

The background of the slide features a white surface with several bright orange, round pills. In the upper left, a group of pills is blurred, while a single pill is in sharp focus in the lower right. A blue curved banner is at the top of the slide.

Public Meeting on Idiopathic Pulmonary Fibrosis Patient-Focused Drug Development

September 26, 2014



Welcome

Soujanya Giambone, MBA

Office of Strategic Programs

Center for Drug Evaluation and Research

U.S. Food and Drug Administration

September 26, 2014

Agenda

- **Setting the context**
 - Opening Remarks
 - Overview of FDA's Patient-Focused Drug Development Initiative
 - Background on Idiopathic Pulmonary Fibrosis and Therapeutic Options
 - Overview of Discussion Format
- **Discussion Topic 1:** Disease symptoms and daily impacts that matter most to patients
- **Discussion Topic 2:** Patients' perspectives on current approaches to treating idiopathic pulmonary fibrosis
- **Open Public Comment**
- **Closing Remarks**

A background image showing a cluster of orange, round pills in the upper left corner, with a single pill in sharp focus in the lower right corner. The pills are set against a white background with a blue curved border at the top.

Opening Remarks

Lydia Gilbert-McClain, MD

Deputy Director, Division of Pulmonary, Allergy and Rheumatology Products (DPARP)

Center for Drug Evaluation and Research

U.S. Food and Drug Administration

September 26, 2014



FDA's Patient-Focused Drug Development Initiative

Theresa Mullin, PhD

Director, Office of Strategic Program
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

September 26, 2014

Patient-Focused Drug Development under PDUFA V

- **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 the assessment of benefit-risk and decision making for new drugs
 - Input can inform FDA's oversight both during drug development and during our review of a marketing application
- **Patient-Focused Drug Development is part of FDA commitments under the fifth reauthorization of the Prescription Drug User Fee Act (PDUFA V)**
 - FDA will convene at least 20 meetings on specific disease areas over the next five years
 - Meetings will help develop a systematic approach to gathering input

Identifying Disease Areas for the Patient-Focused Meetings

- **In September 2012, FDA announced a preliminary set of diseases as potential meeting candidates**
 - Public input on these nominations was collected. FDA carefully considered these public comments and the perspectives of our drug review divisions at FDA
- **FDA selected a set of 16 diseases selected to be the focus of meetings for fiscal years 2013-2015**
 - Another public process will be initiated in 2015 to determine the set for fiscal years 2016-2017

Disease Areas to be the focus of meetings for FY 2013-2015

FY 2013

- Chronic fatigue syndrome
- HIV
- Lung cancer
- Narcolepsy

FY 2014

- Sickle cell disease
- Fibromyalgia
- Pulmonary arterial hypertension
- Inborn errors of metabolism
- Hemophilia A, Hemophilia B, von Willebrand disease, and other heritable bleeding disorders
- **Idiopathic pulmonary fibrosis**

FY 2015

- Female sexual dysfunction (October 27-28)
- Alpha-1 antitrypsin deficiency
- Breast cancer
- Chronic chagas disease
- Irritable bowel syndrome, gastroparesis, and gastroesophageal reflux disease
- Parkinson's disease and Huntington's disease

Tailoring Each Patient-Focused Meeting

- **Each meeting focuses on a set of questions that aim to elicit patients' perspectives on their disease and on treatment approaches**
 - We start with a set of questions that could apply to any disease area; these questions are taken from FDA's benefit-risk framework and represent important considerations in our decision-making
 - We then further tailor the questions to the disease topic of the meeting (e.g., current state of drug development, specific interests of the FDA review division, and the needs of the patient population)
- **Focus on relevant current topics in drug development for the disease at each meeting**
 - E.g., focus on HIV patient perspectives on potential “cure research”
- **We've learned that active patient involvement and participation is key to the success of these meetings.**

“Voice of the Patient” Reports

- Following each meeting, FDA publishes a Voice of the Patient report that summarizes the patient testimony at the meeting, perspectives shared in written docket comments, as well as any unique views provided by those who joined the meeting webcast.
- These reports serve an important function in communicating to both FDA review staff and the regulated industry what improvements patients would most like to see in their daily life.
- FDA believes that the long run impact of this program will be a better, more informed understanding of how we might find ways to develop new treatments for these diseases.

