

# Cystic Fibrosis and Orkambi: This New Drug is Adding Tomorrows

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Zoë Mertz

At three days old,  
my little brother  
was diagnosed  
with a genetic  
disease called  
cystic fibrosis

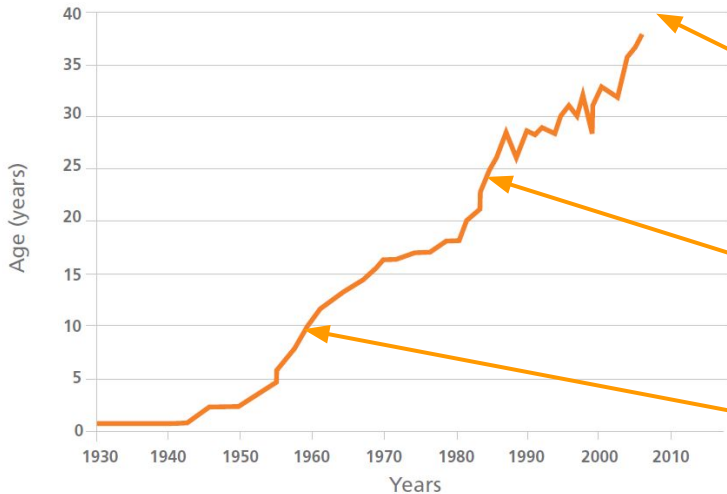


Circa 2001



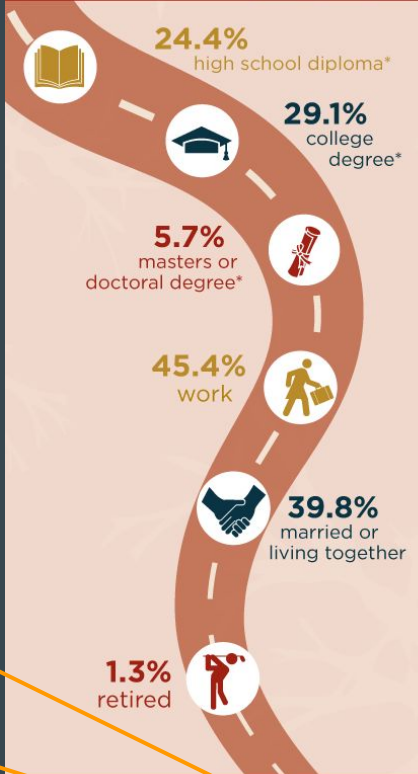
# Cystic fibrosis patients have a significantly shortened lifespan, though life expectancy is improving

## Cystic Fibrosis Life Expectancy



New Health Guide. Cystic Fibrosis Life Expectancy. Updated April, 29, 2015. Accessed at: <http://www.newhealthguide.org/Cystic-Fibrosis-Life-Expectancy.html> on 04.29.2015.

