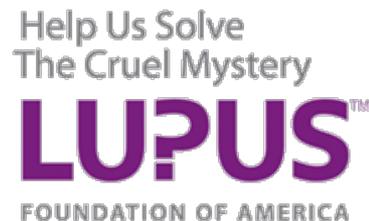


# *Lupus: Patient Voices*

Report Overview  
March 6, 2018





**Welcome**

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**Kathleen Arntsen**, Lupus and Allied Diseases Association

**Sandra Raymond**, Lupus Foundation of America

**Diane Gross**, Lupus Research Alliance



# Goals of Lupus PFDD Initiative

- To understand the lupus patient journey and recognize patient preferences and risk tolerances
- To examine the complexity and heterogeneity of the disease to help better inform trial designs
- To create a practical, scientifically rigorous framework that incorporates patient preferences and patient reported outcomes into lupus clinical research and trials
- To ensure people with lupus understand the value of their participation in the drug development and clinical trials process by acknowledging how their contributions impact decision-making and outcomes



# Overview of Lupus PFDD Initiative

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- FDA responded favorably to the three convening organizations LOI application to hold an externally-led PFDD meeting
  - Initiative is not organized by FDA but is based on the FDA-model for internally-led meetings
- Three main components:
  - Pre-meeting survey
  - Lupus PFDD Meeting
  - *Lupus: Patient Voices* Report



# Lupus PFDD Meeting

Nearly 600 people with lupus and their representatives participated in the September 25, 2017 meeting in Hyattsville, MD





# Lupus PFDD Meeting

- Two main topics:
  - Patient perspectives on disease symptoms and daily impacts that matter most
  - Patient perspectives on current approaches to treating lupus
- Two moderated six-person patient panels
- Two facilitated patient audience discussions
- Presentations by lupus community representative, clinical expert, and FDA officials
- Live polling from patient participants in audience and on webinar
- Open public comment session



# Input for Report

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- Pre-meeting survey (over 2,100 respondents)
- Panel presentations at meeting
- Facilitated discussion at meeting
- Post-meeting comments



# Potential Uses of Report

- As a resource for FDA Center for Drug Evaluation and Research
- As patient experience data in review of new drugs
- As draft benefit-risk framework in drug reviews
- To inform new follow-on initiatives related to PFDD
- As additional data and insights for the lupus community



# Key Report Themes

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- Lupus has a devastating impact on the lives of many people living with the disease
- Lupus disproportionately affects women and minorities
- Lupus is highly variable and heterogeneous, resulting in diagnosis, treatment, and clinical trial design challenges
- Treatments have limited effectiveness and are often associated with substantial adverse effects
- People with lupus desire more effective and less damaging treatments, and are eager to participate in the drug development process



# Report Structure

- Report summary
- Background on lupus and current treatments
- Meeting design and data collection approach
- Summary of findings from meeting and pre-meeting survey
  - Impact of disease symptoms on daily life
  - Perspectives on treatments for lupus
- Draft benefit-risk framework
- Appendices
  - Lupus PFDD Meeting agenda, speakers, discussion questions, and polling questions
  - Pre-meeting survey questions



# Overview of Lupus and Treatments

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- Types of lupus (SLE, subacute cutaneous, discoid, drug-induced)
- More common in women and minorities
- Common symptoms (fatigue, joint/muscle pain, kidney disease, organ involvement, “brain fog”, photosensitivity, and rashes)
- Current treatments
  - Only four treatments currently approved for lupus
  - Many medications approved for other diseases and conditions are used “off label” for lupus
  - Many drugs cause significant side effects

