Appendixes

Appendix A.—Acronyms and Glossary

Acronyms

ADAMHA – Alcohol, Drug Abuse, and Mental Health Administration (PHS)
ALL — acute lymphoblastic leukemia
AMIS — Aspirin Myocardial Infarction Study
AML — acute myelocytic leukemia
BP — blood pressure
CABG — coronary artery bypass graft (surgery)
CAT — computed axial tomography
CCU — coronary care unit
CDP — Coronary Drug Project
CEA — cost-effectiveness analysis
CME — continuing medical education
CSP — Cooperative Studies Program (VA)
CT — computed tomography
DBP — diastolic blood pressure
DES — Drug Efficacy Study
DES — diethylstilbestrol
DHHS — Department of Health and Human Services
DOD — Department of Defense
DRS — Diabetic Retinopathy Study
ECOG — Eastern Cooperative Oncology Group
FDA — Food and Drug Administration
HBGM — home blood glucose monitoring
HCFA — Health Care Financing Administration (DHHS)
HCT — historical control trial
HMO(s) — health maintenance organization
ICU — intensive care unit
IHCE — Institute for Health Care Evaluation
MRFIT — Multiple Risk Factor Intervention Trial
NAS — National Academy of Sciences
NCHCT — National Center for Health Care Technology (PHS)
NCI — National Cancer Institute (NIH)
NEI — National Eye Institute (NIH)
NHLBI — National Heart, Lung, and Blood Institute (NIH)
NIAAA — National Institute on Alcohol Abuse and Alcoholism (ADAMHA)
NIADDK — National Institute of Arthritis, Diabetes, and Digestive and Kidney Diseases (NIH)
NIAID — National Institute of Allergy and Infectious Diseases (NIH)
NIDA — National Institute on Drug Abuse (ADAMHA)
NIEHS — National Institute of Environmental Health Sciences (NIH)
NIGMS — National Institute of General Medical Sciences (NIH)
NIH — National Institutes of Health
NIMH — National Institute of Mental Health (ADAMHA)
NINCDS — National Institute of Neurological and Communicative Disorders and Stroke (NIH)
NRC — National Research Council (NAS)
NSABP — National Surgical Adjuvant Project for Breast and Bowel Cancers
OHTA — Office of Health Technology Assessment (PHS)
OMAR — Office for Medical Applications of Research (NIH)
OTA — Office of Technology Assessment (U.S. Congress)
PHS — Public Health Service (DHHS)
POSCH — Program on the Surgical Control of the Hyperlipidemias
RCT(S) — randomized clinical trials
TAR — Treatment Assessment Research
VA — Veterans Administration

Glossary

Apheresis: A procedure that separates the blood into its basic components (red cells, white cells, platelets, and plasma) and selectively removes one or more of these components from the blood for the purpose of curing, alleviating, or treating a disease and its symptoms.

Blinding: Keeping secret which treatment is assigned to participants in randomized clinical trials. When only the patient is kept unaware of his or her treatment assignment, the study is "single-blind;" when the person administering treatment (e.g., the physician) *also* is unaware, the study is "double-blind." Additional layers of blinding can be added—e.g., when a third individual, the evaluator of outcome, also is unaware of treatment assignments.

Chemotherapy: The treatment of disease by chemical agents.

Concurrent controls: In a clinical trial, individuals

given a "control treatment" during the same time period as experimentally treated individuals, usually used to refer to individuals not formally enrolled in the trial.

Consensus: General agreement on a subject, not necessarily grounded in fact.

Control group: In a randomized clinical trial, the group receiving treatment with which the group receiving experimental treatment is compared. The control treatment is generally a standard treatment, a placebo, or no treatment.

Crossover: In a randomized clinical trial, switching of treatment during the course of the trial. Crossovers can be planned as part of the trial method, or unplanned, a consequence of an individual's changing medical condition.

Device (medical): Any physical item, excluding drugs, used in medical care (including instruments, apparatus, machines, implants, and reagents).

