Apr 23rd, 2:15 PM - 3:45 PM

Cystic Fibrosis in Context: A Look into Patient Advocacy, Race, Genetics, and Big Pharma

Kayla Sloan
Providence College

Kayla Sloan

Sarah Wilcox

Health Policy/Activism

2 May 2020

Cystic Fibrosis in Context: A Look Into Patient Advocacy, Race, Genetics, and Big Pharma

I. Introduction

I was diagnosed with cystic fibrosis about ten years ago, which is one of the most common life threatening genetic diseases with about 70,000 people having CF across the world. CF causes the production of heavy mucus that leads to pulmonary and pancreatic problems and involves dozens of daily treatments and medications. Through my experience as a white person with a disease where most patients are European, I have always wanted to complete more concrete research into what the implications of CF being considered a "white" disease are. Growing up I saw the way that the time my mom had to get involved with the CF Foundation and the connections she had through work with others with time and money helped with our fundraising efforts. It is the time, money, and connections we have that stem from my family's racial and economic privilege that has helped with our advocacy efforts and the organizing of thousands of others in our community. This was the impetus in wanting to look at the struggles and inequities in patient advocacy and how race can compound these obstacles to patients having their voices heard.

The neoliberal approach to the American healthcare system puts in place a priority to act based on financial incentives instead of real life struggle and experience. This neglect to listen to

people's personal experiences makes it more difficult for patient advocates to have their voices heard over those of pharmaceutical executives (Panofsky 2011). The struggle for advocates to be heard by anyone making decisions that will affect their lives are further compounded by systemic racism that inhibits people's ability to be heard by scientists, scholars, doctors, and policy makers. This racial component is seen most clearly through the struggles of people with sickle cell disease, which many consider to be a "black" disease (Wailoo 2006). Sickle cell disease is another one of the most common life threatening genetic disorders and causes patients to experience severe pain due to the abnormal type of hemoglobin in their red blood cells (What is Sickle Cell Disease; Grob 2008). The hemoglobin causes their blood cells to sometimes become sickled and reduces the blood cells ability to pass through small blood vessels, which cuts off blood from body tissue and causes pain. This disease constituency has experienced obstacles with trust in the medical system, struggles with access to time and money, and dangerous myths about the community lacking agency and being "badly behaved" (Benjamin 2013). On the other hand, the CF community being mostly white people gives more of an opportunity for patients to get involved with advocacy and meet all their medical needs (Wailoo 2006). At the same time, this generalization about the CF community having all white patients creates an even smaller space for the voices of people of color that have CF. In order for disease advocacy to flourish, scientists need to focus on the voices of patients in the context of societal issues, which means scientists need to seek out voices and not just acknowledge the ones coming to them.

In this paper, I am going to provide an understanding of patient advocacy organizations with a focus on the comparison between cystic fibrosis and sickle cell disease to highlight these broader themes. I will start by looking at the way that patient advocacy organizations and scientist-patient relationships are built. After getting an idea of this advocacy infrastructure, I am

going to bring in information about how the racial composition of genetic diseases creates further complications with the process of advocacy. Then I am going to look at how stem cell technologies and treatments highlight how scientists and scholars take control of research and neglect patient voices, and discuss the way this issue is worsened for sickle cell patients when it comes to understanding the opposition to stem cell transplants. Next, I will analyze pharmaceutical industries and look at the way they operate under neoliberalism to reinforce advocacy inequities and create problems with access to drugs and treatments.

II. Patient Advocacy

The infrastructure of patient advocacy and scientist-patient relationships set the groundwork for health mobilization success. With more recent opportunities to create digital infrastructure, the way that patient advocacy has worked is forever changed, but the deep seated inequities on racial and economic lines still exist in how disease advocacy operates. Despite these digital changes, there are still disparities between patient and scientist influence or academic expertise vs. lived expertise that play a role in the difficulties of health advocacy (Epstein 2016; Panofsky 2011). Even questions of mobilizing the grassroots vs. letting corporate industry advantage take over to funnel more money into research and care for a particular disease are more pertinent than ever given the increasing power of corporations and the pharmaceutical industry in the American political system (Panofsky 2011). To understand the infrastructure of patient advocacy organizations, it is important to look at how PAO's work, the history of health mobilization, and the future of patient organizing in a digital world.