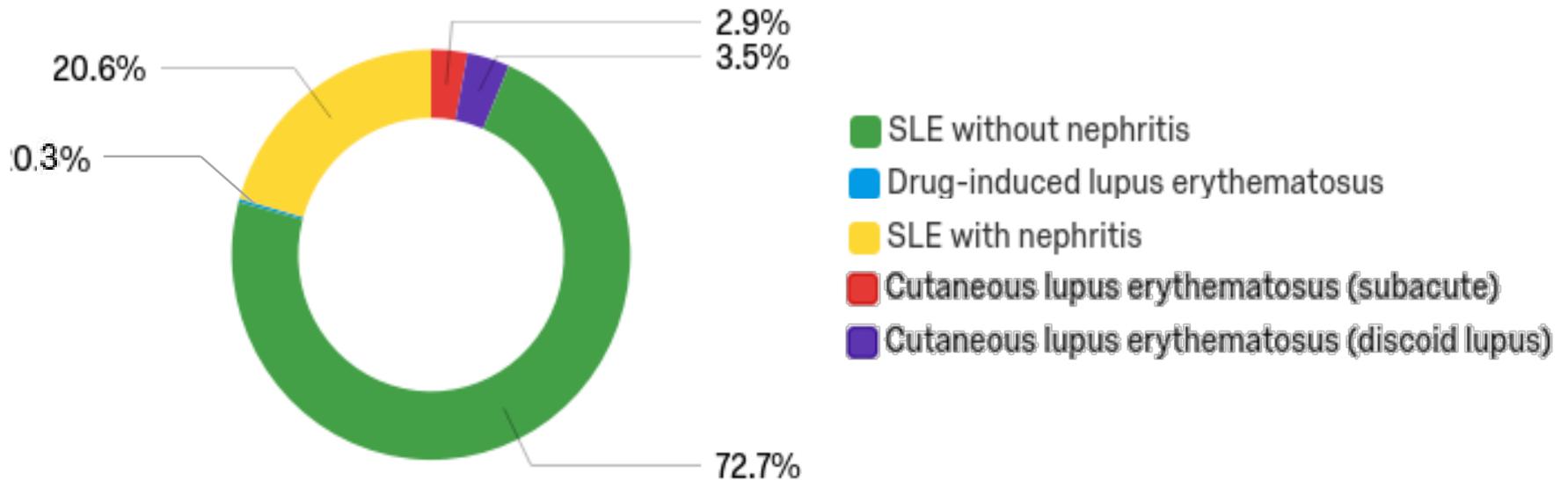


# Demographics of Survey Respondents

Survey Respondents by Race or Ethnicity	
White	65.9%
Black or African-American	14.8%
Hispanic or Latino	11.3%
Asian	3.0%
Native Hawaiian or Other Pacific Islander	0.9%
American Indian or Alaska Native	0.7%
Other	3.4%

Survey Respondents by Age	
Under 18	1.5%
18-29 years	11.7%
30-39 years	19.9%
40-49 years	24.2%
50-59 years	23.8%
60 years or older	18.9%

