



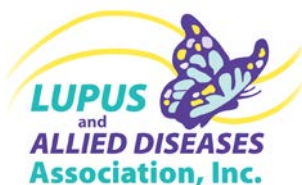
Lupus: Patient Voices

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

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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)**

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A Message of Gratitude

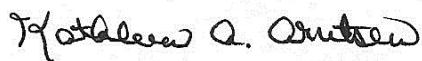
The Lupus and Allied Diseases Association, the Lupus Foundation of America, and the Lupus Research Alliance are pleased to present *Lupus: Patient Voices*, a summary report composed as a result of an Externally-led Lupus Patient-Focused Drug Development (PFDD) Meeting, a parallel effort to the U.S. Food and Drug Administration's (FDA) Patient-Focused Drug Development Initiative. The report reflects the three organizations' account of the perspectives of people with lupus and their representatives who participated in the public meeting, responded to the pre-meeting survey, and/or submitted post-meeting comments.

On behalf of the three collaborating organizations, we would like to recognize and thank the various stakeholders who helped to make this groundbreaking initiative possible. We extend sincere appreciation to the many lupus groups, healthcare providers, and researchers who provided input; the biopharmaceutical sponsors for their generous support; the Food and Drug Administration for working with us; and, above all, the people with lupus and their loved ones who promoted and participated in this project while inspiring us along the way. We gratefully acknowledge the efforts of the entire lupus community for understanding the value of the project in driving new therapies forward and for being engaged from day one in helping to make it a success.

Lupus remains a devastating and poorly understood disease: it is difficult to diagnose, with few proven treatments, and significantly impacts the lives of those affected. Even the current number of people in the U.S. with lupus is unknown, although estimates place it anywhere from 322,000 to 1.5 million. Despite recent improvements in mortality rates, lupus and its associated complications can still be fatal. While many dedicated researchers are diligently working to better understand this elusive disease and identify possible treatments, there is still much to be done in delivering optimal therapies.

For over two years, our organizations have worked tirelessly to make the PFDD meeting and resulting report a reality. Although it was our goal to show the diversity of the population impacted and the variability in disease manifestations through pre-meeting survey data, and over 2,100 people responded, we realize that the information collected is not truly reflective of the overall patient population. We are however, deeply indebted to the many individuals with lupus and their representatives who took the time to complete the survey, which helped us to craft a meaningful meeting agenda and provided valuable insight in understanding their experiences and priorities.

Our community stands united in its desire to improve the lives of people with lupus and this report represents a significant step in advancing therapies for this complex and debilitating disease. We sincerely hope that the FDA and drug developers will use this information to continue to move us forward in achieving that important goal.



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Report Summary

Lupus is a chronic, systemic, and often disabling autoimmune disease with an unpredictable course and inadequate treatment options. Frustrated with the present status of lupus therapies, members of the lupus community shared their perspectives on living with the disease, their experiences with current approaches to treatment, and what they want in new treatments at the *Lupus: Patient Voices* meeting with representatives from the U.S. Food and Drug Administration (FDA). Approximately 300 members of the lupus community participated in the meeting held on September 25, 2017 in Hyattsville, Maryland. An additional 300 people with lupus and their representatives joined the meeting via webcast. The lupus community urged regulators and drug developers to ramp up efforts to advance more effective and safer treatments.

Three partnering organizations – the Lupus and Allied Diseases Association, the Lupus Foundation of America, and the Lupus Research Alliance – organized the meeting as part of the FDA’s externally-led Patient-Focused Drug Development (PFDD) Initiative. The PFDD program was created by the FDA to allow regulators to more effectively understand, in a systematic manner, the unique perspective of people with diseases such as lupus to better assess the risks and benefits of drugs under review. The Lupus PFDD Meeting was designed primarily to provide individuals with lupus and their representatives an opportunity to share their unique experiences to help the FDA and the biopharmaceutical industry to:

- Understand the lupus patient journey and recognize patient preferences and risk tolerance so these may be translated into improved clinical trial designs and selection and/or development of measurable outcomes relevant in lupus drug development. In addition, this information could be incorporated into the benefit-risk framework being used in regulatory decision making;
- Demonstrate the complexity and heterogeneity of lupus, with the end goal of development programs and trial designs that will reflect these aspects of the disease;
- Create a practical, scientifically rigorous framework that incorporates patient preferences and patient reported outcomes into lupus clinical research and trials to ensure that trials are measuring not only statistical success but also demonstrating meaningful benefit to the individual with lupus; and
- Ensure that 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 at all levels to both improve their own health-related quality of life as well as the quality of life for the next generation of people with lupus.

Prior to the Lupus PFDD Meeting, more than 2,100 people completed a detailed survey about their experiences with lupus. The data from these surveys were used to shape the meeting, and similar questions were posed and additional data were collected in the form of polling questions during the meeting via a web-based data collection tool.

Four introductory presentations set the tone of the meeting. The first, by a lupus community representative, provided a personal account of the difficulties facing individuals with lupus. Her remarks were followed by presentations from two FDA representatives and a presentation by a lupus clinical expert.

To supplement the data collected in the survey, additional information about the experience of living with lupus was captured in two live sessions that focused on 1) symptoms of lupus, disease severity and progression, and the impact on individuals’ daily lives, and 2) perspectives on available and future

treatments. For each of these two themes, a panel of individuals with lupus and/or their representatives presented brief summaries of their experiences and preferences. People with lupus and their representatives who attended the in-person meeting or participated via webcast then responded to a series of polling questions similar to those asked in the survey. Polling was followed by a facilitated discussion in which audience members were invited to further share their experiences and preferences.

The second facilitated audience discussion was followed by an Open Public Comment period that included various comments from attendees who had pre-registered to speak. A representative from the FDA Division of Pulmonary, Allergy, and Rheumatology Products then provided summary remarks that captured predominant themes. The moderator closed the meeting by sharing powerful quotes from various participants.

The meeting was broadcast to the public from a dedicated website set up for the initiative. The full recording can be viewed at this link: <http://www.lupuspfdd.org>.

The pre-meeting survey data, remarks from panelists and audience participants at the meeting, meeting polling question data, and public comments yielded important information on the daily impacts of lupus and current treatments. Key themes from this information are summarized below and are explored in greater detail in this Report.

Lupus has a devastating impact on the lives of many people living with the disease.

- The physical and emotional impact of lupus is immense due to the nature of the disease and the side effects of available treatments.
- Lupus has a major impact on peoples' work, social, and family life. Many people with lupus express substantial concern about the future.
- Many people with lupus experience loss of identity and self-esteem, and worsening quality of life, which may be exacerbated by the stigma of the disease and the fact that friends, family members, and co-workers do not understand the impact of a disease that can be largely invisible.

Lupus disproportionately affects women and minorities.

- The majority of people with lupus are women, and most women are diagnosed in their child-bearing years or prime of life, although men, older individuals, and children are also affected.
- African-American, Hispanic and Latino, Asian and American Indian/Alaska Native women are disproportionately affected and experience lupus onset at an earlier age.
- African-American, Hispanic and Latino, and American Indian/Alaska Native individuals with lupus also experience more severe lupus than do whites with lupus, with more internal organ and kidney damage and greater mortality.

Lupus is highly variable and heterogeneous, resulting in diagnosis, treatment, and clinical trial design challenges.

- Fatigue and pain in the joints and muscles were ranked as symptoms that have the most impact on well-being by more than half of survey respondents.
- Cognitive problems such as forgetfulness and lack of concentration – often called “lupus fog” or “brain fog” – also ranked high among the most impactful symptoms.
- Other problems that ranked among the most impactful symptoms included stomach and bowel problems, sun sensitivity, reduced physical strength, increased susceptibility to infections, depression or mood changes, organ inflammation, and kidney disease or kidney failure.

- Most respondents said that on their best days their symptoms have a moderate impact on their daily lives, while on their worst days, symptoms have a moderately high to high impact.
- A plurality of respondents said their symptoms are under moderate control, and most report many “flares.”
- Most respondents said their symptoms have worsened over time.

Treatments have limited effectiveness and often are associated with substantial adverse effects.

- Many pharmacotherapy options are commonly used to treat people with lupus. Only four drugs have been approved for lupus; and only one drug, belimumab, was created specifically to treat lupus.
- Individuals with lupus take many prescription and over-the-counter medications, and also use additional non-pharmacologic approaches including exercise, diet, and mind-body techniques to manage their symptoms.
- The side effects from lupus treatments, such as hair loss, weight gain, heart disease, infertility, and increased risk of infection, may be severe and extremely damaging to the body, increasing the burden of the disease.
- People with lupus often take many concomitant drugs, adding to the burden of managing their symptoms and making it challenging to determine whether symptoms are related to the disease or are side effects from the medications. Taking multiple drugs also increases the chance of people experiencing drug-to-drug interactions.
- Treatment regimens typically must be adjusted frequently, as the effectiveness of medications seems to decrease over time in many persons with lupus.

People with lupus desire more effective and less damaging treatments and are eager to participate in the drug development process.

- The priorities of people with lupus regarding new therapies include treatments that: cause fewer side effects and long-term health problems, improve quality of life, enable greater independence, reduce the number of pills taken, and address individual differences.
- People with lupus prefer treatment options that specifically target the active disease rather than broadly suppressing immune function.
- While most people with lupus have not participated in clinical trials, they express great willingness to consider participation.

The Lupus PFDD Initiative represented an important step for the lupus community in bringing the patient voice into the drug development process. The data collected in the pre-meeting survey and the input provided at the meeting and through post-meeting comments reflect the unique perspectives of people with lupus and are intended to help the FDA better assess drugs under review. People with lupus who participated in this initiative expressed great willingness to participate in research and urged the FDA and pharmaceutical companies to pursue improved treatments with a greater sense of urgency. Additional information on the Lupus PFDD Meeting and related data collection and analysis can be found in the full *Lupus: Patient Voices* Report.

Introduction

On September 25, 2017, members of the lupus community participated in an Externally-led Patient-Focused Drug Development (PFDD) Meeting in which individuals with lupus and their representatives had an opportunity to share their perspectives on living with disease symptoms and their experiences with treatments. Although not organized by the U.S. Food and Drug Administration (FDA), this meeting was attended by FDA officials who were able to hear from people with lupus via panel presentations, facilitated discussions, and public comments. In the months leading up to this meeting, the three convening organizations – the Lupus and Allied Diseases Association, the Lupus Foundation of America, and the Lupus Research Alliance – developed and distributed a survey for people with lupus and their representatives.

This report summarizes the input shared by people with lupus and their representatives during the PFDD meeting, as well as via the pre-meeting survey and the post-meeting comment process. Although much of that input aligns with current research on lupus and available treatments, the report content may not be representative of the entire population of people with lupus. There may be views on symptoms, impacts on daily life, treatments, and other experiences with lupus that are not reflected in this report.

Overview of lupus and available treatments

Lupus is a complex, debilitating, and poorly understood chronic inflammatory autoimmune disease. The most common form of lupus, systemic lupus erythematosus (SLE), is characterized by the production of antibodies against self-tissues, which leads to inflammation, tissue injury, and damage to any organ system of the body including the kidneys, skin, heart, lungs, joints, blood and brain. Other forms of lupus include subacute cutaneous lupus erythematosus, discoid lupus erythematosus, and drug-induced lupus erythematosus. Due to the very different nature of neonatal lupus, it was not included in the survey or data collection for this initiative.

The disease impacts every person differently, resulting in varying levels of pain, photosensitivity, rashes, fatigue, organ damage, and cognitive difficulties or “brain fog,” among other symptoms. Kidney damage is common, particularly in SLE, with the most severe cases leading to kidney failure, dialysis, and transplantation. Skin-related symptoms such as photosensitivity and rashes can occur in any type of lupus, but are frequently seen in people with subacute cutaneous lupus and discoid lupus.¹ Individuals with lupus also experience an increased incidence of premature cardiovascular disease, stroke, and early death. One study showed that a person diagnosed with lupus at age 20 has a one-in-six chance of dying by age 35.² People with lupus also frequently have other autoimmune diseases and co-morbid conditions. This adds to both the substantial burden of the disease and to the treatment challenges.

Lupus predominantly affects women, with 80% of new diagnoses made in women during their childbearing years.³ Children may also be diagnosed with SLE⁴ and may experience more severe disease.⁵ Lupus disproportionately affects women of color, with the highest prevalence rates among Black women, followed by Hispanic and Asian women.^{6,7,8} Minority women tend to develop lupus at a much younger age, experience more serious complications, and have higher mortality rates, up to three times the incidence and mortality of Caucasians.⁹ Responses to medications may also vary by race.

No single test exists to identify lupus, resulting in delayed diagnosis and intervention that can lead to more severe disease manifestations and worse patient outcomes. Since lupus is less common in men, clinicians may fail to recognize it, further delaying diagnosis and treatment. Diagnostic uncertainty also presents a significant obstacle for drug development. The complex, unpredictable, and variable nature of lupus makes it difficult to assess and compare longitudinal disease activity across the patient

population, complicating the design of clinical trials. Better outcome measures and biomarkers are clearly needed. In addition, clinical trials have been limited by an insufficient number of individuals willing or able to participate, particularly from the minority groups that lupus afflicts most. Smaller and shorter trials with better outcome assessments and biomarkers could reduce the burden on participants, thus maximizing the efficiency of trials.

Many different pharmacotherapy options are prescribed for people with lupus, including off-label treatments. However, only four treatments have been approved by the FDA since 1948: aspirin, corticosteroids, hydroxychloroquine, and belimumab. Of those approved treatments, only one – belimumab – was developed specifically for lupus and is only approved in combination with the standard of care. Individuals with lupus also use a variety of over-the-counter medications, supplements, and non-pharmacologic treatments. Antimalarials such as hydroxychloroquine, non-steroidal anti-inflammatory drugs (NSAIDs), and corticosteroids are commonly prescribed as first-line treatment.¹⁰ Immunosuppressive agents such as cyclophosphamide, azathioprine, mycophenolate mofetil, and methotrexate are often prescribed as well. Both corticosteroids and immunosuppressants have been associated with frequent serious adverse effects.¹¹ It has only been recently that new biologic therapies have been explored, which led to the approval of belimumab.¹² Other biologics such as rituximab are also used off-label for lupus, and there are many other immune cell-targeted and anti-cytokine therapies that may be tried as treatments for lupus,¹³ with other biologics in development.¹⁴

Although individuals with subacute cutaneous lupus and discoid lupus are often prescribed many of the same medications used for SLE, additional treatments are also used to manage the different symptoms of these types of lupus. Topical corticosteroids are a common first approach, and retinoids, dapson, and steroids are also frequently prescribed.¹⁵ For advanced cases, thalidomide or high-dose intravenous immunoglobulins may be used, although some providers and people with lupus may opt to avoid these treatments due to the high risk of side effects.¹⁶

The physical and emotional toll of having lupus is high, both because of the devastating impact of the disease itself and the substantial adverse effects of treatment. In one study, for example, over a four-year follow-up period, a third of individuals with lupus who participated in the study stopped working.¹⁷ Given the high morbidity and mortality of lupus, the limited response to current therapies, and the high incidence of adverse events, there is an urgent, unmet need for more therapeutic options.

Meeting design and data collection

Work on the Lupus PFDD Initiative began in late 2015 when the three convening organizations submitted a letter of intent to the FDA with their proposed plan for the meeting, data collection, and report. The initiative, including the design and planning process for the meeting and data collection, was managed by a Steering Committee with representatives from the three convening organizations. The three organizations worked together collaboratively, with support from Faegre Baker Daniels Consulting, to plan the meeting, design and distribute the survey, and analyze the survey data. The following individuals served on the Steering Committee:

- Kathleen Arntsen, President and CEO, Lupus and Allied Diseases Association;
- Pat Wildman, Vice President, Advocacy and Government Relations, Lupus Foundation of America; and
- Diane Gross, National Director of Advocacy and Programs, Lupus Research Alliance.

