



## MEMORANDUM

**To:** Dr. Francis Collins, NIH Transition Team

**From:** Sandra C. Raymond, Lupus Foundation of America, CEO

**Re:** NIH: A Legacy of Underfunding for Lupus Research and its Effects on Women and Health Disparities

**Date:** December 29, 2008

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**President-Elect Obama and the NIH Transition Team have the opportunity to address the devastating health consequences of lupus, the prototypical autoimmune disease that disproportionately affects women, especially women of color.** Over the past ten (10) years, research has produced tantalizing clues about the pathogenesis and pathophysiology of lupus. We now know more about the functions of the immune system but we must continue to build upon these findings. In recent years, the federal investment in medical research has not grown and there has been a serious erosion of the lupus research effort. Overall, underfunding of the NIH has changed the way in which laboratories operate, universities hire and researchers work.

Lupus is a complex disease whose mysteries will only be unlocked by a robust medical research effort. **It is shocking to report that it has been fifty (50) years since the Food and Drug Administration (FDA) last approved a new drug specifically to treat this chronic, life-altering, disabling and, too often, fatal autoimmune disease.** Indeed, many drug development companies consider research and development for lupus treatments to be too high-risk financially to be worthwhile economically. Compounding this challenge is the fact that lupus incidence and prevalence are too great for those affected by the disease to benefit from the industry research incentives contained in the “Orphan Drug Act.”

Research on lupus cuts across many, if not all of the Institutes of the National Institutes of Health (NIH); however, the lead Institute for lupus research is the National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS). A robust biomedical research program, that includes basic, clinical epidemiological and translational research, has great potential to not only directly benefit those who suffer from the disease but may well provide beneficial information about other autoimmune diseases affecting more than 23 million Americans.

Lupus – a prototypical autoimmune disease – stands apart from other chronic diseases in that, not only is there no cure, but most of the therapies currently used: (1) have not been approved by the FDA; (2) have side-effects profiles that often prove worse than the disease itself; and (3) are untenable for long-term use. The lack of specific therapies – caused, principally, because of insufficient public and private investment in research – plays a significant role in the terrible health disparities seen among those who are impacted by this complex and unforgiving disease.

The health disparities seen in lupus are astonishing and unparalleled:



- Ninety percent of people with the disease are young women in their child-bearing years, although men and children also are affected;<sup>1</sup>
- The disease is two to three times more common among African Americans, Hispanic/Latino-Americans, Asian-Americans, and Native Americans, than Caucasians – a health disparity that remains unexplained;<sup>2</sup>
- African-American women with lupus are impacted at an earlier age, experience greater disease severity, have the highest overall death rate among all people with lupus, and are three times more likely to die from the disease than Caucasian women;<sup>3</sup>
- Eighty percent of new cases are diagnosed in women of child-bearing age – striking people in their prime;<sup>4</sup>
- More than 36% of lupus deaths occur in people between the ages of 15 and 44;<sup>5</sup> and
- Women die from lupus at five times the rate of men with the disease.<sup>6</sup>

There is no other disease that has such an adverse impact on women and people of color, for which there are so few safe and effective treatment options. As lupus is a prototypical autoimmune disease, research breakthroughs in lupus also likely will benefit the more than 23 million Americans living with autoimmune diseases.<sup>7</sup>

### Background/Overview

What makes lupus a particularly cruel and unique disease is that it affects virtually every organ and major system in the body and can cause severe and permanent damage to the skin, joints, heart, lungs, blood, kidneys, and brain – resulting in significant disability or death.

- **Lupus annually costs the nation an estimated \$31.4 billion in direct and indirect expenditures.** A recent study found that the average annual cost per person with lupus (between the ages of 18 and 65) was \$20,924.<sup>8</sup> However, each year, the National Institutes of Health (NIH) only invests \$56 per person with the disease.<sup>9</sup> Given the significant negative effects of lupus among women, people of color, women of child-bearing age, and children, the federal investment in lupus must become proportional to its impact on individuals, communities, and the nation.

