



Overcoming Barriers to Drug Development in Lupus

Summary of the report prepared by The Lewin Group

Introduction

The Lupus Foundation of America, Inc. (LFA) commissioned The Lewin Group (Lewin) to prepare a report on the multi-dimensional challenges related to the development and approval of new lupus therapies and to develop recommendations that address the unmet needs of individuals with lupus.

Barriers to Lupus Drug Development

The key barriers to lupus drug development include the following:

- The diversity and impact of symptoms and the unique biology of the disease
- Recruiting appropriate clinical trial participants.
- Selecting the appropriate clinical trial endpoints.
- Adapting currently available instruments and tools for measuring disease activity for use in clinical trials.
- The impact of strong background medications on clinical trial results.

Recommendations

The following recommendations reflect deliberations of more than 40 participants from industry, government, and academia who served on the expert panel convened in Washington, DC, on June 1-2, 2009, and the series of expert interviews conducted prior to that event.

Recommendation 1: A new coordinated national effort should be organized to overcome the barriers to drug development and approval in lupus, with input from representatives from federal agencies, academic institutions, the lupus scientific community, and national advocacy and health professional organizations.

Recommendation 2: The federal biomedical research effort should be greatly expanded to develop a better understanding of the biological mechanisms of lupus, including more basic and translational research on the pathophysiology and pathogenesis of the disease.

Recommendation 3: The scientific community and the National Institutes of Health should collaborate on a research agenda to provide a clear pathway to drug development in lupus. The scientific community, the LFA, and its partners should coordinate a series of workshops and related expert meetings to address the design of clinical

Recommendation 4: The NIH should establish and fund a consortium to expedite the identification and validation of biomarkers for lupus.

Recommendation 5: The scientific community and pharmaceutical and biotechnology industries should establish a technical expert panel to assess the two predominant instruments currently used in lupus clinical trials: the BILAG Index and SLEDAI.

Recommendation 6: The LFA and its partners should conduct a systematic review of cyclophosphamide to assess its treatment effect. Cyclophosphamide is a chemotherapy used to treat moderate-to-severe lupus flares and is considered the standard of care, but has not been approved by the FDA for the treatment of lupus.

Recommendation 7: The current interpretation of regulations pertaining to clinical trial design and related standards of evidence used to evaluate investigational drugs for lupus should be reexamined by the FDA, in cooperation with experts from industry, research, and the health professions.

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