The Role of Patient Advocacy in Clinical Trials

Marilyn G. Geller
Chief Executive
Celiac Disease Foundation
Since our founding in 1990, the Celiac Disease Foundation has played a key role in achieving federal recognition of celiac disease, improving diagnostic tools, and accelerating research for better treatments and a cure.

**Research**

- iCureCeliac® Patient Registry
- Patient Recruitment Services
- iQualifyCeliac Patient Recruitment Platform
- Patient Recruitment Funds Research Prizes and Grants
Since our founding in 1990, the Celiac Disease Foundation has played a key role in achieving federal recognition of celiac disease, improving diagnostic tools, and accelerating research for better treatments and a cure.

**Education**
- Patients, families, healthcare providers, industry, and the public
- Partnerships for educational programs and provider training with Celiac Disease Centers, AGA, NASPGHAN, Academy of Nutrition and Dietetics, SSCD
- Industry Partnership in Patient Education & Advocacy Summit
Since our founding in 1990, the Celiac Disease Foundation has played a key role in achieving federal recognition of celiac disease, improving diagnostic tools, and accelerating research for better treatments and a cure.

**Advocacy**

- Celiac Disease Caucus, NIH, FDA, CDC, HHS, CDMRP
- Patient Education & Advocacy Summit, State Advocacy Ambassador Training Program
- AOECS (Association of European Coeliac Societies)
iCureCeliac® Patient Registry Makes Finding A Cure Possible

Share your or your child's experience living with celiac disease to advance the development of better treatments and one day a cure for celiac disease.

Join the more than 5,000 individuals and families participating in iCureCeliac, solving celiac disease together.

Join the Patient Registry

Our research goals:

- Increase diagnostic rate of celiac disease
- Identify alternative treatments to the gluten-free diet
- Identify long-term implications of celiac disease
- Find a cure
## Consent Agreement

The iCureCeliac® informed consent statement can be found below. This is the information you are asked to agree to, while registering for iCureCeliac®.

It is necessary for you to provide informed consent before participating in any research. The document below describes iCureCeliac® and what your role will be. Please read carefully to ensure you fully understand the initiative before joining.

If you are reading this form as the parent of a participant, “you” refers to your child.

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<tr>
<th>Name of Research Study</th>
<th>A Celiac Disease Patient-Powered Research Network, iCureCeliac®</th>
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<tr>
<td>Sponsor</td>
<td>Celiac Disease Foundation</td>
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☐ I consent to take part and/or for my child to take part in this research study.

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### Consent Agreement

1. My name is Marilyn Gillett.
2. We are asking you to take part in a research study because we are trying to create a database to learn more about what people with celiac disease and other related diseases think and feel about having it.
3. If you decide to be in this study, your parent may ask, or you may read questions, that appear on the screen. You will tell your parent the answers and they will enter them for you. There are a lot of questions so you can take a break at any time and finish them later.
4. Sometimes things happen in research studies. Some of the bad things that could happen are you may not feel comfortable answering some of the questions. It is okay to not answer questions that make you feel uncomfortable. Even though your answers are given to researchers without your name, it is possible your name may become known due to a security issue. We work very hard to make sure this does not happen. Some of these things might happen to you or they might not. It’s things might happen that we don’t know about yet.
5. People also have good things happen to them when they are in research studies. The good things may be that we will better understand celiac disease and other diseases.
6. Please talk this over with your parents before you decide whether or not to take part in this study. We will also ask your parents to give their permission for you to take part in this study. But even if your parents say “yes” you can still decide not to do this.
7. If you don’t want to be in this study, you don’t have to. You may stop being in this study any time. Remember, being in this study is up to you and no one will be upset if you don’t want to take part in this study or ever if you change your mind later and want to stop.
8. You can ask any questions that you have about the study. If you have a question later that you didn’t think of now, you can have your parent call the Celiac Disease Foundation at 818.795.0512 x01 or email them at icureceliac@icureic.com.

☐ Checking the box on this screen and clicking Create Profile means that you have decided to be in this study and have given your permission.

[Submit Registration] [Create Profile] [Cancel]
Posters, Abstracts, and Publications
Patient Recruitment Services

**STUDY DESIGN CONSULTATION**
Offering guidance on the design of research questions, approach, and strategy from early stages of development through study implementation, we help you define measures and outcomes that are clinically significant and meaningful to patients, address bottlenecks in the enrollment process, and create solutions for common barriers to study participation.

**PATIENT ADVISORY BOARD ENGAGEMENT**
 solicit feedback and gather valuable insights from patients to improve your study feasibility, recruitment, and retention. We offer board strategy development, member selection, and meeting coordination and facilitation.

**STUDY SITE SELECTION**
Leverage our database of geotargeted high-performing sites to create your research network and accelerate enrollment into your studies and trials.

**BRANDING & DESIGN**
The right messaging is key to a successful patient recruitment campaign. We provide proven strategies that resonate with the celiac disease community, including in-house design of your study logo, a customized hosted microsite, and creative assets for your multiplatform marketing campaign.

