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Welcome Back!
Lupus Research Action Network: Day 2

October 18, 2020

Today's Agenda

- Recap of Day 1
- History of the Patient Voice
- The Importance of the Patient Perspective in Lupus Drug Development and Clinical Trials
  - Patient Focused Drug Development
  - RAY: Research Accelerated by You
- Advocating to Support Research
- Speaking About Research
- Recap and Opportunities to Take Action

DAY ONE RECAP

DAY 1

- Lupus patient experience – symptoms, treatments, we're all different
- Types of research
- Participating in clinical trials
  - What it means & how to talk about it
- Frustration, but Hope
- Patient experience in research

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The Patient Voice

Evolution of the Patient Voice

- 1980's & 1990's: Patient and disability communities demanded their voices be heard
  - Expanded Access
  - Patient Representative Program
- 1990's & 2000's: Accelerating approvals
  - Prescription Drug User Fee Act (PDUFA)
  - Priority Review

Evolution of the Patient Voice

- 2012-2020: New age of patient-centricity
  - Fast Track, Accelerated Approval, Breakthrough Therapy
  - FDASIA, 21st Century Cures Act
  - Patient Focused Drug Development
    - Patient input to inform drug development and regulatory decision-making

- 2020: The science of patient engagement
  - All stakeholders, defining how and putting it into practice throughout the lifecycle
    - Clinical trials and trial design, outcomes, benefit-risk, burden of disease and treatment, unmet need, quality of life, RWE…the Patient Experience

There is Consensus

- All stakeholders agree…
  - Patients are experts with unique insights who should be treated as partners in research
  - Meaningful engagement with patients works and has led to significant advances in drug development

- Your voice is critical to improving lupus drug development
  - Congress, FDA, industry, clinicians and researchers
  - Patients can drive priorities

Plenty of Work Remains

- Lupus clinical trial participation remains a challenge, especially as it relates to diversity
- Education on the importance of clinical trials and lupus research is needed
- Improving health disparities is critical to success
- Advocacy – funding and policy

LRAN – Opportunities!

- The importance of the patient perspective in lupus drug development and clinical trials
  - Patient-Focused Drug Development
  - LFA's Research Accelerated by You (RAY)
- Advocacy
- Speaking about research
- Recap and opportunities for you to take action

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The Importance of the Patient Perspective in Lupus Drug Development and Clinical Trials

Patient-Focused Drug Development

Overview

- Key Terms
- FDA's Patient Focused Drug Development (PFDD) program
- Lupus Patient Focused Drug Development
- Opportunities For You

Key Terms

- Patient centered
- Meaningful patient engagement
- Patient-focused drug development (PFDD)
- Patient-reported outcome (PRO)
- Real-world data (RWD) and real world evidence (RWE)

Patient Centered

- Any process, program or decision focused on patients in which patients play an active role as meaningfully engaged participants, and the central focus is on optimizing use of the patient perspective

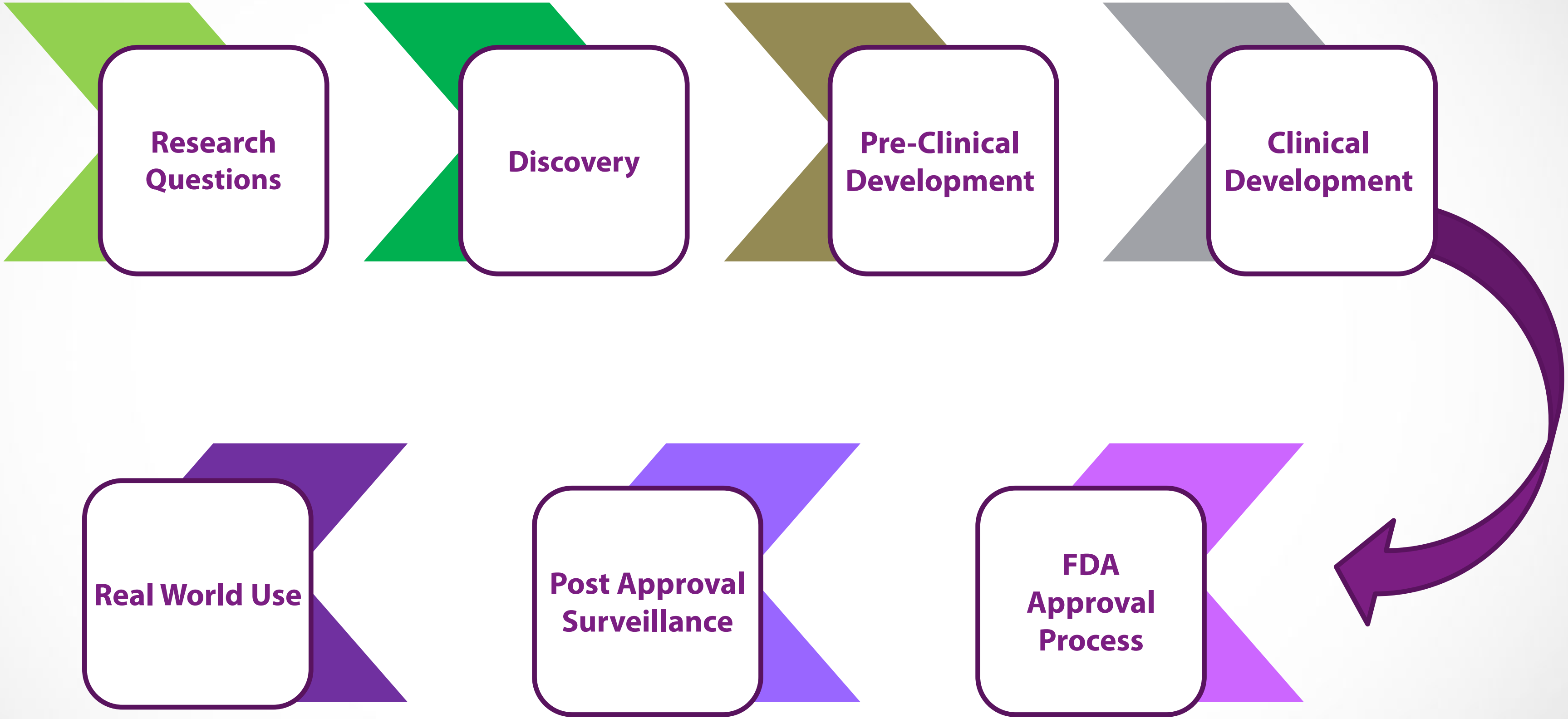
- Doing things <u>**with**</u> -- not **for** or **to –** patients

Meaningful Patient Engagement

- A bi-directional relationship between a patient(s) and another stakeholder(s)
- Characteristics
  - Reciprocal
  - Co-learning
  - Co-development
  - Partnership
  - Trust
  - Transparency
  - Honesty
  - Respect
- Communications are open, honest and clear
- Goals, participants, methods, desired impacts and actual impacts are clearly outlined and transparent

Patient-Focused Drug Development

- The meaningful engagement of patients in the research and development of therapeutic products and the various important roles patients can play in improving the processes



Research Questions

Discovery

Pre-Clinical Development

Clinical Development

FDA Approval Process

Post Approval Surveillance

Real World Use

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Patient-Reported Outcome

- An outcome measure based on a report that comes directly from the patient (e.g., study participant) about the status of the patient's health condition
  - No interpretation from others

Real World Data & Real World Evidence

- RWD = the data from the real world use
    - Electronic health records (EHRs)
    - Claims and billing
    - Registries

- RWE = turning RWD into something meaningful that can help in decision making
    - Clinical evidence from the analysis of RWD


Patient-Focused
Drug Development
FDA Wants
To Hear
From Patients
FDA
U.S. FOOD & DRUG
ADMINISTRATION

FDA Recognition

What is Patient-Focused Drug Development?

