

June 2021 Capitol Hill Briefing Summary:

Advancing Regenerative Medicine and Cell Therapies for Patients in Need



The Need for New Treatments and Cures

On Friday, June 18, 2021, the independent, non-profit Alliance for Cell Therapy Now and NFL Alumni Association (NFL Alumni) hosted a briefing on Advancing Regenerative Medicine and Cell Therapies for Patients in Need, featuring remarks from Congressman Ted Deutch (D-FL), former NFL player, Sterling Sharpe, and four distinguished researchers from across the country.

Mr. Sharpe, five-time Pro Bowler who played for several years with the Green Bay Packers, described the toll that sports can take on the human body. He described the need for hip replacement and the neck injury that forced him to retire from the NFL. He also spoke about how he, and other former football players, would prefer to find a long-lasting treatment that would negate the need to have surgery or take ten or more pills per day.



"I am definitely an advocate for anything that would allow us in our older age, not to have to go through surgeries, because I've had my neck fused, leaving the NFL, which is why I had to retire."

Bipartisan Leadership in Congress



"Federal investments in clinical research have been key to saving lives for cancer, reducing the impact of chronic conditions, and improving the lives of Americans and people around the world. That's why I joined Congressman Billy Long in pushing for federal funding for research and development of cell-based therapies—therapies that are showing great promise for patients who need the hope of breakthrough treatments and cures."

Congressman Ted Deutch (D-FL) offered opening remarks during the event, describing bipartisan efforts in Congress that will support researchers working hard to get out of the lab, to the bedside of patients, and on to regulatory approval.

Representative Deutch described how he has joined with Representative Billy Long (R-MO) to push for federal funding for research and development of cell-based therapies—therapies that are showing great promise for patients who need the hope of breakthrough treatments and cures.

Finally, the Congressman spoke of how he looks forward to working with stakeholders to advance science and bring the promise of advances in cell therapies forward to improve the lives of Americans across the country.

Promising Research

Dr. Colleen Delaney, a physician with more than twenty years of research experience, serves as the Founder, Chief Scientific Officer, and Executive Vice President of Research and Development at Deverra Therapeutics, a spinout focused on technologies she worked on while at the Fred Hutchinson Cancer Research Center in Seattle, Washington. Dr. Delaney has been treating patients with therapies off the platform she has developed since 2006. Most recently, she has embarked on the development of a new therapy off of this platform to boost the immune system of patients who are hospitalized with COVID-19.

Dr. Joshua Hare, Professor of Medicine and Founding Director of the Interdisciplinary Stem Cell Institute at the University of Miami Miller School of Medicine, talked about his research on tissue regeneration using adult stem cells—particularly mesenchymal stromal cells (MSCs) from bone marrow. According to Dr. Hare, MSCs have shown promise for a variety of applications, including aging and frailty, cardiac repair, and musculoskeletal conditions.

Dr. David Pearce, President of Innovation, Research, and World Clinic at Sanford Health discussed his organization's work in the area where there has been the biggest demand from patients-- orthopedics. Sanford Health just completed a clinical trial for safety that used stromal vascular fraction (SVF) for rotator cuff tears. The organization is also in the process of completing a pivotal trial that is being conducted across eight sites in the United States—including Sanford Health, Duke University, Emory University, Georgia Tech, and the Andrews Clinic—for osteoarthritis of the knee. Sanford Health is also working on a clinical trial exploring the use of SVF for osteoarthritis in five joints—the knee, wrist, ankle, shoulder, and hip, and hopes to be able to definitively answer the question of efficacy with respect to these treatments.

Dr. Krishnendu Roy, who serves in many roles at the Georgia Institute of Technology (Georgia Tech), including Director of the National Science Foundation Engineering Research Center for Cell Manufacturing Technologies, described how he is working with engineers, clinical scientists, biologists, chemists, physicists, and data scientists to improve manufacturing capabilities related to cell-based therapies, with the goal of creating scalable, cost-effective, high-quality cell therapies to improve access for people around the world. According to Dr. Roy, what we have seen over the last year or so, is that biomanufacturing has been one of the biggest challenges associated with the pandemic, and remains a challenge in terms of infrastructure for the country, requiring investment.



"I went into this field because I believed that these therapies were going to be the way of treating patients in the future. I still very much believe that as we learn more and more about human biology and how these cellular therapies work; cell therapy, gene therapy, regenerative medicine approaches are going to offer cures that are less toxic and a more natural approach to healing people."

Actions Needed to Bring Therapies to Patients

Panelists had a chance to discuss the challenges researchers in the regenerative medicine and cell therapy field are facing. One of the biggest issues: a lack of funding for translational research.

Dr. Delaney described how many institutions lack the infrastructure to translate basic research from the initial discovery to the patient's bedside. According to Dr. Delaney, there's great science happening at many institutions, but not all of these institutions have the infrastructure needed to support manufacturing. Funding needs to be directed to infrastructure, including consortia and groups "that can help people like me when I was first developing these therapies—I was like a deer in the head lights." Dr. Delaney also described the challenges she experienced, as both an academic and a biotech, as it relates to manufacturing, suggesting the need for a "manufacturing incubator," so that those who are in the early stages of clinical development could more easily have a place to manufacture under good manufacturing conditions.