**MULTIPLATFORM MARKETING CAMPAIGN**
Utilizing our robust website reach at celiac.org, social media channels, and Eat Gluten Free app, we broadcast your study opportunity to those who care about it most. Targeted recruitment to CureCeliac® and iQualifyCeliac participants who meet your subject profile further reduces your cost by identifying the right patients, accelerating enrollment, and improving trial retention and satisfaction.

**QUALIFIED LEAD SCREENING AND IN-HOUSE CALL CENTER**
Branded with your marketing design, our proprietary iQualifyCeliac platform screens patients based upon your study parameters. This, combined with our in-house call center of skilled patient services representatives, produces the highest quality geotargeted-to-site referrals in days—not months.

**STUDY SITE ENGAGEMENT**
We work in tandem with your study site coordinators to ensure a seamless enrollment process. Site coordinators receive virtual training and a secure login to our iQualifyCeliac platform to access pre-screened referrals assigned to their sites. All from their portal, site coordinators can log contact attempts, receive reminders to follow up, schedule first visits, and capture enrollment or randomization status. The sponsor portal allows you to view real-time recruitment statistics and monitor study site contact and enrollment progress.

**PATIENT COMPENSATION**
Through our partnership with Greenphie, we offer Virtual ClinCard management integrated with our iQualifyCeliac platform for seamless patient compensation.

**PATIENT DATA CAPTURE**
Empower patients to capture and submit data on their smartphone or tablet with our user-friendly secure and HIPAA-compliant cloud-based clinical platform. Capture eConsent, eCOA, ePRO, eDiary, symptoms, and wearable device data, customized to your study or trial.

**ICURECELIA**® **PATIENT REGISTRY**
DATA LICENSE
Our online, patient-powered research network, iCureCeliac®, allows patients to share their health data and insights to accelerate research investigating topics important to people with celiac disease. Anonymized data contributed under Informed consent are available to assist you with your study or trial aims.

Survey questions span topics including treatment preferences, quality of life, biomarkers of celiac disease, and many others. Validated instruments include the Celiac Symptoms Index (CSI), Celiac Dietary Adherence Test (CDAT), Celiac Disease Quality of Life Measure (CQOL), SF-36, PROMIS Gastrointestinal, PROMIS 29 Profile, and PROMIS Pediatric 29 Profile.
Multiplatform Marketing Campaign

PROACTIVE CELIAC STUDY

Accidental gluten exposure is inevitable.
When it comes to your celiac disease symptoms and long-term health.
Be PROACTIVE.

Virtual Celiac Symptoms Study

Make an impact, wherever you are.
Celiac research that works with your lifestyle—the virtual study.

Virtual Celiac Symptoms Study

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Virtual Celiac Symptoms Study

Make an impact, wherever you are.
Celiac research that works with your lifestyle—the virtual study.
iQualifyCeliac Study Screening
iQualifyCeliac Study Screening

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### Prospect Screening

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SSCD and CDF Collaborations: Consensus Workshop

ROADMAP

Society for the Study of Celiac Disease position statement on gaps and opportunities in coeliac disease

M. Ines Pinto-Sanchez, Jocelyn A. Silvestro, Benjamin Lebwohl, Daniel A. Leffler, Robert P. Andersson, Amelie Therriault, Ciaran P. Kelly, and Elena F. Verdú

Abstract: Progress has been made in understanding celiac disease, a relatively frequent and underappreciated immune-mediated condition that occurs in genetically predisposed individuals. However, several gaps remain in knowledge related to diagnosis and management. The gluten-free diet, currently the only available management, is not curative or universally effective (some adherent patients have ongoing duodenal injury). Unprecedented numbers of emerging therapies, including some with novel tolerogenic mechanisms, are currently being investigated in clinical trials. In March 2020, the Celiac Disease Foundation and the Society for the Study of Celiac Disease convened a consensus workshop to identify high-yield areas of research that should be prioritized. Workshop participants included leading experts in clinical practice, academia, government, and pharmaceutical development, as well as representatives from patient support groups in North America. This Roadmap summarizes key advances in the field of celiac disease and provides information on important discussions from the consensus approach to address gaps and opportunities related to the pathogenesis, diagnosis, and management of celiac disease. The morbidity of celiac disease is often underestimated, which has led to an unmet need to improve the management of these patients. Expanded research funding is needed as celiac disease is a potentially curable disease.
NIH ADCC Meeting and Accelerating Progress Workshop

Celiac Disease-Focused Autoimmune Disease Coordinating Committee (ADCC) Meeting Agenda
Teleconference/Videocall
May 20, 2020

1:00 PM – 1:10 PM
Welcome and Introductions
Ellen Goldmann, M.D., Ph.D.
Division of Allergy Immunology and Transplantation, NIAID

1:10 PM – 1:25 PM
Updates from Federal Agencies, Private Organizations and Foundations

1:25 PM – 1:40 PM
Unmet Need in Celiac Disease - Patients’ Views
Marilyn Geller, CEO, Celiac Disease Foundation