# Types of Lupus Among Survey Respondents





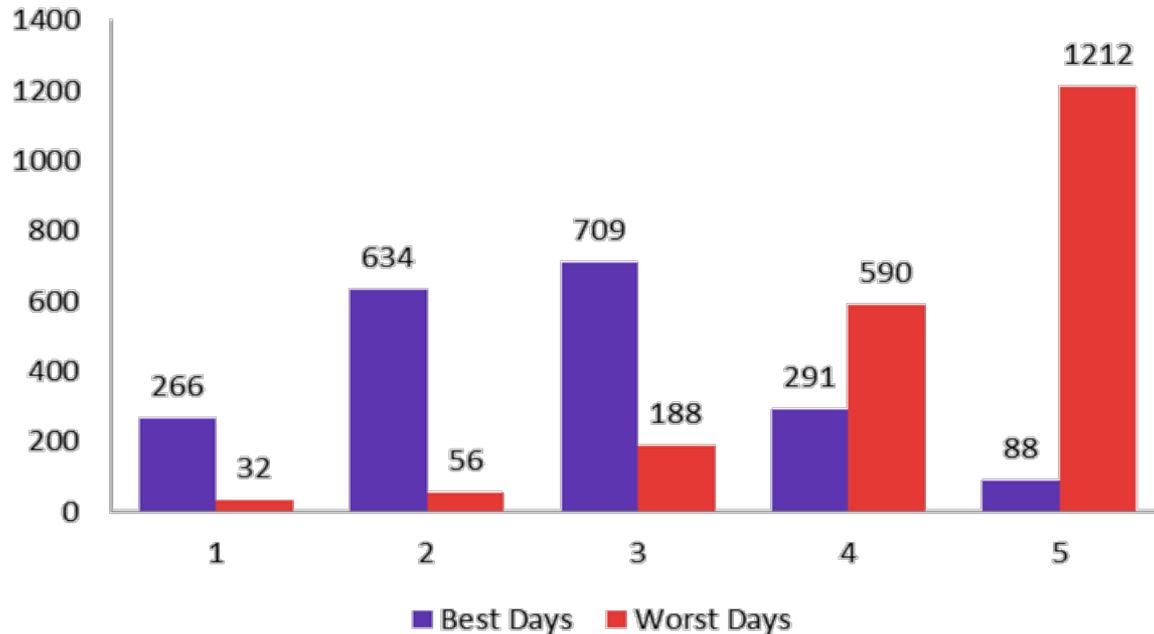
# Symptoms with Most Negative Impact

Symptom	Percentage of Respondents
Joint and muscle pain and/or swelling	31.9%
Fatigue	31.6%
Renal (kidney) disease or renal failure	5.6%
Increased susceptibility to infections	3.6%
Blood clots	3.1%
Stomach or bowel problems	2.9%
Sun sensitivity	2.8%
Reduced physical strength	2.6%
Rashes	2.3%
Pleurisy (inflammation of the lining of the lung)	2.1%
Other symptoms (detailed in report)	11.4%



# Impacts on Daily Life

**Even on good days, people with lupus experience mild- to moderate-level impacts on their daily lives**



Comparison of impact of lupus symptoms on respondents' best and worst days (1 = minimal impact, 5 = high impact)



