



## **Congressional Briefing**

# **Securing Significant Federal Funding for Celiac Disease Research**

**Featured Speaker: Congressman Dwight Evans (D, PA-3, Philadelphia)**

**By: Jon and Leslie Bari**

**U.S. Capitol Visitor Center**

**Congressional Meeting Room North (CVC 268)**

**January 14, 2020**

# Overview

Celiac Disease is a serious autoimmune disease that has historically been underfunded by our government.

Celiac Disease requires serious and sustained Federal funding for medical research to find treatment options and a cure.

Biomedical breakthroughs are only enabled through research, and this requires a transformative commitment with Federal funding!

# What is Celiac Disease?

Celiac Disease is a serious auto-immune disorder that causes more than **200 debilitating symptoms**.

In Celiac Disease patients, Gluten, a protein found in wheat, barley and rye, triggers an immune response leading the body to attack and damage the small intestine.

Moreover, Gluten ingestion for people with Celiac Disease:

- Causes **permanent** immunological scarring
- Doubles the risk of **heart disease**
- **Acts as a carcinogen**, quadrupling the risk of small intestinal cancers.

Source: Public Witness Testimony submitted by Dr. Stefano Guandalini and Dr. Bana Jabri of the University of Chicago, U.S. Senate Appropriations Committee, Subcommittee on Labor, Health and Human Services, and Related Agencies, Department of Health and Human Services, June 3, 2019.

# How Many People Have Celiac Disease?

Celiac Disease afflicts **3 million Americans**, or about 1.0% of the population (by estimate 129,000 Pennsylvanians and 16,000 Philadelphians suffer from Celiac).

Researchers are finding that Celiac Disease prevalence is doubling approximately every 15 years, making it a **public health epidemic**.

Approximately 40.0% of the population carry the Celiac genes, but about 1.0% of the population actually develop Celiac.

This is nearly double the number of people with Crohn's Disease and Ulcerative Colitis combined, and nearly triple the number of those with Type 1 Diabetes.

Source: Public Witness Testimony submitted by Dr. Stefano Guandalini and Dr. Bana Jabri of the University of Chicago, U.S. Senate Appropriations Committee, Subcommittee on Labor, Health and Human Services, and Related Agencies, Department of Health and Human Services, June 3, 2019.

# How Is Celiac Disease Treated?

Strict adherence to a complex and very restrictive Gluten Free diet is the only available treatment option for Celiac Disease patients.

However, respected Celiac Disease researchers including at University of Chicago Celiac Disease Center agree, “There is no such thing as a truly Gluten-FREE diet’ because of the constant risk of cross-contact with Gluten, and Gluten is in 80% of our foodstuffs.”

There is no medicine available to treat Celiac (even in the event of accidental ingestion of Gluten), and there is no known cure.

Celiac is a disease, not a diet! In other words, a Gluten Free diet is all that has been available to date, but it is NOT all that is needed to treat Celiac Disease.

Source: Public Witness Testimony submitted by Dr. Stefano Guandalini and Dr. Bana Jabri of the University of Chicago, U.S. Senate Appropriations Committee, Subcommittee on Labor, Health and Human Services, and Related Agencies, Department of Health and Human Services, June 3, 2019.

# The Celiac Treatment Burden

The lifetime treatment burden of the Gluten Free diet is perceived by patients to be **second only to end-stage renal disease**, and by caregivers, **comparable to caring for a patient with cancer**. [1]

Diagnosed patients report that they miss, on average, **23 days of work and school annually**, resulting in excess utilization of our healthcare resources. [2]

It's Personal – We can attest to the treatment burden including the **continuous concern over intestinal healing, proper nutrition, cross contact, and inclusion of our son in life's numerous daily activities that involve food** at home, at school and away from home in daily life.

## Sources:

[1] "Patient Perception of Treatment Burden is High in Celiac Disease Compared to Other Common Conditions," PMC, National Library of Medicine, National Institutes of Health, July 1, 2014, <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4159418/>, and "What is Celiac Disease?", Celiac Disease Foundation, <https://celiac.org/about-celiac-disease/what-is-celiac-disease/>.

[2] Testimony of Marilyn G. Geller, CEO, Celiac Disease Foundation (Los Angeles, CA), to the U.S. House of Representatives Committee on Appropriations, Subcommittee on Labor, Health and Human Services, Education, and Related Agencies, April 9, 2019, <https://celiac.org/april-2019-congressional-testimony-by-ceo-marilyn-g-geller/>.

