Northern Light Health

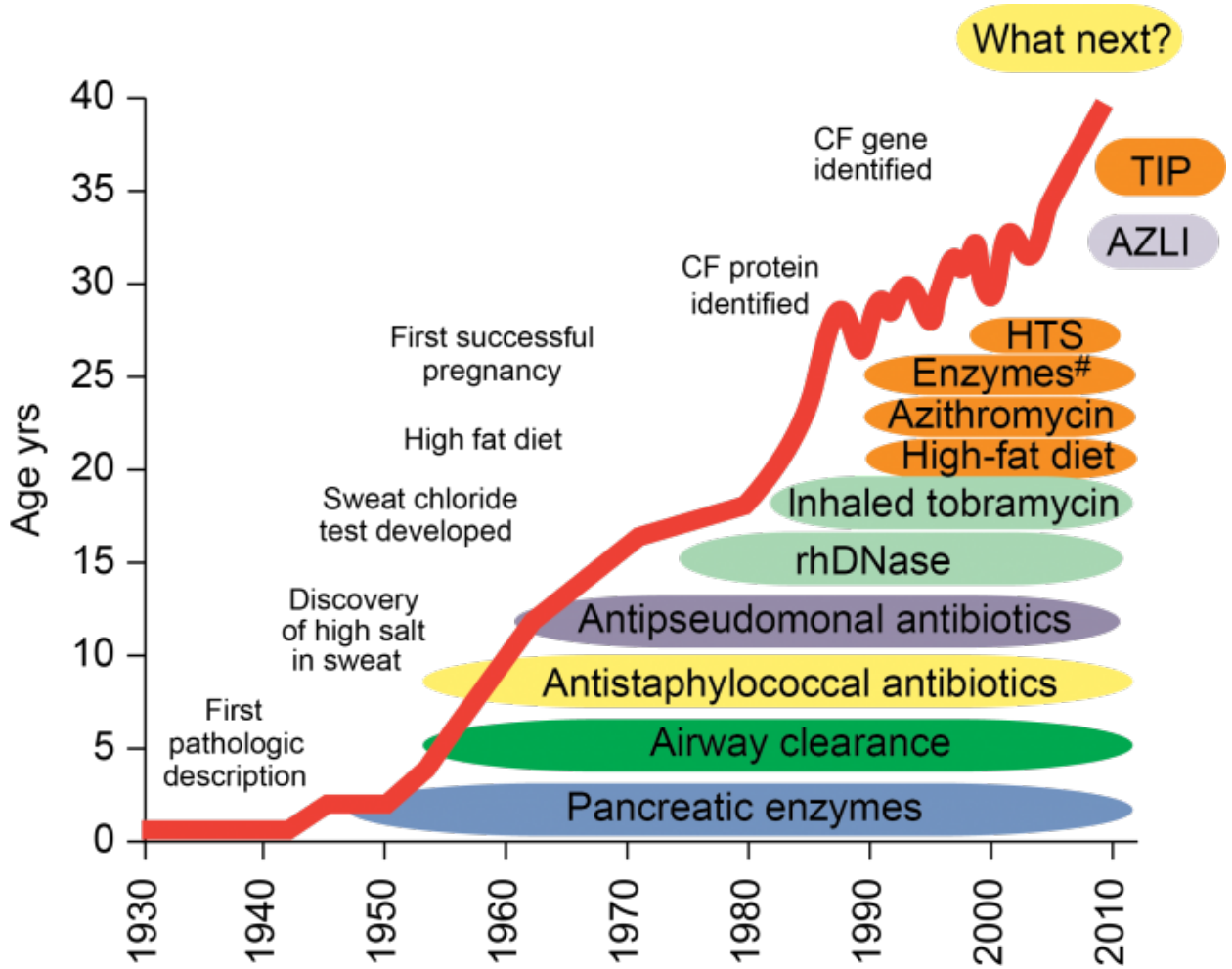
Innovations in Pediatric Medicine
Spring 2022 Maine AAP

CF for the Primary Care Provider: Current Prognosis, New Therapies, and the Importance of CF Center care