Disease prevention: The aversion of disease, traditionally characterized as primary, secondary, and tertiary prevention. Primary prevention aims at avoiding disease altogether. Secondary prevention strategies detect disease in its early stages of development, with the hope of improving outcome. Tertiary prevention attempts to arrest further deterioration in individuals who already suffer from a disease.

Drug: Any chemical or biological substance that may be applied to, ingested by, or injected in order to prevent, treat, or diagnose disease or other medical conditions.

Effectiveness: Same as efficacy (see below) except that it refers to average or usual conditions of use.

Efficacy: The probability of benefit to individuals in a defined population from a medical technology applied for a given medical problem under ideal conditions of use.

Experimental group: In a randomized clinical trial, the group receiving the treatment being evaluated for safety and efficacy. The experimental treatment may be a new technology, an existing technology applied to a new problem, or an accepted treatment about whose safety or efficacy there is doubt.

External controls: In a clinical trial, individuals given a "control treatment" with which the experimentally treated group is compared, but who are not formally enrolled in the trial. External controls may be historical or concurrent.

Historical controls: In nonrandomized clinical trials, individuals treated with a "control treatment" outside the study proper, at some time previous to the trial, against which the experimentally treated individuals are compared.

Mammography: X-ray examination of the breast, used as both a screening procedure on apparently healthy females, and as a diagnostic procedure in clinical situations to detect breast cancer.

Medical technologies: Drugs, devices, and medical and surgical procedures. The organizational and supportive systems through which medical care is provided are part of medical technology in its broadest sense, but are not discussed in this report.

Minimization: In randomized clinical trials, a method of patient allocation which seeks to minimize different distributions of prognostic factors between treatment groups without creating mutually exclusive subgroups.

p **value:** In a randomized clinical trial, the probability of concluding that there is a difference between the treatment groups when, in fact, there is none. Also called "Type I error" or "alpha" and commonly called the "level of statistical significance, " analogous to "false positive. "

Phase I, II, and III drug trials: The sequence of studies in human beings required for new drug approval by the Food and Drug Administration. Phase I includes studies in a small number of relatively healthy patients or normal volunteers to determine safety and pharmacologic effects. Phase 11 includes controlled clinical trials to determine appropriate doses, safety, and effectiveness in a total of about 200 patients. Phase III trials are usually randomized clinical trials (RCTS).

Placebo: A drug or procedure with no intrinsic therapeutic value which mimics the drug or procedure being tested in a randomized clinical trial. A placebo is used in control groups as a means to blind patients and investigators as to whether an individual is receiving the experimental or control treatment.

Prognostic factors: Symptoms, signs, or characteristics of an individual that are known to be predictive for certain disease outcomes.

Random allocation: In a randomized clinical trial, allocation of individuals to treatment groups such that each individual has an equal probability of being assigned to any group.

Randomized clinical trial (RCT): An experiment designed to test the safety and efficacy of a medical technology in which people are randomly allocated to experimental or control groups, and outcomes compared.

Risk: A measure of the probability of untoward outcomes occurring, and the severity of the resultant harm to health of individuals in a defined population associated with use of a medical technology, applied for a given medical problem under specified conditions of use.

Safety: A judgment of the acceptability of risk in a specified situation,

Statistical power: In a randomized clinical trial, the

probability of detecting a difference between the treatment groups when one does exist. Failure to detect an effect is called "Type 11 error" or "beta," analogous to "false negative. "

Statistical significance: See p value.

Stratification: In randomized clinical trials, the categorization of individuals for the purpose of adjusting the groups to take into account unequal distribution of characteristics of prognostic importance. Stratification may be used during patient allocation, creating subgroups within which individuals are randomized to treatments; or stratification maybe applied during data analysis to statistically adjust for differences between the groups.

Synthesis: The integration of findings from different studies and the development of generalizations based on their results.

Type I error: See p value.

Type II error: See statistical power.

Validity: A measure of the extent to which an observed situation reflects the "true" situation. *Internal validity* is a measure of the extent to which study results reflect the true relationship of a technology to the outcome of interest in the study subjects. *External validity* is a measure of the extent to which study results can be generalized to the population which is *represented* by individuals in the study.