PAO's are a critical part of health mobilization that operate as the middle man between a grassroots patient base and a bureaucratic organization run by scientists and research experts

(Panofsky 2011). PAO's can range anywhere from "mom and pop" groups started by a parent who realizes their child's rare disease has no organization to a PAO running for the most common disease in America. PAO's also give disease communities the opportunity to create larger PAOs that act as networks across other similar diseases. For example, the cystic fibrosis community is a part of the rare disease PAO to combine forces with other diseases that have less than 200,000 patients worldwide (Panofsky 2011). The rare disease PAO is then able to fight for policies like the Orphan Drug Laws that incentivize scientists to look for drug and treatment developments, despite the low pool of possible consumers in their individual disease constituencies. PAOs can operate as more casual organizations helping patients make personal connections that allow for sociability that motivate researchers to listen to patient priorities of what should be researched (Panofsky 2011). PAOs can also work as more strict and regulated groups that are seen as a stop on the way to getting a research proposal approved. The difficulty in this kind of advocacy is that scientists often do not take people with lived experience of the disease seriously if they don't have the expertise about the science of the disease and the process of doing research (Esptein 2016). This lack of trust from scientists leads many advocates to feel the need to become lay experts to impress researchers into considering their ideas and opinions. Both types of systems can work if done right, but each are examples of how important it is to have an organization actively working with patients so that research can be done with priority given to those with lived experience.

Organizations mobilizing for health changes throughout history usually focus on ideas about organizing for a particular disease or for universal healthcare for all (Hoffman 2003). Organizations like Act Up have realized the ways that goals of having guaranteed healthcare, reduced drug prices, lowered deductibles and copays, and freedom to see any provider can

overlap between the AIDS disease constituency and the rest of the health policy movement. This act of spreading out the goals of the organization is typically not how disease constituencies work, because they worry that making their goals too general would undermine the specificity of certain policies and funding they need for their specific disease community. These debates over immediate change for a disease constituency vs. more universal improvements become one of the most difficult parts of patient organizing. This struggle also proves the way that patients feel the responsibility to do everything themselves because there are not scientists and policy makers actively listening to them. If scholars and politicians actually implement the more specific community demands of these groups, advocates would have more time to organize for universal needs outside of their disease constituency.

Ever since the Cystic Fibrosis Foundation was established, scientific experts had control as funding came through corporate structures, but with that funding has come improved leadership and educational opportunities and more patient involvement in the future of advocacy and development (Panofsky 2011; Marshall 2009). The use of these corporate funds to create patient involvement has also recently combined with digital social networks that have increased CF patient advocacy. Part of what makes CF organizing difficult compared to other medical conditions is the threat of cross-contamination that prohibits people with CF from being within six feet of each other (Marshall 2009). With the rise in digital opportunity, the ability to organize remotely or just talk with each other has increased exponentially through websites like Cystic Life where people can create online communities and post life updates. The foundation has also started virtual programs like Tomorrow's Leaders that use conference calls to train young people on how to be CF advocates. The Cystic Fibrosis Foundation has also used a public policy program to demand basic healthcare reforms and raise awareness of the difficulties of being

underinsured that has included teen and adult advocacy on the part of those with CF in Capitol Hill. Through the corporate funding of CFF they have also created the Cystic Fibrosis Patient Association to help cover insurance costs for families struggling to pay medical bills (Marshall 2009). The use of corporate funding to incorporate patients into the actions of the organization shows that while corporate structure can create issues with patient voices, it can also be a way to make the money necessary for patient and professional integration.

With these advances in the digital world, it has become much easier for patients to connect with each others online and create social cohesion that "break down 'information monopolies'" (Landzelius and Dumit 2006:593). Breast cancer patients and loved ones have also had a lot of success with online self-help communities by creating relationships with others who understand what they are going through. The communities also have records with research and information, which includes a glossary of breast cancer terms. These kinds of educational sources change the information landscape, allowing patients to swap information with each other rather than solely getting it from doctors who many patients commiserate about not trusting. Online patient run groups give people the opportunity to be each other's advocates and support system without the struggle of being shut out from conversations with research experts or politicians.

A lot of issues about improving patient advocacy are not frequently discussed, especially the question of how to expand access to advocacy for more disadvantaged patients. For example, one of the main strategies to create sociability and personal connection among patients and scientists with the help of PAOs is conferences and formal dinners one patient called "elbow to elbows" (Panofsky 2011). It is unrealistic that someone would be able to attend one of these events that likely involves flying on a plane if they do not even have enough money to pay the

expensive medical bills for themselves or a loved one. This can go for conferences and fancy dinners, but it can also go for raising money through events and asking friends and family for money when you don't have a higher income social group to ask. Even social online groups are often not used by people who are low income because they need access to a computer and many of the people involved in these groups will take trips to meet each other that would be difficult to do if you were struggling financially and had time constraints (Landzelius and Dumit 2006). The issues relating to race and socioeconomic status make it clear that equal opportunity in disease advocacy directly connects to ideas of racial inequity in health mobilization.

