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October 2004

GP45-A

Studies to Evaluate Patient Outcomes; Approved Guideline

This guideline describes the essential issues in planning outcomes research, including resources needed, formulating a research question, validity and sources of error, feasibility, and ethical issues; addresses the design and implementation of a patient outcomes research plan, including study design, study subjects, measurements, interventions, and analysis; summarizes recommendations for reporting patient outcomes research; and includes definitions, references, and resources for those interested in planning, conducting, and using patient outcomes research.

A guideline for global application developed through the Clinical and Laboratory Standards Institute consensus process.

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Studies to Evaluate Patient Outcomes; Approved Guideline

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Abstract

CLSI document GP45-A—*Studies to Evaluate Patient Outcomes; Approved Guideline* provides an overview of patient outcomes studies and health services research to assist healthcare providers, managers of healthcare services, and others in planning, conducting, and reporting patient outcomes research. This guideline describes the essential issues in planning outcomes research, including resources needed, formulating a research question, validity and sources of error, feasibility, and ethical issues; addresses the design and implementation of a patient outcomes research plan, including study design, study subjects, measurements, interventions, and analysis; summarizes recommendations for reporting patient outcomes research; and includes definitions, references, and resources for those interested in planning, conducting, and using patient outcomes research.

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Foreword

A number of factors have converged to make efforts to monitor and improve the quality of health care increasingly important. Rapid changes in the organization and financing of services have led to unprecedented efforts to reduce the use and cost of services, while not adversely affecting either the delivery of services or patient outcomes. As the average lifespan of the population increases, the focus of medical care has shifted from the traditional role of providing treatment for acute care and the prevention of premature mortality to a role of helping people manage more chronic conditions. An increased interest in maintaining, prolonging, and improving the quality of life has prompted patients to want to be better informed about their care options. However, informed decision making requires that measures of patient outcomes are available and understood, and work, in addition to being cost effective.

Large variations in the way health care was practiced across the country became evident from studies done by Wennberg.[1] Importantly, these differences in practice did not lead to obvious differences in patient outcomes. In addition, research by Brooks on appropriateness of care[2] and Eddy on the poor quality of medical evidence[3] indicated that much of the care being provided was either unnecessary or inappropriate, regardless of the intensity of practice variation. Therefore, the assumed scientific basis for much of the established practice was called into question, and it was evident that studies were needed to determine which healthcare practices would be most effective and lead to better patient outcomes.

Recently, society has become increasingly concerned not only about the large variation in healthcare practice, but also with ensuring access to care and with reducing the costs of care. This has led to questions about whether care could be optimized by following specified protocols (i.e., practice guidelines). Several approaches have been developed to determine how best to improve patient outcomes, reduce variations in practice across the country, and contain costs. These include health services research, managed care, and national quality assurance activities.

Finally, researchers have begun to question the value of clinical trials as the gold standard to guide practice. The need to examine outcomes other than clinical endpoints, to look at outcomes of longer duration than those in a typical clinical trial, and to look at procedural interventions beyond drugs and clinical treatment has prompted the development of a whole new field to examine interventions applied to patients on a daily practical basis.

This document provides guidance to providers of healthcare services and manufacturers of healthcare products to assist with designing and conducting studies to evaluate patient outcomes. Using the tools provided in this document will help providers determine what works in their healthcare setting and to become the problem solvers, innovators, and quality improvement experts of the future. In addition, this document will assist those who wish to evaluate previously conducted studies by illustrating the strengths and weaknesses of various study designs. It can also help those involved in patient safety, quality improvement, and quality assurance activities to link their efforts more closely with improving patient outcomes, which is the ultimate goal of all of our efforts.

Key Words

Best practice, cost, effectiveness, efficiency, evidence-based medicine, health services research, patient outcomes, patient safety, processes, quality improvement, structure

Studies to Evaluate Patient Outcomes; Approved Guideline

1 Scope

This guideline can be applied to studies to evaluate patient outcomes by any service in a healthcare organization or manufacturer of a healthcare product. It includes essential elements to consider in either conducting studies, or evaluating previously conducted studies. The principles described are universal and can be used to make decisions about the most appropriate structure and processes to use for delivery of healthcare services. The document has been developed through the NCCLS consensus process and describes general criteria for conducting studies of patient outcomes. It is not intended to be a primer or manual for conducting research. There are several excellent books available for readers interested in more specific information about how to conduct a patient outcomes study, including information on qualitative and quantitative research methods (see the Additional References section).

The focus of this guideline is on primary studies in patient outcomes research. These include observational studies (surveys or cross-sectional studies, case-control studies, and cohort studies) and interventional studies (randomized controlled trials and nonrandomized studies).

The role of systematic overviews, meta-analyses, decision analyses, cost-effectiveness analyses, and simulations is described briefly.

