Help Us Solve
The Cruel Mystery
LUPUS™
FOUNDATION OF AMERICA

Welcome Back!
Lupus Research Action Network: Day 2

October 27, 2021

Today's Agenda

- Recap of Day 1
- Hear from the Experts – Panel
- The Importance of the YOUR voice 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
- Exciting time in lupus drug development
- Patient experience in research

Thank You to Our Sponsors!

gsk

Biogen®

Genentech
A Member of the Roche Group

Help Us Solve
The Cruel Mystery
LUPUS™
FOUNDATION OF AMERICA

Help Us Solve
The Cruel Mystery

LUPUS™

FOUNDATION OF AMERICA

Hear From The Experts

Help Us Solve
The Cruel Mystery
LUPUS™
FOUNDATION OF AMERICA

The Importance of the Your Voice 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

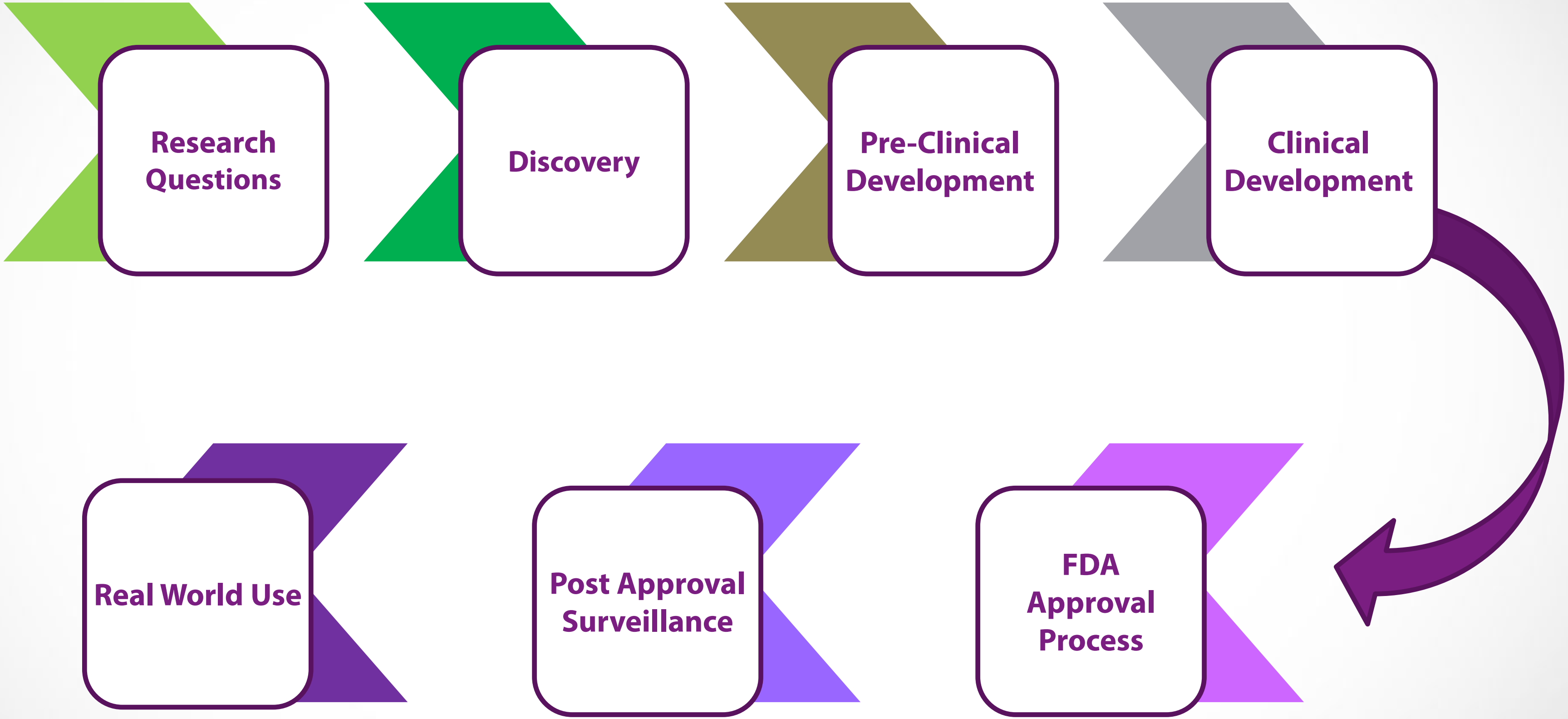
- Doing things <u>**with**</u> -- not **for** or **to –** patients

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

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.

Help Us Solve
The Cruel Mystery
LUPUS™
FOUNDATION OF AMERICA

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
- Patient & caregiver listening sessions
- Focus groups
- 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