III. Race and Genetic Diseases

Cystic fibrosis is known as a "white disease", while sickle cell disease is seen as a "black disease", and while the histories and demographics of these two diseases do prove these associations to be true, the narrative of these two conditions has gone a lot farther than the concrete biological facts (Wailoo 2006). Cystic fibrosis is known as a disease from Europe that possibly started due to a genetic resistance to the European cholera outbreak, while sickle cell disease has been tagged as starting as a genetic resistance to malaria in Africa. Genetic diseases are inherently connected in some way to ideas of race and ethnicity, which complicate the already difficult process of patient advocacy (Wailoo 2001; Wailoo 2006). Not only does race impact the way that all of these elements of advocacy operate, but it also creates changes throughout the history of organizational advocacy in how they strategize to push for access, research, and awareness for their constituents. For diseases constituencies that are seen as marginalized based on race, there is the additional aspect of systemic exclusion from income,

healthcare, and expectations for fair medical practices. That systemic exclusion is why it is integral to improving advocacy to look at the impact of societal disparities.

The politics of race that have been impacting research and legislation for both sickle cell anemia and cystic fibrosis for decades have shifted to connect with different historical moments in America. Growing up with cystic fibrosis, I always associated the disease with something white people have due to its connection to Europe. I would even go into my CF clinic knowing to avoid other people with CF due to cross-contamination risks and thought of the CF give away as a white person with a mask on. Despite the truth that the majority of CF patients are white, CF is a more panethnic disease then it is given credit for, largely due to the pivot in the 1990's that CF was a "Cacuasian disease" to stir up momentum for venture investment research (Wailoo 2006). This strategy worked because venture investment saw a group of white disease constituents as more likely to have the money to fund this drug research, but also because CF's white image gave it the reputation of being the disease of the "majority of America". This perception of the CF community created a better justification for the researchers in why they were doing drug experimentation for a rare disease. This idea that CF represented the population of the "majority of America" also created a lack of racial group identity or solidarity for CF because it wiped over any kind of ethnic or racial diversity within the CF community. Tay-Sachs disease being seen as a Ashkenazi Jewish disease and sickle cell being seen as an African American disease creates a sense of racial/ethnic connection that CF lacks (Wailoo 2006; Burgess 2019).

In the 1970's when America was in the midst of the black power movement and had just experienced the civil rights movement, researchers framed CF as a panethnic disease to better connect its advocacy efforts to the current moment (Wailoo 2001; Wailoo 2006). Sickle cell

anemia has always been seen as a “black” disease and as a result it has been seen as representative of the pain and suffering black people have experienced in America. This connection has led to more awareness and advocacy in the hopes of rewriting wrongs of racism in America, especially during the late 60’s and 70s . Even Richard Nixon passed legislation in hopes to improve conditions for people with sickle cell disease and end the neglect of the pain of black America. Unfortunately, sickle cell’s association as a “black disease” has also created conflict in providing patients with pain management drugs when they are having what is called a pain “crisis” because of the cultural idea of black people being more likely to get addicted to narcotics scaring scientists and physicians (Wailoo 2006).

One of the other critical aspects of disparity between cystic fibrosis and sickle cell anemia is the way they bring in money and attention for critical research. Cystic fibrosis has been described as a “privileged disease” because “there were an estimated 1,155 new cases of [sickle cell] disease [and] 1,206 of cystic fibrosis...[y]et volunteer organizations raise $1.9 million for cystic fibrosis...,but less than $100,000 for sickle cell anemia” (Wailoo 2006:63). The fundraising that has been done for sickle cell anemia, which is a specific type of sickle cell disease, comes in large part from groups connected to the black community due to the group solidarity aspect of the disease (Wailoo 2001). For example, a sickle cell center in Memphis Tennessee run by Lemuel Diggs received funding from a local black sorority and the NIH. Ebony also used its appeal as a magazine targeting a mostly black audience to help sickle cell gain awareness by covering stories of the families that are battling the disease everyday. Not only did this bring recognition to the real faces behind the disease, but the article touched on environmental triggers to disease flare ups and discussed the economic and political component of living with an expensive disease, while being part of a marginalized racial community.

