

AHEAD OF THE CURVE

EMERGING CF THERAPIES 2009



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Ongoing Research into CFTR Modulation – John Paul Clancy, MD

Learning Objective:

1. Describe the function of CFTR modulators and how they differ from current traditional CF treatments.
2. Identify what types of CF patients might benefit from CFTR potentiators.
3. Understand the similarities and differences between different types of CFTR modulator strategies (suppressors of premature termination codons, potentiators and correctors).

Abstract:

So what is a CFTR modulator, and why is it different from other CF therapies that are currently in use? All currently available therapies in CF treat downstream symptoms of the disease (such as clearance of mucus, infection, and inflammation). CFTR modulators, rather than targeting CF symptoms, address the underlying defect(s) that cause CF based on the type of mutation responsible. These therapies are designed to restore function to CF-causing mutant CFTR proteins that don't work normally; addressing primary causes of disease and therefore possibly having effects before disease manifestations. While fully realizing the potential of these therapies is a few years away, treating the root cause of CF through protein restoration is the long-term goal. This presentation will cover some of the ways that CFTR mutations lead to disease, and different agents that are being developed to restore function to mutant CFTR proteins.