

CF and Colon Cancer: My Lived Experience

Anna Payne
Langhorne, PA



Anna Payne

Anna Payne is a 36-year-old cystic fibrosis and colon cancer patient. She is also the founder of the Bucks County CF Alliance, a non-profit organization. In her talk, she describes her experience with CF and living with stage-4 colon cancer. She was diagnosed at the age of 34, well before the recommended screening age of 40. Those with cystic fibrosis have 5 to 10 times the rate of colon cancer as the general public: those post transplant have over 25 times the risk. After her diagnosis, Anna immediately focused her advocacy efforts on educating as many people as possible about the elevated risk of developing colon cancer experienced by CF patients and carriers. She details her story, from diagnosis until today; what she has learned and what we can do to help make sure this doesn't happen to others.

Presenting alongside Dr. Ahmet Uluer, Anna shares the personal side of this two-part medical story: the real-life impact that colon cancer has on people and what it looks like living with two diseases.

Advances in Stem Cell Research for the Treatment of CF

Brigitte Gomperts, MD
University of California, Los Angeles, CA



Brigitte Gomperts

This is a very exciting time in CF research. The first clinical trial of nebulized CFTR mRNA airway delivery has been completed and several more clinical trials are in the pipeline. While we await the results

of these trials, research is moving forward with even more advanced gene therapy approaches. But central to understanding how gene addition or gene editing will work

for CF is knowing which cells in the airway should be targeted. In this presentation, we share our recent findings using single cell RNA sequencing of the multitude of different cell types and subtypes in the human airway and their function. We also discuss how these cell subtypes change in CF. We focus in on which cell types express CFTR and examine the different airway stem cells. Airway stem cells are of particular interest because gene editing of these cells could potentially lead to long-lived correction of CFTR.

We review the current approaches to replace, repair or restore the CFTR gene in the airway, which include mRNA delivery, gene delivery, gene editing, and cell therapy approaches. Cell therapy approaches are in their infancy, and we explore the different kinds of stem cells that could potentially be used and the pros and cons of each stem cell type. There are several barriers to airway delivery of these gene therapeutic approaches which are even more challenging in CF, such as thick, tenacious mucus and inflammation. There are also barriers to systemic gene delivery and gene editing in the body. Cell therapy approaches have their own delivery issues and the additional hurdle of engraftment in the airway. We discuss each of these barriers and potential ways to overcome these obstacles with advances in delivery systems. Overall, we show major progress in the field of gene therapy for CF that is being made through cutting edge research in this area and provides hope that this could provide a therapeutic approach for all CF patients.

Normalizing the Abnormal

Alanah Rosenbloom, MSW
San Jose, CA



Alanah Rosenbloom

Navigating a life with CF is tough... to say the least! Having CF can feel weird, gross, and unpredictable, especially when all you yearn for is to be normal and in control. It has taken Alanah decades to gain

some perspective on the struggle that is living with CF, and while she doesn't necessarily feel normal or in control, she does feel incredibly empowered by the experience

thus far. After living with CF for 37 years, she is ready to share some of her stories.

Join Alanah as she talks about: the funny, the bad, and the ugly; befriending others with CF; using CF to be of service to others. Whether you have CF or not, Alanah hopes this talk generates hope that what once brought shame can one day become cherished.

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