Despite the way black solidarity can increase fundraising for sickle cell, it does not overperform the tens of thousands of CF advocates that are more likely to have the time and money to participate in fundraising due to the demographic makeup of the disease community.

A lot of the way that sickle cell research is framed is concerned with a disproportionate amount of people of color struggling financially and socially due to systemic racism. This kind of concern has caused fear that risky treatments framed as cures like bone marrow transplants are going to be picked by families who fear the economic consequences of raising a child with sickle cell for years to come (Wailoo 2006). The truth of bone marrow transplants is that parents with lots of degrees are more likely to choose this risky procedure and even if the patient survives, there is a high probability that they will develop other expensive conditions. Despite the research not backing up this claim, the idea still permeates the school of thought for sickle cell. This kind of worry leads many researchers to not want to target members of the sickle cell community for more long-term cures and instead focus on more incremental changes that can make life more manageable for people with sickle cell. CF patients are different in that they are more likely to be focused on for experimental drug treatments because venture markets see value in a privileged constituency who represents a washed out wide swath of American society (Wailoo 2006).

The other difficulties of life with sickle cell are endless because of a myriad of racial inequities embedded in our culture and political institutions. One of these obstacles is the overall lack of trust many black Americans feel towards the medical system due to the country's history of medical discrimination most blatantly seen in the Tuskegee experiments (Wailoo 2006). This medical mistrust also carries into forms of control of birth that have been used in the Ashkenazi Jewish community to reduce the birth of babies with Tay-Sachs because women of color in America have a long history of white people controlling their reproduction (Wailoo 2006;

Burgess 2019). There was even a researcher at one point who suggested that people should get tattoos if they carry sickle cell so that they do not fall in love with another sickle cell carrier and have a child with the disease. There is also the direct link between lack of familial income and Medicare coverage that impacts families of color disproportionately and is part of what causes the fundraising disparities in advocacy with organizations like the CF Foundation (Wailoo 2006). Sickle cell is a disease that is most known for sudden increases in pain "crises" which often involve hospitalizations. Hospital treatments for crises only serve to improve symptoms and still cost $30,000 to $50,000 a year. Complications with staying employed and getting coverage from work while raising a child in and out of the hospital create a cyclical effect that complicates life with a chronic illness under systemic racial inequality (Wailoo 2006).

Solving the crisis of racial inequity in treatment and research in sickle cell does not have clear cut and easy solutions. Similar to how issues like asthma in low income communities need to be solved with social programs that get to the root of racial disparities, sickle cell needs to be improved with structural social policy change to alleviate the economic and social stressors of racism (Brown 2003). If issues like medical access and higher economic stability amongst people of color were achieved, patients with sickle cell would have families more financially able to pay for their medications and hospital stays. Issues like expanding Medicare access and lowering drug costs are already more well known and have people rallying behind them. Even if these policies passed without awareness of sickle cell, they would be helping black Americans live with more financial comfort that would be helping people with sickle cell. While this increased financial stability could increase the ability for sickle cell families to get involved in more advocacy, that does not mean scientists and politicians would all of a sudden start listening to the community. Issues relating to bias of white scientists and politicians and the overall lack of trust

in institutions from the black community would still cause a lack of consideration of the needs of the sickle cell community.

IV. Genetic Testing, Technology, and Treatment

Scientists owning the narratives surrounding genetic testing and technology has created a lack of acknowledgement of social disparities creating distrust over genetic treatment for sickle cell patients and little awareness of the complexities of newborn screening for genetic diseases like CF (Benjamin 2013; Grob 2008). The risk of these scientific narratives stem from the disconnect they have with patients and families that have personal experience with the disease. This lack of patient understanding creates false justifications for why people are skeptical or unsupportive of using new kinds of genetic testing and technology. The two aforementioned recent advances in genetic testing and treatment, have lots of advantages seen through the help they have been able to do for people struggling with genetic diseases, but there is much more to these treatments than guaranteed medical help. A lot of the complexities of balancing the ethics of these new treatments comes from a need to work with a spectrum of parents experiencing these problems without using research as an opportunity for scientists and scholars to take further control of the medical narrative.

