

Medical Research: Saving Lives Through New Cures and Treatments

Americans have benefited from tremendous progress in medical research over the past 60 years. Today, people are living longer and healthier lives thanks in significant part to NIH-funded research conducted by physicians and scientists at the nation's medical schools and teaching hospitals. But even though reports of medical discoveries are in the news nearly every day, new cures, treatments, prevention approaches and diagnostics are actually the products of a long and arduous process.

The Road to Discovery

There are three different categories of medical research that take place in laboratories and at the patient's bedside.

Basic Research Debunks Notion that Aging is Inevitable

Ten years ago, biochemist Cynthia Kenyon, Ph.D., found a way to double the lifespan of a microscopic roundworm—by manipulating a single gene. With support from NIH, Kenyon's team at the University of California at San Francisco has been using DNA microarray technology to trace all the genetic changes that flow from that single gene change. Because the same pathway affects the lifespan of fruit flies and mice, it's possible that it also affects the lifespan of humans. And these changes don't just delay aging, they postpone the diseases of aging, including Huntington's disease and cancer, as well. "The consequences are stunning," Kenyon says, "and if we can figure out a way to copy these effects in humans, we might all be able to live very healthy long lives."

Basic research

This type of research seeks to answer important biological questions like:

- How do cells talk to each other?
- How are genes regulated?
- Why does the body sometimes destroy its own tissues?

Basic research typically takes place in a laboratory. While it does not involve human subjects, human tissues or fluids may be used. Basic research often relies on studies in "model organisms" such as yeast, fruit flies or mice. Human cells contain the same molecular building blocks and pathways as those of most other living things, so researchers can learn a great deal about the way cells work by studying these simpler organisms.

Although these studies may not have an immediate impact on health, it is "untargeted," investigator-initiated basic research that most often has led to breakthroughs that transform our understanding of life's complex processes and provide the leads to new medicines, technologies and research tools. Examples of advances that grew out of basic research include: biotechnology; a variety of drugs used to treat conditions from cancer to AIDS; magnetic resonance imaging (MRI), which provides a different way of viewing the body's organs and tissues; and the polymerase chain reaction, a laboratory technique that is the basis of "DNA fingerprinting," which revolutionized criminal forensics. In general, about two-thirds of the grants NIH awards to outside scientists are for basic research.

Clinical Genomics Research Uncovers a Genetic Susceptibility to HIV/AIDS

Sunil K. Ahuja, M.D., an infectious disease expert at University of Texas Health Science Center at San Antonio, wanted to know why some people infected with HIV do not suffer immune system damage. By studying the genetic make-up of more than 4,000 HIV-positive and -negative individuals, Ahuja and his colleagues found that people with more copies of a gene that fights HIV are less likely to become infected with the virus or to develop AIDS than people who have fewer copies of the gene. Their NIH-supported study was published in January 2005 and could lead to a screening test to determine individual susceptibility to HIV/AIDS and enable doctors to adapt treatment regimens or vaccine trials to the patient's needs. Ahuja collaborated on the research with Matthew J. Dolan, M.D. of the U.S. Air Force's Wilford Hall Medical Center and Brooks City-Base in San Antonio.

Clinical Research

Many of the advances pioneered in medical school laboratories are then developed and tested at medical schools and their affiliated teaching hospitals through clinical research programs funded by NIH grants. Clinical research is made possible through the participation of people who volunteer to take part in scientific studies. Clinical trials and epidemiologic studies are two types of the important research that falls within this area.

Clinical trials, also called patient studies, usually aim to produce a therapy—a drug, or a new device or a vaccine, for example—that will slow, cure, or perhaps prevent a disease. These studies are usually done in three phases: Phase I clinical trials aim to find out whether a new experimental treatment is safe in people and to establish the best dose to test in larger studies; Phase II trials test the safety and efficacy of new medications, medical devices or surgical treatments; and Phase III trials test a new treatment or procedure compared to the current standard of care. These trials usually enroll large numbers of people and may be conducted in many teaching hospitals, doctors' offices, and clinics nationwide. Medical schools and teaching hospitals frequently serve as sites for clinical trials to advance treatment for an array of conditions, including cancer, cardiovascular diseases, diabetes, and neurological and orthopedic disorders.

Epidemiologic studies examine factors that influence distributions of diseases in population groups. Harvard Medical School, for example, is conducting several large, long-term population-based studies, including the NIH-funded Nurses' Health Study I and II. In these two studies, participants are surveyed every two years to gather information on diet, smoking, physical activity, medications, health screening behavior, as well as occurrence of cancer, cardiovascular disease, and other serious illnesses, such as diabetes, fractures, kidney stones, and pre-cancerous lesions. Important findings have come from these studies, including the effect of aspirin use on various cancers and the impact of fish diets and omega-three fatty acids on heart disease.

Approximately 37 percent of the NIH's extramural budget supports clinical research.

Translational Research Curtails Heart Disease Deaths

Benefits from basic, clinical and epidemiologic research on heart disease include the development of cholesterol- and blood pressure- lowering drugs and prescriptions for changes in behavior (less dietary fat, no smoking, more exercise). Deaths from heart disease have dropped dramatically as a consequence. And the research continues. Seven years ago, cardiologist Paul Ridker, M.D., at Brigham and Women's Hospital, a teaching hospital in Boston, set a two-part goal: to provide evidence that inflammation produced by an immune-system reaction in the arteries caused heart attacks; and to devise a method for doctors to detect inflammation in their patients. In a series of landmark studies, Ridker's group revealed that serum C-reactive protein (CRP), an indicator of inflammation, could be used to detect and measure inflammation in the arteries as well as predict first-ever heart attacks and strokes. They also provided critical evidence that life-saving "statin" drugs not only lower cholesterol, but also lower CRP. Researchers at the Cleveland Clinic published results supporting that conclusion.

Translational Research

This kind of research seeks to move basic research findings from the laboratory to patients and populations and speed up the back-and-forth exchange between basic and clinical science. Investigators at medical schools and teaching hospitals historically have been the chief agents in this type of research where observations are often first made at the patient's bedside, and then move to the laboratory "bench" where the mechanisms of disease are studied at a molecular or cellular level. The laboratory findings are then brought back to the bedside to improve patient care. This approach requires intense two-way collaborations between basic scientists and clinical researchers and a corps of physician-scientists who are expert in both laboratory science and medical care.

The National Cancer Institute's Specialized Programs of Research Excellence (SPOREs) initiative is an example of the federal government's expanding support for translational research. Most of the 60 SPOREs, which cover 14 different cancers, are located at medical schools, teaching hospitals, and affiliated cancer centers, such as Baylor College of Medicine, Johns Hopkins University School of Medicine, and Duke University Medical Center.

To learn more about how medical schools, teaching hospitals and the NIH are working together to fulfill the promise of medical research, go to www.aamc.org/ftp.