Accelerating Celiac Disease Research: With You, We Can Go Further

With Kristin Voorhees, MA, NFCA’s Director of Healthcare Initiatives
and

Jacob Hughey, PhD, Francisco Leon, MD, PhD, and Joseph A. Murray, MD

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Important Reminders!

① Will this information be available at a later date?
   - Yes, always!
   - Webinar recording will be posted along with the webinar slides within 72 hours after the live webinar ends at CeliacCentral.org/webinars/archive/

② Are continuing education credits available?
   - Yes! Continuing education credits will be offered for this webinar
   - A follow-up email containing a link to download the webinar slides will be sent by Friday, October 30th through an email from NFCA
   - To ensure that you receive this email, make sure that NFCA (National_Foundation_for_Celiac_A@gmail.vresp.com) is on your allowed senders list
     – Accidentally unsubscribed? Register for the webinar with a new email address
– Be sure your email address is spelled correctly when registering

Learning Objectives

1) Identify the patient’s role in research
2) Share examples of how patients have contributed to successful celiac disease research
3) Discuss how a motivated and educated patient community can help to advance their field
4) Understand how patients, researchers, and NFCA can all work together

Welcome!
Jacob Hughey, PhD

• Postdoctoral Fellow at the Institute for Computational Health Sciences at the University of California, San Francisco

• Earned his PhD in Bioengineering from Stanford University

• The goal of his research is to use math and computers to improve our understanding of biology and human health

• Diagnosed with celiac disease almost 3 years ago and has a current project that deals with detecting patients with undiagnosed celiac disease using electronic medical records

Welcome!
Francisco Leon, MD, PhD

- Co-Founder, Chief Executive Officer and Chief Medical Officer of Celimmune, a clinical-stage biopharmaceutical company developing medicines for people with celiac disease
- Adjunct Research Associate Professor of Medicine at Jefferson Medical College in Philadelphia
- Received his MD and PhD from Autónoma University in Madrid, Spain
- Has over 20 years of experience in translational immunology
Welcome!

Joseph A. Murray, MD

• Professor of Medicine with the Division of Gastroenterology at Mayo Clinic

• Past president of the North American Society for the Study of Celiac Disease (NASSCD)

• Studied medicine at National University of Ireland and internal medicine/gastroenterology training at Trinity College, Beaumont Hospital and the University of Iowa

• Has a strong focus on immunology, genetics of the disease and immune responses to food and microbiota

• Member of NFCA’s Scientific/Medical Advisory Council
How have patients contributed to successful celiac disease research?

- Clinical trials for different drug options
- Population-based studies
- Intervention trials
- Sharing the patient’s journey
Experimental non-dietary therapies for celiac disease

Alvine: ALV003 – Ph2b (Abbvie)
DSM: AN-PEP – Ph1b
U. Washington: KumaMax - Pre-clinical
Avaxia: Pre-clinical

BiolineRx: BL-7010 – Ph1

Alba: Larazotide –Ph2b

Zedira: ZED1227 – Ph1
Sitari: Pre-clinical

Provid: Pre-clinical

Celimmune: AMG 714 – Ph2a (Amgen)
Mayo Clinic/NCI: HuMik1b – Ph2a
Calypso: CALY 002 – Pre-clinical (Merck KGa)

ImmusanT: NexVax2 – Ph1b
Adelaide Univ: Necator americanus – P2a
Cour: Pre-clinical
Selecta: Pre-clinical (Sanofi Aventis)
Kanyos Bio: Pre-clinical (Astellas/Anokion)

Schuppan Gastroenterology 2009, modified
Source: Francisco Leon, Celimmune
What is the patient’s role in research?

Healthy controls & at-risk family members

Population surveys

Developing new treatments

Screening of high-risk individuals

Sharing disease history

Public advocacy

Clinical trials
How can a motivated and educated patient community help to advance the field?

Self-managed condition

Constant vigilance

Many are undiagnosed or misdiagnosed

The real challenges of the gluten-free diet
Top 5 Needs of NFCA’s Community

1. Dining outside of the home
2. Traveling without concern about safe gluten-free options and availability of them
3. New treatment options that can replace the gluten-free diet
4. Participating in social activity without concern and/or seeming needy
5. Availability of (and access to) healthcare providers and registered dietitians who are knowledgeable and can manage all aspects of celiac disease

• How can researchers address these needs alongside patients?

• What other needs do we know exist?
What type of participation is needed from patients in research and how can everyone get involved?

Amplify your voice!

Connecting patients and researchers

Patient experience
A Path Forward

- NFCA’s hope is that we can overcome the day-to-day challenges that people face, move beyond celiac disease and build a path forward to finding a cure by putting the patient at the center of celiac disease research.
• Learn how we’re doing this by watching our short video “A Path Forward” that highlights NFCA’s 2015 Research Summit

• YouTube: Search “nfaceliaccentral”
Sign Up to Receive Research News and Announcements!

As celiac disease research advances, NFCA wants to ensure that our community remains up to date and aware of new and ongoing research opportunities. You can do so by signing up for “Beyond Celiac™: NFCA's Research Opt-In.”

How often do you want to receive research news updates?

Pick Your Preference!
Research Opportunity:
The Mayo Clinic is seeking individuals diagnosed with Refractory Celiac Disease

• Currently, there are no established or effective treatments for RCD.

• The goal of the study is to assess the administration of a monoclonal antibody (HUMik-β1) for the treatment of Refractory Celiac Disease (RCD). The drug targets the effects of a signal (IL-15) in the body that is believed to cause much of the damage in the intestine. It is hoped that this drug will lessen the damage in your intestine and/or lessen the severity of RCD.

• The study involves multiple trips to Mayo Clinic in Rochester, Minnesota. Travel and lodging expenses qualify for reimbursement for required visits.
As we finish...

Questions from the audience?

If you are interested or want more information, please contact: Carol Van Dyke by telephone at 507-266-7842 or email Vandyke.carol@mayo.edu
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