# 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.*

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| 9:00 a.m. | **Opening Remarks** | **Suna Seo** *(5 min)* |
| | Considerations for drug development in celiac disease: FDA perspective | *(15 min)* |
| | *Speaker: Irena Lavine* | |
| 9:20 a.m. | **Session 1: Histologic assessment in the evaluation of the underlying disease and treatment benefit in celiac disease** | |
| | ***Moderators: Suna Seo and Dawn Adams*** | |
| | Approach to monitoring disease through histologic assessment in clinical practice | *(15 min)* |
| | *Speaker: Benjamin Lebwohl* | |
| | Unique considerations for using histologic assessments to monitor disease in pediatric patients | *(10 min)* |
| | *Speaker: Jocelyn Silvester* | |

Histologic characteristics to define disease severity and remission: a pathologist's perspective *(10 min)*

*Speaker: Marie Robert*

9:55 a.m. Break *(10 min)*

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

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

10:45 a.m. **Session 2: Pediatric celiac disease**

***Moderators: Lynne Yao and Ritu Verma***

Extrapolation of efficacy: Regulatory considerations *(10 min)*

*Speaker: Mona Khurana*

Pediatric patient's perspective on living with celiac disease and goals of treatment *(10 min)*

*Speaker: Tyler Friedman*

Clinical manifestations, natural history, and unmet needs of pediatric celiac disease *(15 min)*

*Speaker: Maureen Leonard*

FDA perspective: Defining clinical benefit in pediatric clinical trials for celiac disease *(15 min)*

*Speaker: Christopher St. Clair*

11:35 a.m. Break *(15 min)*

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

*Panelists: Prista Charuworn, Alessio Fasano, Tyler Friedman, Kathy and Beckett Hardin, Mona Khurana, Maureen Leonard, Suna Seo, Christopher St. Clair, Marisa Stahl*

12:30 a.m. **Lunch** (*60 min)*

1:30 p.m. **Session 3: Gluten challenge in clinical trials**

***Moderators: Juli Tomaino and Amanda Cartee***

FDA introductory remarks for the session *(5 min)*

*Speaker: Juli Tomaino*

Gluten challenges and unintentional gluten exposure in clinical practice *(10 min)*

*Speaker: Joseph Murray*

Dose and duration of gluten exposure that elicits clinical signs/symptoms and changes in histology in patients *(10 min)*

*Speaker: Jason Tye-Din*

Role of gluten challenge in clinical trials: Industry perspective *(10 min)*

*Speaker: Daniel Leffler*

2:05 p.m. Break *(15 min)*

2:20 p.m. Panel discussion and Q & A* *(45 min)*

*Panelists: Irena Lavine, Benjamin Lebwohl, Dale Lee, Daniel Leffler, Francisco Leon, Joseph Murray, Kelsey Smith, Jason Tye-Din*

3:05 p.m. **Closing Remarks** **Irena Lavine** *(5 min)*

3:10 p.m. **Adjournment**

***To facilitate discussion, please submit all questions for the panel prior to the session break**