December 5, 2014

Janet Woodcock, M.D.
Director
Center for Drug Evaluation and Research
U.S. Food and Drug Administration
Division of Dockets Management (HFA-305)
5630 Fishers Lane, RM. 1061
Rockville, MD 20852

RE: Docket No. FDA- 2012-N-0967 Prescription Drug User Fee Act Patient-Focused Drug Development; Request for Comments

Dear Dr. Woodcock,

The Aging in Motion (AIM) Coalition is a group of patient, caregiver, health and aging organizations pressing for greater awareness, regulatory consideration, and improved treatment of sarcopenia. On behalf of AIM, we would like to thank you for the opportunity to comment on CDER’s preliminary list of disease areas for patient-focused drug development meetings during fiscal years (FYs) 2016-2017. The patient-focused drug development meetings held to date covered aspects of disease that are most important to those living with diseases like Narcolepsy, Fibromyalgia and Lung Cancer. These meetings resulted in valuable publicly-available resources written in the voice of patients to help inform new endpoint development, outcome measure selection in clinical trials, and benefit/risk decision making by the FDA. We thank you for including sarcopenia on the list of nominated diseases and ask that you prioritize a meeting on sarcopenia early in FY 2016 so that current and future trials for sarcopenia could benefit from such useful resources.

Sarcopenia as a specific condition was initially identified in 1989. Originally, it referred to the loss of muscle mass that occurs with age, and was seen as a characteristic state almost universal with aging. Over time however, clinical perspectives on sarcopenia have evolved. Particularly because loss of muscle mass by itself does not necessarily correlate with weakness or functional impairment, sarcopenia has now come to be defined as a clinically significant disorder based on distinct findings and functional issues. It leaves millions of older adults vulnerable to falls and fractures, hospitalization, loss of mobility, frailty, institutionalization, inability to recover from injury, and even death. It is estimated that sarcopenia costs as much as $18 billion in annual healthcare expenses. Though sarcopenia affects a significant number of elderly people and is a contributor to increased cost of care, it is not currently recognized as a distinct diagnosis and there is no clear regulatory pathway for this condition.

To help eliminate these barriers to the treatment of sarcopenia, the AIM Coalition focuses on several areas. AIM is in the process of establishing an ICD-10 diagnosis code for sarcopenia through the Centers for Disease Control and Prevention and the Centers for Medicare and Medicaid Services. This code is defined by areas of consensus developed through the Foundation for the National Institutes of Health Sarcopenia Project, the European Working Group on Sarcopenia in Older Persons, and other national and international groups. Once established, this code will be available for use in providing a recognized diagnosis for the condition.

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While there are some interventions such as nutritional counseling, occupational therapy, and physical therapy that can improve muscle strength, no pharmacologic treatments are approved for use in the treatment of sarcopenia. To help expand the availability of new treatment options for patients with sarcopenia, AIM is pursuing qualification of functional measures through FDA’s Drug Development Tool Qualification Process that we believe will be useful as endpoints in clinical trials for this condition. In addition to pursuing formal qualification of functional measures, AIM facilitates ongoing interactions among patient advocates, clinicians, regulators, members of the academic community and industry to overcome other obstacles that impede the development of treatments for sarcopenia.

In March of this year, AIM held its first meeting on patient reported outcome (PRO) measures for sarcopenia. We felt that this topic was important to pursue in order to inform the development of specific PROs for use in clinical trials for sarcopenia. The proposal made at this meeting was that a good PRO measure for sarcopenia should, among other factors, evaluate the impact of muscle wasting and loss of strength on a person’s life; represent a single impact rather than a multidimensional concept; be relevant to most people with sarcopenia most of the time; be easily understood; and measure a concept likely to change with successful treatment of the condition. A robust measure like this does not currently exist but a structured patient-focused drug development meeting where patients share their experiences on aspects of everyday life with sarcopenia, challenges with carrying out activities of daily living, and issues related to difficulty in functioning would provide important context to those who could develop such a PRO measure or are working to adapt existing measures for use in clinical trials. Two additional areas of need identified during the March meeting were the need to define the target population for intervention by a common set of symptoms or defining features and the need to better understand notable gender differences in age-related muscle and strength loss. These are also critical insights that we believe could be elicited in a patient-focused drug development meeting on sarcopenia.

If sarcopenia is selected for a patient-focused drug development meeting in FY 2016, AIM and other stakeholders in the aging community stand ready to provide input into the topics of discussion, to help disseminate information on the meeting when it is scheduled, and to facilitate the participation of patients and their caregivers. Thank you again for the opportunity to comment and for your careful consideration of the views expressed above. If we can be of assistance to the center as it contemplates its final list of diseases for FY 2016-2017, please contact us at (202) 293-2856.

Sincerely,

Daniel Perry
Chairman

Cynthia Bens
Vice President, Public Policy

CC: Theresa Mullin, Ph.D., Director, Office of Strategic Programs
    Richard Klein, Director, Office of Health and Constituent Affairs
    Jean-Marc Guettier, M.D. Director, Division of Metabolism and Endocrinology Products