danielle [REDACTED]  
[REDACTED]

May 20, 2014

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Melissa [REDACTED]  
[REDACTED]

May 20, 2014

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William [REDACTED]  
[REDACTED]

May 20, 2014

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Seth  
[REDACTED]

May 20, 2014

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Stephanie [REDACTED]  
[REDACTED]

May 20, 2014

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Steven [REDACTED]  
[REDACTED]

May 20, 2014

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Diane [REDACTED]  
[REDACTED]

May 20, 2014

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Nicole [REDACTED]  
[REDACTED]

May 20, 2014

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Amanda [REDACTED]  
[REDACTED]  
May 20, 2014

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Rebecca [REDACTED]  
[REDACTED]  
May 20, 2014

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Ted [REDACTED]  
[REDACTED]  
May 20, 2014

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Sarah [REDACTED]  
[REDACTED]  
May 20, 2014

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Abbey [REDACTED]  
[REDACTED]  
May 20, 2014

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Melissa [REDACTED]  
[REDACTED]  
May 20, 2014

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Regina [REDACTED]  
[REDACTED]  
May 20, 2014

---

Do it!!!

---

Frances [REDACTED]  
[REDACTED]  
May 20, 2014

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Maria [REDACTED]  
[REDACTED]  
May 20, 2014

---

Ann [REDACTED]  
[REDACTED]  
May 20, 2014

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Raffaella [REDACTED]  
[REDACTED]  
May 20, 2014

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Kathleen [REDACTED]  
[REDACTED]

May 20, 2014

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Sue [REDACTED]  
[REDACTED]  
May 20, 2014

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Misty [REDACTED]  
[REDACTED]  
May 20, 2014

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Connie [REDACTED]  
[REDACTED]  
May 20, 2014

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Dan [REDACTED]  
[REDACTED]  
May 20, 2014

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Amy [REDACTED]  
[REDACTED]  
May 20, 2014

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Molly [REDACTED]  
[REDACTED]  
May 20, 2014

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Crystal [REDACTED]  
[REDACTED]  
May 20, 2014

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Justyna [REDACTED]  
[REDACTED]  
May 20, 2014

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gudrun [REDACTED]  
[REDACTED]  
May 20, 2014

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Michael [REDACTED]  
[REDACTED]  
May 20, 2014

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Patty [REDACTED]  
[REDACTED]  
May 20, 2014

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john [REDACTED]  
[REDACTED]  
May 20, 2014

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Carla [REDACTED]  
[REDACTED]

May 20, 2014

---

Please make sure that medical food is available to every child and adult that needs it. The cost is far less than long term care and supports.

Mary [REDACTED]  
[REDACTED]

May 20, 2014

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Nancy [REDACTED]  
[REDACTED]

May 20, 2014

---

sally [REDACTED]  
[REDACTED]

May 20, 2014

---

Paul [REDACTED]  
[REDACTED]

May 20, 2014

---

Crescent [REDACTED]  
[REDACTED]

May 20, 2014

---

This is for my son's future and the other PKU adults!

Laura [REDACTED]  
[REDACTED]

May 20, 2014

---

Kathleen [REDACTED]  
[REDACTED]

May 20, 2014

---

Lauren [REDACTED]  
[REDACTED]

May 20, 2014

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Anne [REDACTED]  
[REDACTED]

May 20, 2014

---

joan [REDACTED]  
[REDACTED]

May 20, 2014

---

David [REDACTED]  
[REDACTED]  
May 20, 2014

---

P J [REDACTED]  
[REDACTED]  
May 20, 2014

---

Please give PKU a voice!

---

Emily [REDACTED]  
[REDACTED]  
May 20, 2014

---

Natalie [REDACTED]  
[REDACTED]  
May 20, 2014

---

This is a life long struggle and those who suffer deserve a better life, they deserve a CURE.

---

shannon [REDACTED]  
[REDACTED]  
May 20, 2014

---

theresa [REDACTED]  
[REDACTED]  
May 20, 2014

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kelly [REDACTED]  
[REDACTED]  
May 20, 2014

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linda [REDACTED]  
[REDACTED]  
May 20, 2014

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Sherry [REDACTED]  
[REDACTED]  
May 20, 2014

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Suzanne [REDACTED]  
[REDACTED]  
May 20, 2014

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Richard [REDACTED]  
[REDACTED]  
May 20, 2014

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Rhonda [REDACTED]  
[REDACTED]  
May 20, 2014

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Bobby [REDACTED]  
[REDACTED]  
May 20, 2014

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Michael [REDACTED]  
[REDACTED]  
May 20, 2014

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Suzanne [REDACTED]  
[REDACTED]  
May 20, 2014

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Eileen [REDACTED]  
[REDACTED]  
May 20, 2014

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T. [REDACTED]  
[REDACTED]  
May 20, 2014

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Cami [REDACTED]  
[REDACTED]  
May 20, 2014

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in honor of my 2nd cousin, 1-year-old [REDACTED]

---

Carreen [REDACTED]  
[REDACTED]  
May 20, 2014

---

PKU needs to be addressed.

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Patricia [REDACTED]  
[REDACTED]  
May 20, 2014

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Nancy [REDACTED]  
[REDACTED]  
May 20, 2014

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Cathie [REDACTED]  
[REDACTED]  
May 20, 2014

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Melinda [REDACTED]  
[REDACTED]  
May 20, 2014

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Kathy [REDACTED]  
[REDACTED]  
May 20, 2014

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Ashley [REDACTED]  
[REDACTED]  
May 20, 2014

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Jim [REDACTED]  
[REDACTED]  
May 20, 2014

---

We need this to help us! It will make our lives a lot more bareble and make us feel that little bit normal! This would make people's lives so much easier. Please help us!

---

Nicola [REDACTED]  
[REDACTED]  
May 20, 2014

---

My five year old son has PKU and he deserves a voice.

---

Berenice [REDACTED]  
[REDACTED]  
May 20, 2014

---

Tony [REDACTED]  
[REDACTED]  
May 20, 2014

---

d [REDACTED] Canada  
[REDACTED]  
May 20, 2014

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Damon [REDACTED]  
[REDACTED]  
May 20, 2014

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Gail [REDACTED]  
[REDACTED]  
May 20, 2014

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Angela [REDACTED]  
[REDACTED]  
May 20, 2014

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Catherine [REDACTED]  
[REDACTED]

May 20, 2014

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Lisa [REDACTED]  
[REDACTED]

May 20, 2014

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olivia [REDACTED]  
[REDACTED]

May 20, 2014

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Sarah [REDACTED]  
[REDACTED]

May 20, 2014

---

No one should suffer because of being denied essential medicines and foods.

Diane [REDACTED]  
[REDACTED]

May 20, 2014

---

Mary Beth [REDACTED]  
[REDACTED]

May 20, 2014

---

John [REDACTED]  
[REDACTED]

May 20, 2014

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Lauren [REDACTED]  
[REDACTED]

May 20, 2014

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Scott [REDACTED]  
[REDACTED]

May 20, 2014

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Holly [REDACTED]  
[REDACTED]

May 20, 2014

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Colleen [REDACTED]  
[REDACTED]

May 20, 2014

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Domenighetti  
[REDACTED]

May 20, 2014

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Mary [REDACTED]  
[REDACTED]

May 20, 2014

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Katie  
[REDACTED]

May 20, 2014

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Marcia [REDACTED]  
[REDACTED]

May 20, 2014

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Jessica [REDACTED]  
[REDACTED]

May 20, 2014

---

For my bro and sis

Michael [REDACTED]  
[REDACTED]

May 20, 2014

---

Jennifer [REDACTED]  
[REDACTED]

May 20, 2014

---

chandler [REDACTED]  
[REDACTED]

May 20, 2014

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Mary [REDACTED]  
[REDACTED]

May 20, 2014

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Connie [REDACTED]  
[REDACTED]

May 20, 2014

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Nicole [REDACTED]  
[REDACTED]

May 20, 2014

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Jill [REDACTED]  
[REDACTED]

May 20, 2014

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Tracy [REDACTED]  
[REDACTED]  
May 20, 2014

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Austin [REDACTED]  
[REDACTED]  
May 20, 2014

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Nathan [REDACTED]  
[REDACTED]  
May 20, 2014

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ASOCIACIÓN [REDACTED] GALICIA  
[REDACTED] Spain  
May 20, 2014

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Thelma [REDACTED]  
[REDACTED]  
May 20, 2014

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Melissa [REDACTED]  
[REDACTED]  
May 20, 2014

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Josie [REDACTED]  
[REDACTED]  
May 20, 2014

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Laurie [REDACTED]  
[REDACTED]  
May 20, 2014

---

It's about time PKU got recognition

---

Mark [REDACTED]  
[REDACTED]  
May 20, 2014

---

kelli [REDACTED]  
[REDACTED]  
May 20, 2014

---

Bobbie [REDACTED]  
[REDACTED]  
May 20, 2014

---

Please help. Thank you

Andy [REDACTED]  
[REDACTED]  
May 20, 2014

---

Kim [REDACTED]  
[REDACTED]  
May 20, 2014

---

Tracy [REDACTED]  
[REDACTED]  
May 20, 2014

---

Tom [REDACTED]  
[REDACTED]  
May 20, 2014

---

In honor of my adult brother with PKU.

Kristina [REDACTED]  
[REDACTED]  
May 20, 2014

---

Lisa [REDACTED]  
[REDACTED]  
May 20, 2014

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Ariel [REDACTED]  
[REDACTED]  
May 20, 2014

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Donna [REDACTED]  
[REDACTED]  
May 20, 2014

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Sarah [REDACTED]  
[REDACTED]  
May 20, 2014

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Tom [REDACTED]  
[REDACTED]  
May 20, 2014

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Sandra [REDACTED]  
[REDACTED]  
May 20, 2014

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Kathy [REDACTED]  
[REDACTED]  
May 20, 2014

---

Help our PKU kids!!!

Brandee [REDACTED]  
[REDACTED]  
May 20, 2014

---

kelli  
[REDACTED]  
May 20, 2014

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Jacy  
[REDACTED]  
May 20, 2014

---

Lisa [REDACTED]  
[REDACTED]  
May 20, 2014

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Debra [REDACTED]  
[REDACTED]  
May 20, 2014

---

I have PKU and I think its just ridiculous that us Pkuers in the US can not get the help thats necessary.

Brandy [REDACTED]  
[REDACTED]  
May 20, 2014

---

SUPPORT to the USA PKU-ers! This is a condition for life and should be treated as such, including financial assistance.

