

**WE NEED**

**NEW TREATMENTS**

**NOW**

Introducing  
a New Pioneering  
Research Study

**ABOUT THE COVER:**

Shannon, diagnosed at age 33 with a 30 day supply of more than 20 medications she takes to manage her lupus.

Help Us Solve  
The Cruel Mystery

**LUPUS**<sup>™</sup>

FOUNDATION OF AMERICA





# HOPE IS NOW

## Lupus is the Cruel Mystery

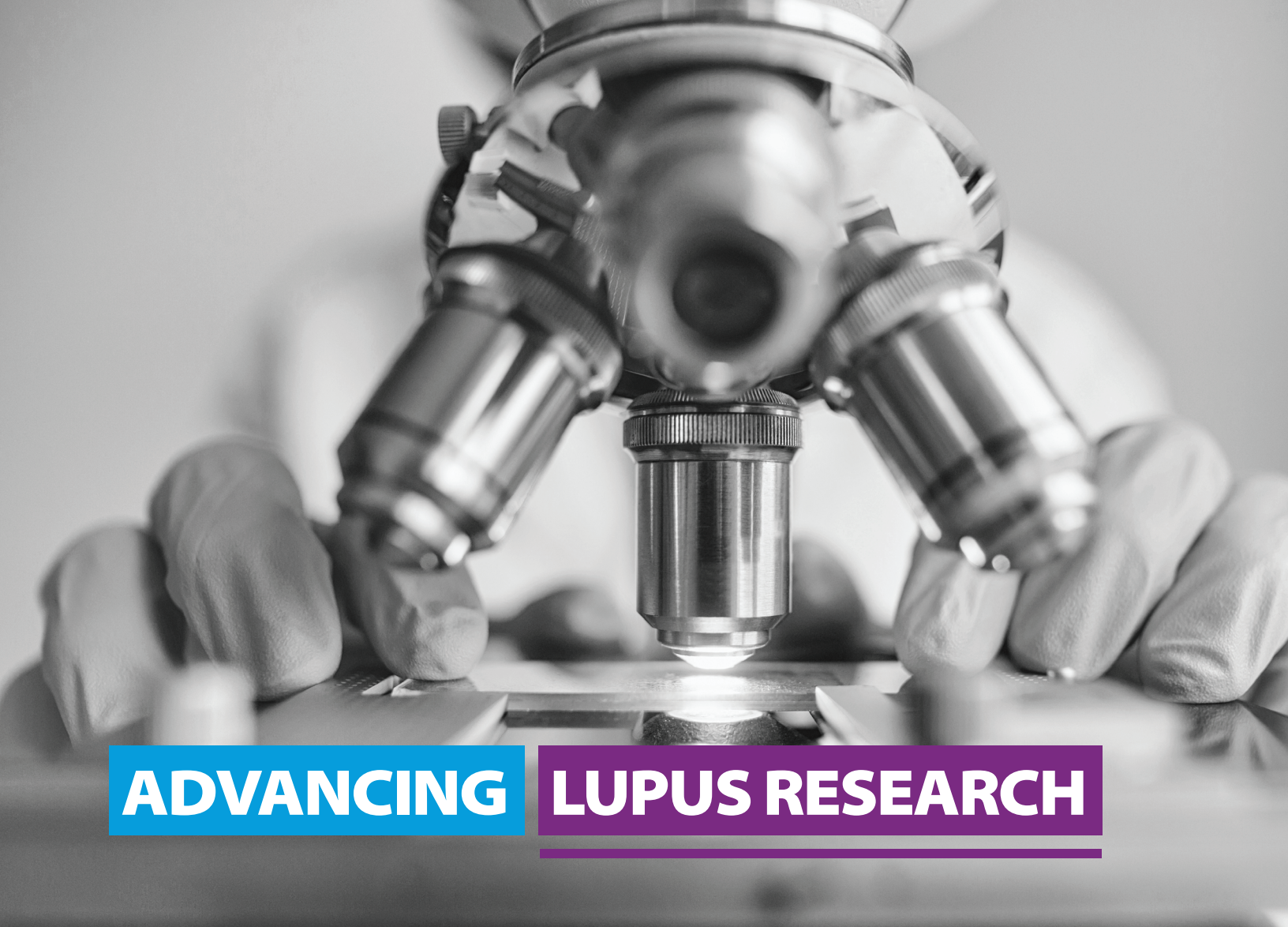
A lupus diagnosis is life-changing. Today, a lupus diagnosis means waiting for new medical advances while managing life-long and potentially fatal symptoms.