Persons with lupus and their representatives (defined as parents or legal guardians of children with lupus) were invited to respond to a survey that was designed to yield better understanding of the

experience of living with lupus. The survey was based on similar polling questions used at FDA-led PFDD meetings and was expanded to capture additional detail about disease symptoms, impacts on daily life, and experiences with treatments. The survey was designed by the three organizing groups with input from lupus experts, including clinicians. The 46-question survey was available for over two months on a dedicated website and in paper form, and both formats were available in English and Spanish. See Appendix 5 for the English version of the survey. The survey was distributed through a variety of channels including lupus community networks, websites, social media, and e-mail blasts and as well as hard copies distributed at events, support groups, and a camp for children with lupus. Complete responses were received from 2,121 individuals in the U.S., of whom 98% were individuals with lupus and 2% were parents or representatives of children with lupus. Responses were received from people across the U.S. and over 100 people with lupus who live in other countries, although the data from international respondents were not included in the analysis for this report.

The survey was used to inform the meeting content and the selection of participants. Meeting panelists and audience participants were chosen based on their survey responses in order to represent robust and diverse perspectives. Characteristics considered in the selection process included: type of lupus, symptomology, age at diagnosis, time from development of symptoms to diagnosis, years since diagnosis, age, race, ethnicity, gender, state/region, severity of disease, and past/current treatments. Many of the selected participants were not previously known to any of the lupus organizations convening the meeting. The meeting was open for public attendance and a number of other people with lupus and members of the lupus community also participated.

The meeting was held in Hyattsville, Maryland to facilitate participation and observation by FDA representatives. James Valentine, JD, MHS, an attorney with Hyman, Phelps & McNamara, PC served as the meeting moderator and began the day by welcoming both in-person and webinar attendees. Valentine previously worked at the FDA where he helped to launch the PFDD program. Lupus community representative Kathleen Arntsen followed with a history of the community, then highlighted the critical need for better therapies that included a firsthand account of the challenges facing individuals with lupus. She ended with a call to action, encouraging those with lupus to openly share their personal stories. Her presentation was followed by remarks from Pujita Vaidya, MPH, Acting Director of the Decision Support and Analysis Team in the Office of Strategic Programs at the FDA's Center for Drug Evaluation and Research (CDER), who described the FDA's PFDD Initiative and Benefit-Risk Framework. In an interview-style session facilitated by Debra Lappin of Faegre Baker Daniels Consulting, Janet Woodcock, MD, Director of CDER provided her insights both as the leader of the drug evaluation team and as a rheumatologist experienced in treating people with lupus. Anca Askanase, MD, MPH, Associate Professor of Medicine and Director of the Lupus Center at Columbia University Medical Center presented a clinical overview of lupus and available treatments. Near the end of the meeting, Nikolay Nikolov, MD, Clinical Team Lead in the CDER Division of Pulmonary, Allergy, and Rheumatology Products provided summary marks of the major themes discussed that day. The moderator concluded the meeting with powerful quotes from meeting participants.

The majority of the meeting consisted of two sessions that each focused on a specific discussion topic:

- **Topic 1 – Perspectives of people with lupus and their representatives on disease symptoms and daily impacts that matter most.** Questions posed included what symptoms have the most impact and why; what activities are no longer possible because of lupus; how the burden of having lupus varies from day to day and over time; and major concerns for the future.

- **Topic 2 – Perspectives of people with lupus and their representatives on current approaches to treating lupus and desires for future treatments.** Questions asked included how well available treatments work; what adverse effects have been experienced; and what they desire from a future treatment.

For each topic, a panel of six individuals with lupus and/or representatives of people with lupus presented brief summaries of their experiences. Meeting panelists were selected by the Steering Committee. Panelists were selected with the goal of representing the broad spectrum and diverse perspectives of people with lupus, while recognizing that the full range of experiences would be impossible to capture in a small panel. Meeting attendees then responded to a series of polling questions similar to those asked in the survey. Polling data are not included in the analysis for this report as the live polling was only intended to guide discussion at the meeting. Polling was followed by a moderated discussion in which audience members were invited to share their perspectives.

Demographic data for survey respondents

Of the 2,121 individuals who submitted complete* surveys, nearly all were female (96.5%). Other demographic characteristics are presented in Table 1. For purposes of this report, only data received from respondents in the U.S. were used. Duplicate or largely incomplete surveys were also excluded from the analysis. Although the meeting organizers were pleased with the robust response to this survey from the lupus community, the data and accompanying analysis are not necessarily representative of the entire population with lupus.

Table 1: Race/ethnicity (one selection allowed per respondent) and age data of survey respondents. N = 2,121.

Survey Respondents by Race or Ethnicity		Survey Respondents by Age	
White	65.9%	Under 18	1.5%
Black or African-American	14.8%	18-29 years	11.7%
Hispanic or Latino	11.3%	30-39 years	19.9%
Asian	3.0%	40-49 years	24.2%
Native Hawaiian or Other Pacific Islander	0.9%	50-59 years	23.8%
American Indian or Alaska Native	0.7%	60 years or older	18.9%
Other	3.4%		

This report includes analysis of the data from many of the questions included in the survey and is intended to capture the most important themes from that data collection effort and the remarks at the Lupus PFDD Meeting. Additional analyses of the survey data may be conducted in the future to gain further insights into the experiences of people with lupus.

Disease characteristics of survey respondents

Nearly three quarters (72.7%) of survey respondents were given a diagnosis of SLE without nephritis (Diagram 1). About 20% were given a diagnosis of SLE with nephritis, and a small minority received a diagnosis of cutaneous lupus erythematosus (either subacute or discoid lupus) or drug-induced lupus erythematosus. Among those with nephritis, only about 4% were on dialysis and/or had undergone a kidney or other organ transplant. When viewing this data across different races and ethnicities (Table 2), SLE without nephritis was the most commonly reported lupus diagnosis in all groups except Native Hawaiians or Other Pacific Islanders, more of whom responded that they were diagnosed with SLE with

* Survey questions on respondents' race/ethnicity, age, sex, ZIP code, and how long they have had lupus were mandatory. All other questions were optional, although responses that were largely incomplete were not included in the analysis for this report.

nephritis. As is supported by other research, Black and Hispanic respondents reported higher rates of diagnosis of discoid lupus than whites. Although the comparison of lupus diagnosis across race/ethnicity groups offers an interesting view of this data, it is also worth noting that the data may be less representative of the overall population of non-SLE groups that had lower representation among survey respondents.

Diagram 1: Lupus diagnosis of survey respondents (select one). N = 2,108.

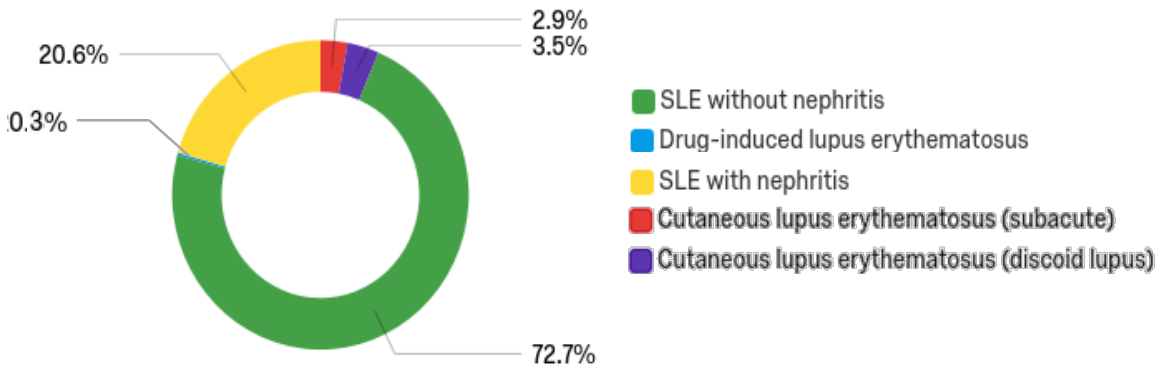


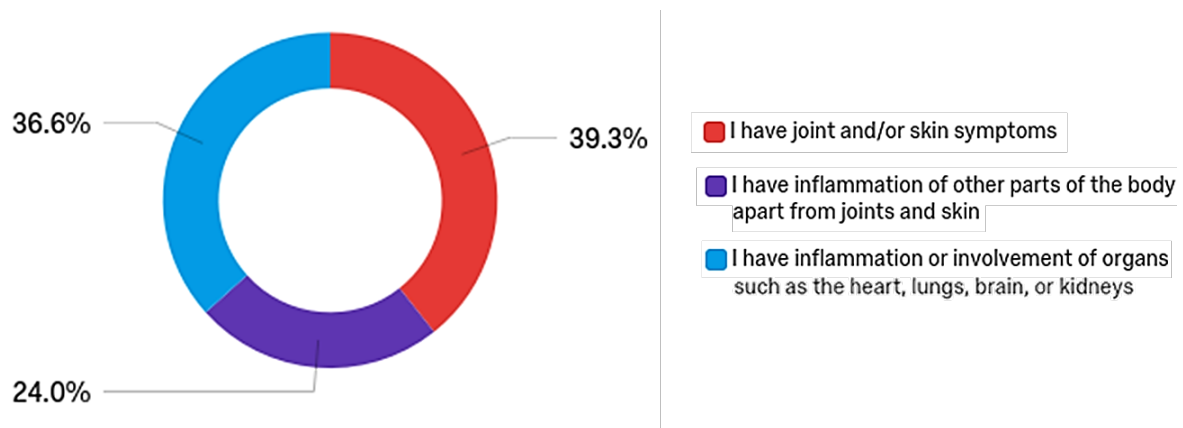
Table 2: Lupus diagnosis by race/ethnicity. N = 2,108.

	American Indian or Alaska Native	Asian	Black or African American	Hispanic or Latino	Native Hawaiian or Other Pacific Islander	White	Other
	%(N)	%(N)	%(N)	%(N)	%(N)	%(N)	%(N)
Cutaneous lupus erythematosus (subacute)	--	1.6(1)	2.9(9)	3.0(7)	--	3.2(44)	1.4(1)
Cutaneous lupus erythematosus (discoid lupus)	--	1.6(1)	6.4(20)	4.2(10)	10.5(2)	2.7(38)	2.7(2)
Drug-induced lupus erythematosus	--	--	0.6(2)	0.4(1)	--	0.2(3)	--
Systemic lupus erythematosus (SLE) without nephritis	76.9(10)	57.8(37)	61.8(194)	67.4(159)	41.2(8)	77.3(1,074)	69.9(51)
Systemic lupus erythematosus (SLE) with nephritis	23.1(3)	39.1(25)	28.3(89)	25.0(59)	47.4(9)	16.6(230)	26.0(19)
Total	13	64	314	236	19	1,389	73

Survey respondents were almost evenly divided in how they described their lupus (Diagram 2). Just over one third (39.3%) said they have joint and/or skin symptoms, 36.6% said they have inflammation or involvement of organs, and 24.0% reported inflammation of body parts other than joints and skin. Most

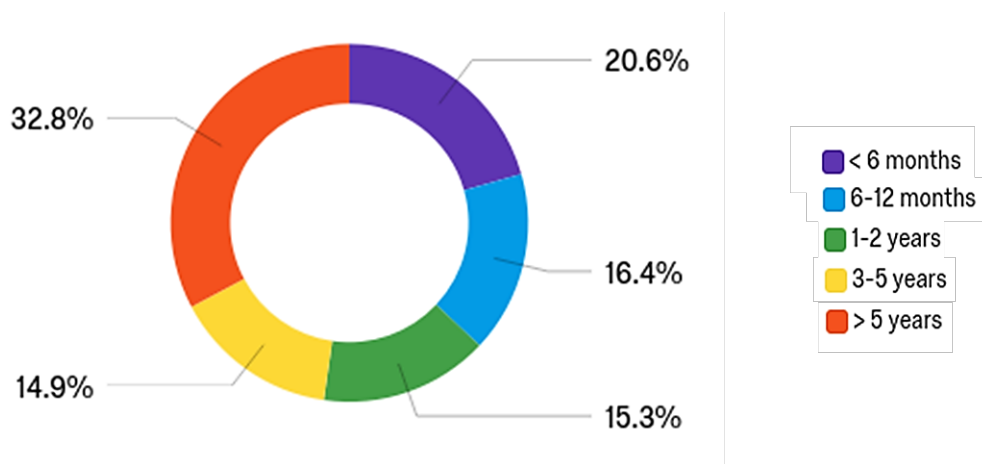
respondents (56.5%), regardless of the type of symptoms they have, said their lupus is under moderate control (some flares, possibly requiring dose changes in current medications).

Diagram 2: Responses to the question, “What best describes your lupus? (Choose one)”. N = 2,110.



Three quarters (77.5%) of survey respondents reported that their lupus symptoms began before the age of 39, with approximately one quarter (23.1%) reporting that their symptoms began before age 18. Of note, however, is that more than five years passed from symptom onset before receiving an official diagnosis in one third (32.8%) of respondents (Diagram 3). Only slightly more than one third (37.0%) received an official diagnosis within one year of the appearance of symptoms. Delayed diagnosis not only delays effective treatment and is associated with increased disease severity, but could also complicate enrollment in clinical trials.

Diagram 3: Time between appearance of lupus symptoms and official diagnosis. N = 2,121.



Topic 1: Impact of Disease Symptoms

Most distressing symptoms

The survey asked individuals with lupus or their representatives to select and rank the symptoms that most negatively impact their lives. As shown in Table 3 below, joint and muscle pain and/or swelling and fatigue were ranked as the most burdensome symptoms by more than half (63.5%) of respondents. As shown below in Tables 4 and 5, the symptoms with the most negative impact varied across different race/ethnicity groups and different types of lupus. Although many of the symptoms on the list were selected by relatively small percentages of respondents, remarks at the meeting and post-meeting comments made it clear that these symptoms have a major impact on individuals' lives. The wide array of symptoms that survey respondents experience and the varying levels of burden that the symptoms have on each person demonstrate the heterogeneity of lupus.

Table 3: Lupus symptoms with the most negative impact. Survey respondents were asked to select and rank the three lupus symptoms that most negatively impact their life (a rank of 1 being most impact). Responses below are for the symptoms that received a rank of 1. N = 2,076.

Symptom	% (N)
Joint and muscle pain and/or swelling	31.9 (662)
Fatigue	31.6 (656)
Renal (kidney) disease or renal failure	5.6 (117)
Increased susceptibility to infections	3.6 (74)
Blood clots	3.1 (65)
Stomach or bowel problems	2.9 (61)
Sun sensitivity	2.8 (58)
Reduced physical strength	2.6 (55)
Rashes	2.3 (48)
Pleurisy (inflammation of the lining of the lung)	2.1 (43)
Depression	1.9 (40)
Pericarditis (inflammation of the lining of the heart)	1.3 (28)
Hair loss	1.2 (25)
Shortness of breath	1.1 (23)
Forgetfulness	1.1 (22)
Lack of concentration	1.0 (21)
Sleep disturbances	0.9 (19)
Weight gain	0.8 (17)
Seizures	0.6 (12)
Mouth sores	0.5 (10)
Miscarriages	0.4 (9)
Fevers	0.3 (7)
Mood changes	0.2 (4)
Peritonitis (inflammation of the lining of the abdomen)	0.0 (0)
Total	2,076

When viewing the impact of symptoms across other characteristics of respondents, comparatively larger percentages of respondents with cutaneous subacute lupus and discoid lupus selected rashes and sun sensitivity, respectively, as having high impacts. These results were not surprising given that these types of lupus more significantly impact the skin. Similarly, respondents who had SLE with nephritis were more likely to choose renal disease or failure as a high-impact symptom. These as well as other symptoms are shown below in Table 4.