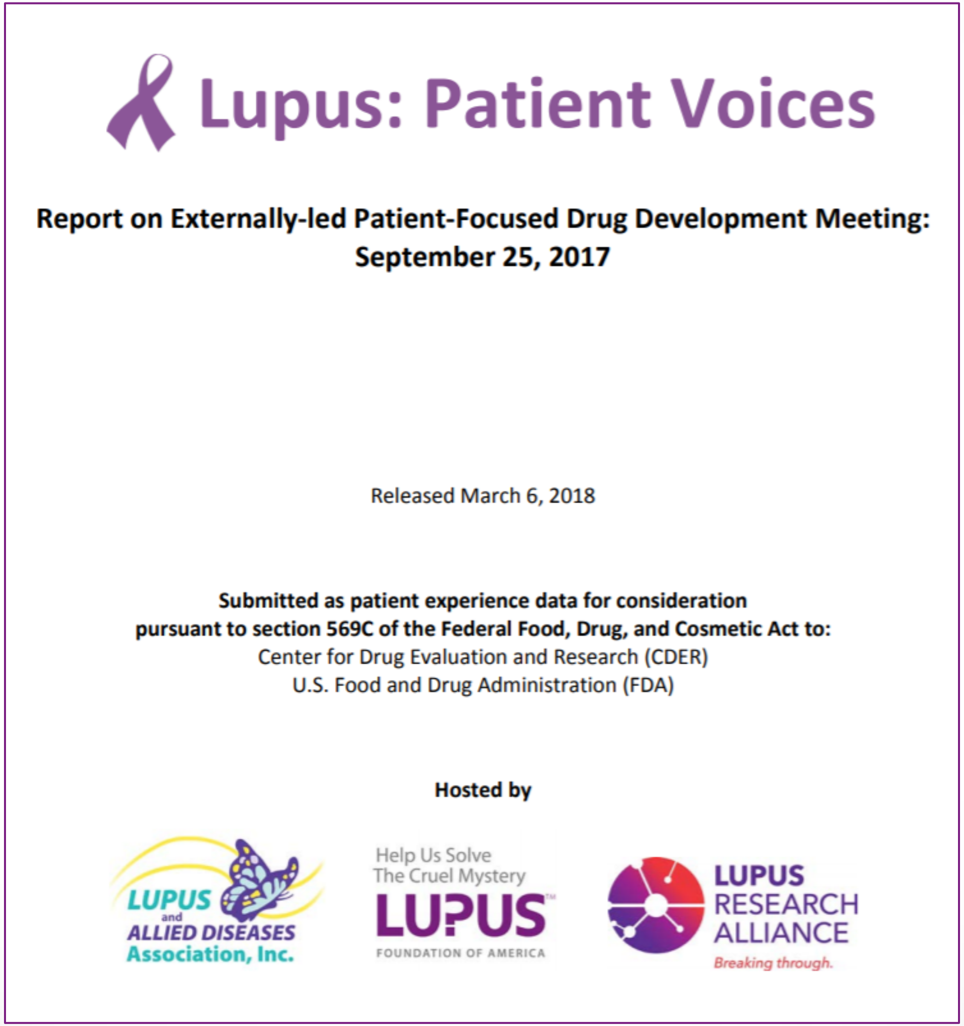
Every medicine comes with benefits and risks. For people with lupus, what is the right balance? How much risk is acceptable for a moderate benefit?

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)

Help Us Solve
The Cruel Mystery
LUPUS™
FOUNDATION OF AMERICA



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.
Help Us Solve The Cruel Mystery LUPUS™ FOUNDATION OF AMERICA
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
- Partner with LFA and other stakeholders at events like these!

Help Us Solve
The Cruel Mystery

LUPUS™

FOUNDATION OF AMERICA

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 you

What is a Patient Registry?

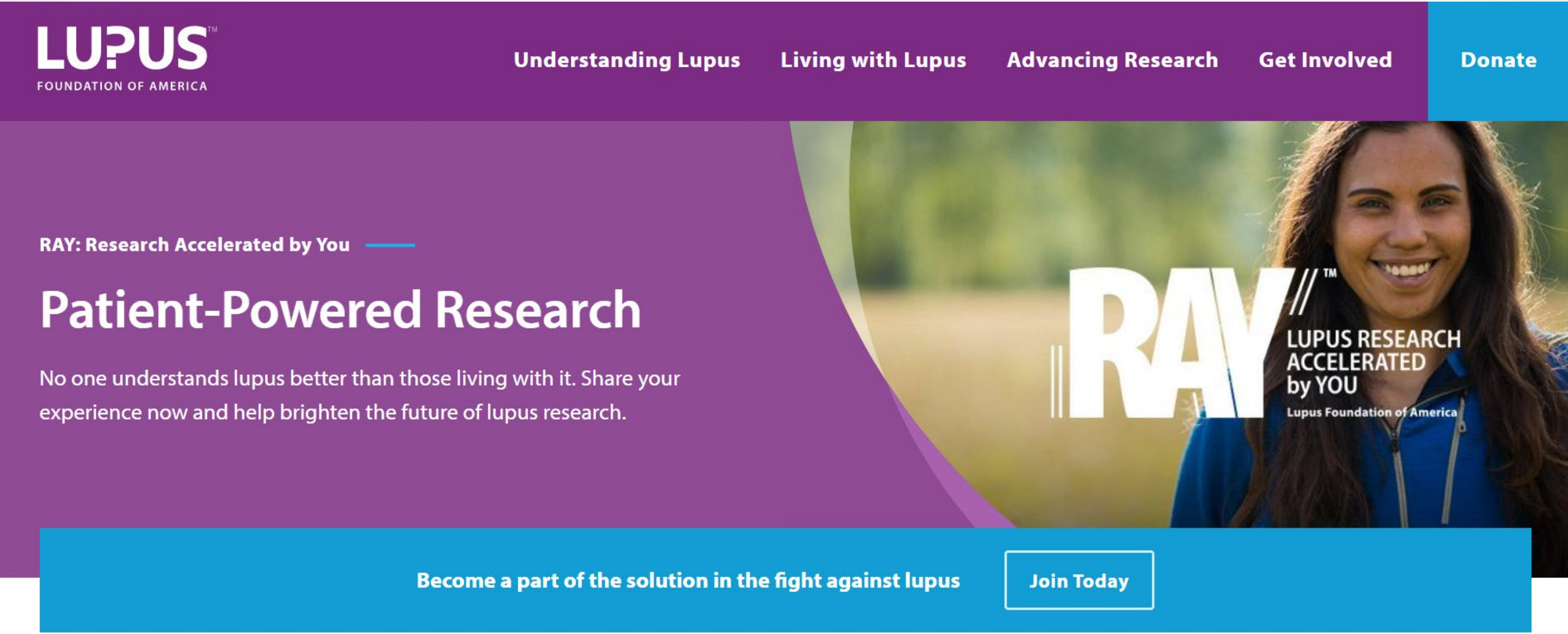
- A place to stored detailed information about individuals with a specific disease
- LFA has built a patient-powered registry meaning all of the information generated comes directly from people living with lupus and their families