# The “Modern” History

The year was 1887...

- Grover Cleveland was President of the United States
- Gustave Eiffel began work on the Eiffel Tower in Paris
- Americans celebrated the 100<sup>th</sup> Anniversary of the Constitution of the United States (1787)
- The NIH was established
- Dr. Samuel Gee presented the first modern definition of Celiac Disease at a lecture at the Hospital for Sick Children in London. Dr. Gee first established the connection between Celiac Disease and diet, and theorized, **“if the patient can be cured at all, it must be by means of diet.”** [1]

**That was 133 years ago!!!**

[1] <https://www.beyondceliac.org/celiac-news/from-mussels-to-bananas-to-gluten-celebrating-samuel-gee-advances-in-celiac-disease-research/>

# The “Modern” History (Continued)

**The year was 1952...**

- Harry S. Truman was President of the United States
- The Korean War was being fought
- Dr. Jonas Salk was poised to [develop and test a Polio vaccine \(\(1953-1954\) which led to the Polio vaccine being licensed in 1955\)](#)
- First discovered that Gluten was the trigger of Celiac Disease [1]

**That was 68 years ago!!!**

Fast forward to 2019, and the only known treatment option for Celiac Disease is a medically required, strict Gluten Free diet, with no exceptions!

[1] <https://www.beyondceliac.org/celiac-news/from-mussels-to-bananas-to-gluten-celebrating-samuel-gee-advances-in-celiac-disease-research/>

# Permanent State of Food & Nutritional Insecurity

The Universal Declaration of Human Rights expressly and directly links “health and well-being” with adequate food – **The Human Right to Adequate Food**.

However, for Celiac patients and their caretakers, the issue of food being cross contaminated with Gluten contributes to a **permanent state food and nutritional insecurity** to holders of this auto-immune disease who medically require a lifelong special diet.

This can cause loss of quality of life, socialization, and health of the individual, both in the short and long term.

Sources:

[“The Principle of Human Right to Adequate Food and Celiac Disease: Advancements and Challenges”](#), by Juliana Nadal (Department of Nutrition. Food Quality and Nutrition Area. Federal University of Parana, Curitiba, PR, Brazil), Sila Mary Rodrigues Ferreira, Islandia Bezerra Da Costa, Suely Teresinha Schmidt (Post-graduation Program in Food and Nutrition Security. Federal University of Parana, Curitiba, PR, Brazil), 2013.

[“Going Gluten Free as a Human Rights Issue”](#), By Yvonne Vissing Ph.D. and Christopher Moore-Vissing, Journal of Gluten Sensitivity, Summer 2016 Issue, July 11, 2016.

# The Status Quo is Unacceptable!

Since it was first theorized in 1887 that treating Celiac Disease patients “[must be by means of diet](#)” (1887 was ironically the same year that the NIH was founded), and it was discovered in 1952 that Gluten was the trigger of Celiac Disease, the status quo from 1952 remains.

Strict adherence to a Gluten Free diet has been the only available treatment!

# What Is the Call to Action?

Celiac Disease research receives virtually no resources from the NIH, and little to no investment from the private sector, in comparison with other diseases, especially when the prevalence, treatment burden, and available treatment options are considered.

It is imperative that Celiac Disease be recognized as an important threat to the health of our citizens by the U.S. Government, including and especially by the NIH, the CDC, the FDA, the DoD and CMS.