Patient-focused drug development (PFDD) is a systematic approach to help ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation. As experts in what it is like to live with their condition, patients are uniquely positioned to inform the understanding of the therapeutic context for drug development and evaluation.

FDA's PFDD Initiative

- Established in 2012
- Goal: Better incorporate the patient's voice in drug development
- Helped accelerate the adoption of patient engagement
- Continues to evolve

Input Throughout Drug Development

- Research agenda
- Development of research questions
- Clinical trial design
- Selection of outcomes
- Recruitment
- Translation and dissemination of results
- Real-world understanding

Lupus Patient-Focused Drug Development

- Listen and partner with YOU
- Externally-led lupus PFDD meeting
- Shape policy
- Data collection

Burden of Disease

How does lupus affect the people living with it? What are the biggest challenges of coping with lupus? Which symptoms are the most difficult to manage?

Burden of Treatment

People with lupus take an average of 8 drugs to manage their disease. Which side effects are the worst? How difficult is it to manage the medications?

Medicine Benefits and Risks

Every medicine comes with benefits and risks. For people with lupus, what is the right balance? How much risk is acceptable for a moderate benefit?

Clinical Trials

What prevents people with lupus from enrolling in clinical trials? How can clinical trials be designed to make lupus patients want to participate?

Lupus PFDD Meeting (2017)

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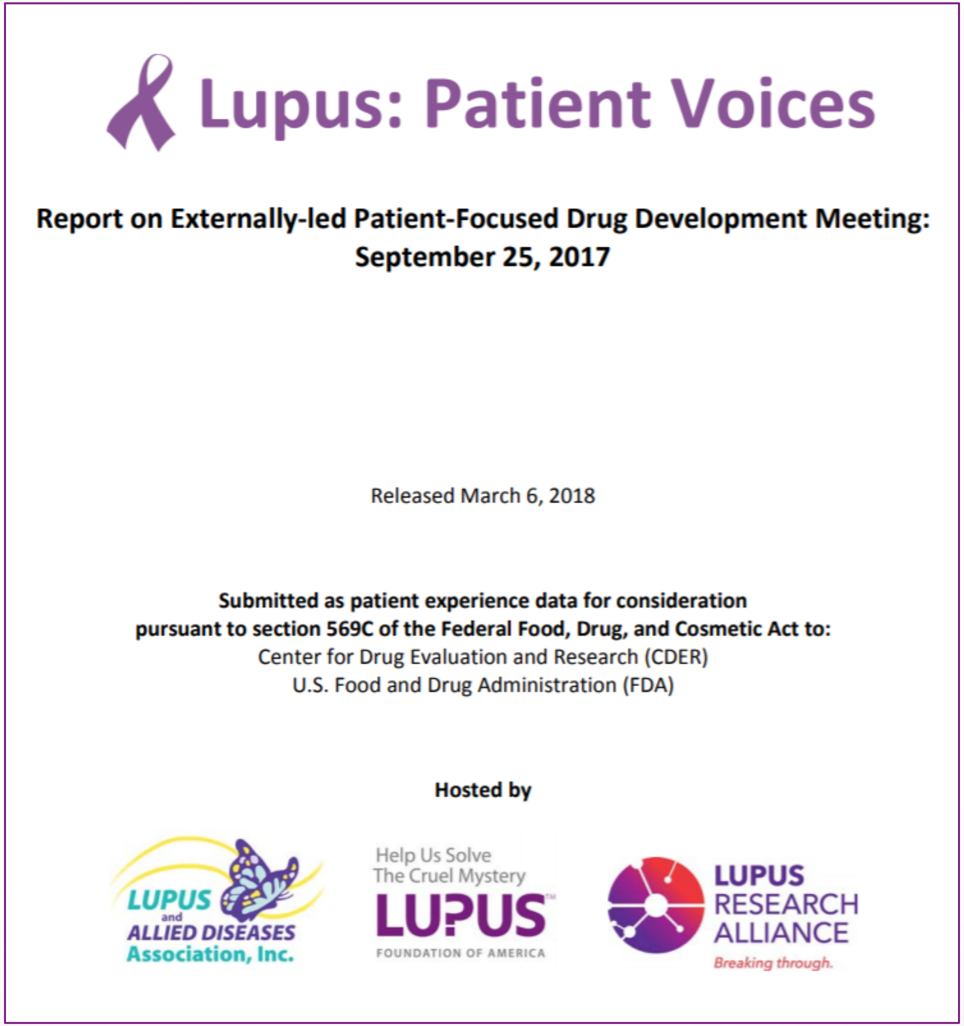

Lupus: Patient Voices

Report on Externally-led Patient-Focused Drug Development Meeting: September 25, 2017

Released March 6, 2018

Submitted as patient experience data for consideration
pursuant to section 569C of the Federal Food, Drug, and Cosmetic Act to:
Center for Drug Evaluation and Research (CDER)
U.S. Food and Drug Administration (FDA)

Hosted by

LUPUS and ALLIED DISEASES Association, Inc.

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LUPUS RESEARCH ALLIANCE
Breaking through.

Policy: Providing the Lupus Perspective

- FDA Patient-Focused Drug Development (PFDD) Guidance Series for Enhancing the Incorporation of the Patient's Voice in Medical Product Development and Regulatory Decision Making
- Benefit-Risk
- Real-World Evidence
- Enhancing the Incorporation of the Patient Perspective in Clinical Trials
- Diversity in Clinical Trials
- Prescription Drug User Fee Act (PDUFA) VII

Opportunities

- FDA Patient Representative Program
- Department of Defense Consumer Reviewers
- LFA Research and Advocacy Initiatives
- RAY: Research Accelerated by You

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RAY: Research Accelerated by You

Overview

- Purpose
- What is a Patient Registry?
- RAY: Research Accelerated by You
- Benefits

The purpose of this registry is to improve our understanding of lupus while engaging you in research opportunities that matter to <u>you</u>

