

**SUPPORT
EDUCATION
RESEARCH**



A non-profit organization
serving the needs of
persons with scleroderma
and related diseases

July 30, 2004

Food and Drug Administration
Division of Dockets Management (HFA-305)
5630 Fishers Lane, Room 1061
Rockville, MD 20852

RE: Docket No: 2004N-0181

The Scleroderma Foundation is pleased to submit these comments in response to the Notice of Comment on the Food and Drug Administration's Critical Path Initiative (2004N-0181), released on April 22, 2004.

The Scleroderma Foundation is a 501(c)(3) national nonprofit organization serving the interests of persons with scleroderma. The Foundation's 40 chapters and 160 support groups nationwide help to carry out its three-fold mission of support, education, and research. The Scleroderma Foundation is the leading nonprofit supporter of scleroderma research-funding over \$1 million of new grants each year to find the cause and cure of scleroderma.

The Scleroderma Foundation is a member of the National Health Council, a private, nonprofit umbrella organization of more than 110 national health-related organizations, working to bring quality health care to all people. We are also a member of the Association of American Autoimmune Related Diseases Association and the National Coalition of Autoimmune Patient Groups.

The Scleroderma Foundation is pleased that the Food and Drug Administration (FDA) is seeking to obtain input from all stakeholders pertaining to the development and design of medical products, and looking for ways to improve the pace and effectiveness of this process as well as potentially reducing the costs. Bringing new and effective treatments to patients with chronic diseases and/or disabilities is a key priority for the Scleroderma Foundation and its members. However, ensuring the safety of such products is of critical concern, as is ensuring that any new products that reach the market are affordable and accessible to all patients who need them.

The FDA has always played a crucial role in ensuring the safety and efficacy of medical products, but has traditionally had a more limited role related to the costs of such products. We applaud the FDA for incorporating this issue into its thinking and encourage FDA to continue to seek ways to enhance the safety and efficacy of medical products while at the same time lowering the cost of development and production.

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First and foremost, the Scleroderma Foundation considers the safety of patients, from clinical research to post-market consumption of products, as the number one priority. We agree that standardization of data collection and submission is a crucial tool to help the FDA review, evaluate, and analyze clinical trial data, and will help lead to greater efficiencies in clinical research and FDA review of New Drug Applications (NDAs). There should also be a new focus on developing better predictive models, using new computer technologies whenever possible, for testing the safety of compounds in the early stages of clinical research.

We commend FDA for recognizing the shift to a chronic care paradigm in health care, and recommend that FDA work closely with academic and industry scientists to develop clinical trials and predictive models that will address the issues of co-morbidities and polypharmacy.

Many patients with autoimmune diseases such as scleroderma have multiple chronic conditions requiring multiple medications, supplements, and other dietary restrictions. For instance, many patients with scleroderma also suffer from pulmonary hypertension, raynaud's syndrome, sjogrens syndrome and autoimmune thyroid conditions. Many people with autoimmune illness suffer chronic long-term symptoms with periods of flare-ups leading to severe disability.

There are over 50 million Americans with autoimmune disease, the nation's leading cause of chronic illness. There are over 100 autoimmune diseases including scleroderma, lupus, multiple sclerosis, juvenile diabetes, rheumatoid arthritis and Crohn's disease. Unfortunately, each illness is viewed separately by the medical establishment and the FDA. There is no medical specialty that diagnoses and treats autoimmune diseases in an integrated way.

In addition, autoimmune diseases such as scleroderma disproportionately affect women. Seventy five percent of all autoimmune patients are women. Diagnosis generally occurs in women from the age of 25 to 50 during the prime child raising and work years. There has been very little attention to or research on the use of most medications for autoimmune diseases and the impact of such medications on a woman's body, her chances of pregnancy while on such medications and on the outcome of the pregnancy.

The Scleroderma Foundation also encourages FDA to work with other federal research agencies and the private/academic sectors to promote the development of tools and techniques to better evaluate, analyze, and predict the safety and efficacy of new compounds and medical products. The National Institutes of Health's "Roadmap" initiative, which focuses on bringing together scientists from multiple disciplines to further the knowledge in basic and translational science, serves as a good model for FDA's work on critical path research.

In addition, the Scleroderma Foundation recommends that FDA should work to ensure that a repository of studies and published data are catalogued and readily available in an

easy-to-navigate format. Previous research can provide tremendous value to scientists, potentially leading to new discoveries at reduced costs.

Conclusion

The Scleroderma Foundation is pleased that FDA is working to provide more innovative products to patients, with lowered costs and enhanced safety and efficacy profiles. We look forward to collaborating with the FDA and other stakeholders to bring scientific advances into the product development process. We thank you for this opportunity to comment on this important initiative.



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