



# Patient-Focused Drug Development

**FDA Wants  
To Hear  
From Patients**

**FDA**

**U.S. FOOD & DRUG  
ADMINISTRATION**

# Public Meeting on Patient-Focused Drug Development for Alopecia Areata

September 11, 2017

# Welcome

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

September 11, 2017

# Agenda

- Opening Remarks
- Setting the context
  - Overview of FDA's Patient-Focused Drug Development
  - Overview of Alopecia Areata
  - Road from PFDD Meetings to Clinical Trial Endpoints
  - Overview of Discussion Format
- Discussion Topic 1
- Break
- Discussion Topic 2
- Open Public Comment
- Closing Remarks

# Opening Remarks

Tatiana Oussova, MD

Deputy Director for Safety, Division of Dermatology and Dental Products (DDDP)

Office of New Drugs

Center for Drug Evaluation and Research

U.S. Food and Drug Administration

September 11, 2017

# FDA's Patient-Focused Drug Development Initiative

Theresa Mullin, PhD  
Director, Office of Strategic Programs  
Center for Drug Evaluation and Research  
U.S. Food and Drug Administration

September 11, 2017

# 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 authorization of the Prescription Drug User Fee Act (PDUFA V)**
  - FDA is convening 24 meetings on specific disease areas in FY 2013-2017
  - Meetings will help develop a systematic approach to gathering patient input

# Disease Areas to be the focus of meetings for FY 2013-2017

Fiscal Year 2013	Fiscal Year 2014	Fiscal Year 2015	Fiscal Year 2016	Fiscal Year 2017
<ul style="list-style-type: none"> <li>• Chronic fatigue syndrome/ myalgic encephalomyelitis</li> <li>• HIV</li> <li>• Lung cancer</li> <li>• Narcolepsy</li> </ul>	<ul style="list-style-type: none"> <li>• Sickle cell disease</li> <li>• Fibromyalgia</li> <li>• Pulmonary arterial hypertension</li> <li>• Inborn errors of metabolism</li> <li>• Hemophilia A, B, and other heritable bleeding disorders</li> <li>• Idiopathic pulmonary fibrosis</li> </ul>	<ul style="list-style-type: none"> <li>• Female sexual dysfunction</li> <li>• Breast cancer</li> <li>• Chagas disease</li> <li>• Functional gastrointestinal disorders</li> <li>• Huntington’s disease and Parkinson’s disease</li> <li>• Alpha-1 antitrypsin deficiency</li> </ul>	<ul style="list-style-type: none"> <li>• Non-tuberculous mycobacterial lung infections</li> <li>• Psoriasis</li> <li>• Neuropathic pain associated with peripheral neuropathy</li> <li>• Patients who have received an organ transplant</li> </ul>	<ul style="list-style-type: none"> <li>• Sarcopenia</li> <li>• Autism</li> <li>• <b>Alopecia areata</b></li> <li>• Hereditary angioedema (September 25)</li> </ul>



# “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.

# An Overview of Alopecia Areata

Melissa Reyes, MD, MPH, DTMH  
Division of Dermatology and Dental Products (DDDP)  
Office of New Drugs  
Center for Drug Evaluation and Research  
U.S. Food and Drug Administration

September 11, 2017

# An Overview of Alopecia Areata



- Clinical Features
- Epidemiology
- Treatment Options
- Impact of Alopecia Areata

# Alopecia Areata



- Alopecia areata is a relatively common disorder affecting the hair follicles
  - Three patterns: focal, totalis, universalis
- Predominantly skin manifestations, but can affect nails

# Clinical Presentation



## Alopecia Areata - focal

# Clinical Presentation



## Alopecia Areata - focal

# Clinical Presentation



Alopecia Totalis

# Clinical Presentation



Alopecia Universalis

# Clinical Presentation



Nail Involvement

# Epidemiology of Alopecia Areata



- Affects  $\approx$  0.15% of the U.S. population<sup>1</sup>
  - $\sim$  490,000 individuals
  - Majority with onset by age 40 years, almost half by age 20<sup>2</sup>
- Occurrence likely equal in females and males<sup>2</sup>
- In children, mean age of onset between 5-10 years of age

<sup>1</sup>Interventions for alopecia areata. Cochrane Database Syst Rev. 2008.

<sup>2</sup>Fricke, et al. Epidemiology and burden of alopecia areata: a systematic review. Clin Cosmet Investig Dermatol. 2015;8:397-403.

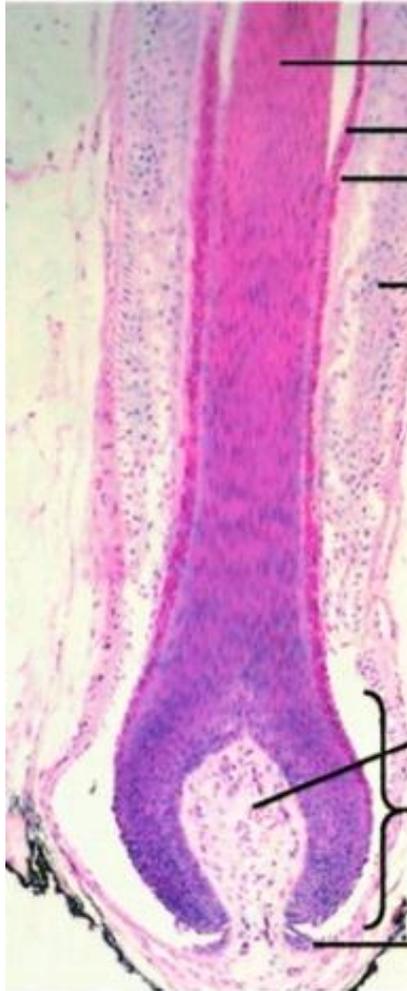
# Epidemiology of Alopecia Areata



- Onset in first two decades associated with more severe disease<sup>2</sup>
- Alopecia totalis and universalis: almost always before age 30 years<sup>2</sup>

<sup>2</sup>Fricke, et al. Epidemiology and burden of alopecia areata: a systematic review. Clin Cosmet Investig Dermatol. 2015;8:397-403.

# Pathology



# Treatment Options for Alopecia Areata



## **FDA-approved**

- None

# Treatment Options for Alopecia Areata



## Guidelines

- American Academy of Dermatology – Alopecia Areata Update. Part II. Treatment – 2010
- British Academy of Dermatology – 2012
- Japanese Dermatologic Association – 2010
- Cochrane review “Interventions for Alopecia Areata (Review)” - 2008

# Treatment Options for Alopecia Areata



## Local

- Corticosteroids
- Calcineurin inhibitors
- Immunotherapies
- Minoxidil
- Prostaglandin analogs
- Platelet-rich plasma
- Retinoids
- Cryotherapies
- 308 nm Excimer laser and phototherapy

# Treatment Options for Alopecia Areata



## Systemic

- Corticosteroids
- Immunosuppressants
- Immunomodulators
- Retinoids
- Statins

# Impact of Alopecia on Quality of Life



- Social, psychological and economic impacts
  - adult versus pediatric experience
- AA patients have lower Health-Related Quality of Life than control patients<sup>3</sup> and comparable to other chronic skin conditions such as psoriasis and atopic dermatitis.

<sup>3</sup>Health-related quality of life among patients with alopecia areata: A systemic review. J Am Acad Dermatol 2016;75:806-12.

# Treatment Options for Alopecia Areata



- Cochrane Review, 2008
  - “desperate need for large well conducted studies that evaluate long-term effects of therapies on quality of life”

# Patient-Focused Drug Development



- The FDA is aware of unmet medical needs experienced by patients who have Alopecia.
- FDA is conducting this public meeting to hear comments about the impact of Alopecia on patients, caregivers and family members.

Thank you for taking the time, and making the trip to share your comments with us today.