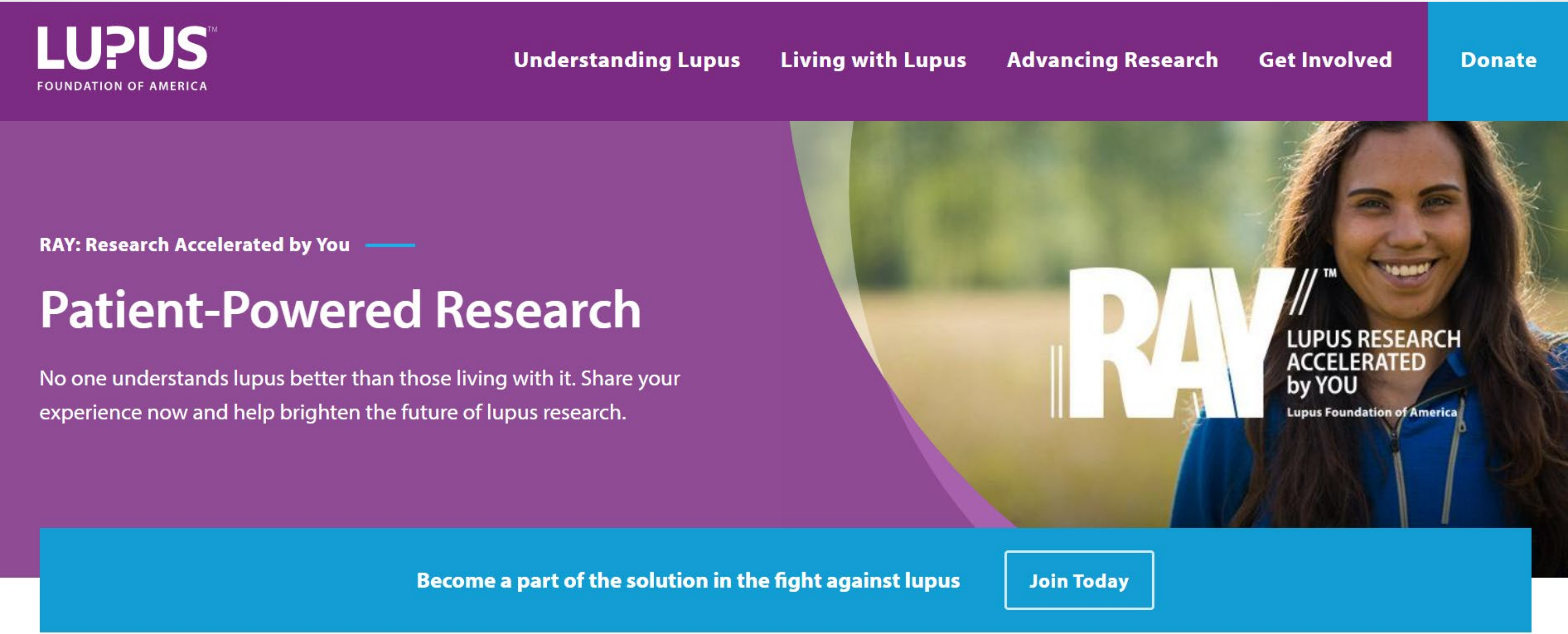
What is a Patient Registry?

- A place to stored detailed information about individuals with a specific disease

- LFA has built a patient-powered registry meaning all of the information generated comes directly from people living with lupus and their families

Steering Committee

- Persons with lupus and caregivers
- Scientific academic leaders
- Biopharmaceutical industry



RAY: Research Accelerated by You

Patient-Powered Research

No one understands lupus better than those living with it. Share your experience now and help brighten the future of lupus research.

Become a part of the solution in the fight against lupus

What is it?

- An online set of questions for you to answer about your lupus experience and preferences
- A platform to collect and store information about your health, including your lupus and medical history
- A platform that combines your information with other participants' to help us learn more
- A place where you can learn about new research opportunities

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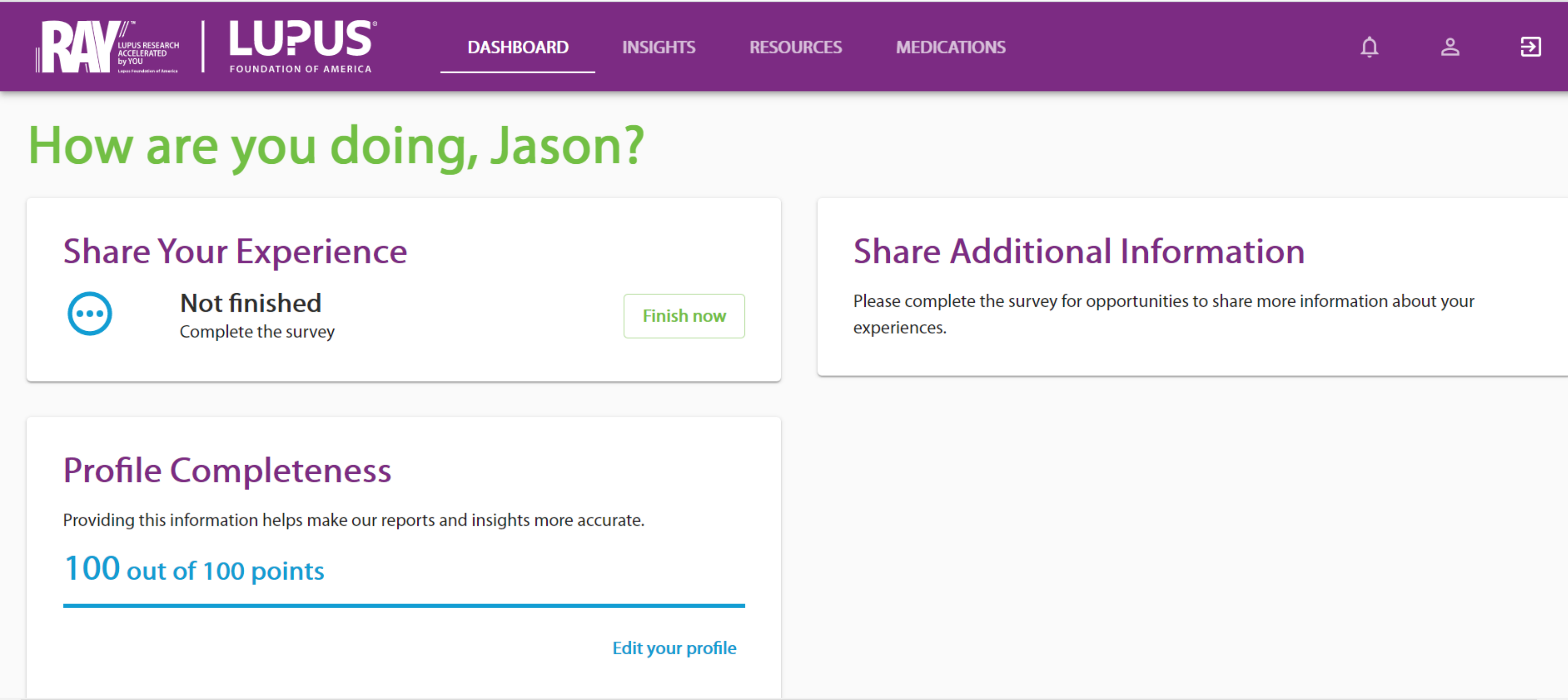
How does it work?

- Once you join, it allows us to provide you with the most up-to-date information about research that may interest you
  - We tailor the information we send you based on the information you provide
- Helps researchers incorporate the patient perspective every step of the way
  - The Registry can help identify people that may be eligible to participate in studies, and so much more

Data Security and Privacy

- We take this very seriously!
- We will never share your contact information with anyone outside of LFA
- Your personal information will be associated with a unique code that is specific to you
- Only LFA and our Registry team will be able to link the code with your contact information
- LFA-approved partners and/or researchers will only be provided access to de-identified, anonymous information



How are you doing, Jason?

