

Request for Scientific Conferences

Request submitted for September 2007 X March 2008 ___ Round

Title: Integrating Cost-Effective Analysis into Biobehavioral Research for Rare Diseases

Rare disease(s) represented: Multiple rare diseases

Anticipated costs:

Total: 30,000.00

IC Contribution: 5,000.00

Requested ORD cofunding: 25,000.00

Funding mechanism and grant number: National Institute of Nursing Research (NINR) resources to fund a working group to develop a Funding Opportunity Announcement (FOA) for R01 or R21.

Responsible program contact (include title, e-mail/phone/fax): Susan Marden, Ph.D., R.N., Health Scientist Administrator, mardens@nih.gov, 301-594-5973/ 301-480-8260

Responsible budget officer or grants management specialist (include e-mail/phone/fax): NINR Budget Officer, Rebecca Erickson/301-402-5737/fax:301-480-4969

Anticipated participants and other ICs/agencies/organizations (include patient support or advocacy groups or foundations, and organizations in the pharmaceutical industry): Agency for Health, Research and Quality; Federal Drug Administration; National Cancer Institute; National Heart, Lung and Blood Institute; National Organization for Rare Disorders; National Institute on Aging; National Institutes of Allergy and Infectious Disease; and National Institute of Neurological Disorders and Stroke.

Has your IC or Office received ORD conference support on this subject in the last 3 years?
NO.

If so, please explain why you are making this request (follow-up meeting, rapidly evolving area of opportunity, etc.).

Tentative meeting date(s): Summer 2008

Proposed meeting location: Bethesda, Maryland

Brief narrative description (approximately 1 page, single-spaced):

Background/relevant history: Because any single rare disease occurs in a small number of patients (< 200,000), the economic impact is surmised to be less than another illness that affects a wider population. However, with over 6,000 rare diseases identified in the United States, this group of disorders may pose a significant societal burden. Rare diseases are often associated with disability, loss of role and employment status, chronic morbidity, and early mortality requiring the extended use of expensive treatment modalities. New health care technologies and interventions, including interventions for self-management, for rare diseases are emerging. Thus,

it will be important to develop the capacity to identify technologies and interventions that maximize health outcomes and optimize the cost-benefit ratio. Rare disease experts such as the 2004 NINR sponsored working group on “Increasing Opportunities in Biobehavioral Research: Expert Working Group Utilizing Allergic Bronchopulmonary Aspergillosis as a Framework” have recommended increased attention to the economic impact of rare diseases for both the individual and society. Therefore, a research agenda focused on cost-effective analysis (CEA) will be a critical step in shaping future policy initiatives to support the allocation of scarce health resources for the treatment of rare diseases. Integrating cost-effectiveness with evaluation of biobehavioral interventions promotes a research agenda of long standing interest to the NINR.

Proposed content/scientific issues to be addressed: The use of CEA in research and its relevance to the health policy process will be presented. The tools involved in CEA to include cost-utility analysis, discounting and inflation, sensitivity analysis, and a complementary approach, cost-consequence analysis will be examined. Current trends and issues integral to applying CEA in research of rare diseases will be discussed as well as strategies for assembling an interdisciplinary research team to perform a CEA of a biobehavioral intervention.

Anticipated goals and use of results: The purpose of the working group is to discuss current knowledge related to CEA in rare diseases, identify gaps in this body of knowledge and strategize next steps for incorporating CEA for the evaluation of biobehavioral interventions. It is anticipated that the working group will provide recommendations that will promote the development of a research agenda that integrates this important tool in the study of rare diseases.

Proposed format: A one and half day working group. Brief didactic presentations will be enhanced by opportunities to explore CEA through case examples and group discussion.

Plans for publicizing meeting and dissemination of meeting outcome: The working group will be publicized on the websites of the NINR, Office of Rare Diseases, National Organization for Rare Disease. Dissemination of workshop proceedings will be posted on the NINR website and potentially lead to an NINR FY08 or 09 trans-NIH FOA on cost-effectiveness and quality of life interventions.

Plans to assess the meeting’s effectiveness in terms of goals, use of results, or development of specific research initiatives: The development of a FOA and investigator response rate to it.

Proposed agenda (please provide as an attachment to this form or provide outline here): Topics to be explored include:

- CEA and the Policy Making Process
- Measuring Costs over Time
- Cost-Utility Analysis
- Health Benefit Valuation
- Inflation and Discounting
- Current Trends and Issues in CEA Research in Rare Diseases
- Developing an Effective Team to Conduct Cost-Effectiveness Research

Potential participants (please provide as an attachment to this form or provide list here): Experts in CEA and clinical research scientists focused on biobehavioral interventions in rare diseases will be invited to participate in this working group.

Speaker list (draft):

Kevin Frick, PhD is an Associate Professor in the Department of Health Policy and Management at the Johns Hopkins Bloomberg School of Public Health.

Michael Drummond, DPhil is Professor of Health Economics, Centre for Health Economics, University of York, Heslington, York, UK.

Peter Neuman, M.D., Sc.D., Professor of Medicine, Institute for Clinical Research and Health Policy Studies, Tufts-New England Medical Center, Boston MA.

David Feeny, Ph.D. is a health economics researcher and Professor of Economics; Institute of Economics, University of Alberta, Canada.

William Lawrence, M.D., M.S. is a Senior Service Fellow in Outcomes Research in the Center for Outcomes and Evidence at the Agency for Healthcare Research and Quality.

Joanna Siegel, RN, ScD is Director, Research Initiative in Clinical Economics, Center for Outcomes and Evidence at the Agency for Healthcare Research and Quality.

Patricia Stone, PhD, MPH, RN is an Assistant Professor, Columbia University, School of Nursing.

Web link(s) (If available): Not available