# The NIH Has Historically Underfunded Celiac Disease Research

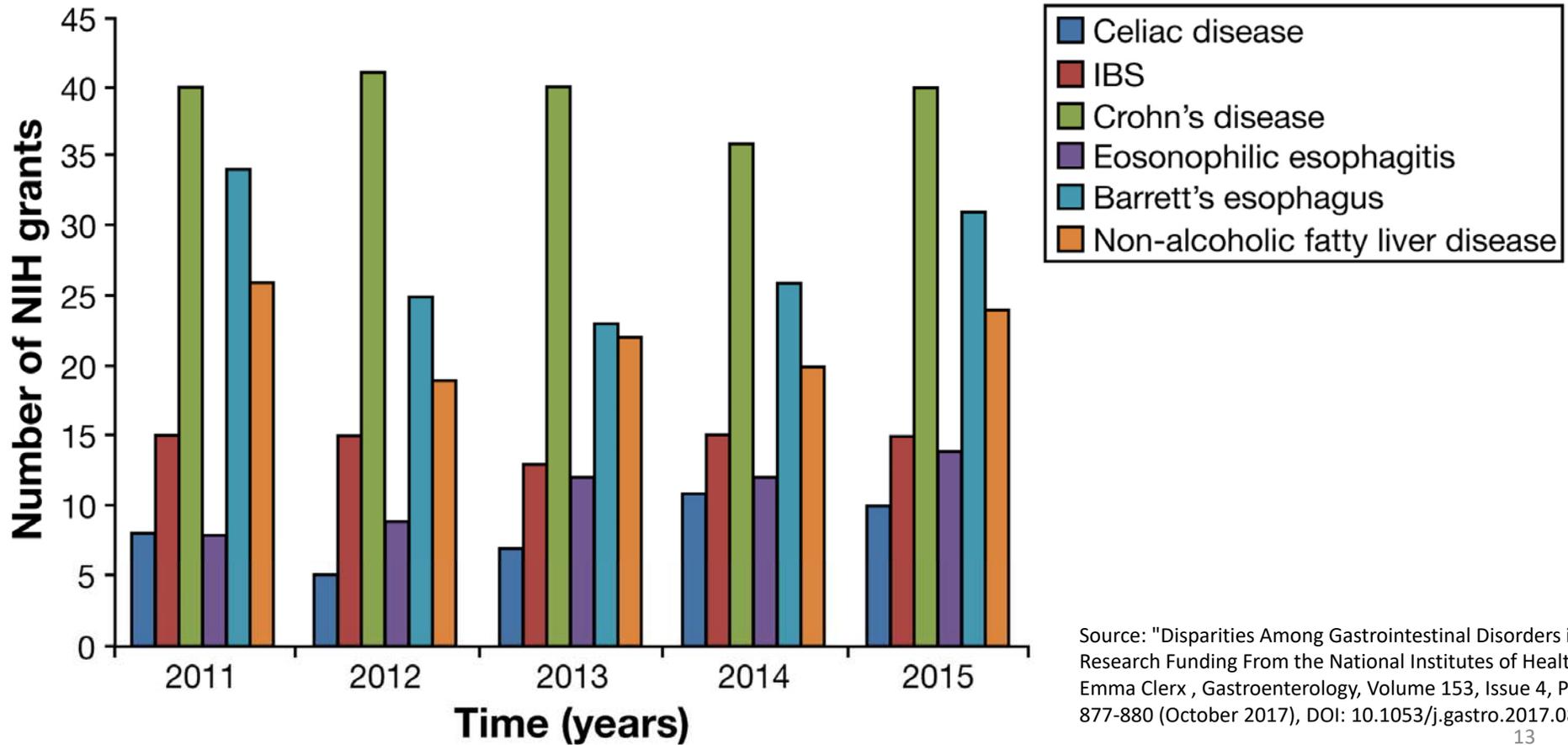
Out of various Gastrointestinal Disorders, from 2011-2015:

- A. **“Celiac disease consistently received the lowest amount of NIH funding over the 5-year period, at approximately \$3 million per year.”**
- B. **“Celiac disease consistently received the lowest amount of NIH grants, at approximately eight grants per year.”**
- C. **“In conclusion, NIH funding of GI diseases is not proportional to disease prevalence or mortality.** These data further suggest that a few diseases, including IBS and celiac disease, are underfunded in comparison with other diseases, especially when the prevalence, burden, and available treatment options are considered.”

Source: “Disparities Among Gastrointestinal Disorders in Research Funding From the National Institutes of Health,” The American Gastroenterological Association, By: Emma Clerx, Harvard University; Sonia Kupfer, Celiac Disease Center at University of Chicago; and Daniel Leffler, North American Society for the Study of Celiac Disease, Beth Israel Deaconess Medical Center; September 4, 2017, [https://www.gastrojournal.org/article/S0016-5085\(17\)36084-5/pdf](https://www.gastrojournal.org/article/S0016-5085(17)36084-5/pdf)