A background image showing several orange, round pills scattered across a white surface. One pill is in sharp focus in the lower right foreground, while others are blurred in the background.

Background on Idiopathic Pulmonary Fibrosis (IPF) and Therapeutic Options

Banu A. Karimi-Shah, MD

Medical Officer, DPARP
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

September 26, 2014

Introduction

- Rare, chronic, progressive, interstitial lung disease of unknown etiology affecting ~5 million patients worldwide
- Affects males > females, diagnosis occurs between 5th and 7th decade of life
- Progression of disease is variable among individuals
- Progressive fibrosis (scarring) leads ultimately to death with a median survival of 3 to 5 years after diagnosis

Signs and Symptoms

- Dyspnea (shortness of breath)
 - Rapid, shallow breathing
 - With Exertion
- Non-productive (dry) cough
- Gradual, unintended weight loss
- Feeling tired
- Clubbing of the fingers and toes

Treatment Options

- The goals for the treatment of IPF are to reduce symptoms and signs, improve quality of life, slow/halt disease progression, and increase survival.
- Current therapies:
 - Lung transplantation
 - No recommended/approved therapies specifically to treat IPF
 - Even some therapies which were once considered “standard of care” have been shown to be harmful when studied in clinical trials

Challenges in Drug Development for IPF

- Small number of patients with the disease
- Diverse phenotypes (disease characteristics)
- Important unanswered questions:
 - What signs and symptoms should be measured, and how?
 - Do the disease symptoms progress in a time course that can be captured adequately in a clinical trial?
 - Do biomarkers exist and do they have relevance as an endpoint?

Patient-Reported Outcomes

- For conditions like IPF, which are not fully understood, input from patients is especially important
- Patient-reported outcomes (PROs) can represent direct measures of treatment benefit – how a patient feels or functions
- All measurements need to be evaluated in adequate and well-controlled randomized trials
- Patient input is essential to capture important and clinically-relevant disease symptoms in PROs

A background image showing a cluster of orange, round pills in the upper left corner, with a single orange pill in sharp focus in the lower right corner. The pills are set against a white background with a blue curved border at the top.

Overview of Discussion Format

Soujanya Giambone, MBA

Office of Strategic Programs

Center for Drug Evaluation and Research

U.S. Food and Drug Administration

September 26, 2014

Discussion Overview

Topic 1: The symptoms that matter most to you

- Which symptoms have the most significant impact on your life?
- How do these symptoms affect your ability to do specific activities?
- How have your symptoms changed?

Topic 2: Current approaches to treating IPF

- What are you doing to treat IPF?
- What are their biggest downsides?
- What would you look for in an “ideal” treatment?

Discussion Format

- **We will first hear from a panel of patients and caregivers**
 - The purpose is to set a good foundation for our discussion
 - They reflect a range of experiences with IPF

- **We will then broaden the dialogue to include patients and patient representatives 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
 - Patients and patient representatives 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

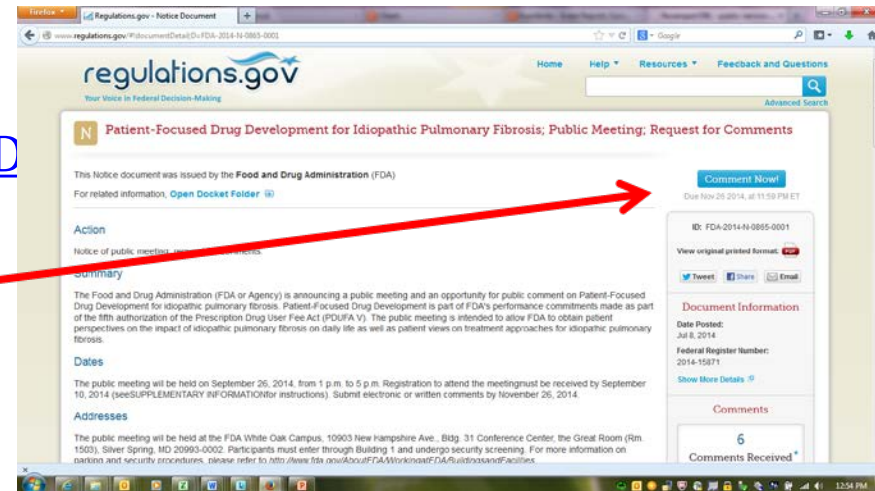
Send us your comments!