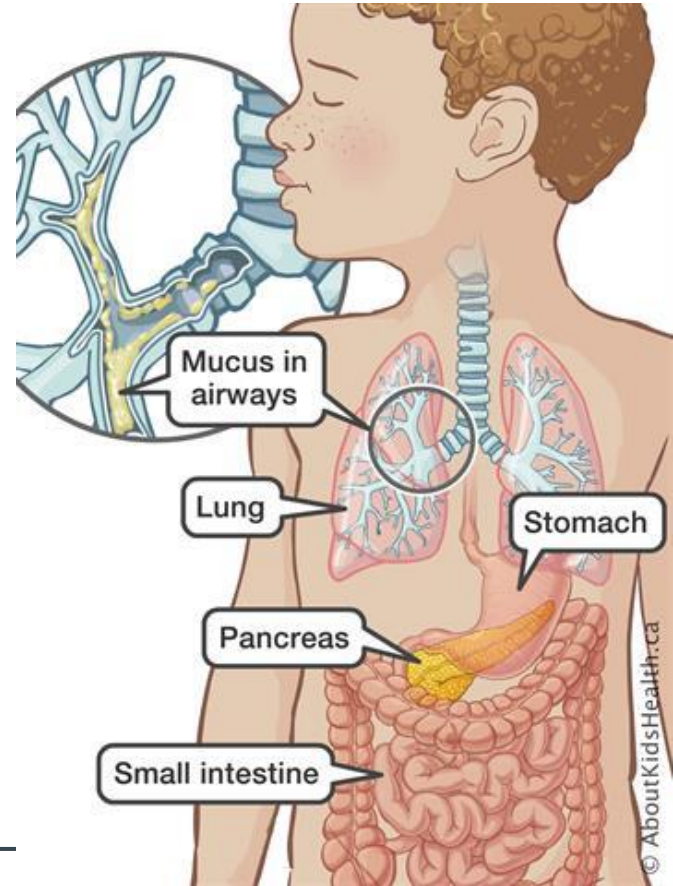
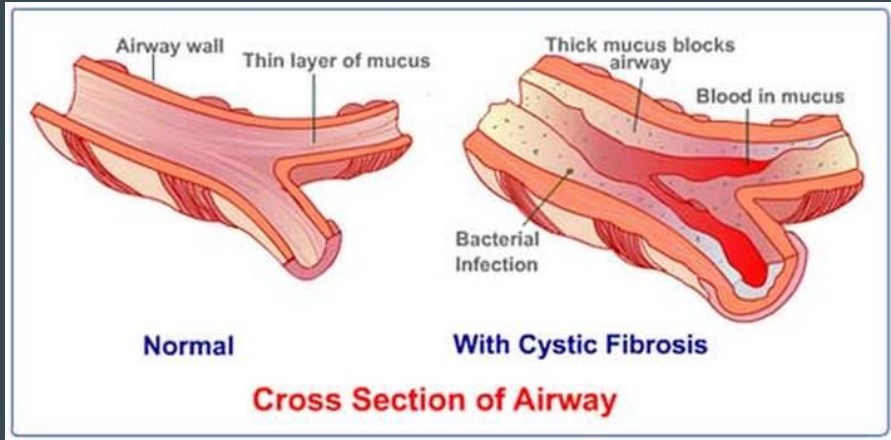
## ADULTS WITH CYSTIC FIBROSIS REACHING KEY LIFE MILESTONES



## PREDICTED MEDIAN SURVIVAL



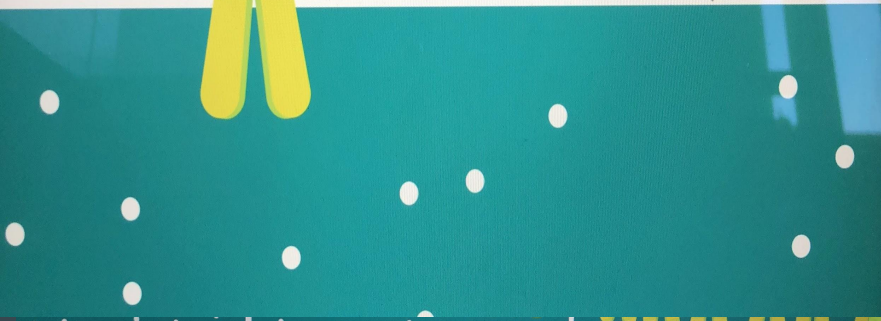
Cystic fibrosis can cause damage to the lungs, pancreas, and other organs due to a buildup of thick, sticky mucus