Paul [REDACTED]  
[REDACTED]  
May 20, 2014

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Lindy [REDACTED]  
[REDACTED]  
May 20, 2014

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Brenda [REDACTED]  
[REDACTED]  
May 20, 2014

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Eric [REDACTED]

May 20, 2014

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Jill [REDACTED]

May 20, 2014

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Andrea [REDACTED]

May 20, 2014

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rose [REDACTED]

May 20, 2014

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Krystal [REDACTED]

May 20, 2014

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Kristi [REDACTED]

May 20, 2014

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Jason [REDACTED]

May 20, 2014

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Ginny [REDACTED]

May 20, 2014

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patricia [REDACTED]

May 20, 2014

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Carol [REDACTED]

May 20, 2014

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donald [REDACTED]

May 20, 2014

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Carrie [REDACTED]

May 20, 2014

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Amanda [REDACTED]  
[REDACTED]  
May 20, 2014

---

Sign petition

Jacqueline [REDACTED]  
[REDACTED]  
May 20, 2014

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SHERI [REDACTED]  
[REDACTED]  
May 20, 2014

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Scott [REDACTED]  
[REDACTED]  
May 20, 2014

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Anthony [REDACTED]  
[REDACTED]  
May 20, 2014

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Peggy [REDACTED]  
[REDACTED]  
May 20, 2014

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Hope [REDACTED]  
[REDACTED]  
May 20, 2014

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Stacy [REDACTED]  
[REDACTED]  
May 20, 2014

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Lindsay [REDACTED]  
[REDACTED]  
May 20, 2014

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Betty [REDACTED]  
[REDACTED]  
May 20, 2014

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MG [REDACTED]  
[REDACTED]  
May 20, 2014

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catherine  
[REDACTED]

May 20, 2014

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Barb [REDACTED]  
[REDACTED]

May 20, 2014

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Stefanie [REDACTED]  
[REDACTED]

May 20, 2014

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William [REDACTED]  
[REDACTED]

May 20, 2014

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Dale [REDACTED]  
[REDACTED]

May 20, 2014

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Kendra [REDACTED]  
[REDACTED]

May 20, 2014

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Jimmie [REDACTED]  
[REDACTED]

May 20, 2014

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Laura [REDACTED]  
[REDACTED]

May 20, 2014

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rachael [REDACTED]  
[REDACTED]

May 20, 2014

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Tracey [REDACTED]  
[REDACTED]

May 20, 2014

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diane [REDACTED]  
[REDACTED]

May 20, 2014

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kristen [REDACTED]  
[REDACTED]

May 20, 2014

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Kimberly [REDACTED]  
[REDACTED]

May 20, 2014

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Nikki [REDACTED]  
[REDACTED]  
May 20, 2014

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Fiona [REDACTED]  
[REDACTED]  
May 20, 2014

---

Find a cure!

DeAnn [REDACTED]  
[REDACTED]  
May 20, 2014

---

For Mick

Tina [REDACTED]  
[REDACTED]  
May 20, 2014

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Pasquale [REDACTED]  
[REDACTED]  
May 20, 2014

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Denise [REDACTED]  
[REDACTED]  
May 20, 2014

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Lilianne [REDACTED]  
[REDACTED]  
May 20, 2014

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Lynnette [REDACTED]  
[REDACTED]  
May 20, 2014

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Joan [REDACTED]  
[REDACTED]  
May 20, 2014

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suzanne [REDACTED]  
[REDACTED]  
May 19, 2014

---

Please do what is right!

Kylie [REDACTED]  
[REDACTED]

May 19, 2014

---

Cara

[REDACTED]

May 19, 2014

---

Ellen

[REDACTED]

May 19, 2014

---

Mr Jeffrey

[REDACTED]

May 19, 2014

---

Please help the fight for a cure!

Susan

[REDACTED]

May 19, 2014

---

Ruth

[REDACTED]

May 19, 2014

---

Dave

[REDACTED]

May 19, 2014

---

melissa

[REDACTED]

May 19, 2014

---

Deborah

[REDACTED]

May 19, 2014

---

Catherine

[REDACTED]

May 19, 2014

---

Matt

[REDACTED]

May 19, 2014

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Annemarie

[REDACTED]

May 19, 2014

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Marty [REDACTED]  
[REDACTED]  
May 19, 2014

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Kristen [REDACTED]  
[REDACTED]  
May 19, 2014

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Kimberly [REDACTED]  
[REDACTED]  
May 19, 2014

---

Cigall [REDACTED]  
[REDACTED]  
May 19, 2014

---

Medical food and formula for PKU patients should be covered 100% on all health plans! It is more cost effective than treating the patient who suffers the repercussions of not following the diet!

---

Wendy [REDACTED]  
[REDACTED]  
May 19, 2014

---

Susan [REDACTED]  
[REDACTED]  
May 19, 2014

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Carrie [REDACTED]  
[REDACTED]  
May 19, 2014

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Alissa [REDACTED]  
[REDACTED]  
May 19, 2014

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Byron [REDACTED]  
[REDACTED]  
May 19, 2014

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Julie [REDACTED]  
[REDACTED]  
May 19, 2014

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Peg [REDACTED]  
[REDACTED]  
May 19, 2014

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Elisha [REDACTED]  
[REDACTED]  
May 19, 2014

---

This a serious concern!

Johnny F [REDACTED]  
[REDACTED]  
May 19, 2014

---

PKU, be gone!

Trudy [REDACTED]  
[REDACTED]  
May 19, 2014

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Will [REDACTED]  
[REDACTED]  
May 19, 2014

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Mary [REDACTED]  
[REDACTED]  
May 19, 2014

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Iwona [REDACTED]  
[REDACTED]  
May 19, 2014

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Chris [REDACTED]  
[REDACTED]  
May 19, 2014

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I support PKU

Suzanne [REDACTED]  
[REDACTED]  
May 19, 2014

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Yvonne [REDACTED]  
[REDACTED]  
May 19, 2014

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Janet [REDACTED]  
[REDACTED]  
May 19, 2014

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Samantha [REDACTED]  
[REDACTED]

May 19, 2014

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Christie [REDACTED]  
[REDACTED]

May 19, 2014

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Josephine [REDACTED]  
[REDACTED]

May 19, 2014

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Terri [REDACTED]  
[REDACTED]

May 19, 2014

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Vicki [REDACTED]  
[REDACTED]

May 19, 2014

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JUST DO WHAT NEEDS TO BE DONE ALREADY! BE FAIR & CARING. WHAT WOULD YOU DO IF ONE OF YOUR LOVED ONES NEEDED YOUR HELP? THINK!!!!

Diane [REDACTED]  
[REDACTED]

May 19, 2014

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Jeannine [REDACTED]  
[REDACTED]

May 19, 2014

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Beth [REDACTED]  
[REDACTED]

May 19, 2014

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Kim [REDACTED]  
[REDACTED]

May 19, 2014

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Alice [REDACTED]  
[REDACTED]

May 19, 2014

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I have PKU

Derek [REDACTED]  
[REDACTED]

May 19, 2014

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My boyfriend has PKU

Lauren

[REDACTED]

May 19, 2014

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George

[REDACTED]

May 19, 2014

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Amanda

[REDACTED]

May 19, 2014

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Nancy

[REDACTED]

May 19, 2014

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Tracy

[REDACTED]

May 19, 2014

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Laura

[REDACTED]

May 19, 2014

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Aron

[REDACTED]

May 19, 2014

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Marsha

[REDACTED]

May 19, 2014

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Jen

[REDACTED]

May 19, 2014

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Carol

[REDACTED]

May 19, 2014

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Kelly Anne

[REDACTED]

May 19, 2014

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Tom

[REDACTED]

May 19, 2014

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John [REDACTED]  
[REDACTED]

May 19, 2014

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Angel [REDACTED]  
[REDACTED]

May 19, 2014

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James L [REDACTED]  
[REDACTED]

May 19, 2014

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Dana [REDACTED]  
[REDACTED]

May 19, 2014

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PLEASE.. CLOSE THE GAP .. THESE PATIENTS DESERVE ACCESS TO THE BEST AVAILABLE TREATMENT TO THEM .

judith k. [REDACTED]  
[REDACTED]

May 19, 2014

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Melissa [REDACTED]  
[REDACTED]

May 19, 2014

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steve [REDACTED]  
[REDACTED]

May 19, 2014

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This is for my 2 daughters who have PKU!

Katrina [REDACTED]  
[REDACTED]

May 19, 2014

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Amy [REDACTED]  
[REDACTED]

May 19, 2014

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Brenda [REDACTED]  
[REDACTED]

May 19, 2014

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Kathleen [REDACTED]  
[REDACTED]

May 19, 2014

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christina [REDACTED]  
[REDACTED]

May 19, 2014

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Meribeth  
[REDACTED]

May 19, 2014

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Tom [REDACTED]  
[REDACTED]

May 19, 2014

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Heidi [REDACTED]  
[REDACTED]

May 19, 2014

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Laura  
[REDACTED]

May 19, 2014

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Karen [REDACTED]  
[REDACTED]

May 19, 2014

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Rachel [REDACTED]  
[REDACTED]

May 19, 2014

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my girlfriend has PKU and the fact that her insurance company says that her formula isnt necessary makes me enraged.. It's not like these people choose to have PKU. They have had it since birth and it very much so should be covered

Alex [REDACTED]  
[REDACTED]

May 19, 2014

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Prayers for all the children with this condition

Michele [REDACTED]  
[REDACTED]

May 19, 2014

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Cherie [REDACTED]  
[REDACTED]

May 19, 2014

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Nicky

[REDACTED]  
May 19, 2014

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Please close the gap between PKU and the readily available treatment known as the category of medical food, a designated orphan therapy. Patients deserve access to the latest innovations in the nutritional management of PKU, which stem from NIH research but continue to be denied and are often not reimbursed.

Alice

[REDACTED]  
May 19, 2014

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sarah

[REDACTED]  
May 19, 2014

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Ruth

[REDACTED]  
May 19, 2014

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Hollon

[REDACTED]  
May 19, 2014

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Emylee

[REDACTED]  
May 19, 2014

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Cynthia

[REDACTED]  
May 19, 2014

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Vincent

[REDACTED]  
May 19, 2014

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Debbie

[REDACTED]  
May 19, 2014

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Please help make PKU treatable for all sufferers.

Kathleen

[REDACTED]  
May 19, 2014

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Deborah

[REDACTED]

May 19, 2014

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Zack [REDACTED]  
[REDACTED]

May 19, 2014

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Jennifer  
[REDACTED]

May 19, 2014

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Lauren [REDACTED]  
[REDACTED]

May 19, 2014

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Liz [REDACTED]  
[REDACTED]

May 19, 2014

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Celena [REDACTED]  
[REDACTED]

May 19, 2014

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Medical treatment for PKU is mandated as a life-long treatment. Yet, there is usually funding for children born with PKU, but only to a certain age. If they do not have insurance that pays for the treatment after that age, they are receiving no treatment at all. There is absolutely no sense to this at all. The dietary restrictions have been proven to have a maximum effect when continued lifelong. Stopping these restrictions, will cause all kinds of absolutely preventable consequences.

Linda [REDACTED]  
[REDACTED]

May 19, 2014

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erna [REDACTED]  
[REDACTED]

May 19, 2014

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Dolores [REDACTED]  
[REDACTED]

May 19, 2014

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Judi [REDACTED]  
[REDACTED]

May 19, 2014

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Go Allison!

