



## Cystic Fibrosis

### What is it?

- ❖ A Genetic Disease that affects the lungs and pancreas

### How Does It Happen?

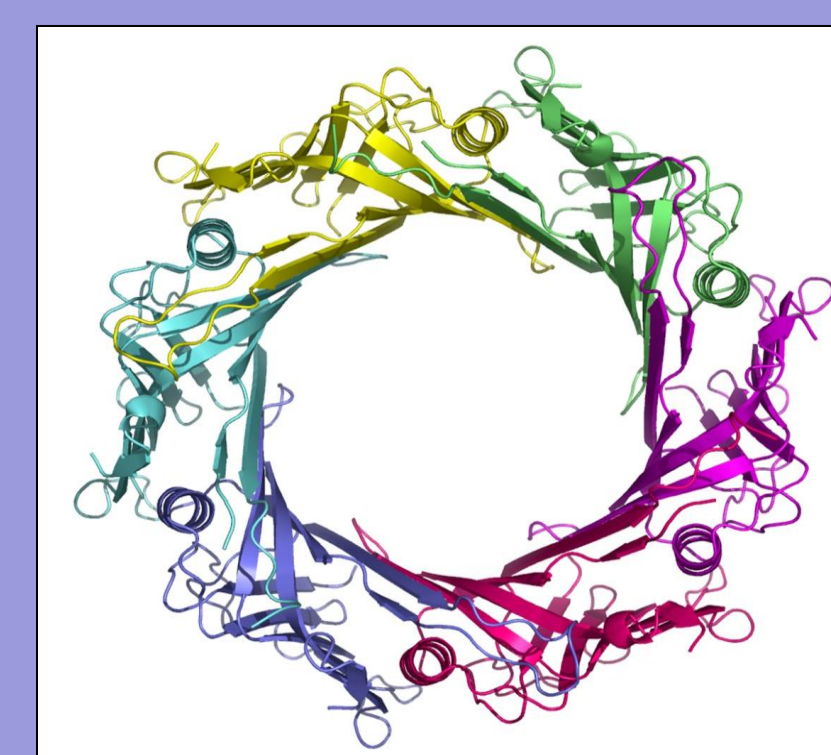
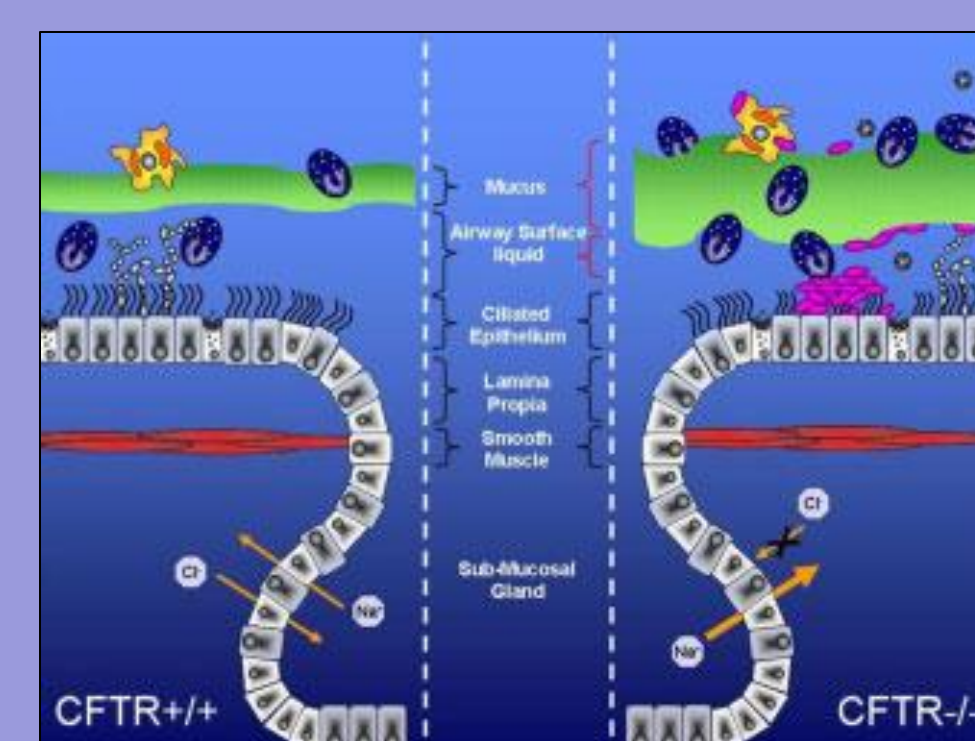
