Raeka Aiyar, PhD, Symposium Moderator | Stem Cells and the Future of Medicine

https://www.youtube.com/watch?v=r6bPIo7l_YQ&index=6&list=PLl4AfLZNZEQPxjqF4ojAO3wdCFMeriNBK&t=0s

Raeka Aiyar: Okay, there have been some emotional speeches. I wasn't ready for that.

I'm really happy to be back here at this excellent meeting. I'm really grateful to Ron for inviting me to come back. [Can you not hear? Sorry. Okay, yes, I will speak louder.] Sorry, I'm just a little bit emotional after hearing this morning's speeches.

So I'm really grateful to Ron and to the entire community for welcoming me back to this meeting. I'm really excited to be here at this event. It was a really inspiring event last year. And last year at this time, as he [Ron] mentioned, I was working with him at Stanford and with this great team. And I'd like to tell you a little bit today - before we get things kicked off with some of our very ME/CFS- focused talks - what I've been up to since then. Because I wasn't just recruited away. I was drawn to another really exciting area in science that I think is going to have profound implications for disease research, including for ME/CFS. And I'm looking forward to telling you about that today.

I'm working now in in the area of stem cell research, which I believe has the potential to absolutely transform the way that we research and treat diseases in a similar way that we've seen genomics transform research. And that was something I got to participate in during my early research career, my PhD, and working in Ron's lab of course as one of the drivers of that. I think stem cell research is about to do the same thing. It's still earlier days for that, but I think it's really exciting. I want to share that excitement and potential with you.

So right now I'm working at the New York Stem Cell Foundation. We are a non-profit foundation. We are a patient advocacy organization. Our CEO is is the mother of a patient, much like the Open Medicine Foundation. And like many of the of the foundations that support ME/CFS, and our mission is to accelerate cures for the major diseases of our time through stem cell research.

We do this in several different ways. The first is that we have a worldwide community of innovators that we fund all over the world, this includes early career investigators in stem cell and neuroscience research. It's a really excellent community. We convene them every year, kind of similar to this, and try to promote collaboration and exchange of ideas. We also have a huge focus on outreach through our conferences and symposia. We do an annual conference as well with about 500 people. We have a lot of events that are dedicated to educating patients about the different diseases that we study and how stem cell research can help them. And finally, we have a Research Institute where we do our own research in-house. So it's a bit of a unique model. I was really drawn to that because there's the opportunity for both education and outreach, which I've come to love so much in working with the ME/CFS community, and for being at the cutting edge of this new field of stem cell research. We also have these collaborations all over the world. I'm hoping to build some as well with this community while I'm here to drive stem cell research forward and really accelerate these cures.

So what do we research at the New York Stem Cell Foundation? We do a lot of different areas, this includes bone regeneration. Cancer. We have diabetes, which was one of our founding programs in autoimmune diseases. We also have a program for macular degeneration to restore eyesight in patients with age-related macular degeneration.

Our major focus is on neurological disorders, and that's particularly exciting for me because I'm hoping that there will be some lessons that we can also translate from, especially diseases like multiple sclerosis, to ME/CFS. But we also work on other neurodegenerative disorders, like Parkinson's and Alzheimer's disease as well as neuropsychiatric disorders.

So there's a really broad range of diseases that you can study a lot better with stem-cell research. I realize that not everybody might know what I mean by stem cell research, because there's a lot of things people mean when they say that, so I just want to show a very short video to explain why we think this is exciting.

[Broadcasting Video]:

Stem cells are the superheroes of the cellular universe. They are the only cells in the human body with the ability to turn into more than 200 types of cells, including heart, brain, spinal cord, kidney, liver, eyes, skin, and more. Stem cells also have the power to regenerate indefinitely, filling up the entire room you are sitting in. These cells are uniquely powerful because they allow us to do three things that we have never been able to do before.

First, by studying the stem cells of patients, we can see how those cells become damaged, causing disease. Second, we are able to test the safety and effectiveness of drugs on those diseased patient cells. And, third, we are learning how to replace those cells affected by disease with the patient's own healthy cells.