2 Introduction

The guidance described in this document can be used to evaluate patient outcomes by anyone in the healthcare field. It is designed to meet the needs of both the providers of healthcare services, who are under increasing pressure to provide effective and efficient patient care, and the manufacturers of medical devices and kits, who are in an increasingly competitive market and must demonstrate the added value of their products. The techniques described provide the means that anyone in the healthcare field could use to answer basic questions about the quality and effectiveness of the services they provide, pay for, or oversee. In particular, these techniques should be useful to those who must find ways to evaluate and improve the quality of service they offer. Evaluation of the impact of changes in structure or processes on patient outcomes can help identify ways to reduce errors in processes and practices, to avoid mistakes, and to evaluate the validity of claims by others. Since patient outcomes is the ultimate measure of success or failure in health care, it is essential that those who work in the field know what works and what does not work in order to produce better patient outcomes.

2.1 Potential Impact of Outcomes Studies

Appropriately designed patient outcomes studies conducted at even a single institution can have a significant impact on local, regional, and even national policies, practices, and future health care. Advances in the field of outcomes research have progressed to the point that it is increasingly recognized that: (1) evidence, not opinion, should guide healthcare decisions; (2) more patient outcomes studies are needed to help determine the best way to deliver the benefits and avoid the risks of the complex, but technologically advanced healthcare system; (3) methods are available to conduct patient outcomes studies; and (4) such studies could lead to patient care alternatives that could provide better patient outcomes, sometimes at lower cost.

Properly conducted patient outcomes studies have the capacity to provide decision makers with the evidence they need to make changes in policies, procedures, and practices. Studies have many uses, but are most often applied in the following ways:

- to advocate changing customary practice to evidence-based practice;

- to evaluate technologies or procedures in a different or specific setting not previously studied;

- to evaluate the effect of economic or social issues on outcomes (Outcomes and effectiveness research seeks to understand the end results of particular healthcare practices and interventions. By linking the care people receive [taking into account their social and economic environment] with outcomes they experience, outcomes research becomes the key to developing better ways to monitor and improve the quality of care.);

- to develop clinical practice guidelines, which are systematically developed statements to assist practitioner and patient decisions about appropriate health care for specific clinical circumstances (The National Guidelines Clearinghouse (NGC) at http://www.guideline.gov is intended to make evidence-based clinical practice guidelines widely available to healthcare professionals.);

- to develop criteria for accreditation programs (For example, in the U.S., the National Committee for Quality Assurance (NCQA) HEDIS® (Healthplan Employers Data and Information Set) measures, the Joint Commission on Accreditation of Healthcare Organizations (JCAHO) ORYX program, and The Foundation for Accountability (FACCT) help identify and promote patient-oriented measures of healthcare quality.);

- to assist government programs with decisions about reimbursement and other policies;

- to determine whether the processes and practices we employ in our healthcare services are of the quality required to provide adequate and appropriate patient care;

- to improve patient safety (The critical issue of medical error and patient safety has received a great deal of attention as a result of the Institute of Medicine (IOM) report,[4] which estimated that as many as 98,000 patients in the United States die as the result of medical errors in hospitals each year. More studies are needed to determine how often medical errors occur and result in patient injury.);

- to evaluate the effectiveness of introduction of a new structure or standardized protocol (Healthcare organizations need to test the effectiveness of the transfer and application of systems-based best practices to reduce medical errors and improve patient safety. Such research will help identify high-risk patients or patient groups, providers, healthcare processes and settings, as well as develop generalizable methods for error reduction.);

- to find better ways to manage patient care (Quality management can be thought of as the broad umbrella that those responsible for the management of an organization place over the entire organization. This includes the policies, practices, and processes needed to ensure that the facility, the personnel, the technical aspects of the service, etc. meet the intended goals for patient care. Studies of patient outcomes assist efforts to improve the provision of services.); or

- to define the best practices in health care (The results of properly designed, conducted, and analyzed patient outcomes studies contribute to the body of evidence for best practices. The conscientious, explicit, and judicious use of current best evidence to make decisions has been termed "evidence-based medicine."[5])

2.2 The Need for a Guideline for Outcomes Research

In our present environment of limited healthcare resources, providers of patient care are often unprepared to provide or obtain data needed for decision making about ways to improve the quality or reduce the cost of the care they offer. They are even less able to demonstrate a difference in patient outcomes as a result

The Quality System Approach

NCCLS subscribes to a quality system approach in the development of standards and guidelines, which facilitates project management; defines a document structure via a template; and provides a process to identify needed documents through a gap analysis. The approach is based on the model presented in the most current edition of NCCLS document HS1—*A Quality System Model for Health Care.* The quality system approach applies a core set of "quality system essentials (QSEs)," basic to any organization, to all operations in any healthcare service's path of workflow. The QSEs provide the framework for delivery of any type of product or service, serving as a manager's guide. The quality system essentials (QSEs) are:

Documents & Records	Equipment	Information Management	Process Improvement
Organization	Purchasing & Inventory	Occurrence Management	Service & Satisfaction
Personnel	Process Control	Assessment	Facilities & Safety

Path of Workflow

A path of workflow is the description of the necessary steps to deliver the particular product or service that the organization or entity provides. For example, GP26-A2 defines a clinical laboratory path of workflow which consists of three sequential processes: preanalytic, analytic, and postanalytic. All clinical laboratories follow these processes to deliver the laboratory's services, namely quality laboratory information.