# The Road from Patient-Focused Drug Development Public Meetings to Clinical Study Endpoints



**Michelle Campbell, Ph.D.**  
Clinical Outcome Assessments Staff  
Office of New Drugs  
Center for Drug Evaluation and Research  
U.S. Food and Drug Administration



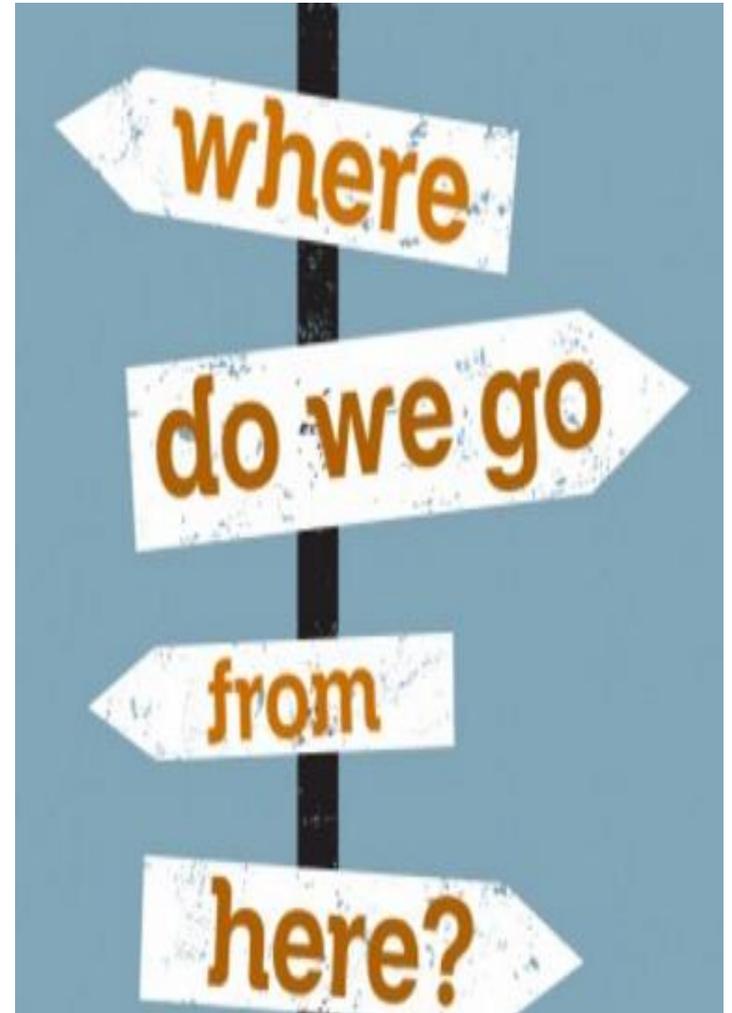
# Disclaimer

The views expressed in this presentation are those of the speaker, and do not necessarily represent an official FDA position.

**PATIENT-FOCUSED  
DRUG DEVELOPMENT  
(PFDD) MEETINGS**



**But...**



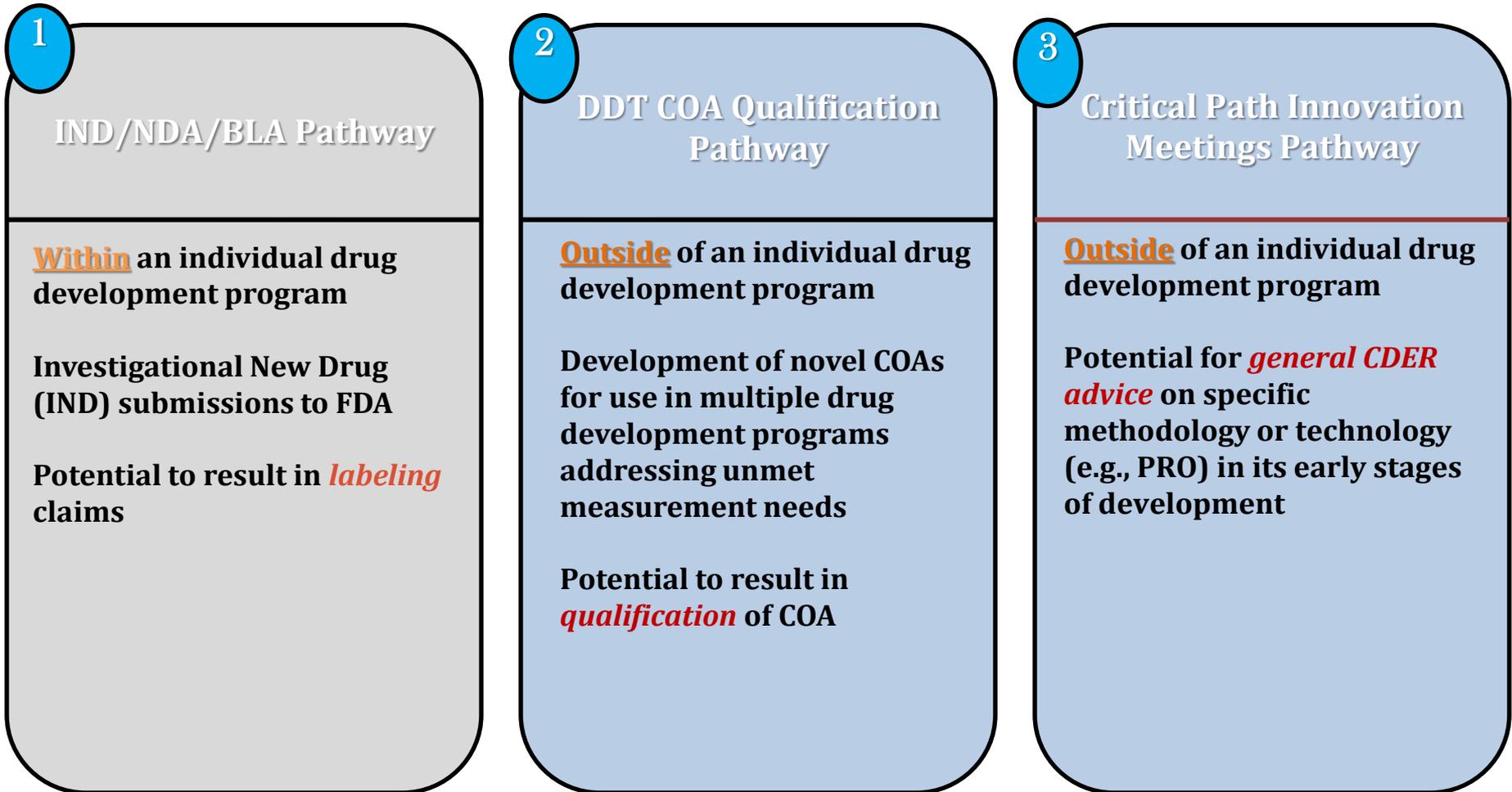
# Your Voice







# Pathways for FDA Clinical Outcome Assessment Review & Advice



DDT = Drug Development Tool; COA = Clinical Outcome Assessment; PRO = Patient-Reported Outcome  
NDA = New Drug Application; BLA = Biologics Licensing Application



# Key Takeaways

- PFDD meetings are a “starting point” for developing patient-focused outcome measures and endpoints
- The outcomes of PFDD meetings will support and guide FDA risk-benefit assessments in drug reviews
- Individual and caregiver input ultimately helps determine:
  - WHAT is measured to provide evidence of treatment benefit
  - HOW best to measure concepts in a clinical study
  - WHAT a meaningful improvement is in treatment benefit

# Relevant Resources



- **FDA COA Staff Website:**  
<http://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDER/ucm349031.htm#Endpoints>
- **PRO Guidance:**  
<http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM193282.pdf>
- **DDT COA Qualification Guidance:**  
<http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM230597.pdf>
- **DDT COA Qualification Website:**  
<http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugDevelopmentToolsQualificationProgram/ucm284077.htm>
- **Critical Path Innovation Meeting Website & Guidance:**  
<http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugInnovation/ucm395888.htm>

# Overview of Discussion Format

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

September 11, 2017

# Discussion Overview



## **Topic 1: Health Effects and Daily Impacts of Alopecia Areata**

- Which health effects have the most significant impact on your life?
- Are there 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 do these health effects impact your life day to day?
- How have your experiences with alopecia areata changed over time?
- What worries you most about your condition?

## **Topic 2: Current Approaches to Treatment**

- What are you currently doing to treat your condition or its symptoms?
- How well do your current treatments manage your condition?
- What are the significant downsides to your current treatments?
- What are you looking for in an ideal treatment for alopecia areata?
- What factors do you take into account when making decisions about selecting a treatment?

# Discussion Format

- **We will kick off our discussion with comments from a panel of individuals and family members**
  - The purpose is to set context for a broader discussion with the audience
  - Panel commenters reflect a range of experiences with alopecia areata
  - Some panelists are affiliated with advocacy or support organizations
  
- **We will then broaden the dialogue to include individuals and family members 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
  - Individuals or family members 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 13, 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-2017-N-3067-0001>

Or Search “alopecia areata FDA public meeting” on [www.regulations.gov](http://www.regulations.gov)

**And Click Comment Now!**



The screenshot shows the 'regulations.gov' website interface. The main heading is 'Patient-Focused Drug Development for Alopecia Areata; Public Meeting; Request for Comments'. Below this, there is a 'Comment Now!' button highlighted with a red arrow. The page also displays the document ID 'FDA-2017-N-3067-0001', the date posted 'Jun 23, 2017', and the number of comments received, which is '6 Comments Received'. A testimonial quote is visible at the bottom right of the page.



# Resources at FDA

A first stop for individuals and families:

- **FDA Office of Health and Constituent Affairs**
  - Contact: [PatientNetwork@fda.hhs.gov](mailto:PatientNetwork@fda.hhs.gov), (301) 796-8460
  - Liaison between FDA and stakeholder organizations
  - Runs the Patient Network and Patient Representative Program

A first stop for advocacy and support healthcare providers:

- **CDER Professional Affairs and Stakeholder Engagement (PASE)**
  - Contact: [CDERPASE@fda.hhs.gov](mailto:CDERPASE@fda.hhs.gov)
  - Facilitates communication and collaboration between CDER and stakeholders on issues in drug development, review, and safety.