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<sup>1</sup> The Epidemiology of Systemic Lupus Erythematosus *Dubois' Lupus Erythematosus*, Bevra H. Hahn, Daniel J. Wallace, M.D., 1997.

<sup>2</sup> National Institute of Arthritis and Musculoskeletal and Skin Diseases, Strategic Plan for Reducing Health Disparities, November 2006.

<sup>3</sup> Centers for Disease Control and Prevention (CDC), Trends in Deaths from Systemic Lupus Erythematosus—United States, 1979—1998.

<sup>4</sup> *The Lupus Book: A guide for Patients and Their Families*, Daniel J. Wallace, M.D., 1995.

<sup>5</sup> CDC, Trends in Deaths from Systemic Lupus Erythematosus—United States, 1979—1998.

<sup>6</sup> Ibid.

<sup>7</sup> Progress in Autoimmune Diseases Research, National Institutes of Health Autoimmune Diseases Coordinating Committee, Report to Congress, March 2005.

<sup>8</sup> Healthcare Costs And Costs Associated With Changes In Work Productivity Among Persons With Systemic Lupus Erythematosus, Pantelis Panopalis, et. al., October, 2008.

<sup>9</sup> NIH reports it spent \$84 million in FY 2008 on lupus research. Estimate of Funding for Various Diseases, Conditions, Research Areas, National Institutes of Health Website, February 2008. <http://www.nih.gov/news/fundingresearchareas.htm>



- **An accurate diagnosis of lupus can take as long as four or more years and visits to three or more physicians.** Much of this delay in diagnosis is due to inadequate health professional awareness, education, and training. Late diagnosis and delayed treatment contribute to poor outcomes and increased morbidity and mortality. As such, improved knowledge of lupus among health professionals will result in earlier and more accurate diagnoses; this, in turn, will improve disease management, diminish adverse effects, and increase quality-of-life among those affected.<sup>10</sup>
- **The last drug specifically to treat lupus was approved by the FDA during the Eisenhower Administration.** Currently, there are only three approved medications to treat lupus – hydroxychloroquine, prednisone, and aspirin. While these drugs do provide benefit to people with lupus, hydroxychloroquine and prednisone can have significant side effects such as eye/vision changes, Type 2 diabetes, increased risk of infections, bone loss and osteoporosis, and other adverse health effects.

Due to the absence of new lupus treatments in the last 50 years, physicians have had no other choice but to turn to drugs approved for other indications to provide some relief for their patients. Based on clinical data, some of these off-label drugs have been found to be effective in treating lupus symptoms, and they, along with the “old” approved therapies, have been considered by treating physicians as the standard of care for the treatment of lupus. Unfortunately, these therapies can cause severe complications because they suppress the immune system, which can help bring symptoms under control, but put people at risk for a variety of infections and other diseases, including cancer. Some of the off-label treatments also are known to be teratogenic and over longer periods of use, often compromise fertility by causing premature menopause or ovarian failure. Too often, women of child-bearing age with lupus – a majority of those affected by the disease – have to choose between managing their disease and having children: A choice no young woman should have to make.

- **Access to affordable health care must be ensured for people with lupus.** Affected individuals must have affordable, unencumbered access to existing therapies that their health care providers determine are necessary and appropriate. Many health plans often do not cover these necessary off-label medications for the treatment of lupus, or, if they do, these therapies are categorized in a higher tier, placing them financially out-of-reach for the majority of people with lupus. Too many people with lupus are denied coverage and/or adequate reimbursement for much-needed treatments, forcing them to either face significant and, very frequently, untenable out-of-pocket costs, or forego treatment altogether. Adequate and affordable coverage and reimbursement for lupus treatment, available today and in the future, will help enhance quality-of-life and improve outcomes for people living with the disease.