Newborn screening has grown out of the prenatal screening that began about thirty years ago and by the late 2000's newborn screening had become so commonplace that only three states even asked for consent to complete the test (Grob 2008). Newborn screening was initially used to test for diseases like phenylketonuria that were threats to newborn's health, but had universal treatment options. After these screenings expanded in use, the genetic diseases tested for grew with it, which means that diseases like CF that are usually not putting infants in danger and often

do not have visible symptoms for weeks, months, or years are being tested for. Unfortunately, scientists and scholars did not take the struggles newborn parents who have a baby that just tested positive for a genetic disease into account (Grobb 2008).

Since a disease like CF can be asymptomatic for newborns, the difference between "sickness" and "disease" becomes very clear as parents see a healthy baby, but know that their child has a genetic disease. One mother in Grob's study recalls looking at her baby and wanting to fall in love with them, but being worried to do so because she didn't know how long her baby would be alive. Meanwhile, parents whose children get diagnosed with CF later on, can go through the normal bonding development process with their child before getting the diagnosis so that it doesn't change the core of their relationship. Especially for first time parents, this experience can be anxiety inducing as they try to parce what is normal for a baby vs. a possible dangerous CF symptom. This anxiety can lead to an over reliance on doctors that expand the medicalization process of their baby and could jeopardize the patient-doctor relationship if the doctor feels annoyed and overwhelmed by the parents' constant messages (Grob 2008).

The CF parents in Grob's survey do understand the need to have the newborn screening as an option, but said that personally they would have wanted to be able to wait for symptoms before diagnosis. Many parents surveyed did wait to get their children tested in the future since they had the genetic potential to have cystic fibrosis. Since I was diagnosed with CF at nine years old, I have thought about how different my life would be if I was born a couple of years after 2001 and received an automatic newborn screening. While it might have been better for me to live my whole life knowing I had CF instead of having to process that shift in my consciousness and identity later, my parents have mentioned that on their end it would have probably been more stressful for the reasons mentioned earlier about the struggles of being a parent of a newborn

with CF. My experience matched some of the survey participants' in that though stressful there was a sense of relief in my diagnosis given that my parents had not been taken seriously when concerned about my CF symptoms in the past. What this wide spectrum of experiences show from CF parents is that families need to be included in the discussion over the rules and legislation about newborn testing. Currently scientists only listen to a small group of active advocates whose newborns have or could have been saved by newborn screening, and scientists should seek out other voices touched by the issue in addition to hearing these important experiences (Grob 2008).

There has also been a lack of sickle cell voices in explaining the reason that many patients and families chose not to do stem cell transplants, which creates a roadblock in working to help the sickle cell community going forward. After seeing the disparity between African American patients and Asian American patients in receiving experimental stem cell research, scientists came up with false justifications for these disparities by saying people of color lack agency and the ability to comply with doctor's orders (Benjamin 2013). Research on this topic has continued to fail to analyze the way that "social processes" create these disparities in the way that "distrust is socially produced in the everyday experiences of patient families in and outside of the clinic" (Benjamin 2013:2). In a similar way to how information on the disproportionate levels of health crisis like COVID-19 in the black community can be misinterpreted as people of color not being careful and hygenic, sickle cell research can wrongly place blame on people of color for not having "agency" (Chowkwanyan and Reed 2020).

Many sickle cell patients not attending all appointments and using every medication is a multifaceted issue connecting with possible struggles to pay for the medication and finding time to pick it up, but it also relates to a societal distrust of medical professionals through horrors like

the Tuskegee experiment (Benjamin 2013). One participant interviewed in Benjamin's article talks about not wanting her daughter to use an inhaler because she is unaware of the outcomes and possible side effects it could have and has seen other sickle cell patients get over prescribed medications that worsen their condition. Considering why these racial disparities within these experimental stem cell treatments exist for sickle cell families can be like "sweeping up broken glass while the... flames in their lives are left to wreak havoc" (Benjamin 2013:7). In order to be a sickle cell advocate who is trying to expand stem cell research, families would need the time, money, and connections to answer questions like this and get scientists to listen to them, which is a rare combination of factors for someone in this situation.

When it comes to raising funds and grants to expand stem cell research, there is also a large disconnect between the sickle cell community and the people involved in politics, pharmaceuticals, and biotech companies (Benjamin 2013). Sickle cell research has often been used politically in conjunction with the fight for racial justice, but these political rallying cries have not always brought forward the voices of the actual patients fighting this disease. When California tried to pass a state stem cell research proposition it did not even listen to the Sickle Cell Disease Foundation and received backlash for being a ploy to help large biotech companies. The other large flaw in these pushes for more stem cell funding is that none of these groups are looking to solve the societal issues that are causing most sickle cell patients to stay away from stem cell transplants (Benjamin 2013). This means that when care does look to more long term solutions, it fails to fix the societal problems relating to race that are more structural and would help people of color as whole (Wailoo 2006). These societal changes would improve sickle cell patient's ability to advocate for their interests and work with their doctors, but instead politicians

are pushing for more of a treatment that will likely be rejected anyway by a large sector of the sickle cell community.