- **You can send us comments through the “public docket”.**
 - The docket will be open until November 26, 2014
 - Share your experience, or expand upon something discussed today
 - Comments will be incorporated into our summary report
 - Anyone is welcome to comment

Visit:

<http://www.regulations.gov/#!/documentDetail;D=FDA-2014-N-0865-0001>

Click Comment Now!

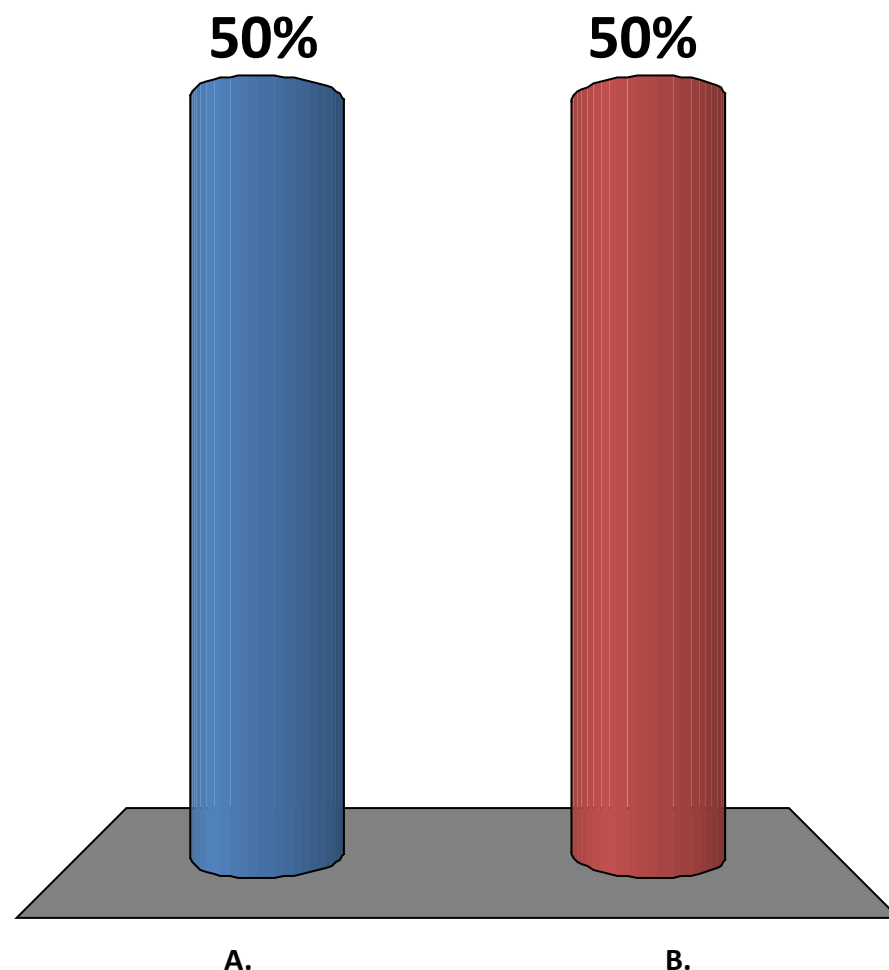


Discussion Ground Rules

- We encourage patients to contribute to the dialogue—caregivers and advocates are welcome too
- FDA is here to listen
- Discussion will focus on symptoms and treatments
 - Open Public Comment Period is available to comment on other topics
- The views expressed today are personal opinions
- Respect for one another is paramount
- Let us know how the meeting went today; evaluations at registration desk

Where do you live?