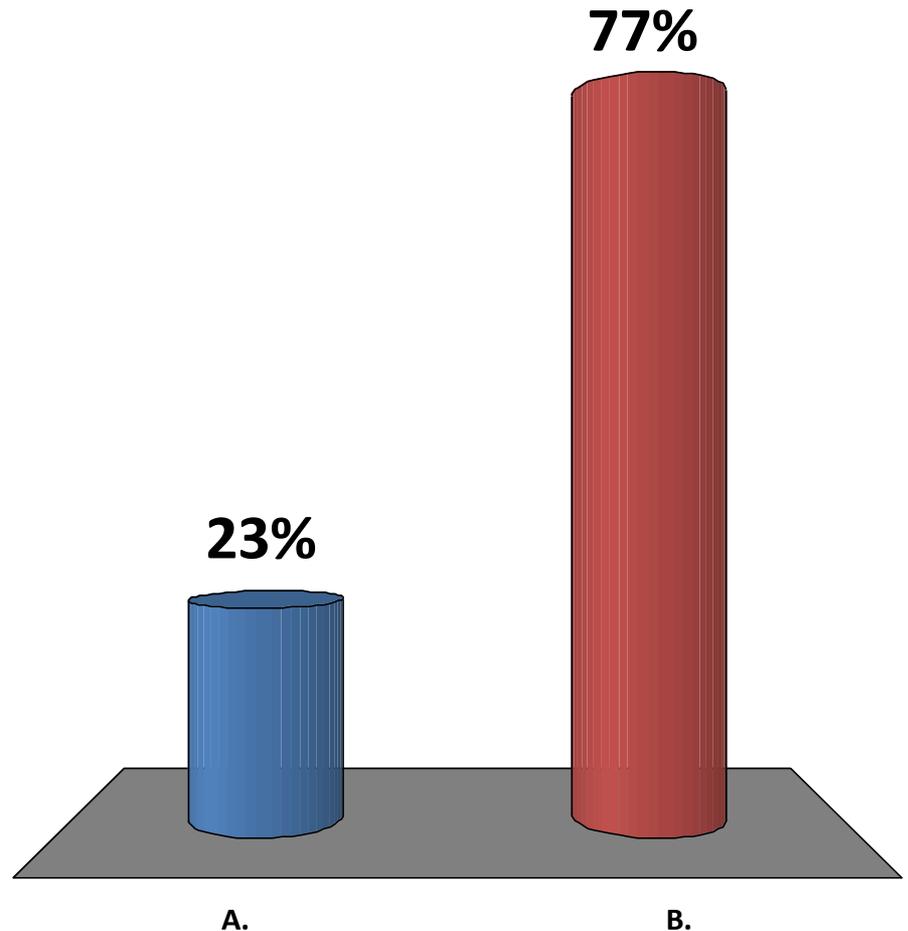


# Discussion Ground Rules

- We encourage all individuals and family members to contribute to the dialogue
- FDA is here to listen
- Discussion will focus on alopecia health effects 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; evaluation forms are available at the registration table

# Where do you live?

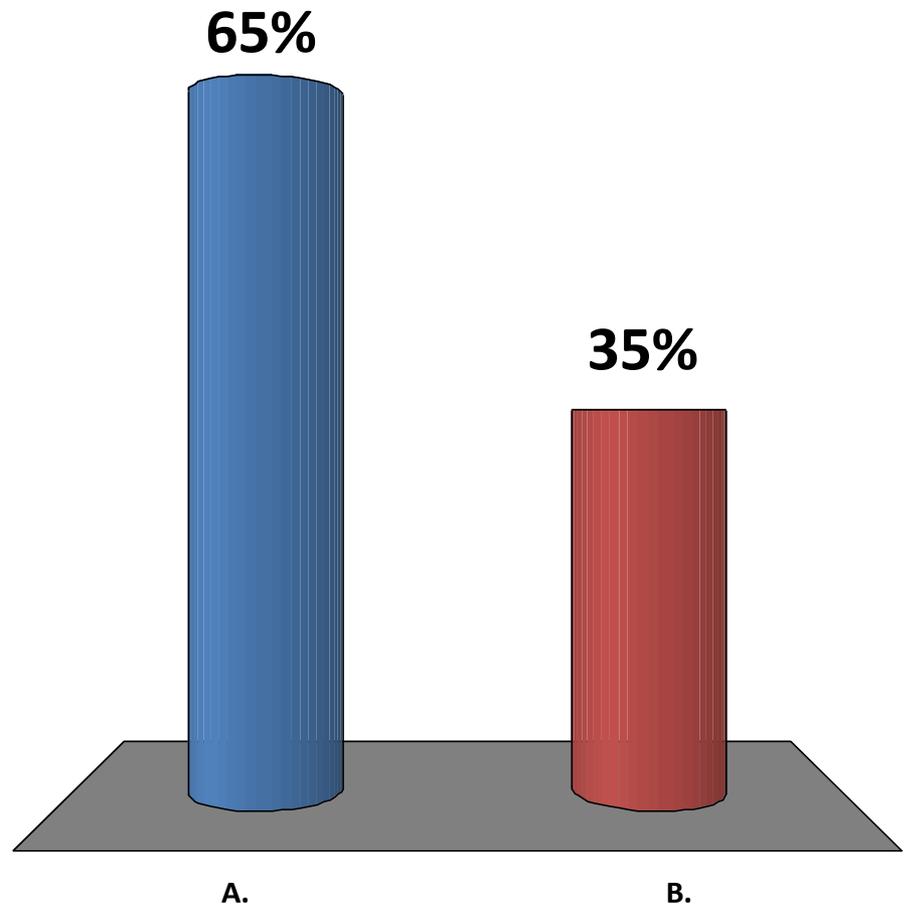
- A. Within Washington, D.C metropolitan area (including the Virginia and Maryland suburbs)
- B. Outside of Washington, D.C. metropolitan area