Help Us Solve
The Cruel Mystery
LUPUS™
FOUNDATION OF AMERICA

Steering Committee

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

Help Us Solve
The Cruel Mystery
LUPUS™
FOUNDATION OF AMERICA



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.

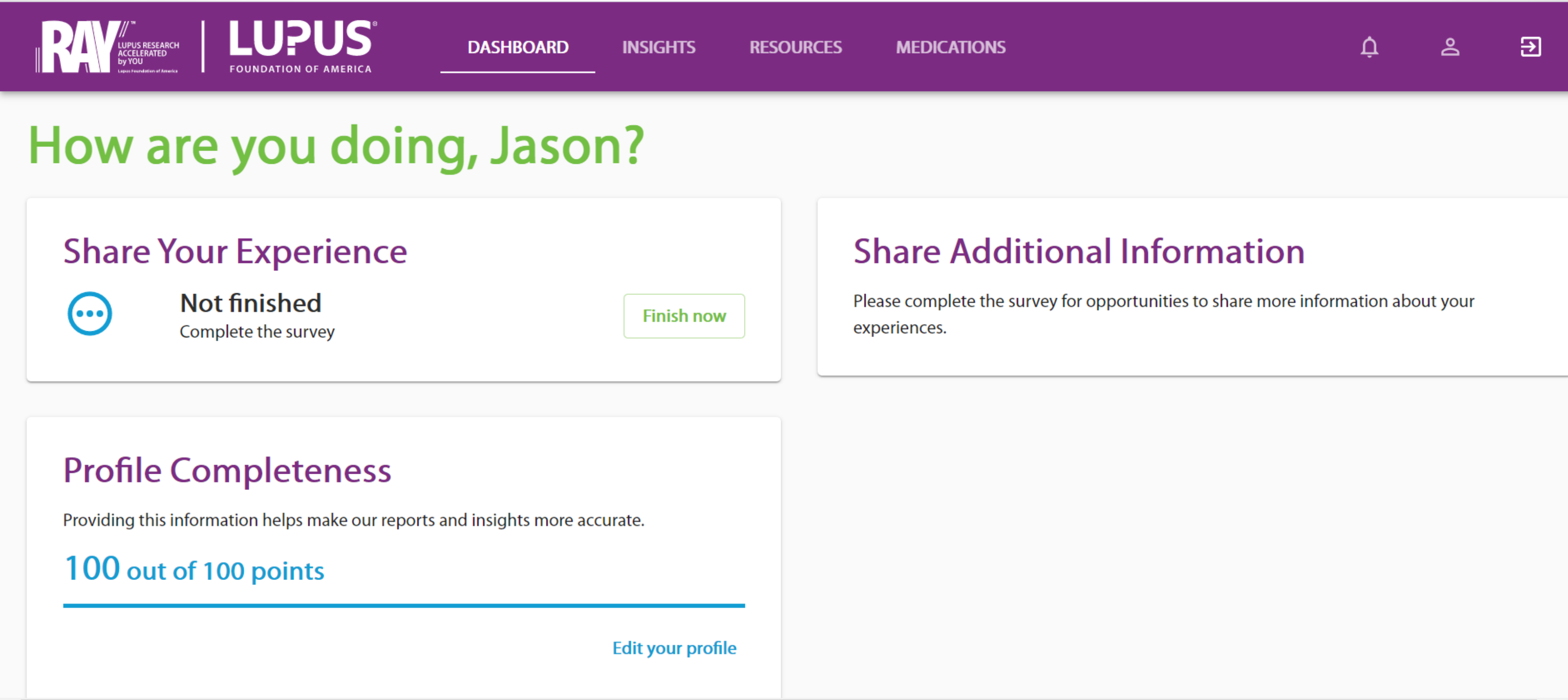
Become a part of the solution in the fight against lupus

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

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



How are you doing, Jason?