Share Your Experience

Not finished

Complete the survey

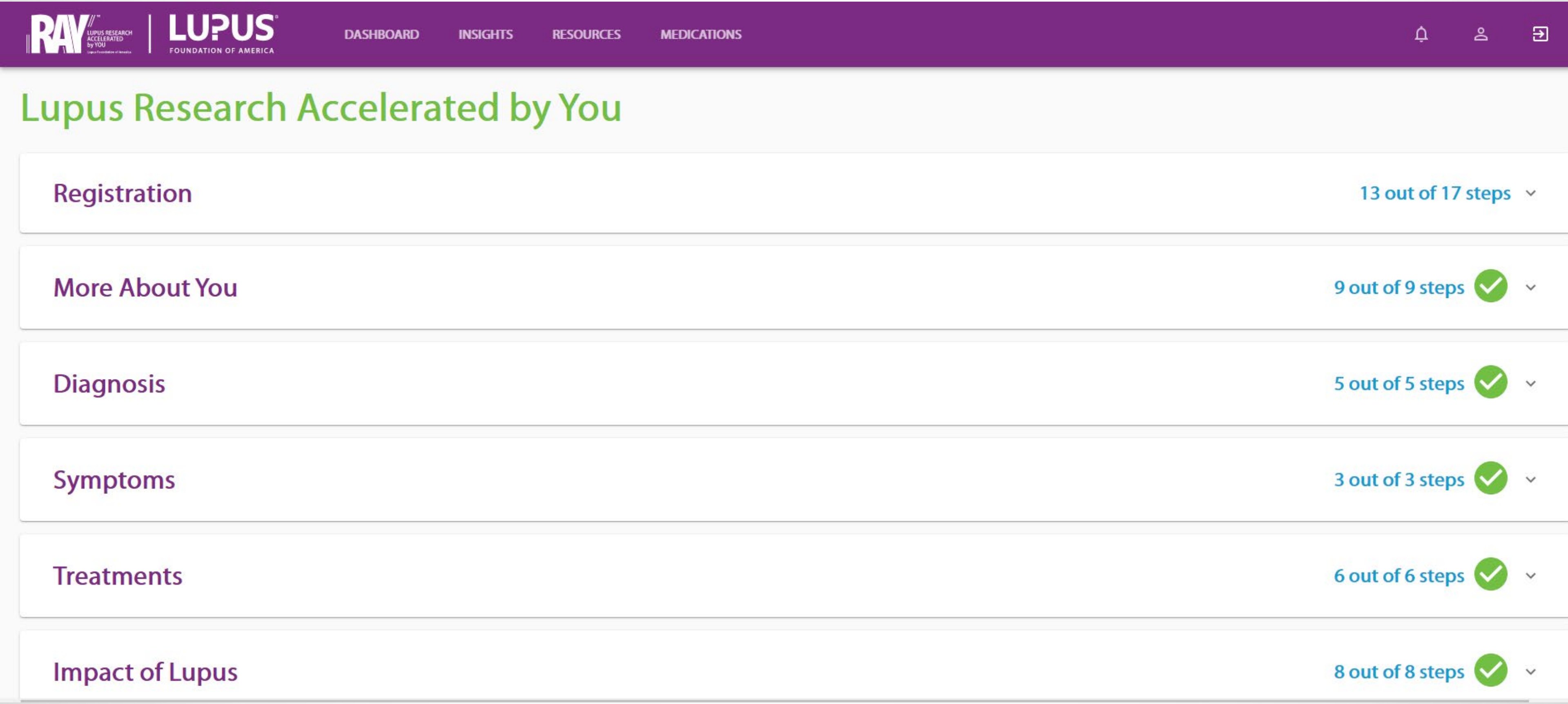
Share Additional Information

Please complete the survey for opportunities to share more information about your experiences.

Profile Completeness

Providing this information helps make our reports and insights more accurate.

100 out of 100 points



Lupus Research Accelerated by You

Registration: 13 out of 17 steps

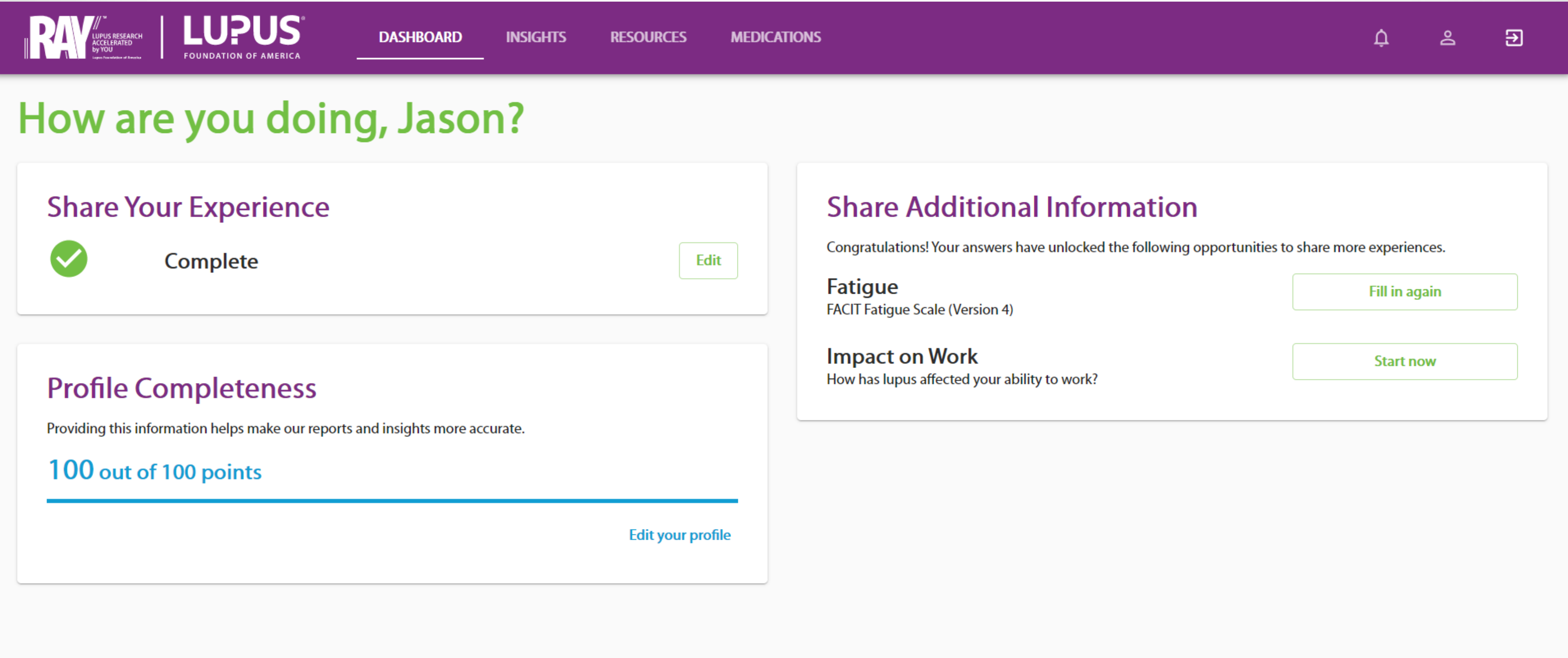
More About You: 9 out of 9 steps ✓

Diagnosis: 5 out of 5 steps ✓

Symptoms: 3 out of 3 steps ✓

Treatments: 6 out of 6 steps ✓

Impact of Lupus: 8 out of 8 steps ✓



How are you doing, Jason?

Share Your Experience

✓ Complete

Profile Completeness

Providing this information helps make our reports and insights more accurate.