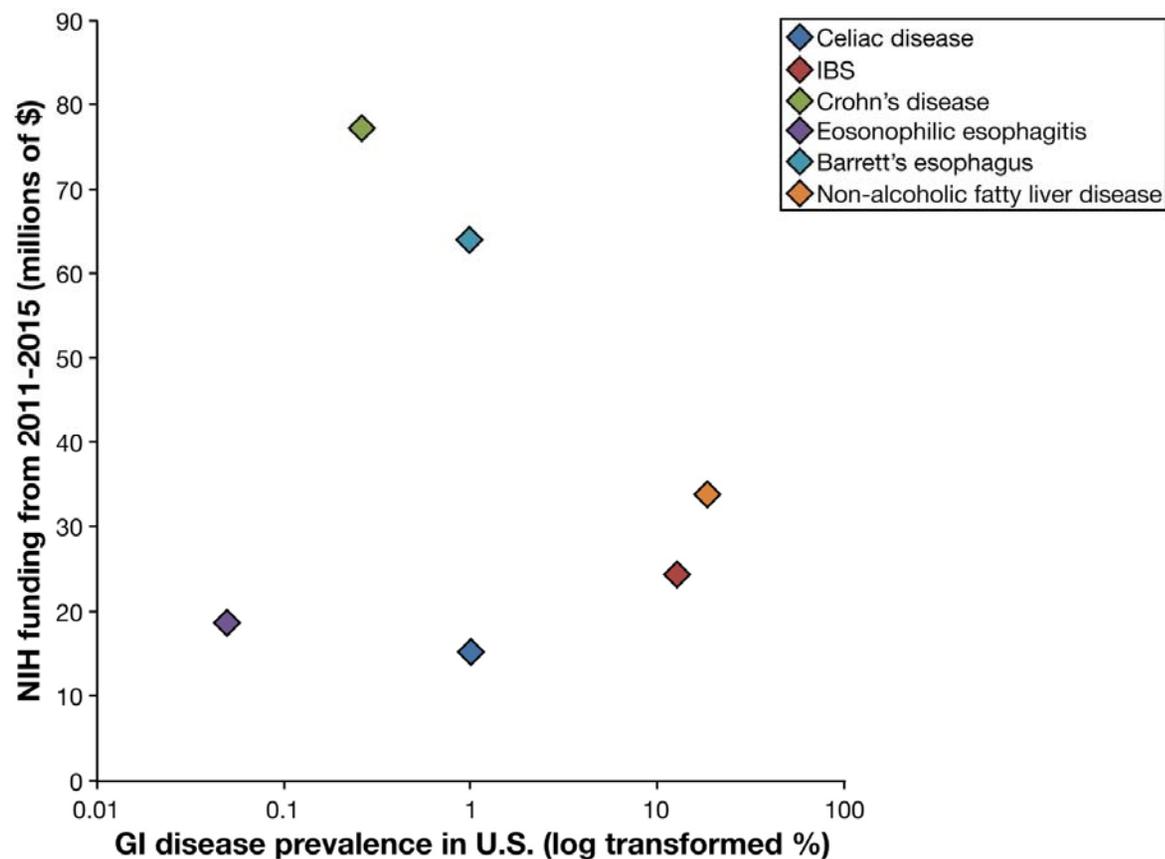
## Number of National Institutes of Health (NIH) Grants Funded of Different Gastrointestinal Disorders from 2011 to 2015

(IBS, irritable bowel syndrome)