Share Your Experience

Not finished
Complete the survey

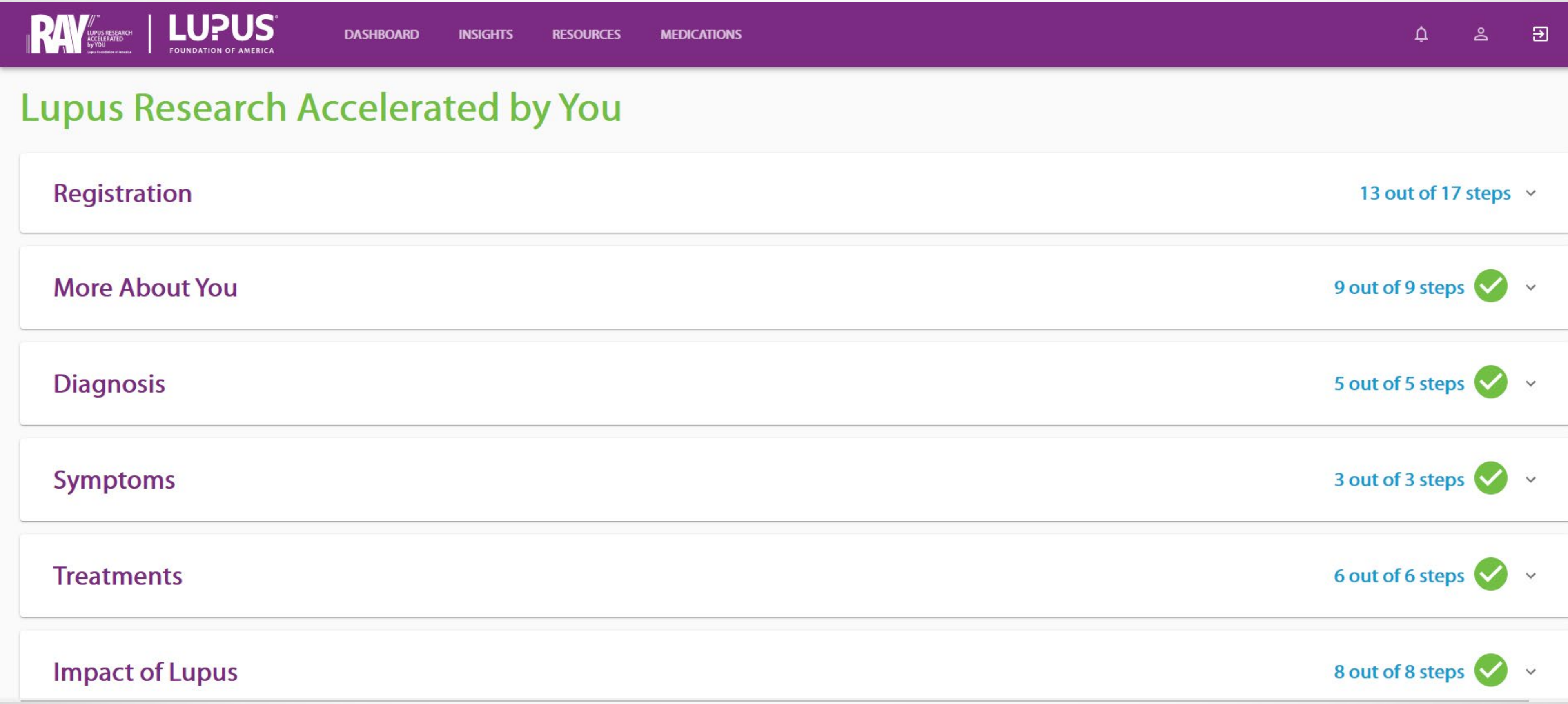
Share Additional Information

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

Profile Completeness

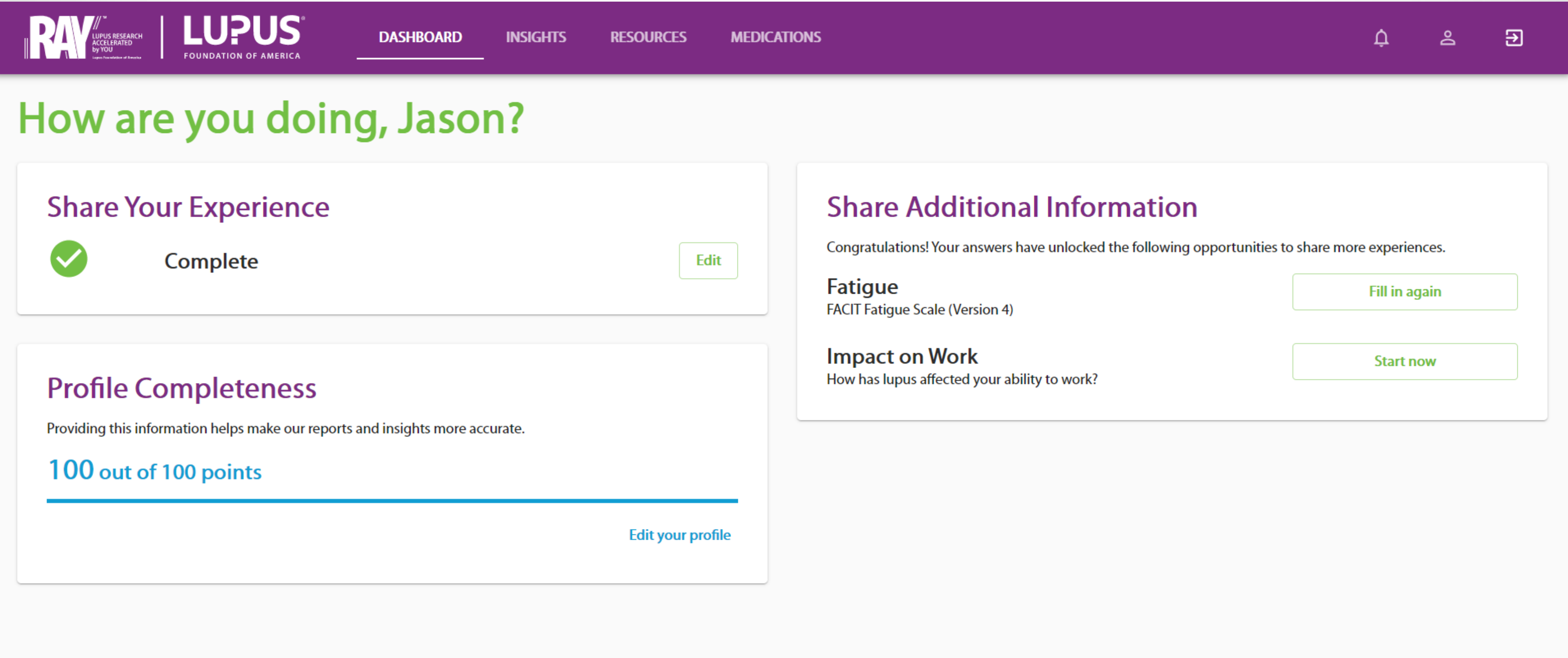
Providing this information helps make our reports and insights more accurate.