To increase quality-of-life, productivity, health, and well-being among people with lupus, and to reduce the private and public sector economic costs, the Lupus Foundation of America (LFA) maintains that greater federal and private sector resources must be allocated to:

- Basic, stem-cell, biomarker, clinical, epidemiological, translational, and behavioral research;
- Lupus awareness and education efforts; and
- Initiatives to facilitate public-private partnerships which advance research on lupus.

This investment will help ensure that the disease is better understood and that another 50 years will not pass without safe, tolerable, and effective lupus-specific drugs becoming available to the women, men, and

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<sup>10</sup> Lupus Foundation of America, *Survey of People with Lupus Reveals the Disease Remains Difficult to Diagnose and Manage*, Summer 2000.



children who need them. Adequate coverage of, reimbursement for, and access to all lupus treatments are essential. Moreover, without sufficient coverage, payment, and access policies for lupus treatment, new breakthroughs will be meaningless for people living with this and other chronic, life-threatening diseases.

### **Opportunities for Change**

While lupus poses significant challenges to biomedical researchers, rheumatologists, and other health professionals, there are a number of policies and programs that, if implemented today, would have immediate, positive results for the people lupus affects and, likely, would have application to the 23 million other Americans with autoimmune-related conditions. As Centers for Disease Control and Prevention (CDC) Medical Epidemiologist, Dr. Chad Helmick, asserts, “Understanding lupus is important for understanding other autoimmune diseases, and understanding why there are such disparities by race and sex in lupus.” According to Dr. Daniel J. Wallace, Clinical Professor of Medicine at UCLA, gains in the understanding of lupus have “helped not only [lupus] patients, but also those with immune-related disorders, such as allergies, cancer, HIV, and other infectious processes.”<sup>11</sup>

Through President-Elect Obama’s leadership and support, our nation, finally, could make progress in addressing a disease that, for too long, **has been under-served by the federal government to the detriment of the women, people of color, and children living with lupus.**

To that end, we provide the following information to you, with the hope that President-Elect Obama, upon taking office in January, would consider taking executive action in the areas in which he has authority and would, otherwise, urge Congress to deliver to him legislation that seeks to address the other issues outlined below. We respectfully encourage President-Elect Obama and the NIH Transition Team to:

- (1) **For the next three fiscal years (FY 2010, 2011, and 2012), submit a budget to Congress that increases the federal investment in lupus in the following ways:**
  - **Doubles the overall annual amount of NIH funding specifically allocated to lupus** at the various institutes and centers involved in lupus research, including the National Center on Minority Health and Health Disparities. For each of the next three years, new funding should be allocated in the following ways to expand: studies in health disparities (\$5 million); basic and stem-cell research (\$5 million); clinical and translational research (\$10 million); behavioral research (\$2 million); biomarker identification and validation (\$3 million); studies in genetics and the environment (\$5 million); and questions that have a direct relationship to unraveling the complex nature of the disease.<sup>12</sup>
  - **Increases funding for the National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS).** The mission and work of NIAMS touch every American family, yet it historically has been woefully underfunded in relation to the incidence and prevalence of the diseases that fall under its scope. While increasing overall NIH funding is essential, during the last doubling effort, NIAMS did not receive its proportional share – this legacy of under-funding has

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<sup>11</sup> *The Lupus Book: A guide for Patients and Their Families*, Daniel J. Wallace, M.D., 1995.

<sup>12</sup> Estimates of Funding for Various Diseases, Conditions, Research Areas, National Institutes of Health Website, February 2008. <http://www.nih.gov/news/fundingresearchareas.htm>



severely impacted the agency's ability to fulfill its mission and hinders much-needed progress in lupus and other autoimmune conditions.

We very much appreciate your attention to our concerns and thank you for considering our recommendations. Please know we stand ready to work with the NIH Transition Team and other policymakers to ensure that we reduce and prevent suffering from lupus and other autoimmune diseases. If you have any questions regarding the information or policy proposals contained in this memo, or if we can be of any assistance to you, please do not hesitate to contact Julie Venners, Senior Director of Government Relations at [venners@lupus.org](mailto:venners@lupus.org).