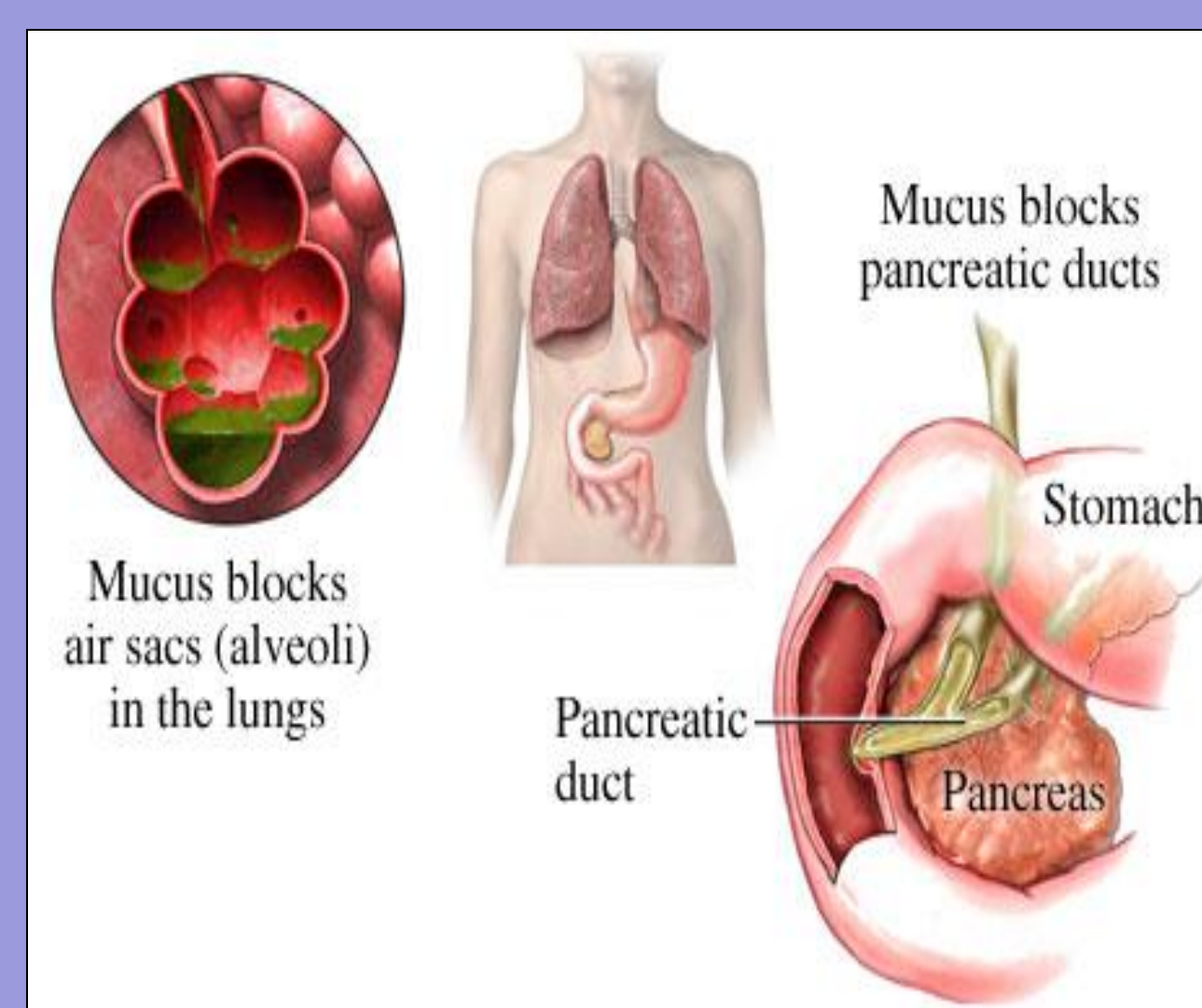
- ❖ A Gene (CFTR) on chromosome seven is mutated
- ❖ The CFTR protein is either mutated or not made at all
- ❖ Without the correct protein the Chloride level is unbalanced