# Have you ever been diagnosed as having alopecia areata?

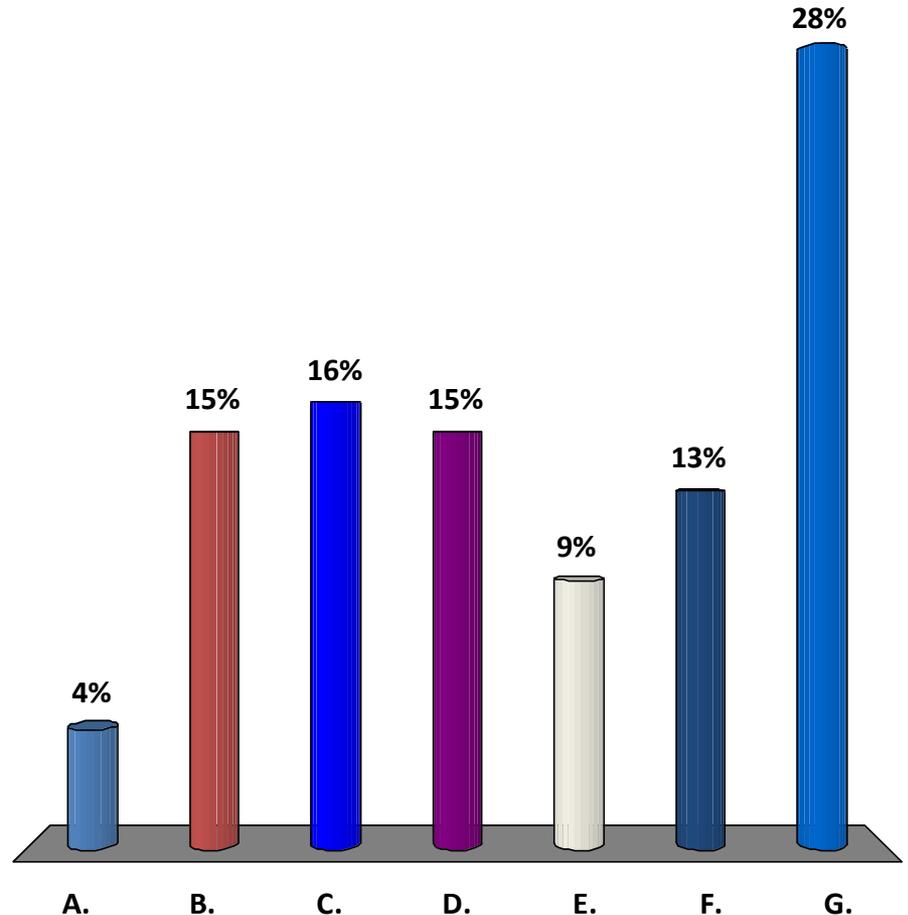
A. Yes

B. No



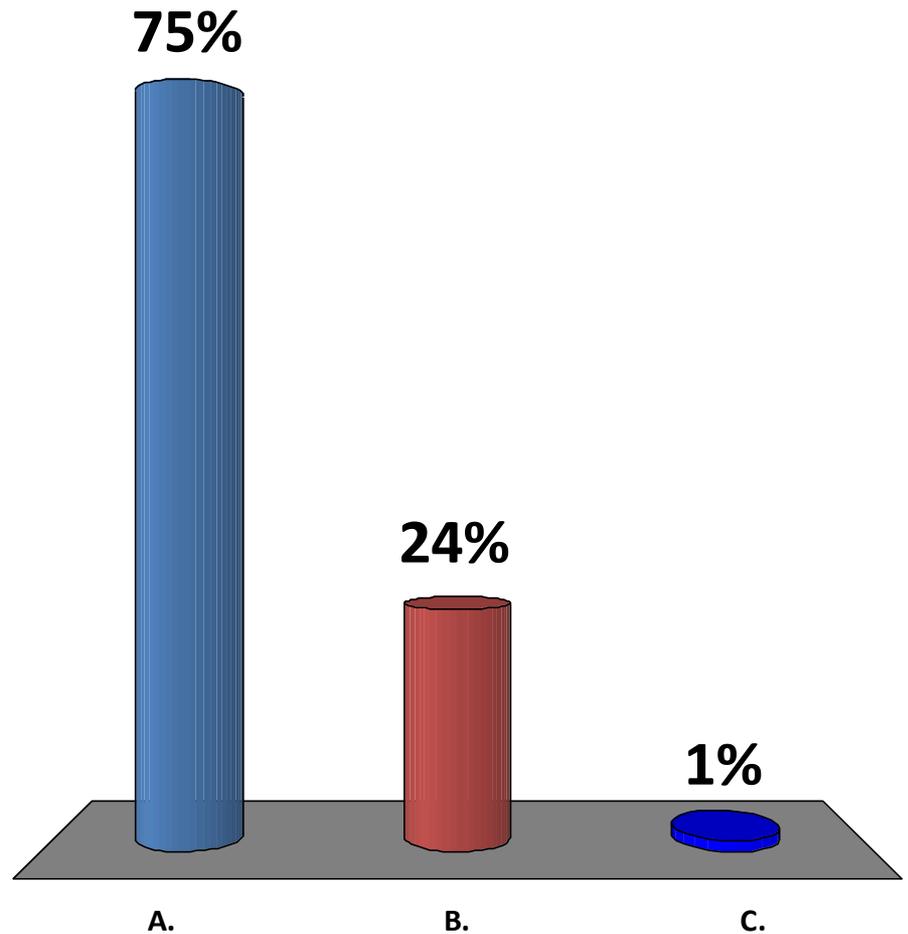
# What is your age?

- A. Younger than 6 years old
- B. 6 – 12
- C. 13 – 17
- D. 18 – 29
- E. 30 – 39
- F. 40 – 49
- G. 50 years old or older



# Do you identify as:

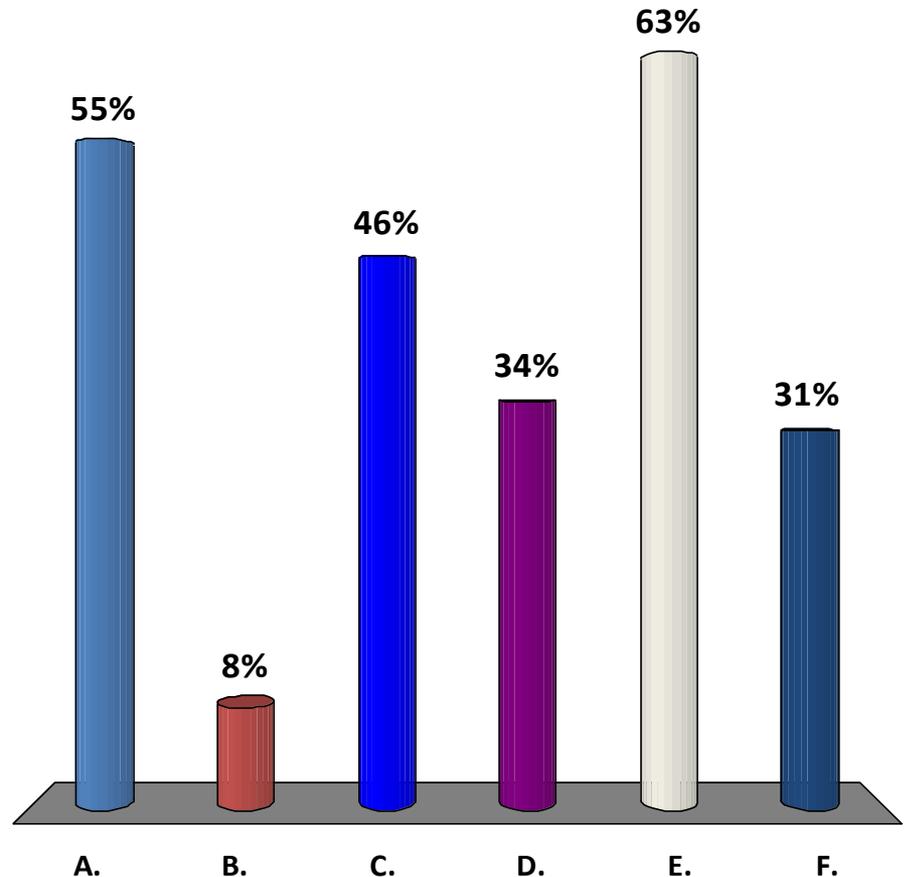
- A. Female
- B. Male
- C. Other



# Where is your alopecia areata located?

Check all that apply.

- A. Scalp
- B. Beard, side burns or moustache
- C. Eyebrows
- D. Eyelashes
- E. All areas
- F. Other areas not mentioned (such as nails)



# Discussion Topic 1

## Health Effects and Daily Impacts of Alopecia Areata

Meghana Chalasani  
Facilitator

September 11, 2017

# Topic 1 Panel Participants

- Elizabeth (Liz) DeCarlo
- Harrison and Sarah Evans
- Samantha Cunningham
- Deirdre Nero
- Megha Thyagarajan



Liz's High School Senior Photo

# Topic 1 Panel Participants

- Elizabeth (Liz) DeCarlo
- Harrison and Sarah Evans
- Samantha Cunningham
- Deirdre Nero
- Megha Thyagarajan



Harrison  
2011



First Haircut  
2012



Harrison with  
his dad Woody  
2014

# Topic 1 Panel Participants

- Elizabeth (Liz) DeCarlo
- Harrison and Sarah Evans
- Samantha Cunningham
- Deirdre Nero
- Megha Thyagarajan

Samantha



# Topic 1 Panel Participants

- Elizabeth (Liz) DeCarlo
- Harrison and Sarah Evans
- Samantha Cunningham
- Deirdre Nero
- Megha Thyagarajan



Deirdre



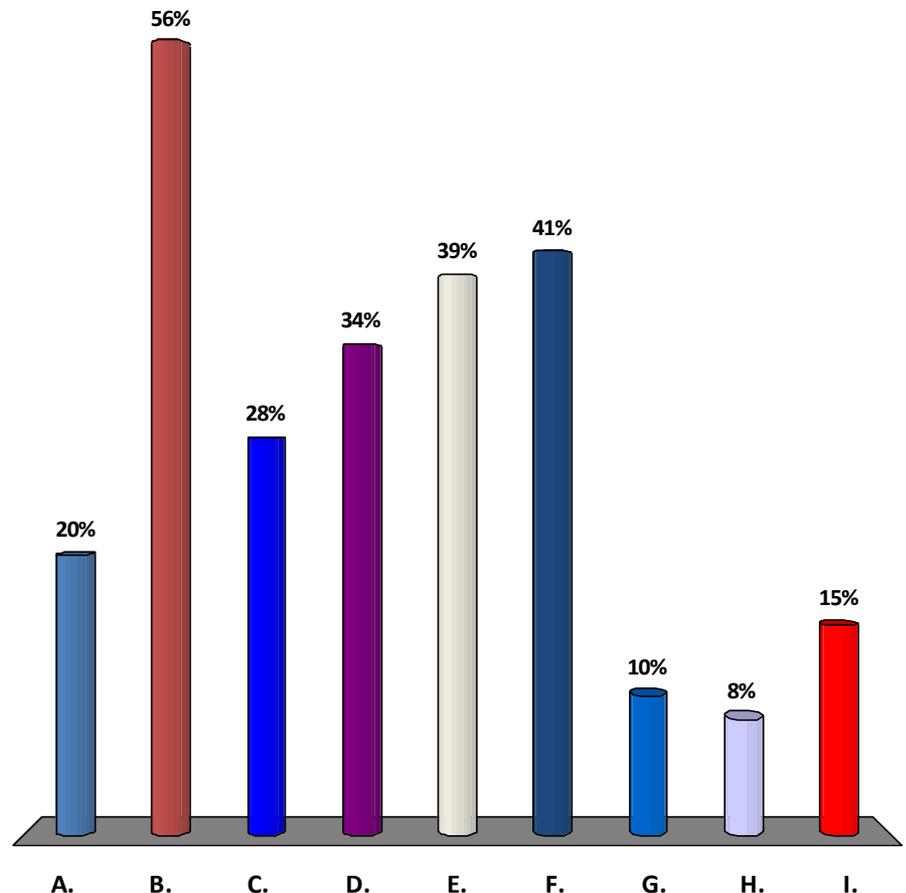
# Topic 1 Panel Participants

- Elizabeth (Liz) DeCarlo
- Harrison and Sarah Evans
- Samantha Cunningham
- Deirdre Nero
- Megha Thyagarajan

