Patient Centered Outcome Research
&
Comparative Effectiveness Research

Health and Research Ambassadors
NAAF Annual Patient Conference, June 23, 2017
Learning Objectives

• **Understand Patient-Centered Outcomes Research (PCOR)**
  • What is it and why is it relevant to people with alopecia areata?
  • How does it differ from traditional research to find treatments?
  • Why is it important to the FDA, the biopharmaceutical industry and insurance companies now?
  • How PCOR connects to the upcoming Patient-Focused Drug Development meeting with the FDA

• **Understand Comparative Effectiveness Research (CER)**
  • What is it and why is it relevant to people with alopecia areata?
  • How will it help you, your doctors, the FDA and insurance companies make better decisions about the right care for you?

• **Understand why training and equipping our community to participate in all aspects of the research and care-delivery process is important**
  • To ensure that treatments developed are meaningful to you!
  • To equip you with tools for effective Shared Decision-Making with your doctors!

• **Understand NAAF’s Health and Research Ambassador (HARA) PCOR/CER training program**
  • Become interested and involved
Who Are We?

• Health and Research Ambassadors (HARAs) providing training in PCOR and CER
  • HARAs are patient advocates seeking to engage, inspire, and equip a new cadre of patient stakeholders ready to meaningfully participate in the research process
  • HARAs are individuals living with alopecia areata, caregivers or family members
  • HARAs work through local networks and venues such as NAAF Support Groups, academic medical centers, local hair salons or groups like the Rotary Club to share insights and training about PCOR/CER and its relevance to alopecia areata
  • The audience can be people living with alopecia areata, researchers, service providers from beauticians to dermatologists and immunologists, biopharmaceutical industry representatives, the FDA and health insurance providers.
What is Patient-Centered Outcomes Research (PCOR)?

PCOR:
research focused on
affecting and measuring
what matters most to patients
What is a Patient-Centered Outcome?

Blood Cancer Example

- **Reduce Spleen Size** – quantity that researchers and clinicians can measure
- **Reduce pain** – what industry guessed patients would care about
- **Reduce bloating and discomfort** – true patient-centered outcome
Why Are We Talking About It Now?!
Research on Alopecia Areata is Accelerating

Publications on “Alopecia Areata” per year over past 40 years

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Clinical Research on Alopecia Areata is Accelerating

Clinical Trials on Alopecia Areata Started Per Year (ClinicalTrials.gov)

- Clinical Trials Started Per Year

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Clinical Trials are increasing year by year.
Research on PCOR is Accelerating

Publications on “Patient-Centered Outcomes” per year

- Publications

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Natural Alopecia Areata Foundation
What’s driving focus on PCOR & CER?

National Academies

IOM 2001
Crossing the Quality Chasm

NRC 2011
Toward Precision Medicine

Healthy People 2020

From Advisory Groups

US Department of Health and Human Services

ODPHP 2000 and 2010

AHRQ 2011

From Within Us

Human Genome Sequenced 2003

Retail Genome and Whole Exome Sequencing for $99 - $499 in 2016
How are the government, insurance, research, and patients interconnected?

The Patient Drug User Fee Act (PDUFA) required the FDA to make sure patient voices would be considered in the risk/benefit analysis of medical therapy research.

The American Recovery and Reinvestment Act (ARRA) funds CER which encourages treatments for specific individuals. CER helps inform health care insurance company decisions on which treatments should be covered.

The Affordable Care Act (ACA) funds PCORI which conducts CER and prepares patients and advocates to engage in PCOR and CER such as funding this workshop for all of you!
Why PCOR Training NOW?
FDA (and therefore Industry) are required to listen to US Legislation requiring increasing patient engagement and patient-centered outcomes

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<td>American Recovery &amp; Reinvestment Act</td>
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<td>2008</td>
<td>Patient Protection &amp; Affordable Care Act</td>
<td>Patient-Centered Outcomes Research Institute (PCORI): Investigate comparative effectiveness Inform Medicare coverage decisions</td>
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What is a biomarker?