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.

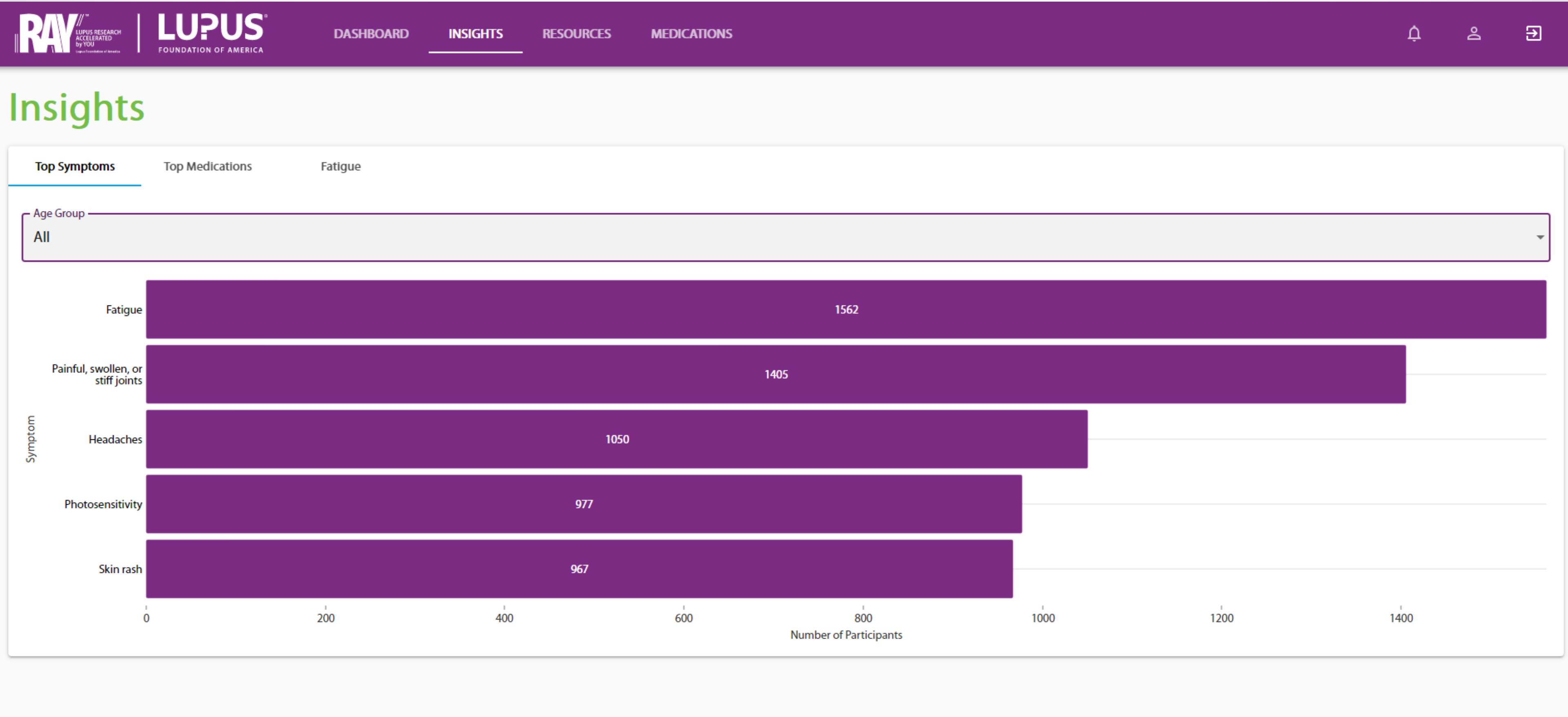
100 out of 100 points

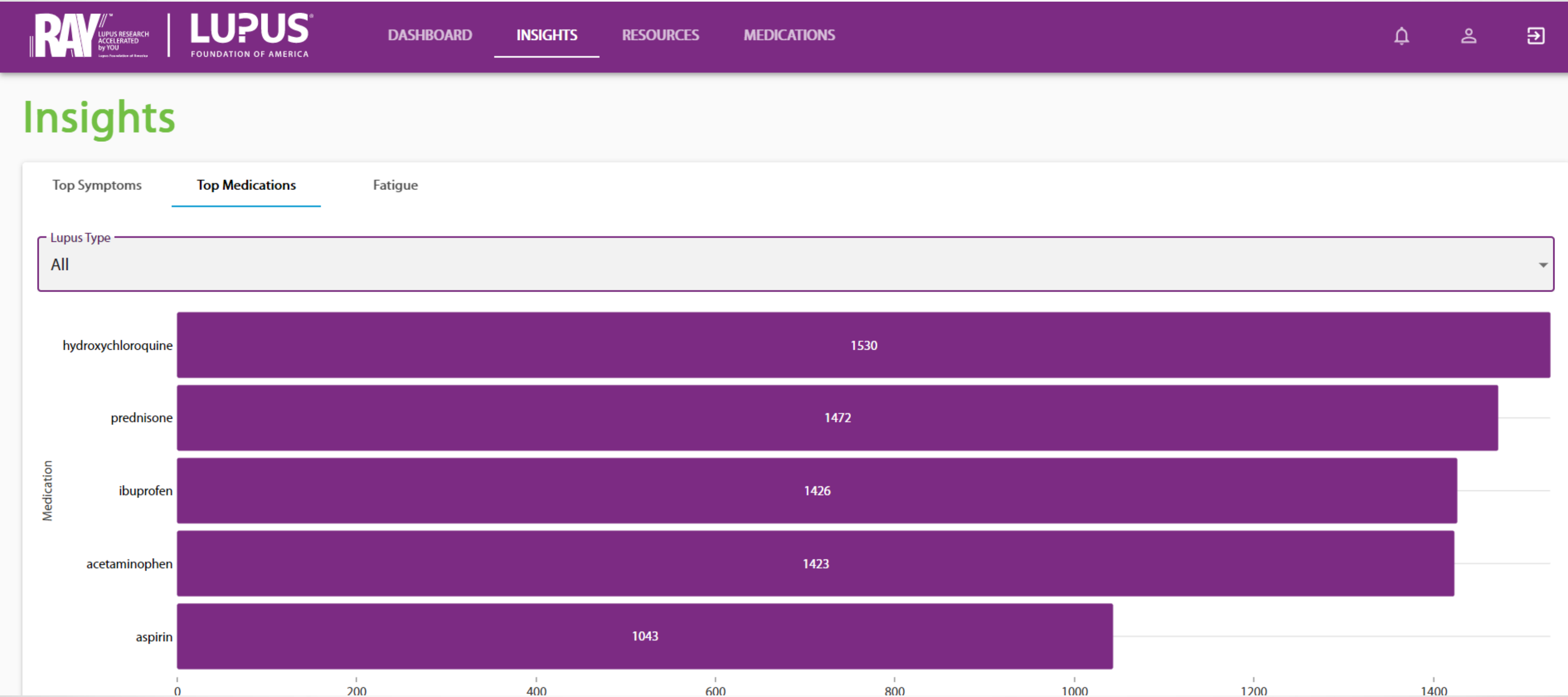
