amyloidosis research consortium

June 7, 2016

Dr. Janet Woodcock
Center for Drug Evaluation and Research
Food and Drug Administration
10903 New Hampshire Ave
Silver Spring, MD 20993-0002

Re: Draft "Voice of the Patient" Report Submission

Dear Dr. Woodcock and Colleagues at the FDA,

This correspondence constitutes the formal submission of a draft "Voice of the Patient" report for amyloidosis arising from an externally-led Patient-Focused Drug Development (PFDD) meeting. This report was generated by the Amyloidosis Research Consortium. The effort to create this report is consistent with the FDA's PFDD Initiative.

On November 16, 2015, the Amyloidosis Research Consortium, had the opportunity to host a PFDD meeting in Silver Spring, MD with participation from the FDA. The meeting was organized as a result of teleconference with Dr. Woodcock on April 21, 2016. Recognizing the devastating effects of amyloidosis, the complexity of these diseases, and the benefit of having a greater understanding of the experiences of patients to support the development and evaluation of novel agents, it was agreed that it would be of great value for the Amyloidosis Research Consortium to take the lead and organize a public meeting.

There were over 240 participants at this meeting, including patients, caregivers, and patient advocates. Participants represented a broad spectrum of experiences across the disease. They described the symptoms that matter most, the impact the disease has on their daily lives, and the patient experience with currently available treatments. FDA staff present included members of FDA's Divisions of Cardiovascular and Renal, Neurology, and Hematology Products.

This submission summarizes the input provided by patients and caregivers at this meeting. In addition, individually written patient testimony is included as an addendum to the meeting summary, representing many patients that were not able to attend the meeting. We request that the FDA review the document and reissue it as part of the PFDD initiative. A recording of the meeting is available at http://www.arci.org/advocacy/

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We believe that this report will serve an important function in providing insight to the FDA and industry regarding the severe burden and critical unmet medical needs that exist across the spectrum of amyloidosis diseases. It contributes additional context on what benefits amyloidosis patients want most from future therapies, the risks they are willing to take for potential treatment benefit, as well as an informed understanding of the patient experience in order to support the development of new treatments.

We look forward to your response and would welcome any questions that you may have regarding this submission. We feel that PFDD is an important initiative and the Amyloidosis Research Consortium is very grateful for the FDA's continued responsiveness and support in this effort.

Yours sincerely,

Isabelle Lousada
President & CEO
Amyloidosis Research Consortium, Inc.
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FDA attendees included:
Dr. Janet Woodcock, Director, CDER
Dr. Jonathan Goldsmith, Associate Director for Rare Diseases, OND, CDER
Dr. Preston Dunnmon, Medical Office, Division of Cardiovascular and Renal Products, OND, CDER
Dr. Devanand Jillapalli, Medical Officer, Office of Orphan Products Development, OC
Dr. Ann Farrell, Director, Division of Hematology Products, OHOP, OND, CDER
Dr. Rich Moscicki, Deputy Director for Science Operations, CDER
Dr. Ronald Farkas, Clinical Team Leader, Division of Neurology Products, OND, CDER

Submission:
Draft Voice of the Patient Report for Amyloidosis
Submitted Stories for AL amyloidosis
Submitted Stories for ATTR amyloidosis