Treating the root cause of cystic fibrosis, rather than just the symptoms, may be an ideal approach to improving patient outcomes for people with the disease. Thanks to a new study conducted by scientists at Howard Hughes Medical Institute and University of Illinois at Urbana-Champaign, a treatment strategy based on this concept may be possible in the future. Principal investigator Martin D. Burke and colleagues have developed a small molecule channel that performs a function similar to that of the defective protein in cystic fibrosis (known as the CFTR protein).
According to a news release from the Journal of the American Chemical Society, which published Dr. Burke’s article, cystic fibrosis is caused by a genetic mutation that affects certain cellular proteins that make up “channels,” which act like gates managing the flow of ions in and out of cells. “In cystic fibrosis, some of these gates don’t work like they’re supposed to.” Since multiple small molecules exist that allow directional movement of specific ions but not other ions or molecules, Dr. Burke’s team believed they could use imperfect mimicry of functioning channels to restore proper channel function.

All of the studies in Dr. Burke’s laboratory were conducted on yeast with missing ion channel proteins, as reported in the article, “Restored Physiology in Protein-Deficient Yeast by a Small Molecule Channel.” The small molecule capable of proper ion flow was amphotericin B, a molecule most commonly known for treating fungal infections. When the researchers applied amphotericin B to yeast without the correct proteins to transport ions into and out of its cells, the yeast grew almost as well as normal yeast.

According to the team, amphotericin is a channel-forming small molecule capable of working with the protein pumps and channels in the yeast cell membranes to restore the correct flow of ions. While this technology is far from translation into clinical studies with cystic fibrosis patients, the research platform provides an interesting new mechanistic framework for future therapeutic strategies.

With the approval of new therapies such as Kalydeco and Orkambi, CF treatments are beginning to move in the direction of addressing the underlying cause of the disease. This new research could further advance that trend in CF drug development.
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Maureen Newman is a senior Science and Research writer for Cystic Fibrosis News Today. Maureen is currently a PhD student studying biomedical engineering at University of Rochester, working towards a career of research in biomaterials for drug delivery and regenerative medicine. She is an integral part of Dr. Danielle Benoit’s laboratory, where she is investigating bone-homing therapeutics for osteoporosis treatment.

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