Table 4: Lupus symptoms with the most negative impact – breakout by type of lupus. Survey respondents were asked to select and rank the three lupus symptoms that most negatively impact their life (a rank of 1 being most impact). Responses below are for the symptoms that received a rank of 1. N = 2,061.

Diagnosis	Cutaneous lupus erythematosus (subacute)	Cutaneous lupus erythematosus (discoid)	Drug-induced lupus erythematosus	SLE without nephritis	SLE with nephritis
	% (N)	% (N)	% (N)	% (N)	% (N)
Blood clots	--	--	--	3.1 (48)	3.7 (16)
Depression	3.2 (2)	4.1 (3)	--	1.3 (20)	3.2 (14)
Fatigue	37.1 (23)	21.9 (16)	33.3 (2)	33.0 (506)	24.2 (105)
Fevers	--	--	--	0.4 (6)	0.2 (1)
Forgetfulness	--	1.4 (1)	--	1.1 (17)	0.9 (4)
Hair loss	6.5 (4)	2.7 (2)	--	0.9 (14)	1.2 (5)
Increased susceptibility to infections	1.6 (1)	1.4 (1)	1 (1)	3.7 (56)	3.5 (15)
Joint and muscle pain and/or swelling	16.1 (10)	34.2 (25)	33.3 (2)	33.8 (518)	24.2 (105)
Lack of concentration	1.6 (1)	1.4 (1)	--	1.0 (16)	0.7 (3)
Miscarriages	--	--	--	0.4 (6)	0.7 (3)
Mood changes	--	2.7 (2)	--	0.1 (2)	--
Mouth sores	--	1.4 (1)	--	0.6 (9)	--
Pericarditis	--	1.4 (1)	--	1.3 (20)	1.6 (7)
Peritonitis	--	--	--	--	--
Pleurisy	--	--	--	2.5 (39)	0.9 (4)
Rashes	11.3 (7)	6.8 (5)	16.7 (1)	1.8 (27)	1.6 (7)
Reduced physical strength	4.8 (3)	1.4 (1)	--	2.9 (44)	1.6 (7)
Renal (kidney) disease or renal failure	--	--	--	1.7 (26)	21.0 (91)
Seizures	--	1.4 (1)	--	0.5 (7)	0.7 (3)
Shortness of breath	--	--	--	1.2 (18)	1.2 (5)
Sleep disturbances	3.2 (2)	--	--	0.5 (8)	2.1 (9)
Stomach or bowel problems	4.8 (3)	4.1 (3)	--	2.9 (44)	2.3 (10)
Sun sensitivity	4.8 (3)	9.6 (7)	--	2.6 (40)	1.4 (6)
Weight gain	--	--	--	0.7 (10)	1.6 (7)
Total	59	70	6	1,499	427

When considering this data across different races and ethnicities, (Table 5), fatigue and joint and muscle pain and/or swelling were listed among the top three highest impact symptoms for each group. A comparatively higher percentage of Black and American Indian or Alaska Native respondents selected renal disease or failure as a high-impact system. Hispanic or Latino and white respondents were also more likely to list stomach or bowel problems in their top three symptoms. Given the smaller sample sizes of some race/ethnicity groups, however, these data are not necessarily representative of the entire population with lupus.

Table 5: Lupus symptoms with the most negative impact – breakout by race/ethnicity. Survey respondents were asked to select and rank the three lupus symptoms that most negatively impact their life (a rank of 1 being most impact). Responses below are for the symptoms that received a rank of 1. N = 2,076.

Race/Ethnicity	American Indian or Alaska Native	Asian	Black or African American	Hispanic or Latino	Native Hawaiian or Other Pacific Islander	White	Other
	% (N)	% (N)	% (N)	% (N)	% (N)	% (N)	% (N)
Blood clots	7.7 (1)	3.2 (2)	2.6 (8)	3.0 (7)	--	3.3 (44)	4.3 (3)
Depression	--	3.2 (2)	3.9 (12)	4.3 (10)	--	1.0 (14)	2.9 (2)
Fatigue	15.4 (2)	17.7 (11)	26.1 (80)	23.9 (56)	31.6 (6)	34.4 (465)	22.9 (16)
Fevers	7.7 (1)	--	1.3 (4)	0.9 (2)	--	--	--
Forgetfulness	--	--	1.0 (3)	0.9 (2)	--	1.0 (14)	4.3 (3)
Hair loss	--	--	2.3 (7)	1.7 (4)	--	0.9 (12)	2.9 (2)
Increased susceptibility to infections	--	11.3 (7)	2.0 (6)	3.0 (7)	5.3 (1)	3.7 (50)	4.3 (3)
Joint and muscle pain and/or swelling	46.2 (6)	32.3 (20)	30.0 (92)	32.9 (77)	31.6 (6)	32.9 (444)	24.3 (17)
Lack of concentration	--	3.2 (2)	0.3 (1)	0.4 (1)	--	1.1 (15)	2.9 (2)
Miscarriages	--	1.6 (1)	--	0.4 (1)	--	0.4 (6)	1.4 (1)
Mood changes	--	--	0.3 (1)	--	--	0.1 (1)	2.9 (2)
Mouth sores	--	--	1.3 (4)	0.4 (1)	--	0.4 (5)	--
Pericarditis	--	--	1.0 (3)	0.9 (2)	5.3 (1)	1.6 (22)	--
Peritonitis	--	--	--	--	--	--	--
Pleurisy	--	1.6 (1)	3.6 (11)	2.1 (5)	--	1.9 (26)	--
Rashes	--	1.6 (1)	3.3 (10)	3.0 (7)	--	2.1 (28)	2.9 (2)
Reduced physical strength	7.7 (1)	1.6 (1)	3.6 (11)	3.0 (7)	--	2.4 (33)	2.9 (2)
Renal (kidney) disease or renal failure	7.7 (1)	14.5 (9)	9.4 (29)	7.7 (18)	15.8 (3)	3.6 (49)	11.4 (8)
Seizures	7.7 (1)	--	0.3 (1)	0.4 (1)	--	0.7 (9)	--
Shortness of breath	--	--	1.3 (4)	1.7 (4)	--	1.0 (14)	1.4 (1)
Sleep disturbances	--	1.6 (1)	0.7 (2)	2.6 (6)	5.3 (1)	0.6 (8)	1.4 (1)
Stomach or bowel problems	--	--	2.3 (7)	3.8 (9)	--	3.1 (42)	4.3 (3)
Sun sensitivity	--	6.5 (4)	2.0 (6)	2.6 (6)	5.3 (1)	3.0 (40)	1.4 (1)
Weight gain	--	--	1.6 (5)	0.4 (1)	--	0.7 (10)	1.4 (1)
Total	13	62	307	234	19	1,351	70

Examples of comments below by meeting attendees powerfully illustrate the impact of disease symptoms on the lives of people with lupus, and several symptoms emerged that may not have been captured by survey data.

Fatigue

Many individuals with lupus or their representatives spoke of debilitating fatigue, lack of energy, difficulty getting out of bed in the morning, and their need for many hours of sleep, including daytime

naps. In separate questions in the survey, a large majority of respondents (85.4%) said they experienced disrupted sleep/wake cycles; both of which could be related to fatigue or drug side effects.

An American Indian woman in her 30s said, “I require far more sleep than any of my friends do. While most people consider naps a luxury, they are an absolute necessity for me. I find it difficult many days to plan activities and chores while leaving a block of time open to just simply rest.”

“I feel like I never really fully recharge, and I feel like if most of us could find a solution to the fatigue we could deal with a lot of the other symptoms that we’re dealing with because we at least are able to do things for ourselves and not feel just so debilitated by something that we have no control over,” said a white woman in her 40s.

Joint and muscle pain

Joint and muscle pain were one of the most common symptoms that individuals with lupus said most negatively impact their lives. Just over half (54.0%) of respondents said it takes them more than one hour to get moving or loosen up in the morning. Many individuals with lupus also have other conditions that can cause joint and muscle pain, such as arthritis or fibromyalgia. Regardless of the source, pain is a predominant issue for people with lupus.

“I’ve been in constant pain for 18 years. Some days it’s less painful but every day I am in pain...I get it all over and I also have fibromyalgia and arthritis so it’s basically from my shoulders to my toes. It’s the tingling, burning, inflammation pain,” said a Black woman in her 50s.

The mother of a Latina child with lupus said, “There was a point that [my daughter] was not able to walk more than 40 minutes due to joint pain. Literally, she was not able to walk.”

Pain beyond the joints and muscles

Individuals with lupus reported pain in many other parts of the body in addition to joints and muscles. In some cases, this is related to the area of the body most affected by lupus, or the particular form of lupus or other concomitant conditions, such as Raynaud’s disease. Pain also impacts other aspects of the disease. For example, pain can make sleep difficult, which can exacerbate fatigue.

A white woman in her 30s said, “Pain is a constant in my life. It varies from not being able to even walk because I cry every time I bend my knees to ... can I open that jar today. Sometimes I can’t be touched because my skin hurts so badly. Even soft clothes hurt. It feels like needles are being stabbed into my skin, over and over and over again.”

“My doctors said I have discoid lupus. This affects my skin and causes painful lesions and plaques on my fingers, toes, genitals, scalp, ears, in my ear canals, and in my nasal passages. At times these plaques sting and burn; they have led to hair loss, and some of them bleed on occasion,” said an Asian man in his 40s.

“One of the hardest symptoms I struggle with is chronic pain in my stomach. It can be hard to function in school and social situations when you have constant excruciating pain that will not go away no matter what you try,” said a Black female in her teens.

Brain fog, cognitive impairment

Survey respondents selected forgetfulness and lack of concentration as having a substantial negative impact on their lives. At the meeting, many individuals with lupus referred to this as “brain fog.” Some

respondents related this to fatigue. Participants described specific situations where an inability to think clearly has had a disabling effect on their ability to function at work or in other activities.

“My profession requires that I think on my feet and contribute to complex discussions. It’s difficult for me to participate in these discussions because I often lose my train of thought,” said a Black man in his 40s who works as an attorney.

A white woman in her 50s said, “Someone will tell me their name and a minute later I cannot remember it. Or I’ll be in a conversation and lose my train of thought. And that happens frequently; if I’m more tired it’s even worse.”

Gastrointestinal symptoms

About one in six survey respondents (16.2%) chose stomach and bowel problems as one of the top three symptoms that had the most negative impact on their lives. For example, one white woman in her 20s with a G-tube said that she cannot eat or drink much of anything and has to carry around a gastroparesis pump in a backpack at all times. Other individuals with lupus described dramatic and unexplained weight loss as a symptom that caused them and/or their families great consternation and that was one of the symptoms that led to their diagnosis.

“My lupus story started around the age of 16 when I inexplicably lost 30 pounds over the course of one summer,” recalled an American Indian or Alaska Native woman in her 30s.

“I had gone from 100 to 65 pounds in the course of a summer,” noted a Black female in her teens about her weight loss early after her lupus developed.

Sun sensitivity

Increased sensitivity to the sun – even sitting beside a window – affects persons with lupus in different ways, including causing severe fatigue, headache, nausea, rashes, and other painful skin lesions. Their inability to be outside can significantly limit their ability to take part in activities, including family events.

One Latina of unknown age described her challenges with sun sensitivity during the meeting. “I’m not able to be in the sunlight, not even a minute... SPF doesn’t really do much for me. I develop a rash that is painful--painful and just unbelievable, itchy.... If I’m by a window and I get sunlight, my fatigue increases 300 percent.”

Reduced physical strength

Survey respondents cited reduced physical strength as a symptom that often negatively impacted their lives. For example, a Black woman in her 50s said that because of reduced strength, she cannot open canned goods. “I have literally gone to tears trying to open up a can of corn with those flip tops,” she said.

Increased risk of infections

Given that many of the medications used to treat lupus suppress the immune system, people with the disease are at increased risk of contracting infections, which can be life-threatening. For example, one Latina in her 30s recalled that she picked up a stomach bug from her 3-year-old child, which led to hospitalization for kidney involvement, “Even a paper cut to us can cause an infection,” she said.

Depression and anxiety

About one in ten survey respondents (10.9%) cited depression as one of their most debilitating symptoms. A higher rate of depression (12.7%) was reported in people with cutaneous (subacute) lupus,

suggesting that depression may be connected with the social stigma associated with the presence of visible skin lesions. When considering differences by age group, the highest rate of depression (12.1%) was reported in the under-18 group, suggesting that the mental and emotional impact of the disease may be particularly concerning in children. Among meeting attendees and individuals who submitted post-meeting comments, depression and anxiety also were mentioned as serious symptoms with a dramatic impact on their lives.

A Black female in her teens said, “Because of the trauma of being diagnosed with lupus at my age, I developed depression/anxiety leaving me feeling hopeless and sad most days... It feels like every day I’m putting on a show and a smile pretending that I’m doing fine when all I want to do is cry.”

A white woman in her 40s added, “The anxiety and depression this brings is like no other. Wondering if I should plan for my financial future and retirement or if I should focus on estate planning and trusts for my son. Wondering if I should be paying off my mortgage as quickly as possible in anticipation that I may become one of the 40% disabled by systemic lupus ... But tisk tisk... we lupus patients can't afford the luxury of worry for we know anxiety and depression worsen our lupus symptoms.”

Kidney problems

Lupus can be extremely damaging to the kidneys, particularly in SLE with nephritis, which can cause edema, scarring, and, in an estimated 22% of people, end stage renal disease.¹⁸ The survey results showed that about one fifth of respondents (20.6%) were diagnosed with this type of lupus. Kidney involvement may lead to the need for dialysis or even a transplant, but even in less severe cases, it can be extremely serious.

An Asian woman in her 30s said, “A flare up for me usually includes a high-grade fever, increased edema in my lower extremities and a high creatinine reading. At its very worst, experiencing these symptoms all at once can have a negative impact in the most life-disrupting ways. The edema can become so heavy and painful that it can prevent me from walking without assistance, as well as driving or bathing without assistance. It can also affect the shape of my body which means I can only wear certain footwear or clothing. All of this affects my abilities to remain high-functioning in my full-time job and day-to-day routine.”

“Because of my limited kidney function, I cannot take pain medication,” added a Black man in his 40s.

Problems with other organ systems

Lupus can affect almost any part of the body. While the joints, skin, and kidneys are most often affected, the survey revealed a significant number of individuals who experienced inflammation of the heart and/or lungs. People with lupus who spoke at the meeting also mentioned these problems, as well as issues with the central nervous system, blood, and reproductive organs.

“Today my major issues revolve around chest pains caused by inflammation of my heart and lungs that some days make breathing both difficult and painful,” said an American Indian woman in her 30s. She added that she spent many months in the hospital while pregnant due to life-threatening blood clots in her legs and groin. “My legs have become so damaged from the chronic clotting that I now depend on prescription pain medications and compression stockings just to spend time on my feet,” she said.

A Black woman in her 40s recalled that a lupus flare led to cardiac arrest. “I was resuscitated twice, placed on hypothermia, woke up on dialysis with a defibrillator inserted to make sure that my heart would not stop beating again,” she said. “I’ve had kidney failure, respiratory failure, sepsis, bowel obstruction, had a colostomy placed and then reversed. So lupus has attacked many of my vital organs and I was being treated for symptoms and conditions as they appeared, which made it very hard for me especially since lupus can attack any organ or system in your body without warning.”

An Asian man in his 20s said, “I’ve been invaded with CNS vasculitis. From 2015 to almost June of 2016 I suffered five strokes. The last of which had me--I couldn’t--I just fell. I was taking my medicine and I fell on the ground. And I couldn’t move my left arm or my left leg.”

