Lupus Federal Working Group
October 22, 2003

Summary Minutes

Dr. Stephen Katz, National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS) Director, greeted the attendees who were representing NIH institutes and centers, other Federal agencies, voluntary and professional organizations, and industry. Because of its mission, NIAMS, along with other NIH institutes and centers (ICs), has had a long-term interest in autoimmunity. He noted that much is happening in research on lupus and autoimmunity at NIAMS and other components of the NIH, and referred as an example to the Autoimmune Diseases Coordinating Committee Research Plan.

Dr. Katz said that he sees the role of the Lupus Federal Working Group (LFWG) as similar to that of the Federal Working Group on Bone Diseases, which brings together the key players and facilitates communication and coordination. The LFWG, he believes, will be successful because its members will use the committee for mutual learning and sharing. He explained that the current meeting is designed to allow the various organizations to learn about lupus research and related activities in intramural and extramural programs.

Dr. Susana Serrate-Sztein, Director, NIAMS Rheumatic Diseases Branch, spoke of the LFWG as a common starting point for gathering together interested NIH ICs, other Federal agencies, voluntary and professional organizations, and industry to discuss intramural and extramural activities and to stimulate and facilitate cooperation and collaboration in the early stages of planning. Dr. Sztein asked the members to play an active role in making the LFWG an effective tool for the lupus community by suggesting agenda items and identifying organizations that should be represented. Dr. Sztein said that she would like to concentrate on a specific issue at the next LFWG meeting and encouraged the participants to suggest agenda items. She also asked everyone to email items of interest they would like to share with the membership.

Dr. Sztein then invited the representatives around the table to give a brief description of their organizations' lupus-related activities.

Dr. Peter Lipsky, NIAMS Scientific Director, discussed the need to develop and validate biomarkers for lupus. He spoke about the NIAMS intramural program biomarker initiative and interest in biological agent interventions. He referred to the Food and Drug Administration (FDA) Arthritis Advisory Committee's draft guidance document for developing new lupus drugs. Dr. Lipsky noted the lack of new drug development for lupus in recent years. The next meeting of the FDA's Arthritis Advisory Committee will be held in 2004.

Dr. Barbara Mittleman, NIAMS/Intramural Research Program (IRP), spoke about the potential for collaboration between the intramural and extramural programs. She

reiterated the need for lupus biomarkers and commented on how intramural staff and resources are uniquely suited to move this science along. She stated the need for more interdisciplinary activity in lupus research and asked how the parties should talk to each other both organizationally and scientifically. Dr. Mittleman distributed a handout from an October 14, 2003, meeting that was convened by NIAMS/IRP to discuss the development and validation of biomarkers. The meeting followed a meeting in April sponsored by the FDA and lupus voluntary organizations.

Dr. Lee Simon, Center for Drug Evaluation and Research, FDA, reported on the lupus guidance document developed by the Arthritis Advisory Committee to gain insight into biomarkers, and spoke about the necessity for stimulating the field. He also reported that FDA is aggressively pursuing outcome measures for drugs used to treat lupus. In addition, he informed the attendees about a new FDA office focused on safety and considerations of risk to benefit. Dr. Simon noted that FDA is a regulatory agency and does not conduct research.

Dr. Jeff Siegel, Center for Drug Evaluation and Research, FDA, discussed the review of bio-agents and drugs under development, including monoclonal antibodies for autoimmune diseases. He also talked about clinical trial design, safety, and efficacy, and mentioned the FDA's interest in encouraging pharmaceutical companies to pursue new products for lupus. He spoke about the importance of the guidance document and requirements being developed. Dr Siegel also discussed FDA data from failed trials. He explained that the data are proprietary, but the FDA has been successful in negotiating with pharmaceutical companies to allow data to be reanalyzed and he offered this as a possibility on a case-by-case basis.

Dr. Liana Harvath, National Heart Lung and Blood Institute (NHLBI), explained that NHLBI has no intramural research in lupus. She reported on 13 grants related to lupus and coronary heart disease, thrombosis, immune activities of coagulation, and patterns of inflammation. Dr. Harvath told the attendees that her Institute is interested in collaborative interactions.

Ms. Barbara Boyts, Alliance for Lupus Research (ALR), spoke about the Alliance's grant portfolio and interest in supporting investigators looking for new treatment approaches, scientific gaps, genetic links to lupus, pregnancy loss, and CD20 therapies. Ms. Boyts described the ALR as a catalyst for moving research to clinical trials. She referred the attendees to a lupus study on prevention of atherosclerosis progression in adults with lupus. She also noted that the ALR Scientific Board, chaired by Dr. Joseph Craft, is interested in identifying scientific gaps and in partnering with other organizations, particularly patient groups.

Dr. Gladys Hirschman, National Institute of Diabetes and Digestive and Kidney Diseases, (NIDDK), spoke about NIDDK's 21 grants (R01s) in lupus nephritis. She described a prednisone and cyclophosphamide randomized clinical trial examining the inflammatory component of lupus, nephrogenic autoimmunity, and mechanisms of immune-mediated injury.

Ms. Margaret Dowd, Lupus Research Institute (LRI), explained the genesis of LRI, which is sponsored by patients and families and focuses on encouraging new scientific ideas and young investigators by funding good hypotheses with little preliminary data. Although concerned about duplication of effort, she said that every effort is needed. She ended by saying that the LRI is interested in coordination with other organizations.

Dr. Jane Salmon, representing the American College of Rheumatology (ACR), described the ACR as an important source of information for rheumatologists. She described the Research and Education Board, career development awards, and research on mechanisms of disease and clinical research. Dr. Salmon spoke about the organization's interest in the development of organ-specific outcome instruments and global outcome instruments.

