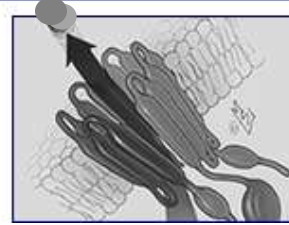


# AHEAD OF THE CURVE

## EMERGING CF THERAPIES 2009



### Pathophysiology & Therapeutic Targets – Richard B. Moss, MD

#### Learning Objectives:

1. Differentiate the stages of CF pathogenesis and pathophysiology amenable to discrete therapeutic intervention.
2. Review several competing/complementary theories of pathogenesis affecting timing and choice of therapeutic intervention.
3. Describe the promising areas of new targeted interventions.

This speaker has disclosed that his/her presentation may reference the following unlabeled/unapproved use of drugs or products: Denufosal, mannitol, hypertonic saline, azithromycin, colistin, PTC124, VX770, VX809, Tobi

#### Abstract:

Defective CFTR leads to defective ion transport in the respiratory tract. It is now widely accepted that airway surface liquid depletion is a central driver of pathogenesis. The creation of obstruction in airways is due to resultant defective mucociliary clearance but also due to the presence of infection and inflammation, and the latter are also likely to drive progressive loss of lung function. Better understanding of the temporal sequence of these early events is expected to lead to more effective early intervention. Ideally correction of defective CFTR function will ameliorate or resolve these “downstream” events.