

# Public Meeting on Patient-Focused Drug Development for Sarcopenia\*

\*Muscle loss and weakness associated with aging

# Welcome

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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

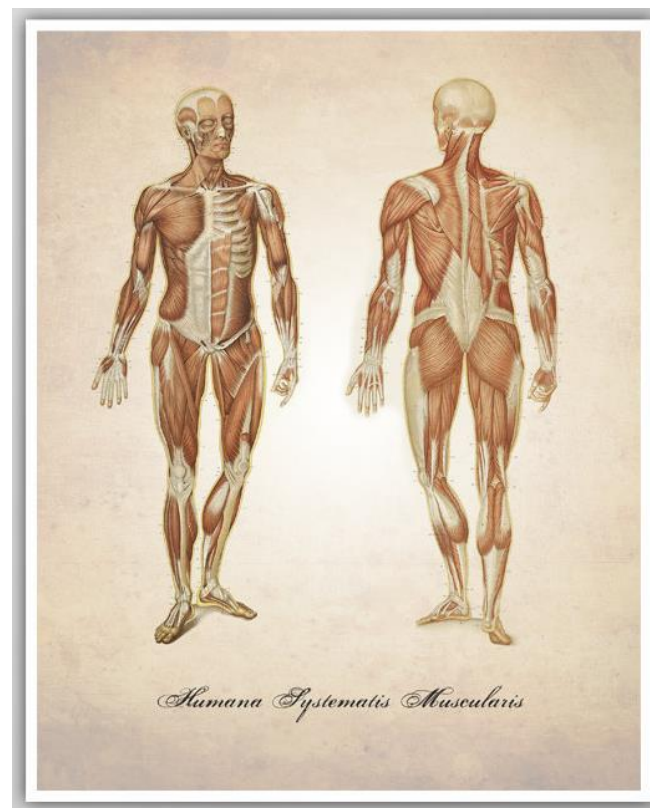
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# What is sarcopenia?

- **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

# Overview of FDA's Patient-Focused Drug Development Meeting

Sara Eggers, PhD

Office of Strategic Programs

Center for Drug Evaluation and Research

U.S. Food and Drug Administration

April 6, 2017

# 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

Visit:

<https://www.regulations.gov/document?D=FDA-2016-N-4198-0001>

Or Search “sarcopenia patient-focused drug development” on [www.regulations.gov](http://www.regulations.gov)

