

**SUMMARY STATEMENT
(Privileged Communication)**

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Release Date: 04/26/2004

Application Number: 1 R13 NS049950-01

WHITLEY, CHESTER B MD, PHD
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MINNEAPOLIS, MN 55455

Review Group: ZNS1 SRB-W (09)
National Institute of Neurological Disorders and Stroke Special Emphasis Panel

Meeting Date: 04/13/2004
Council: MAY 2004
Requested Start: 05/13/2004

RFA/PA: PAR03-176
PCC: TAGLEDNG
Dual PCC: DVS CONF
Dual IC(s): DK

Project Title: WORLD Lysosomal Research Network Annual Symposium

SRG Action: Priority Score: 175
Human Subjects: 10-No human subjects involved
Animal Subjects: 10-No live vertebrate animals involved for competing appl.

Project Year	Direct Costs Requested	Estimated Total Cost
1	20,000	20,000
TOTAL	20,000	20,000

ADMINISTRATIVE BUDGET NOTE: The budget shown is the requested budget and has not been adjusted to reflect any recommendations made by reviewers. If an award is planned, the costs will be calculated by Institute grants management staff based on the recommendations outlined below in the COMMITTEE BUDGET RECOMMENDATIONS section.

1 R13 NS049950-01**WHITLEY, CHESTER B.**

RESUME AND SUMMARY OF DISCUSSION: This application requests partial support for the first annual symposium on lysosomal diseases (LD), to be held May 13-15, 2004, in Minneapolis, Minnesota. This was judged to be an important topic that has not been covered appropriately by other meetings. A thoughtfully organized and carefully constructed program is proposed. A relatively unique feature of the proposed meeting is the integration of sessions on ethics and clinical trials with basic science sessions. Little information was provided about plans for involvement of junior investigators or about the methods to be used to recruit women, ethnic minorities or individuals with disabilities. The overall impression that this application left with the reviewers was that this will be an excellent "here is what we know in 2004" meeting. However, it was not clear what would be the outcome of this meeting other than publication of the abstracts. The application was recommended for support with excellent enthusiasm.

DESCRIPTION (provided by applicant): The World Organization for Research on Lysosomal Diseases (WORLD) Clinical Research Center at the University of Minnesota will host the first annual symposium on lysosomal diseases (LD), May 13-15, 2004, in Minneapolis, Minnesota. This symposium is organized to provide an annual multidisciplinary forum to facilitate discussion of basic/clinical research and care issues in LD. Participants include clinicians, geneticists/genetic counselors, neurologists/neuropsychologists, researchers, health care professionals, patients/families, support organizations and industry professionals. Specific aims are to: 1) foster interdisciplinary collaboration with the overall goal of improving knowledge of basic discoveries and clinical manifestations of these diseases; 2) identify and discuss the latest findings in the natural history of LD, diagnostic testing and screening, and treatment; and 3) identify areas requiring additional basic/clinical research. The 2004 meeting is organized into 4 symposium topics, which will include a combination of expert invited speakers, platform presentations selected from submitted abstracts and panel discussions. The Symposium on Longitudinal and Quality of Life (QOL) Studies will identify critical elements in designing and assessing longitudinal and QOL studies for LD. The Symposium on Bioethics and Regulatory Issues will identify and discuss bioethical issues that impact rare disease clinical research. A Symposium on Innovative Therapies will provide an understanding of the pathophysiology and molecular biology of LD and will present new diagnostic techniques/treatment advances, with a focused session on advances in hematopoietic cell transplantation and enzyme replacement therapy. The Symposium of Excellence on Mucopolysaccharidosis: Airway Safety First will identify the role of disease and patient-specific factors in pre-, peri-, and post-operative assessment and management, and pulmonary and respiratory conditions that impact the health and well being of MPS patients. Patients/family sessions will include educational presentations, support services, and round table discussions.

CRITIQUE 1:

Further understanding of the pathophysiology and clinical manifestations of lysosomal disease, improvement of diagnostics and screening, and treatment and identifying areas of further research in this multifaceted set of diseases is an important and timely issue. The group of heritable disorders that result from defects in lysosomal form or function may have severe neurological (and other) manifestations. Prior meetings have addressed issues related to some of these disorders and represent a logical progression of topics from 1988 through 2000. The PI has been involved in the planning and implementation of the past meetings. The May 2004 (current) meeting proposal will cover a broad but well integrated set of topics that are of direct clinical relevance. The planners will also consider needs for future research. The meeting will draw a diverse group of clinicians, basic scientists, and representatives of patient groups and it has good potential for developing novel collaborative efforts and for advancing knowledge and clinical practice. These goals fit well with the NINDS research agenda.