V. Big Pharma

The pharmaceutical industry has been known to prioritize their own profits over the needs and interests of disease communities, but this focus on profits has also created inequities in drug development between the cystic fibrosis and sickle cell constituencies. Even though corporate profits have been tied to medicine since the 1940's, the era of neoliberalism that started in the 70's has taken the private pharmaceutical industries to a different level (Hemphill 2010; Baker 2016). Since the 1970's, the patents have intensified by being held for longer amounts of time and the international laws and regulation surrounding patents and their copyrights has extended to international waters to match the globalization of the era (Baker 2016). As patents have become stronger, productivity has gotten weaker, total factor productivity rate decreasing from 1.2% in the 70's to under 1% in the 2000's.

Neoliberalism has also been embedded into big pharma through the orphan drug system in order to have drug development for small disease constituencies fit the industry's need to make a profit. Policy like the 1984 Orphan Drug Act and tax credit incentives for big pharma to develop drugs for small disease groups are the only way that these companies will feel they have a reason to prioritize these groups (Hemphill 2010). Pharmaceutical companies have even caused disease inflation where they expand what it means to be sick so that drugs that are meant for a targeted audience can be expanded to a wider pool to increase the profits of the private pharmaceutical industries. In order for disease constituencies to work around this disadvantage in having a smaller demographic for drug developments that don't match the incentive model for

private pharmaceutical companies, they have to get creative in how they network with big pharma.

The CF Foundation has had more support throughout history with the pharmaceutical industry than most other disease constituencies, choosing to ally with private industry groups through their venture pharmaceutical program with Vertex. This partnership creates a dynamic where a nonprofit is working with a pharmaceutical company to bring in profit on both sides that is meant to go back into research (Cohen and Raftery 2014)). CF advocacy has also benefited from wealthy individuals like Joe O'Donnell who has a personal connection with CF and has put on high price fundraising events to bring in money for the organization. This dynamic is also at play in less extreme ways given the higher percentage of white people that have CF and the realities of racial inequities in America and their impact on socioeconomic divisions. Because of this economic advantage in industry and grassroots financial growth, the CF Foundation has succeeded in having lots of research and drug trials and approvals compared to conditions like SCD (Farooq 2020).

As a result of this venture pharmaceutical model, the CF Foundation has had success with multiple drugs whose effects would have been unheard of fifteen years ago. Trials started in 2011 for ivacaftor which is the first drug in the CF community to address the underlying causes of the CFTR protein malfunction compared to treating the symptoms of CF like Pulmozyme or antibiotics like TOBI that were approved by the FDA in the 1990's (CF Foundation). Ivacaftor/Kalydeco, was then approved by the FDA in 2014 for people in the CF population that have one out of the eight different mutations, which represented about 5% of the CF population (Cohen and Raftery 2014). Then in 2015, Kalydeco was approved for people with one or two F508del mutations, which accounted for 90% of the CF Foundation (Johnson 2019). Since then,

more trials have been done to try to expand the drug to the rest of the CF community and to expand the drug from single therapy to the double and triple combination treatments called Symdeko and Trikafta (CF Foundation).

It is also important to mention that the 10% of the CF population that are not covered by any of these groundbreaking drugs are disproportionately minority members of the community that are already statistically likely to have lower life expectancies than white people with CF (Johnson 2019). The drugs also cost $311,000 a year and even though the patent ivacaftor is on for five years after being released and the passage of the orphan drug laws helped the drug get created, there has not been much change to accommodate the price beyond insurance coverage patients can hope to have through their employers (Johnson 2019; Cohen and Raftery 2014). This venture philanthropy model that the Cystic Fibrosis Foundation took up has brought all of these new drugs that are having drastic impacts on patients' health, but questions around access to the drugs and the patient's voice in a process based in corporate profits still exist. Since the success of the CF venture pharmaceutical model in developing new drugs, many other disease constituencies have developed similar plans, but the disparity in resources and trust in the medical establishment that the sickle cell community has makes these kinds of private alliances more difficult. (Farooq 2020).