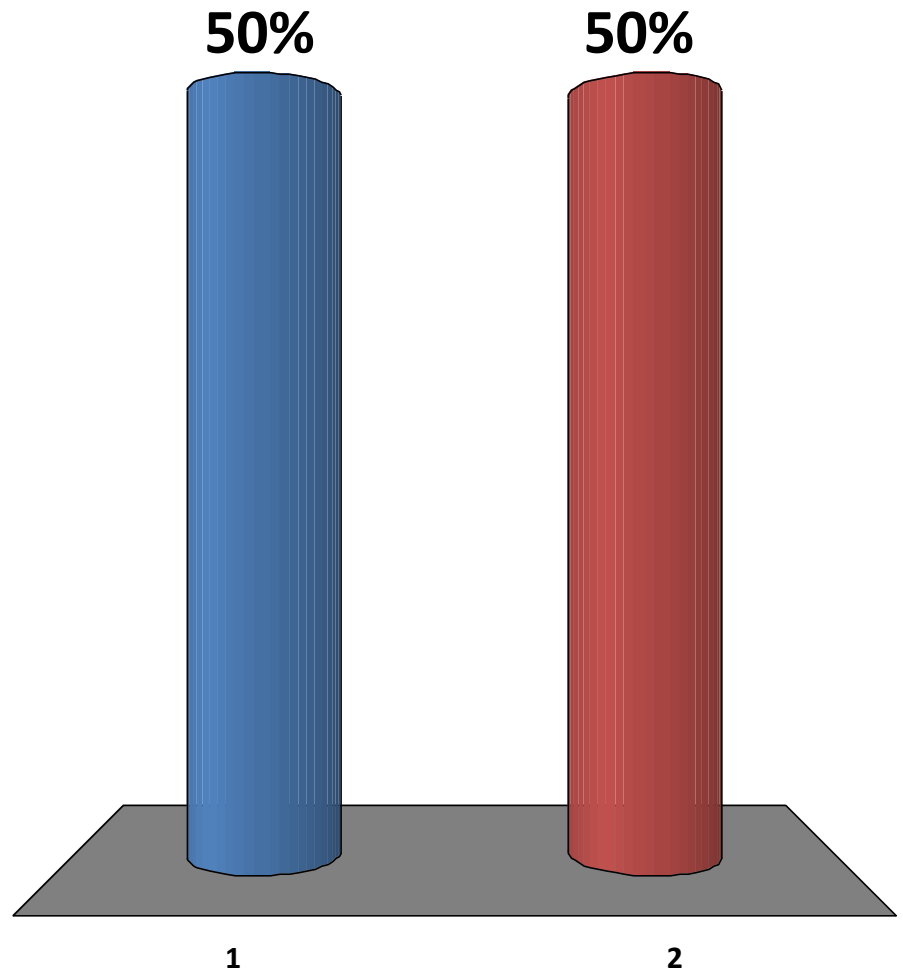
**And Click Comment Now!**



The screenshot shows the regulations.gov website interface. At the top, there is a search bar with the text 'sarcopenia' and a search icon. Below the search bar, the page title is 'Public Meeting on Patient-Focused Drug Development for Sarcopenia; Request for Comments'. The main content area includes a notice from the Food and Drug Administration (FDA) regarding a public meeting and an opportunity for public comment. A red arrow points from the text 'And Click Comment Now!' to a blue button labeled 'Comment Now!' in the top right corner of the notice. Below the button, there is a 'Document Information' section with details such as 'Date Posted: Dec 14, 2016' and 'Federal Register Number: 2016-28998'.

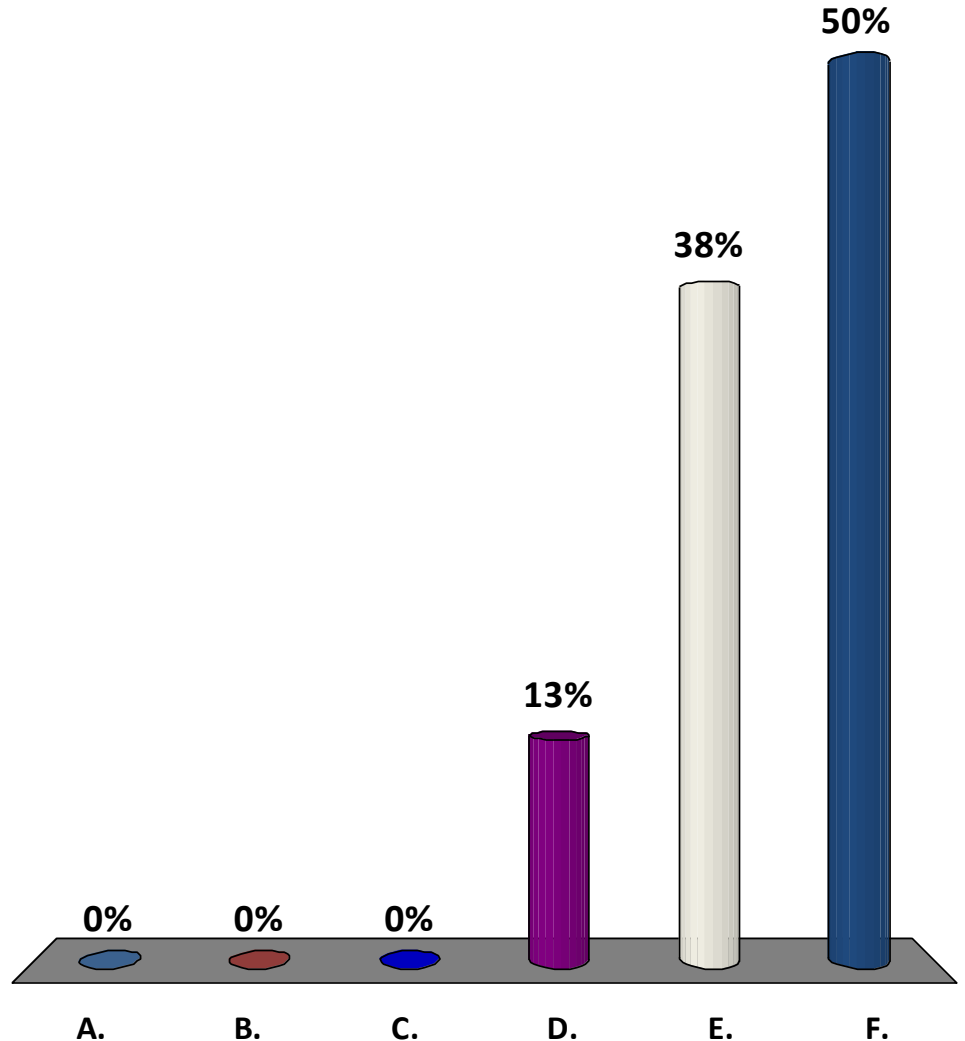
# Have you ever consulted a healthcare professional about age related loss of muscle mass, strength, or function (sarcopenia)?