A measurable substance in an organism whose presence is indicative of some phenomenon such as disease, infection, or environmental exposure.
Example: Alopecia Areata Biomarker (ALADIN)

• Molecular measure of health
  • unaffected individuals (black)
  • people with patchy alopecia (green)
  • those with universalis and totalis (red)

• This is helpful in:
  • Disease Progression/Treatment Response
  • Select likely responders up-front
  • Affect insurance company coverage decisions
I’m not a researcher. How can I play a role in research?
• PDUFA is requiring progressively more patient engagement (guidance docs, FDA) such that patients are being included earlier and earlier in the research process.

• In 2010, the Affordable Care Act authorized PCORI to help advance patient-centered comparative clinical effectiveness research (CER) and disseminate the results in ways that the end-users will find useful and valuable.

• The **alopecia areata community** has the opportunity to take advantage of the legal framework to help our community.
  • This PCOR/CER training
  • Provide us your testimony
  • PFDD Meeting with FDA & Have a voice in PRO Instrument Development

• We are at the **beginning of alopecia areata clinical trials**. We guide the researchers into focusing on what we want.
The system we are aiming for

- All phases of basic and clinical research
- FDA Approval
- Insurance company coverage decisions

Biomarker data
Patient input
Easy-to-understand research

Informed partnership between the experts (shared decision-making)
What PCOR means to different stakeholders
What is Shared Decision Making?

Doctors
share information in light of patient desires

Patients
Share what is important to them and consider options

Together, they make a decision
Patient-Centered Outcomes Research (PCOR)

• Using your experiences as a patient to inform what and how research on your disease is performed.

• Partnering with researchers and collaborating to ensure that research questions and results are meaningful, understandable, and accessible to the patient community.

• Sharing decision-making with your doctor about treatments.

• Getting data on what you care about:
  • to the FDA when approving treatments
  • to Insurance companies making pay/no pay decisions
Answering patient-centered questions about:

• Expectations
  • “Given my personal characteristics, conditions, and preferences, what should I expect will happen to me?"

• Options
  • "What are my options, and what are the potential benefits and harms of those options?"

• Outcomes
  • "What can I do to improve the outcomes that are most important to me?"

• Decisions
  • "How can clinicians and the care delivery systems they work in help me make the best decisions about my health and health care?"
PCOR + Alopecia Areata

Angela M. Christiano

Vera H. Price, MD

A Patient like You

Health Insurance
How Clinical Development Works: From Trials to Treatment

**Discovery Science**
- Studies in the lab
- Studies in animal models

**Clinical Trials**
- **PHASE I:** study of a small number of patients to evaluate safety and dosing
- **PHASE II:** study of more patients to assess drug’s effectiveness and further evaluate safety
- **PHASE III:** study with the largest number of patients to confirm drug is effective and safe; assess side-effects and compare this drug to the current treatments
Patient Engagement Across the Clinical Trial Continuum

- Affecting what gets measured
- Help finalize eligibility criteria within the study protocol
- Assist in creating the informed consent form
- Advise on study recruitment
- Serve on study steering committee

- Serve on a Data Safety Monitoring Board
- Report on patient feedback regarding sites, investigators, and study experience

- Serve on FDA advisory committees
- Provide testimony at FDA hearings including the upcoming PFDD meeting for alopecia areata
Traditional Research

“Do you want to buy this drug?”
Patient-Centered OUTCOMES RESEARCH

Patient input throughout the research process
PCOR/CER

Leo/NAAF Focus Group

NAAF PRO Consortium

AA PFDDI Meeting
PCOR Tends to Be More Qualitative

- **Traditional** research tends to be **Quantitative**
  - Count the number of new hairs

- **Patient-Centered** research tends to be **Qualitative**
  - Ask if the patient is happy with their appearance considering the quality and placement of new hair.
Alopecia Areata PRO Consortium: Making a Validated Instrument

Alopecia Areata KOLs

Biopharmaceutical Industry Partners

Evidera

Evidence. Value. Insight.