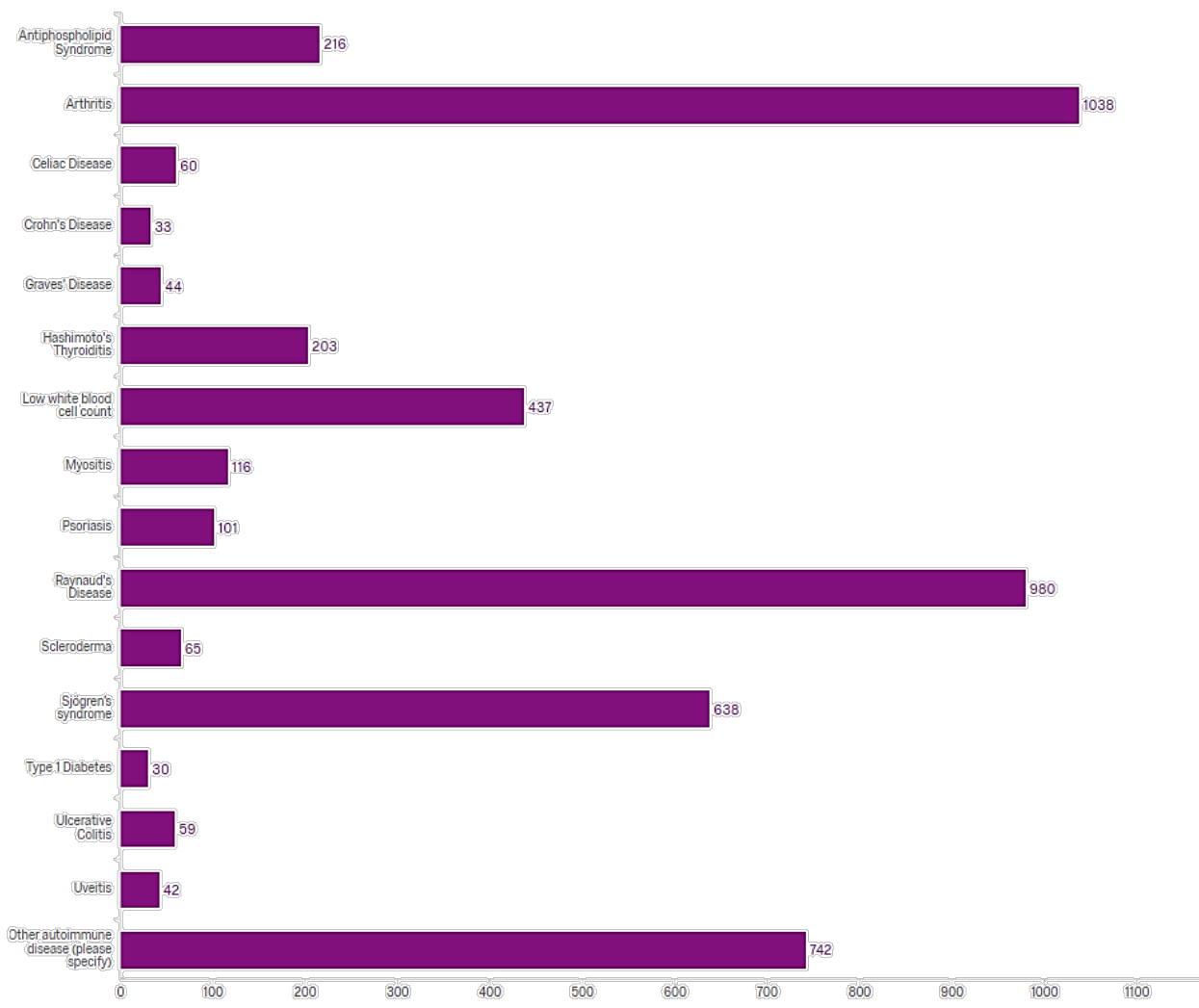
“Another symptom I was experiencing was fainting spells. The scariest is when I was home in Chicago and I had actually just spoken at a lupus event. I woke up that morning feeling fine. I was excited to go to the event. I was a block away from the train station to go home. I was crossing one of the biggest intersections in Chicago and I fainted in the middle of the street with oncoming traffic,” said a Latina in her 30s.

A white woman in her 50s added, “The thing lupus really robbed me of was the chance to have kids because I had ovarian failure when I was 27.”

Symptoms possibly related to other conditions

Individuals with lupus frequently have other autoimmune disorders, as well (Diagram 4). The survey results supported the high presence of overlapping conditions, with respondents reporting being diagnosed with an average of 2.3 other autoimmune diseases. The actual number of autoimmune diseases could be higher given that many respondents indicated that they had one or more other diseases not included as an option in the survey. Approximately half of individuals reporting having a diagnosis of arthritis (49.0%) and/or Raynaud’s (46.2%), and about one third (30.1%) reported having Sjögren’s syndrome.

Diagram 4: Other autoimmune diseases diagnosed in respondents (select as many as apply). N = 2,119.



Meeting attendees described their symptoms this way:

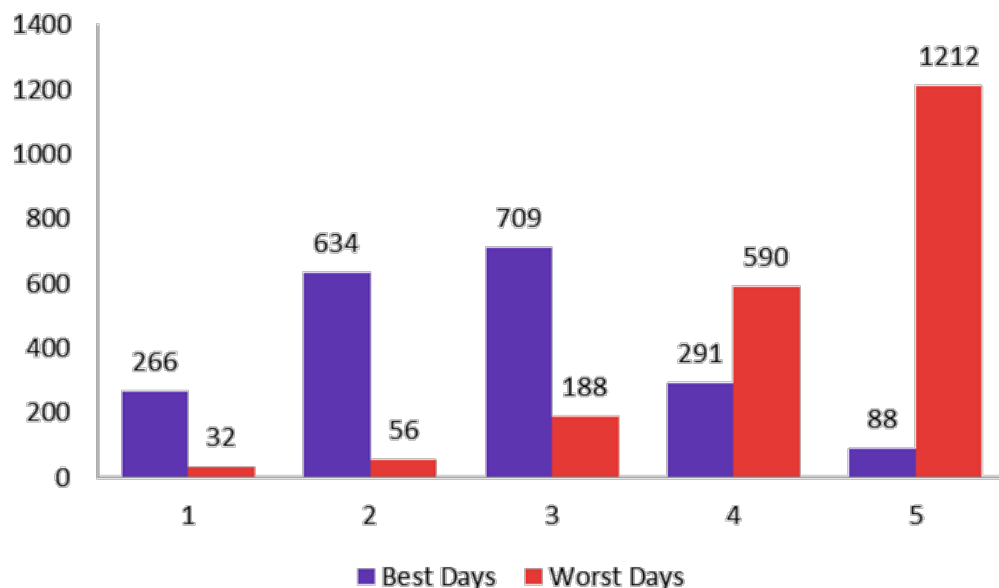
One woman of unknown race and age noted that Sjögren's syndrome presents some of the most challenging symptoms. "The dry mouth, the dry eyes, especially this time of year, the leaves are blowing and so is the dust into my eyes, or in the summer you know it seems like the sun once it gets that summer equinox in July it just dries out every piece of moisture in my eyes...water just doesn't satisfy you. Drinking soda, coffee, and tea will exhaust you later in the day. It--I just never knew that something as dryness could really change your life like that."

"Raynaud's is so painful a handshake feels it would crush me like a hammer to glass," said a white woman in her 40s.

Impact of lupus symptoms on daily life

The survey asked respondents to evaluate the impact of lupus and its symptoms on their daily lives on their best and worst days (Diagram 5). On their best days, most respondents said their symptoms have moderate impact on their daily lives, while most said that on their worst days, symptoms have a moderately high to high impact. Overall, a plurality stated that their symptoms are only under moderate control, and most report many “flares”, implying that control is not optimal.

Diagram 5: Comparison of impact of lupus symptoms on respondents’ best days and worst days. A score of 1 indicates minimal impact and a score of 5 indicates high impact. N = 1,988 for best days question, N = 2,078 for worst days question.



Survey respondents also answered a question about how frequently activities of daily living are impacted by lupus (Table 6). These results showed the significant impact of lupus on people’s daily lives, with 72.0% of respondents reporting that their ability to do one or more of these activities is impacted on a daily basis. Overall, survey results showed that respondents’ ability to do one or more of these activities is far more likely to be impacted on a daily basis than on a less frequent basis. Given the sun sensitivity that many people with lupus experience, it was not surprising that relatively high percentages of respondents indicated that their ability to take part in outdoor activities was impacted on a daily basis. The specific link is not clear from the data, as this could be from preventative avoidance or actual discomfort experienced when in the sun. Far more people reported daily, rather than less frequent, challenges for all of the activities listed, highlighting the significant impact of lupus on daily life.

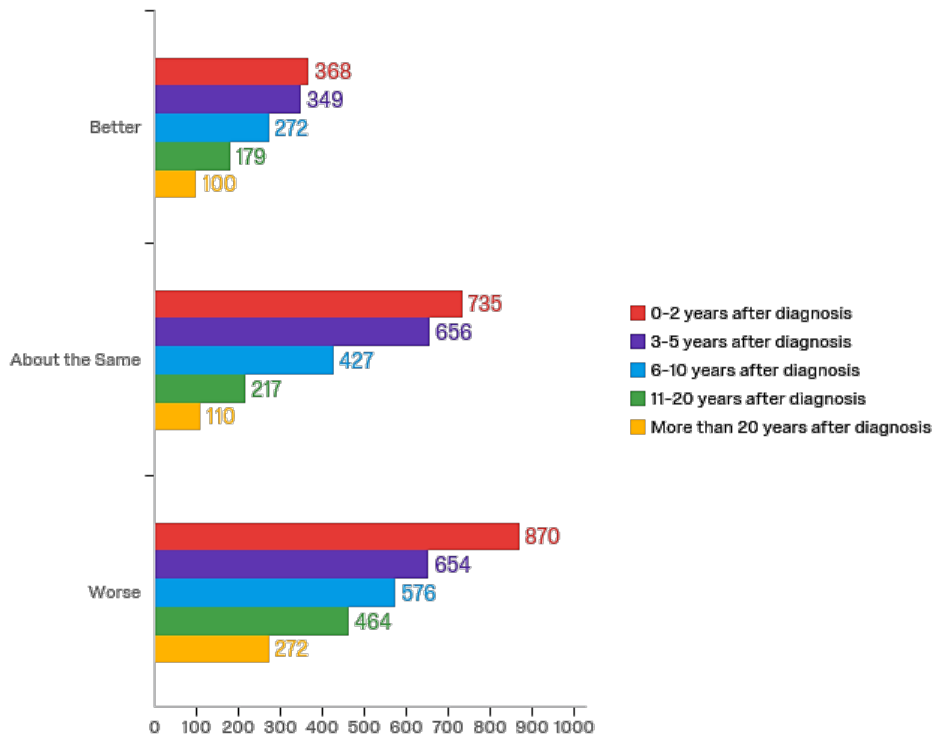
Having daily activities frequently impacted by their disease contributed to a number of meeting participants feeling that lupus contributed to a loss of independence. One mother of a Latina child with lupus noted that she has to help her daughter shower and get dressed because the pain from lupus makes it impossible for the child to do these things on her own. An African American woman in her 50s noted that fatigue and reduced physical strength make it difficult to run basic errands, saying, “I live alone. When I go grocery shopping, I call my son.”

Table 6: How often certain activities are impacted by lupus (select as many activities as are impacted by your lupus). N = 2,121.

	Daily	Several Days/Week	Weekly	Monthly	Seasonally
Activity	% (N)	% (N)	% (N)	% (N)	% (N)
Attending school	3.0 (143)	2.3 (72)	2.3 (50)	1.9 (40)	3.7 (70)
Attending work	9.6 (455)	6.8 (210)	7.5 (162)	12.4 (259)	15.4 (289)
Performing daily hygiene	6.3 (297)	13.4 (414)	11.7 (252)	8.4 (176)	10.7 (201)
Managing personal finances	4.8 (228)	7.3 (225)	10.0 (216)	14.8 (310)	8.3 (156)
Being intimate with a spouse or partner	7.4 (351)	8.4 (259)	13.7 (295)	14.9 (312)	7.4 (140)
Driving a motor vehicle	4.6 (218)	8.6 (264)	8.9 (191)	11.6 (243)	13.1 (247)
Doing outdoor activities involving direct sunlight	22.3 (1,054)	9.3 (287)	8.5 (182)	4.9 (103)	18.1 (340)
Participating in sports or social activities	16.2 (768)	13.2 (407)	12.3 (264)	11.3 (235)	10.0 (188)
Performing household chores	13.5 (640)	18.2 (559)	16.6 (356)	11.4 (239)	6.5 (122)
Taking care of children or others	8.5 (404)	10.1 (311)	7.3 (157)	7.1 (149)	6.0 (113)
Other	3.6 (170)	2.3 (71)	1.2 (25)	1.1 (22)	0.7 (14)

The survey data also indicate that people with lupus experience a worsening of symptoms over time (Diagram 6). Analyses by type of lupus showed similar patterns. Analysis by race and ethnicity, however, showed that Hispanic/Latino survey respondents reported the most dramatic worsening early after diagnosis. Comments from meeting attendees also indicate progressive worsening of disease.

Diagram 6: Changes in lupus and symptoms from time of diagnosis. N = 2,108.



Those who spoke at the meeting and submitted post-meeting comments referenced the many other ways that lupus has negatively impacted their lives, such as impact on careers, social life, family life, and concerns about the future. What follows are a few examples of these comments.

Impact on career

“I tried to go back to work with no success. I would work for a day and then I would be sick for three. If my students were sick I would catch it and I’d be out for a month. The doctors finally told me that I was no longer allowed to work,” said a Latina in her 30s.

A white woman in her 50s said, “I was a practicing RN and my specialty was HIV. And I had to give up my profession because I was no longer able to do mathematical functions and calculate drug dosages or I couldn’t remember someone’s blood pressure after I took it.”

A Black woman in her 30s said, “I was working in a laboratory, human genetics, I had to stop doing that; I had to take myself and begin working from home, but recently due to the aneurysm and the headaches that accompany it, I was let go from my most recent position.”

Impact on social and family life

An Asian man in his 40s said, “Sometimes my fatigue is so severe that I do not get out of bed until well after the kids are already in school and my wife is at work. This means that often I do not have breakfast with my family in the mornings, I do not drive our morning carpool, and I cannot have all the meaningful interactions that I would like to have with our children... the worst effect of discoid lupus has been the lesions and plaques that appear on my genitals. These affect urination and how my wife and I can be intimate with each other. Of course, this adds to even more stress on top of all of the other symptoms that I experience.”

An American Indian or Alaska Native woman in her 30s said that quality time with her sons is spent at home. “When we do venture out on good days I can only offer them short spurts of energy such as pushing them on the swings and then I must find a place to sit and rest a while. I would give anything to run and play with my sons, but lupus has stolen that from me. When friends or family invite me to spend time with them or attend functions I often RSVP with every intention of going and having a wonderful time. However, 60% of the time roughly, on the day of the event I’m too ill to leave the house and I have to cancel last minute. This unreliability is embarrassing and so disappointing to everybody involved. Many people have walked out of my life with the assumption that I’m just a terrible friend, when in fact lupus is the culprit.”

Concern about the future

“Having SLE gives me a plethora of things to worry about. I spend many sleepless nights terrified of what this cruel mystery disease has in store for my future. The idea of a shortened life expectancy and leaving my boys too soon devastates me,” said an American Indian woman in her 30s.

A woman of unknown race and age with Sjögren’s syndrome said that she worries most about the effect on her children. “My primary caregiver is 15 years old, my daughter, and she knows when I’m flaring before I do. ‘Your cheeks are red. You’re walking slow. Let me get that for you...’ I do worry about the children or the caregivers who take care of us because they see the progression and it’s so hard. Sometimes it’s so hard for them.”

A Black woman in her 60s with Raynaud's said that one of her biggest concerns arises from the hereditary nature of her illness. "I have it on my mother's side and father's side and two of my children have autoimmune illnesses, so that's a real big concern is your family and loved ones developing an autoimmune disease."

