



How Has Basic Science Research Improved Treatment For Lung Diseases?

What is basic science research?

Basic science research:

- Is conducted to better understand what causes a disease, to test how current treatments work, and to develop new treatments.
- Is done in a laboratory using test tubes, not people, and looks at the cellular and molecular level of life.
- Has greatly improved the treatment of many lung diseases. A few examples are listed below.

Lung Cancer

Lung cancer is the leading cause of cancer death in the United States and the world. Recent developments in the understanding of the molecular genetics of lung cancer have identified key molecules that can predict how well a cancer treatment will work. The best example is the work that focused on the Epidermal Growth Factor Receptor (EGFR), a protein that is present in many lung cancers. Treatments that block the function of the EGFR protein are now being used to treat advanced lung cancers, and can improve survival in some lung cancer patients. This is just one of several examples of the application of basic science research being used to guide treatment and improve survival of lung cancer patients.

Cystic Fibrosis

Cystic fibrosis (CF) is one of the most common genetic diseases among Caucasians. An important breakthrough in our understanding of CF occurred when scientists identified the mutated gene that causes CF, the cystic fibrosis transmembrane conductance regulator (CFTR). This landmark finding has allowed scientists to develop molecules that improve the function of the mutated CFTR. Two such molecules are currently being tested in CF patients. Early results of these

trials are very encouraging and may signal the arrival of the first therapies to address the genetic defect that causes CF.

Respiratory Distress Syndrome

One of the most compelling stories in lung research is the development of treatment for premature babies suffering from Respiratory Distress Syndrome. Investigators showed that a molecule called surfactant prevented the collapse of the lung during normal breathing. They then found that the lungs of premature babies did not have surfactant. This lack of surfactant was responsible for Respiratory Distress Syndrome. These findings led to the development of artificial surfactant for prevention of Respiratory Distress Syndrome. Use of artificial surfactant has led to a 25% decline in deaths of premature babies.

Emphysema

Emphysema causes a heavy health and economic burden both in the United States and around the world. In some patients, scientists discovered that the lack of an enzyme, alpha-1 antitrypsin (AAT), caused emphysema. Health care providers are now able to test their patients for AAT and provide effective counseling. This includes advising patients to stop smoking, which delays the onset and severity of the emphysema. In addition, patients can be given supplemental AAT, to reverse the lack of AAT. Knowing how AAT works has helped to understand how emphysema develops, even in patients who do not have AAT deficiency, and offers hope for future treatments for emphysema.

These examples show how basic science research has used findings from the laboratory to improve care at the bedside that leads to an improvement in quality of life and survival in lung diseases.

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