1. Yes
2. No



# What is your age?

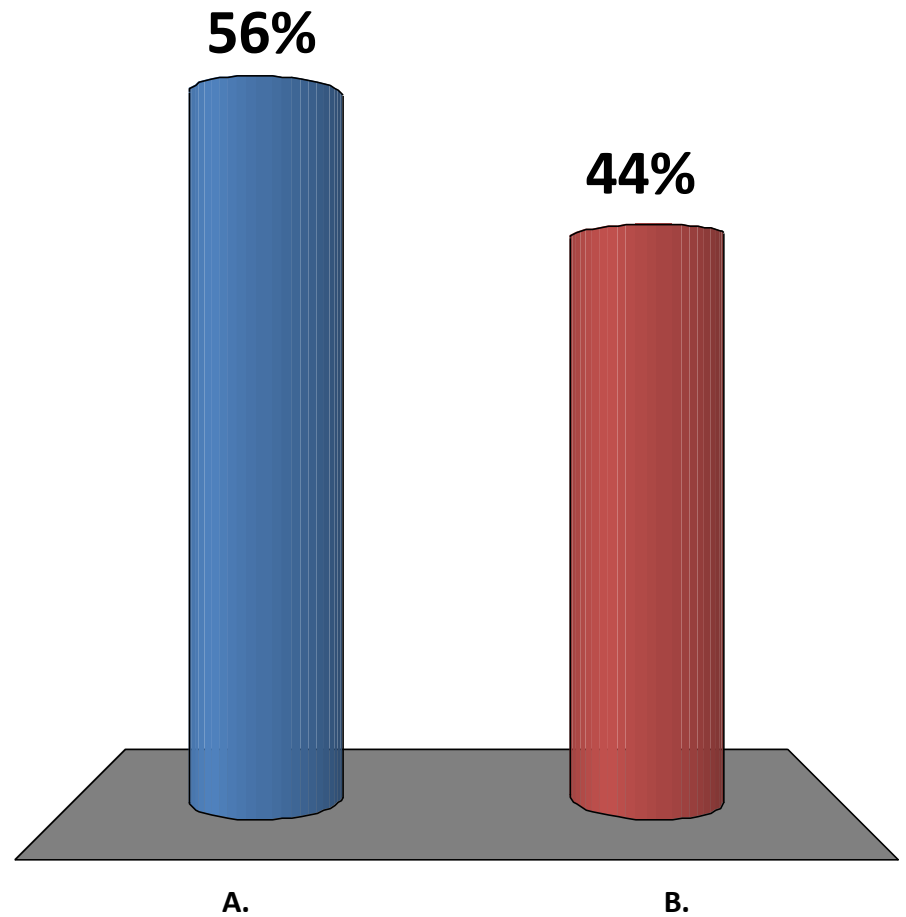
- A. Younger than 40
- B. 40 – 49
- C. 50 – 59
- D. 60 – 69
- E. 70 – 79
- F. 80 or greater