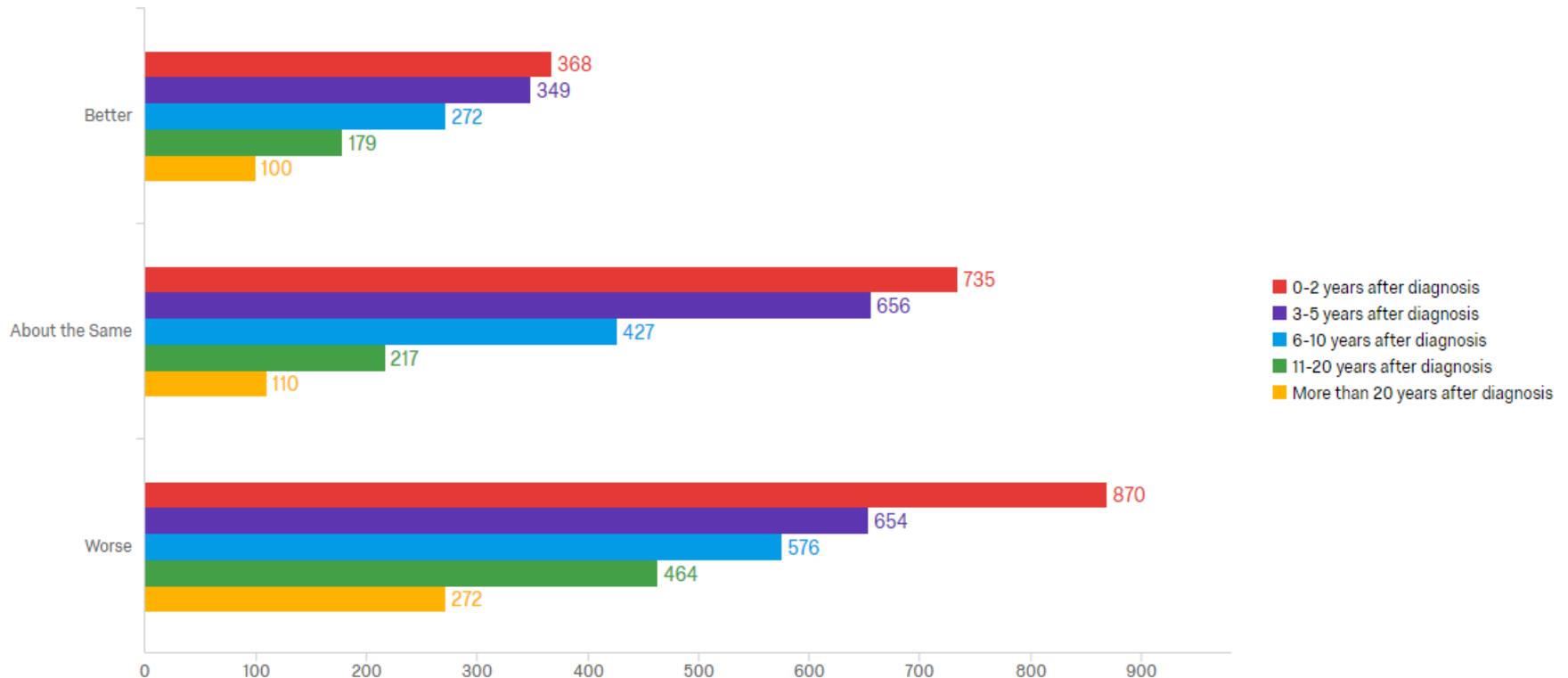
# Significant Impacts on Daily Life

- 72% of respondents reported that lupus impacts their ability to do one or more activities on a daily basis
- Activities frequently impacted by lupus include:
  - Doing outdoor activities involving direct sunlight
  - Participating in sports or social activities
  - Performing household chores
  - Attending work or school
  - Taking care of children or others
  - Being intimate with a spouse or partner



# Changes in Symptoms Over Time

**Survey data indicate that people with lupus experience a worsening of symptoms over time**





# Current Treatments: Summary Points

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- People with lupus take multiple medications to manage their symptoms.
- Many people use a combination of prescription and over-the-counter as well as non-pharmacologic approaches.
- Side effects of medications can be quite severe and difficult to distinguish from disease symptoms.
- What works for one person at one time may not work for others or for that same person at a different time.

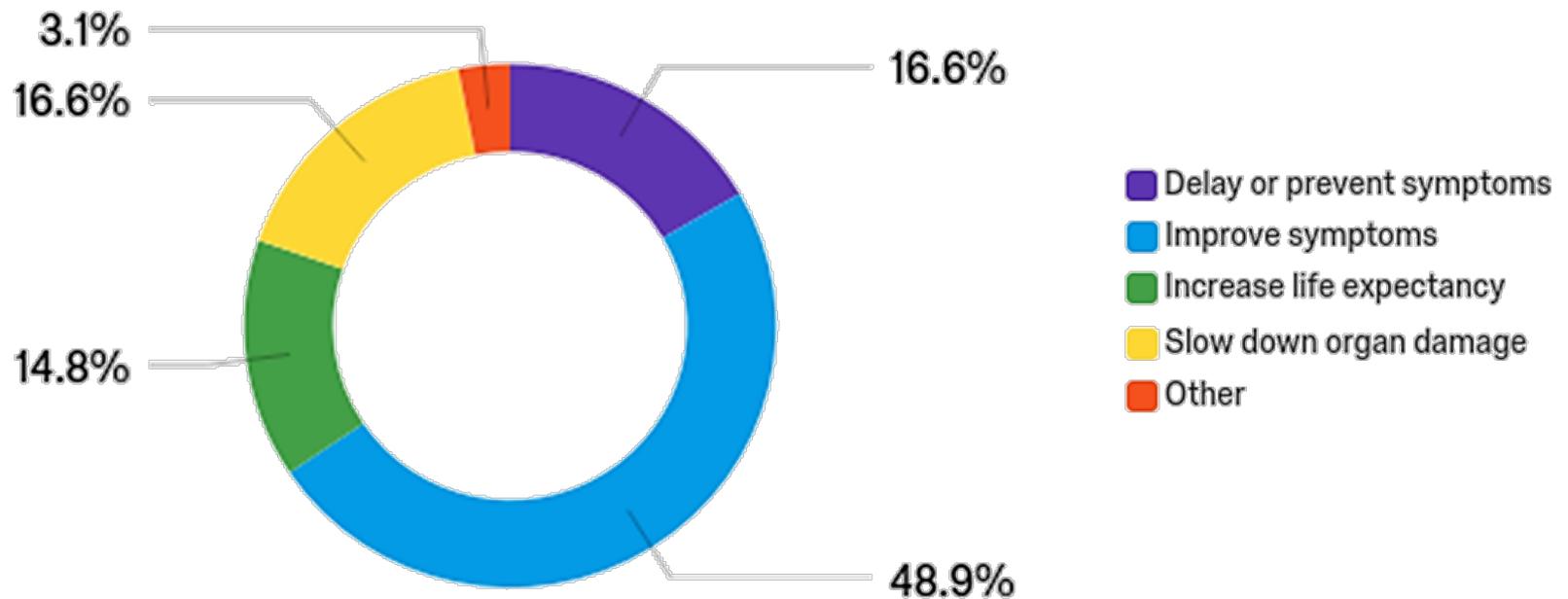


# Common Treatments

- **Prescription medications:**
  - Antimalarials
  - Corticosteroids
  - Immunosuppressants
- **Over-the-counter medications:**
  - Vitamins/minerals
  - Anti-inflammatory agents (NSAIDs)
  - Analgesics
- **Non-pharmacologic approaches:**
  - Exercise
  - Diet modification
  - Heat packs