Loss of identity, self-esteem

"I used to be a competitive cheerleader and dancer that dreamed of moving on to the college level. But the symptoms I experience with lupus have prevented that from ever happening... I was always the most outgoing person in my friend groups and I never struggled in school. Since I have begun experiencing my lupus symptoms I have had to learn that this is no longer who I am," said a white woman in her teens.

A white woman in her 50s said, "You felt like a fairly high intelligence level at one point and I don't really feel like my intelligence has changed, but my ability to function and have a high-level conversation you know it's kind of affected my confidence in that level."

Quality of life

"I no longer had control over my life. I felt like it was hopeless and that I had no quality of life. Lupus became this bully that I would have to face on a day-to-day battle and I felt like I was losing," said a Latina in her 30s.

Stigma, dealing with 'invisible' illness

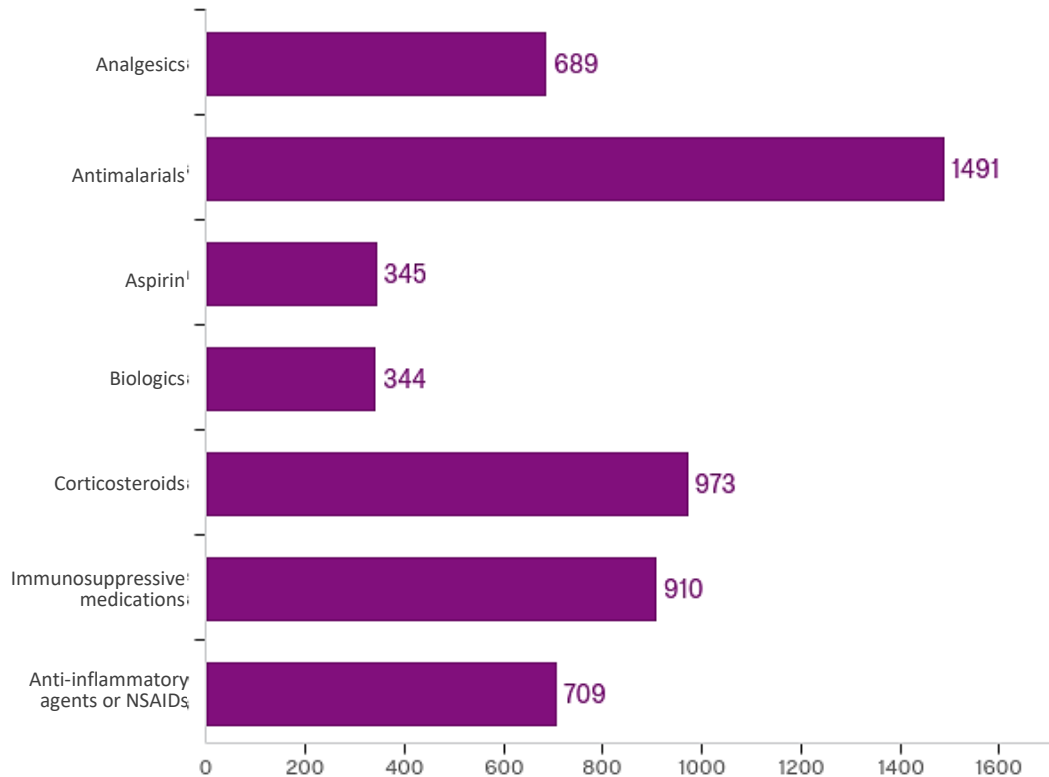
A Black female in her teens said, "My high school did not share my medical condition or medications I was taking with the staff. Because of this I looked perfectly fine and just passed out all of the sudden. The school nurse and my teacher thought I was faking it and told the paramedics that I was faking it."

"I've started to limit my activities with others because you need to walk slower or be dropped off at the door and it's hard sometimes for people to correlate how you look with how you feel," said a Black woman in her 40s.

Topic 2: Perspectives on Treatments for Lupus

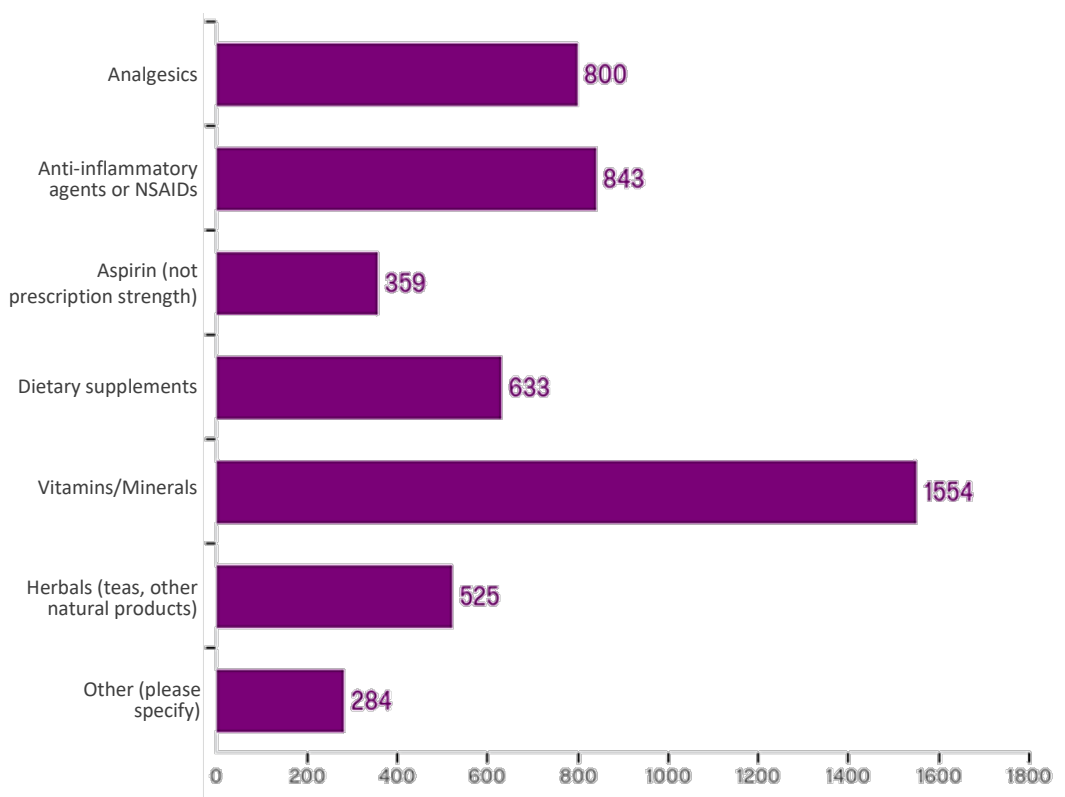
Individuals with lupus often take multiple types of medications and supplements to manage their symptoms and reduce disease activity. Respondents reported taking an average of 2.6 different types of prescription medications. Among respondents to the survey, antimalarial agents were the most common prescription medications taken to treat lupus, followed by corticosteroids, immunosuppressive medications, anti-inflammatory agents, and analgesics (Diagram 7). Prescription aspirin and biologics were used less frequently.

Diagram 7: Prescription medications currently taken for lupus (select as many as apply). N = 1,990.



In addition to prescribed medicines, many people also take over-the-counter medications such as non-steroidal anti-inflammatory drugs (NSAIDs) and analgesics for pain (Diagram 8). Just over three quarters (78.7%) of respondents take more than one over-the-counter medication for their lupus, with respondents taking drugs from an average of 2.7 of the categories included in the survey. Sometimes these over-the-counter medications are taken concurrently with prescription drugs. Most individuals who responded to the survey also take supplements, particularly vitamins and minerals (79.9% of respondents). Dietary supplements and herbals (teas and other natural products) are taken by many individuals as well: 32.6% and 27.0% of respondents, respectively. Probiotics, marijuana, fish oil, and other essential oils are also used by a number of survey respondents.

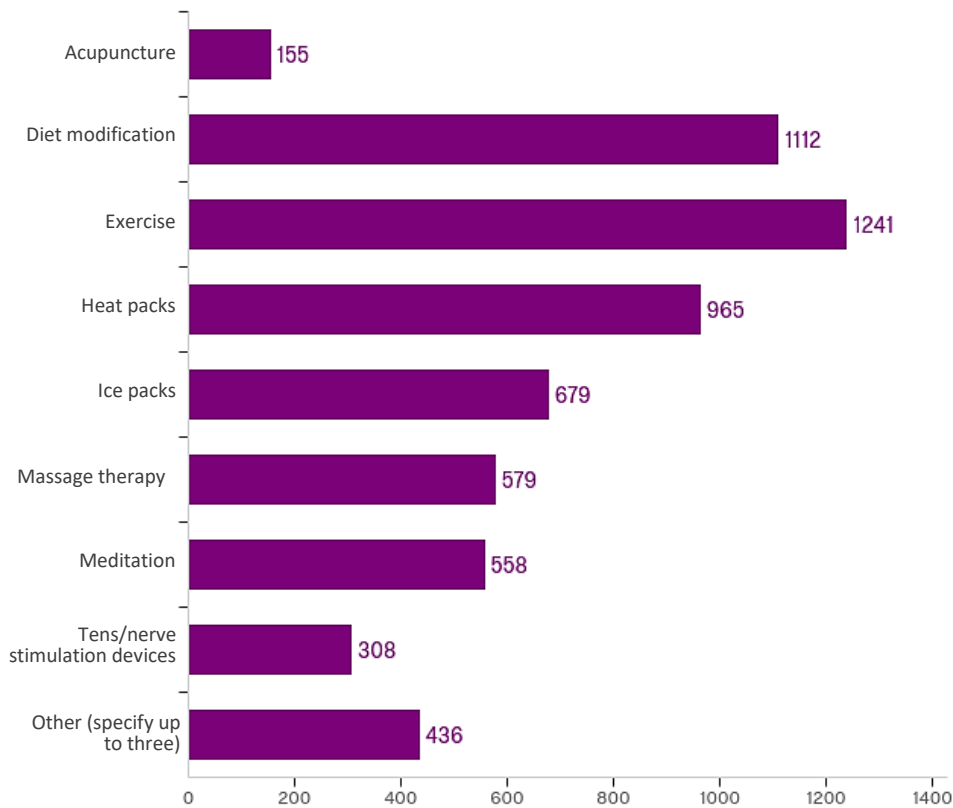
Diagram 8: Over-the-counter medications currently taken for lupus (select as many as apply). N = 1,944.



Two thirds of survey respondents (66.9%) take additional medications for other conditions, such as depression, high blood pressure and other cardiovascular conditions, osteoporosis, diabetes, anxiety, migraine, and thyroid dysfunction.

Besides prescription and over-the-counter products, most survey respondents reported using other non-pharmacologic approaches to help manage their disease (Diagram 9). Non-pharmacological approaches include diet modification, exercise, heat and ice packs, massage therapy, meditation and other mind-body approaches such as yoga, nerve stimulation devices, acupuncture, physical therapy, prayer, and rest. Diet modification and exercise appear to be the most commonly used approaches, with 57.3% and 64.0% of survey respondents, respectively, reporting that these techniques help them to manage their disease. Meeting attendees and those who submitted public comments also mentioned exercise, diet, and mind-body approaches as being beneficial. For example, one white woman in her 40s said, “I have taken a proactive approach to eating better and exercising regularly. I see an acupuncturist regularly. I wish that physical and emotional therapy were considered a part of lupus treatment, because lupus impacts the whole person physically, emotionally and spiritually.”

Diagram 9: Non-pharmacologic approaches currently used to manage lupus and its symptoms (select as many as apply). N = 1,939.



Positive impact of treatments

The survey asked people which treatments have had a positive impact on their lives. Among the prescription medications mentioned were belimumab, the antimalarial medicine hydroxychloroquine, prednisone, meloxicam, chemotherapy, and lisinopril. Survey respondents also mentioned the benefits of non-pharmacologic approaches such as acupuncture, ice packs, diet modification, and rest. Meeting participants and those who submitted public comments also referenced the benefits of belimumab, hydroxychloroquine, prednisone, and chemotherapy.

“Plaquenil [hydroxychloroquine] definitely helped with lupus fatigue or extreme fatigue... Plaquenil may improve joint pain, skin rashes, but it’s not sufficient treatment for other severe lupus symptoms and it can take up to six months to see the full benefits,” said the mother of a Latina child with lupus.

A white woman in her 30s said, “It took six months for the drug [Plaquenil] to kick in, but once it did, that was the best I’ve been in this 13-year journey... After two and a half years, Plaquenil started to not work as well for me.”

“I loved Benlysta for myself frankly. It did give me energy and that was the biggest thing, right; we all hear about the fatigue. It improved my fatigue and my daily living was significantly improved,” said a Latina in her 20s.

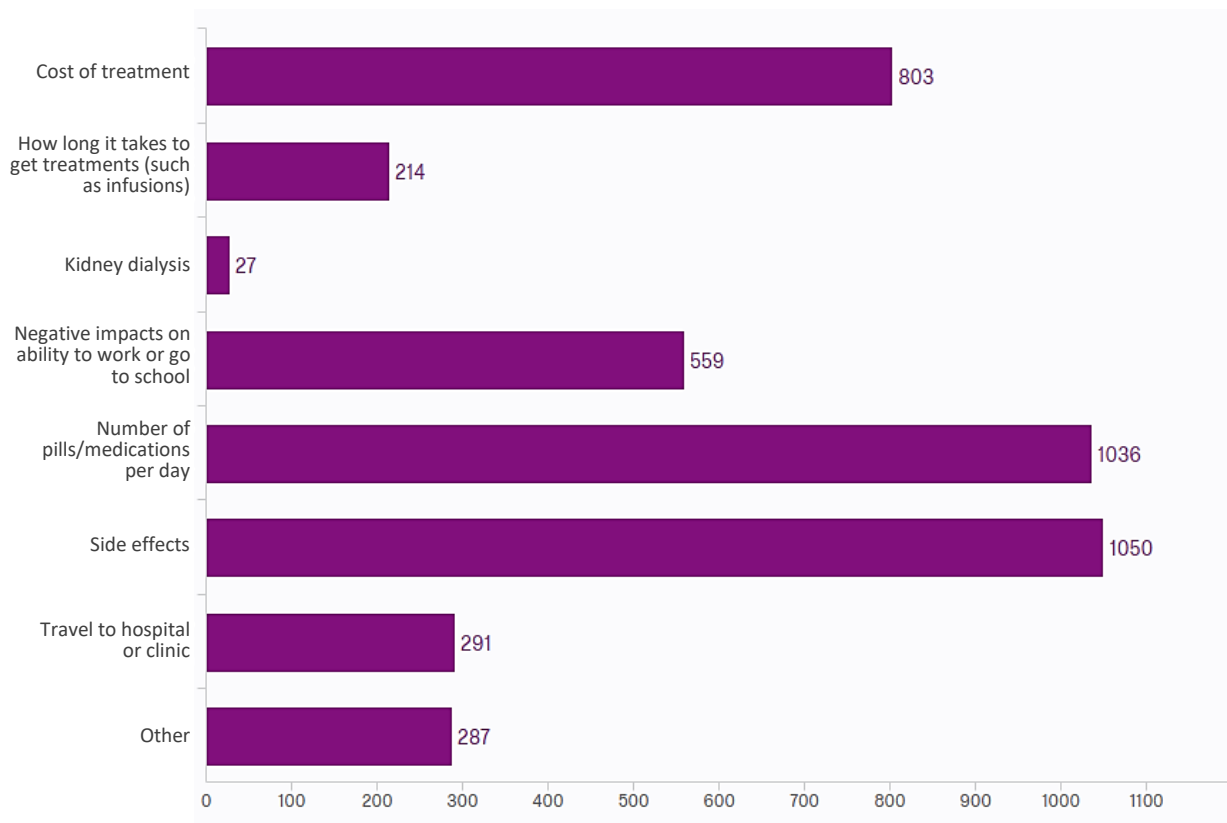
“I’m probably one of the only people in this room who will admit that I actually like prednisone. Personally for me it does wonders. When I start to wean off of prednisone I tend to flare. When I’m on high doses I seem to be fine,” said a Black woman in her 20s.