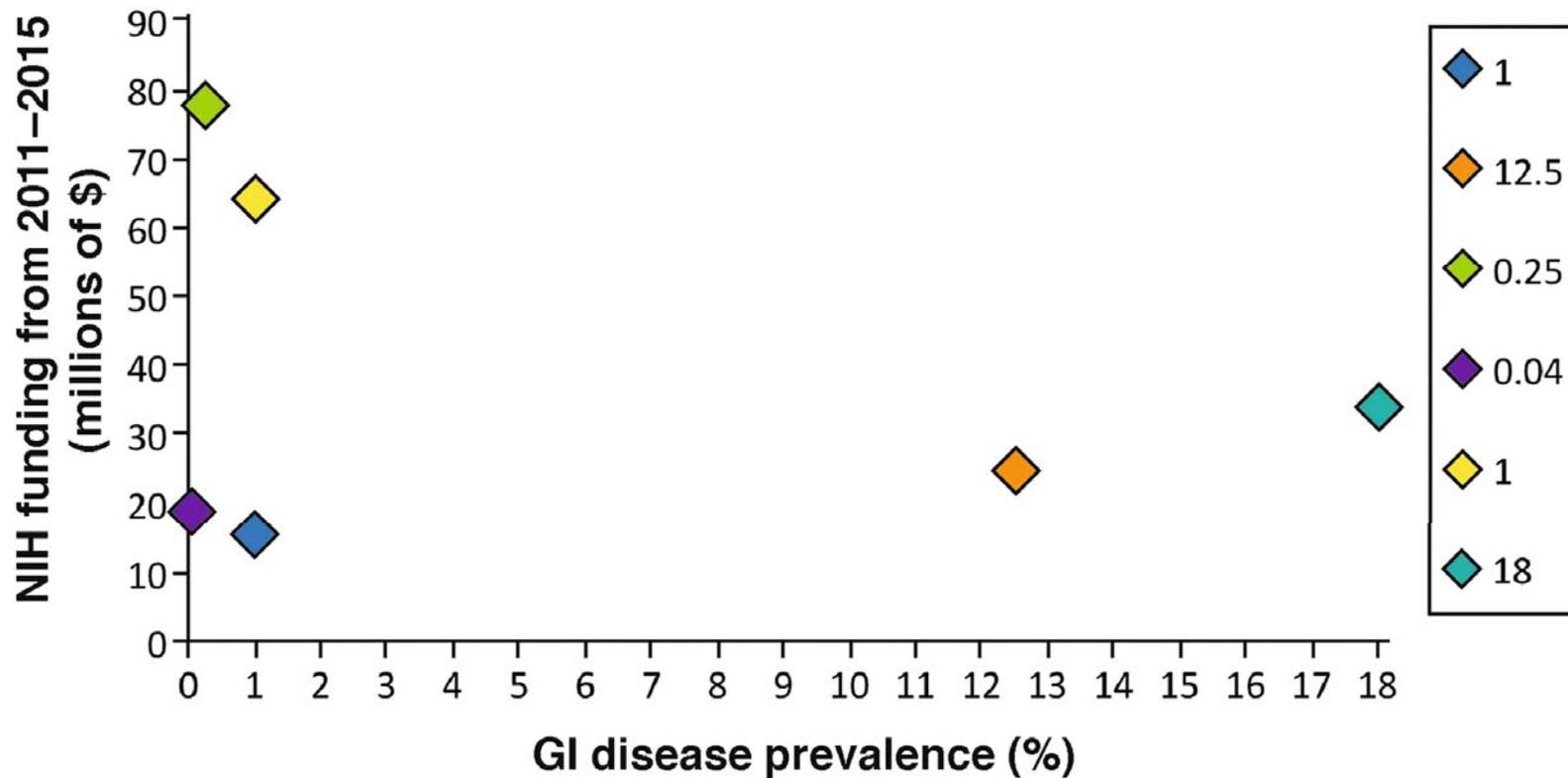
Source: "Disparities Among Gastrointestinal Disorders in Research Funding From the National Institutes of Health", Emma Clerx, Gastroenterology, Volume 153, Issue 4, Pages 877-880 (October 2017), DOI: 10.1053/j.gastro.2017.08.051

## Number of Clinical Versus Basic/Translational Studies on Gastrointestinal (GI) Diseases Funded by the National Institutes of Health (NIH) from 2011 to 2015 (IBS, irritable bowel syndrome)



Source: "Disparities Among Gastrointestinal Disorders in Research Funding From the National Institutes of Health", Emma Clerx, *Gastroenterology*, Volume 153, Issue 4, Pages 877-880 (October 2017), DOI: 10.1053/j.gastro.2017.08.051

## National Institutes of Health (NIH) Funding of Different Gastrointestinal Diseases from 2011 to 2015 Versus Gastrointestinal Disease Prevalence in the United States (IBS, irritable bowel syndrome)



Source: "Disparities Among Gastrointestinal Disorders in Research Funding From the National Institutes of Health", Emma Clerx, Gastroenterology, Volume 153, Issue 4, Pages 877-880 (October 2017), DOI: 10.1053/j.gastro.2017.08.051

# NIH Has Historically Underfunded Celiac Disease Research

NIH's total budget for 2015 was \$30.4 billion, or \$95 per person living in the United States. But of that \$95, only about one penny (\$0.01) per person was allocated to Celiac Disease research. Another way of looking at it is that the NIH has spent only about \$1.00 per American with Celiac per year from 2011-2015.

Unfortunately, not much has changed since that seminal analysis was published in 2017 by the American Gastroenterological Association entitled, "Disparities Among Gastrointestinal Disorders in Research Funding From the National Institutes of Health."

In spite of a lower number of disease specific mortalities as well as many available and emerging treatment options, Crohn's disease received about 40 NIH grants per year averaging a total of about \$16 million annually from 2011-2015, in comparison to Celiac Disease which received about 8 NIH grants per year averaging a total of about \$3.0 million annually from 2011-2015.