# Are you:

A. Male

B. Female



# Discussion Topic 1 & 2

## Setting the Context on Patients Experiences and Perspectives

Meghana Chalasani and Sara Eggers  
Facilitators

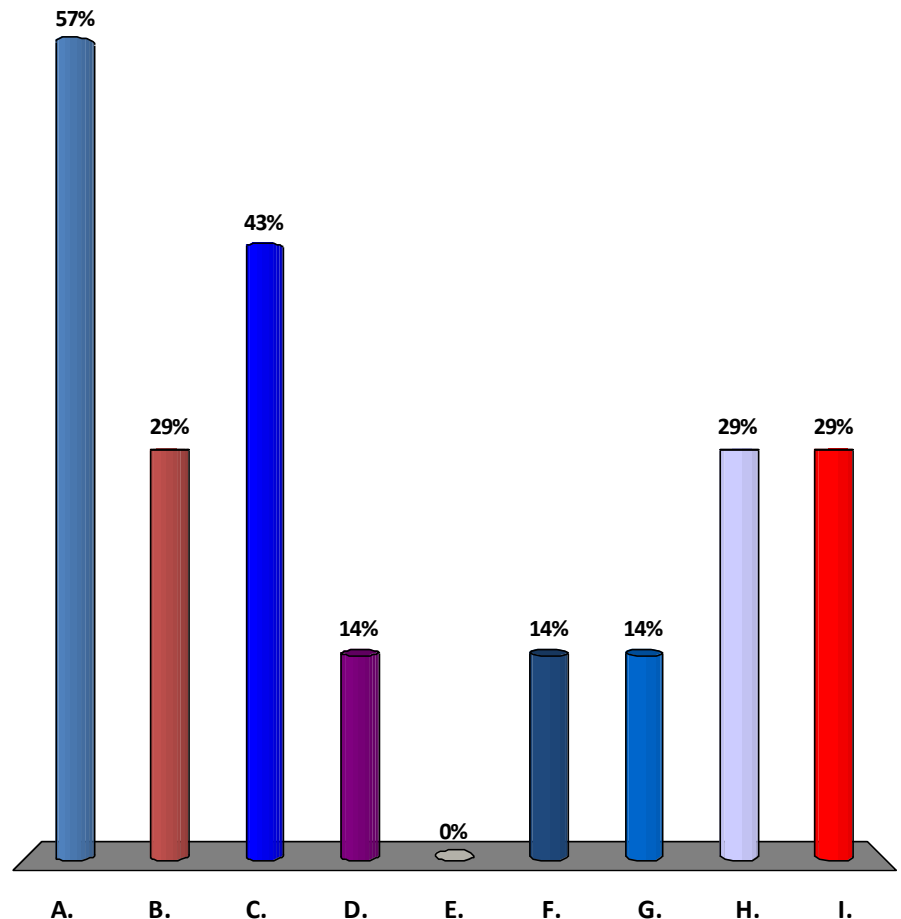
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# Panel Participants