04.30.2022 Suchari Rutledge MD

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

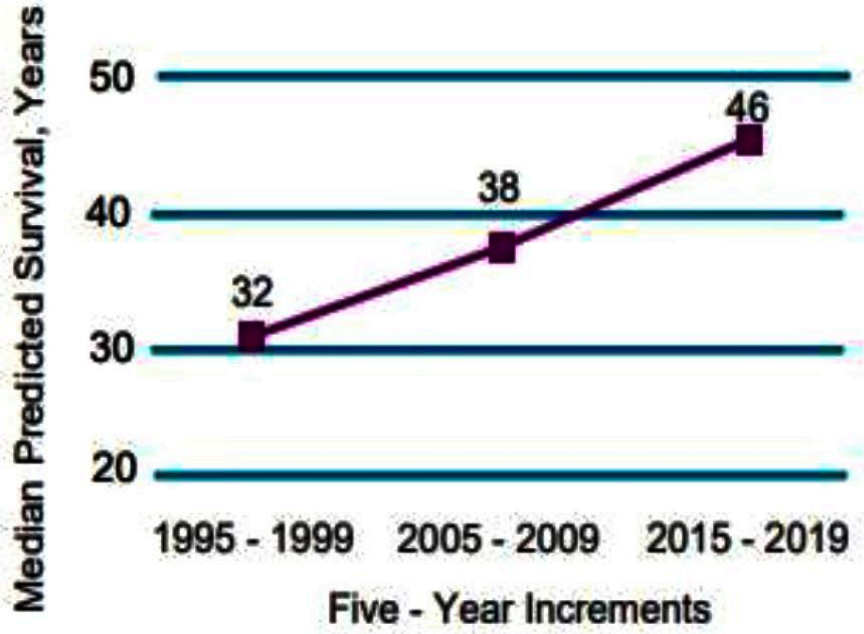
Figure courtesy of Elborn JS. Eur Respir Rev. 2013 Mar 1;22(127):3-5

Median Predicted Survival (US)