# PEDIATRIC AND YOUNG ADULT: What aspects of your alopecia areata are most bothersome to you?

**Please choose up to three answers.**

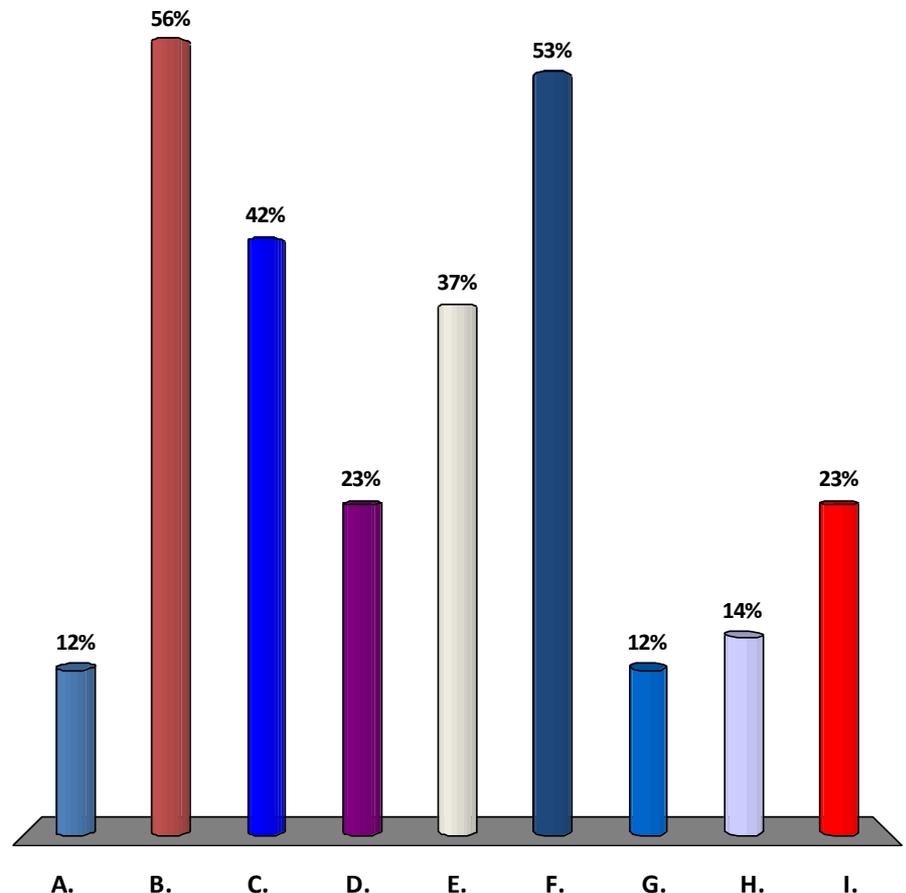
- A. Patchy hair loss
- B. Widespread hair loss
- C. Location of my hair loss
- D. Repeated episodes of hair loss and regrowth
- E. Unpredictability of when or where hair loss will occur
- F. Skin sensitivity (such as to sun, temperature, or sweat)
- G. Itching, burning or stinging
- H. Brittle, spotted, pitted, rough, or ridged nails
- I. Other health effects that may be associated (e.g., thyroid disease, vitiligo)



# ADULT: What aspects of your alopecia areata are most bothersome to you?

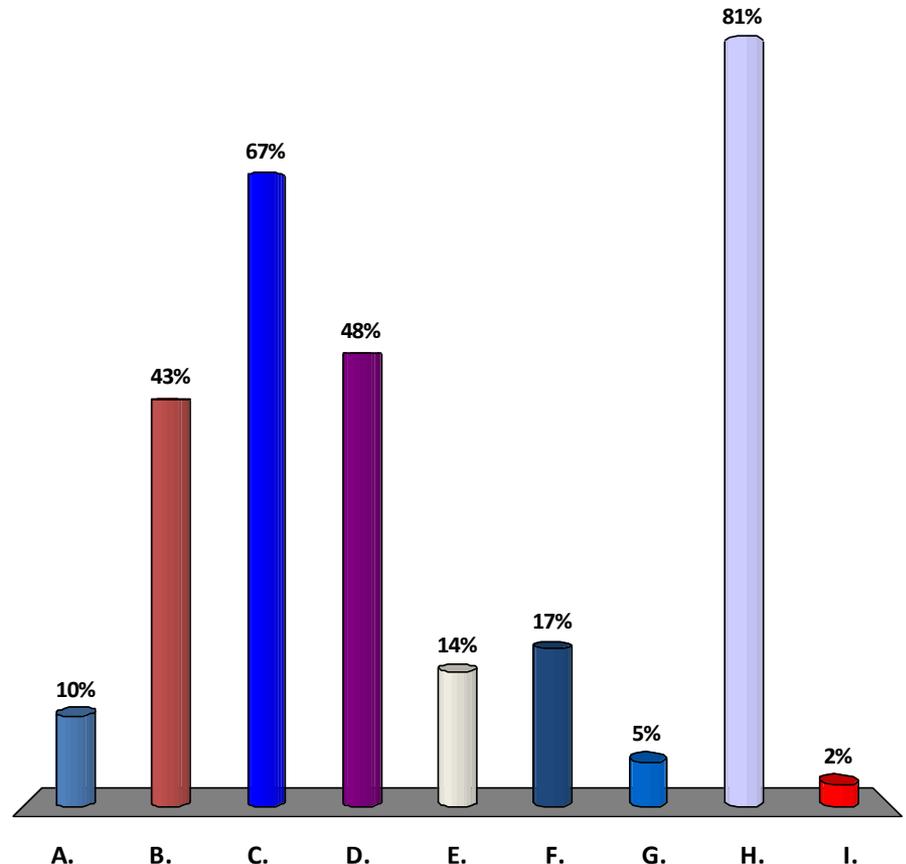
Please choose up to three answers

- A. Patchy hair loss
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- H. Brittle, spotted, pitted, rough, or ridged nails
- I. Other health effects that may be associated (e.g., thyroid disease, vitiligo)



# PEDIATRIC AND YOUNG ADULT: What do you find to be the most bothersome impacts of alopecia areata on your daily life? Please choose up to three answers.

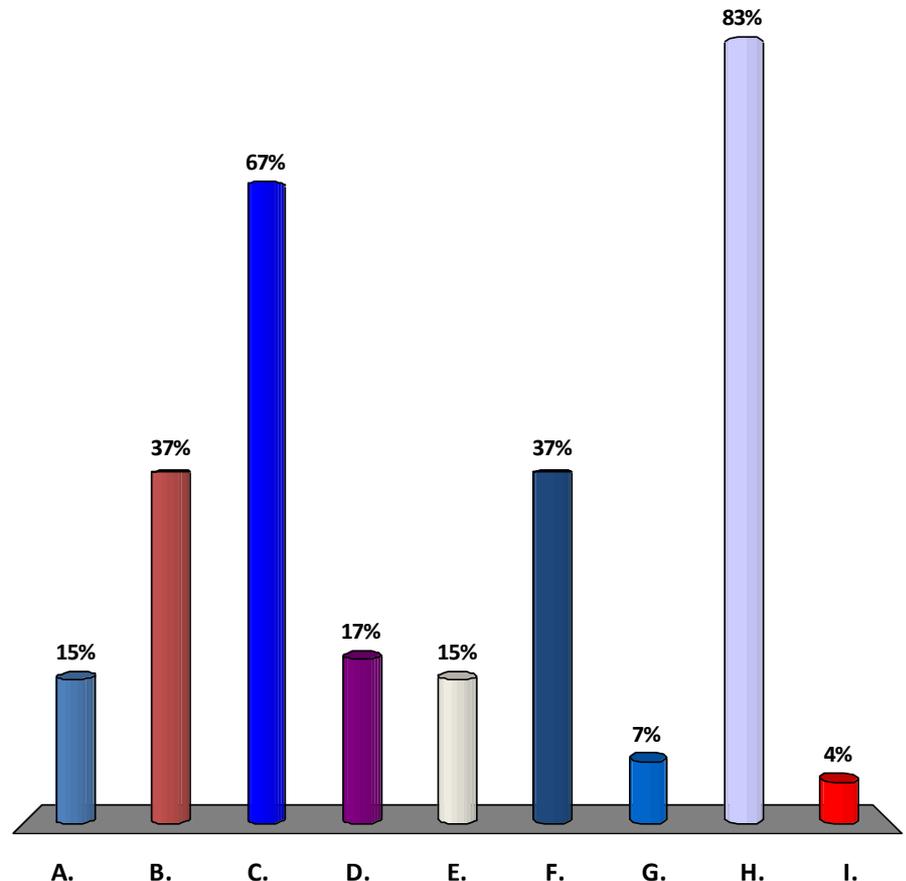
- A. Time or cost of daily maintenance
- B. Refraining from activities (such as school, work, sports, social activities)
- C. Self-consciousness or embarrassment
- D. Bullying or discrimination
- E. Impact on relationships with family and friends
- F. Impact on intimate relationships
- G. Physical impacts (such as pain or difficulty concentrating)
- H. Emotional or psychological impacts (such as anxiety, fear, depression, etc.)
- I. Other impacts not mentioned



# ADULT: What do you find to be the most bothersome impacts of alopecia areata on your daily life?

Please choose up to three answers.