In 2018, the [NIH RePORT](#) suggests that Celiac Disease research received a modest increase to 13 grants totaling approximately \$4.7 million. In contrast, in 2018, NIH research funding for Crohn's disease encompassed 210 grants totaling \$69 million.

# NIH Appropriations Scarcity Has Been Causing a Negative Feedback Cycle for Celiac Research

Public funding is perceived as validation of the seriousness of a disease and its research needs.

Absent public funding validation, a vacuum is created, which causes private and philanthropic funding to be scarce.

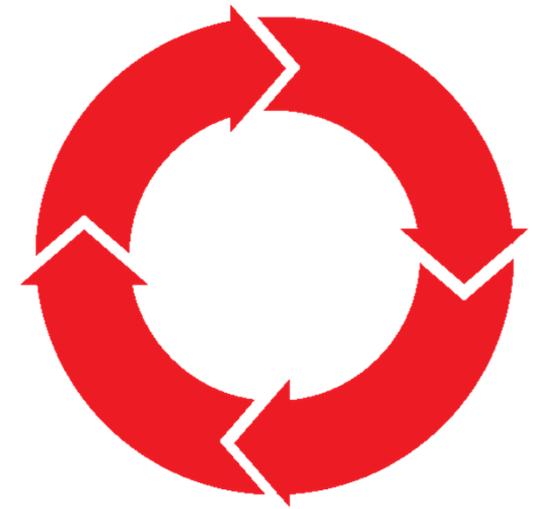
Research in Celiac Disease has lagged behind in the biomedical imagination of other more well NIH funded research GI and Autoimmune diseases.

In contrast to Celiac Disease, the NIH funded Crohn's disease research model is an example of "success breeds success" that has created a **Positive Feedback Loop** (network effects) with ongoing and meaningful government validation, more established research programs recruiting more young investigators, increased NIH grant submissions, increased private sector funding, and increased philanthropic funding.

# Summary of Negative Feedback Cycle Caused by Scarcity of NIH Funding for Celiac Research

## The Biomedical Research Vacuum is Created by:

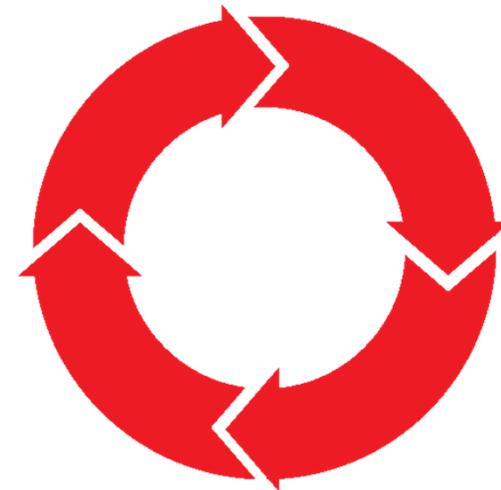
- Lack of meaningful Federal funding
- Lack of validation for Celiac as a serious disease
- Perception that the Gluten Free diet is all that is needed for treatment, as opposed to all that has been available
- Small number of established research, advocacy and education programs
- Under resourced research, advocacy and education programs
- Recruitment of young investigators has been inhibited
- Brain drain of scientific and medical talent away from Celiac Disease
- Fewer NIH grant submissions
- Narrow expertise of NIH review committees over time continuing through today



# Summary of Negative Feedback Cycle Caused by Scarcity of NIH Funding for Celiac Research (Continued)

## The Biomedical Research Vacuum is Created by:

- NIH did not put out any requests for Celiac Disease funding from at least 1999-2017
- Scarcity of corporate funding
- Scarcity of philanthropic funding
- NIH Web site language in 2020 suggests that: 1) Celiac Disease is just a digestive disorder and not an auto-immune disease with many debilitating symptoms, 2) a GF diet is all that is needed, 3) little to no recognition of food insecurity and treatment burden.
- Celiac research funding has principally come from just National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK), and not also National Institute of Allergy and Infectious Diseases – suggests an either/or approach by NIH, rather than a holistic strategy.
- Ample Federal funding for other diseases have made those diseases more desirable among research institutions and investigators, so focus has been directed elsewhere.



