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Great Achievements in Pediatric Research

IN THE PAST 40 YEARS

1 Preventing Disease with Life-Saving Immunizations

Millions of lives around the world have been saved because of research, development, and delivery of vaccines. Diseases like rotavirus and Haemophilus influenzae type b, which can be fatal, are now preventable thanks to vaccines.

2 Saving Premature Babies by Helping them Breathe

Deaths from Respiratory Distress Syndrome, a major killer of premature babies, have been reduced by two thirds since the introduction of surfactant.

3 Reducing Sudden Infant Death with Back to Sleep

The Sudden Infant Death Syndrome (SIDS) rate has declined by half due to research and a campaign urging parents and caregivers to put babies to sleep on their backs initiated by a coalition including federal agencies, the American

Academy of Pediatrics and parent advocacy groups.

4 Curing a Common Childhood Cancer

More than 90% of children with the most common childhood cancer, acute lymphocytic leukemia, now survive because of research discoveries. As recently as the 1970s, only 57% of these children survived longer than five years.

5 Preventing HIV Transmission from Mother to Baby

Twenty years ago, one in four mothers with HIV transmitted HIV to their babies. Today, because research demonstrating that medication combinations given during pregnancy could reduce mother-to-child transmission to less than 2%, the majority of these babies are born HIV-free.

6 Increasing Life Expectancy for Children with Chronic Diseases

Forty years ago, U.S. children with sickle cell disease or cystic fibrosis had a life expectancy of just 14 years. Now, the life expectancy for people with these conditions is over 40 years.

7 Saving Lives with Car Seats and Seat Belts

Motor vehicle injuries remain the leading cause of death in the United States for both children and adults up to age 44. Research leading to vehicle safety laws has significantly reduced child and adolescent motor vehicle deaths.

American Academy
of Pediatrics



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Preventing Disease with Life-Saving Immunizations

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 annually had Hib infections every year, and up to 1,200 children died.

Discovery

Vaccine research includes laboratory vaccine development, testing effectiveness in humans, testing ways to get children vaccinated and reducing barriers to immunizations.

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.

VACCINES SAVE LIVES

Vaccinations are now a routine part of children's lives in developed countries like the United States, though their immunizing effects are often taken for granted.

Research leading to the development of new vaccines continues to save millions of lives worldwide.

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 8 newborns is born prematurely, about 500,000 children each year.

Respiratory Distress Syndrome (RDS) is a life-threatening respiratory condition frequently diagnosed in premature



newborns, and develops when the immature lungs are unable to produce a compound known as surfactant in quantities needed for the infant to breathe.

Discovery

Research identified that the lungs of premature babies were deficient in surfactant.

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.



Pictured throughout: Nathaniel and Collin Cull, twin boys born prematurely and given surfactant, now thriving 16-year-olds.

Reducing Sudden Infant Death Syndrome 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 be 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.

"BACK-TO-SLEEP" BECAME THE STANDARD ADVICE GIVEN BY PHYSICIANS DURING ROUTINE PEDIATRIC VISITS.

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
- 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.



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.

Outcome

Because of the last 40 years of research, **90%** of newly diagnosed ALL pediatric patients are expected to have greater than 5 year survival.

Preventing HIV Transmission from Mother to Baby

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.

While a diagnosis of HIV used to be grim, many people with HIV are now able to live full lives.

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.

As a result 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 antibiotic administration 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.



"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, 15-year-old whose mother died of complications from sickle cell disease in 2004

Pictured: Omolara Olaniyan and her son Muyiwa

Saving Lives with Car Seats and Seat Belts

Problem

Motor vehicle accidents are the leading cause of death in children aged 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.

In infants, using car seats decreases mortality by **71%**

This injury prevention research has driven policy change across the country, including required use of car seats, booster seats, and seat belts for children and several national public

awareness campaigns, such as Click It or Ticket, to encourage compliance with the law.

Outcome

In children younger than 1 year, using car seats decreases mortality by 71%.

In children 1-4 years old, car seats and booster seats reduce injury by 45% when compared to only using a seatbelt.

In older children the use of seat belts decreases mortality by about 50%.

Conclusion

- 1 Preventing Disease with Life-Saving Immunizations
- 2 Saving Premature Babies by Helping them Breathe
- 3 Reducing Sudden Infant Death with Back to Sleep
- 4 Curing a Common Childhood Cancer
- 5 Preventing HIV Transmission from Mother to Baby
- 6 Increasing Life Expectancy for Children with Chronic Diseases
- 7 Saving Lives with Car Seats and Seat Belts

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.

Strong federal support for pediatric research is needed to continue to build on the investments made in child health research to date.