MEDICAL INNOVATION: CHILDHOOD PNEUMOCOCCAL VACCINE (PHARMACEUTICAL: BIOLOGICALS)

Academic Institution: Ronald Eby, Dace Madore, Velupillai Puvanesrajah
Industry: Praxis Biologics

Situation

A million childhood deaths a year

Pneumococcal diseases are conditions caused by contact with the pneumococcus bacterium that are especially devastating to young children. Among the worst of these diseases are pediatric pneumonia, severe ear infections, meningitis (infection of the brain and spinal cord lining) and a dangerous blood infection known as bacteremia.

Prior to the development of a childhood vaccine in 2000, one million children died from pediatric pneumonia each year worldwide, and in the United States, a form of pneumococcus bacteria known as Streptococcus pneumonia caused some 17,000 cases of bacteremia, 700 cases of meningitis and 17,000 cases of pneumonia in children annually.

Physician-Industry Collaboration

A novel approach to vaccine development

While a vaccine against most types of pneumococcal bacteria had been developed for adults in 1977, it was not effective in children under the age of five, because their immune systems were not sufficiently developed to be able to react to the vaccine. In the mid-1980s, Dr. Ronald Eby of Praxis Biologics and two of his colleagues set out to change that through an innovative approach to vaccine development, described in detail by Innovation.org:

Existing vaccine technologies used the outer coat of bacteria to trigger an immune response from the body. Dr. Eby used this approach, but linked a protein to the bacteria's outer coat that could be recognized by an infant's immune system. The catch was that S. pneumoniae comes in seven main forms, so Dr. Eby had to create a separate compound for each of these and roll all of them into one seven-part vaccine.

Dr. Dace V. Madore, whose specialty was in clinical trials, was called upon to help in the development of a clinical program that could bring this new vaccine to the public. In order to introduce a new therapy for widespread use, scientists have to conduct rigorous studies to show that it is both safe and effective in humans. Because the vaccine was unique in that it was targeting seven forms of one bacterium, she had to develop new methods for running the clinical testing.

Once the vaccine was shown to be safe and effective, the challenge of making the quantities of vaccine required for the millions of children worldwide was left to Dr. Velupillai Puvanesarajah. He had already made the 30,000 to 40,000 doses that clinical trials alone required and now 10,000 times that number would be needed for distribution to millions of children. The large-scale production of this vaccine required an innovative multi-step process to ensure safety and consistency from batch to batch. The resulting manufacturing process, equipment and facilities pioneered by Dr. Puvanesarajah have successfully manufactured more than 72 million doses of the vaccine, known as Prevnar, to date since it was first approved in 2000.

Innovation Benefits

A devastating childhood disease all but eliminated in the U.S.

In just over a decade, the vaccine has been credited with all but wiping out invasive pneumococcal disease in young children in the U.S. and other developed countries that administer it regularly, and it has become recognized as one of the greatest vaccine breakthroughs of the late twentieth century. The Centers for Disease Control and Prevention (CDC) estimates that the vaccine prevented well over a hundred thousand cases of invasive pneumococcal disease in young children in the U.S. in the first eight years since it was developed.

In 2010, the U.S. Food and Drug Administration (FDA) approved an updated version of Prevnar -- using the same novel approach to vaccine development -- that protects against 13 of the most common pneumococcal bacteria, instead of simply the seven covered by the earlier vaccine. This new vaccine is rapidly becoming the worldwide standard for childhood vaccination against the disease.

Patient Benefits

'We nearly lost our twin boys…'

The CDC tells a powerful story of one mother's battle to save her twin boys who came down with invasive pneumococcal disease from a bacterial strain that the original vaccine didn't cover, but one that the newer version of Prevnar would protect against:

"Pneumococcal disease attacked our family with such force that we nearly lost our twin boys," says Andrea, the boys' mother.

The family's fight against pneumococcal disease began in the fall 2003. Andy and Peter were three years old when they became sick. Andy developed a high fever and also had a seizure. He was rushed to the hospital by an ambulance. Both boys had pneumonia and empyema (a severe lung complication of pneumonia). Andy had hemolytic uremic syndrome (a severe kidney and blood problem). Both boys were admitted to the hospital and had tubes put into their chests to drain fluid from around their lungs.

Andy's case became more serious as the infection attacked several organs. He was placed in the intensive care unit (ICU) and put on life support. Andrea explains, "Seeing Andy on life support and dialysis to help his kidneys function was our worst nightmare. We weren't sure if he was going to make it."

Fortunately, the treatments Andy received worked, and he was taken off life support after four days. After almost three weeks, the boys went home from the hospital. The twins still had a long recovery. Andrea explains, "Andy was so sick and didn't talk for so long. Peter needed physical therapy to help him walk. It was so sad to see them regress because of a vaccine-preventable disease." The twins eventually made a full recovery, but their life was impacted for months.

Andrea says, "I feel so lucky that my boys are still here with us today. I encourage every parent to get the pneumococcal vaccine for their children."