VI. Conclusion

Throughout American history, patients have struggled to have their voices heard by scientists and politicians, always feeling like they are "pitted against each other" for care (Hoffman 2003:82). The way that research has worked has been more based on pharmaceutical and biotech incentives then what patients have been saying should be researched and prioritized.

Researchers and politicians have also failed to take systemic inequalities into account that are integral to success in getting advocates' voices across (Benjamin 2013). Patients and families should not be begging for scraps with scientists and policy makers to have their voices heard, and these "begging sessions" shouldn't require a three hundred dollar plane ticket to be able to happen in the first place (Panofsky 2011).

The people that need to act to rewrite this wrong are politicians who need to both listen to patients and act to pass structural societal change so that racial inequities stop creating obstacles to advocacy. It is still important to acknowledge that passing racial justice policy would still not be enough to end this advocacy divide due to the racial prejudice through unconscious bias impacting how willing people are to listen and act on the interests of people of color. The other large issue when it comes to racial inequities in medicine is creating more trust in the medical profession among the black community, which like the last issue, is not going to be fixed with one clean and easy solution. One option to fix this predicament, is to put value and support behind leaders who are trusted in communities of color that can encourage others to develop more trust in the system (Godoy 2020). Another important aspect of advocacy inequity to consider is the fact that if people with more privilege have more opportunities to get involved, the young people around them are more likely to have first hand experience seeing someone do this kind of work. Giving this same kind of education in schools may allow people who don't know anyone doing disease advocacy to see what it means and how important it is to our healthcare system.

Patients have been working hard for decades to have their voices heard for improvements on issues that impact their lives and those of others, whether they have a disease that affects ten other people or ten million (Panofsky 2011). These people are working for their own futures and

deserve better than getting neglected for gains in pharmaceutical revenue. Despite these difficulties, it does look like there is hope for disease constituencies going forward. One source of hope comes from the new opportunities for patients and families to use technology for activism and social connection that allow patients to "break down 'information monopolies'" and make their voices heard (Landzelius and Dumit 2006:593). With issues like racial justice and the inept nature of the American healthcare system coming to a boiling point in 2020 with protests over police brutality and the dangerous holes in our healthcare system being highlighted by COVID-19, it looks like a perfect storm for changing the neglect of patients and especially patients of color. It looks like 2021 could bring change and scientists and politicians could work with patients to regain trust by being a part of that change. In order for scientists and politicians to step up and actually help patients, they can't just listen to the people who can fly out to D.C. for conferences and political action days, but need to seek out voices that may have never been heard before.

References

Baker, D. (2016). "The Old Technology and Inequality Scam: The Story of Patents and Copyrights" from *Rigged: How Globalization and the Rules of the Modern Economy Were Structured to Make the Rich Richer*: Center for Economic and Policy Research, 77-129.

Barnes, R. (2002). Patient advocacy and cystic fibrosis: political correctness or a real force for good?. *Journal of the Royal Society of Medicine*, 95 (Suppl 41), 52-57, https://slc.on.worldcat.org/oclc/8138044931.

Benjamin, R. (2013). Races For Cures. In *People's Science: Bodies and Rights on the Stem Cell Frontier* (pp. 113-134). Stanford University Press. https://slc.on.worldcat.org/oclc/852759287.

Brown, P. et al. (2003). The Health Politics of Asthma: Environmental Justice and Collective Illness Experience in the United States. *Social Science and Medicine*, 57(3), 453-464, https://my.slc.edu/ICS/icsfs/brown_health_politics_of_asthma.pdf?target=fc79e4d0-787f-4be1-ba06-60611f9a40a9.

Burgess, P et al. (2019). Michigan Medicine. University of Michigan, https://www.uofmhealth.org/health-library/tv7879?.

CF Foundation Venture Philanthropy Model. *Cystic Fibrosis Foundation*, https://www.cff.org/About-Us/About-the-Cystic-Fibrosis-Foundation/CF-Foundation-Venture-Philanthropy-Model/.

Chowkwanyn, M. and Reed, A. (2020). Perspective: Racial Health Disparities and COVID-19-Caution and Context. *New England Journal of Medicine*, 383, 201-203, https://my.slc.edu/ICS/icsfs/Chowkwanyun_Racial_Health_Disparities_and_Covid-19.pdf?target=166070fc-3ba9-45b5-8418-b4f626b58dcf.

Cohen, D. and Raftery J. (2014). Paying twice: questions over high cost of cystic fibrosis drug developed with charitable funding. BMJ: British Medical Journal, 348, 1-4, https://www.jstor.org/stable/2651391.