GP45-A describes a path of workflow for evaluating patient outcomes. The steps included in the path of workflow are indicated by an "X."

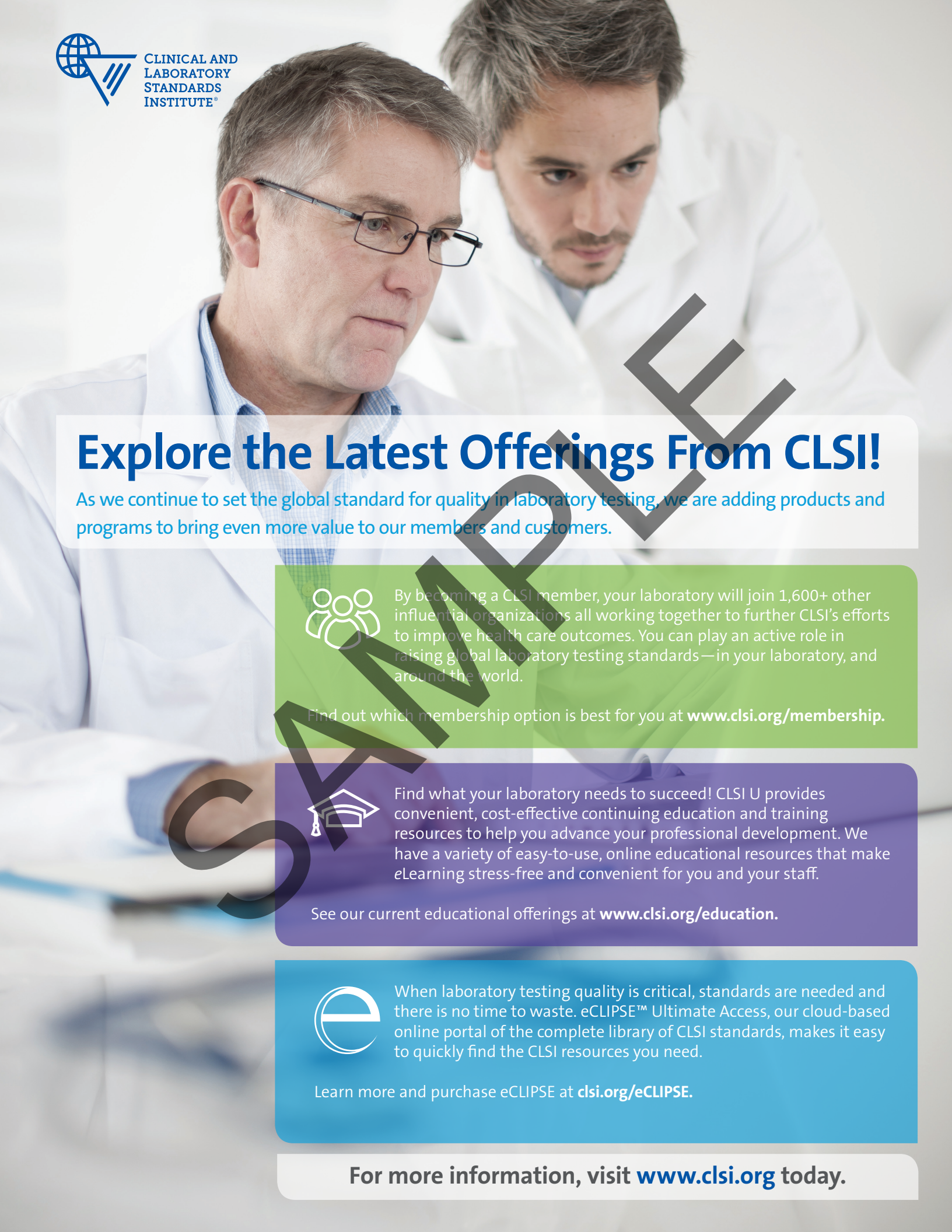
Planning the Study															Conducting the Study										Reporting/ Disseminating the Study		
Formulate the research question	Assess feasibility	Assess scientific merit	Review literature	Assess relevance to practice	Develop specific aims and hypothesis	Select study design	Choose study setting and sites	Develop criteria for subject eligibility and exclusion	Plan analysis and sample size	Assess threats to validity (chance, bias, confounding)	Assess human subjects and ethical issues	Write protocol	Submit to institutional review committees	Obtain administrative approval	Secure funding	Assemble team	Train staff	Establish procedures and operations	Recruit subjects	Measure baseline characteristics	Apply intervention	Measure outcomes	Analyze results	Implement methods to reduce chance, bias, confounding	Interpret findings	Submit for publication	Report results to institution and funding agencies	Translate research into practice
X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X

Adapted from NCCLS document HS1—*A Quality System Model for Health Care*.

Related NCCLS Publication*

HS1-A **A Quality System Model for Health Care; Approved Guideline (2002**). This document provides a model for healthcare service providers that will assist with the implementation and maintenance of effective quality systems.

* Proposed- and tentative-level documents are being advanced through the NCCLS consensus process; therefore, readers should refer to the most recent editions.



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