FOOD AND DRUG ADMINISTRATION (FDA)

Center for Drug Evaluation and Research (CDER)

Gastroenterology Regulatory Endpoints and the Advancement of Therapeutics 3 (GREAT 3)
Workshop on Celiac Disease

FDA White Oak Campus, Building 31, the Great Room
White Oak Conference Center, Silver Spring, Maryland
March 31, 2015

AGENDA

The goal of today's workshop is to discuss the appropriate target population for pharmacological therapy in celiac disease, and the definition and measurement of efficacy in celiac disease clinical trials intended to support marketing approval, including the role and timing of assessment of specific endpoints.

8:00 a.m.	Opening Remarks	**Donna Griebel/Andrew Mulberg**
8:10 a.m.	**Session 1: Defining target patient population(s) for pharmacological therapies**	**<u>Moderators:</u> Sheila Crowe + Andrew Mulberg**
	Current management of celiac disease and identifying an appropriate patient population(s) for pharmacologic therapies in adult patients *(15 min)*	**Joseph Murray**
	Current management of celiac disease and identifying an appropriate patient population(s) for pharmacologic therapies in pediatric patients *(15 min)*	**Alessio Fasano**
8:40 a.m.	**Panel Discussion** *(40 min)*	**<u>Panel:</u> Joe Murray, Alessio Fasano, Sonia Kupfer, Alice Bast, Juli Tomaino, George Dukes, Ritu Verma**
9:20 a.m.	**Public Q & A** *(30 min)*	
9:50 a.m.	**Break** *(20 min)*	
10:10 a.m.	**Session 2: Defining clinical benefit in celiac disease trials intended to support marketing approval**	**<u>Moderators:</u> Sonia Kupfer + Juli Tomaino**

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Patient's perspective on a clinically meaningful benefit	**Alice Bast**
(10 min)	

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	Clinician's perspective on a clinically meaningful benefit in adult patients. *(10 min)*	**Sheila Crowe**
	Clinician's perspective on a clinically meaningful benefit in pediatric patients. *(10 min)*	**Ivor Hill**
	Defining clinical benefit in clinical trials: FDA perspective *(15 min)*	**Jessica Lee**
10:55 a.m.	**Panel Discussion** *(40 min)*	**<u>Panel:</u> Sheila Crowe, Anthony DiMarino, Alice Bast, Ivor Hill, Stefano Guandalini, Ciaran Kelly, Donna Griebel, Jessica Lee, George Dukes**
11:35 a.m.	**Public Q & A** *(35 min)*	
12:10 p.m.	**Lunch** *(60 min)*	
1:10 p.m.	**Session 3: Measuring clinical benefit in celiac disease trials intended to support marketing approval**	**<u>Moderators:</u> Peter Green + Jessica Lee**
	Clinical outcome assessments to demonstrate clinical benefit *(20 min)*	**Elektra Papadopoulos**
	Role of histology to measure clinical benefit and appropriate timing of assessment(s) *(20 min)*	**Benjamin Lebwohl**
	Role of serology to measure clinical benefit and appropriate timing of	**Daniel Leffler**

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3:25 p.m.	**Break** *(15 min)*	
3:40 p.m.	**Public Q & A** *(40 min)*	
4:20 p.m.	**Closing Comments**	**Juli Tomaino**
4:30 p.m.	**Adjournment**	