# NIH - It's Time to “Reverse These Troubling Trends”, April 15, 2019

**Bipartisan letter to the Senate Appropriations Committee signed by 62 Senators:**

“As NIH grants become more competitive, researchers can easily spend half their careers working before receiving a grant. Secure NIH funding allows scientists to do what they do best - discover tomorrow’s therapies and cures; preventing promising, talented young researchers from leaving the field of biomedical research; and ensuring seasoned investigators do not abandon scientific research altogether or conduct their research outside the United States. We hope that the renewed commitment to NIH funding will help reverse these troubling trends.”

Letter to the Senate Appropriations Committee (Senators Richard Shelby, Roy Blunt, Patrick Leahy, and Patty Murray) on FY2020 funding for NIH, signed by Senators Robert P. Casey, Richard Burr and 60 (sixty) other U.S. Senators, April 15, 2019.

## A Guiding Framework – “The Best Interests of the Child”

According to the **United Nations Convention on the Rights of the Child**:

- “In all actions concerning children, whether undertaken by public or private social welfare institutions, courts of law, administrative authorities or **legislative bodies, the best interests of the child shall be a primary consideration.**” (emphasis added) (Article 3.1)
- “States Parties **recognize the right of the child to the enjoyment of the highest attainable standard of health** and to facilities for the treatment of illness and rehabilitation of health. States Parties shall strive to ensure that no child is deprived of his or her right of access to such health care services.” (Article 24.1)

To address the historical underfunding of Celiac Disease, including with Federal research funding, the “legislative bodies” such as the U.S. Congress and the “administrative authorities” such as the NIH and CDC should use the Convention on the Rights of the Child as a guiding principle in the allocation of funding resources: **“the best interests of the child shall be a primary consideration.”**

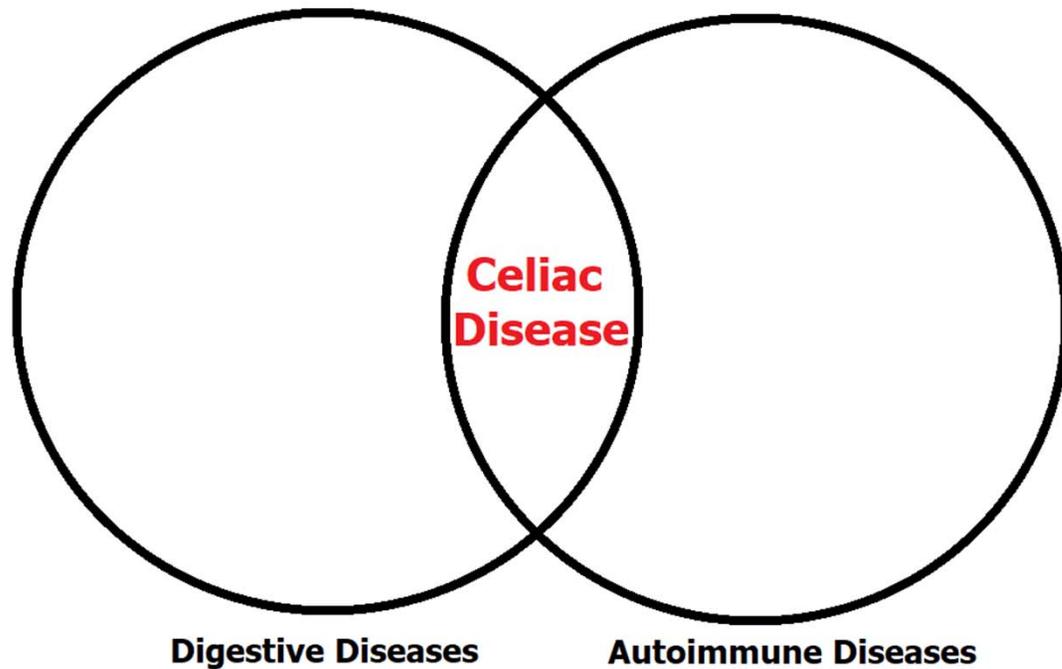
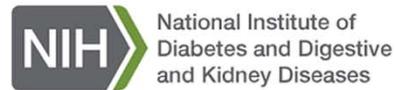
# NIH Report Language – The Opportunity

On December 19, 2019, President Trump signed into law in the FY2020 Federal Budget Resolution which included a historic first for Celiac Disease with this language,

**"Celiac Disease.** The agreement encourages NIH to devote sufficient, focused research to the study of celiac disease, including the autoimmune causation underpinning the affliction. The agreement urges NIAID to better coordinate existing research and focus new research efforts toward causation and, ultimately, a cure of this disease. NIAID is encouraged to coordinate with other Institutes and Centers as appropriate and **to submit its plan for coordination and execution of this research to the Committees no later than 90 days [by March 20, 2020] after enactment of this Act.**" (emphasis added)

# Celiac is at the Intersection of Autoimmune & Digestive Diseases

“It was not the ice cream. It was the cone!”



