Patient Perspectives on the Impact of Rare Diseases: Bridging the Commonalities

Welcome

April 29, 2019

Docket Number: FDA-2019-N-0077

JANET MAYNARD, MD, MHS

Director, Office of Orphan Products Development
U.S. Food and Drug Administration

ANDREA FURIA-HELMS, MPH

Director, Patient Affairs Staff
U.S. Food and Drug Administration

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Key Information

WIFI:

- Network: FDA Public
- Passcode: publicaccess

Electronic or written comments can be submitted to the public docket (Docket# FDA-2019-N-0077) through May 30, 2019.

The webcast recording will be available approximately 1 week after the meeting.

A transcript from the meeting will be available on the FDA meeting website: https://www.fda.gov/NewsEvents/MeetingsConferencesWorkshops/ucm628352.htm

Agenda

1:00 PM	Opening Remarks
1:10 PM	Meeting Overview
1:20 PM	First Session: Panel Discussion and Facilitated Group Discussion
2:40 PM	Remarks by FDA's Principal Deputy Commissioner
2:55 PM	BREAK
3:15 PM	Second Session: Panel Discussion and Facilitated Group Discussion
4:15 PM	Open Public Comment
4:50 PM	Closing Remarks
5:00 PM	Adjourn

We want to connect with you!

Patient Affairs Staff:

www.fda.gov/RequestToConnect

PatientAffairs@fda.gov

Twitter: @FDAPatientInfo

301-796-8460

Office of Orphan Products Development:

www.fda.gov/Orphan

Orphan@fda.gov

301-796-8660

Tweet with our hashtag!

#RareDiseaseFDA

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Rules of Engagement

- The views expressed are personal opinions
- Please be respectful; allow participants to finish without interrupting
- FDA panel members may ask follow-up questions
- Participants in the room must use a microphone

FDA Panelists

Andrea Furia-Helms, MPH (Patient Affairs Staff)

Lucas Kempf, MD (CDER)

Janet Maynard, MD, MHS (Office of Orphan Products Development)

Susan McCune, MD (Office of Pediatric Therapeutics)

Douglas Silverstein, MD (CDRH)

Rachel Witten, MD (CBER)

SUSAN CHITTOORAN, MSW

Patient Affairs Staff
U.S. Food and Drug Administration

Topic 1: Commonalities in symptom management and treatment considerations

1. What are your 2-3 most burdensome symptoms and what are you currently doing to help manage the symptoms of your rare disease or condition? (Examples may include, but are not limited to, prescription medicines, over-the-counter products, devices, non-drug therapies, or support services)

Topic 1: Commonalities in symptom management and treatment considerations

2. What factors do you take into account when making decisions about how to manage your symptoms?

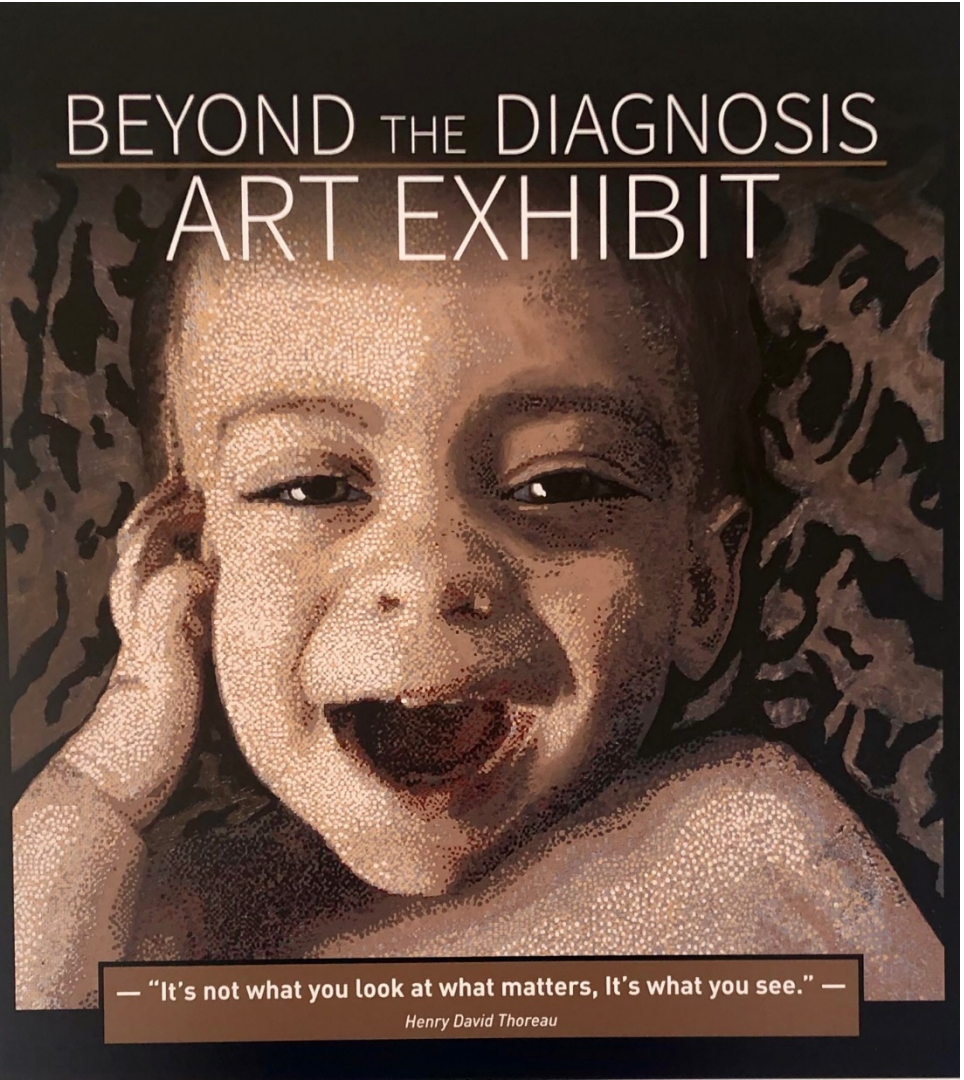
a) What potential benefits factor most into your decisions?

b) How do you weigh potential benefits versus potential common side effects?

c) How do you weigh potential benefits versus potential less common but serious risks?

AMY ABERNETHY, MD, PhD

Principal Deputy Commissioner and Acting Chief Information Officer
U.S. Food and Drug Administration



The FDA Rare Disease Council is pleased to host the *Beyond the Diagnosis Art Exhibit*.

This exhibit encourages us to look beyond the diagnosis and focus on the rare disease patient.

BREAK

SUSAN CHITTOORAN, MSW

Patient Affairs Staff
U.S. Food and Drug Administration

Topic 2: Commonalities in clinical study considerations and registry considerations

1. What factors have you or would you consider when thinking about participating in a clinical study or registry (Examples may include, but are not limited to, side effects, travel, time commitment, or trial design)?

Open Public Comment

- Speakers will have 2 minutes to speak.
- We will call each speaker by name; please come to the microphone in the middle of the room.
- Timer Lights:
  - Green – speak
  - Yellow – 30 seconds remaining
  - Red – stop
- Docket: FDA-2019-N-0077 open through May 30, 2019

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Thank you for Attending!

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