46
YEARS

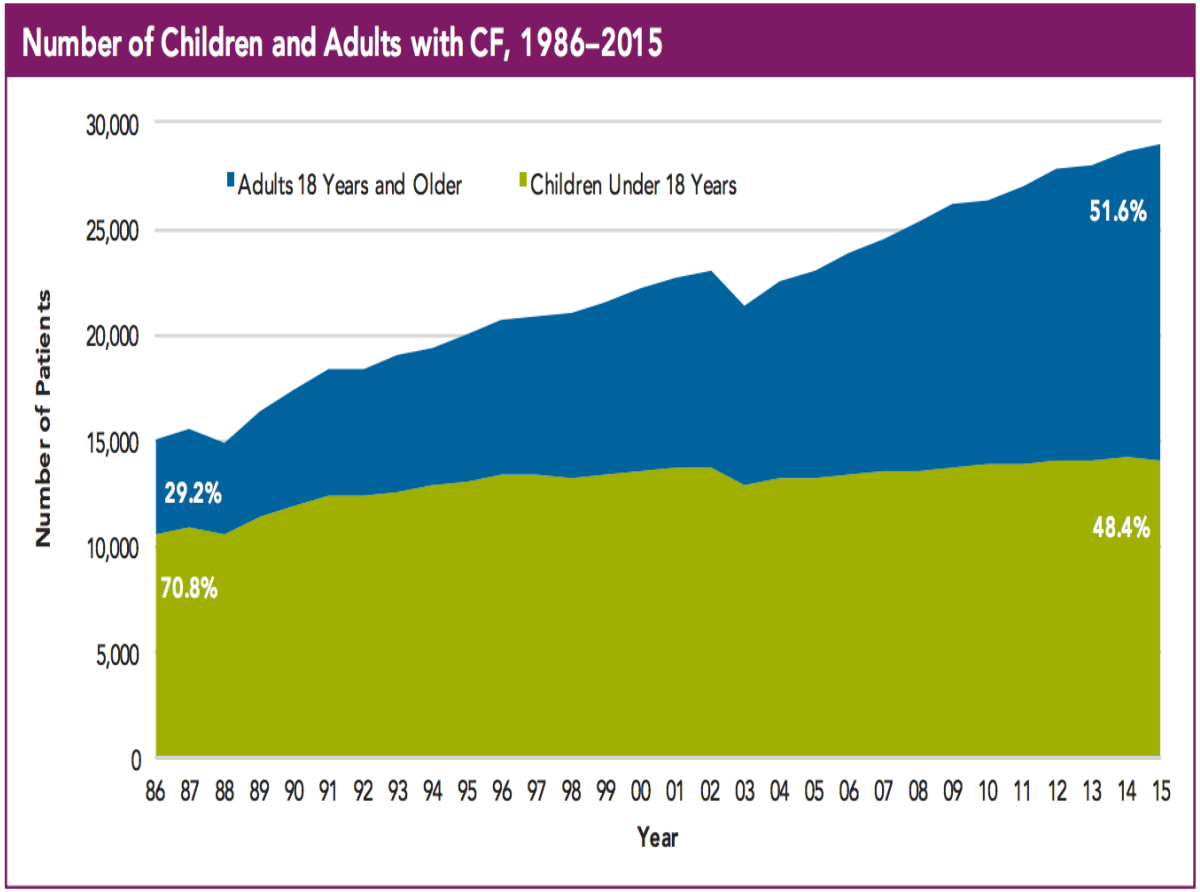
2015 - 2019

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

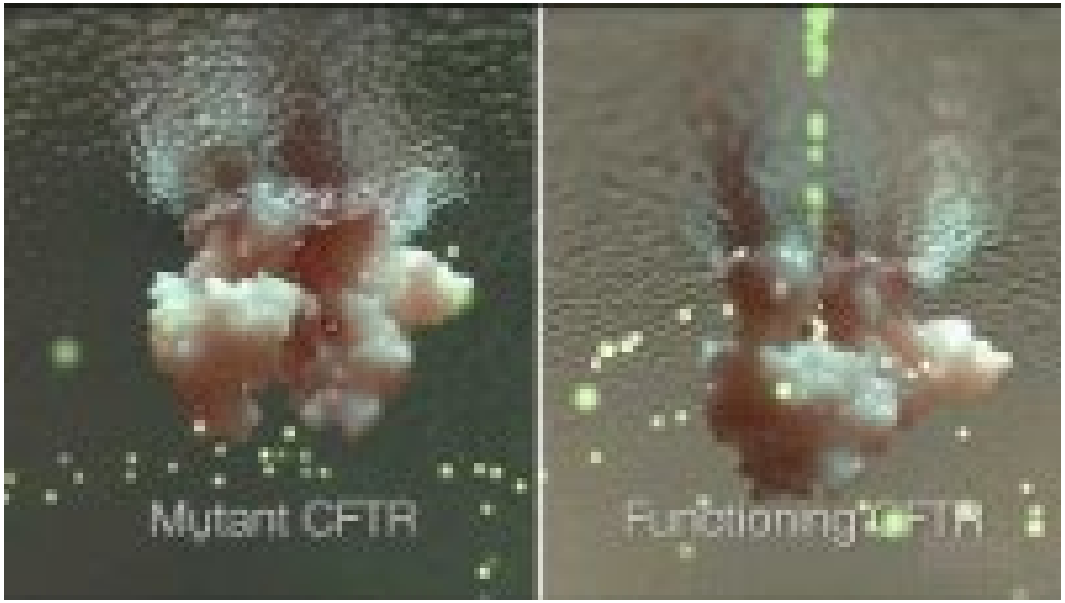


In 2015 for the first time, the number of adults living with CF exceeded the number of children.

Increasing median life expectancy means shift to larger proportion of adults living with CF

Current State 2022: “Highly Effective Modulators”

Trikafta (elexacaftor/tezacaftor/ivacaftor)



Video from HHMI BioInteractive

12+ and one+ delF508 mutation:

10/21/2019

Expansion of mutations eligible:

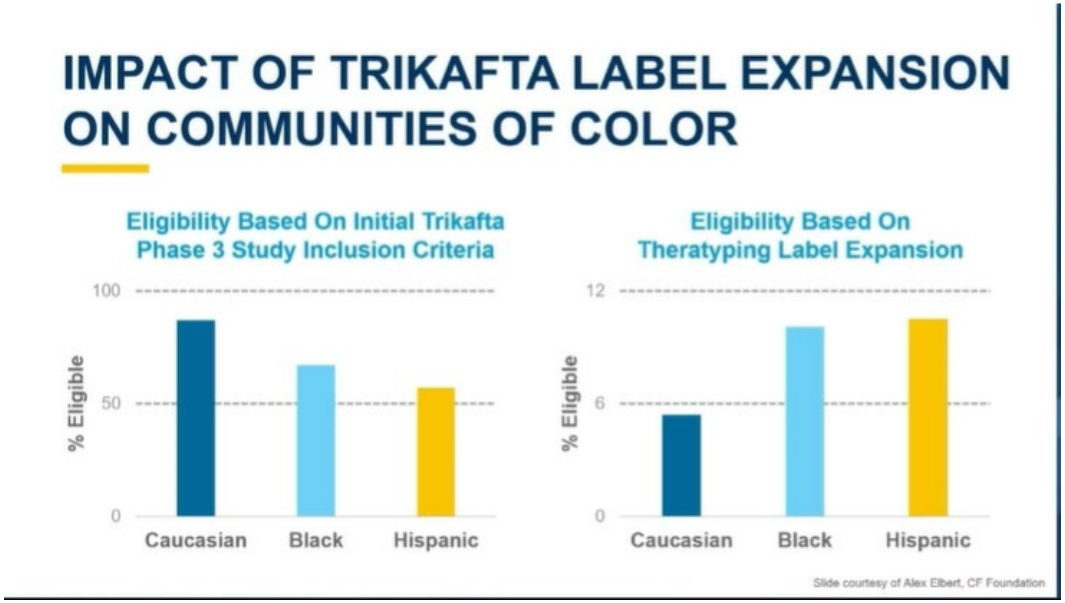
12/21/2020

Expanded down to age 6:

6/9/2021

Trials now down to age 2 years

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

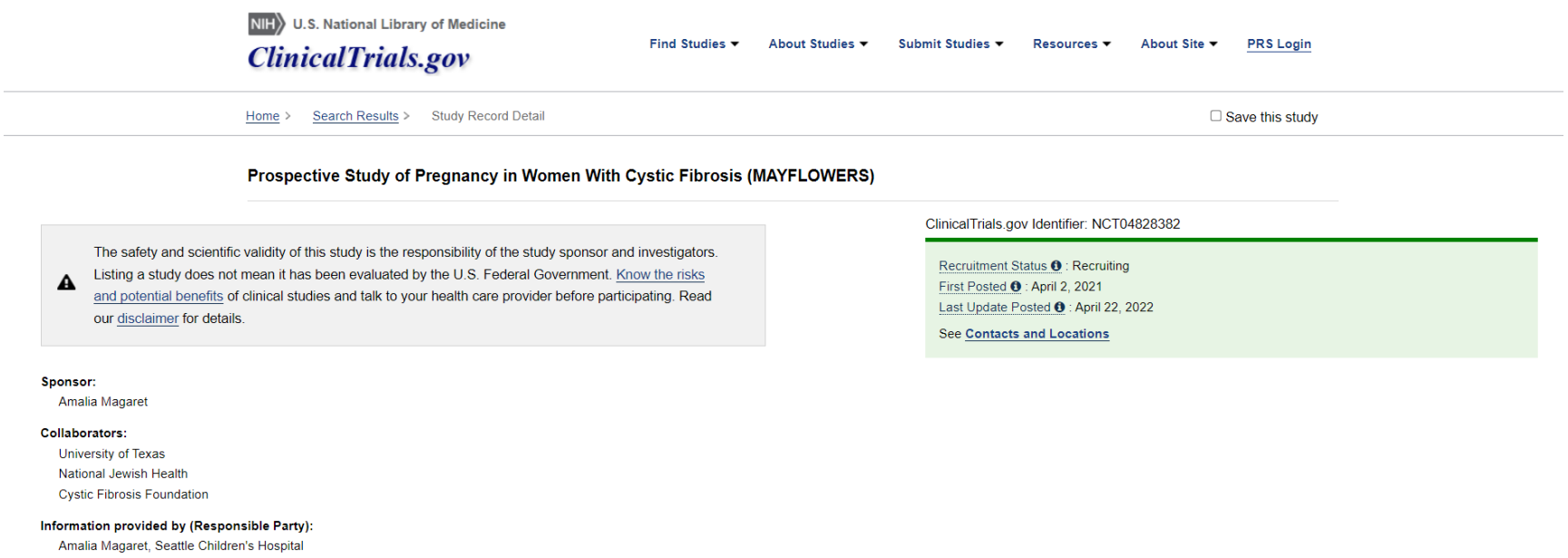
J Cyst Fibros. 2021 Sep;20(5):835-836.

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[1], Julie M Seguin[2], Denise M Kay[3]

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

MAYFLOWERS & ethics: how much is enough?



U.S. National Library of Medicine
ClinicalTrials.gov

Find Studies | About Studies | Submit Studies | Resources | About Site | PRS Login

Home > Search Results > Study Record Detail

Save this study

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.

ClinicalTrials.gov Identifier: NCT04828382

Recruitment Status : Recruiting
First Posted : April 2, 2021
Last Update Posted : April 22, 2022

See Contacts and Locations

Sponsor:
Amalia Magaret

Collaborators:
University of Texas
National Jewish Health
Cystic Fibrosis Foundation

Information provided by (Responsible Party):
Amalia Magaret, Seattle Children's Hospital

Maine CF Care Centers

MMC: 207 662 5522 option 8
EMMC: 207 275 4251