1:40 PM – 2:05 PM
Basic Research, Unmet Needs and Opportunities
B. Jabri, M.D., Ph.D., Professor of Pediatrics, The University of Chicago

2:05 PM – 2:30 PM
Clinical-Translational Research: Gaps and Opportunities in Prevention and Diagnosis
Joseph A. Murray, M.D., Professor of Medicine, The Mayo Clinic

2:30 PM – 2:55 PM
Non-dietary treatments for celiac disease: Why? What?
Carin P. Kelly, M.D., Professor of Medicine, Harvard Medical School

2:55 PM - 3:00 PM
Wrap up and Discussion of Future Meetings
Ellen Goldmann, M.D., Ph.D.

Accelerating Progress in Celiac Disease Research Workshop

Agenda and Faculty List

Day 1: Thursday, March 18, 2021

9:00 AM – 10:00 AM – Speaker Check-in (Check slides, audio, PowerPoint)

10:00 – 10:05
Meeting Overview and Logistics
NIAID Workshop Organizers

10:05 – 10:10
NIH Workshop Organizers
Dr. Antonio E. Rodriguez (NIDDK)
Dr. Traci Ann Johnson (NIAID)
Dr. Rebecca Gershon (NIDDK)

10:10 – 10:15
Workshop Co-Organizers
Dr. Joseph Murray (Celiac Disease Foundation)
Dr. Albert Faillace (Celiac Disease Foundation)

Session 1: Celiac Disease Genetics
Session Moderator: Dr. Britton Lee (Columbia St. Vincent)
Q&A Moderator: Dr. Antonio E. Rodriguez (NIDDK)

10:15 – 10:30
Dr. Benjamin Levitt (Columbia, NIDDK)

10:30 – 10:45
Dr. Marilyn Geller (Celiac Disease Foundation)

10:45 – 11:00
Dr. Mark Andreason (CCGF)

11:00 – 11:15
Dr. Ronni J. Wilkins (University of Connecticut)
NIH Notice of Special Interest in Celiac Disease and RCDC

### Apply for Research Funds To Accelerate Progress of Celiac Disease Research

**Funding News Edition:** December 15, 2021

See more articles in this edition.

If you are a researcher who can advance our understanding of the etiology and pathogenesis of celiac disease research, apply for funds through the "NIH Notice of Special Interest in Celiac Disease Research" at [NIH's website](#). NIAID participates in the NIDDK's Center for Complementary and Integrative Health (CCIH), National Cancer Institute (NCI), National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS).

**Research Objectives**

Clinical manifestations of celiac disease are multifaceted, and its pathogenesis can involve a combination of predisposing genes, gluten, and environmental factors. While many aspects of the underlying mechanisms are well understood, there remains a urgent need in understanding celiac disease pathogenesis, including a limited understanding of pathways of disease and tissue destruction, and the cause of intolerance loss to a component of food.

This NOB encourages research applications that include the following NIAID relevant research topics:

- Underlying mechanisms of loss of cell tolerance
- Autoimmune triggers in celiac disease
- Strategies that can eliminate and revert the pathogenic memory gluten-specific CD4 memory T cells
- Cellular circuits and mediators involved in tissue destruction
- The role of microcirculation in the loss of oral tolerance and tissue destruction
- The discovery of novel nutritional interventions and strategies to prevent celiac disease and/or restore tolerance
- Biomarkers that predict tissue destruction in celiac disease

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FDA CDER and Patient Advocacy in Drug Development

FOOD AND DRUG ADMINISTRATION (FDA)
Center for Drug Evaluation and Research (CDER)

Gastroenterology Regulatory Endpoints and the Advancement of Therapeutics VI (GREAT VI)
Workshop on Celiac Disease

Virtual meeting
July 22, 2021

AGENDA

The goal of today’s workshop is to discuss the overall approach to drug development in celiac disease that includes an assessment of both clinical symptoms and histology. The workshop will focus the discussion on the histologic endpoints to assess treatment benefit in patients with celiac disease; regulatory framework for pediatric drug development in celiac disease; and the role of gluten challenge in clinical trials to provide a forum for open discussion between stakeholders to facilitate drug development.

10:05 a.m. Panel discussion and Q & A* (40 min)

Panelists: Prista Charanworn, Stephen Lagana, Irena Lavine, Benjamin Lebwohl, Edwin Liu, Marie Robert, Jocelyn Silvester, Kelsey Smith

11:50 a.m. Panel discussion and Q & A* (40 min)

Panelists: Prista Charanworn, Alessio Fasano, Tyler Friedman, Kathy and Beckett Hardin, Mona Khurana, Maureen Leonard, Suna Seo, Christopher St. Clair, Marissa Stahl
Thank you to the SSCD
For more information visit celiac.org/iqualifyceliac

Marilyn G. Geller
Chief Executive
Celiac Disease Foundation

SSCD Clinical Trial Webinar Series • July 27, 2022