Patrick [REDACTED]  
[REDACTED]

May 19, 2014

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please help these pku patients!! They deserve a quality healthy life too.

karyn [REDACTED]  
[REDACTED]  
May 19, 2014

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Kem [REDACTED]  
[REDACTED]  
May 19, 2014

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For my niece [REDACTED]

Crystal [REDACTED]  
[REDACTED]  
May 19, 2014

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Sibel [REDACTED]  
[REDACTED]  
May 19, 2014

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Julie [REDACTED]  
[REDACTED]  
May 19, 2014

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Maryann [REDACTED]  
[REDACTED]  
May 19, 2014

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Rhoda [REDACTED]  
[REDACTED]  
May 19, 2014

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Melissa [REDACTED]  
[REDACTED]  
May 19, 2014

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Christine [REDACTED]  
[REDACTED]  
May 19, 2014

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Brent [REDACTED]  
[REDACTED]  
May 19, 2014

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My sister has PKU, and she has a hard time taking all the formula stuff that she needs everyday. We need a better, more efficient way to adminster the formula at least.

Kaissac [REDACTED]  
[REDACTED]

May 19, 2014

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Nancy [REDACTED]  
[REDACTED]

May 19, 2014

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Bob [REDACTED]  
[REDACTED]

May 19, 2014

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Chairman Upton, I beg you to include PKU for medical food for this terrible metabolic disorder. What you spend now will cost you less in the future for the necessary medical care involed in treating PKU patients  
Thank you

john [REDACTED]  
[REDACTED]

May 19, 2014

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Gloria [REDACTED]  
[REDACTED]

May 19, 2014

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Wellington [REDACTED]  
[REDACTED]

May 19, 2014

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Angela [REDACTED]  
[REDACTED]

May 19, 2014

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Lauren [REDACTED]  
[REDACTED]

May 19, 2014

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Liza [REDACTED]  
[REDACTED]

May 19, 2014

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Ryan [REDACTED]  
[REDACTED]

May 19, 2014

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catherine [REDACTED]  
[REDACTED]

May 19, 2014

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bobbie  
[REDACTED]

May 19, 2014

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Kathy [REDACTED]  
[REDACTED]

May 19, 2014

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Clare [REDACTED]

Kylie [REDACTED]  
[REDACTED]

May 19, 2014

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Joyce [REDACTED]  
[REDACTED]

May 19, 2014

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Susan [REDACTED]  
[REDACTED]

May 19, 2014

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Jessica [REDACTED]  
[REDACTED]

May 19, 2014

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Cynthia  
[REDACTED]

May 19, 2014

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Brendan [REDACTED]  
[REDACTED]

May 19, 2014

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Unbelievable help these people enjoy foods that we r able to enjoy daily!

ann [REDACTED]  
[REDACTED]

May 19, 2014

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Brenna [REDACTED]  
[REDACTED]

May 19, 2014

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Stacey [REDACTED]  
[REDACTED]

May 19, 2014

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PLEASE HELP THESE CHILDREN NOW OTHERWISE IT WILL COST YOU MORE DOWN THE LINE  
IN MEDICAL CARE.

Mary [REDACTED]  
[REDACTED]

May 19, 2014

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In support of [REDACTED] 14 years old.

Bob [REDACTED]  
[REDACTED]

May 19, 2014

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Angel [REDACTED]  
[REDACTED]

May 19, 2014

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Sheilah [REDACTED]  
[REDACTED]

May 19, 2014

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Elizabeth [REDACTED]  
[REDACTED]

May 19, 2014

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Barbara [REDACTED]  
[REDACTED]

May 19, 2014

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Their special food is their lifeline just as important as insulin to a diabetic. Please pass this for all of their  
sakes!

Deborah [REDACTED]  
[REDACTED]

May 19, 2014

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Rebecca [REDACTED]  
[REDACTED]

May 19, 2014

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Mary Beth [REDACTED]  
[REDACTED]

May 19, 2014

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Meredith [REDACTED]  
[REDACTED]  
May 19, 2014

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Allison [REDACTED]  
[REDACTED]  
May 19, 2014

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Alex [REDACTED]  
[REDACTED]  
May 19, 2014

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Dawn [REDACTED]  
[REDACTED]  
May 19, 2014

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Mindy [REDACTED]  
[REDACTED]  
May 19, 2014

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Lauren [REDACTED]  
[REDACTED]  
May 19, 2014

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Please help

Thomas [REDACTED]  
[REDACTED]  
May 19, 2014

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maureen [REDACTED]  
[REDACTED]  
May 19, 2014

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Thank you!

Kathleen [REDACTED]  
[REDACTED]  
May 19, 2014

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Christina [REDACTED]  
[REDACTED]  
May 19, 2014

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Tracy [REDACTED]  
[REDACTED]  
May 19, 2014

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Karen [REDACTED]  
[REDACTED]  
May 19, 2014

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Debbie [REDACTED]  
[REDACTED]  
May 19, 2014

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Brandon [REDACTED]  
[REDACTED]  
May 19, 2014

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stephanie [REDACTED]  
[REDACTED]  
May 19, 2014

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Jenny [REDACTED]  
[REDACTED]  
May 19, 2014

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Deb [REDACTED]  
[REDACTED]  
May 19, 2014

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Melinda [REDACTED]  
[REDACTED]  
May 19, 2014

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Theresa [REDACTED]  
[REDACTED]  
May 19, 2014

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Yucera [REDACTED]  
[REDACTED]  
May 19, 2014

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Signed for my little handsome nephew and my adult cousin and all others that need this help!!

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Tammy [REDACTED]  
[REDACTED]  
May 19, 2014

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Robert [REDACTED]  
[REDACTED]  
May 19, 2014

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Matt [REDACTED]  
[REDACTED]

May 19, 2014

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Tamara [REDACTED]  
[REDACTED]

May 19, 2014

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Melanie [REDACTED]  
[REDACTED]

May 19, 2014

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Heather [REDACTED]  
[REDACTED]

May 19, 2014

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My amazingly brilliant 10 year old niece has PKU. Her brilliance is due to adherence to the Diet for Life started at 8 days old. Medical food is critical. While it is incredibly expensive, as a family we are extremely fortunate to find a way to fit medical food into the budget. Others are not as fortunate - please help.

Kirsten [REDACTED]  
[REDACTED]

May 19, 2014

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stefania [REDACTED]  
[REDACTED]

May 19, 2014

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My granddaughter has PKU and they need all the help they can get.

Kitty [REDACTED]  
[REDACTED]

May 19, 2014

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Camille [REDACTED]  
[REDACTED]

May 19, 2014

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Michael [REDACTED]  
[REDACTED]

May 19, 2014

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Brenda [REDACTED]  
[REDACTED]

May 19, 2014

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Dana [REDACTED]  
[REDACTED]

May 19, 2014

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Colleen [REDACTED]  
May 19, 2014

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christine [REDACTED]  
May 19, 2014

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Michele [REDACTED]  
May 19, 2014

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Cathy [REDACTED]  
May 19, 2014

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Tyler [REDACTED]  
May 19, 2014

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hugo [REDACTED]  
May 19, 2014

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Kayren [REDACTED]  
May 19, 2014

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Martina [REDACTED]  
May 19, 2014

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Robyn [REDACTED]  
May 19, 2014

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Sarah [REDACTED]  
May 19, 2014

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Julie [REDACTED]  
May 19, 2014

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As the grandparents of 2 young grandsons, ages 8 and 10 yrs, we kindly ask for the readily available treatment known as the category of medical food, a designated orphan therapy. Fortunately the diagnosis was found when our grandsons were born thanks to the wonders of medical research in the 70s. We humbly ask Chairman Upton to include the voices of all PKU families throughout the nation be granted access to the latest innovations in the nutritional management of PKU. Iowa families are denied respectively reimbursements of their very important daily formulas designated orphan therapy. Thank you. Lawrence & Santina [REDACTED]

Lawrence & Santina [REDACTED]  
[REDACTED]

May 19, 2014

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PLEASE help these children & adults with PKU. They're just as deserving as everyone else with metabolic & genetic conditions. The ACA was 'supposed' to cover their medical foods & formula ... but little by little they're being denied by insurance companies and employers. PLEASE, PLEASE help us!

Mamie [REDACTED]  
[REDACTED]

May 19, 2014

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Tabitha [REDACTED]  
[REDACTED]

May 19, 2014

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Colleen [REDACTED]  
[REDACTED]

May 19, 2014

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Jane [REDACTED]  
[REDACTED]

May 19, 2014

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Milton [REDACTED]  
[REDACTED]

May 19, 2014

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Megan [REDACTED]  
[REDACTED]

May 19, 2014

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Lynnette [REDACTED]  
[REDACTED]

May 19, 2014

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Monica [REDACTED]  
[REDACTED]

May 19, 2014

---

The food should be covered because it's medically necessary to sustain the health of anyone afflicted with PKU.

Sally [REDACTED]  
[REDACTED]

May 19, 2014

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Dena [REDACTED]  
[REDACTED]

May 19, 2014

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Marge [REDACTED]  
[REDACTED]

May 19, 2014

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Chelsey [REDACTED]  
[REDACTED]

May 19, 2014

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No email solitary on thank you. Please support the the voice of pay cure

Lucy [REDACTED]  
[REDACTED]

May 19, 2014

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Jean [REDACTED]  
[REDACTED]

May 19, 2014

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Joyce [REDACTED]  
[REDACTED]

May 19, 2014

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Janet [REDACTED]  
[REDACTED]

May 19, 2014

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William [REDACTED]  
[REDACTED]

May 19, 2014

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I'm a medical geneticist who used to treat kids with PKU. These foods are medically necessary and the only hope for these individuals to be healthy are reach their potential as productive human beings!

Jennifer [REDACTED]  
[REDACTED]

May 19, 2014

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Valerie [REDACTED]  
[REDACTED]  
May 19, 2014

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Ann [REDACTED]  
[REDACTED]  
May 19, 2014

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Kelly [REDACTED]  
[REDACTED]  
May 19, 2014

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I have PKU to. Please support people like me!

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amanda [REDACTED]  
[REDACTED]  
May 19, 2014

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cynthia [REDACTED]  
[REDACTED]  
May 19, 2014

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Kristen [REDACTED]  
[REDACTED]  
May 19, 2014

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Carla [REDACTED]  
[REDACTED]  
May 19, 2014

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I had 2 sister with PKU. Please help the PKU patients!!!

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Sharilyn [REDACTED]  
[REDACTED]  
May 19, 2014

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Gloria [REDACTED]  
[REDACTED]  
May 19, 2014

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Charlotte [REDACTED]  
[REDACTED]  
May 19, 2014

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Roswitha [REDACTED]  
[REDACTED]  
May 19, 2014

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Michelle [REDACTED]  
[REDACTED]  
May 19, 2014

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Melissa [REDACTED]  
[REDACTED]  
May 19, 2014

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Kylie [REDACTED]  
[REDACTED]  
May 19, 2014

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Kirsten [REDACTED]  
[REDACTED]  
May 19, 2014

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Elizabeth [REDACTED]  
[REDACTED]  
May 19, 2014

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Michaela [REDACTED]  
[REDACTED]  
May 19, 2014

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Cindy [REDACTED]  
[REDACTED]  
May 19, 2014

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Kristin [REDACTED]  
[REDACTED]  
May 19, 2014

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Donna [REDACTED]  
[REDACTED]  
May 19, 2014

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Linda [REDACTED]  
[REDACTED]  
May 19, 2014

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Kellsey [REDACTED]  
[REDACTED]  
May 19, 2014

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Joanne [REDACTED]  
[REDACTED]  
May 19, 2014

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Vanessa [REDACTED]  
[REDACTED]

May 19, 2014

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Michele [REDACTED]  
[REDACTED]

May 19, 2014

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Amanda [REDACTED]  
[REDACTED]

May 19, 2014

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Geoffrey [REDACTED]  
[REDACTED]

May 19, 2014

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Tammie [REDACTED]  
[REDACTED]

May 19, 2014

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Emily [REDACTED]  
[REDACTED]

May 19, 2014

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I'm 48 I have cpku I have 2 sets of twins one living / one set past I went k on my diet with all my pregsbut I'm not on my diet bc I can't afford it I also love re food I used to steal food that I couldn't have bc eveybody ate in front of me/ make fun of me 0a my diet if we could get a cure then we wouldn't b embaressed ab r diet 0a made fun of.maybe we could actually get some insurance like ebelse

teresa [REDACTED]  
[REDACTED]

May 19, 2014

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My grandchildren's future as healthy, contributing American citizens, depends on this coverage.