Lupus is an unpredictable and misunderstood autoimmune disease that can impact any organ, including the joints, skin, kidneys, brain, heart and lungs. It has no known definitive cause and no known cure. Health effects linked to lupus can range from a skin rash to a heart attack to kidney failure, and even death. The impact of lupus and the medications used to treat the disease on patients are devastating: disability, interruption of careers, infertility and more. Research on lupus also remains woefully underfunded relative to its scope and devastation.

The Lupus Foundation of America's mission is to improve the quality of life for all people affected by lupus. We will continue to attack lupus from every direction until we find the causes and cures for this disease, while providing care, support and answers to all those impacted by lupus.

## About Lupus:

- A recent study led by the University of California, Los Angeles, found that lupus is a leading cause of death in young women ages 15-24.
- Only one medication is currently on the market specifically to treat lupus. It took half a century of research and scientific advances for this drug to be FDA-approved for the treatment of lupus.
- Lupus is two to three times more prevalent among women of color than among Caucasian women.
- People with lupus can experience significant symptoms such as pain, extreme fatigue, hair loss, cognitive issues and other physical impairments that affect every facet of their lives.
- People with lupus surveyed take an average of eight medications to manage all of their symptoms and related conditions.
- The annual total cost per lupus patient can be as high as \$50K due to health care costs and lost productivity.



## **ADVANCING LUPUS RESEARCH**

### **Our goal is to fund research that delivers results in the shortest time possible.**

Research is the key to solving the cruel mystery of lupus. There is only one medication specifically to treat lupus. Side effects of current treatments can be debilitating and are often severe. They include increased risks of infection, diabetes, cancer and infertility, bone loss and osteoporosis.

Developing therapies that can fight lupus is a challenge. Every person with lupus is unique and no two cases of lupus are alike. But we at the Lupus Foundation of America are committed to finding answers and ending the brutal impact of this disease. That's why we focus our support on research that delivers the greatest impact on peoples' lives in the shortest time possible. And we are making progress.

The Lupus Foundation of America is funding the research of Dr. Gary Gilkeson and colleagues who are evaluating mesenchymal stem cells as a potentially promising new therapy for lupus. This study has the potential to help people with some of the most severe cases of lupus, and for whom current medications are not working. Our hope is that this research could be a powerful success story in the long, difficult journey towards better treatments for all people with lupus. Together, we can provide hope for the future for millions of people with lupus.

**We are continuing our commitment to improve and transform the lives of those with lupus by raising \$4 million to support an innovative phase II trial to evaluate mesenchymal stem cells as a potential treatment for lupus.**



## Stem Cell Research: Providing Hope And Improving Lives

### Trailblazer for Stem Cell Research

The Lupus Foundation of America has long been a leader in funding pioneering basic and clinical adult stem cell research. We were among the earliest supporters and first organizations to see the potential of stem cell therapy in people with lupus. Over the past 10 years we have invested and provided funding to researchers to explore this important area.

### An Innovative Step Forward in Lupus Treatment

The Lupus Foundation of America has been supporting Dr. Gary Gilkeson and Dr. Diane Kamen, specialists in rheumatology and immunology at the Medical University of South Carolina (MUSC), in their mesenchymal stem cell (MSC) research since 2011. In the spring of 2018, the National Institute of Allergy and Infectious Diseases (NIAID), part of the National Institutes of Health (NIH), joined with the Foundation to co-fund the phase II multi-center study. This study will help researchers determine whether the stem cell therapy is effective, and which people could possibly benefit from this potential treatment.