Epstein, S. (2016). The politics of health mobilization in the United States: the promise and pitfalls of "disease constituencies." *Social Science & Medicine*, 165, 246–254, https://slc.on.worldcat.org/oclc/6798056772.

Farooq, F. et al. (2020). Comparison of US Federal and Foundation Funding and Research for Sickle Cell Disease and Cystic Fibrosis and Factors Associated with Research Productivity. *JAMA Network*, 1-12, https://jamanetwork.com/journals/jamanetworkopen/fullarticle/2763606.

Godoy, M. (2020). Biden Advisory Board Co-Chair Says Addressing Health Disparities Will Be A Key Focus. *NPR*, https://www.npr.org/2020/11/13/934718160/biden-advisory-board-co-chair-says-addressing-health-disparities-will-be-a-key-f.

Grob, R. (2008). Is my Sick Child Healthy? Is my Healthy Child Sick? Changing Parental Experiences of Cystic Fibrosis in the Age of Expanded Newborn Screening. *Social Science & Medicine*, 67(7), 1056-1064, https://slc.on.worldcat.org/oclc/5903248997.

Hemphill, T. (2010). Extraordinary Pricing of Orphan Drugs: Is It a Socially Responsible Strategy for the U.S. Pharmaceutical Industry? *Journal of Business Ethics*, 94(2), 225-242, https://www.jstor.org/stable/40784598.

Hoffman, B. (2003). Health Care Reform and Social Movements in the United States. *American Journal of Public Health*, 93(1), 75-85, https://my.slc.edu/ICS/icsfs/Hoffman_Health_Care_Reform_and_Social_Movements_in.pdf?target=20308181-9a91-4a1e-a7a1-09fe7b01f2ad**.**

Johnson, C. (2019). Long-awaited cystic fibrosis drug could turn deadly disease into a manageable condition. *Washington Post*, https://www.washingtonpost.com/health/2019/10/31/long-awaited-cystic-fibrosis-drug-could-turn-deadly-disease-into-manageable-condition/.

Landzelius, K. and & J. Dumit. (2006). Introduction: Patient organisation movements and new metamorphoses in patienthood. Special Issue: Patient Organisation Movements. *Social Science and Medicine*, 62(3), 529-537, https://www-sciencedirect-com.remote.slc.edu/science/article/pii/S0277953605003072.

Landzelius, K. and J. Dumit (2006). "To me it's my life": Medical communication, trust, and activism in cyberspace. Special Issue: Patient Organisation Movements. *Social Science and Medicine*, 62(3), 591-601, https://www-sciencedirect-com.remote.slc.edu/science/article/pii/S0277953605003060.

Marshall, B.C. et al. (2009). Cystic fibrosis foundation: achieving the mission. *Respiratory care 2009 Jun*, 54(6), 788-795, https://slc.on.worldcat.org/oclc/352925314**.**

Our History. *Cystic Fibrosis Foundation*. https://www.cff.org/About-Us/About-the-Cystic-Fibrosis-Foundation/Our-History/.

Panofsky, A. (2011). Generating sociability to drive science: patient advocacy organizations and genetics research. *Social Studies of Science*, 41(1), 31–57, https://slc.on.worldcat.org/oclc/5792313874.

Wailoo, K. (2006). A Perilous Lottery for the Black Family: Sickle Cells, Social Justice, and the New Therapeutic Gamble. In *The troubled dream of genetic medicine: ethnicity and innovation in Tay-Sachs, cystic fibrosis, and sickle cell disease* (pp. 116-160). Johns Hopkins University Press. https://slc.on.worldcat.org/oclc/61211245.

Wailoo, K. (2006). Risky Business in White America: Gene Therapy and Other Ventures in the Treatment of Cystic Fibrosis. In *The troubled dream of genetic medicine: ethnicity and innovation in Tay-Sachs, cystic fibrosis, and sickle cell disease* (pp. 61-115). Johns Hopkins University Press. https://slc.on.worldcat.org/oclc/61211245.

Wailoo, K. (2001). Sickled Cells, Black Identity, and the Limits of Liberalism. In *Dying in a City of Blues: Sickle Cell Anemia and the Politics of Race and Health* (pp. 137-164). University of North Carolina Press. https://slc.on.worldcat.org/oclc/44794906.

What is Sickle Cell Disease (SCD). *Sickle Cell Disease Association of America, Inc*, https://www.sicklecelldisease.org/sickle-cell-health-and-disease/types/#.