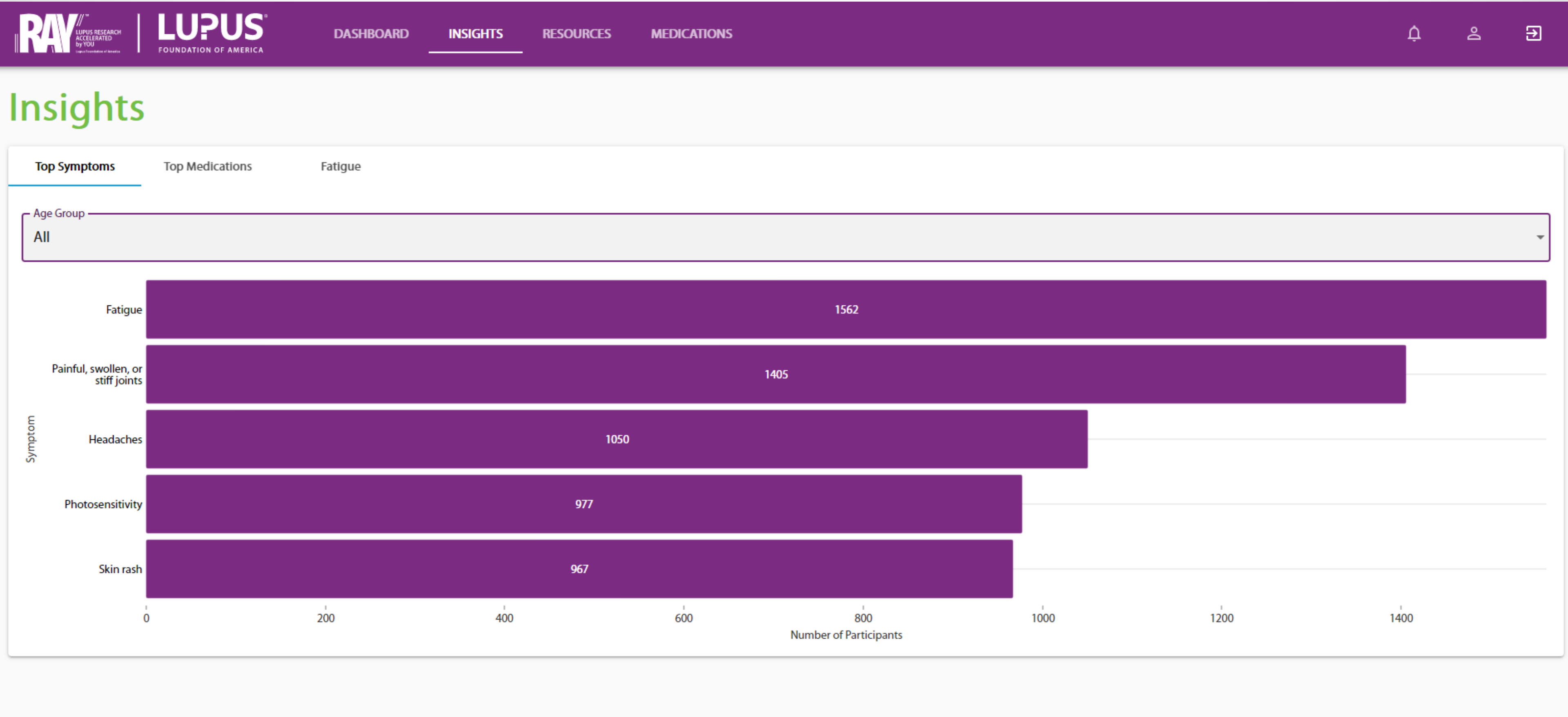
100 out of 100 points

Share Additional Information

Congratulations! Your answers have unlocked the following opportunities to share more experiences.

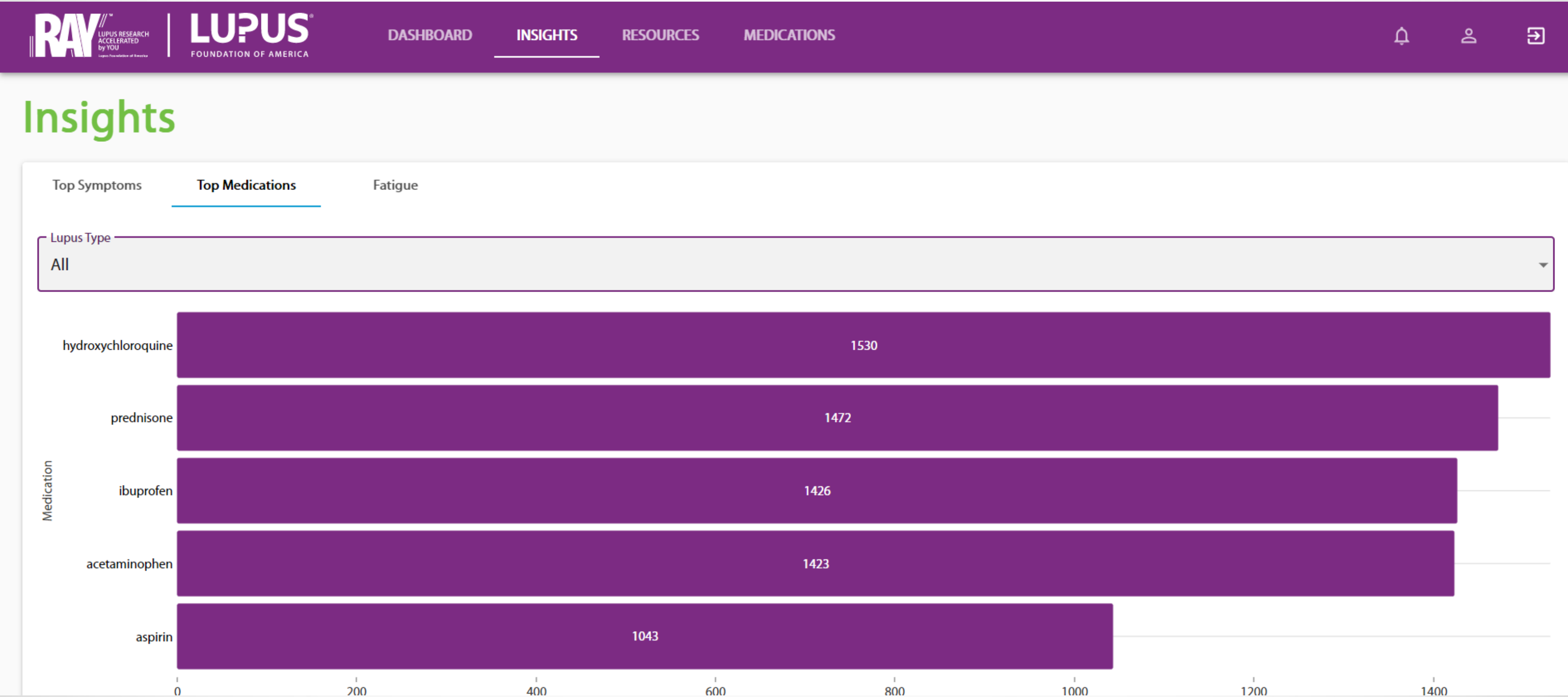
Fatigue
FACIT Fatigue Scale (Version 4)

Impact on Work
How has lupus affected your ability to work?

