# Ahead of the Curve – Emerging CF Therapies 2009: Managing Patient Expectations Patients' Case Scenarios

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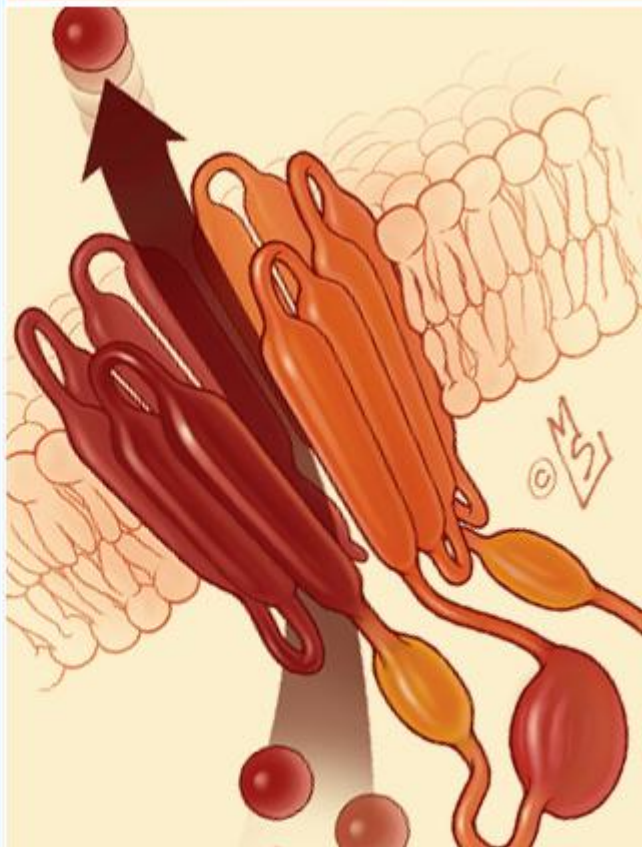

# Case 1

23 year old CF female with an $FEV_1$ of 55% of predicted. Sputum is culture positive for *Pseudomonas aeruginosa*. The patient qualifies for three different ongoing clinical trials that are open for enrollment at the site.

# Question Case 1

The patient is reluctant to enroll in a research study because she does not want to get the placebo. She thinks she will lose ground and have disease progression in the study.

THE CFTR MODULATORS

- The study agent is investigational and not FDA approved. What should the PI do?

THE MUCUS CLEARERS

- The study agent is FDA approved for another indication. What should the PI do?

# Question Case 1

THE CFTR Modulators

- The patient wants to be in a study and is eligible for three studies, one of which is under-enrolled. How should the PI present the clinical research at the site?

THE MUCUS CLEARERS

- The patient really wants to participate in the study but is on an excluded agent.  What should you do as a PI?

# Case 2

- A family meets with the CF team regarding their 2 ½ year old CF child.  He was diagnosed on new born screening and has no symptoms and a normal chest radiograph.  The FDA has just approved a novel agent for CF which improved CFTR function in adolescents and adults.

# Question Case 2

## THE CFTR MODULATORS

- The family requests that their child receive this novel treatment.  No further clinical trials of this therapy are planned.  What should the physician do?

## THE MUCUS CLEARERS

- A new study has started to enroll children age 2-14 years of age in an RCT studying this FDA approved agent. The family requests that the CF team treat the patient outside of the clinical trial.

# Question Case 2

FOR EVERYONE

The FDA approval is for a specific genotype (type 2). The family requests the drug despite having a different genotype (type 1). What should the physician do?