

Notes from the Kit Taylor Memorial Lecture April 24, 2007

- ❑ Speaker- Bonnie Ramsey from Seattle, Washington. Spends most of her time working on new treatments.
- ❑ Mutations tell us what is wrong with protein
- ❑ Developing treatments based on the most common mutations
- ❑ Two ways to develop new treatments- Drug Discovery and Drug Evaluation
- ❑ Drug Discovery is the process of looking for new drugs
- ❑ Drug Evaluation is the process of looking at existing drugs to see if they can benefit those living with CF
- ❑ One goal is to fix the bad CFTR protein. Two step process: get this protein moving correctly and make it open correctly
- ❑ The company that is doing this is Vertex in San Diego. They are able to test a magnitude of potential compounds. They have set a very enthusiastic timetable to achieve receive results. They have met them every time
- ❑ They start with testing millions of compounds...they are looking for hits during this process...hoping for 100's of results...they take these results and look for validating 10-20 of them...From here they look for 4-6 leads...Down to 2-4 developmental candidates
- ❑ **The first developmental candidate is in human trials!!!!**
- ❑ The next thing that they are looking at is Restoring the Ion and Chlorine transports
- ❑ Bronchitol is an Australian based drug that hopes to be approved by 2009-10
- ❑ This dry powder inhaler draws water out to improve hydration and prevent the mucus from becoming thick
- ❑ Denufosal is currently in phase 3 and enrolling participants.
- ❑ Another area they are looking at is Other Channel Transports...my interpretation of this is that there are a number of channel transports for the protein. We are looking at developing “detours” using the good channels.
- ❑ Aztreanam Lysinate reduces pseudanomas and increases FEV1. Use opposite Tobi. Looking for younger participants to test. Very promising. Goal is mid 2008.
- ❑ New Tobi Delivery system.
- ❑ Bigger Companies are starting to get involved.
- ❑ Especially in the area of drug evaluation...looking at existing drugs to see if there is a crossover.