- A. Time or cost of daily maintenance
- B. Refraining from activities (such as school, work, sports, social activities)
- C. Self-consciousness or embarrassment
- D. Stigma or discrimination
- E. Impact on relationships with family and friends
- F. Impact on intimate relationships
- G. Physical impacts (such as pain or difficulty concentrating)
- H. Emotional or psychological impacts (such as anxiety, fear, depression, etc.)
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# National Suicide Prevention Lifeline

1-800-273-8255

**BREAK**



# Patient-Focused Drug Development

**FDA Wants  
To Hear  
From Patients**

**FDA**

**U.S. FOOD & DRUG  
ADMINISTRATION**

# Discussion Topic 2

## Current Treatment Options

Meghana Chalasani  
Facilitator

September 11, 2017

# Topic 2 Panel Participants

- Katie Krueger
- Katie
- Tyrone Folliard-Olson
- Andrea Alberti
- Gracielle Palma



Katie when she was  
9 years old and  
at her First  
Communion

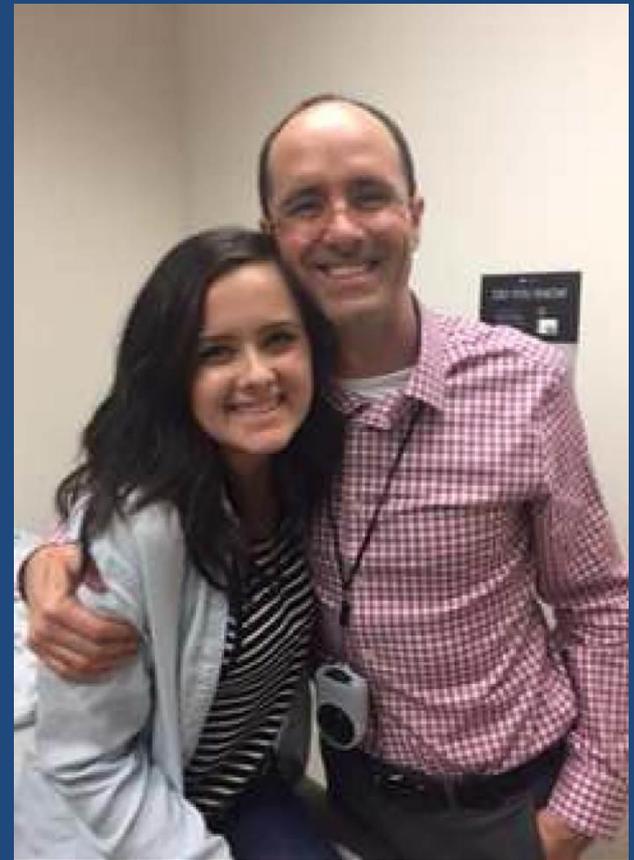
# Topic 2 Panel Participants

- Katie Krueger
- Katie
- Tyrone Folliard-Olson
- Andrea Alberti
- Gracielle Palma

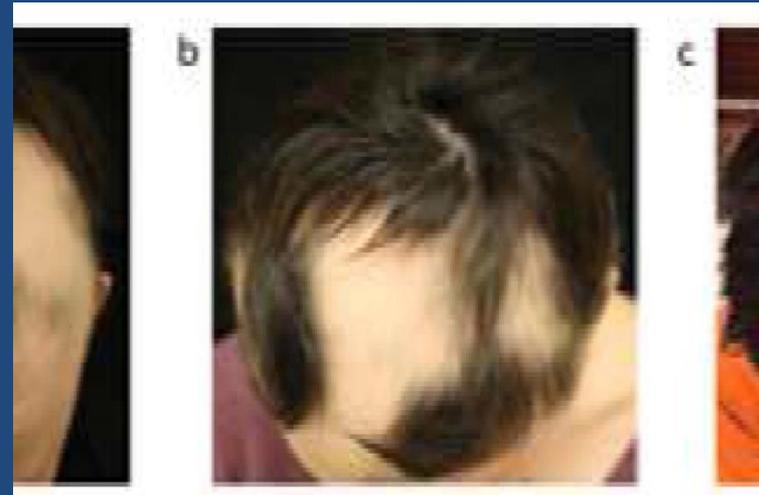
Katie



Katie with her physician Dr. King



Katie with treatment of Xeljanz



# Topic 2 Panel Participants

- Katie Krueger
- Katie
- Tyrone Folliard-Olson
- Andrea Alberti
- Gracielle Palma



Andrea

# Topic 2 Panel Participants

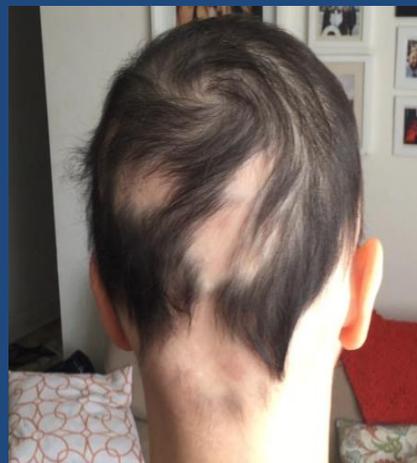
- Katie Krueger
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- Gracielle Palma