Share Additional Information

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

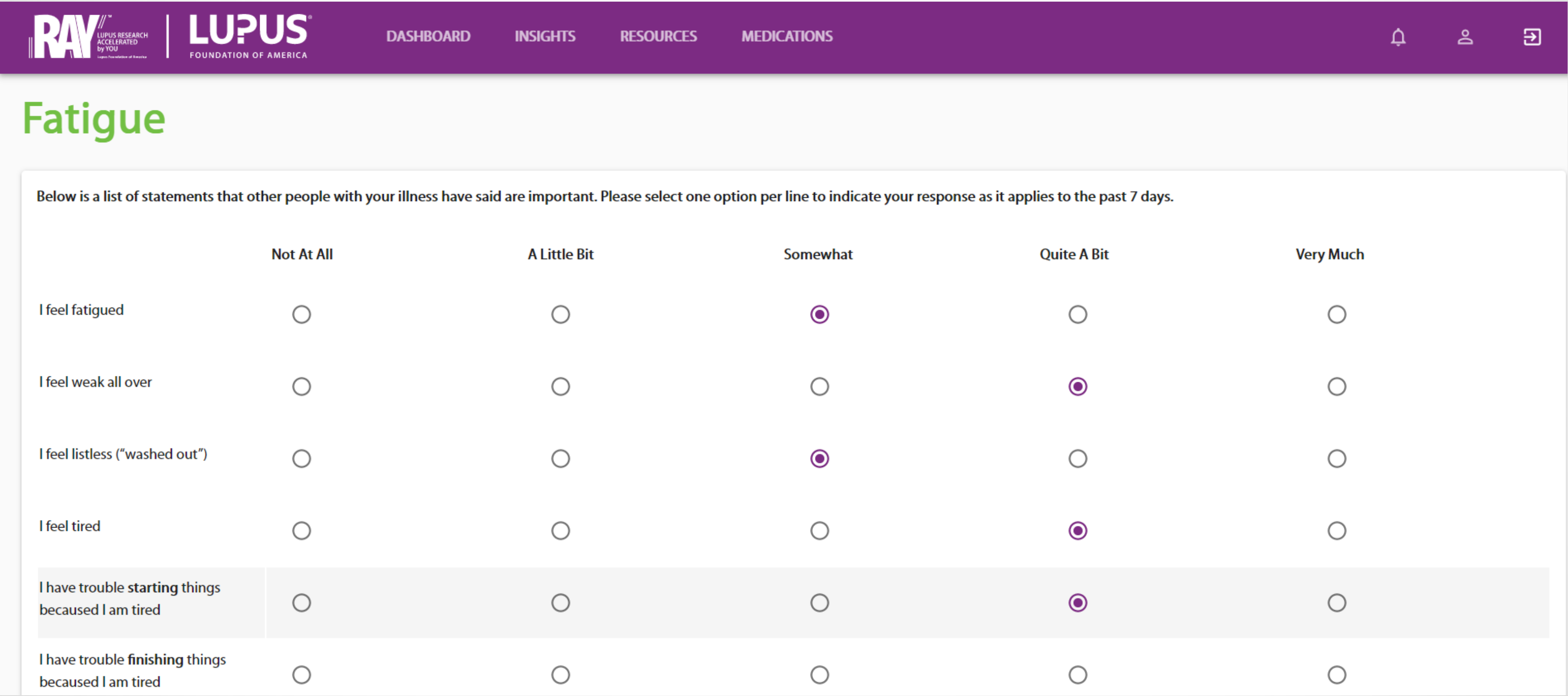
Fatigue
FACIT Fatigue Scale (Version 4)