### Why is it harmful?

- ❖ It causes thick and sticky mucus to build up
- ❖ The mucus in the lungs creates respiratory problems (difficulty breathing, bacterial infections, etc)
- ❖ The mucus in the pancreas creates digestive issues

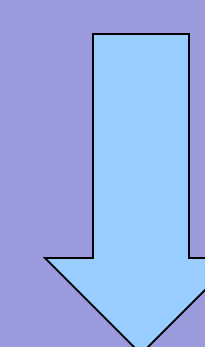
### Who Does It Affect?

- ❖ CF affects over 70,000 people worldwide
- ❖ Most common in people of Northern European descent
- ❖ It's affects can start from either infancy or adolescence

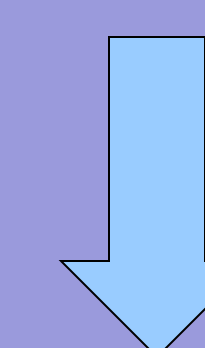


## Problem:

- ❖ Over 30,000 people suffer from Cystic Fibrosis in the U.S.

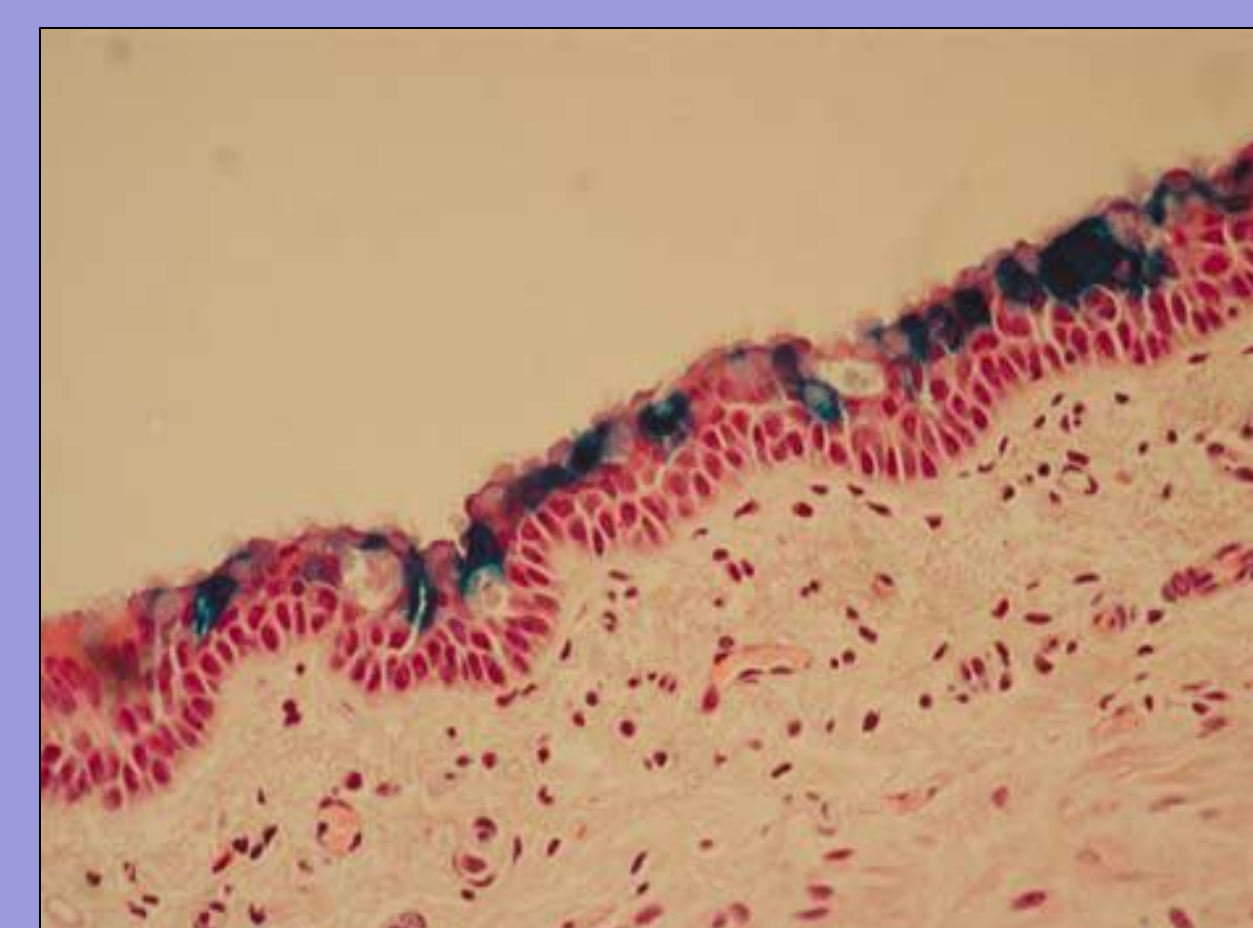


- ❖ Gene therapy is a potential cure.



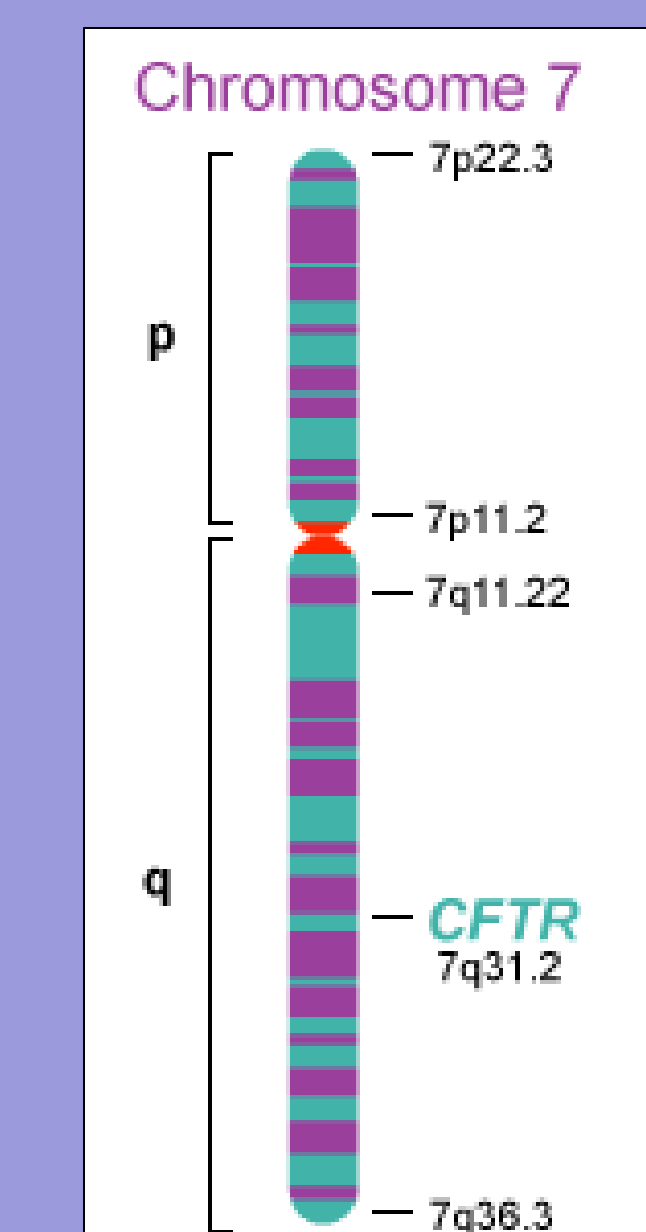
## Solution:

- ❖ Awareness can be spread by educating the population about the treatment of CF with gene therapy. This would in turn improve the success of the treatment and potentially lead to an official cure.



