

DEPARTMENT OF HEALTH AND HUMAN SERVICES
NATIONAL INSTITUTES OF HEALTH

FY 2008 Budget for the National Institutes of Health:
A New Vision for Medical Research (Part I)

Witness appearing before the
Senate Subcommittee on Labor-HHS-Education Appropriations

Duane Alexander, MD, Director, NICHD
National Institute of Child Health and Human Development

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Mr. Chairman and Members of the Committee:

I am pleased to present the Fiscal Year (FY) 2008 President's budget request for the National Institute of Child Health and Human Development (NICHD). The FY 2008 budget includes \$1,264,946,000.

With continuous support from this Committee, the NICHD has made significant discoveries that have improved the health and well-being of children and adults. For instance, in the 45 years since the NICHD was founded, our research has been largely responsible for a decline in infant mortality of more than 70 percent, a 93 percent reduction in the rate of mother-to-child transmission of the AIDS virus, the elimination of five major causes of mental retardation, successful treatments for infertility, an effective intervention for reducing a major cause of premature birth, and many other benefits. Our scientists around the country are grateful to this committee for providing the opportunity to pursue research in these areas.

The Institute's research agenda builds on the discoveries from the last decade, addresses some of our country's and the world's most critical health needs, and moves us closer to major breakthroughs against diseases and conditions such as infertility, birth defects, infections, limb loss, premature birth, and maternal death.

PRESERVING FERTILITY FOR WOMEN FACING CANCER TREATMENT

The chemotherapy and radiation used to treat cancer can irreparably damage the body's reproductive tissues and render men and women infertile. Males may have the pre-treatment option of storing their frozen sperm for later use, but no comparable option currently exists for women. Eggs seldom survive the freezing and subsequent thawing processes required for storage. Currently, the only option for women facing the prospect of such infertility is *in vitro* fertilization and long-term storage of the embryos, which tolerate freezing. However, this option is not always suitable. Young women with cancer may be forced to forego having their own children in order to receive life-saving treatment. The NICHD's new Fertility Preservation Research Program seeks to develop treatments to preserve fertility among patients with cancer or environmental risks for infertility. Building on current research, such as using a gelatin mixture to surround the follicle containing the egg, our scientists will be developing new techniques to protect the egg during the freezing, thawing, and maturation process. The goal is to allow a small section of the ovary to be removed and frozen for later use. When the woman is ready to start a family, the frozen follicles could be thawed and then cultured. The resulting eggs could be fertilized and implanted in the uterus to establish a pregnancy.

PROTECTING OUR CHILDREN AS WE TREAT THEIR ILLNESSES

The Best Pharmaceuticals for Children Act (BPCA) — enacted by Congress to increase information about the safety, usefulness, and dosage of medications for infants and children—is an important part of the nation’s ongoing effort to assure that our treatments for children do not harm them. As we have learned, children’s immature body systems and metabolic rates make pediatric clinical trials essential for studying the impact of widely prescribed drugs on children and infants. Within its work on the BPCA, the NICHD, in consultation with the Food and Drug Administration, identifies and prioritizes drugs for pediatric clinical study. The NICHD collaborates with manufacturers and academia in designing and implementing preclinical and clinical studies of drugs that are widely used or integral to the care of children with specific medical conditions. Currently 29 studies are under way evaluating drugs to provide information for labeling to guide pediatric use.

PREVENTING DISABILITIES THROUGH NEWBORN SCREENING

Imagine being able to know if an infant has one of hundreds of genetic or metabolic disorders by testing a single drop of a newborn’s blood. Imagine being able to treat the condition as soon as it is identified, sparing that infant an early death or a lifetime of mental retardation or physical disability. This screening and treatment, developed in large part through NICHD research, now is provided universally in the U.S. for a few such disorders. For example, the National Newborn Screening and Genetic Research Center reports that congenital hypothyroidism (CH) occurs once in every 3,000 births, affecting 1,300 children each year in the United States. Without treatment, an infant with CH will suffer irreparable brain damage within months and require a lifetime of special care. Because an NICHD grantee developed a screening test for the disorder in the 1970s, children with CH are now routinely identified at birth and treatment begins immediately. One thyroxine pill daily spares them from the brain damage that would otherwise result, thus eliminating CH as a significant cause of mental impairment. The cost of treatment: a few pennies a day; the lifetime net dollar savings: \$140 million each year; the human suffering prevented: priceless.