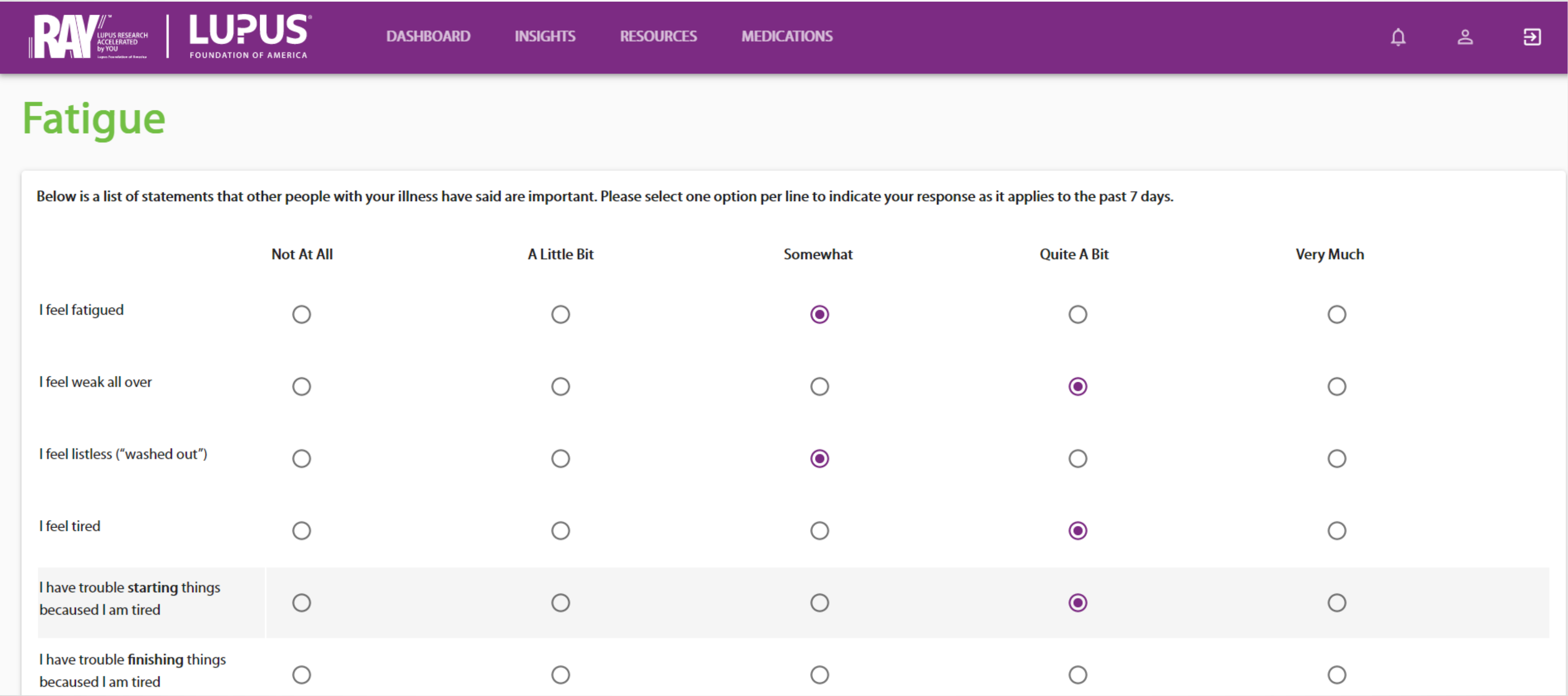


Insights

Age Group: All



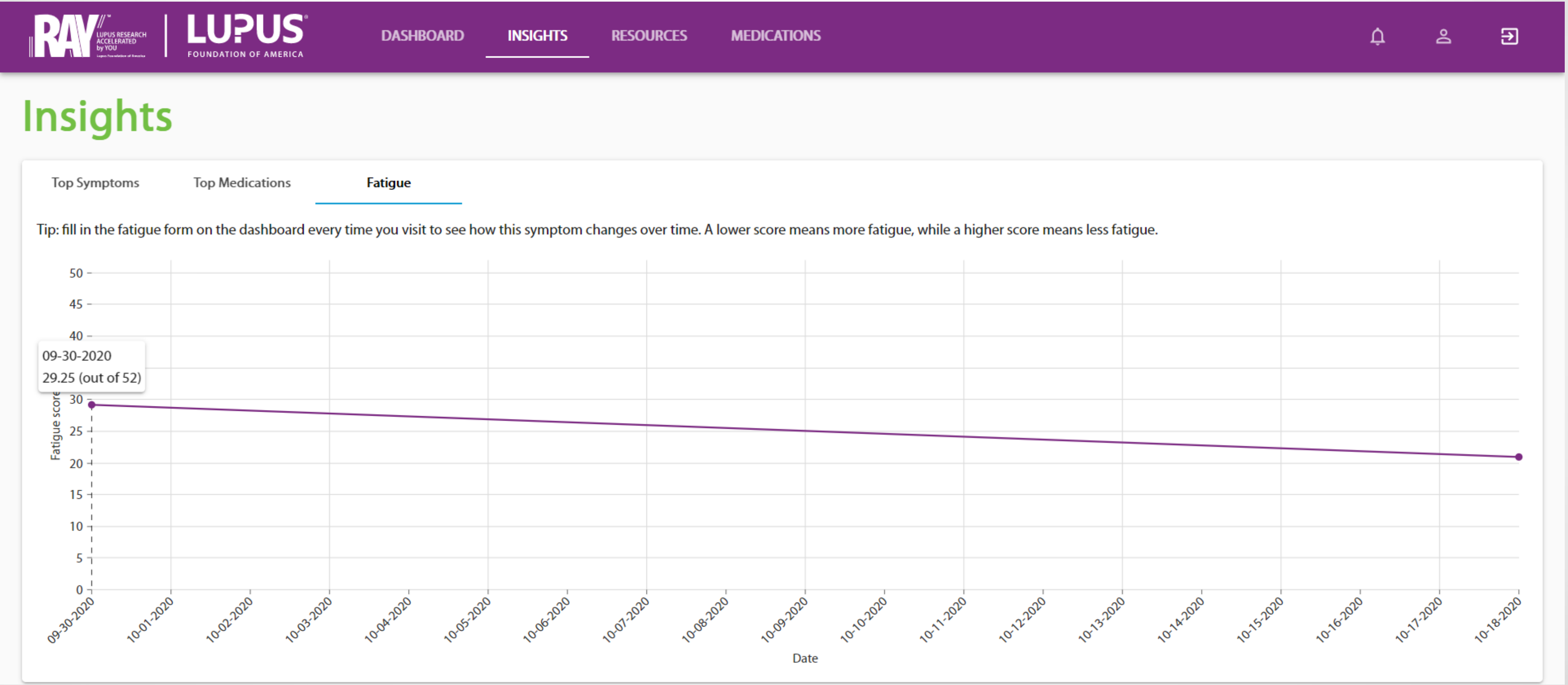
Lupus Type: All



Fatigue

Below is a list of statements that other people with your illness have said are important. Please select one option per line to indicate your response as it applies to the past 7 days.

