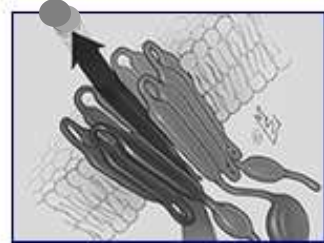


AHEAD OF THE CURVE

EMERGING CF THERAPIES 2009



Transcript: Introduction & Course Goals: Peter Mogayzel, MD

Thank you all for coming to hear what I think is going to be a very interesting and fun program. As you heard, there is going to be audience participation through keypads, so that we can get your feelings about what you've learned and do some cases to see if you can figure out the best approaches to some interesting problems that are going to be coming up in the future. This talk is not about what you do now, these are a series of talks about what is going to happen in the future, in the not too distant future.

I have disclosures related to things that come to Johns Hopkins, the Cystic Fibrosis Center there. The conference goals, as you've heard, are really to engage in an interactive learning experience so that you can understand some of the therapeutic targets of research, and to integrate information about emerging therapies.

The learning objectives are listed in your book and summarized here. And we have had several wonderful speakers. Dr. Richard Moss is going to talk to us about the pathophysiology of cystic fibrosis and selective targets for correcting the ion defect in CF. JP Clancy, from the University of Alabama is going to talk to us about emerging therapies related to CFTR modulation. Pam Zeitlin from Johns Hopkins is going to take the opposite approach and say why do we need CFTR, there is all this other stuff that we can use as a therapeutic target. And then finally Chris Goss from the University of Washington is going to speak about issues in managing expectations of patients, both now while we're doing trials, and in the future after these drugs are approved.

The final business, official thing I need to tell you, is the disclaimer. This program is provided by an unrestricted grant from Vertex Pharmaceuticals, which means that they gave us the money to do this program, but it's been created

independently with no input from Vertex and no input on the presentation at all.

In addition, it's vigorously vetted by the Johns Hopkins CME Department and the content has been peer reviewed to insure the program is free of commercial bias. needs to be done in that area.

So with that as an introduction, allow me to introduce Richard Moss who is a Professor of Pediatrics and directs the Cystic Fibrosis Center at Stanford, and he is going to talk to us about the pathophysiology of CF and some emerging targets.