# The CF Delta F508 mutation has two main effects on operation of cells

Too few CFTR proteins reach the cell surface

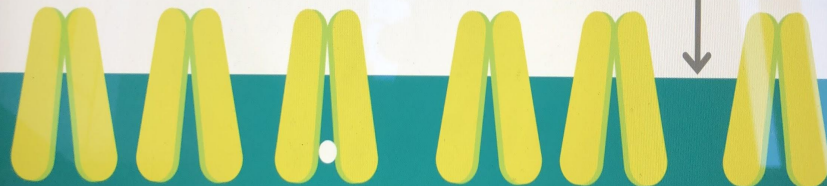
cell surface



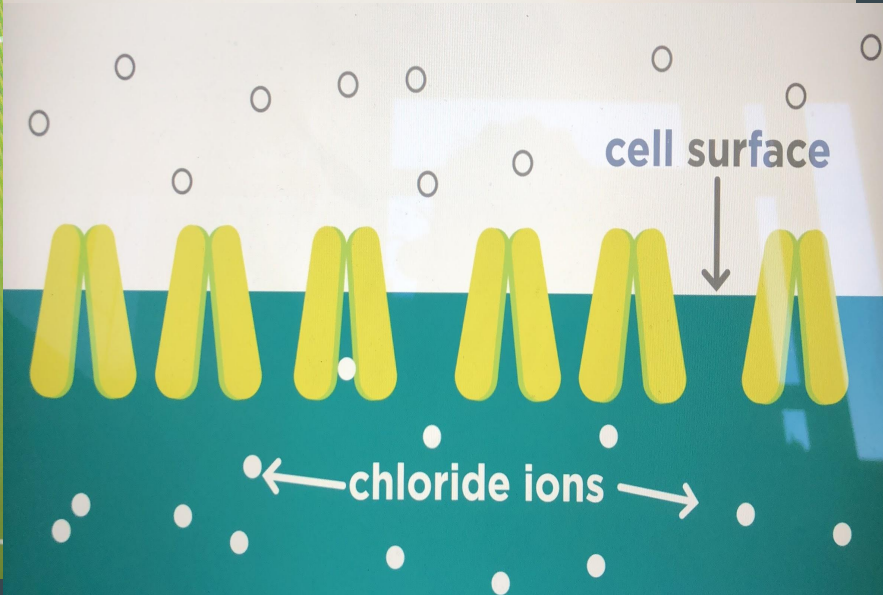
HOW MANY  
CFTR PROTEINS

PTS OF THE  
MUTATION

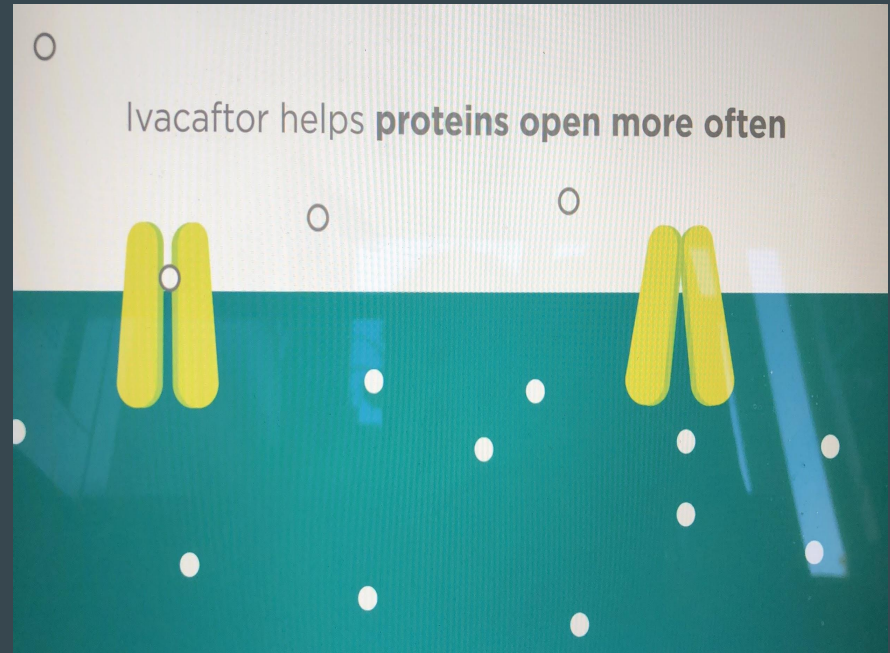
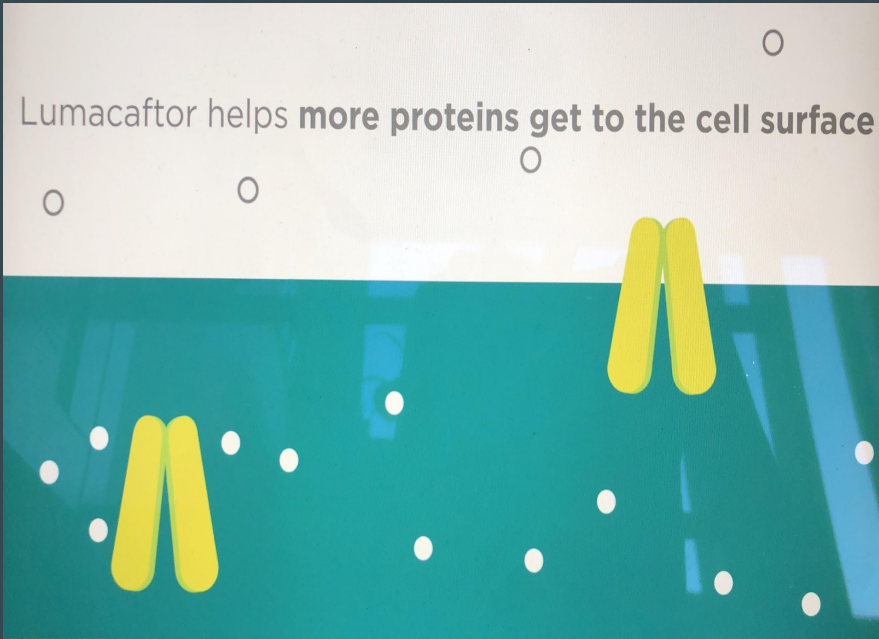
cell surface