A Black woman in her 30s said, “Cytoxan. It is dangerous of course; we all know it’s chemo. It’s, you know, poison. However, it was my miracle drug. You know it gave me the closest thing that I had to remission... it was helpful with the fatigue but somehow it did help with the joint pain.”

Negative impact of treatments

The most negative aspects of treatment, according to survey respondents, are side effects and the number of pills or medications taken per day (Diagram 10). Lupus community representative Kathleen Arntsen said, for example, “I presently take 46 drugs a day. I’ve endured decades of destruction from the toxic treatments I have taken, and I used to weigh 220 pounds. I have interstitial nephritis and my entire digestive tract is impaired, so it takes five different drugs to allow me to eat food each day. The veins in my arms are useless, so I’m now on my second Infusaport for all blood work and infusions. And I am blind in my right eye.” Survey data also showed that about half of respondents (51.4%) had stopped taking a medication due to negative side effects.

Diagram 10: Biggest downsides of current treatments (select up to three). N = 1,934.



Nearly everyone who spoke at the meeting mentioned serious side effects they have experienced in their lupus journey.

In a post-meeting comment, one white woman in her 40s said, “The treatment options are so few and so heavily riddled with side effects that I have spent a good amount of time fretting over quality of life vs. quantity of life.”

“[When I was diagnosed 14 years ago] my rheumatologist started me on Prednisone that I stopped after a couple of weeks on my own because it caused my vision to blur. It increased my weight, made me feel jittery, and gave me insomnia,” said a Black woman in her 40s.

A woman of unknown race in her 50s said, “I was put on Prednisone as one of my first medicines back in 1992. I have never been off of it since that fateful day... It has broken down my skin, changed my appearance, enlarged the circumference of my head; I have a hump on my back and have gained over one hundred pounds. My hip is now degenerated to the point it needs replacement... I have cataracts and multiple growths on my skin.”

Another Black woman in her 30s said that high doses of steroids triggered steroid-induced psychosis. “I think I was hospitalized for four months; almost lost my life,” she said.

“I take methotrexate and I can actually show you pictures from an endoscopy to what some of the medications do to our stomachs. I have holes in my abdomen and I bleed through my abdomen because of some of the medications,” said a white woman in her 40s.

A woman of unknown race in her 40s said, “A series of cell inhibitors left me without an immune system. I have tried all of the non-IL-6 inhibitors. I have tried every medication unsuccessfully. At one point, a switch from methotrexate to something that was supposed to be ‘less toxic’ – azathioprine – left me hospitalized for 6 months with bilirubin, creatinine and ALT levels through the roof. Pancreatitis Amylase and lipase levels that were so high they had never been recorded that high at this hospital ever. I experienced massive headaches that left me crumpled on the floor clutching my head as the toxicity crossed the blood brain barrier and I developed septic shock.”

In addition to side effects and the number of pills required, those who responded to the survey noted other problems associated with available treatments, most notably the cost of treatment. Some treatments impacted their ability to go to work or school, both because of the time required for treatments such as infusions and because medication side effects made them feel too ill. The time and travel required to access kidney dialysis or infusion therapy was also selected as an impediment for many people.

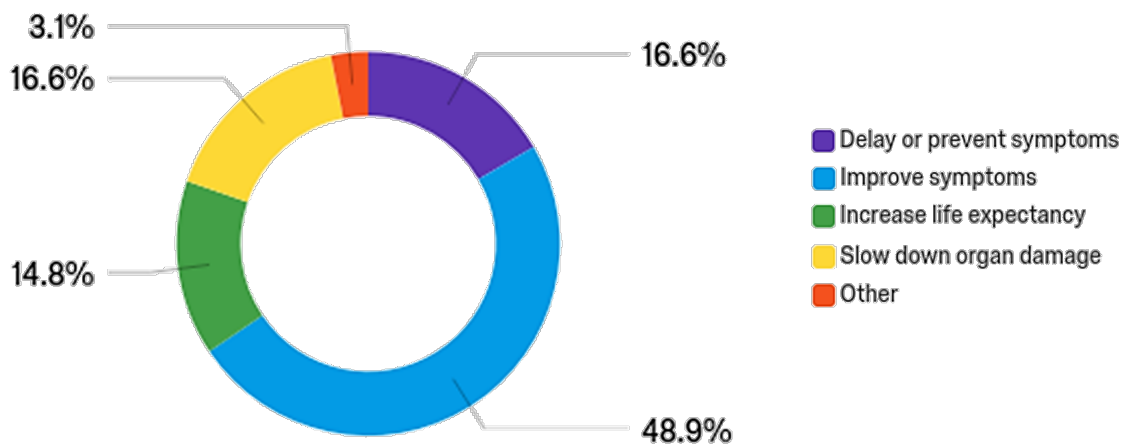
Cost and insurance concerns were also raised by meeting attendees and those who submitted public comments. Several people mentioned being denied insurance coverage because drugs are prescribed “off label.” But even belimumab, the one drug approved specifically for the treatment of lupus, has proven difficult to acquire for a number of people because of cost and insurance issues. One recently diagnosed woman of unknown race and age said that because insurance coverage was denied, she abruptly stopped taking belimumab, resulting in the return and exacerbation of symptoms. A white woman in her 20s who was diagnosed five years ago added that for those without insurance, “The cost of this life-changing drug is not only not affordable, but unattainable to all without insurance unless you are incredibly wealthy and can cover ~\$8k a month.” Medicare patients may be particularly affected,

noted another white woman in her 30s, since they may not be eligible for drug discount programs offered by pharmaceutical companies.

Factors that impact choice of treatment

When survey respondents were asked what they viewed as most important when choosing a treatment, nearly half (48.9%) said the most important benefit would be improved symptoms (Diagram 11). The other half of respondents were split almost equally on three other important considerations: delaying or preventing symptoms (16.6%), slowing down organ damage (16.6%), and increasing life expectancy (14.8%).

Diagram 11: Factors most important when choosing a treatment (select one). N = 2,086.

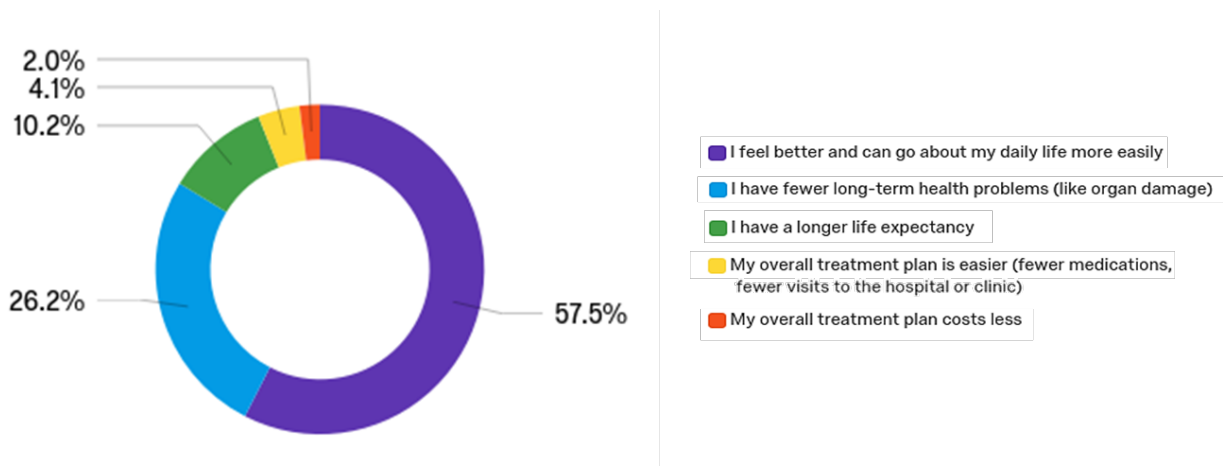


The survey also asked how much certain common side effects affect their decision to choose a treatment. The side effects that would most likely cause them to reject a treatment were eye problems (not including cataracts or glaucoma); physical effects such as skin problems, hair loss, or weight loss or gain; stomach and bowel effects, fatigue, headache, bone thinning, and mood swings. Of less concern were injection and infusion site reactions. Among less common but serious side effects, those that would most likely cause them to decide against having a treatment included stroke, kidney damage, cancer, heart damage, and liver damage. Somewhat responded said that atherosclerosis, blood disorders, glaucoma, diabetes, osteoporosis, infections, and cataracts could lead them to reject a treatment. Other side effects of concern to many individuals were birth defects and sterility, which were more frequently selected as concerns by respondents ages 18-29 years.

Treatment preferences and perspectives on an ideal treatment

The survey asked individuals with lupus and their representatives what the most important result of a new treatment would be, assuming there is no complete cure for lupus (Diagram 12). Just over half of respondents (57.5%) expressed a desire to feel better and go about their daily lives more easily. Approximately a quarter of respondents (26.2%) indicated that having fewer long-term health problems such as organ damage would be most important. A longer life expectancy, having an easier treatment plan with fewer drugs and hospital visits, or lower cost treatments were ranked as most important by small percentages of respondents.

Diagram 12: The most important result that a treatment could provide (assuming there is no complete cure for lupus)



The comments of meeting attendees reinforced the survey results. People spoke about their hopes for treatments with fewer side effects, treatments that would allow them to live more independent lives and improve their quality of life. The mother of a Latina child with lupus said, “I have helped my daughter take a shower and get dressed because she was in so much pain that she was not able to do it on her own. Creating new treatments for this will give her and many others a sense of independence.”

Respondents stated the need for treatments that recognize the individual differences in the experiences of people with lupus. For example, a white man in his 50s advocated “treatments that address the active disease in individuals, perhaps combining multiple drugs, rather than relying on treatments that broadly suppress immune function.”

At the meeting and in the post-meeting comments, people with lupus expressed a preference for treatment options that would mitigate impacts and voiced frustrations with the treatments currently available. “I’m really tired of treatments that force us to make long-term decisions in a short period of time... I want a better quality of life that’s going to last longer than the treatment or a side effect would last,” said a Black woman in her 30s. A Latina in her 50s added, “I want a drug and I want treatment that is not going to affect every organ in my body.” For people with multiple chronic illnesses, this frustration is amplified, as noted by a white woman in her 40s. “Because a treatment for one problem will worsen another... We need drugs where severe side effects are less common so that young people are not forced to make Faustian bargains with their health,” she said.

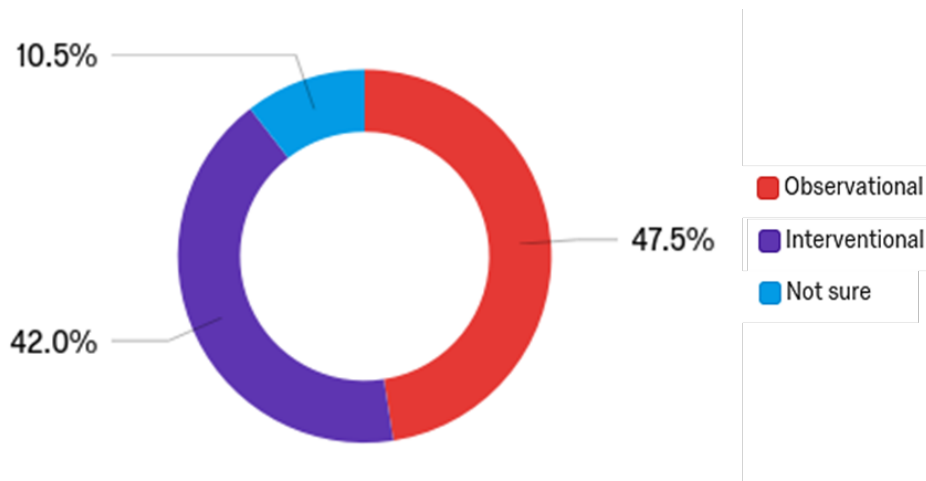
In a few post-meeting comments, members of the community provided perspectives on areas where they would like to see further progress in drug development for lupus. One woman of unknown race in her 40s encouraged drug developers and regulatory authorities to explore whether drugs that were originally developed for other conditions but not approved could be considered as possible lupus treatments. A white woman in her 60s added, “I would like to see more medical studies jointly looking at western drugs with alternative treatments; i.e. acupuncture, dietary supplements, body work, etc.” and shared her own positive experience with these types of treatments.

Participation in clinical trials

Approximately one in seven survey respondents (14.2%) indicated that they had participated in a clinical trial, and respondents have participated in both observational and interventional trials (Diagram 13). Their most common reason for participation was a desire to advance research, followed closely by a trial being recommended by a doctor. The vast majority of respondents (87.0%) who had participated in a

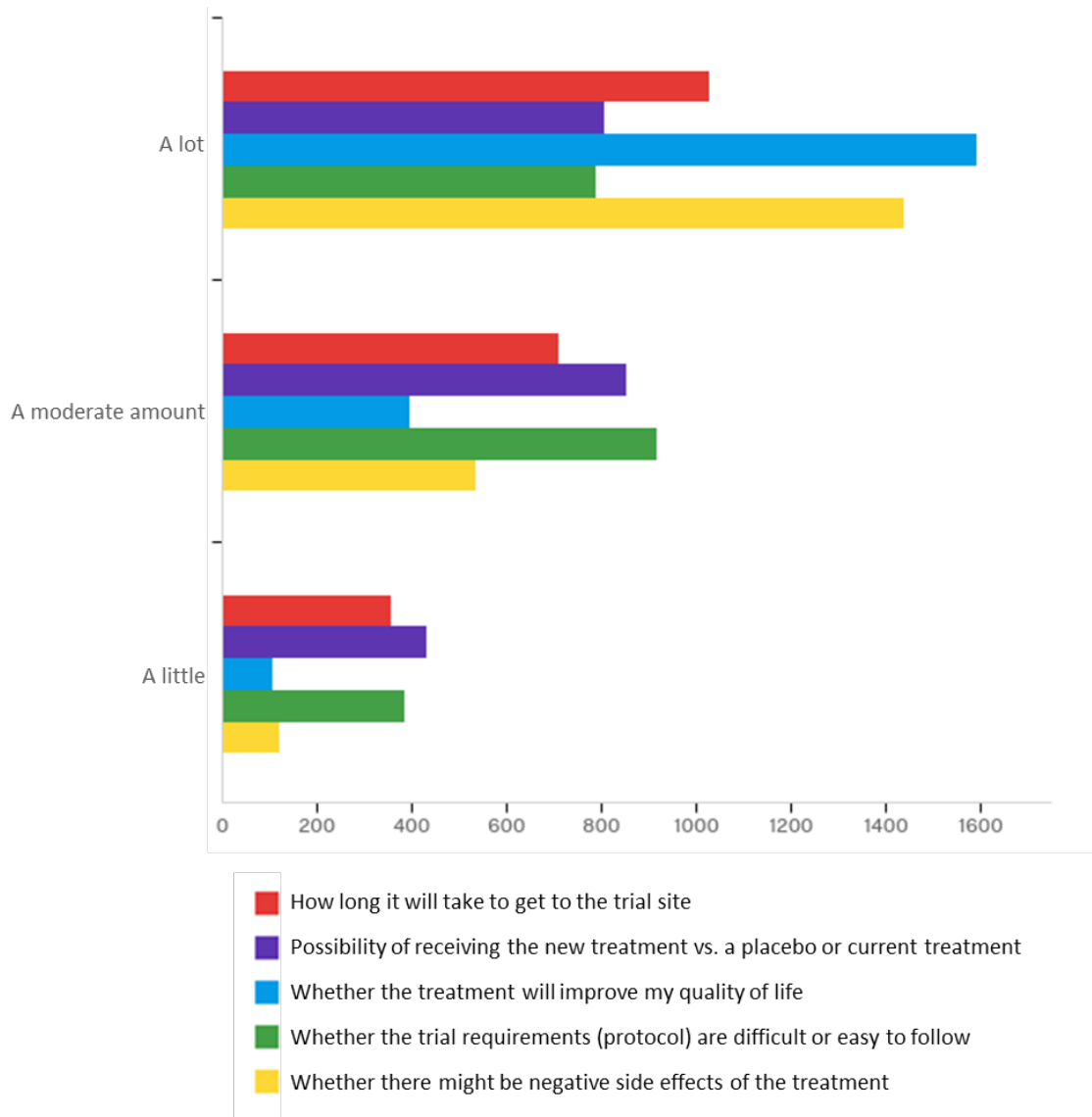
trial indicated that they would join a trial again. Of those who had not participated in a trial, about one in six (16.6%) responded that they had tried but had not been eligible to participate in a trial.