Jane [REDACTED]  
[REDACTED]

May 19, 2014

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Mike [REDACTED]  
[REDACTED]

May 19, 2014

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Heather [REDACTED]  
[REDACTED]

May 19, 2014

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Christa [REDACTED]

May 19, 2014

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Kathy [REDACTED]

May 19, 2014

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Julie [REDACTED]

May 19, 2014

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Marie [REDACTED]

May 19, 2014

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Jim [REDACTED]

May 19, 2014

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My son has Classic PKU.

deborah [REDACTED]

May 19, 2014

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Amanda [REDACTED]

May 19, 2014

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Jim [REDACTED]

May 19, 2014

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Judy [REDACTED]

May 19, 2014

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Jessica [REDACTED]

May 19, 2014

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Shana [REDACTED]

May 19, 2014

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Lisa [REDACTED]

May 19, 2014

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Beth

[REDACTED]

May 19, 2014

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Valerie

[REDACTED]

May 19, 2014

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Michael

[REDACTED]

May 19, 2014

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Elisa

[REDACTED]

May 19, 2014

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chancy

[REDACTED]

May 19, 2014

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Rochelle

[REDACTED]

May 19, 2014

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Tracie

[REDACTED]

Tracie

[REDACTED]

May 19, 2014

---

The [REDACTED] Boys!!!

Grace

[REDACTED]

May 19, 2014

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Tiffany

[REDACTED]

May 19, 2014

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Aleksandra

[REDACTED]

May 19, 2014

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Tina

[REDACTED]

May 19, 2014

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jean [REDACTED]  
May 19, 2014

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Selena [REDACTED]  
May 19, 2014

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Vail [REDACTED]  
May 19, 2014

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Darla [REDACTED]  
May 19, 2014

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Lisa [REDACTED]  
May 19, 2014

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Irene [REDACTED]  
May 19, 2014

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Sherry [REDACTED]  
May 19, 2014

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Delbert [REDACTED]  
May 19, 2014

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Caitlin [REDACTED]  
May 19, 2014

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Brent [REDACTED]  
May 19, 2014

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Katherine [REDACTED]  
May 19, 2014

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Sue [REDACTED]  
May 19, 2014

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Ashley [REDACTED]  
[REDACTED]  
May 19, 2014

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Theresa [REDACTED]  
[REDACTED]  
May 19, 2014

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Dan [REDACTED]  
[REDACTED]  
May 19, 2014

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Katherine [REDACTED]  
[REDACTED]  
May 19, 2014

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A [REDACTED]  
[REDACTED]  
May 19, 2014

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Pam [REDACTED]  
[REDACTED]  
May 19, 2014

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Paula [REDACTED]  
[REDACTED]  
May 19, 2014

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Mary [REDACTED]  
[REDACTED]  
May 19, 2014

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Amber [REDACTED]  
[REDACTED]  
May 19, 2014

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Katherine [REDACTED]  
[REDACTED]  
May 19, 2014

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Terry [REDACTED]  
[REDACTED]  
May 19, 2014

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This metabolic condition that individuals are born with is not a choice. It is a genetic condition. Survival and normalcy are dependent upon this medical food. All those who have PKU without a doubt should be fully covered. Fully reimbursed! In addition, more funding should be included towards the research & development of pill form rather than liquid formula. Reach out and make this your Critical Attention. This is a clear cut issue that should not have any discrepancies & requires immediate attention. The denial is very unreasonable and life threatening. Afterall - how does the PKU disorder survive without this vital nutrition? Listen up and Change the LAW !

Carolyn [REDACTED]  
[REDACTED]

May 19, 2014

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I am an adult living with PKU. I struggle with coverage for formula and food. When not able to get what is necessary for me to function properly my levels go up high. Then it becomes a challenge to daily tasks that most others find easy. when my levels are not where they need to be I have concentration problems, tremors, spasms, and other things happen. This is a diet for life so it needs to be covered. The cost is insurmountable. Without coverage, out of pocket expenses are too much. Please help us.

Wendy [REDACTED]  
[REDACTED]

May 19, 2014

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Emily [REDACTED]  
[REDACTED]

May 19, 2014

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Alan [REDACTED]  
[REDACTED]

May 19, 2014

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This is an urgent cause

Yvette [REDACTED]  
[REDACTED]

May 19, 2014

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Jesse [REDACTED]  
[REDACTED]

May 19, 2014

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Michael [REDACTED]  
[REDACTED]

May 19, 2014

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geri [REDACTED]  
[REDACTED]

May 19, 2014

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Quint

██████████  
May 19, 2014

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Kyle

██████████  
May 19, 2014

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Michael

██████████  
May 19, 2014

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Jessica

██████████  
May 19, 2014

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Amanda

██████████  
May 19, 2014

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Mike

██████████  
May 19, 2014

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Molly

██████████  
May 19, 2014

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Amy

██████████  
May 19, 2014

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Mary Jane

██████████  
May 19, 2014

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Kelley

██████████  
May 19, 2014

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Jill

██████████  
May 19, 2014

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raymond

██████████  
May 19, 2014

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PKU is one of the conditions tested for through newborn screening. Doesn't make sense to me, to test for it but not help with the cost of treatment.

Mary Ann [REDACTED]  
[REDACTED]  
May 19, 2014

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Amanda [REDACTED]  
[REDACTED]  
May 19, 2014

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Molly [REDACTED]  
[REDACTED]  
May 19, 2014

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Please!!!

Kathy [REDACTED]  
[REDACTED]  
May 19, 2014

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Marg [REDACTED]  
[REDACTED]  
May 19, 2014

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Genetic medical conditions treatments whether food or pill to sustain a "normal" day to day life without further damage should be covered if ordered by a physician!

Kelly [REDACTED]  
[REDACTED]  
May 19, 2014

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This is for you Joshua and Jonathan!!! Love, The Texas [REDACTED]

Sarah [REDACTED]  
[REDACTED]  
May 19, 2014

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Chris [REDACTED]  
[REDACTED]  
May 19, 2014

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All medical conditions should be treated without restrictions.

Mary [REDACTED]  
[REDACTED]  
May 19, 2014

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Beatriz [REDACTED]

May 19, 2014

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Medical foods are not a shot or pill, not a traditional form of treatment, but are THE treatment for PKU. With the Federal Lunch Guidelines recognizing the importance of diet compliance in the schools, and mandating the schools to purchase and prepare the low protein foods, it's unbelievable that mandating for the other meals of the day has been overlooked. Without the proper diet these children and adults suffer unnecessary health issues only leading to bigger costs than the medical food and formula. A family struggling with bills shouldn't have to choose whether to pay the bills or to provide the needed treatment for their child.

Sandy [REDACTED]

May 19, 2014

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Doris [REDACTED]

May 19, 2014

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Sherry [REDACTED]

May 19, 2014

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Michele [REDACTED]

May 19, 2014

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Lindsay [REDACTED]

May 19, 2014

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Natalie [REDACTED]

May 19, 2014

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Danielle [REDACTED]

May 19, 2014

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Michelle [REDACTED]

May 19, 2014

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Blythe [REDACTED]

May 19, 2014

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Without my PKU formula, I would not be an employed, civically engaged, tax-paying citizen I am today. However my formula costs approximately \$60 per can, and I use about one can of formula per day. Medical insurance covers only a fraction of this cost and sometimes denies coverage altogether. Affording treatment to allow me to be a normally functioning adult should not be cost prohibitive for patients who depend upon this life-saving care!

Virginia [REDACTED]  
[REDACTED]  
May 19, 2014

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Mary Ellen [REDACTED]  
[REDACTED]  
May 19, 2014

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Dana [REDACTED]  
[REDACTED]  
May 19, 2014

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Sonia [REDACTED]  
[REDACTED]  
May 19, 2014

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Sarah [REDACTED]  
[REDACTED]  
May 19, 2014

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STEVE  
[REDACTED]  
May 19, 2014

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My 6 year old son has Classic PKU! March of Dimes provides are formula and we receive assistance for low-protein foods as long as we stay under a specific income bracket. If we lose any of this help it will devastate my family! We can not afford to pay \$60 for a can of medicated formula (we go through a can every 3 days). We also can not afford the low-protein food. A box of mac 'n' cheese (the size of a Kraft box) is \$12 without the shipping! We need your help!

Dayna [REDACTED]  
[REDACTED]  
May 19, 2014

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Debbie [REDACTED]  
[REDACTED]  
May 19, 2014

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I have PKU and am pregnant with my second baby, my other daughter has autism, it so important to keep my bloods controlled would them to get a cure for PKU, life can get soo hard

Lynsey [REDACTED]

[REDACTED]  
May 19, 2014

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Susan [REDACTED]  
[REDACTED]  
May 19, 2014

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kenny [REDACTED]  
[REDACTED]  
May 19, 2014

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Diane [REDACTED]  
[REDACTED]  
May 19, 2014

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Christina [REDACTED]  
[REDACTED]  
May 19, 2014

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Ben [REDACTED]  
[REDACTED]  
May 19, 2014

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Shahia [REDACTED]  
[REDACTED]  
May 19, 2014

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Kathrine [REDACTED]  
[REDACTED]  
May 19, 2014

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Erica [REDACTED]  
[REDACTED]  
May 19, 2014

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Lissa [REDACTED]  
[REDACTED]  
May 19, 2014

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These people need help. The costs are burdening families. Make this happen, please.

Barry [REDACTED]  
[REDACTED]  
May 19, 2014

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kathryn [REDACTED]  
[REDACTED]  
May 19, 2014

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Charles [REDACTED]  
[REDACTED]  
May 19, 2014

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ernie  
[REDACTED]  
May 19, 2014

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Tiffany [REDACTED]  
[REDACTED]  
May 19, 2014

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Please make it easier for people with pku to get the help they need. Thank you

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billie kay [REDACTED]  
[REDACTED]  
May 19, 2014

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Nicholas [REDACTED]  
[REDACTED]  
May 19, 2014

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cindy [REDACTED]  
[REDACTED]  
May 19, 2014

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elvira  
[REDACTED]  
May 19, 2014

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For my little sister

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Michelle [REDACTED]  
[REDACTED]  
May 19, 2014

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please help with their PKU treatments

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sally [REDACTED]  
[REDACTED]  
May 19, 2014

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Patricia [REDACTED]  
[REDACTED]  
May 19, 2014

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crystal [REDACTED]  
[REDACTED]

May 19, 2014

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Dawn [REDACTED]  
[REDACTED]

May 19, 2014

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Stephanie  
[REDACTED]

May 19, 2014

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It would be a blessing to be able to get medical foods, any kind of formula I needed, or drug therapies at an affordable rate or covered completely! Right now I can't afford any of them and if Michigan stops providing free formula then I don't know what I'm going to do! It's expensive to even buy the lower protein health food options from the store!