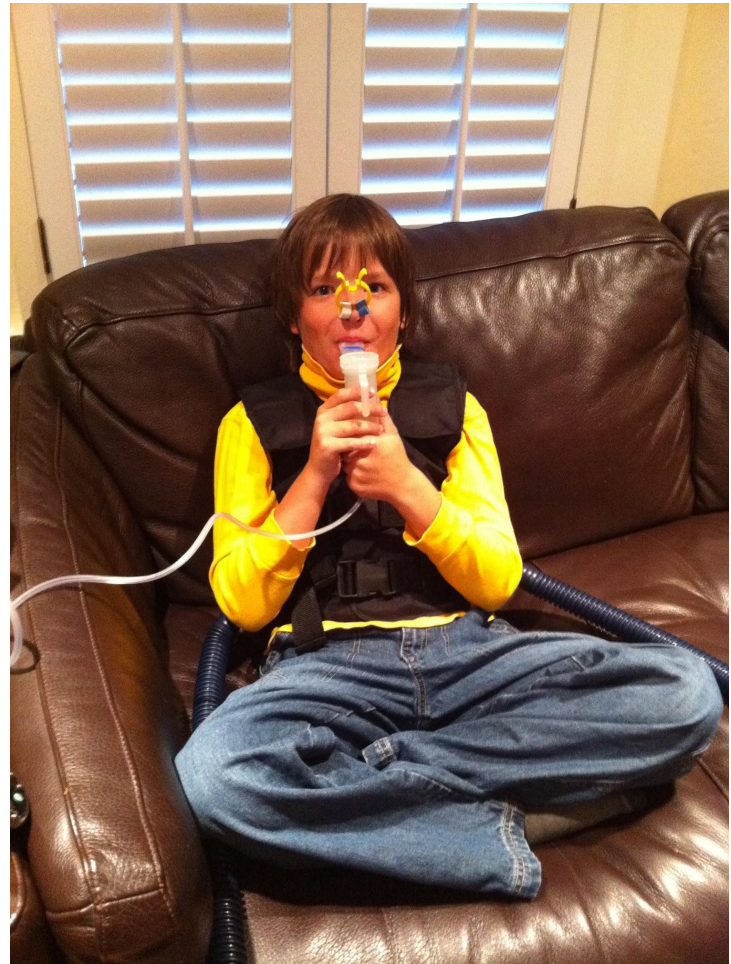
← chloride ions →



# Orkambi is a miraculous breakthrough for patients with the Delta F508 CF mutation – addressing the cause, not just the symptoms



# So What? Who Cares?



Circa 2010

# Bibliography

Cystic Fibrosis Foundation. (n.d.). Lumacaftor ivacaftor (Orkambi®). Retrieved September 27, 2018, from <https://www.cff.org/Trials/Pipeline/details/86/Lumacaftor-ivacaftor-Orkambi->

Cystic Fibrosis Foundation. (n.d.). About Cystic Fibrosis. Retrieved September 27, 2018, from <https://www.cff.org/What-is-CF/About-Cystic-Fibrosis/>

How ORKAMBI® (lumacaftor/ivacaftor) Works | Watch the Video. (n.d.). Retrieved October 7, 2018, from <https://www.orkambi.com/how-orkambi-works>



# Goals for Presentation:

- Speak slowly!
- Learn new things about science
- Try out / practice new presenting techniques