Dr. Roy agreed with the need for investment, stating that, "if you look at other countries, especially the U.K. and Canada, they have really invested hundreds of million dollars in this space and the United States has not done it. So, companies within the U.S. are going to the U.K. and going to Canada to get help versus the U.S., because that infrastructure for translation that Colleen was just mentioning does not exist right now." According to Dr. Roy, "that gap of fundamental science discovery tools and technologies to scale up manufacturing and regulatory support, as well as prepare clinical trials for FDA approval takes a lot of investment. And it is really a public-private enterprise that is going to solve that."

According to Dr. Hare, we are in what he would call the "valley of death," where there are a large number of trials that have successfully completed a phase two trial and are ready to go to that pivotal, most expensive trial, but there's a funding gap.

There is preliminary data, we have the means to produce, and we have fantastic investigators—like Krish Roy and his team—working on the issues of scale-up that will be required. We also have the patient base; we have the technology and we have the need, but we're missing the resource—the financial resource—to be able to decisively go and do that phase three trial.



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And if we can get that, then I think you'll see a massive waterfall effect where we'll get answers in a very short period of time and get the necessary approvals so that these cell-based drugs can be responsibly used by the medical community in the appropriate patients in the very near future. According to Marchibroda, more than 90 percent active clinical trials in the U.S. are in phase one or two, and most are being conducted by academic medical centers or small biotechnology companies. Dr. Roy and Dr. Hare emphasized that to find out whether a therapy works, large, multi-center, randomized, placebo-controlled clinical trials are needed—an expensive investment that they hope the government will support through partnerships with academia and industry.

Dr. Delaney pointed out that all of the things panelists are talking about, including investments in this early stage, are about de-risking, including getting the right data and moving these things forward. Doing these things in an appropriate way will enable researchers to attract industry partners, which is necessary for commercialization.

Actions Needed to Bring Therapies to Patients

According to Dr. Pearce, “the data is there, the safety is there, and the efficacy is there in some respects; but we just have to take it to the next level, and it requires a little bit more focus on the science, a little bit more focus on the regulation, and certainly some resources.”

Dr. Roy also noted that one of the top issues is the workforce: globally, there are too few trained professionals in the cell therapy field, and the United States could be the world leader in regenerative medicine and cell therapy if the country invests in developing that workforce.

Finally, Dr. Pearce, Janet Marchibroda, and the other panelists noted the need to support patients, some of whom are desperate for new treatments. Dr. Pearce noted that patients in need are seeking out treatments and, oftentimes, are spending money on therapies that don't work: “policymakers need to be giving profound support for the FDA, not just in providing funding but endorsing that they have the ability to make these decisions and are making the right decisions... people are spending copious amounts of money on treatments that they have no idea if they are efficacious and, in many cases, they probably aren't.”



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With guidance from voluntary experts and leaders, Alliance for Cell Therapy Now and NFL Alumni are working to develop a patient primer to help individuals identify where to participate in clinical trials or seek Food and Drug Administration (FDA)-approved cell-based treatments. The FDA, with additional support, can also help provide clarity for patients in need. Marchibroda also recognized FDA's actions during the COVID-19 pandemic, which produced tests, treatments, and vaccines in record time: “I think about all the innovation at FDA, under Dr. Marks' leadership, and all that they did during the pandemic around rapid turnaround times... sustaining and supporting FDA's innovation, efforts, and leadership around the regulatory process is really important.”

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Closing Remarks



Billy Davis and Kyle Richardson, Co-Directors of Health Care Initiatives at the NFL Alumni Association closed the briefing by thanking the speakers and highlighting the personal nature of this issue for former NFL players.

A former NFL player, Davis has had more than 20 surgeries, and he hopes that cell-based therapies can improve his quality of life in the future. He also recognizes that regenerative medicine and cell therapy are a vital industry that ultimately needs to become the standard of care.

"I have personally had 20+ surgeries. And I'm hopeful that these therapies will give me a quality of life that I can enjoy in my older years. This is a vital industry that needs to be the standard of care."

Also a former NFL player and Board Member of NFL Alumni, Kyle Richardson is hopeful that these therapies can help address the musculoskeletal and neurological conditions that many former players (as well as those in the general public) experience.

Richardson also stated that these therapies won't get to patients unless there is more federal funding for clinical trials and manufacturing of regenerative medicine and cell therapies.



"We know and understand that these therapies won't get there to the patients unless we see more federal funding for clinical trials and manufacturing of regenerative medicine and cell therapies."

About Alliance for Cell Therapy Now

Alliance for Cell Therapy Now (Alliance) is an independent, non-profit organization guided by leaders representing academic and medical institutions, industry innovators, and patients, that is working to advance safe and effective regenerative medicine and cell therapies for patients in need. For more information, go to <http://allianceforcelltherapy.org/>

About NFL Alumni

Founded in 1967 by a small group of successful retired NFL players, NFL Alumni is one of the oldest and well-respected retired player organizations in professional sports. NFL Alumni's mission is to inform, assist, and serve players in their post-NFL lives. NFL Alumni's mission is focused on "caring for our own," "caring for children," and "caring for the community." NFL Alumni Health is a wholly-owned subsidiary of NFL Alumni, which is devoted to improving the health and wellness of NFL Alumni members as well as the general public, by providing informational resources, programs, services, and other programs. Visit www.nflalumnihealth.org.