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In this presentation, we discuss how CF clinical trial enrollment, newborn screening, and global availability create inequities. We also discuss how policies and practice must advance so that every person with CF has a fair and just opportunity to attain their highest level of health.

Panel: Parenting with CF

Lucy Barnes; Matthew DeFina; Carl Robinson
Ashland, OR; Napa, CA; Danville, CA
Moderated by Mary Helmers, RN
Lucile Packard Children's Hospital Stanford, Palo Alto, CA


Lucy Barnes


Matthew DeFina


Carl Robinson

Improved treatments for cystic fibrosis – most notably CFTR modulators – and the resulting increased life expectancy have translated to larger numbers of individuals with CF becoming parents. Speakers on this engaging panel share their individual paths to parenthood, including adoption, in vitro fertilization (IVF), and pregnancy / childbirth. Moderated by Mary Helmers, who has counseled many of her patients with CF about available options as they weigh the decision to become parents, the discussion explores panelists' range of experiences related to reproductive health discussions with their CF care providers; their partners' key roles; finding balance with parenting, working and managing CF; the impact of pregnancy and / or parenthood upon health maintenance; and addressing psychosocial care needs.

Advances in mRNA Therapy: New Applications for Cystic Fibrosis

Deepika Polineni, MD, MPH
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Deepika Polineni

It is estimated that up to 10% of people with cystic fibrosis (CF) in the United States are unable to benefit from currently FDA-approved CFTR modulator therapies due to their CFTR mutations (i.e., genotype) or a history of side effects to such treatments. Research is underway for alternative strategies to improve the health of people with CF who do not benefit from CFTR modulators. Messenger ribonucleic acid (mRNA) therapy is one such treatment option that is under investigation for people with CF and has often recently been included under the term "genetic therapies" for CF. Importantly, mRNA therapy is distinct from gene therapy and gene editing. mRNA is a type of ribonucleic acid that is present in human cells and represents one step in the process of the DNA genetic code becoming translated into a functional protein. Using mRNA replacement therapy as a treatment in CF involves the careful delivery of mRNA coding for CFTR into airway cells to use the cells' own machinery to create normal CFTR protein in the lungs. The success of this depends on many factors including the stable maintenance and delivery of the therapy.

Recently, mRNA was utilized successfully in development of mRNA-based SARS CoV-2 vaccines for COVID-19 during the pandemic. Lessons learned from these vaccines and the pandemic will continue to inform investigations of mRNA in therapeutic development and use. mRNA therapy is now under early phases of study for safety and tolerability in people with CF who cannot benefit from CFTR modulators based on their CFTR mutations. mRNA could also have broader future applications for people with CF independent of their CFTR mutations. In summary, mRNA therapy in CF is a new treatment under investigation that could have potential to improve lung disease for people with CF irrespective of their CFTR genotype. This presentation provides a review of mRNA as a novel therapeutic option.

Embracing the Future: Aging with CF

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Ahmet Uluer

Cystic fibrosis (CF) is a multisystem disorder primarily affecting the respiratory and digestive systems. Advancements in treatment, including highly effective modulators, have significantly improved quality of life for individuals with CF.

The incidence of cancer involving the GI tract occurs at higher rates for people with CF than the general population, particularly those receiving organ transplant. Regular screening and surveillance are essential to detect and not only manage but even prevent malignancies. Individuals with CF are at an increased risk of developing diabetes and over 35% have this listed as a diagnosis. Monitoring for elevated blood pressure also important, especially for those with diabetes. Furthermore, people with CF are also at risk of hearing loss and kidney disease associated with life-saving treatments. Monitoring and screening measures are important to prevent and manage both kidney disease and hearing loss. If necessary, interventions involving hearing aids and cochlear implant can impact quality of life. Cardiovascular disease is also emerging as a concern among adults with CF. Regular cardiovascular screening, including monitoring of lipid profiles and assessment of cardiac function, is necessary to identify and manage cardiovascular complications promptly. Early identification and aggressive management are essential to mitigate their impact on overall health and well-being.

As the adult CF population continues to grow, it is imperative to recognize the complications associated with aging in individuals with CF. Screening, early treatment, and understanding of effective management strategies are essential to improve the quality of life for adults living with CF.