Diagram 13: Types of clinical trials joined by people with lupus (only asked of survey respondents who indicated that they have been in a trial). N = 324.



Most respondents (73.0%) indicated that they would be willing to participate in a trial. The factors that would most impact their decision on whether to participate in a trial include whether the treatment would improve their quality of life and whether there might be negative side effects from the treatment (Diagram 14). The possibility of receiving the new treatment versus placebo or current treatment, whether the trial requirements are difficult or easy to follow, and how long it would take to get into a trial were also noted as important factors.

Diagram 14: How much these factors matter when considering whether to join a clinical trial (select as many as apply). N = 2,095.



Incorporating Patient Input into a Benefit-Risk Framework for Lupus

In 2013, the FDA published a draft implementation plan for a structured approach to benefit-risk assessment in drug regulatory decision making.¹⁹ This framework calls for assessing five decision factors: Analysis of Condition, Current Treatment Options, Benefit, Risk, and Risk Management. When completed for a specific product, it summarizes each decision factor and explains the FDA’s rationale for its regulatory decision. The benefit-risk framework is important for both regulatory and treatment decisions.

The PFDD process is designed to allow the patient voice to help construct a benefit-risk framework for use in the evaluation of new treatments. People living with the disease have a unique perspective on the dimensions that are most important and critical to regulatory decision making, the unmet medical needs of others with their condition, and the benefit-risk tradeoffs that may be acceptable across the continuum of the disease. Their input should be the foundation of therapeutic development, especially in terms of developing treatments that are clinically meaningful and that address aspects of disease that are most critical to people living with the disease. Moreover, by reflecting the perspective of people with lupus, drug developers will be better able to design clinical trials with a high chance of success.

The input provided by people with lupus and their representatives at the Lupus PFDD Meeting, through the pre-meeting survey, and via e-mailed responses following the meeting is summarized here in this sample framework (Table 7) to provide an understanding of the benefit/risk aspects for two of these decision factors: Analysis of Condition and Current Treatment Options. This sample framework is likely to evolve over time and could be incorporated into a benefit-risk assessment framework for a drug under review.

This Externally-led Lupus PFDD Meeting demonstrates that people with lupus are highly engaged and enthusiastic about working with the FDA and drug developers to advance better treatments for this debilitating disease, including repurposing drugs that have been developed and approved for other conditions.

Table 7: Sample benefit-risk framework for lupus

Decision Factor	Evidence and Uncertainties	Conclusions and Reasons
Analysis of Condition	<p>Lupus is a complex, debilitating and poorly understood chronic inflammatory autoimmune disease that may affect any organ system in the body. There are multiple types of lupus and those with different diagnoses may experience different symptoms. Even within each type of lupus, symptoms are highly heterogeneous and sometimes life-threatening.</p> <p>The most common and often debilitating symptoms of lupus include joint and muscle pain, photosensitivity, rashes, fatigue, organ damage, and cognitive</p>	<p>Lupus is a progressive, multi-system, heterogeneous disease that may cause serious disability and loss of function. It can substantially affect an individual’s quality of life and place a large burden on the family. Lupus impacts individuals’ abilities to carry out everyday activities such as household chores, socialize and be outdoors. Disease onset typically coincides with critical years for education and career advancement, which can lead to long-term impacts on individuals’ ability to work.</p>

Decision Factor	Evidence and Uncertainties	Conclusions and Reasons
	<p>difficulties. Approximately 80% of new diagnoses are made in women in their childbearing years. African-Americans, Latinos, and Asian individuals are disproportionately affected.</p> <p>Many of lupus symptoms can be chronic and pervasive and people with the disease often find it difficult to complete activities of daily living as a result of their symptoms. Activities frequently impacted include attending work or school, participating in sports or social activities (particularly those involving direct sunlight), performing household chores, taking care of children or other dependents, and being intimate with a spouse or partner.</p> <p><i>See the Lupus: Patient Voices Report for a more detailed narrative.</i></p>	<p>Even on good days, people with lupus typically experience mild- to moderate-level impacts of the disease on their daily lives. Regardless of lupus diagnosis, individuals experience significant impacts from the disease on their worst days.</p>
<p>Current Treatment Options</p>	<p>Multiple drugs are commonly used to treat people with lupus, but only four have been approved by the FDA and only one was specifically developed for lupus and is not intended for use in all types of lupus. Most of these are non-specific modulators of immunity or inflammation or provide symptomatic relief. Side effects from treatment are frequent and often severe and it can be difficult to distinguish between symptoms that are caused by the disease from those that are drug side effects.</p> <p>Most people with lupus take more than one drug concurrently and the heterogeneous nature of the disease means that it is typical for people with lupus to cycle through multiple drugs throughout their lifetime. Over-the-counter and non-pharmacologic treatments are also frequently used in combination with prescribed drugs.</p>	<p>Drug treatments are urgently needed that more effectively treat lupus symptoms and slow disease progression without causing serious and debilitating side effects. Lupus is considered a disease of unmet medical need because of the lack of efficient diagnostic tools, effective therapies, and well-designed clinical trials.</p> <p>The absence of reliable biomarkers and limitations on clinical outcome measures contribute to clinicians' difficulty in providing optimal patient care and developing new lupus treatments.</p> <p>Lupus therapeutic research should focus on the goals of controlling symptoms, preventing complications, limiting organ damage, increasing survival, improving overall health and</p>

Decision Factor	Evidence and Uncertainties	Conclusions and Reasons
	<p><i>See the Lupus: Patient Voices Report for a more detailed narrative</i></p>	<p>day-to-day functioning for people with the disease.</p> <p>More basic, translation, and clinical research is needed to examine the complexity and heterogeneity of the disease to help better inform trial designs, including inclusion and exclusion criteria, allowing for full implementation of improved enrichment of trial designs that capture these complexities and lead to successes in lupus research and drug development. Designing smaller, more focused studies that examine flare prevention, combination therapies and reducing steroids are also needed.</p>

Conclusion

The Lupus and Allied Diseases Association, the Lupus Foundation of America, and the Lupus Research Alliance convened a meeting for people with lupus and their representatives as part of the FDA's Externally-led Patient-Focused Drug Development (PFDD) Initiative. Through the use of a broadly distributed survey and an in-person meeting and live webcast held on September 25, 2017, they gathered data that reflects the unique perspective of people with lupus on the experience of the disease and the urgent need for better treatments. These data are intended to help the FDA better assess the risks and benefits of drugs under review.

The major themes that emerged from the meeting included the substantial burden of disease in women, particularly among women of color; the considerable variability and heterogeneity of symptoms among people with lupus across the lifespan; the broad impact of the disease and treatment side effects on individual's work, social, and family life, self-esteem, and quality of life; and the inadequacy of currently available treatments.

People with lupus expressed great willingness to participate in clinical trials, and implored the FDA and drug developers to move forward with greater urgency in the development of better treatments for lupus.

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<http://www.fda.gov/downloads/ForIndustry/UserFees/PrescriptionDrugUserFee/UCM329758.pdf>

Appendix 1: Meeting Agenda



Monday, September 25, 2017
10:00 a.m. – 4:15 p.m.
College Park Marriott Hotel & Conference Center
Potomac Ballroom
Hyattsville, Maryland

9:00 – 10:00 a.m.	Registration
10:00 a.m.	Welcome James Valentine, Moderator
10:00 – 10:15 a.m.	Opening Remarks Kathleen A. Arntsen Lupus Community Representative
10:15 – 10:25 a.m.	Overview of FDA’s PFDD Initiative and Road to Clinical Trial Endpoints Pujita Vaidya, MPH Acting Director, Decision Support and Analysis Team, Office of Strategic Programs, Food and Drug Administration Center for Drug Evaluation and Research
10:25 – 10:40 a.m.	FDA Insights on PFDD Initiative and Lupus Janet Woodcock, MD Director, Food and Drug Administration Center for Drug Evaluation and Research Debra Lappin, Facilitator
10:40 – 10:55 a.m.	Overview of Lupus and Available Treatments Anca D. Askanase, MD, MPH Associate Professor of Medicine and Director, Lupus Center, Columbia University Medical Center
10:55 – 11:05 a.m.	Overview of Discussion Format James Valentine, Moderator
11:05 – 11:50 a.m.	Panel on Discussion Topic 1: Patient Perspectives on Disease Symptoms and Daily Impacts that Matter Most Panelists James Valentine, Moderator

11:50 a.m. – 12:50 p.m.	Facilitated Discussion: Topic 1 Persons with lupus and their representatives James Valentine, Moderator
12:50 p.m. – 1:50 p.m.	Lunch
1:50 – 1:55 p.m.	Afternoon Welcome James Valentine, Moderator
1:55 – 2:40 p.m.	Panel on Discussion Topic 2: Patient Perspectives on Current Approaches to Treating Lupus Panelists James Valentine, Moderator
2:40 – 3:40 p.m.	Facilitated Discussion: Topic 2 Persons with lupus and their representatives James Valentine, Moderator
3:40 – 3:55 p.m.	Open Public Comment Period
3:55 – 4:10 p.m.	Summary Remarks Nikolay P. Nikolov, MD Clinical Team Leader, Division of Pulmonary, Allergy, and Rheumatology Products, Food and Drug Administration
4:10 – 4:15 p.m.	Closing Remarks James Valentine, Moderator

Appendix 2: Meeting Speakers

Panel 1

- Tiffany Alsbury, person with lupus
- Elizabeth Beck, person with lupus
- Shanna Garcia, person with lupus
- Rajiv Aaron Manglani, person with lupus
- Chris Reed, person with lupus
- Laura Tanner, person with lupus

Panel 2

- Addy Batiquin, person with lupus
- Lorenzo Hall, person with lupus
- Kevin Johnson, person with lupus
- Elizabeth SantaCruz, parent of a child with lupus
- Diane Thomas, person with lupus
- Donna Wood, person with lupus
- Carlene Harrison, person with lupus (alternate)

Other Speakers

- Kathleen A. Arntsen, Lupus Community Representative
- Anca D. Askanase, MD, MPH, Associate Professor of Medicine and Director, Lupus Center, Columbia University Medical Center
- Debra Lappin, Faegre Baker Daniels Consulting, Facilitator
- Nikolay P. Nikolov, MD, Clinical Team Leader, Division of Pulmonary, Allergy, and Rheumatology Products, Food and Drug Administration
- Pujita Vaidya, MPH, Acting Director, Decision Support and Analysis Team, Office of Strategic Programs, Food and Drug Administration Center for Drug Evaluation and Research
- James Valentine; Hyman, Phelps & McNamara; Moderator
- Janet Woodcock, MD, Director, Food and Drug Administration Center for Drug Evaluation and Research

Appendix 3: Meeting Discussion Questions

Topic 1: Patient perspectives on disease symptoms and daily impacts that matter most

- Of all the symptoms that you experience because of your condition, which 1-3 symptoms have the most significant impact on your life?
- 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?
- How do your symptoms and their negative impacts affect your daily life on the best days? On the worst days?
- How have your condition and its symptoms changed over time?
- What worries you most about your condition?

Topic 2: Patient perspectives on current approaches to treating lupus

- What are you currently doing to help treat your condition or its symptoms?
- How well does your current treatment regimen control your condition?
- What are the most significant downsides to your current treatments, and how do they affect your daily life?
- Assuming there is no complete cure for your condition, what specific things would you look for in an ideal treatment for your condition?
- What factors do you take into account when making decisions about selecting a course of treatment?

Appendix 4: Meeting Polling Questions

The following questions were posted to people with lupus and their representatives who participated in the September 25, 2017 Lupus PFDD Meeting. In-person attendees and those participating via webinar were invited to respond to these questions. The results from these questions were used as a discussion aid for the facilitated discussion sessions.

Demographic/General Questions

1. Where do you live?
 - a. Northeast
 - b. Mid-Atlantic
 - c. Midwest
 - d. South
 - e. Mountain
 - f. West

2. Are you:
 - a. An individual living with lupus
 - b. A parent or legal guardian of a child with lupus
 - c. A representative of an adult with lupus

3. Age
 - a. Younger than 18
 - b. 18-29 years
 - c. 30-39 years
 - d. 40-49 years
 - e. 50-59 years
 - f. 60 or older

4. Do you identify as
 - a. Male
 - b. Female
 - c. Other

5. How long ago were you officially diagnosed with lupus?
 - a. Less than 2 years
 - b. 2-5 years
 - c. 6-10 years
 - d. 11-20 years
 - e. More than 20 years

6. What type of lupus do you have? Check all that apply.
 - a. Cutaneous (skin) lupus erythematosus (subacute)
 - b. Cutaneous lupus erythematosus (discoid lupus)
 - c. Drug-induced lupus erythematosus
 - d. Systemic lupus erythematosus (SLE) without nephritis (kidney disease)
 - e. Systemic lupus erythematosus (SLE) with nephritis

- f. I'm not sure
7. What best describes your lupus?
- a. I have joint and/or skin symptoms
 - b. I have inflammation of other parts of the body apart from joints and skin
 - c. I have inflammation or involvement of organs such as the heart, lungs, brain, or kidneys

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

1. Of all the symptoms you have experienced because of lupus, which do you consider to have the most significant impact on your daily life? Select your top three.
- a. Depression
 - b. Fatigue
 - c. Forgetfulness
 - d. Increased susceptibility to infections
 - e. Joint and muscle pain and/or swelling
 - f. Rashes
 - g. Reduced physical strength
 - h. Renal (kidney) disease or kidney failure
 - i. Organ inflammation (such as pericarditis, peritonitis, or pleurisy)
 - j. Sun sensitivity
 - k. Other symptoms not mentioned
2. Some people find that their lupus makes it hard to do certain activities. Select up to three activities that are most impacted by your lupus.
- a. Attending school or work
 - b. Performing daily hygiene
 - c. Managing personal finances
 - d. Being intimate with a spouse or partner
 - e. Driving a motor vehicle
 - f. Doing outdoor activities involving direct sunlight
 - g. Participating in sports or social activities
 - h. Performing household chores
 - i. Taking care of children or others
 - j. Other activities not mentioned
3. On a scale of 1-5, how much do your lupus symptoms negatively impact your life on the best days? A score of 1 is minimal impact (a few limitations on daily activities) and a score of 5 is high impact (completely unable to complete basic daily activities).
4. On a scale of 1-5, how much do your lupus symptoms negatively impact your life on the worst days? A score of 1 is minimal impact (a few limitations on daily activities) and a score of 5 is high impact (completely unable to complete basic daily activities).
5. Is your lupus currently:
- a. Under very good control (stable, little or no flares)

- b. Under moderate control (some flares, possibly requiring dose changes in current medications)
- c. Not under control (active disease, requires treatment changes, possible hospitalization)