# Factors Impacting Treatment Choice

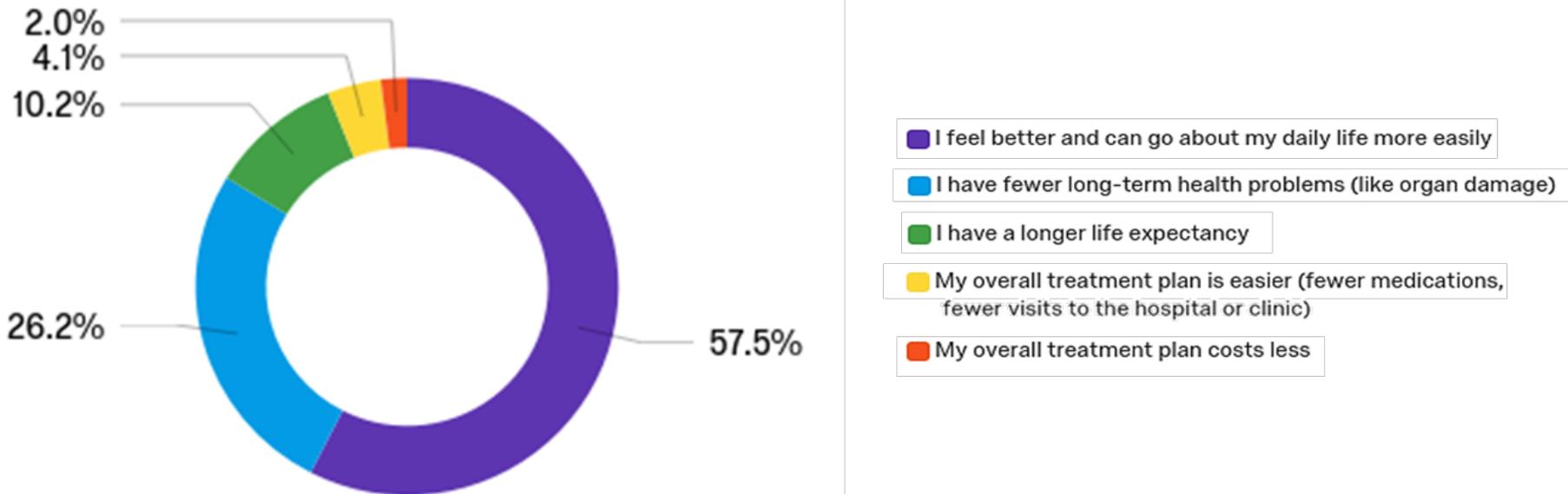
**Symptom improvement is the most important factor for people when choosing a treatment for lupus**





# Treatment Preferences

**Assuming there is no complete cure for lupus, respondents place the most value on feeling better and being able to more easily go about their daily lives**





# Clinical Trials Participation and Interest

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- 14.2% of respondents have participated in a clinical trial
  - Mix of observational and interventional trials
  - 87.0% said they would join a trial again
- 73.0% of respondents said they are willing to participate in a trial
- Most important factors when considering whether to join a trial:
  - Whether the treatment will improve quality of life
  - Possible negative side effects of treatment
  - Travel time to trial site



# The Report Demonstrates

- The importance of patient engagement and the significant value and impact of people sharing their personal experiences
- The value of patient experience data and how it will be used – throughout the drug development process from preclinical to post approval to inform regulatory decisions at FDA and drug development within industry
- The significant unmet need and benefit-risk in lupus, and the urgent need for the FDA and drug developers to understand them



# Questions or Comments?

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General questions and comments: **info@lupuspfdd.org**

To learn more about this initiative, visit <http://lupuspfdd.org>

To read the report, visit <http://lupuspfdd.org>



# Thank You

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We extend sincere appreciation to the many individuals and organizations who made this initiative possible including the lupus groups across the country, researchers, healthcare providers, biopharmaceutical sponsors, the FDA, and most of all, **people with lupus and their loved ones.**

**We couldn't have done this without you. Thank you.**