- 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

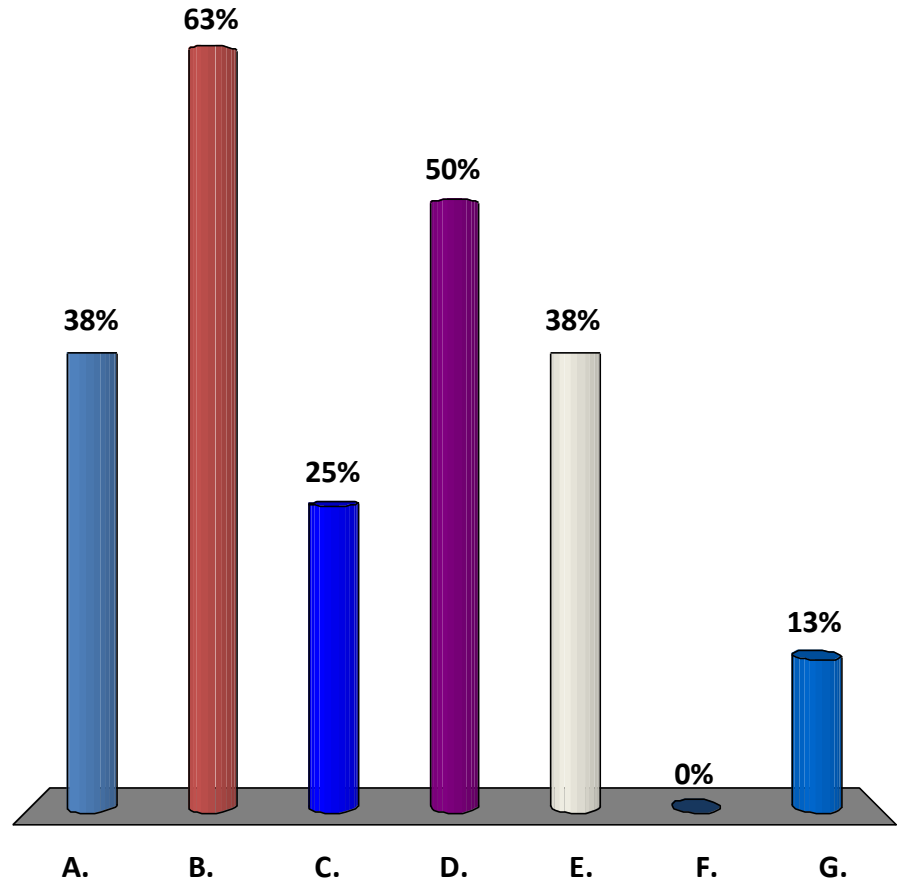




# Which health effects are most bothersome to you?

Please choose up to three health effects.

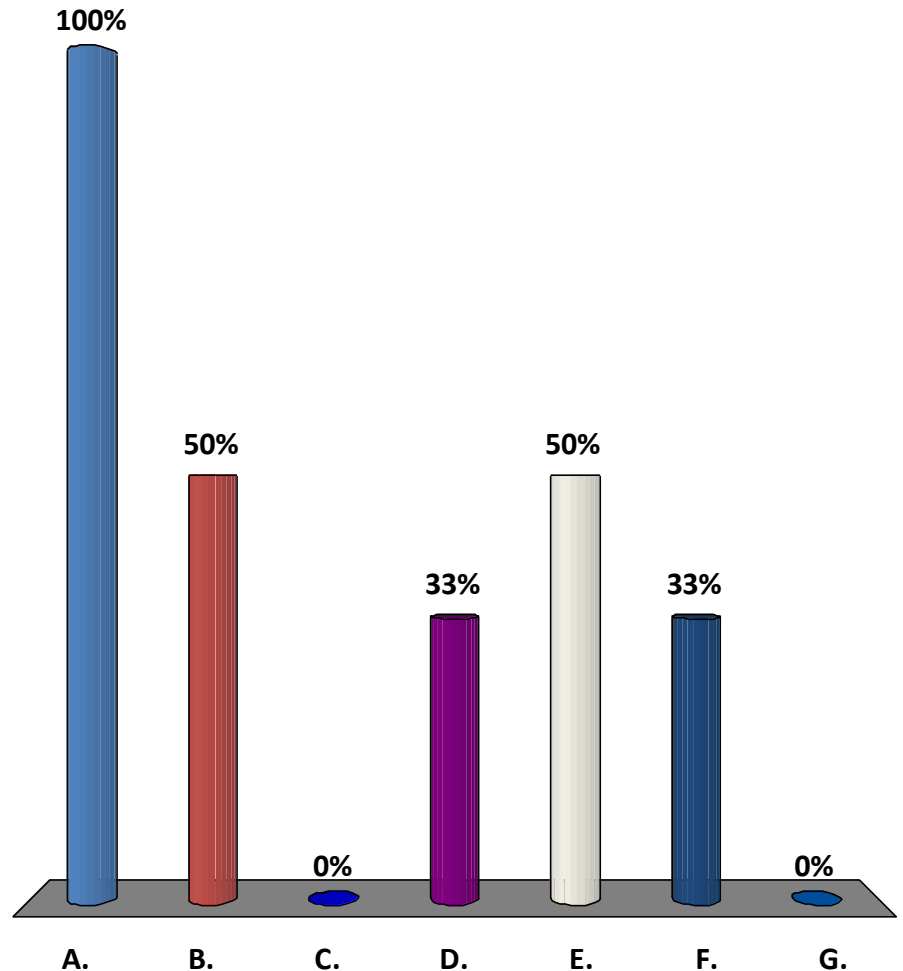
- A. Pain
- B. Fatigue or lack of energy
- C. Poor balance
- D. Difficulty walking
- E. Reduced muscle strength or increase muscle weakness
- F. Depression
- G. Other symptoms not mentioned