Kylee  
[REDACTED]

May 19, 2014

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Anne [REDACTED]  
[REDACTED]

May 19, 2014

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Please ad pku!!!

stephanie  
[REDACTED]

May 19, 2014

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Ami [REDACTED]  
[REDACTED]

May 19, 2014

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Carrie [REDACTED]  
[REDACTED]

May 19, 2014

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Moya [REDACTED]  
[REDACTED]

May 19, 2014

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I have a pku son , all every parent wants is to give their child a "normal" type of life. Unfortunately the cost of these products for many is to expensive to have a on regular basis like the "norm".

Elizabeth [REDACTED]  
[REDACTED]

May 19, 2014

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Medical food For PKU is bank breaking especially when we want out child to have as much of a normal life as possible. because it takes so much money to feed him it takes away from him and the family in other areas. It makes it very hard to provide for the family on other ways when just 2 half full boxes of mac'n cheese and wheat starch for bread comes to 73 dollars inducing the shipping. Please help us!

Amanda [REDACTED]  
[REDACTED]  
May 19, 2014

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Kathy [REDACTED]  
[REDACTED]  
May 19, 2014

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LeeAnne [REDACTED]  
[REDACTED]  
May 19, 2014

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Rachel [REDACTED]  
[REDACTED]  
May 19, 2014

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Please help these children. They need this!

Karen [REDACTED]  
[REDACTED]  
May 19, 2014

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Charlotte [REDACTED]  
[REDACTED]  
May 19, 2014

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Elizebeth [REDACTED]  
[REDACTED]  
May 19, 2014

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Kelly [REDACTED]  
[REDACTED]  
May 19, 2014

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Stephanie [REDACTED]  
[REDACTED]  
May 19, 2014

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Christan [REDACTED]  
[REDACTED]  
May 19, 2014

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Anna [REDACTED]  
[REDACTED]

May 19, 2014

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Thank you for your time.

Mandy [REDACTED]  
[REDACTED]

May 19, 2014

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Charity [REDACTED]  
[REDACTED]

May 19, 2014

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My 5 year old daughter has PKU and we have consistently had to battle for insurance coverage. It is a nightmare. Please help!!

Emily [REDACTED]  
[REDACTED]

May 19, 2014

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Dianna [REDACTED]  
[REDACTED]

May 19, 2014

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Barbara [REDACTED]  
[REDACTED]

May 19, 2014

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Melissa [REDACTED]  
[REDACTED]

May 19, 2014

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Lori [REDACTED]  
[REDACTED]

May 19, 2014

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Stacey [REDACTED]  
[REDACTED]

May 19, 2014

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David [REDACTED]  
[REDACTED]

May 19, 2014

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Lauren [REDACTED]  
[REDACTED]

May 19, 2014

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Yes, my name and address are the same.

Sandra [REDACTED]  
[REDACTED]  
May 19, 2014

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Karen [REDACTED]  
[REDACTED]  
May 19, 2014

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Martha [REDACTED]  
[REDACTED]  
May 19, 2014

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Catherine  
[REDACTED]  
May 19, 2014

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Chelsea  
[REDACTED]  
May 19, 2014

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Wendi [REDACTED]  
[REDACTED]  
May 19, 2014

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Sarah  
[REDACTED]  
May 19, 2014

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Jackie [REDACTED]  
[REDACTED]  
May 19, 2014

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Lisa [REDACTED]  
[REDACTED]  
May 18, 2014

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A cure needs to be found for PKU. Although newborn screening is commanded by law, it isn't a law to treat PKU. And with no cure out there at the moment a lot of parents are deciding to let their children's mental health suffer because of it. I believe if there was a cure for PKU more parents would get treatment for their young ones, the future of our nation.

Laura [REDACTED]  
[REDACTED]  
May 18, 2014

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Hi I am 42 years old I have PKU. Without the diet and the medication I'm on for my PKU I wouldn't be able to live as normal as I am now as I got older more symptoms got worse. Hand tremors, sever mood swings depression, falling asleep straight away after high protein foods amongst other side affects. Now I've been on my PKU diet for 10 years and I will never go back. It's the best decision I have ever made. I really believe that every child and adult deserve to have the right medication and food to help them feel as normal as possible. It's made such a difference to my life having a PKU diet along with the support of the hospital, dietatians, and the government backing. I'm very lucky in Australia and I hope that others are able to have the same benifits we do here in Australia.

Nicole [REDACTED]  
[REDACTED]  
May 18, 2014

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Tamara  
[REDACTED]  
May 18, 2014

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Please put funding in finding a cure for PKU

Renee [REDACTED]  
[REDACTED]  
May 18, 2014

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Micheal [REDACTED]  
[REDACTED]  
May 18, 2014

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Marshall [REDACTED]  
[REDACTED]  
May 18, 2014

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Samantha [REDACTED]  
[REDACTED]  
May 18, 2014

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Selina [REDACTED]  
[REDACTED]  
May 18, 2014

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Justine [REDACTED]  
[REDACTED]  
May 18, 2014

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April [REDACTED]  
[REDACTED]  
May 18, 2014

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Jessica [REDACTED]

May 18, 2014

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I have a 9 year old daughter with PKU. In order to keep her healthy these foods are a necessity.

erin [REDACTED]

May 18, 2014

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For my daughter and cousin, both with PKU.

Harold [REDACTED]

May 18, 2014

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I have PKU and it's very difficult to receive any kind of help with formula and low protein foods as it isn't covered in my state and it is necessary to have them both as PKU is diet for life and if off diet for a person with PKU there are many side affects to it.

Jennifer [REDACTED]

May 18, 2014

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Jennifer [REDACTED]

May 18, 2014

As a parent with a child living with arthritis, the treatment experience has been extremely emotional, frustrating, tiring, and helpful. As you can imagine having to negotiate/fight with insurance to cover your daughters much needed biologics or MRIs to determine severity of arthritis damage is exhausting and frustrating to say the least. Then to finally get the approval and have to pay an exorbitant of money for such treatment, with good (according to my HR and comparison to what other people have) insurance. All the while, you are internally fighting with your emotions of "why my child" and my daughters emotional breakdowns or "Why her?" or "Please no more needles mommy." is truly draining.

Just the thought that if we spent more time researching or studying other forms of treatment outside of big machines and needles their mental state and the parents can be so much better. This disease is also very taxing on marriages, beyond dealing with the fact that your child has an autoimmune disease the financially repercussions on the families quality of life.

However, as I type this email and think of everything my family has been through both emotionally and financially over the last two years I can't help but be thankful for our amazing doctors, the biologics that are available that allow her to be an active 5 year old, and the ability to have healthcare. It saddens me for the people that drive hundreds of miles to get to the nearest Pediatric Rheumatologist or don't have the financial resources to pay for the appointments, test, or treatments. This should not be allowed or pushed to the side. Something must be done to impact the number one debilitating disease.

Please reach out to me if you have any further questions or need additional information. Thanks for fighting for those that can't or don't know how.

Parent of a child with JIA,

Jennifer [REDACTED]

My Dad, uncle and I have or had pkd. There was no treatment for then 40 years ago and there is not much more now. My dad did not get dialysis then and at least I have that when I need it and now transplantation is available but there is nothing on the horizon I know of.

I am not looking forward to either of those and at my age I may not ever get a transplant.

I would like my children to have more offered to them.

To the US Congress,

Because the disease is passed on from family to family (genetic) my story will be similar to many others. I lost my father and brother to PKD and I am also afflicted with this horrible disease. I have heard it kills more people than many other diseases in this country that has the attention and funding support of the government. I would just like to bring PKD to the attention of the House and Energy Committee to please consider this disease in the 21st Century Cures initiative. As you know 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).

██████████  
██████████  
██████████

Retired - Bee Keeper - Water Master - Outdoor Enthusiast

Polycystic Kidney Disease (PKD) has afflicted my family for 70+ years. To lose my grandmother, mother, aunts, uncles and cousins to PKD; and to have **19** family members; the 5th generation of family members dealing with the affects of PKD today, tells me that there definitely is not enough being done for my family. Because PKD is a common life threatening disease, I know many other families have suffered the same loss and heartbreak as I have suffered. Presently I am confident in saying, "There is a major 'gap' in the number of treatments available to patients of PKD". In fact, are you aware that presently there is NO treatment or cure for PKD? Once our kidneys fail, dialysis and transplantation is our only option! This is a very frightening situation to be facing. I know, as I have faced both of them. I feel strongly that more needs to be done in securing a number of treatments, or a cure. We need to look at this situation, promote research which could find treatments for Polycystic Kidney Disease.

I feel the government has played a small role in helping PKD patients. Research money has been cut each year that could benefit both parties. Let's think about this. If the government were to spend money on research, help find a treatment or a cure, PKD patients would not need dialysis - which is covered by Medicare.

I hope that a good deal of attention will be given to the fact that there is NO treatment for someone diagnosed with PKD. Further, that 21st Century Cures does keep the PKD patient in mind, as we seek to find hope in brining this disease to its knees!

Thank you,

JoAnn [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]

Hello, my name is John [REDACTED] had been on dialysis for almost 32 years until I received a transplant on April 29, 2014. Please bear with me because what I am about to tell you will improve the rate of cure and number of people cured with end stage renal disease. Over the past decade for profit dialysis centers have been dominating the dialysis landscape.

One large corporation which is one of the biggest players in the for profit dialysis business is Fresenius. The problem with the current business setup with Fresenius is they provide the service at the dialysis centers and they manufacture the critical equipment. By equipment I mean the dialysis machine and the dialyzer. Since they make money on the operation of the dialysis unit, they have no incentive to improve the dialysis equipment. For example if they had the technology to turn a four hour treatment into a one treatment, they probably would not implement that because it would hurt their profits coming from operating the units. Another example would be if they could manufacture a dialysis unit which could be implanted in the human body (an artificial implantable kidney). Dialysis centers would vanish and they would not pursue breakthrough research because it would end their dialysis center business.

The basic problem I am trying to convey here is there is a definite conflict of interest and it goes directly against what is in the best interest of the patient. The best interest of the patient is being compromised by corporate profit.