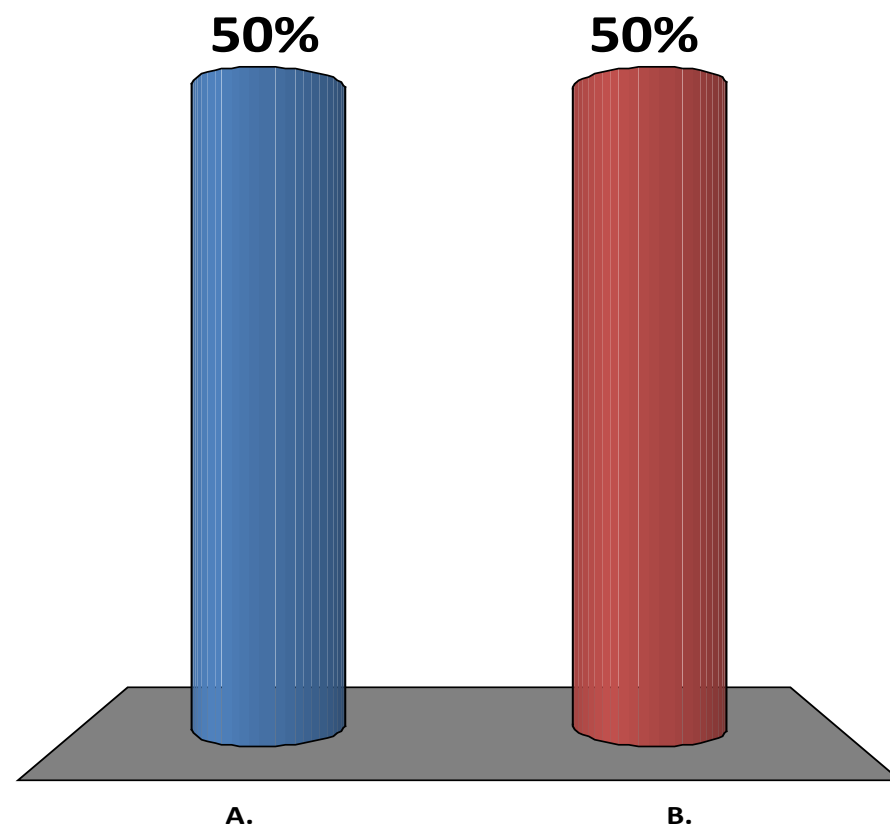
- A. Within Washington, D.C. metropolitan area (including the Virginia and Maryland suburbs)
- B. Outside of the Washington, D.C. metropolitan area



Have you ever been diagnosed as having idiopathic pulmonary fibrosis?

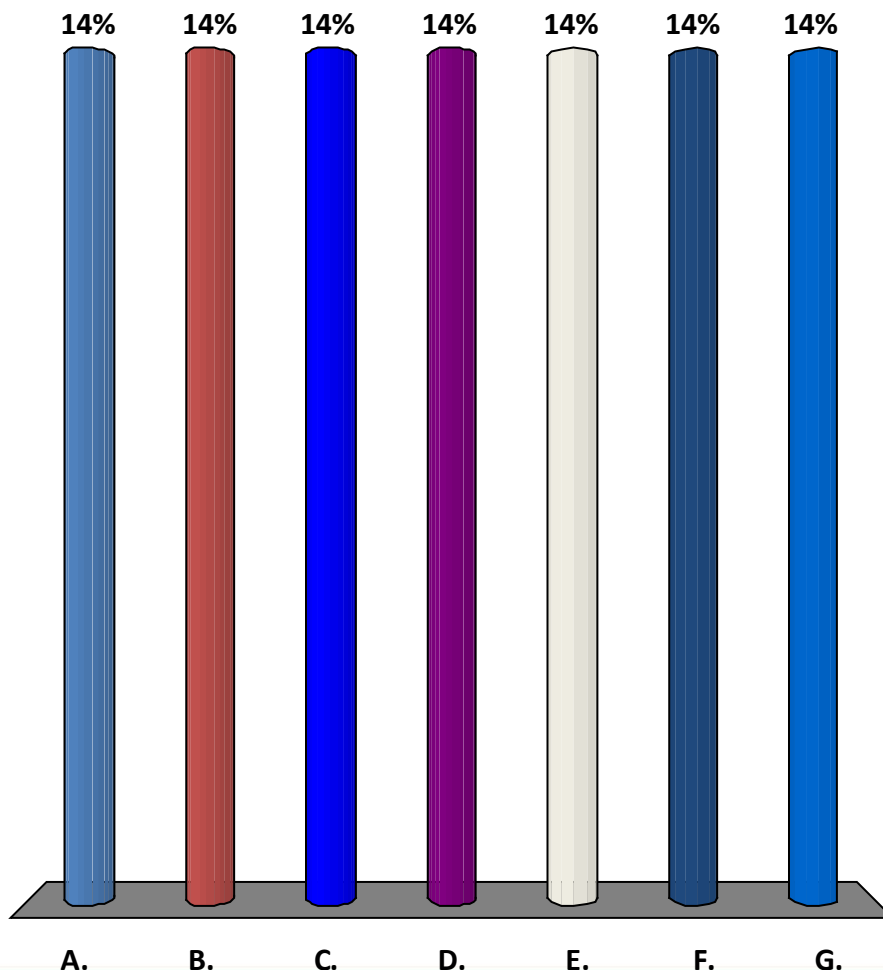
A. Yes

B. No



What is your age / your loved one's age?