National Alopecia Areata Foundation

FDA
How PRO/PCO’s Are Measured & The Importance of a Validated Instrument

Definitions of PRO/PCO

- Patient reported is NOT always patient centered
- Measure what is important to the patient. (FDA, ISPOR, PCOR)
- Patient-centered is the most important

Why need a validated instrument?

- To accurately measure meaningful change on items that matter to patients
- To demonstrate those changes to FDA for drug evaluation and approval
- To compare different treatments for healthcare decision-making
Review Key Difference with PCOR

- **Traditional** research tends to be

- **Patient-Centered** research tends to be
Comparative Effectiveness Research (CER)

- Designed to inform health-care decisions by providing evidence on the effectiveness, benefits, and harms of different treatment options
- The evidence is generated from studies that compare drugs, medical devices, tests, surgeries, or ways to deliver health care
- Ex: Steroid injections for alopecia areata
  - Clinical trial comparing the efficacy, safety, convenience of use for doctor & patient of two devices:
    1. Subcutaneous injection luer syringe
    2. DERMOJET (VS syringe without a needle)
The Importance of Comparative Effectiveness Research (CER)

• If you don’t get the best possible information about your treatment choices, you might not make an informed decision on what treatment is best for you.

• When choosing the right medicine or the best treatment, clear and dependable information can be difficult to find.

• Some treatments may not work for everyone, and some treatments may work better for some people than others. CER can help identify the treatments that may work best for you.

• CER reports often have information with pros & cons of all the options, so that you can make the best decision with your physician, given your unique experiences, medical history, and values (shared decision-making).
What can I do?
How YOU can get involved right now!

• Become a Health and Research Ambassador
• Become a Legislative Liaison
• Participate in the Patient Focused Drug Development Initiative
• Respond to Patient Reported Outcomes Survey
• Participate in clinical trials
• Stay tuned!
Become a HARA

• Health and Research Ambassadors (HARAs) providing training in PCOR and CER
  • HARAs are patient advocates seeking to engage, inspire, and equip a new cadre of patient stakeholders ready to meaningfully participate in the research process
  • HARAs are individuals living with alopecia areata, caregivers or family members
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  • The audience can be people living with alopecia areata, researchers, service providers from hairstylist to dermatologists and immunologists, biopharmaceutical industry representatives, the FDA and health insurance providers.

For more information contact Maureen Smith: Maureen@naaf.org
Become a Legislative Liaison
Legislative Liaisons Do What?

- Contacting local representatives
- Getting involved with bill development and education
- Bringing awareness to policies related to alopecia areata
- For more information, contact Gary Sherwood: Gary@naaf.org
NIH Annual Investment in Alopecia Areata

NIH Investment in Alopecia Areata Funding has increased 579% through efforts of NAAF Legislative Liaisons and personal meetings between NAAF staff, AA Key Opinion Leaders and NIAMS staff.

Mostly due to the increasing amount of Excellent Research that is worthy of NIH funding.

2016 includes $1.6 Million for year one of a 5-year Alopecia Areata Center for Research Translation (AACORT) grant to Columbia:
Provide testimony for the FDA Patient-Focused Drug Development meeting

Alopecia areata was selected as one of 39 diseases out of 12,000 known conditions having an unmet need and, in our case, no FDA-approved drug, and therefore deserving of PFDDI consideration!

Our meeting with the FDA will take place September 11, 2017 in Washington DC!

Register: [https://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm554443.htm](https://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm554443.htm)
Respond to the Alopecia Areata PFDD Survey

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

1. Of all the symptoms that you experience because of your condition, which 1-3 symptoms have the most significant impact on your life?

2. 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?

3. How do your symptoms and their negative impacts affect your daily life...
   - On the best days?
   - On the worst days?

4. How have your condition and its symptoms changed over time?
   - Would you define your condition today as being well managed?

