Public Meeting on Patient-Focused Drug Development for Sarcopenia*

*Muscle loss and weakness associated with aging
Welcome

Meghana Chalasani
Office of Strategic Programs
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

April 6, 2017
Agenda

• Opening Remarks

• Setting the context
  – Background on sarcopenia
  – Background on the meeting format

• Discussion

• Open Public Comment

• Closing Remarks
Opening Remarks

Jean-Marc Guettier, M.D.
Director, Division of Metabolism and Endocrinology Products
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

April 6, 2017
Sarcopenia: Overview and Current Treatment Options

John Sharretts, M.D.
Division of Metabolism and Endocrinology Products
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

April 6, 2017
What is sarcopenia?

- **Sarcopenia** is a word derived from Greek
  - It literally means a lack of muscle or flesh
  - As a medical term, it is a term used to describe the loss of muscle mass, strength, and function associated with aging

- At this time, there is no widely agreed upon definition of sarcopenia, or agreed upon way to diagnose it

- Sarcopenia affects 1% to 33% of people over age 60
  - These are the best current estimates by public health researchers
How is sarcopenia measured?

• Muscle mass or lean body mass
  – Body scans or imaging
  – Blood tests

• Muscle strength
  – Grip strength
  – Knee extension

• Muscle function
  – Walking speed
  – Rising from chair
  – Stair climbing
What causes sarcopenia?

• The cause of sarcopenia is unknown

• Risk factors include:
  – Lack of exercise
  – Decreased muscle growth
  – Changes in nerve supply to muscles
  – Other illnesses: cancer, heart disease
  – Other/unknown factors
Why is sarcopenia important?

• Muscle weakness is a risk factor for falls
  – Falls cause fractures and other serious injuries in older adults

• Loss of muscle strength and function may lead to:
  – Disability
  – Loss of independence (inability to care for self)

• One type of decreased muscle function—slow walking speed—is associated with a higher risk of dying, especially in people older than 75 years of age
  – Walking speed also depends on factors other than muscle
  – It is important to remember that an association between two conditions does not necessarily mean that one causes the other
Treatment options for sarcopenia

• Exercise
  – Best studied and most effective treatment
  – Improves muscle strength and function
  – Effects on muscle mass are inconsistent

• Nutritional supplementation
  – Effects on muscle in research studies are inconsistent
  – May depend on the type of supplement and specific deficiencies of individual patients

• There are no medications approved by the FDA for the treatment of sarcopenia
Why is it important to hear from people who may have sarcopenia?

• Information from these sessions may help doctors and researchers to:
  – Understand how the condition impacts the lives of patients
  – Understand what patients most want in therapies which aim to improve the condition

• Patient information helps
  – Identify concepts that are important to patients living with the condition
  – Develop instruments that can be used to measure how patients with the condition feel or function
  – Develop instruments to measure the treatment benefit of therapies aiming to improve the condition
What is FDA’s Patient-Focused Drug Development Initiative?

- FDA is developing a more systematic way of gathering patient perspective on their condition and available treatment options
  - Patient perspective helps inform our understanding of the context for assessing the benefits and risks of potential new drugs
  - It can inform FDA’s oversight both during drug development and during our review of a marketing application

- FDA is holding 24 Patient-Focused Drug Development meetings in 2013-2017
  - This is part of a commitment under the Prescription Drug User Fee Act
  - These meetings are helping us develop approaches to gathering patient input

- Each meeting is focused on a specific disease or disease area
  - After each meeting, FDA publishes a Voice of the Patient report that summarizes what we have heard from these meetings
  - These reports provide information that can be useful to FDA staff, researchers, drug developers and others
What are we discussing today?

**Topic 1: How sarcopenia affects your daily life**
- Which symptoms related to muscle loss and weakness are most bothersome to you?
- How do these symptoms affect your ability to do specific activities?
- How have your symptoms changed over time?
- What worries you the most about your muscle loss and weakness?

**Topic 2: Treatments for sarcopenia**
- What are you doing to manage muscle loss and weakness?
- How well are these treatments addressing your muscle loss and weakness?
- What would you consider to be a meaningful improvement in muscle strength or muscle function?
What will our discussion look like?