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


DASHBOARD
INSIGHTS
RESOURCES
MEDICATIONS
Insights
Top Symptoms
Top Medications
Fatigue
Age Group
All
Symptom
Fatigue
1562
Painful, swollen, or stiff joints
1405
Headaches
1050
Photosensitivity
977
Skin rash
967
0
200
400
600
800
1000
1200
1400
Number of Participants



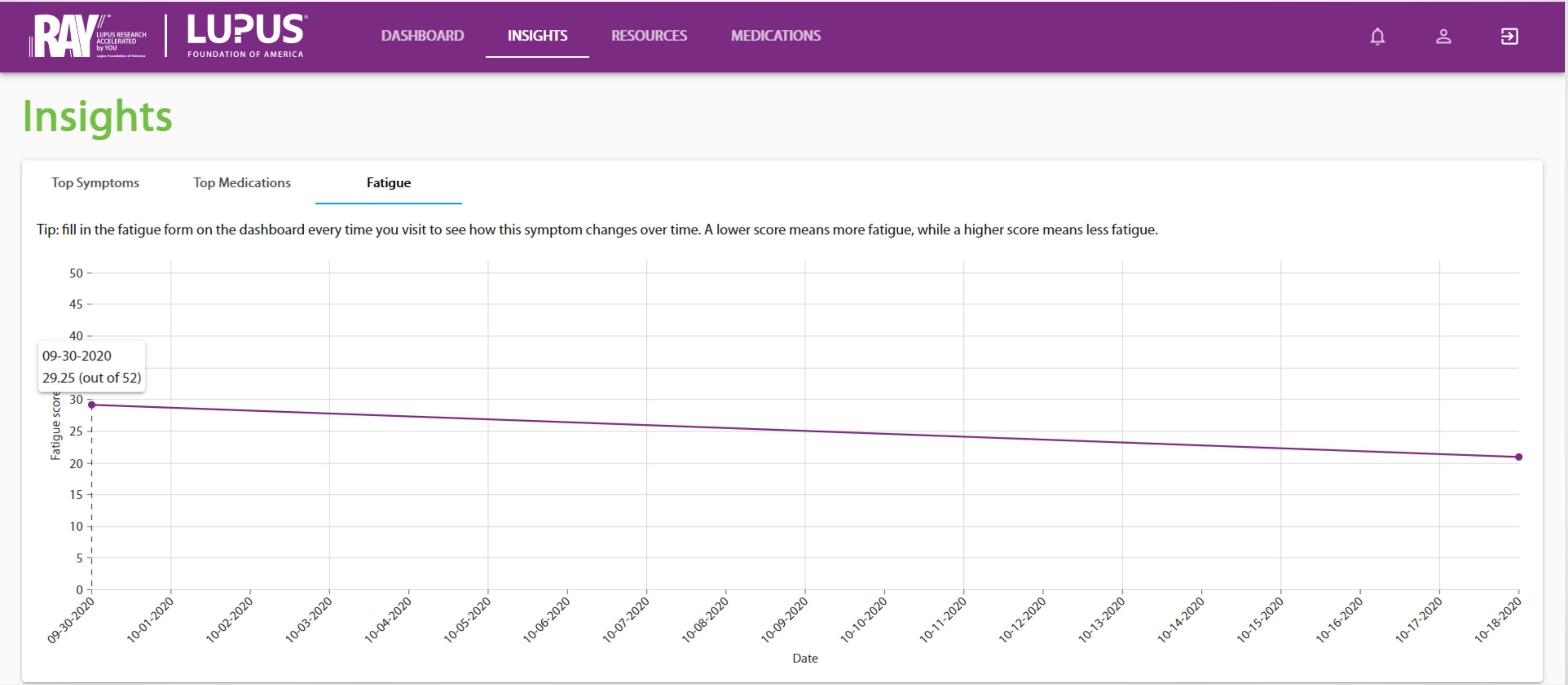
Lupus Type: All



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.