Dr. John Klippel, President and Chief Executive Officer of the Arthritis Foundation (AF), explained that his organization has 50 Chapters, and he mentioned research, advocacy, and public health policy as their main areas of activity. He referred to co-funding research with many organizations represented around the table. Dr. Klippel noted that the AF has focused a large part of its efforts on autoimmunity in young people. He also emphasized the need to explore commonalities and associations with other autoimmune diseases, and to understand the prevention of autoimmune diseases better. He commented on the need to understand heart disease and lupus and remarked about AF reaching out to organizations, such as the American Heart Association, to improve understanding of heart disease and lupus. Dr. Klippel described the National Arthritis Action Plan, which looks at lupus from the public health perspective. He also spoke about the AF's role in supporting the Childhood Arthritis and Related Diseases Research Agenda (CARRA).

Ms. Sandra Raymond, Lupus Foundation of America (LFA), spoke about the Foundation's 341 grants to 81 institutions, its collaborations with other patient organizations and with NIAMS, and its role in professional education. She talked about the importance of cooperation and the need for one voice among groups working for lupus treatment and cure. She asked about the possibility of a lupus initiative similar to the NIAMS Osteoarthritis Initiative. Ms. Raymond provided information on a Lupus World Conference, May 9-13, 2004, under the auspices of the Office of Women's Health, Deprtment of Health and Human Sciences (DHHS), that will focus on international activities. Speakers include experts from many countries, the World Health Organization and the PanAmerican Health Organization. She also mentioned LFA's new quarterly magazine "Lupus Now."

Dr. Glinda Cooper, National Institute of Environmental Health Sciences (NIEHS), discussed the importance of the environment in fully understanding the role of genetics in lupus. She referred to Dr. Fred Miller's sibling study in lupus. NIEHS, which focuses more on toxicology than epidemiology research, plans to stimulate more epidemiology research in collaboration with a Canadian network of rheumatologists. Dr. Cooper also mentioned increased activity related to lupus in the NIEHS intramural research program.

Dr. Rosaly Correa-de-Araujo, Agency for Healthcare Research and Quality, told the attendees about her agency's report on the quality of health care. She also described evidence-based Practice Center reports and the evaluation of interventions. She said that although lupus is not the specific focus of these activities, they are relevant because they address the health of women and minorities. She pointed out that collaborations would be helpful in the evaluation of health disparities and patient safety.

Dr. Ellen Goldmuntz, National Institute of Allergy and Infectious Diseases (NIAID), described the Institute's extramural research support in pediatric rheumatology and its interest in immunology and in genetics in general, as well as in lupus specifically. She explained that NIAID supports research from basic science to clinical trials and discussed grant and training grant support in immunology. She mentioned the Immune Tolerance Network, an international network that includes rheumatoid arthritis and lupus, and the multiple autoimmune diseases genetics research registry for families in which two or more members have an autoimmune disease. NIAID also chairs the NIH Autoimmune Diseases Coordinating Committee that promotes trans-NIH collaborations.

Dr. Kenneth Schwartz, Genelabs Technologies, Inc. (by conference phone), discussed the 1994 study of hormonal aspects of lupus, and he also spoke about Genelab's Phase III study on bone loss due to glucocorticoid treatment.

Dr. Lisa Begg, NIH Office of Research in Women's Health (ORWH), mentioned the long-standing interest of ORWH and of its Director, Dr. Vivian Pinn, in lupus research. She referred to their lead role along with NIAMS and DHHS, in the September 2003 meeting, "Lupus Today: Research into Action."

Dr. Charles Helmick, Centers for Disease Control and Prevention (CDC), discussed population-based research in osteoarthritis, rheumatoid arthritis, and lupus. He spoke about mortality data from the population-based lupus registry at two sites (Michigan and Georgia) and surveillance on appropriate disease management and treatment, including both primary care patients and those seeing rheumatologists. He reported on the increase in lupus mortality in middle-aged black women and commented on the challenge of identifying lupus. Dr. Helmick was enthusiastic about the idea of a collaboratively developed registry of diagnosed lupus patients found in multiple settings (hospitals, private practice, etc.), that would include natural history, disease factors, progression of disease, and misdiagnosis and over-diagnosis.

Ms. Frances Ashe-Goins, Office of Women's Health (OWH), DHHS (by conference phone), described the collaboration between OWH-DHHS, ORWH-NIH, and NIAMS collaboration on the conference, "Lupus Today: Research into Action" on September 5-6, 2003. She spoke about the OWH-DHHS commitment to lupus awareness and the community health centers concerned with health disparities that serve the poor.

Dr. William Robinson, Health Resources and Services Administration (HRSA), explained his agency's national network of community health centers, serving about 11 million people and the role they play in providing primary health care services to low-

income patients and families in urban, underserved, and remote areas. He then talked about HRSA's community-based patient education and outreach initiative on lupus awareness.

Dr. Ellen McCroskery, Alexion Pharmaceuticals, Inc., reported that she is an interested observer. Her organization has no trials in lupus at this time.

Dr. Ursula Utz, National Institute of Neurological Diseases and Stroke (NINDS), was not able to attend the meeting but sent in information on three relevant studies. They are "Protein Methylation in the Brain," Midcareer Investigator Award in Patient-Oriented Research: "Neuropsychiatric Lupus," and "Stroke: Role of aPL and Hemostatic Markers."

There was a brief discussion about future activities, including the need for better coordination of lupus meetings, evolving interest in development of biomarkers for lupus, and the need to coordinate patient awareness and education activities.

Dr. Katz then closed the meeting by thanking all the participants for openly sharing information, and he expressed his enthusiasm for continued coordination and collaborations.