With respect to Recommendation 6, improving the FDA's tools for monitoring and communicating clinical risks and benefits, I would ask that the Committee consider not only the FDA's tools for monitoring risks and benefits, but also the pharmaceutical and biomedical device industries' ability to communicate with physicians and patients as to the full range of medical and scientific information known to companies and outside investigators as it may develop. Companies often are the best source of such information as to clinical risks and benefits of medicines. Absent a safe harbor rule issued by the FDA that clarifies what companies may do with respect to scientific exchange, physician continuing education, consultative engagements, medical compendia information, and health economics data, they may not act without serious risk of incurring criminal and civil liability under the FDCA and the False Claims Act for the dissemination of off label information under those legal theories developed and advanced by the US Dep't of Justice. This remains the case despite several Federal court decisions that emphasize the limits of FDA regulatory authority in consideration of the First Amendment to the Constitution dating back to the late 1990s (Washington Legal Foundation, IMS v Sorrell, Caronia).

Thank you for your consideration.

John [REDACTED]  
[REDACTED]  
[REDACTED]

Dear Sir or Madam:

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

My daughter [REDACTED] was diagnosed at 22 in 2010 with a progressive disorder called Friedreich's Ataxia. One of the worsts part of this disease is it typically shows itself between 5 and 15 just as kids are starting to dream of their futures and then it trashes those dreams leaving the victims cognitively intact but increasingly physically inoperative. My daughter got this terrible disease during what is called "let onset," but it is every bit as terrible and perhaps in some ways more so in that it destroys a young adults future just as they are getting started in life.

Here is a website to learn more about Friedreich's Ataxia:

<http://www.curefa.org/whatis.html>

I have answered your questions below as fully as I can but for the best quality input I recommend you arrange a meeting with the leaders of FARA, Friedreich's Ataxia Research Alliance, for the best comprehensive and focused inputs about the FDA clinical trial processes and how they might be re-balanced and streamlined.

I am happy to speak to anyone verbally. My telephone number is [REDACTED]

Sincerely,

John [REDACTED]

Dad to [REDACTED]

\* 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. Thanks in large part to our FA-family-created FARA we are in the blessed position (compared to most other rare/orphaned disorders) of having 8 drugs in clinical trial right now. None of them are approved though, 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?

In 1998 some parents of FA'ers saw the need for a FA organization to focus resources and increase awareness and so FARA, FA Research Alliance, was born in that September. Now 15+ years later it is the model organization for other rare disorders. Funding comes from many quarters; grassroots fundraising, a couple of FARA-run fundraisers, many FARA sponsored fundraisers, direct donations, several well-funded individuals (FA cares not about social standing nor financial success), etc. See <http://www.curefa.org/mission.html> on the right side.

FARA recognized early-on the importance of having an effective research and approval process infrastructure. FA-interested drug companies and researchers now come to FARA for

- The FA registry (<http://www.curefa.org/registry.html>),
- FA clinics trained and ready as trial sites: (<http://www.curefa.org/network.html>),
- The FA Natural History Study
- Grants (<http://www.curefa.org/grant.html>) to help further their basic and advanced research work.

FARA also recognized the value of collaboration and teamwork among researchers, government agencies and drug companies. Much of their progress is due to this.

By browsing the FARA website (<http://www.curefa.org/index.html>) you will have a much better understanding of FARA's "value-added" to FA research and the FA community.

Also in 1998 Sue Kittel, under the urgings of other FA families, started FAPG, FA Parents Group, an emotional, problem solving, and research communication support group whose 600+ members support FARA in various ways. <http://www.faparents.org/fapg/> More recently Facebook has been added as a research communication media.

\* 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. How much money do you invest for a disorder that only 10 people in the world has? I do not have that answer.

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.

You've already done the perks for orphan designation and fast track. I'm not qualified to suggest other programs.

\* 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. And you cannot reduce the budgets of the FDA and NIH while expecting them to take this on. Won't happen. You ask for more you give them more.

\* How do you coordinate your research and outreach with other (FA) 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?

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

\* 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.

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

We look to the NIH to help on basic research and collaboration efforts.

We look to the FDA to protect our children from "bad" drug treatment alternatives and to move all drug treatments forward posthaste to separate the "wheat" from the "chaff".

The FDA is underfunded and understaffed. They also work in an atmosphere of fear-of-retaliation. You cannot work in this field of creation, exploration and marketing without some risk of a "bad" drug slipping through the best of processes. When this has happened "government" did not "have their back" and instead hung them out to dry. Should we wonder why they are risk-averse?? The role of government should be to confirm the "standard of the day" and "process adherence" and then back them up! Unfortunately that is not the governmental "rule of the day". "Backing one up" is a matter of political expediency and voter damage control. I have no idea how can "incentivize" any organization in this climate of "cover your buns". Good luck.

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

Tough question. The FDA has struggled with this for many years. There is the medical-professional benefit-risk statistical perspective and then there is the personal benefit-risk. For the professional choose a historical-precedent model and run the numbers. Pass/Fail? Relatively simple. Look at the exceptions, intervention, side "events". Make a judgement call (erring on the safe side; ie, "Do no harm"). For the FA parent or patient it is much more visceral and emotional. Watch your child or yourself decline knowing there is no "cure" nor progression stopping/slowing treatment available. Other patients die around you. Desperation mounts; "We need it now!!" is the cry. "But at what risk?" is usually not heard and if it is heard it probably is not integrated as a real possibility/probability; "Not us/me" is the thought. How do you "work with" that dichotomy of views?

And does the FDA really want to absorb individually the voices of so many emotional people, and should they?

- Focused surveys within a specific disorder and even a specific drug with knowledge of side effects might be a good approach. A survey administered by the FDA to the 650+ world-wide membership of FAPG for instance might make knowledge and evaluable information from the din of individual input.

- Or working with a FARA assigned group in several meetings focused on a specific drug might be useful.

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

Another big question books might be written on.

For me public funding should be used when private funding is not forthcoming or inadequate. Again the tough question is that of volume. How much public funding should be expended on disorders that affect only "5 or 10" people in the whole world? I don't have that answer.

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

FARA is a success story of the highest order and should be looked at as a model for other disorders, diseases and other areas where research collaboration is needed.

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

The Email group FAPG and several Facebook FA groups tie us together daily with opportunities to network and hug at annual FA and Ataxia conferences. Regionally the various fundraising events draw us to one another occasionally.

\* What is the financial burden of your disease?

The financial and mental health burden varies state to state, how FA presents itself and the individual phase of progression.

- 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/accupuncture 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 would better treatments and cures help save money for your family and the federal government?

You know the answer to this as well as I. Less to no caregiving, less to no Medicaid medical costs, some FA'ers might even be employable and off SSI.

\* How can Congress help?

Fund the NIH and the FDA at levels appropriate with the responsibilities you mandate them with.

Back them up instead of throwing them to the sharks when a "bad" drug is discovered.

As a patient suffering (and dying) from ADPKD, I understand the need for rigorous testing and validation of any treatment before use on the human population. However, when a drug has been shown to have positive effects on a disease where there are no other non-surgical alternatives, we need to look even more closely at 1) managing and monitoring its use among those with the disease, and 2) expanding the work already done with this treatment to see where it can be safely used. I am speaking of course, about Tolvaptan. To date it is the ONLY drug shown to have ANY positive effect on this debilitating disease, has been approved (for marketing and prescription) elsewhere in the world, but not in the United States. The direct medical costs associated with this disease are estimated to be over \$1.5 billion annually (taken from the PKD Foundation website), but we have been handcuffed by our own regulatory authorities to make any type of decision that benefits the patient. I was diagnosed in early 1999 and have slowly progressed to stage 3 or 4 of this disease. In that time, not one treatment has been approved for treatment in any way, shape or form. We do not even attempt to evaluate alternatives when a single provider is involved. Rather than allow for the improved health of its population, the United States government would prefer to prevent a potential monopoly of a proven drug treatment. Sometimes, the ship is just too big to turn in its own harbor.

**John** [REDACTED]  
[REDACTED]

I was diagnosed with severe RA in 1976. At that time gold injections were the only known drug that could modify the symptoms of RA. This worked for me for 3 years. Since that time several new drugs have been found to prevent the crippling effects, but a cure is yet to be found for this autoimmune illness. I'm struggling now to maintain my mobility. The pain and fatigue is non-stop 24/7. The cost of the drug treatments for me have been \$50000 to \$80000 a year. More research is needed to find a cure, and new treatments need to be affordable.

Sincerely,

Joleen [REDACTED]

To Members of the Energy and Commerce Committee:

Polycystic Kidney Disease (PKD) is one of the most common life-threatening genetic diseases. It affects an estimated 1 in 500 people, including newborns, children and adults regardless of sex, age, race or ethnic origin. There is currently no treatment to slow or stop the growth of the kidney cysts that are characteristic of this disease and that ultimately cause kidney failure. The only remedies for PKD patients once their kidneys fail are dialysis and transplantation.

My father-in-law suffered from PKD and died two years ago, just 7 years after receiving a transplanted kidney from his son. My wife, Susan, also has PKD, and her long-term health is a source of concern. I desperately wish to grow old with her, to enjoy our retirement years together, and for our sons to benefit from their mother's love and guidance well into adulthood. We hope for dramatic advances that will enable Susan to live an active, healthy life well after the time when most PKD patients' kidneys fail and health deteriorates, but resources are scarce and progress feels achingly slow.

I urge you to appropriate funds for research into treatments and a cure for PKD. Advances in the treatment of PKD would enhance the lives of hundreds of thousands of Americans and their families.

Thank you for your interest in accelerating the pace of cures in America and for soliciting feedback from the American people.

Respectfully,

Jonathan [REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

On behalf of the estimated 600,000 American afflicted with Polycystic Kidney Disease (PKD), I am writing to applaud your efforts on the 21st Century Cures Initiative and to give you a glimpse into what a cure would mean to me and my family.

The dominant form of PKD, which has affected generations of my family including my maternal grandmother, mother, sister, brother and myself, robs us of our vitality in our fourth or fifth decade of life. While we have been working as volunteers and contributing financially for decades to support the basic science and clinical work that may one day lead to treatments and a cure, we have lost my grandmother, cousins and mother to this killer disease. Our immediate family has undergone 3 kidney transplants and there are still two of us who fight every day to maintain what we have left of our kidney function and our normal lifestyles before we too are faced with the unacceptable options of either transplant or dialysis.

The real sadness for me lies in the next generation- that of my children, nieces and nephews - each of whom has a 50% chance of having inherited PKD from an affected parent. A treatment and cure really could be in the realm of possibility for them if only we had appropriate funding to support the dozens of clinical trials and further the work on promising new compounds shown to be effective in combatting PKD. For that we need the power of an initiative such as 21st Century Cures. Imagine the impact of offering the first treatment or cure to the estimated 1 out of every 500 Americans afflicted with this disease; we would be free of the pain and suffering, the worries over getting health insurance and employment to insure we can keep the same, the fear of passing on this terrible legacy to the next generation and of the burden we end up placing on our government through Medicare when we have no further options but to have a transplant or go on dialysis. It would be a win-win-win scenario.

You can help make it happen. We pledge to continue our work as volunteers and fundraisers for the PKD Foundation. We would be honored to see you take up our valiant cause as your own.

Sincerely,

Judith [REDACTED]  
[REDACTED]

Please find attached a paper published in NEJM providing evidence of biomedical R&D expenditure shifting away from the US, which may be relevant to motivating recommendation #8, economic incentives to promote innovation in drug development in the US.