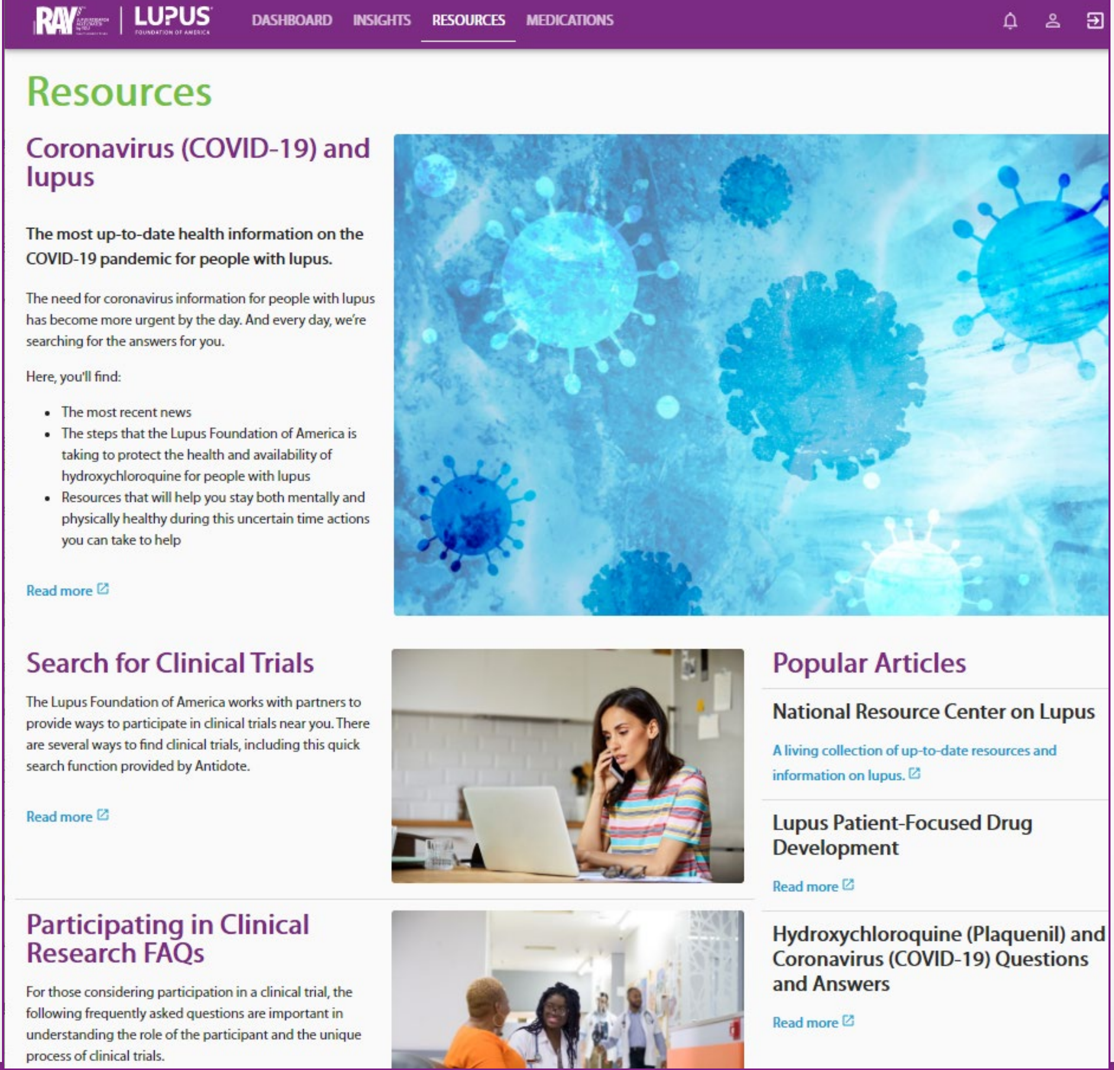
	Not At All	**A Little Bit**	**Somewhat**	**Quite A Bit**	**Very Much**
I feel fatigued	○	○	●	○	○
I feel weak all over	○	○	○	●	○
I feel listless ("washed out")	○	○	●	○	○
I feel tired	○	○	○	●	○
I have trouble **starting** things becaused I am tired	○	○	○	●	○
I have trouble **finishing** things becaused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

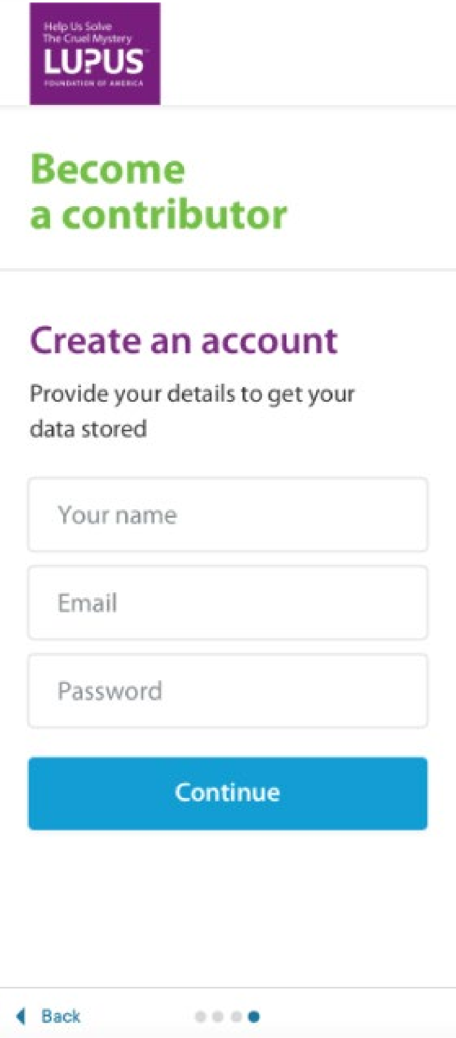
Read more

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

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

Help Us Solve
The Cruel Mystery
LUPUS™
FOUNDATION OF AMERICA

Help Us Solve
The Cruel Mystery
LUPUS™
FOUNDATION OF AMERICA

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

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

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

What is Advocacy?

On the Hill & In Our Backyard = Having a Voice

Help Us Solve
The Cruel Mystery
LUPUS™
FOUNDATION OF AMERICA

Group Discussion

Help Us Solve
The Cruel Mystery
LUPUS™
FOUNDATION OF AMERICA

Opportunities to Take Action

Next Steps

- LFA will summarize this weekend in a report
- LFA to provide resources and opportunities for you to speak about
- By next week please complete the survey to give us feedback on the last two days
- LRAN in 2022 in conjunction with the FDA Office of Minority Health
  - We hope you'll join us again!

Help Us Solve
The Cruel Mystery
LUPUS™
FOUNDATION OF AMERICA

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

Thank You to Our Sponsors!

gsk

Biogen®

Genentech

A Member of the Roche Group