## Gene Therapy

- ❖ Experimental treatment correcting abnormal genes
- ❖ Not routine yet
  - With more testing can improve techniques
- ❖ Two types
  - Germ-line: reproductive cells
  - Somatic Gene Therapy: tissue or cellular level
- ❖ Used to treat diseases
- ❖ Normal gene replaces diseased gene
  - Integration of DNA
- ❖ Enter through vector
  - Retroviruses
    - Easily cloned
    - Best in actively dividing cells
    - Ex Vivo
  - Adenoviruses
    - Most common vector
    - Non-dividing cells
    - Less chance of infection of the patient
  - Non-viral
    - Liposomes
    - Raw recombinant DNA injection
- ❖ Difficulties
  - All diseased genes must be corrected
  - Possible threat of injecting viruses into body



## Gene Therapy in CF

Cystic Fibrosis is caused by a mutation in the CF Transmembrane Conductance Regulator (CFTR).

- ❖ Gene therapy is used to 'correct' the mutation in the CFTR gene by replacing it with a normal gene.

- ❖ Gene replacement during the neonatal period (while in the womb) would decrease morbidity and mortality.

- ❖ The role of the CFTR gene is not completely understood

- ❖ Clinical trials need to be done to see how efficient the gene must be.

### Vectors

- ❖ Viral vectors:
  - Utilizes adenoviruses and adeno-associated viruses ( AAV) to carry out gene replacement.
  - Used in *in vivo* gene delivery to the lungs
  - Had inflammatory responses due to large amounts of vectors.
- ❖ Non-viral vectors:
  - Cationic liposomes; positively charged cationic ends interact with DNA and lipophilic (lipid) ends interact in membrane transfer.
  - Do not have the inflammatory response of viral vectors
  - Have proven to be effective.

## Our Plan

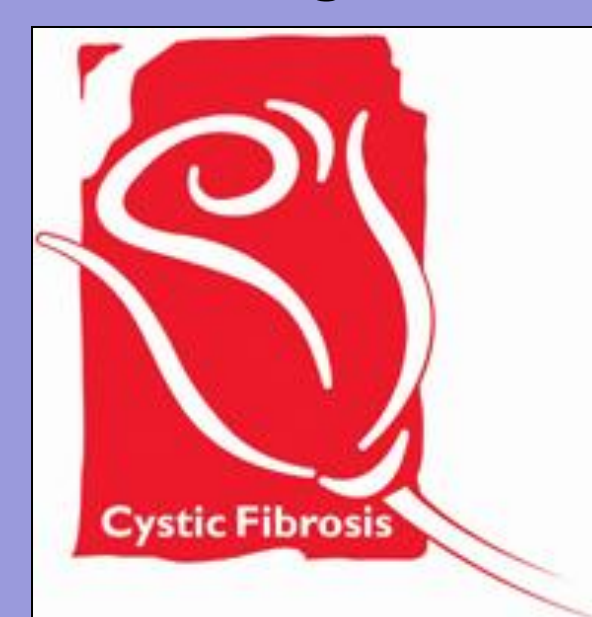
- ❖ Educational Approach

- ❖ Provide conferences to educate medical professionals about the treatment of Cystic Fibrosis with gene therapy

- Encourage further awareness of gene therapy by the medical professionals telling their patients
- Further experiment/test this technique to provide additional success

- Note that this is not an "official" cure and that with continued research and testing it could reach that point

- ❖ Assistance from Red Cross and The Cystic Fibrosis Foundation



- ❖ Conferences will be held at the top ten hospitals in the U.S.

- 1) Johns Hopkins Hospital: Baltimore, Maryland
- 2) Mayo Clinic: Rochester, Minnesota
- 3) Cleveland Clinic: Cleveland, Ohio
- 4) Massachusetts General Hospital: Boston, Massachusetts
- 5) University of California, Los Angeles Medical Center: Los Angeles, California
- 6) New York Presbyterian Hospital: New York, N.Y.
- 7) Duke University Medical Center: Durham, North Carolina
- 8) Barnes-Jewish Hospital: St. Louis, Missouri
- 9) University of California, San Francisco Medical Center: San Francisco, California
- 10) University of Washington Medical Center: Seattle, Washington

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