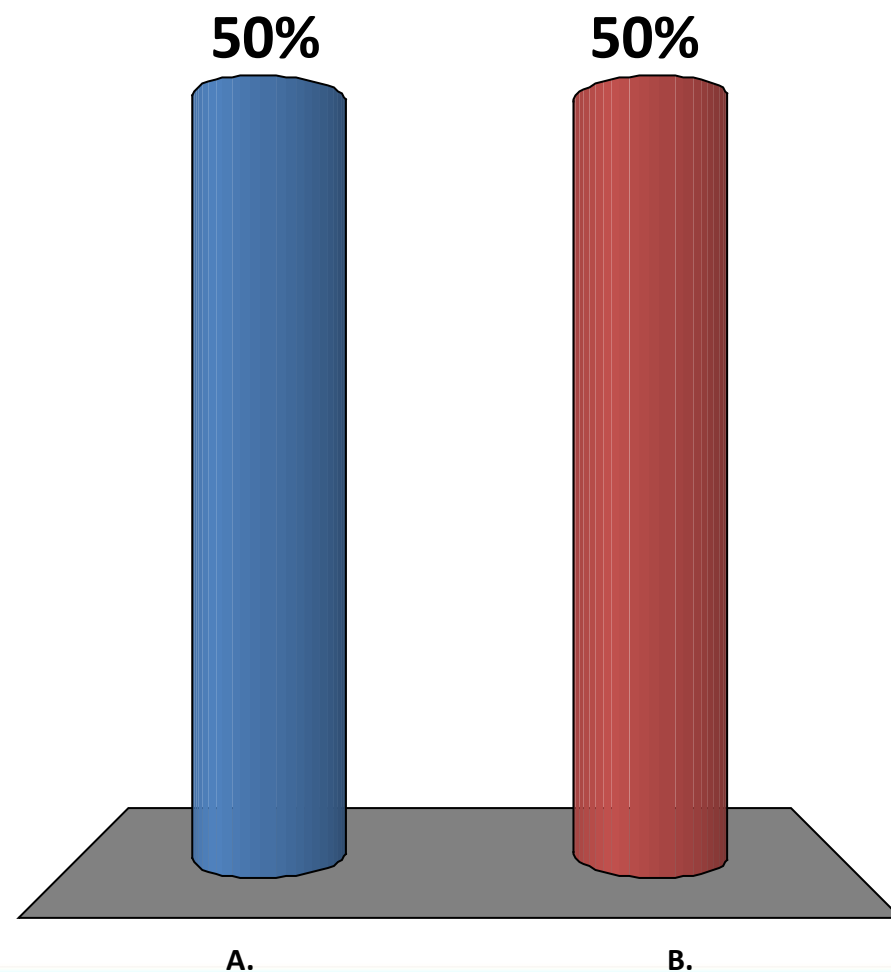
- A. Younger than 30
- B. 31 – 40
- C. 41 – 50
- D. 51 – 60
- E. 61 – 70
- F. 71 – 80
- G. 81 or greater



Are you / is your loved one:

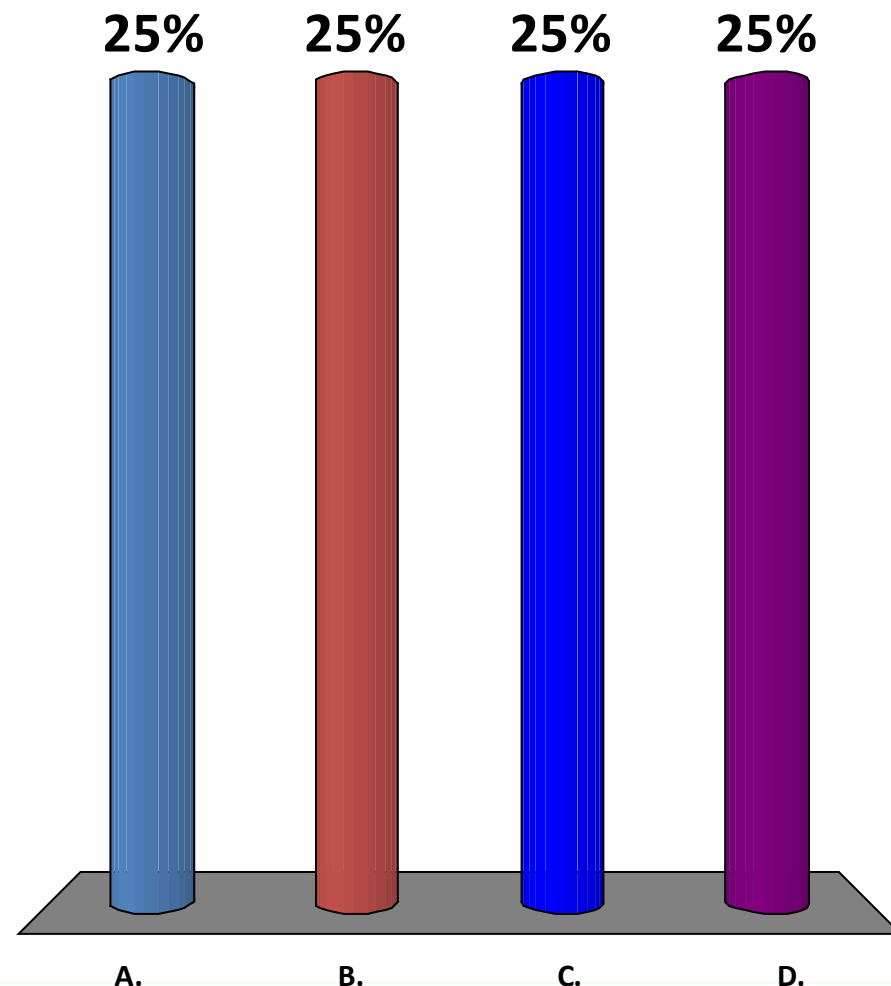
A. Male

B. Female



What is the length of time since your diagnosis?

- A. Less than 1 year ago
- B. 1 year ago to 3 years ago
- C. 3 years ago to 5 years ago
- D. More than 5 years ago





Discussion Topic 1

Disease symptoms and daily impacts that matter most to patients

Soujanya Giambone

Facilitator

Topic 1 Panel Participants

Faye MacInnis

Laura Roix

Curtis Thompson

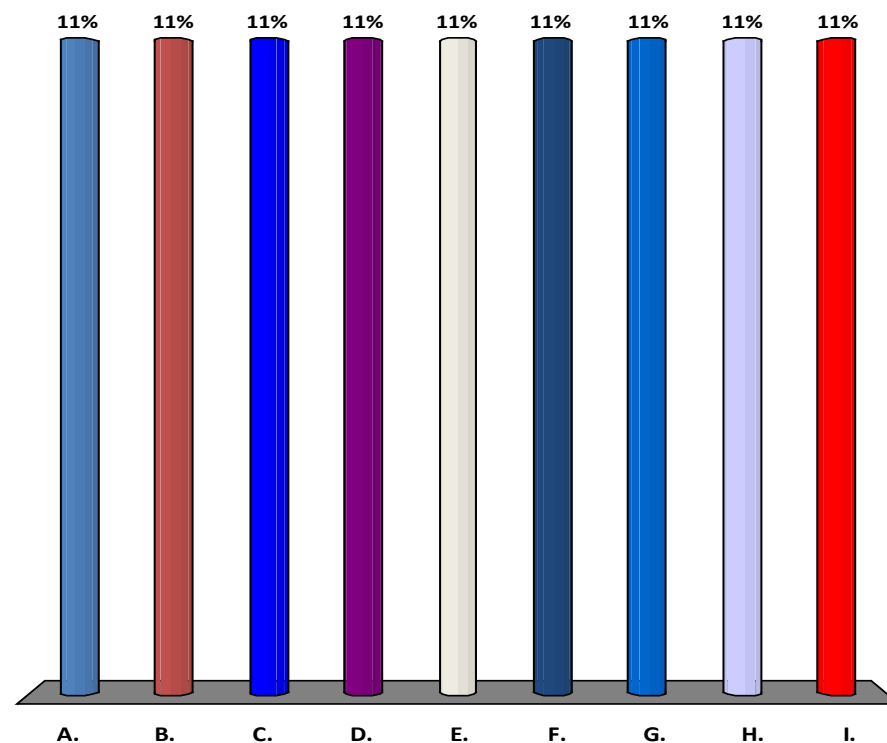
Diane Reichert

Topic 1 Discussion: Disease symptoms and daily impacts that matter most to patients

- Of all the symptoms that you experience because of your condition, which 1-3 symptoms have the most significant impact on your life?
- Are there specific activities that are important to you but that you cannot do at all or as fully as you would like because of your condition?
- How have your condition and its symptoms changed over time?

