



NIH BACKGROUND

National Institutes of Health

Re-engineering the Clinical Research Enterprise Translational Research

One of the ways the Re-engineering the Clinical Research Enterprise Roadmap effort will support translational research is by developing new technologies to improve the assessment of clinical outcomes. Many of the most debilitating chronic illnesses gradually erode patients' quality of life because of the associated fatigue, pain, and mood changes. Currently, these critical symptoms cannot be objectively measured in the same way, for example, as blood sugar levels or blood cell counts. More sensitive, well-validated tools are needed to improve and standardize measurements of these types of symptoms. Technologies, such as a computerized adaptive health assessment, could revolutionize how symptoms and treatment outcomes are assessed. Equipped with these tools, scientists will be better equipped to understand how patients perceive changes in their health status resulting from new treatments, thereby directing research to therapies that would be most highly valued by patients.

Measuring patient-reported outcomes is particularly important in research studies where changes in clinical measurements or imaging results may not translate into recognizable benefits to patients. Assessing patient-reported outcomes is also important in clinical trials where two treatments may have similar effects in controlling or curing disease but different effects on symptoms, function, or other quality of life issues. Having a validated, dynamic system to measure patient-reported outcomes efficiently in study participants with a wide range of chronic diseases and demographic characteristics would greatly enhance the clinical outcomes research enterprise and facilitate comparisons between research studies. Ultimately, this type of system will be useful in medical practice, for the purpose of measuring treatment response and guiding therapy.

Advances in computer technologies and in modern measurement theory now make it possible for researchers to develop, maintain, and modify so-called item banks, where the items are key questions intended to assess degree of functioning on a specific dimension of health such as mobility or pain. This approach allows scientists to compare items and conduct statistical modeling of patient responses. Computerized adaptive testing methods allow subsets of items to be tailored to individual patients. This initiative will establish a collaborative network of researchers that will develop and implement a publicly available system consisting of a large item bank and computerized adaptive tests, both of which will be managed by a statistical coordinating center. The Patient-Reported Outcomes Measurement Information System (PROMIS) will focus on the collection of self-reported data from a diverse population of chronic disease patients, including those from racial and ethnic minority groups. PROMIS will support a comprehensive, integrated approach to data collection, storage, and management.

The URL for the NIH Roadmap web site is nihroadmap.nih.gov. For more information on the Re-engineering the Clinical Research Enterprise Dynamic Assessment of Patient-Reported Chronic Disease Outcomes initiative, contact Deborah Ader, Ph.D., National Institute of Arthritis and Musculoskeletal and Skin Diseases, (301) 594-5032, aderd@mail.nih.gov. Further information about NIH can be found at its Web site: www.nih.gov.

To improve human health, scientific discoveries must be translated into practical applications. Such discoveries typically begin at "the bench" with basic research--in which scientists study disease at a molecular or cellular level--then progress to the clinical level, or the patient's "bedside."

Scientists are increasingly aware that this bench-to-bedside approach to translational research is really a two-way street. Basic scientists provide clinicians with new tools for use in patients and for assessment of their impact, and clinical researchers make novel observations about the nature and progression of disease that often stimulate basic investigations.

Translational research has proven to be a powerful process that drives the clinical research engine. However, a stronger research infrastructure could strengthen and accelerate this critical part of the clinical research enterprise. The NIH Roadmap attempts to catalyze translational research in various ways, including the following two projects:

Regional Translational Research Centers

Key to building a strong infrastructure is increasing interactions between basic and clinical scientists and easing the movement of powerful new tools from the laboratory to the clinic. One strategy to achieve this involves NIH facilitating the development of Regional Translational Research Centers, or RTRCs. These centers will provide sophisticated advice and resources to help scientists master the many steps involved in bringing a new product from the bench to medical practice. Such steps involve laboratory studies to understand how a therapy works and animal studies to determine how well a therapeutic agent is absorbed into the body, how it is distributed to target tissues, how effective it is, and how likely it may be to cause unanticipated side effects.

For additional information on the RTRC initiative contact Anthony Hayward, M.D., Ph.D., National Center for Research Resources, (301) 435-0791, haywarda@mail.nih.gov.

Translational Research Core Services

Once a potential new drug is developed, sufficient amounts must be made according to rigorous standards for testing--first in animals, then in people. The NIH Roadmap also envisions enhancing clinical researchers' access to sophisticated manufacturing capacity and expert advice to ensure that drug-development regulations are observed. This program, entitled Translational Research Core Services, will be modeled on or may evolve through expansion of the National Cancer Institute's Rapid Access to Innovation Development program, which currently provides such resources to the cancer research community. Their availability to the broader research community should expedite discoveries for other major public health challenges.

For more information on the Translational Research Core Services program, contact Josephine Briggs, M.D., National Institute of Diabetes and Digestive and Kidney Diseases, (301) 496-6325, briggsj@extra.niddk.nih.gov.

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