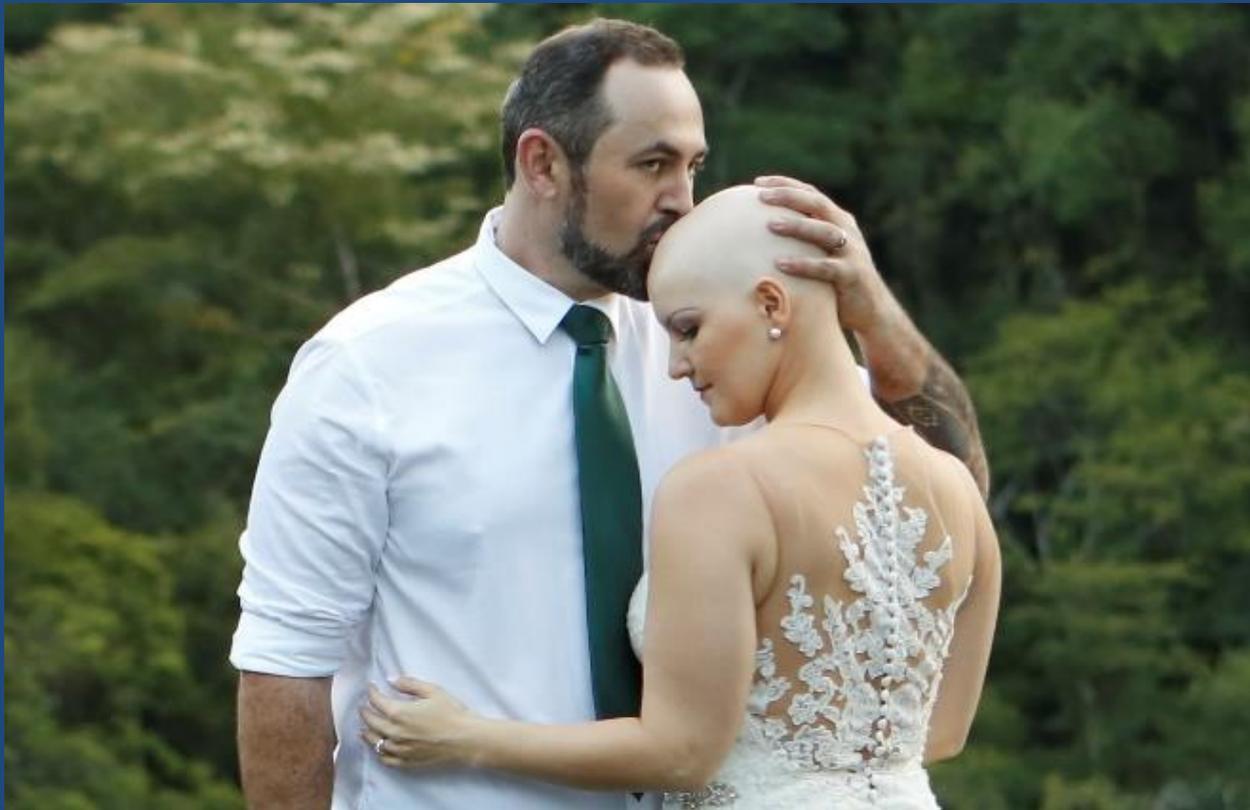
Sept 1998



Sept 2005



August 2017

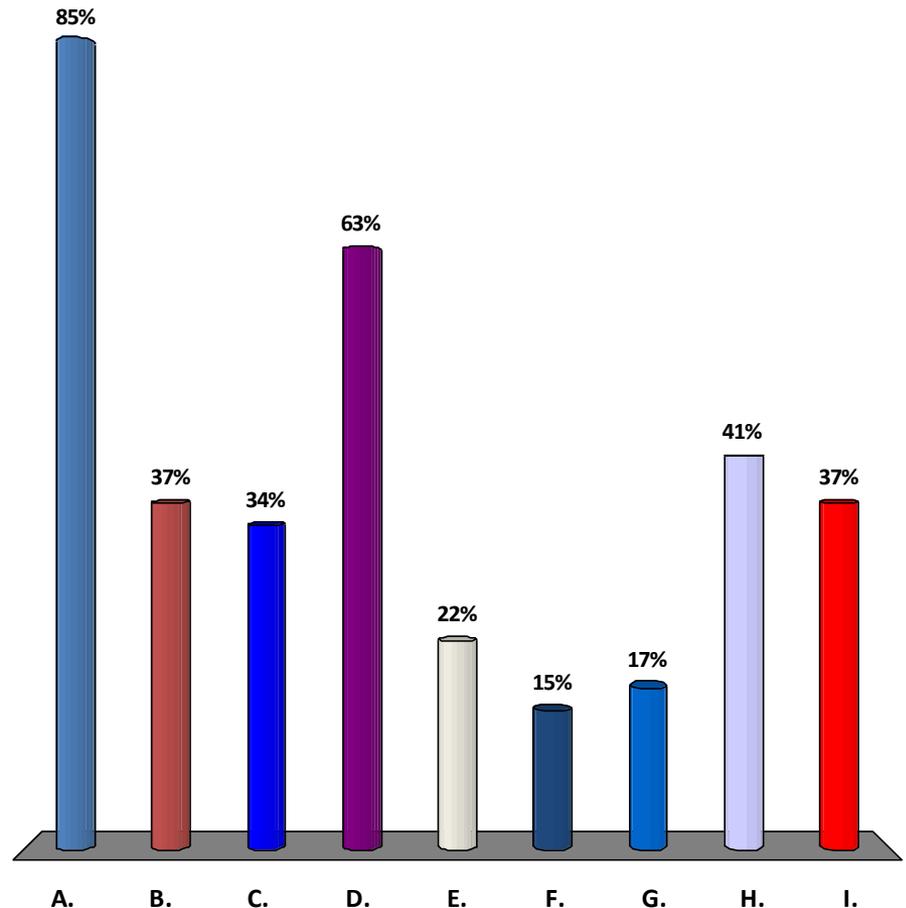


February 2017

Gracielle

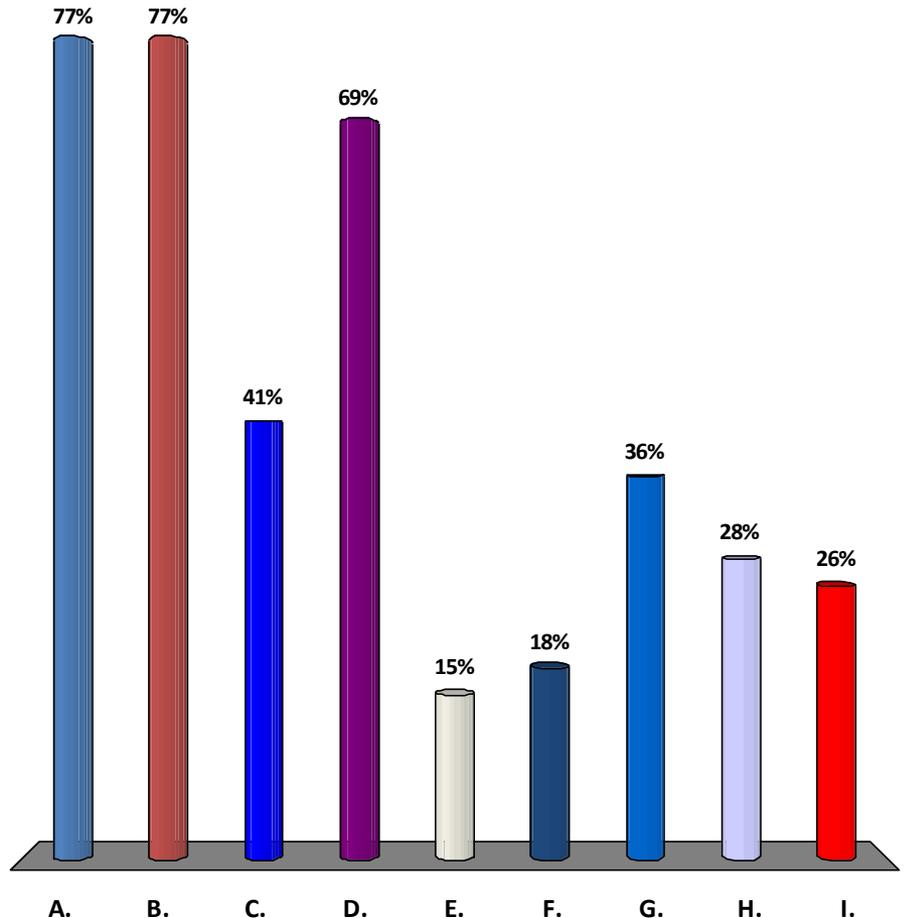
# PEDIATRIC AND YOUNG ADULT: Have you ever used any of the following drug therapies or medical devices to treat your alopecia areata? **Check all that apply.**

- A. Topical corticosteroids
- B. Injectable corticosteroids
- C. Oral corticosteroids
- D. Other topical treatments (such as minoxidil (Rogaine), anthralin, immunotherapy (such as diphencyprone/DPCP))
- E. Immunomodulatory therapies (such as Xeljanz, Jakafi, etc.)
- F. Light treatment (laser, phototherapy)
- G. Other prescription medicine (such as psychiatric or pain medication)
- H. Other drug therapies or medical devices not mentioned
- I. I'm not using any drug therapies or medical devices



ADULT: Have you ever used any of the following drug therapies or medical devices to treat your alopecia areata?  
**Check all that apply.**

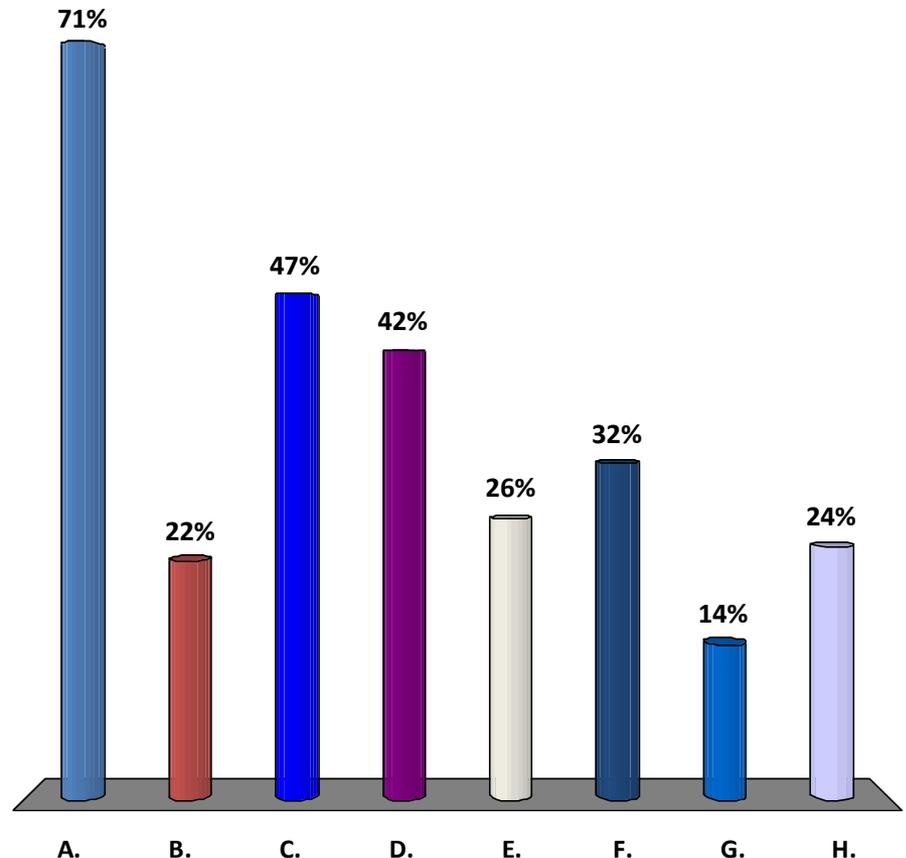
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- F. Light treatment (laser, phototherapy)
- G. Other prescription medicine (such as psychiatric or pain medication)
- H. Other drug therapies or medical devices not mentioned
- I. I'm not using any drug therapies or medical devices



Besides the therapies mentioned previously, what else are you doing to manage any symptoms or manifestations you experience because of your alopecia areata?