Of all the symptoms you have experienced because of idiopathic pulmonary fibrosis, which do you consider to have the most significant impact on your daily life? **Please choose up to three symptoms.**

- A. Shortness of breath
- B. Fatigue or malaise
- C. Coughing, especially dry or hacking coughs
- D. Chest pain
- E. Gradual, unintended weight loss
- F. Decreased appetite
- G. Clubbing, (the widening and rounding of the tips of the fingers or toes)
- H. Depression
- I. Other symptoms not mentioned





BREAK



Discussion Topic 2

Patients' perspectives on current approaches to treating Idiopathic Pulmonary Fibrosis

Soujanya Giambone

Facilitator

Topic 2 Panel Participants

Heather Snyder

Michael Henderson

Taleena Koch

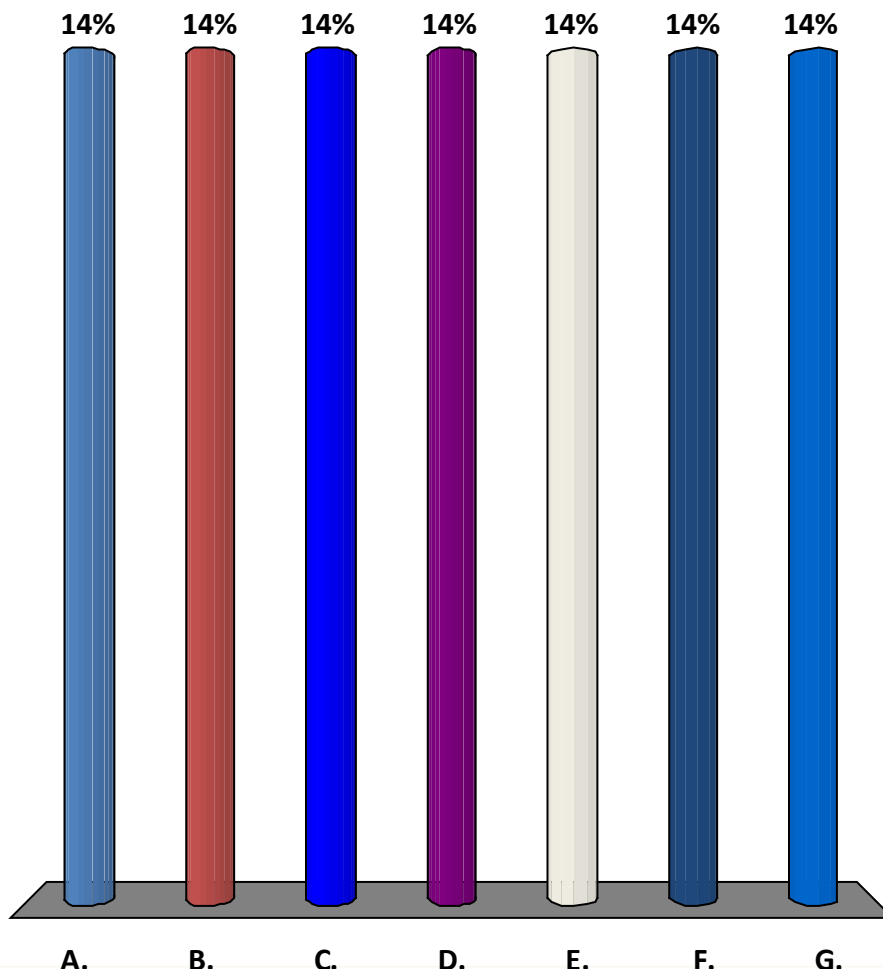
Teresa Barnes

Topic 2 Discussion: Patients' perspectives on current approaches to treating Idiopathic Pulmonary Fibrosis

- What are you currently doing to help treat your condition or its symptoms?
- Have the medications for Idiopathic Pulmonary Fibrosis made a difference to you? If so, in what ways?
- What are the most significant downsides to your current treatments, and how do they affect your daily life?
- What specific things would you look for in an ideal treatment for your condition?