Thank you.

**Justin** [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]

rant increased reimbursement. The hope is that some of the pilot projects currently under way sponsored by the Center for Medicare and Medicaid Innovation (Innovation Center) or by private payers will provide insights to answer this question. For example, can the various models for medical homes and accountable care organizations (ACOs) or other strategies being tested consistently produce savings, and are any early savings that are produced by voluntary participants likely to be generalizable and sustainable? This is clearly a stretch goal, at least in the near term, since most evaluations are still in a relatively early stage, and some of the more advanced models of medical homes are only now beginning to be implemented.

Unfortunately, there are some important strategies that are not being piloted — most notably, projects that assess alternative ways to pay for physician services other than bundling them with institutional services (e.g., those

of hospitals or nursing homes) or strategies involving alternative ways to pay for specialty care (e.g., episode-based payments).

There are a few efforts under development that will begin to focus more systematic attention on changing incentives for specialists. Blue Cross Blue Shield of Michigan, for example, is planning to extend its “fee for value” incentive program to specialists in 2014, and the American Medical Association is in the early stages of developing a condition-based payment system for specialists. Obviously, the results for these activities are years off. Specialists may need to consider whether they will be able and willing to accept more financial risk than they have in the past. The success of physician-led ACOs may clarify their ability to do this successfully.

This year’s interest in fixing the SGR is more promising than it has been in the decade during which Congress has engaged in this year-end ritual. But as in so

much of health care, the devil is in the details, and those have yet to be spelled out.

Disclosure forms provided by the author are available with the full text of this article at [NEJM.org](http://NEJM.org).

From Project HOPE, Bethesda, MD.

This article was published on December 11, 2013, at [NEJM.org](http://NEJM.org).

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## Asia’s Ascent — Global Trends in Biomedical R&D Expenditures

Justin Chakma, B.Sc., Gordon H. Sun, M.D., Jeffrey D. Steinberg, Ph.D., Stephen M. Sammut, M.A., M.B.A., and Reshma Jagsi, M.D., D.Phil.

The National Institutes of Health (NIH) has been a key enabler of the global dominance of the United States in biomedical research and development (R&D). In 2012, NIH funding accounted for \$30.9 billion of the R&D investment in the United States. U.S. government funding contributed to the development of 48% of all drugs approved by the Food and Drug Administration (FDA) and 65% of drugs that

have received priority review between 1988 and 2005.<sup>1</sup>

Owing to cuts mandated by the Budget Control Act of 2011, the NIH budget for fiscal year 2013 was reduced by \$1.7 billion, to \$29.2 billion — a 5.5% reduction that continued a trend of declining federal funding for biomedical research that began in 2003.<sup>2</sup>

A key consideration in NIH budget discussions is the country’s

competitive international standing in biomedical R&D funding.<sup>3</sup>

However, in determining that standing, policymakers have relied on historical trends within the United States and on data on gross expenditures for R&D in science and technology. Since the Global Forum for Health Research reported global expenditures of \$160.3 billion on biomedical R&D for 2005, no analysis, to our knowledge, has examined

Biomedical R&D Expenditures by the Public Sector and Private Industry in the United States, Canada, Europe, and the Asia-Pacific Region, Adjusted for Inflation, 2007–2012.*						
Region	2007	2008	2009	2010	2011	2012
	<i>billions of U.S. \$</i>					
United States	131.3	123.8	119.1	126.3	120.0	119.3
Public	48.0	46.9	47.9	51.4	50.6	48.9
Industry	83.3	76.9	71.2	74.9	69.4	70.4
Canada	6.0	6.1	5.6	5.6	5.6	5.3
Public	4.0	4.1	3.8	3.5	3.4	3.3
Industry	2.0	2.0	1.8	2.1	2.2	2.0
Europe	83.6	90.0	85.6	80.9	84.9	81.8
Public	27.7	31.1	29.0	28.0	28.4	28.1
Industry	55.9	58.8	56.7	52.9	56.5	53.6
Asia–Oceania	41.1	45.6	49.3	52.9	59.8	62.0
Total						
Public	13.5	14.4	15.9	17.3	19.1	19.3
Industry	27.6	31.3	33.4	35.6	40.7	42.7
China	2.0	2.9	4.6	4.0	7.0	8.4
Public	0.6	1.1	1.2	1.1	1.7	2.0
Industry	1.5	1.8	3.4	2.9	5.4	6.3
Japan	28.2	31.3	33.1	34.9	37.5	37.2
Public	7.3	7.6	8.6	9.0	9.6	9.5
Industry	20.9	23.7	24.5	26.0	27.9	27.6
South Korea	3.5	3.6	3.4	4.3	4.9	6.0
Public	0.9	0.9	0.8	1.0	1.0	1.1
Industry	2.6	2.7	2.6	3.3	3.9	4.9
India	1.4	1.7	1.7	1.8	1.8	2.0
Public	0.4	0.4	0.4	0.4	0.4	0.4
Industry	1.1	1.3	1.3	1.3	1.4	1.6
Australia	4.4	4.3	4.6	5.8	6.3	6.1
Public	3.3	3.1	3.6	4.4	4.9	4.7
Industry	1.1	1.2	1.0	1.4	1.4	1.4
Other Asia–Pacific	1.6	1.8	1.9	2.1	2.2	2.4
Public	1.2	1.2	1.3	1.4	1.5	1.6
Industry	0.5	0.5	0.6	0.7	0.7	0.8
Total	262.1	265.6	259.6	265.7	270.3	268.4
Total in nominal values	226.6	240.4	241.8	254.9	266.6	268.4

\* Unless otherwise noted, all values are shown in billions of U.S. dollars, adjusted for inflation to 2012, with the use of the National Institutes of Health Biomedical Research and Development Price Index according to the mean exchange rate for U.S. dollars for each year.

those expenditures in particular (although commentators have described government funding of biomedical R&D in key Asian countries<sup>4</sup>).

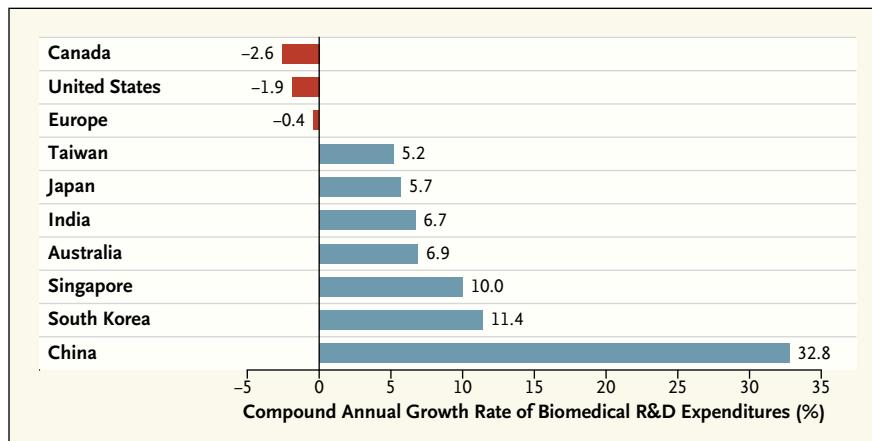
Although a sizable fraction of funding for biomedical R&D in such countries as China, South Korea, Taiwan, and India comes

from national economic-development programs, industry also shoulders a substantial proportion of these costs. To clarify the trends in spending levels, we compared biomedical R&D expenditure levels in the public sector and private industry in the United States, Canada, Europe

(the European Union, Switzerland, Norway, and Iceland), and Asia–Oceania (Australia, China, India, Japan, Singapore, South Korea, and Taiwan) for the period between 2007 and 2012.

We obtained data on biomedical R&D expenditures from government agencies, statistical bureaus, and industry associations and separated them into public spending and industry spending (see Table S1 in the Supplementary Appendix, available with the full text of this article at NEJM.org). Public spending included expenditures by government agencies, educational or research institutions, and charitable organizations, whereas industry spending included expenditures by biotechnology, medical-device, and pharmaceutical firms. We converted all currencies into U.S. dollars using the mean daily exchange rate for each expenditure year and interpolated data where values were missing (see Supplementary Appendix). We adjusted values for inflation using the NIH Biomedical Research and Development Price Index and for differences in the relative value of currencies when calculating percentage share using “purchasing-power parity” data from the International Monetary Fund.

Overall nominal (unadjusted for inflation) R&D expenditures in the United States, Canada, Europe, and Asia–Oceania increased by \$41.8 billion (18.4%), from \$226.6 billion to \$268.4 billion, between 2007 and 2012 (see table). Overall inflation-adjusted expenditures (calculated on the basis of 2012 currency values) increased by \$6.3 billion (2.4%), from \$262.1 billion to \$268.4 billion. But these expenditures increased only in Asia–Oceania (from \$41.1 billion to \$62.0 billion), whereas



**Compound Annual Growth Rate of Biomedical R&D Expenditures by Country, Adjusted for Inflation, 2007–2012.**

The compound annual growth rate was calculated on the basis of total inflation-adjusted biomedical R&D expenditures in U.S. dollars for 2007 and 2012.

they decreased in the United States (from \$131.3 billion to \$119.3 billion), Europe (from \$83.6 billion to \$81.8 billion), and Canada (from \$6.0 billion to \$5.3 billion).

Japan's increase of \$9.0 billion was the largest in absolute dollars. China showed the largest percentage increase — 313.0%, from approximately \$2.0 billion in 2007 to just over \$8.4 billion in 2012, for a compound annual growth rate of 32.8% (see graph). The United States' share of biomedical R&D expenditures among these regions fell from 51.2% in 2007 to 45.4% in 2012 (see Fig. S1 in the Supplementary Appendix). Europe's share remained essentially unchanged — it was 28.5% in 2007 and 29.2% in 2012 — while the proportion spent by Asia–Oceania increased from 18.1% to 23.8%.

Public-sector expenditures (adjusted for inflation) increased in all regions — by \$859 million in the United States, \$452 million in Europe, and \$5.8 billion in Asia–Oceania, driven primarily by a \$2.2 billion increase in Japan and a \$1.4 billion increase in

China. U.S. public-sector R&D spending kept pace with inflation over this period, with decreases in NIH spending being offset by increases from the American Recovery and Reinvestment Act. In 2013, however, the effects of this stimulus were diminished because of sequestration. The United States continued to contribute the largest share of total global public-sector expenditures — 52.9% in 2007 and 50.8% in 2012 — followed by Europe with 26.7% in 2007 and 27.4% in 2012; Asia–Oceania increased its share from 16.6% to 19.1%.

The decline of \$12.0 billion in inflation-adjusted U.S. expenditures from 2007 to 2012 was therefore driven by a \$12.9 billion reduction in industry's investment in R&D. The U.S. share of global industry R&D expenditures decreased from 50.4% in 2007 to 42.3% in 2012. In Europe, industry R&D expenditures decreased by \$2.3 billion, but Europe's share was essentially unchanged (from 29.6% to 30.2%) because its purchasing power increased by 7%. In Asia–Oceania, an increase of

\$15.1 billion in industry's R&D expenditure (from 19.0% to 26.5%) was driven primarily by a \$6.7 billion increase in Japan and a \$4.8 billion increase in China.

