CF for the Primary Care Provider: Current Prognosis, New Therapies, and the Importance of CF Center care
A Patient Story
Rapid changes in prognosis over a single lifetime

Legend:
- **TIP** = tobramycin inhaled powder
- **AZLI** = aztreonam nebs, aka Cayston
- **HTS** = 7% hypertonic saline nebs
- **rhDNase** = dornase alpha nebs, aka Pulmozyme

Figure courtesy of Elborn JS. Eur Respir Rev. 2013 Mar 1;22(127):3-5
Median Predicted Survival (US)

46 YEARS
2015 - 2019

Among people with CF born between 2015 and 2019, half are predicted to live to 46 years old or more. This does not reflect individual variability in survival seen among people with CF.

Survival statistics for the years 2015 through 2019.
No longer a disease primarily of children

In 2015 for the first time, the number of adults living with CF exceeded the number of children.

Increasing median life expectancy means shift to larger proportion of adults living with CF.
Current State 2022: “Highly Effective Modulators”

Trikafta (elexacaftor/tezacaftor/ivacaftor)

- 12+ and one+ delF508 mutation: 10/21/2019
- Expansion of mutations eligible: 12/21/2020
- Expanded down to age 6: 6/9/2021
- Trials now down to age 2 years

Video from HHMI BioInteractive
The other 10+

- Nasal cell theratyping to identify rare mutations that are eligible for Trikafta
- Antisense oligonucleotides targeting individual nonsense mutations using the NMD pathway
A very different patient story

Normal pancreatic function and false-negative CF newborn screen in a child born to a mother taking CFTR modulator therapy during pregnancy

Christopher N Fortner, Julie M Seguin, Denise M Kay

F. w/ CF (delF508/other); M partner delF508 carrier
Unable to conceive before HEM; elected to continue in pregnancy
? Echogenic bowel at wk 20, resolved at wk 32
F. infant b. 39 wks, wt > 98th %ile
NBS: IRT < cut off for testing (false negative)
Sweat chloride @ 5 weeks 60/67, 62/63 @ 6 months (barely positive)
Genetic testing (done due to hx) confirmed CF+ delF508/delF508 infant
Fecal elastase initially normal, progressing over time toward a need for enzymes
Excellent growth and weight gain in first year
Prospective Study of Pregnancy in Women With Cystic Fibrosis (MAYFLOWERS)

The safety and scientific validity of this study is the responsibility of the study sponsor and investigators. Listing a study does not mean it has been evaluated by the U.S. Federal Government. Know the risks and potential benefits of clinical studies and talk to your health care provider before participating. Read our disclaimer for details.

Sponsor:
Amaia Magaret

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