# The Bigger Picture – Unlocking the Mysteries of Other Auto-immune Diseases

## “Why Is the Study of Autoimmune Diseases a Priority for NIAID?”

The chronic and debilitating nature of these diseases, which can lead to high medical costs and reduced quality of life, is a burden on patients and also affects their families and communities.”[1]

According to the University of Chicago Celiac Disease Center,

“Those with an autoimmune disorder are prone to get other autoimmune disorders...

The most common disorders associated with Celiac Disease are thyroid disease and Type 1 Diabetes.” [2]

However, Celiac has not received meaningful funding from NIAID over the years!

## The On Off Switch

Celiac is the only autoimmune disease with an on-off switch (Gluten). In turn, there is a larger societal benefit of solving Celiac whereby it may help to unlock the medical mysteries behind other Autoimmune Diseases

[1] <https://www.niaid.nih.gov/diseases-conditions/autoimmune-diseases>

[2] <https://www.cureceliacdisease.org/faq/what-other-autoimmune-disorders-are-typically-associated-with-those-who-have-celiac-disease/>

# Next Steps – FY2020 and Beyond

## What's Needed for the NIH Report on Celiac Disease to the Congressional Committees on Appropriations:

- Allocate discretionary funding to seed to new projects in FY2020 for Celiac Disease
- Request proposals for biomedical breakthrough issued by NIH for Celiac research
- Establish Multi-Institute NIH Collaboration and Coordination, including between NIDDK (where most of the limited Celiac funding has come from to date) and NIAID, with sustained multi-year investments, accountability and performance metrics, timelines, etc.
- Secure more in-house relevant Celiac Disease expertise at NIH (NIDDK and NIAID), including from outside experts
- Have NIH treat Celiac as seriously as Chron's Disease and nut allergies, as a "priority" and as a "critical topic"

# Next Steps – FY2020 and Beyond (Continued)

## What's Needed for the NIH Report on Celiac Disease to the Congressional Committees on Appropriations:

- Incorporate more patient perspective and advocacy directly to NIH on Celiac treatment burden and pathways forward
- Change NIH mindset and revise existing NIH language that suggests an implicit bias whereby Celiac is not a serious disease, and that a GF diet is all that is needed
- Focus on research ready initiatives at translational research centers, including with microbiome, biorepository, big data, AI, stem cell research, etc.
- Have Celiac Disease added to the list of conditions eligible for research under the Peer Reviewed Medical Research Program (PRMRP) which is a valuable program within DoD, offering creative, long-term insights into significant medical issues such as Celiac that afflict members of the armed services and their families and do not always receive the investments they require in the private sector
- Appropriate **\$100 Million annually in line item Federal funding** for Celiac Disease research!

# The Ask: Discretionary Spending in FY2020 and \$100 Million in FY2021

Notwithstanding the Herculean efforts by some brilliant, small and under resourced research teams and advocacy groups, the paucity of NIH funding has created a vacuum and stifled innovation in the efforts to treat and cure Celiac Disease.

## **Research Ready Initiatives**

This can change starting in FY 2020 with meaningful NIH funding that validates research ready initiatives at multi-institute translational research centers including, for example, at the:

- Celiac Disease Center at Columbia University
- Harvard Medical School Celiac Research Program
- Children's Hospital of Philadelphia Center for Celiac Disease
- Children's National Hospital
- Stanford
- University of Chicago Celiac Disease Center

**FY 2021 – \$100 Million transformative appropriation to accelerate Celiac research and help improve the lives of the 3 million Americans who suffer from Celiac Disease with developing treatment options, finding a cure and preventing new activations.**

# About the Presenters

Leslie and Jon Bari are the Pennsylvania parents of Jax (6) and Lexi (14). In August 2018, Jax was diagnosed with Celiac Disease.

Up until August 2018, we took our food freedom for granted. We enjoyed our food privilege of being able to eat whatever and wherever we wanted.

We are working to foster greater understanding and inclusion of those who suffer from Celiac Disease. We are also working to drive change in the research ecosystem so that hopefully in the short run, treatment options and a cure can be found, and we can return to our pre-Celiac life.

## **Jon Bari**

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