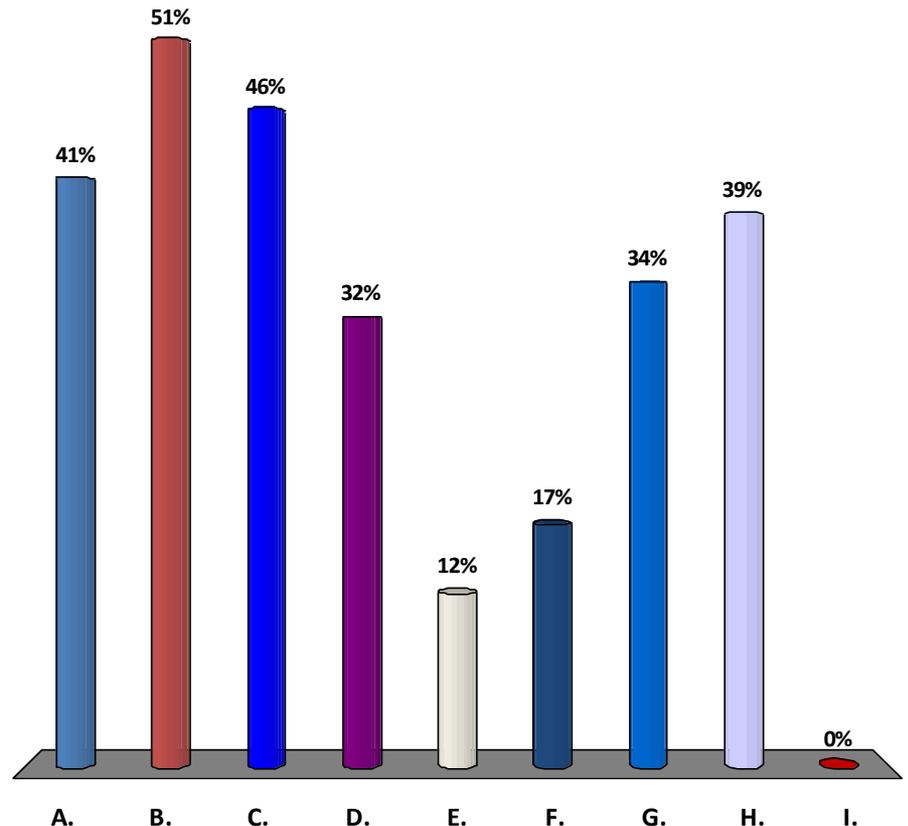
**Check all that apply.**

- A. Temporary cosmetic measures (e.g., wig, hat, hair weave, makeup, etc)
- B. Cosmetic procedures (such as hair transplants or permanent makeup)
- C. Dietary and herbal supplements
- D. Diet modifications
- E. Over the counter products
- F. Complementary or alternative therapies
- G. Other therapies not mentioned
- H. I am not doing or taking any therapies to treat my alopecia areata



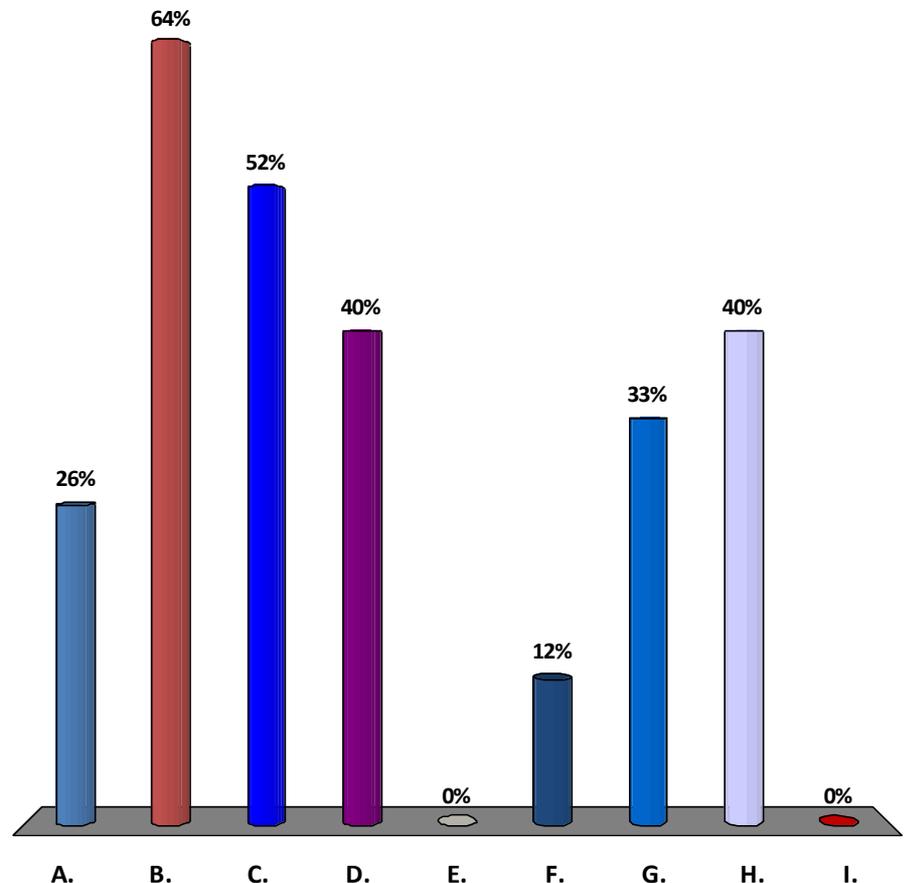
PEDIATRIC AND YOUNG ADULT: For the therapies you use, what do you consider to be the most burdensome downsides? Please choose up to three answers.

- A. How the medication is administered (such as a topical cream or an injection)
- B. Difficulty in accessing treatment (for example, insurance)
- C. The treatment only provides minimal benefit
- D. The treatment is effective only for a short-term
- E. Change in the texture or color of my hair
- F. Bothersome side effects of the treatment
- G. Concern about serious risks of the treatment
- H. Uncertainty about long-terms effects of treatment
- I. Other downsides not mentioned



# ADULT: For the therapies you use, what do you consider to be the most burdensome downsides? Please choose up to three answers.

- A. How the medication is administered (such as a topical cream or an injection)
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- H. Uncertainty about long-term effects of treatment
- I. Other downsides not mentioned



# Hypothetical Scenario

Imagine that you have been invited to participate in a clinical trial to study an experimental treatment for alopecia areata. Your doctor believes that you may be a good candidate for this medication.

This experimental treatment is a weekly self-injection. Early research in animals and people show that this treatment may reduce patchy hair loss on the scalp by up to 30% in some people. The purpose of this study is to better understand how well this treatment works and its safety.

More common side effects of this therapy may include fatigue, headaches, weight gain, sore throat, and gastrointestinal issues. Rarer but more serious side effects may include liver problems, cancer, stroke, infertility, or birth defects.

This clinical study will last 18 months and clinic visits will occur every month for the first 12 months, and once every 2 months in the remaining 6 months. Visits will involve routine blood work.

**What first thoughts come to mind as you hear this scenario?**

**What questions would you ask your doctor about this treatment?**

Based only on the information presented in the scenario,  
would you consider participating in this clinical trial?

**Please choose one response.**

- A. Yes, I would consider participating in this study
- B. No, I would not consider participating in this study
- C. I'm not sure

# Send us your comments!

- **You can send us comments through the “public docket”**
  - The docket will be open until November 13, 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-2017-N-3067-0001>

Or Search “alopecia areata FDA public meeting” 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 navigation bar with 'Home', 'Help', 'Resources', and 'Contact Us'. Below the navigation bar is a search bar with a magnifying glass icon and the text 'Advanced Search'. The main content area features a document titled 'Patient-Focused Drug Development for Alopecia Areata; Public Meeting; Request for Comments'. The document is issued by the Food and Drug Administration (FDA). A red arrow points from the text 'And Click Comment Now!' to a blue 'Comment Now!' button located on the right side of the document page. Below the button, there is a 'Comments' section showing '6 Comments Received' and a small text box with a testimonial: 'Right now as a regimen I've started now back up with a wonderful Rheumatologist, Dr. Robert M. Lissac, MD, PhD of...'

# Open Public Comment

Sara Eggers, PhD  
Office of Strategic Programs  
Center for Drug Evaluation and Research  
U.S. Food and Drug Administration

September 11, 2017

# Closing Remarks

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Center for Drug Evaluation and Research

U.S. Food and Drug Administration

September 11, 2017