The PI has organized several past and apparently successful meetings that have logically led to this meeting. A large planning committee from the University of Minnesota will assist. The agenda covers four important and relevant topics and is well integrated. At the time of writing, most participants had agreed to attend. The format for the meeting includes presentations and panel discussions which should promote interaction among attendees. There are also plans for public outreach programs that include patient/family educational, support, and discussion sessions, and for disease specific breakout sessions. The meeting appears to have been well publicized and there are plans to publish the meeting abstracts in an appropriate journal. The PI notes that efforts were made to recruit women, ethnic minorities and the disabled. Although no details were given on the methods of recruitment, the data on participation of these groups indicates success. Involvement of junior investigators was not specifically addressed and only a few CVs suggest that junior faculty are highlighted. Furthermore, projections for attendance by this group were not mentioned. Opportunities for open discussion may be slightly limited in general.

The meeting format is by and large, standard. It is an informational meeting that lacks slightly a solid justification of the intended outcomes. The patient outreach sessions are innovative. The topics selected are relevant and timely.

The PI is experienced in the relevant research and in organizing and planning conferences. He has a clear plan and implementation appears to be progressing well.

CRITIQUE 2:

The WORLD Lysosomal Clinical Research Network Annual Symposium plans to bring together basic researchers and clinicians studying all types of lysosomal storage diseases so as to allow cross-discipline dissemination of knowledge, clinical guidelines, and recommendations for diagnosis, evaluation and treatment. The conference should lead to consensus on diagnosis, evaluation and treatment of various lysosomal diseases. If consensus cannot be reached for individual disorders, then new areas for active research will have been identified. NINDS has an interest in the lysosomal diseases and other rare diseases.

The format and agenda for the meeting are appropriate for achieving the goals of the conference. The meeting is timely for the subject matter. NIH's interest in rare diseases has resulted in the funding of 8 consortia studying various rare diseases. It is in this group's interest to take appropriate steps so that it can join the clinical research network in the future. This may be the major reason for having this meeting. The proceedings of the meeting are to be published as a supplement to a journal, such as the Journal of Inherited Diseases. Of the 35 speakers invited to the conference, 37% are female and 11% are from a minority group. There was no discussion about the involvement of Junior Investigators in the application. However, there is one post-doctoral fellow and three assistant professors on the invited speaker list. There is also a breakout session for patients and advocates with specific diseases or disease interests. There do not appear to be formal plans to evaluate the success of the meeting.

CRITIQUE 3:

There is little discussion in the application of specific advances that require a meeting, but the agenda indicates several key areas requiring collaboration for further progress. The meeting will address preclinical modeling of lysosomal storage disorders and mechanisms of intervention as well as issues about clinical interventions, genetics testing, QOL, and medical management. The application reports that this is the only meeting specific to this group of disorders.

The meeting is well organized, albeit not innovative in format. Eleven percent of the speakers are minorities. Patient groups are involved but do not seem to have as major role as might be expected in certain sessions such as the ethics section. Proceedings will be published in an appropriate journal.

THE FOLLOWING RESUME SECTIONS WERE PREPARED BY THE SCIENTIFIC REVIEW ADMINISTRATOR TO SUMMARIZE THE OUTCOME OF DISCUSSIONS OF THE REVIEW COMMITTEE ON THE FOLLOWING ISSUES:

COMMITTEE BUDGET RECOMMENDATIONS: The budget was recommended as requested.

NOTICE: The NIH has modified its policy regarding the receipt of amended applications. Detailed information can be found by accessing the following URL address:
<http://grants.nih.gov/grants/policy/amendedapps.htm>

NIH announced implementation of Modular Research Grants in the December 18, 1998 issue of the NIH Guide to Grants and Contracts. The main feature of this concept is that grant applications (R01, R03, R21, R15) will request direct costs in \$25,000 modules, without budget detail for individual categories. Further information can be obtained from the Modular Grants Web site at <http://grants.nih.gov/grants/funding/modular/modular.htm>

MEETING ROSTER

**National Institute of Neurological Disorders and Stroke Special Emphasis Panel
NATIONAL INSTITUTE OF NEUROLOGICAL DISORDERS AND STROKE
ZNS1 SRB-W (09)
April 13, 2004**

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Consultants are required to absent themselves from the room during the review of any application if their presence would constitute or appear to constitute a conflict of interest.