Health Awareness

Finding The Key To New Medical Breakthroughs

(NAPSA)—When it comes to medical breakthroughs, basic research is the first important step to understanding how diseases develop and where to intervene. Whether studying animals or plants, the payoff can be big.

By studying algae—pond scum—scientists moved closer to new treatments for a dangerous condition called polycystic kidney disease (PKD). PKD is a genetic disorder that affects more than 600,000 Americans and 12.5 million people worldwide. Half of those diagnosed progress to kidney disease by age 60.

Doctors have known about PKD for years, but it wasn't until recently that a few researchers, lab mice and some algae helped reveal the disorder's true cause: a defective type of small, common, hair-like structures called primary cilia. Now this finding is leading to breakthroughs involving many other diseases.

A Surprise Finding

Cilia are found in just about every type of organism, from one-celled creatures to multicelled human beings. Although known to help with certain vital functions elsewhere in the body, primary cilia in the kidneys were long thought not to do much of anything.

But then cell biologists studying cilia shocked the scientific world. They discovered that when a gene involved in the development of normal cilia in algae mutated (was altered), the algae couldn't build normal cilia. Switching to mice, scientists were able to show that genetic mutations leading to defective kidney cilia led mice to die of PKD soon after birth.

Research on one disease led to breakthroughs for other conditions.

A Wave Of Discovery

Doctors were surprised and questioned the results. But as the evidence accumulated, it became commonly accepted that abnormal cilia were key to PKD. Further discoveries in different fields linked defective cilia to heart disorders, male infertility, extra fingers and toes and some rare birth defects marked by poor kidney function.

Understanding how disease develops—the mechanisms—requires basic research like this. Then scientists develop and test drugs to target those mechanisms. This takes time. To speed the search for new medicines, scientists are using zebra fish with cystic kidney disease to test drugs already approved by the U.S. Food and Drug Administration for other diseases. Researchers have already identified one drug that has stopped PKD-related cysts in their tracks in animals. Testing in humans may be just a few years away.

Future Breakthroughs

The story of PKD and cilia is just one example of how unexpected discoveries in basic research can lead to unsuspected connections for human health. As scientists identify more disease mechanisms and drug targets through basic research, new treatments could be just around the corner. U.S. National Institutes of Health funds this—and other—basic research across the U.S.

Visit http://tiny.cc/ASCBPKD for more information on cilia, PKD or NIH.