Great Achievements in Pediatric Research
in the last 40 years

American Academy of Pediatrics
DEDICATED TO THE HEALTH OF ALL CHILDREN™

PEDIATRIC POLICY COUNCIL
Advancing child health through public policy
PROBLEM

Rotavirus infection remains the leading cause of severe diarrheal illness and dehydration in children worldwide. In 2008, about 450,000 children worldwide under 5 years old died from vaccine-preventable rotavirus infection.

Prior to vaccine development, rotavirus caused 20-60 deaths each year in U.S. children under 5 years of age. Haemophilus influenzae type b (Hib) is a bacteria that causes many different types of disease in children younger than 5 years of age, including brain infection (meningitis), lung infection (pneumonia), and severe throat infection (epiglottitis).

Prior to the Hib vaccine, about 20,000 U.S. children had Hib infections every year, and up to 1,200 children died.

DISCOVERY

Research into the development of a vaccine to protect against rotavirus infection started in the mid-1970s. Studies elucidated the effect of rotavirus on a child’s immune system and how initial exposure to the virus protected that child from future illness.

Once the prototypal rotavirus vaccine was created, subsequent research tested ways of improving its efficacy while maximizing safety.

Following research and many efficacy and safety studies, the first rotavirus vaccine for widespread public use was approved in 2006.

Studies showed that the most severe infections from Hib tended to occur in young infants, due to their immature immune systems. A multi-dose vaccine schedule was created in order to maximize the protection of infants from infection with Hib, with the first dose given at 2 months of age.

OUTCOME

Receiving the full schedule of rotavirus immunization decreases the occurrence of gastroenteritis by 86% and required hospitalization for gastroenteritis by 96%.

Since the administration of the vaccine, the yearly cases of Hib infection have decreased by 99%.

Currently, most mortality from Hib occurs in developing countries, where vaccination is not routine.
REDUCING SIDS WITH “BACK-TO-SLEEP”

PROBLEM
Sudden Infant Death Syndrome (SIDS), also referred to as “crib death,” occurs without warning and is associated with a sleep period.

A diagnosis of SIDS is given when no explanation can found for the baby’s death after a complete postmortem investigation.

SIDS is the leading cause of death for infants between 1 month and 1 year of age. In 1993 alone, nearly 4,700 U.S. infants died from SIDS.

DISCOVERY
Research found that if infants were placed to sleep on their stomachs, their risk of dying from SIDS increased by at least two-fold.

OUTCOME
As a result, the “Back-to-Sleep” Campaign was initiated in 1994 by a collaboration between the National Institute of Child Health and Development, the American Academy of Pediatrics (AAP), the Maternal and Child Health Bureau of the Health Resources and Services Administration and SIDS groups.

The focus of the campaign was to encourage parents to put their babies to sleep on their backs in order to reduce the risk of SIDS.

The AAP Task Force on SIDS published a policy statement in 2005 encouraging the practice of “Back-to-Sleep” for all infants.

Research showed that between 1993 and 2010 the percent of infants placed to sleep on their backs increased from 17% to 73%. Following the initiation of the “Back-to-Sleep” campaign, the number of infants dying from SIDS has decreased to 2,063 per year as of 2010.

CURING A COMMON CHILDHOOD CANCER

PROBLEM
Acute lymphocytic leukemia (ALL) is the most common childhood cancer. In the United States alone, about 2,900 children and adolescents are diagnosed with ALL each year.

In 1975, a diagnosis of ALL often meant death in the near future. Only 60% of children under 15 years of age survived 5 years, and only 28% of 15-19 year olds survived 5 years.

DISCOVERY
Cancer research has led to the creation of various medications and treatments that destroy cancer cells.

OUTCOME
Research into various combinations of medications to treat ALL has yielded the now standard protocol of induction chemotherapy, which consists of vincristine, L-asparaginase, and corticosteroid for a 2-3 year period.

Because of the last 40 years of research, 90% of newly diagnosed ALL pediatric patients are expected to have greater than 5 year survival.
**SAVING PREMATURE BABIES**
**BY HELPING THEM BREATHE**

**PROBLEM**
Prematurity is the main cause of global death in newborn infants.

In the United States alone, 1 in 9 newborns is born prematurely, about 450,000 children a year.

Respiratory Distress Syndrome (RDS) is a life-threatening respiratory condition frequently diagnosed in premature newborns. Research showed that it develops when the immature lungs are unable to produce a compound known as surfactant in quantities needed for the infant to breathe.

**DISCOVERY**
Studies demonstrated that instillation of surfactant to the lungs of premature animals reduced respiratory distress.

Clinical trials of supplementary surfactant for newborns with RDS led to the medication’s approval by the FDA for widespread use in 1990.

**OUTCOME**
As a result of surfactant administration, newborn deaths from RDS have decreased by 41% between 1985-1991.

*Kingston McKinnon (then):* Born prematurely, unable to breathe on his own.

