Celiac Disease - FDA-requested Listening Session
February 20, 2019

FDA held a 1.5 hour Listening Session with celiac disease patients and caregivers on February 20th, 2019 to better understand the celiac patient perspective.

Patient Listening Sessions are intended to be a resource for the medical product Centers to expeditiously engage with patients or their advocates. Listening Sessions can either be FDA-requested (in cases where FDA has a specific set of questions to ask of a particular patient sub-population) or patient-led (when a patient community wants to share perspectives with the FDA), and are generally focused on conditions of interest to staff across the medical product Centers. This listening session was requested by the Center for Drug Evaluation and Research (CDER), Division of Gastroenterology and Inborn Errors Products (DGIEP), and had participation from others in CDER, the Center for Biologics Evaluation and Research (CBER), and the Office of the Commissioner (OC).

Discussions in FDA Listening Sessions are informal and not meant to replace, but rather compliment, existing patient engagement opportunities in the Agency. All opinions, recommendations, and proposals are unofficial and nonbinding on FDA and all other participants. This report summarizes the input provided by patients and patient representatives at the meeting. To the extent possible, the terms used in this summary to describe specific manifestations of celiac disease, and the health effects and impacts, reflect those of the participants. This report is not meant to be representative of the views and experiences of the entire celiac disease patient population or any specific group of individuals or entities. There may be experiences that are not mentioned in this report.

Summary of topics discussed

1. **Impactful symptoms: The celiac patients and caregivers described a variety of symptoms that impact their daily life:**
   - Gastrointestinal (GI) symptoms were the most common symptoms mentioned by patients/caregivers and included gas, bloating, diarrhea, cramping, and vomiting. The severity, time to onset, and duration of the symptoms varied per individual..
   - Patients also experience non-GI symptoms after gluten ingestion, including aching sensations in the arms and legs, rashes, anxiety, and cognitive impairment. Patients shared that it can sometimes be hard to differentiate whether these symptoms are due to celiac disease or other (non-celiac) conditions with overlapping symptoms.
   - Patients have found it difficult to participate in social activities that involve food and beverages because of the risk of accidental ingestion of gluten or cross-contamination in food preparation. Patients will often pack their own food when eating outside of their homes, but this can be challenging with travel. Patients cannot always trust gluten-free options at restaurants.

2. **Treatment for celiac disease: The celiac patients described their perspective about potential treatments for celiac disease:**
   - Patients were generally open to the idea of a treatment for accidental exposure to gluten such as cross-contamination in food, although there was hesitancy. If such a treatment was available, the patients indicated they would continue to maintain a strict gluten-free diet.
   - Patients were generally not open to the idea of a treatment intended to be taken regularly that does not promote healing of the underlying disease. Patients would rather maintain a gluten-free diet than rely on a medication. However, if a treatment did promote disease healing, some patients would be receptive to it.

- Patients generally felt that strictly maintaining a gluten-free diet is the best method of preventing celiac disease symptoms.
- The patients typically had undergone at least one endoscopy with biopsy, which was often but not always performed when they were diagnosed. For patients who had more than one endoscopy, they found it helpful to see progress in healing of the villi and confirmation that they are adhering to a gluten-free diet.
- Some patients would be willing to undergo more endoscopies for research purposes while maintaining their gluten-free diet; however, patients generally expressed that they were not willing to ingest gluten for the purpose of a clinical trial.

3. **<u>Patient/caregiver concerns:</u> The celiac disease patients and caregivers described several concerns regarding their disease:**
    - Many patients and caregivers shared the concern about other autoimmune diseases that might be associated with celiac disease. Some worry that they are predisposed or at higher risk for developing other diseases such as diabetes.
    - There is concern about whether celiac disease is affecting other parts of the body besides the GI tract. Some patients expressed concern about the potential of passing on a gene (and celiac disease) to the next generation.
    - There is a need for more education about celiac disease. Many online sources contain incorrect information, such as suggesting that celiac patients can reintroduce gluten into their diets. More accurate and trusted sources are needed with an emphasis on standardization. Some patients were concerned that many people don’t understand the disease or take it seriously, which can impact a patient’s ability to eat out or attend social events.

FDA centers and divisions represented

- Office of the Commissioner, Patient Affairs Staff (organizer)
- Center for Drug Evaluation and Research (CDER), Division of Gastroenterology and Inborn Errors Products (DGIEP)
- Center for Drug Evaluation and Research (CDER)
- Center for Biologics Evaluation and Research (CBER)
- Office of the Commissioner (OC)

Patients represented

7 patients and caregivers including:

- Segment of patients diagnosed as young children
- Segment of patients diagnosed as adults

Conflict of Interest

- More than one participant indicated that they had a conflict of interest; however, these individuals are not receiving compensation for this listening session.