• **We will first hear from a panel of seniors and representatives**
  – The purpose is to set a good foundation for our discussion
  – They reflect a range of experiences with sarcopenia

• **We will then broaden the dialogue to include all seniors in the audience**
  – The purpose is to build on the experiences shared by the panel
  – We will ask questions and invite you to raise your hand to respond
  – Please state your name before answering
Discussion Format, continued

• You’ll have a chance to answer “polling” questions
  – Their purpose is to aid our discussion
  – In-person participants, use the “clickers” to respond
  – Web participants, answer the questions through the webcast
  – Seniors or a family member only, please

• Web participants can add comments through the webcast
  – Although they may not all be read or summarized today, your comments will be incorporated into our summary report
  – We’ll occasionally go to the phones to give you another opportunity to contribute
Discussion Ground Rules

• We encourage seniors to contribute to the dialogue
  – Family, healthcare providers and advocates are welcome as well
  – Open Public Comment is available for others who want to comment

• FDA is here to listen

• The views expressed today are personal opinions

• Respect for one another is paramount

• Let us know how the meeting went today; evaluation forms are available on the tables
Send us your comments!

- You can send us comments through the “public docket”
  - The docket will be open until June 6, 2017
  - Share your experience, or expand upon something discussed today
  - Comments will be incorporated into our summary report
  - Anyone is welcome to comment


Or Search “sarcopenia patient-focused drug development” on www.regulations.gov

And Click Comment Now!
Have you ever consulted a healthcare professional about age related loss of muscle mass, strength, or function (sarcopenia)?

1. Yes
2. No
What is your age?

A. Younger than 40
B. 40 – 49
C. 50 – 59
D. 60 – 69
E. 70 – 79
F. 80 or greater
Are you:

A. Male
B. Female
Discussion Topic 1 & 2
Setting the Context on Patients Experiences and Perspectives

Meghana Chalasani and Sara Eggers
Facilitators

April 6, 2017
Panel Participants

• Greta Dershimer
• Fred Bartlit
• Rose Clifford
• Ray Lipicky
Do you currently have any of the following conditions?  
Check all that apply.

A. Arthritis or Osteoporosis
B. Cancer
C. Cardiovascular disease (CVD)
D. Kidney disease
E. Lung disease (such as COPD)
F. Neurological conditions (such as stroke, cognitive impairment)
G. Psychiatric conditions (such as anxiety, depression)
H. Other comorbid condition(s)
I. I do not have any comorbid conditions that I am aware of
Which health effects are most bothersome to you?
Please choose up to three health effects.

A. Pain
B. Fatigue or lack of energy
C. Poor balance
D. Difficulty walking
E. Reduced muscle strength or increase muscle weakness
F. Depression
G. Other symptoms not mentioned
How does sarcopenia affect your life the most?
Please choose up to three impacts.

A. Ability to perform work or hobbies
B. Ability to care for myself independently
C. Ability to leave the home
D. Risks to safety of self or others
E. Impact on relationships with family and friends
F. Emotional impacts
G. Other impacts not mentioned
Have you ever used any of the following therapies to treat your sarcopenia? Check all that apply.

A. Exercise routine
B. Physical therapy, massage, or acupuncture
C. Dietary and herbal supplements
D. Over-the-counter products
E. Experimental therapies (such as part of a clinical trial)
F. Other prescription medicine (such as pain medication)
G. Other therapies not mentioned
H. I am not doing or taking any therapies to treat my symptoms
When considering a new treatment for sarcopenia, which ONE of the following benefits would you consider to be most meaningful? Please choose one.

A. Reduced pain
B. Reduced fatigue or lack of energy
C. Improved balance
D. Increased endurance during physical activity
E. Increased mobility (such as walking across a room, getting out of a chair)
F. Improved muscle strength or reduced muscle weakness
G. Other
A Hypothetical Treatment

- FDA has approved a new monthly injectable medication to treat sarcopenia.
- Your doctor believes that you may be a good candidate for this medication.
- In the clinical studies, one-half of older adults taking this medication achieved a 20% increase in walking speed within three months.
- Common side effects include fatigue, headaches and weight gain.
- The medication is also believed to cause rare, but serious side effects, such as liver problems and cancer.

What first thoughts come to mind as you hear this scenario? What questions would you ask your doctor about this treatment?
Open Public Comment

Pujita Vaidya, MPH
Office of Strategic Programs
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

April 6, 2017
Closing Remarks

Jean-Marc Guettier, M.D.
Director, Division of Metabolism and Endocrinology Products
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

April 6, 2017