*Kingston McKinnon (now):* 3 years old, happy, healthy and strong.
PROBLEM

Human Immunodeficiency Virus (HIV) is a virus that kills the cells comprising the immune system. The more these cells are killed, the harder it is for the body to fight infection. When enough of these cells are killed, HIV infection causes Acquired Immunodeficiency Syndrome (AIDS), with the affected person eventually dying from overwhelming infection.

HIV can be transmitted via the blood, vaginal secretions, semen, anal secretions and breast milk.

Mothers infected with HIV can transmit the virus to their children before the child is born, during the birthing process and after the child is born via breast milk.

This mother-to-infant transmission of HIV can be as high as 40%.

In 1991, the rate of mother-to-baby HIV transmission peaked to 1,650 newborns becoming infected with HIV.

DISCOVERY

Research showed that the use of Zidovudine, an antiretroviral medication, given to mothers during pregnancy and to their infants after birth decreased this rate of transmission by up to two-thirds.

Additional research showed that combinations of antiretroviral medications could reduce the transmission rate even more, to less than 2%.

OUTCOME

In the United States using combinations of anti-retrovirals is now standard in pregnancy and around the time of birth, leading to a decrease in the rate of mother-to-infant HIV transmission of more than 90%.

Currently, fewer than 200 babies become infected with HIV in the United States each year, even though more women with HIV than ever before are giving birth. Research has also led to a reduction in infant HIV infection worldwide with a goal for elimination in 2015.

HIV research was critical in this dramatic reduction in HIV transmission. Other research initiatives have led to increased life expectancy for persons – including children - who are HIV-infected.
INCREASING LIFE EXPECTANCY FOR CHILDREN WITH CHRONIC DISEASE

PROBLEM

Sickle Cell Anemia is an inherited disorder that causes a person’s red blood cells (RBCs) to change their shape and go from being round to forming a C-shape. This change causes the cells to be more easily destroyed and also to clump together forming clots.

Clotting prevents the blood from flowing normally, causing extreme pain, infection, and organ damage including strokes.

Sickle cell disease affects about 90-100,000 Americans.

Forty years ago, the life expectancy for someone living with Sickle Cell Anemia was just 14 years.

DISCOVERY

Research has shown the inheritance patterns of sickle cell disease, including the gene mutations. As a result, all U.S. newborns are routinely screened for sickle cell disease.

Because of this research, a life-saving medication, hydroxyurea has been created and studied, which increases fetal hemoglobin to reduce the effects of sickle cell disease and helps the red blood cells to remain the correct shape and to be less fragile.

The use of prophylactic antibiotics and vaccines have also been studied and incorporated into the standard of care for a child with the disease, preventing important and often fatal illnesses, such as meningitis.

Bone marrow transplantation has been used as a curative therapy, and more gentle means of transplantation are being developed that are effective.

OUTCOME

Combining preventive care with standardized medical management of sickle cell crises have helped to increase the current life expectancy to more than 40 years of age.

Policy efforts to increase pediatric access to care, such as the Children’s Health Insurance Program (CHIP), have increased the reach of these best-practice algorithms.

If not for improvements in sickle cell research...

“I would not have been born in the first place if it were not for improvements in sickle cell research.

“I hope that further research continues and that one day, a cure will be discovered to prevent any more loved ones from being lost.”

Tokunbo Olaniyan, 16-year-old whose mother died of complications from sickle cell disease in 2004
SAVING LIVES WITH CAR SEATS AND SEAT BELTS

PROBLEM
Motor vehicle accidents are a leading cause of death in children ages 1-18.
One in 3 children who die in a motor vehicle collision are unrestrained.

DISCOVERY
Researchers have studied the behaviors of drivers and passengers, and used crash test dummy analyses to better characterize the contributory elements of motor vehicle fatalities.
This testing has shown how the bodies of children can sustain many kinds of fatal injuries, such as being thrown from the vehicle, if not properly restrained.

OUTCOME
In children younger than 1 year, using car seats decreases mortality by 71%.
When compared to only using a seatbelt, car seats reduce the risk of injury by 54% in children 1-4 years old, and in children 4-8 years old, booster seats reduce the risk by 45%.
In older children and adults, the use of seat belts decreases the risk for death and serious injury by about 50%.
THE **NEXT** 7 GREAT ACHIEVEMENTS IN PEDIATRIC RESEARCH

These 7 achievements would not have been possible without pediatric research. Significant strides have been made toward treating chronic diseases, preventing injury, and eliminating illness in children. This work has led to new therapies, interventions, vaccines, and diagnostic tests that have improved the lives of children worldwide.

While major advancements have been achieved through research to improve the child health, children in the United States and across the globe continue to suffer from serious and deadly health conditions that deserve more research.

*Stronger federal support for pediatric research is needed to continue to build on investments in child health.*

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