## What You Need to Know

- MSCs have immunosuppressive qualities that scientists do not fully understand. Some researchers predict that, in people with lupus, MSCs may help turn down an overactive immune system.
- MSCs do not carry the same ethical concerns that embryonic stem cells may have because they are derived from umbilical cord blood or adult bone marrow.
- MSC therapy holds the promise of hope for people with lupus, especially those who are not benefitting from their current medications.
- Results from an early study in China show that a majority of study participants may have benefited from treatment. While the Chinese study was not a randomized, placebo-controlled trial, 65-70% of participants achieved an initial partial response, a much higher response rate than current treatments for lupus. Adverse reactions were also rare.

## About the Trial

- 81 individuals with lupus who are not responding well to current treatments are eligible to enter the full trial.
- In addition to MUSC, which will serve as the coordinating center, six additional academic centers will participate including Emory University (Atlanta); University of North Carolina at Chapel Hill; Northwestern University (Chicago); Cedars Sinai Medical Center (Los Angeles); University of Rochester (Rochester); and University of California (San Diego).
- This study is unique because unlike other adult stem cell protocols, this therapy does **NOT** require an individual's immune system to be suppressed.
- The study is a randomized, placebo-controlled clinical trial. One third of the participants in the control group will receive a placebo infusion and the other two-thirds will receive MSCs from the umbilical cord of an unrelated donor.

## MiSLE Study Timeline

### 2012

Received a **\$100,000 grant from LFA** for a study of MSCs in lupus through the LFA Peer-Review Research Program.

### 2014

Data gained from the LFA funded study laid the groundwork for a **\$255,067** planning grant from NIH to proceed with the **first-ever** multi-center double blind placebo controlled trial of MSC in lupus.

“ This study will help us better understand how mesenchymal stem cells work in lupus. We will also evaluate whether MSC therapy can **decrease lupus disease activity, reduce the need for medications** like steroids, which can have harmful side effects, and **prevent further damage to vital organs.**

– **GARY GILKESON, MD, Professor of Medicine and Associate Dean for Faculty Affairs and Faculty Development with the Medical University of South Carolina**

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## POTENTIAL FOR IMPACT

This research has the potential to generate progress toward finding a new and improved treatment for lupus. While the results from initial studies in China appear promising, this stem cell therapy must be shown through controlled clinical trials to be both safe for people with lupus and more effective than the standard of care treatment alone. Our hope is that stem cell therapy will also show promise for lupus, and ultimately be proven to be a safe and effective treatment for people with lupus

**2015**

Submitted an Investigational New Drug Application (IND) to the FDA for a Phase II clinical trial.

**2016**

Received funds from LFA in order to begin **harvesting cell lines.**

**2017**

Received funds from LFA to begin enrolling patients in phase I study.  
**First patient enrolled** in May 2017.  
LFA commits **\$4M over 5 years** to completion of the Phase II study.

**2018**

**NIAID**, part of the National Institutes of Health, commits to **co-fund the Phase II study.**

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The symptoms pile on top of each other. Each one needs a different medication to treat it. I have taken every medication and treatment available— **and none of them have worked for me.** My story is not unique. This study gives me hope. People’s lives are depending on better treatments.

— **BARBI**

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The reason this trial is happening now is because of the vision of Dr. Gilkeson and his team and the support and resources of the Lupus Foundation of America and the National Institutes of Health. We owe it to our patients and the next generation to continue to fight for more research funding, and better, safer treatment options. **Now is not the time to look around to see who else is going to do it.**

– **S. SAM LIM, MD, Emory University**



**YOU CAN MAKE  
A DIFFERENCE  
NOW!**

We are rallying support for this innovative research so it will get the attention and funding needed to move forward.

**Our promise is to continue supporting Dr. Gilkeson's and Dr. Kamen's research by contributing \$4 million to their stem cell trial over the next five years.**

But we can only support this cutting-edge research with your help! The funding needed for a study of this scope is significant in order to cover everything from the necessary laboratory equipment to the development of the stem cell lines, to the care of participants enrolled in the trial. Together, we can provide hope and change the future for millions of people with lupus NOW.

If you would like more information on how you can support this important research study, please contact:

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