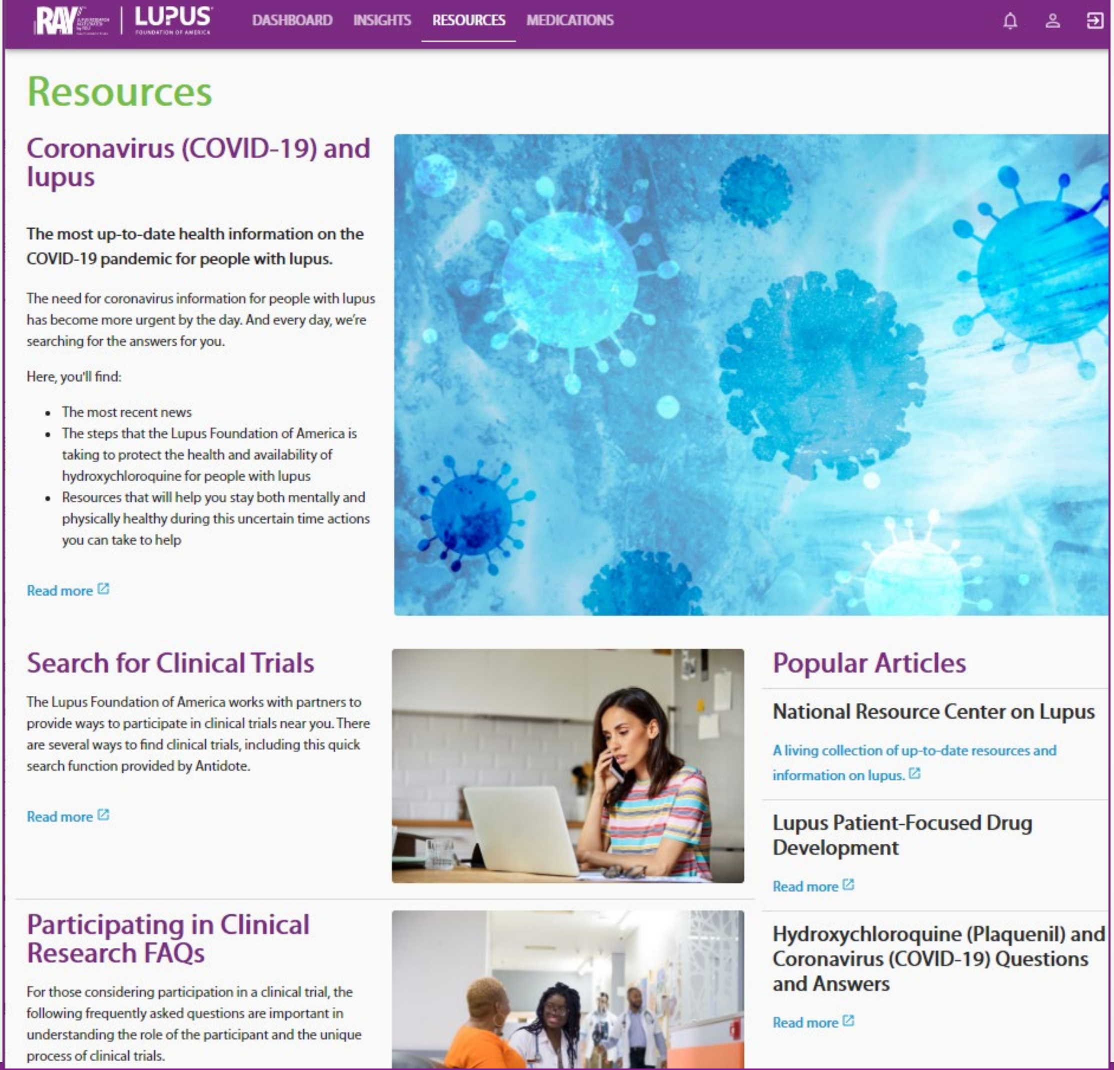
	Not At All	A Little Bit	Somewhat	Quite A Bit	Very Much
I feel fatigued	○	○	●	○	○
I feel weak all over	○	○	○	●	○
I feel listless (“washed out”)	○	○	●	○	○
I feel tired	○	○	○	●	○
I have trouble **starting** things becaused I am tired	○	○	○	●	○
I have trouble **finishing** things becaused I am tired	○	○	○	○	○



Insights

Tip: fill in the fatigue form on the dashboard every time you visit to see how this symptom changes over time. A lower score means more fatigue, while a higher score means less fatigue.

09-30-2020
29.25 (out of 52)



Resources

Coronavirus (COVID-19) and lupus

The most up-to-date health information on the COVID-19 pandemic for people with lupus.

The need for coronavirus information for people with lupus has become more urgent by the day. And every day, we're searching for the answers for you.

Here, you'll find:

- The most recent news
- The steps that the Lupus Foundation of America is taking to protect the health and availability of hydroxychloroquine for people with lupus
- Resources that will help you stay both mentally and physically healthy during this uncertain time actions you can take to help

Read more

Search for Clinical Trials

The Lupus Foundation of America works with partners to provide ways to participate in clinical trials near you. There are several ways to find clinical trials, including this quick search function provided by Antidote.

Read more

Participating in Clinical Research FAQs

For those considering participation in a clinical trial, the following frequently asked questions are important in understanding the role of the participant and the unique process of clinical trials.

Popular Articles

National Resource Center on Lupus

A living collection of up-to-date resources and information on lupus.

Lupus Patient-Focused Drug Development

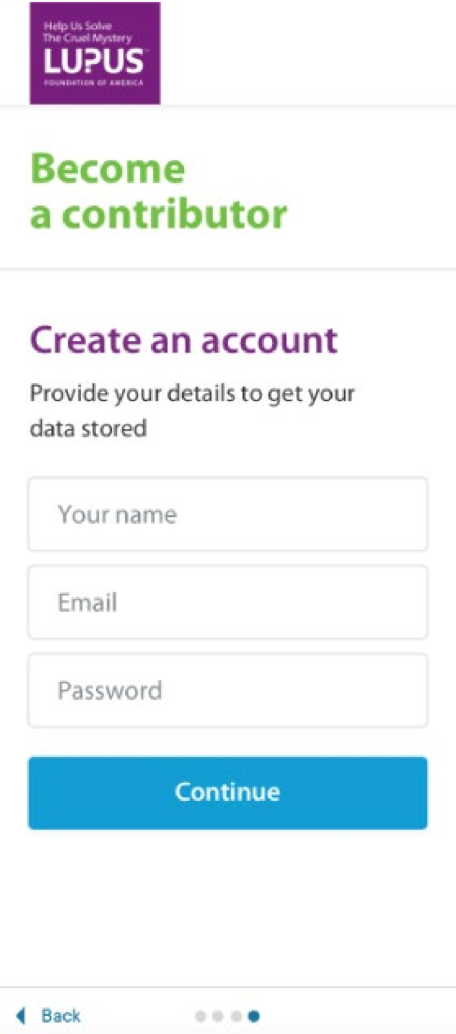
Read more

Hydroxychloroquine (Plaquenil) and Coronavirus (COVID-19) Questions and Answers

Read more

Lupus Clinical Trial Resources

- Variety of resources about clinical trials and value of volunteer participants
  - Specific resources available tied to minority participation in research
- Learn about clinical trials
  - What is a clinical trial?
  - Why should I volunteer?
  - What lupus trials are enrolling?
  - How do I find a trial near me?
- Find lupus clinical trials
- Explore ways to get involved in research
  - lupus.org/advancing-research/get-involved-in-research



Become
a contributor

Create an account

Provide your details to get your data stored

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Why Should I Join?

- Contribute to lupus research from your home
- See how you compare to others living with lupus
- Refer to it at a doctor's visit
- For those interested – get connected to clinical trial and other research information
- Use it as a central hub for the latest in lupus research

Review

- Patient powered
- Benefits
- Feedback from the lupus community will drive improvements and enhancements

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Advocating to Support Research

ADVOCATING FOR INCREASED RESEARCH FUNDING

Key Players and Key Dates

- **LUPUS ADVOCATES**
  - The most important voice, the most effective storytellers

- Congressional Lupus Caucus
  - Champions in the House of Representatives
  - Lead Dear Colleague, submit funding requests

- “Typical” appropriations timeline
  - February: President’s budget
  - March: deadline to submit funding requests, Dear Colleague
  - April – September: Subcommittee & Committee markups, floor votes

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Opportunities for engagement

- March
  - Thanking CLC cochairs for leading the Dear Colleague
  - Encouraging Representative to sign the Dear Colleague
  - Advocacy Summit – meetings with members of Congress
- Subcommittee, Committee, Floor markups & votes
  - Telling the lupus story to members
  - Continuing to build the case for funding requests, generate support
- Passage and signed by the President
  - Spreading the news, expressing gratitude to members and Caucus

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Fiscal Year 2021 Appropriations

- Lupus Foundation Requests:
  - $10 million for the National Lupus Patient Registry at CDC
  - $10 million for the Lupus Research Program at DoD
  - $2 million for the lupus program at the Office of Minority Health
  - $47 billion for the National Institutes of Health

- July 31 – House passes LHHS, DoD spending bills
  - $8.5 million for the National Lupus Patient Registry at CDC
  - $10 million for the Lupus Research Program at DoD
  - $2 million for the lupus program at the Office of Minority Health
  - $47 billion for the National Institutes of Health

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Fiscal Year 2021 Appropriations

- Senate has yet to act on any of its FY21 spending bills
- Sept. 30 – continuing resolution (CR) passed, signed
  - Continues existing government funding until December 11
- Options prior to December 11:
  - Pass another CR, likely until Jan. 20 (inauguration)
  - Senate passes their legislation, or takes up the House bills
- **Once the Senate begins acting, we will need advocates to maintain (and increase) House-passed numbers**

MAXIMIZING THE IMPACT OF EVERY DOLLAR

Steering funding to areas of need

- Report language within spending bills
  - Guides the Agency on how the funding is to be utilized
  - CDC grant to CARRA for juvenile surveillance
- Lupus Federal Working Group
  - Unites all agencies working in lupus to ensure their efforts are building on each other, and there is no duplication
- Coalition work on federal funding landscape
  - Research!America
  - Ad Hoc Group for Medical Research
- DoD: nominations for peer reviewers

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What is Advocacy?