Currently, the number of conditions for which newborns are screened varies widely from state to state. The March of Dimes notes that nearly all of the 4.1 million American infants born each year undergo screening for some disorders, and about 5,000 are diagnosed with an abnormality. Treatments exist for the conditions for which we now screen, as well as for others for which screening is not yet possible. To remedy this situation, the NICHD is funding a series of contracts to develop gene-based technologies that can identify hundreds of rare genetic disorders in a single test. In addition, the Institute will fund new projects to spur research on new treatments for potentially screenable disorders. Examples of conditions in these categories are Spinal Muscular Atrophy, the leading genetic cause of infant death, and Fragile X Syndrome, the leading inherited cause of mental retardation. Expanded efforts in FY 2008 will

include creating a multi-site newborn screening translational research network to test the most promising new screening technologies and experimental treatments in collaboration with state newborn screening programs.

REDUCING ANOTHER CAUSE OF INFANT MORTALITY: NEC

Through research led by the NICHD, one cause of infant mortality after another has yielded to treatments based on new discoveries. Respiratory distress syndrome, severe jaundice, meningitis, and Sudden Infant Death Syndrome cause far fewer deaths today. One remaining problem is necrotizing enterocolitis (NEC). This condition affects 10 to 12 percent of infants weighing less than three pounds, and about 30 percent of those affected will not survive. NEC attacks and destroys their intestines. Unfortunately, its incidence and mortality rate have not changed in 40 years. Now, new NICHD studies give hope that prevention or effective treatment can become a reality. One study in mice demonstrated that epidermal growth factor, administered orally, was highly protective against NEC. Another study, in humans, demonstrated protection against NEC from interleukin-10 in breast milk. These and other potential therapies will be tested in a new NICHD initiative on NEC to be launched in FY 2008.

DEVELOPING IMPROVED PROSTHETICS

As the country's Armed Forces return from stations abroad, and as the nation's population continues to age, increased attention is needed on medical rehabilitation. The Institute's National Center for Medical Rehabilitation Research is a leader in such efforts and provides a Federal focal point for research in this important field. Among the initiatives in the Center's portfolio is developing mechanical limbs that allow for better comfort and mobility. Advances in this area can be particularly helpful to veterans who have lost limbs in combat. One exciting new finding from this research: an amputee can move and have functional use of a prototype prosthetic arm simply by thought. Thinking about moving the arm stimulates the chest muscles to contract. Microprocessors in the arm read the nerve signals sent by the chest muscles, and movement flows with relative ease and greater speed and precision. Researchers hope to use similar technology to restore natural movement and sensation to the limbs of individuals paralyzed by injury or stroke.

HELPING DEVELOPING NATIONS OVERCOME DISEASE

Every 30 seconds, malaria takes the life of a child somewhere in the world. The mosquito-borne disease kills more than one million people each year and severely sickens millions more in developing countries, crippling economic growth. It is one of the world's leading health concerns. Researchers at the NICHD's Laboratory of Developmental and Molecular Immunity—in partnership with researchers in the Malaria Vaccine Development Branch of the National Institute of Allergy and Infectious Diseases, and the Biotechnology Unit of the National Institute of Diabetes and Digestive and Kidney Diseases—may have a solution.

These researchers have developed an experimental vaccine that stops the spread of malaria, mosquito by mosquito. The vaccine eliminates the parasite responsible for malaria from the digestive tract of a malaria-carrying mosquito after it has fed on the blood of a vaccinated individual. Future bites from this mosquito then no longer transmit the disease. If it is proven safe and effective, the vaccine could free entire geographic regions from this destructive disease.

The NICHD's research investments to improve health in developing nations go beyond laboratory benches. The Institute supports the Global Network for Women's and Children's Health Research, an initiative devoted to addressing the leading causes of illness and death in pregnant women and their infants in developing countries. This year one network study, a randomized double blind clinical trial conducted by birth attendants in rural India, demonstrated that giving women a single dose of misoprostol, a uterine muscle constrictor, just after delivery nearly eliminated the incidence of severe post-partum hemorrhage, a leading cause of maternal mortality in developing countries worldwide. India immediately took action to make misoprostol treatment available as standard care throughout the country, and other nations are doing the same. This one simple and cost effective intervention will save the lives of millions of women throughout the developing world.

Mr. Chairman and members of the committee, I would like to thank you for your continued support of the Institute's research as we strive to understand disease and improve the health and well-being of men, women, children, and future generations in the United States and around the world. I will be pleased to answer any questions.