The balance between public-sector and industry expenditures changed little over this period: 35.6% public-sector and 64.4% industry in 2007 and 37.1% public-sector and 62.9% industry in 2012. In an analysis adjusting for the size of a region's economy as defined by nominal gross domestic product (GDP), we found that in 2012, the biomedical R&D expenditure as a percentage of GDP was greatest in the United States — approximately 0.76%, as compared with 0.46% in Europe and 0.28% in Asia–Oceania (see Table S2 in the Supplementary Appendix). As a percentage of GDP, U.S. public expenditures actually increased by 5.7%, while U.S. industry expenditures decreased by 12.3%. However, even after adjustment for Asia–Oceania's GDP growth, which was six times that of the United States, overall U.S. expenditures grew 13% more slowly than those in Asia–Oceania.

Our analysis reveals that U.S. inflation-adjusted R&D expenditures and the U.S. share of global expenditures decreased from 2007 through 2012. The decline is remarkable because the United States has provided a majority of the funding for biomedical R&D globally for the past two decades — a share that some previous analyses suggested was as high as 70 to 80%.<sup>2</sup> Moreover, the decline was driven almost entirely by reduced investment by industry, not the public sector, between 2007 and 2012. Sequestration of NIH funding in 2013 and beyond will exacerbate this reduction by causing U.S. public-sector expenditures to decline.

One explanation for the shift in global R&D expenditures may be the attractive cost of conducting R&D in Asia–Oceania, where labor is cheaper and greater government subsidies are available, especially as the development costs per FDA drug approval have increased considerably.<sup>4,5</sup> Accordingly, we found that the U.S. share declined even as global R&D investments by industry remained flat (after adjustment for inflation), which suggests that industry is simply reallocating R&D funding to Asia–Oceania. Because U.S. public-sector expenditures as a percentage of GDP are already 200 to 300% the size of those in Europe and Asia, increasing NIH funding alone may not be a sustainable way of retaining long-term R&D leadership. Instead, even as it boosts NIH funding, the U.S. government might also develop strategies to

provide incentives to industry for investing in biomedical R&D.

Although our data set has its limitations, our findings reveal a decline in U.S. financial competitiveness in biomedical R&D and may have implications for the debate over appropriate federal policy in this area. The lack of a coordinated national biomedical R&D strategy is disappointing, at a time when mature economies such as those of Japan and Europe have maintained their level of investment in this area.

Disclosure forms provided by the authors are available with the full text of this article at NEJM.org.

From Thomas, Mc Nerney & Partners, La Jolla, CA (J.C.); the Robert Wood Johnson Foundation Clinical Scholars Program (G.H.S., R.J.), the Center for Clinical Management Research, Veterans Affairs Ann Arbor Healthcare System (G.H.S.), the Center for Bioethics and Social Sciences in Medicine (R.J.), and the Departments of Otolaryngology–Head and Neck Surgery

(G.H.S.) and Radiation Oncology (R.J.), University of Michigan Health System — all in Ann Arbor; the Singapore Bioimaging Consortium, Agency for Science, Technology, and Research, Singapore (J.D.S.); and the Department of Health Care Management, Wharton School, University of Pennsylvania, Philadelphia (S.M.S.).

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## The Road toward Fully Transparent Medical Records

Jan Walker, R.N., M.B.A., Jonathan D. Darer, M.D., M.P.H., Joann G. Elmore, M.D., M.P.H., and Tom Delbanco, M.D.

Forty years ago, Shenkin and Warner argued that giving patients their medical records “would lead to more appropriate utilization of physicians and a greater ability of patients to participate in their own care.”<sup>1</sup> At that time, patients in most states could obtain their records only through litigation, but the rules gradually changed, and in 1996 the Health Insurance Portability and Accountability Act entitled virtually all patients to obtain their records on request. Today, we’re on the verge of eliminating such requests by simply providing patients online access. Thanks in part to federal financial incen-

tives,<sup>2</sup> electronic medical records are becoming the rule, accompanied increasingly by password-protected portals that offer patients laboratory, radiology, and pathology results and secure communication with their clinicians by e-mail.

One central component of the records, the notes composed by clinicians, has remained largely hidden from patients. But now OpenNotes, an initiative fueled primarily by the Robert Wood Johnson Foundation, is exploring the effects of providing access to these notes.<sup>3</sup> Beginning in 2010, at Beth Israel Deaconess Medical Center (which serves ur-

ban and suburban Boston), Geisinger Health System (in rural Pennsylvania), and Harborview Medical Center (Seattle’s safety-net hospital), more than 100 primary care doctors volunteered to invite 20,000 of their patients to read their notes securely online.<sup>4</sup>

Although only a small minority of these doctors’ patients used the portals, the initial findings were striking. At the end of a year, four of five patients had read the notes, and among those who responded to a survey, large majorities reported having better recall and understanding of their care plans and feeling more in control of their health care. More-

To whom it make concern:

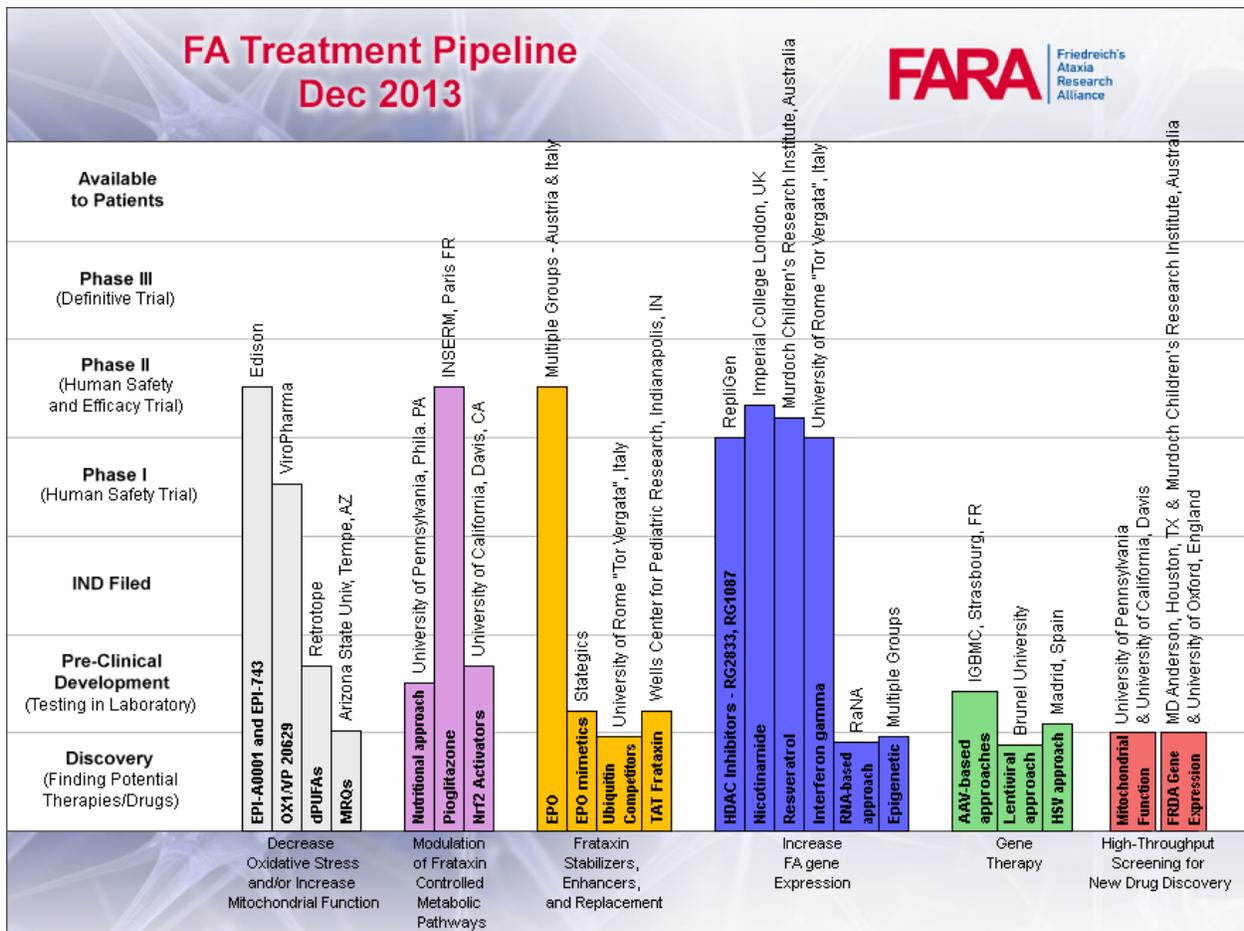
The questions are in bold and my answers are below each of them.

**What is the state of discovery of cures and treatments for your disease?**

My 10 year old has Friedreich's Ataxia which is a progressive neurological disease with no cure or treatment at this time. It is a life shortening disease that affects his heart and balance & coordination. Cognitively he is not effected, but his nerves are deteriorating and so are his functions.

**Are there cures and treatments now or on the horizon?**

See the progress/status in the chart below:



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

FARA has a website for patient registries along with a parent support group. For more details, see [www.curefa.org](http://www.curefa.org)

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

Have a database that is easily accessible to patients along with a general email notification that maybe sent when updates are made.

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

Speed up the FDA process and in severe cases evaluate the situation on an individual basis. Reduce all the paperwork and use technology to help advance and collaborate information more quickly and effectively.

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

FARA fundraises and organizes various doctors, researches and pharmaceutical companies to meet regularly and collaborate information to increase the chances of a cure to be found.

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

FARA and CHOP (Children's Hospital of Philadelphia) send out emails regarding treatment options and possible drug trials in the pipelines. They work hand in hand with the doctors that communicate to their patients things to watch out for.

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

Trials are held under strict guidelines and taken extremely seriously. There are limited spaces available and limited sites that the trials are taking place.

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

Obviously, funding is a huge component but also speeding up the process to get effective drugs on the market is very important. People around the world are effected so if a drug has gone through the necessary safety measures already, the need to start from scratch may not be needed as much.

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

Evaluating risks versus benefits is important but ultimately the patient and their families should be the decision maker as their lives are directly effected.

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

FARA is a non-profit organization that ultimately uses the funding from the private sector to enhance any of the medicine advancements.

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

My son participated in a trial study from October 2013 to February 2014. Though the drug did not help with his balance and coordination, it did increase his overall energy level. This resulted in a better quality of life. He was able to do more activities, he recovered quicker from illnesses and ultimately he was happier during this time as this was a sign of hope.

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

There is a parent support email that allows everyone registered to communicate freely and openly. People can discuss things that have helped, things that are unsafe for this condition, experiences, etc....

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

Recently, we had to move from a two storey home into a bungalow. Various equipment such as wheelchair, grab bars, patient lifts and special dietary foods are costly but needed. If a cure is found, many of these items wouldn't be needed. Also, since this condition is progressive, constant upgrades and changes are needed.

## **How can Congress help?**

Congress can help by reducing the cost of trials, providing more funding so that more opportunities are available. Spread trials across the spectrum so that more people can participate. Make exceptions for special circumstances if it means increasing someone's life.

Sincerely,

Juzer 