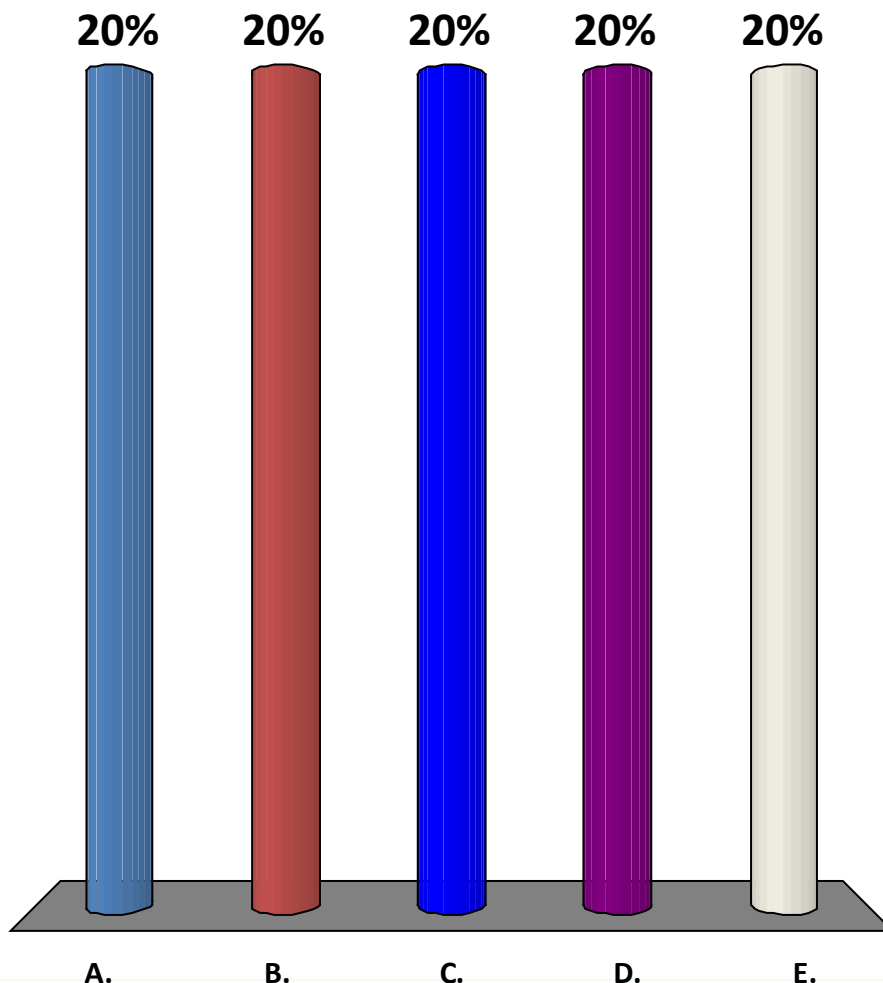
Have you ever used any of the following drug therapies to help reduce the symptoms of idiopathic pulmonary fibrosis? Check all that apply.

- A. Pulmonary rehabilitation
- B. Corticosteroids
- C. Immunosuppressants
- D. NAC (N-acetylcysteine)
- E. Investigational therapy
- F. Other prescription medicines (e.g. acide reflux therapy, inhalers)
- G. I'm not sure



Besides your drug therapies, what else are you doing to help reduce your symptoms of idiopathic pulmonary fibrosis? Check all that apply.

- A. Oxygen therapy
- B. Surgery (such as lung transplantation)
- C. Lifestyle changes, such as limit activity, changes in your home
- D. Other therapies not mentioned
- E. I am not doing or taking any therapies to treat symptoms





Open Public Comment Period

A background image showing several orange, round pills scattered across a white surface. The pills are out of focus in the upper left and become sharper as they move towards the right. One pill is in sharp focus in the lower right quadrant.

Closing Remarks

Banu A. Karimi-Shah, MD

Medical Officer, DPARP

Center for Drug Evaluation and Research

U.S. Food and Drug Administration