Imagine being able to use your own personal cells to replace an organ, bone, or other damaged tissue. Not by waiting in line for a transplant match that may never come, but through a safe, natural process for regenerating healthy tissues and organs [Music; end of video]

Just to summarize what you just saw in that video. There are three major areas that we are pursuing in stem-cell research. One of those is disease research, understanding the molecular basis of disease. What we heard from Ron and from many of us over the last three days, is that if we do want to cure a disease like ME/CFS, we need to understand how it works . And we need to try to do that in many different ways. Another major area is drug discovery. We believe that by testing drugs on patient-derived cells that you can get an idea of which drugs might be effective for which patients, and that's especially important in a disease as heterogeneous as ME/CFS. And finally there's cell replacement therapy. As I mentioned we're working on this for macular degeneration and people are working on it for Parkinson's disease to replace the cells that are lost. Now that's not really I don't think a possibility yet in ME/CFS because we don't know what cells are being damaged or need to regenerate. But if we do figure that out, as it happened for Parkinson's disease when it was determined that the mid-brain dopaminergic neurons are the prominent cells that are lost, then we can move forward with that. And I do want to point out about Parkinson's disease. When it was first discovered people thought it was a psychosomatic disease driven by viral infection. A lot of the same things were being said. So we might just be where that was 50 years ago.

So the reason that all of this is possible is because of technological revolutions. We can actually make your patients', your stem cells, from your skin or blood. We can induce them and that's why they're called induced pluripotent stem cells. And this was a discovery made in 2006 by Shinya Yamanaka. And just six years later he won the Nobel Prize in medicine. That's an incredibly fast turnaround for a Nobel Prize, and that's because of the promise that they've shown to revolutionize research.

So why is this exciting technology? Because we don't have to do invasive surgeries. We can use a skin biopsy or blood cells and we can make your stem cells in a dish and convert them into all of these different cell types that are affected in disease to understand what's going on. These are what your skin cells look like. They're actually quite pretty when you zoom in on them in a microscope. And then we can convert them into these induced pluripotent stem cells.

So just to give you a bit more of an idea of what I mean here. This gives you a bit of a window into disease. When you convert stem cells in the lab, you can convert them into the specific cells that are affected in disease. So in neurodegenerative diseases this means neurons, this means microglia, astrocytes, oligodendrocytes, all the major cell types that are involved. And you can study those in a dish. This really gives you windows into many different kinds of diseases. And having these in a patient-specific way allows you then to test different drugs on those cells in the lab and see what their effects are. And as I wanted to point out especially in ME/CFS. This can help us to get an idea of which drugs might work for which patients. Again, once we are able to see phenotypes or characteristics of these cells that make them different from healthy controls, and I think we're able we're going to be able to do that shortly.

I want to show you an example of cardiomyocytes. These are heart muscle cells. When you grow them from stem cells in a dish, they beat at the same rate as a human heart beats. You can test drugs on those, and you can detect arrhythmias that drugs that are going to induce arrhythmia on certain people or not. You can make neurons and see how well their electrical potential carries throughout a dish and how connected they are. So there are all these kinds of exciting features of these cells that you can see that are able to distinguish patients from healthy people just in a dish. Without having to go in and get your neurons out of your brains, which is definitely preferable to everyone in this room, I'm sure.

Finally as I mentioned with cell replacement therapy. If we find out that ME/CFS is a degenerative disease of some kind. If certain cells are being damaged, then what the possibilities are right now for macular degeneration (this is where a specific type of cell is lost in the eye). And clinical trials have actually started to show promise in restoring eyesight in some of these patients using stem cell-derived versions of these cells. The idea is the same in neurodegenerative diseases. And trials are now underway for Parkinson's Disease to replace those dopaminergic neurons that are lost . And we've just started a preclinical program to work on macular degeneration. We're collaborating with the Parkinson's disease researchers that I mentioned. So it's a really exciting time. What we're trying to push for at our Institute is "disease in a dish" and "clinical trial in a dish" models to really accelerate this. Provide an extra model system to study ME/CFS in and understand the molecular basis of it. And understand this on a patient-specific level so we can figure out better treatments that work for each patient. And we really have a mandate to accelerate all of this research at a community-wide level.

I was going to show you this quickly. We have a fully robotic system for generating these stem cells from skin cells or blood cells. And I will go through that.

One last cautionary note. There are a lot of clinics that offer stem cell therapies now for a variety of diseases. I would urge you to exercise caution with those because these treatments have not been approved yet and they can actually be harmful. These clinics are looking to financially exploit desperate patients. So it's important to remember that at this stage if anybody is advertising a stem-cell therapy or an injection for ME/CFS, I would advise you to exercise caution as none of these have been approved yet.

But we're hopeful that this is going to be the future of medicine that we will be able to treat your disease with your cells. We will be able to study your disease with your cells and understand it better at that level. So with that I want to thank the community that's made this all possible and thank you all for your attention.

[Applause]

Thank you to our wonderful team of transcribers for transcribing the Community Symposium.