# 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



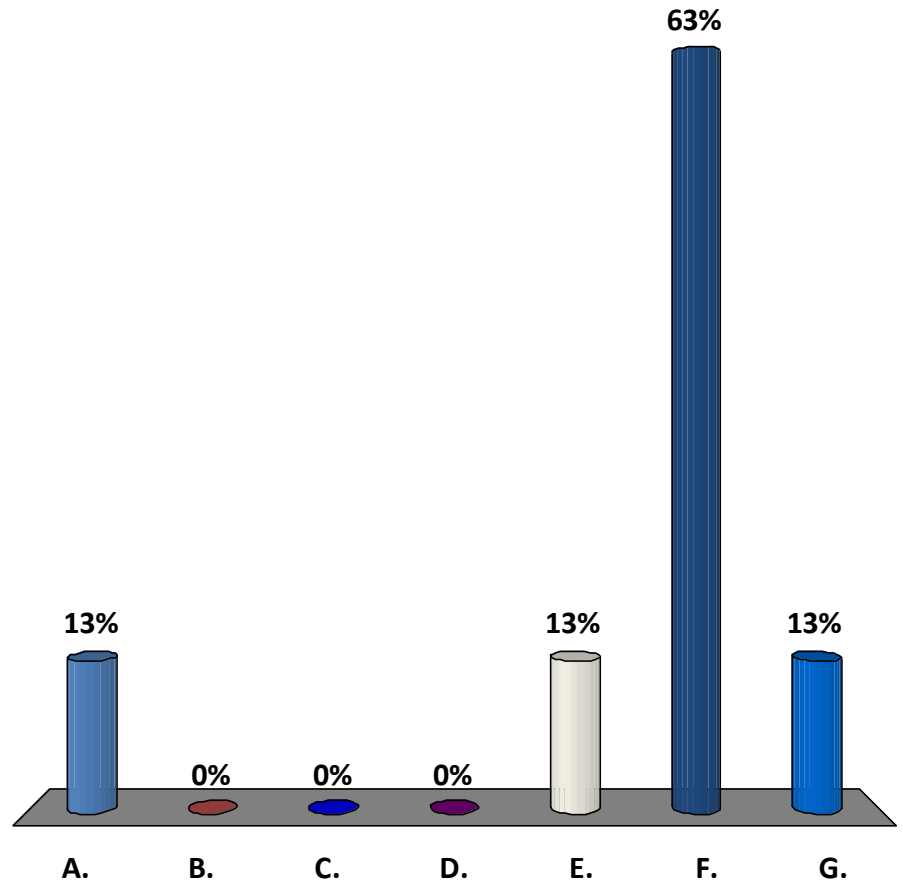
**BREAK**

**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

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U.S. Food and Drug Administration

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# Closing Remarks

Jean-Marc Guettier, M.D.

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