5. What worries you most about your condition?

research.net/r/NAAF_PFDD
Respond to the Alopecia Areata PFDD Survey

**Topic 2: Patients’ perspectives on current approaches to treatment**

1. What are you currently doing to help treat or manage your condition or its symptoms?
   - How has your treatment regimen changed over time, and why?

2. How well does your current treatment regimen control your symptoms?
   - How well do your treatments address your specific symptoms?
   - Which symptoms are not addressed as well?
   - How well have these treatments worked as your condition has changed over time?

3. What are the most significant downsides to your current treatments, and how do they affect your daily life?

4. Since there is no cure or FDA approved treatment for alopecia areata, what specific things would you look for in an ideal treatment for your condition?
   - What would you consider to be a meaningful improvement (symptom or functional) in your condition that a treatment could provide?

research.net/r/NAAF_PFDD
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PFDD Meeting Outcomes

• Each meeting results in a “Voice of the Patient” Report
• Helps the FDA conduct a systematic Benefit-Risk Assessment for products under review
• Provide guidance for drug sponsors on their drug development programs
• It might also support drug development more broadly:
  • Help identify areas of unmet need within the particular patient population
  • Help develop tools that assess benefit of potential future therapies
  • Help raise awareness within the patient community (ie NAAF-Alopecia community)
PFDD Meeting - How You Can Get Involved

• Respond to the Alopecia Areata PFDD Survey!
  research.net/r/NAAF_PFDD

• Provide written testimony for the FDA PFDD Meeting!
• Join us for the PFDD Meeting on September 11, 2017

For more information, contact:
Gary Sherwood Gary@naaf.org or Maureen Smith Maureen@naaf.org
Participate in Clinical Trials that are Recruiting

- **Efficacy Study of a Cosmetic Lotion in the Treatment of Alopecia Areata in Males in Females** (MEXIS/M6S Patent – lotion against alopecia)
- **Topical Garlic Concentrate for Alopecia Areata in Children** (Garlic concentrate)
- **Excimer Light for Alopecia Areata** (UVB excimer light)
- **Intralesional Steroids in the Treatment of Alopecia Areata** (Intralesional Triamcinolone 2.5, 5, 10 mg/ml; Placebo)
- **A Pilot Study of Tralokinumab in Subjects with Moderate to Severe Alopecia Areata** (Tralokinumab vs. Placebo)
- **Pilot Study of the Safety and Efficacy of Apremilast in Subjects with Moderate to Severe Alopecia Areata** (Apremilast vs. Placebo)
- **Study to Evaluate the Efficacy and Safety Profile of PF-06651600 and PF-06700841 in Subjects with Alopecia Areata** (Comparing two JAK inhibitors with Placebo)
- **PAI-1 Expression in Non-scarring Hair Loss** (observational case-control study comparing PAI-1 levels in people with AA and controls without AA)
- **The Efficiency of the Methotrexate at Patients affected by Grave Pelade** (comparing low doses of methotrexate, methotrexate combined with prednisone, and placebo)
Participate in Clinical Trials that are recruiting

- Studies enrolling on ClinicalTrials.Gov
  - Anti IL-17 - Tralokinumab
  - PDE4 Inhibitor - Apremilast
  - Cosmetic Lotion
  - Topical Garlic Concentrate in Children
  - Excimer Light
  - Intraleisonal Steroids vs Placebo
  - Comparing two JAK inhibitors with Placebo
  - JAK3 inhibitor
  - PAI-1 Biomarker Expression
  - Biocellular-Cellular Regenerative Treatment with adipose and PRP
Stay tuned for CER in Alopecia Areata!

Compare patient satisfaction with

• dermatology office visits that address the emotional and mental health burden-
• office visits that only address the hair loss.
Summary of what we learned today—ways to get involved!

- Become a Health and Research Ambassador
- Become a Legislative Liaison
- Patient-Focused Drug Development
  - Fill out the NAAF’s PFDD Survey by June 30th
    research.net/r/NAAF_PFDD
  - Participate in the Meeting September 11th
- Be interviewed on Patient Reported Outcomes
- Participate in clinical trials
- Stay tuned!
Questions?

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