On the Hill & In Our Backyard = Having a Voice

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Speaking About Research

IMPORTANCE OF MINORITY PARTICIPATION IN CLINICAL TRIALS

WENDY RODGERS, M.ED., MPH,
LUPUS WARRIOR

MINORITIES & LUPUS

- Lupus is **two to three times more prevalent among women of color**—African Americans, Hispanics/Latinos, Asians, Native Americans, Alaska Natives, Native Hawaiians and other Pacific Islanders—than among Caucasian women.
- Lupus **affects 1 in 537** young African American women.
- LUMINA (*Lupus in Minority Populations: Nature vs. Nurture*) study reported that African American lupus patients are **more likely to have organ system involvement, more active disease, and lower levels of social support** compared with white lupus patients.
- Minority women tend to **develop lupus at a younger age**, experience more serious complications, and have higher mortality rates.

FDA APPROVALS FOR LUPUS THERAPIES

1948	Aspirin approved for lupus treatment.
1948	Approval of corticosteroids, such as prednisone.
1955	Approval of antimalarial drug Plaquenil (hydroxychloroquine).
2011	**Benlysta**—the first targeted therapy for lupus.
The Future	Will you be part of the next therapy’s approval?

Resource: fda.gov/consumers/consumer-updates/lupus-therapies-continue-evolve, 2017

WHY SHOULD MINORITIES PARTICIPATE IN CLINICAL TRIALS…

- To be a part of the solution.
- To understand the common side effects in us.
- To discover if treatments are less effective in individuals with varying genetics or health conditions.
- We cannot afford not to – all people need to benefit.

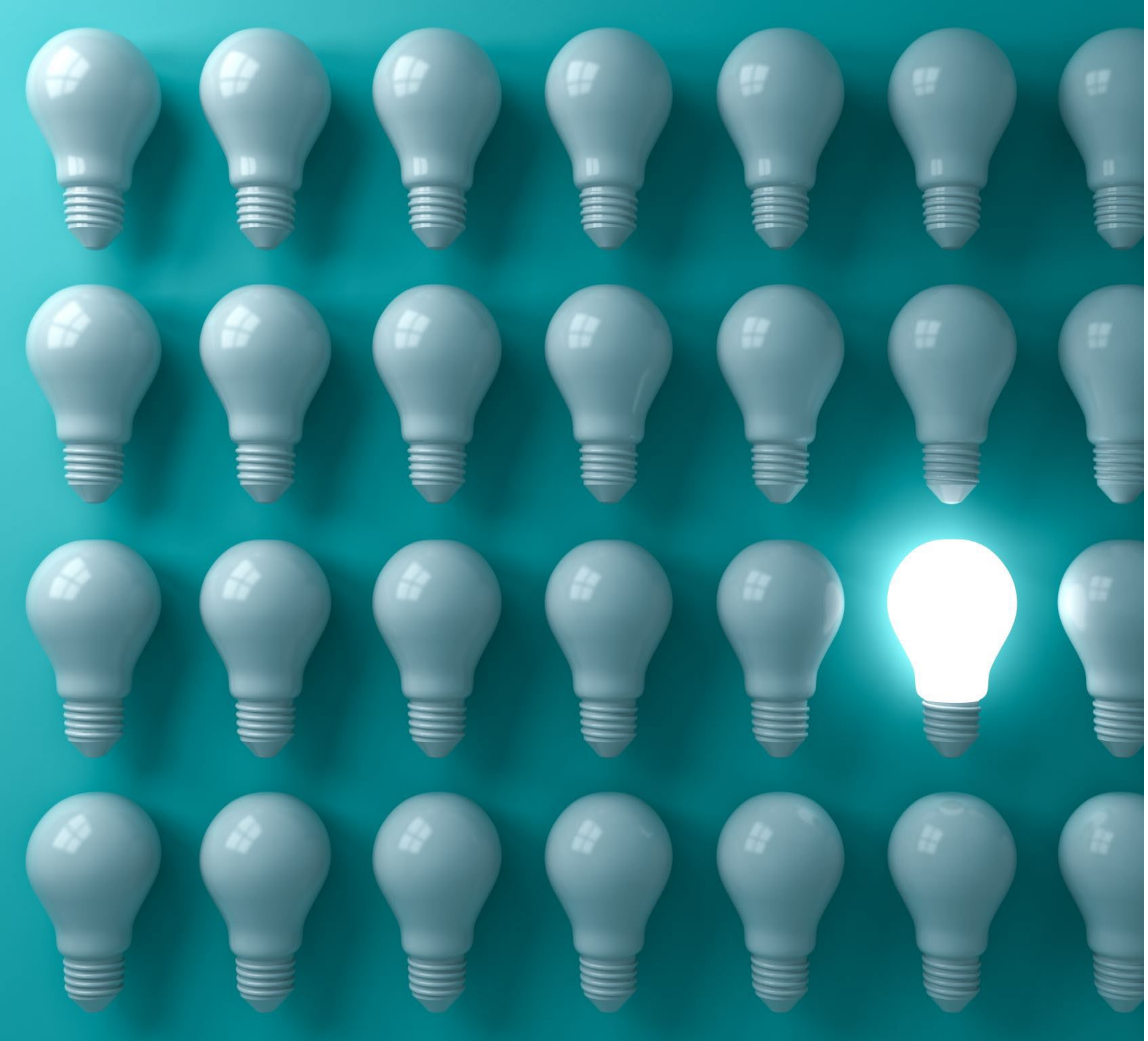
HOPE IS NOW

OVERCOMING MISTRUST IN CLINICAL TRIALS

Science is improving and diversifying approaches.

More focus is given to medical ethics and reducing bias in care.

Informed consent means– YOU have control and can stop at any time.



OTHER CONSIDERATIONS…

- Clinical trial participation does not always mean taking a medication.
- You **MUST** be deemed eligible for a trial.
- Involve your doctor – seek advice.
- Request a navigator to walk you through the process.

Group Discussion

Resources

Lupus Clinical Trial Resources

- Variety of resources about clinical trials and value of volunteer participants
  - Specific resources available tied to minority participation in research
- Learn about clinical trials
  - What is a clinical trial?
  - Why should I volunteer?
  - What lupus trials are enrolling?
  - How do I find a trial near me?
- Find lupus clinical trials
- Explore ways to get involved in research
  - lupus.org/advancing-research/get-involved-in-research

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Recap and Opportunities to Take Action

LRAN Day 2

- The power of your voice
- Opportunities to take action
  - RAY: Research Accelerated by You
  - FDA Patient Representative Program
  - Department of Defense Consumer Reviewers
  - LFA Research and Advocacy Initiatives

Next Steps

- LFA will summarize this weekend in a report
- Provide resources
- Please complete day 2 feedback survey
- LRAN in 2021

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