Topic 2: Patient Perspectives on Current Approaches to Treatment

1. Have you ever used any of the following prescription medications to help treat your lupus? Select all that apply.
 - a. Prescription-strength analgesics (Darvon-N/Propoxyphene, Oxycontin/Oxycodone)
 - b. Antimalarials (Aralen/Chloroquine, Plaquenil/Hydroxychloroquine)
 - c. Aspirin
 - d. Biologics (Benlysta/Belimumab, Rituxan/Rituximab)
 - e. Immunosuppressive medications (Cellcept/Mycophenolate Mofetil, Cytoxan/Cyclophosphamide, Imuran/Azathioprine)
 - f. Anti-inflammatory agents or NSAIDs (Celebrex/Celecoxib, Naprosyn/Naproxen)
 - g. Other prescription medications not mentioned
 - h. I haven't used any of these medications
2. Besides your drug therapies, what else are you doing to manage any symptoms you have experienced because of your lupus? Select all that apply.
 - a. Dietary and herbal supplements
 - b. Diet modifications
 - c. Over the counter medications (such as Acetaminophen, Ibuprofen, Naproxen, Aspirin)
 - d. Complementary or alternative therapies
 - e. Other therapies not mentioned
 - f. I am not doing or taking any therapies to treat symptoms
3. When choosing a treatment, what is most important to you? Select one.
 - a. Delaying or preventing symptoms
 - b. Improving symptoms
 - c. Increasing life expectancy
 - d. Slowing down organ damage
 - e. Other
4. What are the biggest downsides of your current treatment? Select your top three.
 - a. Cost of treatment
 - b. How long it takes to get treatments (such as infusions)
 - c. Kidney dialysis
 - d. Number of pills/medications per day
 - e. Side effects
 - f. Travel to hospital or clinic
 - g. Other
5. What factors do you take into account when making decisions about choosing a treatment plan? Select your top three.
 - a. How closely it needs to be checked by blood tests
 - b. How it will impact my ability to get through the work or school day

- c. How much it costs and how much I have to pay
 - d. If it will change my appearance (examples: weight gain, weight loss, acne, hair loss)
 - e. How it will impact my ability to spend time with my friends and family
 - f. If it will put me at risk for cancer in the future
 - g. Likelihood that it will work
 - h. What my doctor thinks
 - i. Another factor not listed
6. Assuming there is no complete cure for your lupus, what would be the most important result that a treatment could provide?
- a. I feel better and can go about my daily life more easily
 - b. I have fewer long-term health problems (like organ damage)
 - c. I have a longer life expectancy
 - d. My overall treatment plan is easier (fewer medications, fewer visits to the hospital or clinic)
 - e. My overall treatment plan costs less

Appendix 5: Pre-Meeting Survey Questions

The following survey questions were distributed to members of the lupus community prior to the Lupus PFDD Meeting. The survey was intended for people with lupus or parents/representatives of children with lupus. The survey was available on a dedicated website (<http://www.lupuspfdd.org>) from May 23, 2017 to August 5, 2017. Email-blasts, social media posts, and posts on other websites were used to raise awareness of the survey. Hard copies of the survey were also distributed at events, support groups, and a camp for children with lupus. The online and paper surveys were available in both English and Spanish.

1.1 Please provide your first and last name: _____

1.2 Please provide a phone number and/or email address

Phone Number _____

Email Address _____

1.3 Please provide your ZIP code: _____

1.4 Are you a person with lupus or a legally authorized representative of a child with lupus?

- Person with lupus
- Legally authorized representative

1.5 If you are interested in attending the September 25, 2017 Lupus PFDD meeting in Hyattsville, MD, would you be willing to do any of the following?

- Speak on a panel
- Ask questions or give comments during open discussion sessions
- I'm interested in attending the meeting, but don't want a speaking role
- I'm just filling out the survey, not interested in attending the meeting

1.6 How did you learn about this survey?

- Lupus organization website
- Doctor or other healthcare provider
- Friend or family member
- Lupus PFDD Meeting website
- Other Internet site or social media
- Flyer or other printed material
- Other

2.1 Please indicate your sex.

- Male
- Female
- Other

2.2 Which best describes your race or ethnicity?

- American Indian or Alaska Native

- Asian
- Black or African American
- Hispanic or Latino
- Native Hawaiian or Other Pacific Islander
- White
- Other

2.3 How old are you?

- Under 18 years
- 18-29 years
- 30-39 years
- 40-49 years
- 50-59 years
- 60 years or older

2.4 How old were you when your lupus symptoms first began?

- Under 18 years
- 18-29 years
- 30-39 years
- 40-49 years
- 50-59 years
- 60 years or older

2.5 How long ago were you officially diagnosed with lupus?

- Less than 2 years
- 2-5 years
- 6-10 years
- 11-20 years
- More than 20 years

2.6 How much time passed between when your lupus symptoms started and when you were officially diagnosed?

- Less than 6 months
- 6-12 months
- 1-2 years
- 3-5 years
- More than 5 years

3.1 What type of lupus diagnosis were you given by your doctor?

- Cutaneous (skin) lupus erythematosus (subacute)
- Cutaneous lupus erythematosus (discoid lupus)
- Drug-induced lupus erythematosus
- Systemic lupus erythematosus (SLE) without nephritis (kidney disease)
- Systemic lupus erythematosus (SLE) with nephritis

3.2 Are you on dialysis?

- Yes
- No

3.3 Have you had a kidney or other organ transplant because of your lupus?

- Yes
- No

3.4 What best describes your lupus?

- I have joint and/or skin symptoms
- I have inflammation of other parts of the body apart from joints and skin
- I have inflammation or involvement of organs such as the heart, lungs, brain, or kidneys

3.5 Rank the 3 lupus symptoms that most negatively impact your life by writing the numbers 1-3 next to those symptoms, with 1 meaning the biggest impact. Please rank only three symptoms.

Symptoms	
_____	Blood clots
_____	Depression
_____	Fatigue
_____	Fevers
_____	Forgetfulness
_____	Stomach or bowel problems
_____	Hair loss
_____	Increased susceptibility to infections
_____	Joint and muscle pain and/or swelling
_____	Lack of concentration
_____	Miscarriages
_____	Mood changes
_____	Mouth sores
_____	Rashes
_____	Reduced physical strength
_____	Renal (kidney) disease or renal failure
_____	Pericarditis (inflammation of the lining of the heart)
_____	Peritonitis (inflammation of the lining of the abdomen)
_____	Pleurisy (inflammation of the lining of the lung)
_____	Seizures
_____	Shortness of breath
_____	Sleep disturbances
_____	Sun sensitivity
_____	Weight gain

3.6 Some people find that their lupus makes it hard to do certain activities. Select how often your lupus stops or makes it hard to do the following activities.

	Daily	Several days a week	Weekly	Monthly	Seasonally	Not applicable
Attending school	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Attending work	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Performing daily hygiene	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Managing personal finances	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Being intimate with a spouse or partner	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Driving a motor vehicle	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Doing outdoor activities involving direct sunlight	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Participating in sports or social activities	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Performing household chores	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Taking care of children or others	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Other	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

3.7 How much time does it take you to get moving or loosen up in the morning?

- One hour or less
- More than one hour

3.8 Does your lupus have a negative impact on your circadian rhythms (disrupted sleep/wake cycles)?

- Yes
- No

3.9 On a scale of 1-5, how much do your lupus symptoms negatively impact your life on the best days? A score of 1 is minimal impact (a few limitations on daily activities) and a score of 5 is high impact (completely unable to complete basic daily activities).

_____ Level of impact

3.10 On a scale of 1-5, how much do your lupus symptoms negatively impact your life on the worst days? A score of 1 is minimal impact (a few limitations on daily activities) and a score of 5 is high impact (completely unable to complete basic daily activities).

_____ Level of impact

3.11 How has your lupus and its symptoms changed over time?

	Better	About the Same	Worse	Not applicable
0-2 years after diagnosis	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3-5 years after diagnosis	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
6-10 years after diagnosis	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
11-20 years after diagnosis	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
More than 20 years after diagnosis	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

3.12 What worries you most about your lupus? List and rank three items (1 being your biggest concern).

Concern 1 _____

Concern 2 _____

Concern 3 _____

3.13 Is your lupus currently:

- Under very good control (stable, little or no flares)
- Under moderate control (some flares, possibly requiring dose changes in current medications)
- Not under control (active disease, requires treatment changes, possible hospitalization)

3.14 What other autoimmune diseases has your doctor diagnosed for you?

- Antiphospholipid Syndrome
- Arthritis
- Celiac Disease
- Crohn's Disease
- Graves' Disease
- Hashimoto's Thyroiditis
- Low white blood cell count
- Myositis
- Psoriasis
- Raynaud's Disease
- Scleroderma

- Sjögren's syndrome
- Type 1 Diabetes
- Ulcerative Colitis
- Uveitis
- Other autoimmune disease (please specify) _____
- None of the above

3.15 What other conditions has your doctor diagnosed for you?

- Depression
- Fibromyalgia
- High blood pressure
- Stomach or bowel issues (such as irritable bowel syndrome (IBS), not including Crohn's Disease or ulcerative colitis)
- Low blood pressure
- Neuropathy (nerve pain or numbness)
- Osteoporosis (low bone density)
- Non-autoimmune thyroid problems
- Type 2 Diabetes (adult onset)
- Other (please specify) _____
- None of the above

4.1 What prescription medications are you currently taking to treat your lupus?

- Analgesics* (prescription strength such as Celebrex/Celecoxib, Darvon-N/Propoxyphene, Dilaudid/Hydromorphone, Duragesic/Fentanyl, Duramorph/Morphine, Percocet/Acetaminophen-Oxycodone, Oxycontin/Oxycodone, Ultram/Tramadol, Vicodin/Acetaminophen-Hydrocodone)
- Antimalarials* (such as Aralen/Chloroquine, Atabrine/Quinacrine, Plaquenil/Hydroxychloroquine)
- Aspirin* (prescription strength such as Easprin/Aspirin, Ecotrin/Aspirin)
- Biologics* (such as Actemra/Tocilizumab, Benlysta/Belimumab, Orencia/Abatacept, Rituxan/Rituximab)
- Corticosteroids* (such as Prednisone/Deltasone, Methylprednisolone/Medrol)
- Immunosuppressive medications* (such as Azasan/Azathioprine, Cellcept/Mycophenolate Mofetil, Cytoxan/Cyclophosphamide, Hecoria/Tacrolimus, Imuran/Azathioprine, Myfortic/Mycophenolic Acid, Neoral/Cyclosporine, Prograf/Tacrolimus, Protopic/Tacrolimus, Rasuvo/Methotrexate, Sandimmune/Cyclosporine, Trexall/Methotrexate)
- Anti-inflammatory agents or NSAIDs* (prescription strength such as Actron/Ketoprofen, Anaprox/Naproxen, Celebrex/Celecoxib, Clinoril/Sulindac, Feldene/Piroxicam, Indocin/Indomethacin, Ketoprofen ER/Ketoprofen, Mobic/Meloxicam, Naprosyn/Naproxen, Orudis/Ketoprofen, Oruvail/Ketoprofen, Relafen/Nabumetone, Vivlodex/Meloxicam, Voltaren/Diclofenac)
- None of the above*

4.2 What over-the-counter (OTC) medications do you currently take to treat your lupus?

- Analgesics (not prescription strength; such as Panadol/Acetaminophen, Tylenol/Acetaminophen)
- Anti-inflammatory agents or NSAIDS (not prescription strength; such as Advil/Ibuprofen, Aleve/Naproxen, Motrin IB/Ibuprofen)
- Aspirin (not prescription strength; such as Anacin, Bayer, Bufferin, Excedrin)
- Dietary supplements
- Vitamins/Minerals
- Herbals (teas, other natural products)
- Other (please specify) _____
- None of the above

4.3 Besides prescription medicines and over-the-counter products, what are you currently doing to help manage your lupus or its symptoms?

- Acupuncture
- Diet modification
- Exercise
- Heat packs
- Ice packs
- Massage therapy
- Meditation
- Tens/nerve stimulation devices
- Other (specify up to three) _____

4.4 Are you also taking medication for any of these other conditions?

- Depression
- Diabetes mellitus
- High blood pressure
- Osteoporosis (low bone density)
- Other cardiovascular (heart or blood vessel) disease
- Other (please specify) _____

4.5 Have you stopped taking any medications due to negative side effects? If so, list along with reason stopped.

- No
- Yes _____

4.6 What treatment has had the most positive impact on your life? Why?

4.7 What are the biggest downsides of your current treatment? Select up to three.

- Cost of treatment
- How long it takes to get treatments (such as infusions)
- Kidney dialysis
- Negative impacts on ability to work or go to school
- Number of pills/medications per day

- Side effects
- Travel to hospital or clinic
- Other _____

4.8 What factors do you take into account when making decisions about choosing a treatment plan? Select up to three.

- How closely it needs to be checked by blood tests
- How it will impact my ability to get through the work or school day
- How it will impact my ability to spend time with my friends and family
- How much it costs and how much I have to pay
- If it will affect my ability to have children
- If it will change my appearance (examples: weight gain, weight loss, acne, hair loss)
- If it will put me at risk for cancer in the future
- Odds that it will work
- What my doctor thinks
- Whether I can take medication at home or need to go to a doctor's office, clinic, or hospital

4.9 When choosing a treatment, what is most important to you?

- Delay or prevent symptoms
- Improve symptoms
- Increase life expectancy
- Slow down organ damage
- Other _____

4.10 How do the following common side effects affect how likely you are to choose a certain treatment?

	Minor (would not prevent me from taking it)	Moderate (would make me carefully consider but might still take drug)	Major (would likely prevent use)	No concern
Bone thinning	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Eye problems (not including cataracts or glaucoma)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Fatigue	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Headache	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Injection and infusion site reactions	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Mood swings	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Stomach and bowel effects (such as constipation, heartburn, nausea, vomiting)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Physical effects (such as skin problems or rashes, hair loss, weight loss or gain)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Other	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

4.11 How do the following less common but serious risks affect how likely you are to choose a certain treatment?

	Minor (would not prevent me from taking it)	Moderate (would make me carefully consider but might still take drug)	Major (would likely prevent use)	No concern
Atherosclerosis (hardening of the arteries)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Birth defects	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Blood disorders	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Cancer	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Cardiovascular (heart) damage (heart attack, irregular heart rhythm)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Cataracts	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Diabetes	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Glaucoma	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Infections	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Kidney damage	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Liver damage	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Osteoporosis (low bone density)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Sterility	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Stroke	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Other	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

4.12 Assuming there is no complete cure for your lupus, what would be the most important result that a treatment could provide?

- I feel better and can go about my daily life more easily
- I have fewer long-term health problems (like organ damage)
- I have a longer life expectancy
- My overall treatment plan is easier (fewer medications, fewer visits to the hospital or clinic)
- My overall treatment plan costs less

5.1 Have you ever participated in a clinical trial?

- Yes
- No

5.2 If you answered Yes to question 5.1, what type of clinical trial(s) have you been in?

- Observational* – observational trials assess health outcomes. Participants continue their regular medical care and are not assigned to specific interventions.
- Interventional* – in an interventional trial, participants receive specific interventions. These interventions may be medical products, such as drugs or devices; procedures; or changes to participants' behavior, such as diet.
- Not sure*

5.3 If you answered Yes to question 5.1, what was the main reason you participated in the clinical trial?

- No other treatment options
- Suggested by my doctor
- Suggested by another person with lupus
- Desire to advance research
- Other

5.4 If you answered Yes to question 5.1, would you consider joining a clinical trial again?

- Yes
- No

5.5 If you answered No to question 5.1, have you ever tried to participate in a clinical trial but have not been eligible?

- Yes
- No

5.6 If you answered No to question 5.1, would you consider joining a clinical trial?

- Yes
- No

5.7 If you had to decide whether to participate in a clinical trial, how much would these things matter to you?

	A lot	A moderate amount	A little
How long it will take to get to the trial site	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Possibility of receiving the new treatment vs. a placebo or a current treatment	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Whether the treatment will improve my quality of life	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Whether the trial requirements (protocol) are difficult or easy to follow	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Whether there might be negative side effects of the treatment	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>