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Draft for Review

## World-Renown Cancer Experts Reveal Solid Tumors as Next Target in Panel Discussion about Cancer Gene Therapy at ACGT Spon- sored Event

*Panel Featured Carl June, George Yancopoulos, Usman Azam, Andre Choulika, and Leukemia  
Survivor and CAR T Gene Therapy Recipient Rachel Elliott*

**NEW YORK, N.Y., April 23, 2018** — Cancer gene therapy had a banner year in 2017 with the FDA's first-time approval of two cancer gene therapy treatments. It is not surprising then that Alliance for Cancer Gene Therapy (ACGT) had a full-house at its recent panel discussion, **Cancer Gene Therapy: The Next Chapter - Perspectives from the Frontline**, held in New York City. The panel featured heavyweights in the cancer field: **Carl June, MD**, the Director of the Center for Cellular Immunotherapies at the Perelman School of Medicine at the University of Pennsylvania; **George Yancopoulos, MD, PhD**, president and chief scientific officer of Regeneron; **Usman "Oz" Azam, MD**, president and CEO of Tmunity Therapeutics; **Andre Choulika, PhD**, co-founder and CEO of Cellectis; and **Rachel Elliott**, a 21 year-old leukemia survivor and CAR T gene therapy recipient treated in Dr. June's clinical trial at the Children's Hospital of Philadelphia (CHOP). The panel was moderated by **Meg Tirrell**, biopharma reporter with CNBC.

ACGT is the nation's only charitable organization dedicated exclusively to funding cancer cell and gene therapy research and was one of the early funders of Dr. Carl June's breakthrough CAR T gene therapy research at the University of Pennsylvania to treat leukemia. Dr. June received his first ACGT grant in 2004 and a second in 2008, back when gene therapy was considered a "risky proposition" according to Barbara Netter, ACGT's co-founder. Fast forward to 2018 and the field has changed dramatically with major pharmaceutical companies and research institutions vying for the next big discovery using gene therapy or immunotherapy.

"When my late husband Edward and I first founded ACGT in 2001, gene therapy was a risky proposition," said Barbara Netter, co-founder of ACGT. "It had had some setbacks; but Edward was convinced that gene therapy was the way to successfully treating cancer, so we moved forward and raised funds solely with the focus of funding cancer gene therapy research. Dr. Carl June was one of our first grantees. My husband sadly passed away in 2011, just a few short months before Dr. June's success with treating leukemia with CAR T therapy was published in the [New England Journal of Medicine](#). He would be overjoyed to know that his vision is being fulfilled today."

While so much progress has been made in the last six-to-seven years, Dr. June acknowledged that, "It's still the early days of gene therapy. You have to remember that this is Generation One of CAR cells. For the future, we are working on being able to turn them on and off so that the body can rest, instead of it being always on as it is in the current version."

Dr. Azam, president and CEO of Tmunity Therapeutics and the former Head of the Cell & Gene Therapies Unit at Novartis, noted, “If someone had said to me in 2012 that there would be an approved product with a cell therapy that involved gene-editing technology, I’d have said you’re smoking something, it can’t be done. A miracle happened. To go from a proof of concept in a handful of academic centers to getting a full-blown approved product at that pace, is amazing. I’ve been in this industry more than 20 years and I never thought it could happen. And it happened! Now the challenge will be how will we approve upon that and get to the next generation.”

So what’s next? Solid tumors are on the horizon for cancer researchers. Given the recent success with immunotherapy for blood cancers, solid and metastatic cancers are the next big targets. Solid tumors present different challenges as they are more complex than blood cancers and put up more barriers to the immune system.

“What we are doing today is still probably the ‘pre-history’ of what is going to happen in the next five years,” added Dr. Chouluka, CEO of Cellectis, a clinical stage biopharmaceutical company focused on developing a new generation of cancer immunotherapies based on gene-editing CAR T cells.

Dr. June went on to note that, “If you look back five or six years ago when we first treated patients with CAR T therapies, I think we’re at the same point today with solid tumors. We’ve seen some glimpses of success, but no home-runs yet.” June believes combination therapies, checkpoint inhibitors and the use of CRISPR technology will be in the running for treating solid tumors within the next several years.

Pricing was also a topic of discussion with the newly approved immunotherapy treatments being priced at several hundred thousand dollars. Dr. George Yancopoulos, president and Chief Scientific Officer of Regeneron, noted that funding of cancer trials is very expensive. He stressed the importance of the open market for medical research, emphasizing that the risk is greater than the reward at this point and society as a whole needs to encourage scientific research that impacts quality of life directly. “We need to encourage more medical and scientific research and reward those who devote their lives to creating breakthroughs that will improve and sustain life,” he stated. “Without those rewards, there is no hope for our healthcare system to be one of the best.”

The scientists also discussed the rapid progress of scientific research and clinical trials in China. China and the United States are the only two countries currently devoting major resources to cancer gene therapy on a large-scale and are competing head-to-head on who will have the most breakthroughs first. While research is being done in other parts of the world, it is insignificant compared to the research taking place in these two countries. The United States alone has more than 1,000 trials happening today.

Margaret Cianci, executive director of Alliance for Cancer Gene Therapy, remarked on the quality of the panel: “These scientists are changing the way we think about and treat cancer at the cell and gene level and how to combine the most cutting-edge treatments for successfully treating all cancer types. This science is life-changing for patients and we are proud to be a part of it.”

The panel wrapped up with a moving testimony from Rachel Elliott, a 21 year-old leukemia survivor and CAR T gene therapy patient. Rachel was treated at the Children’s Hospital of Philadelphia (CHOP) as part of Dr. Carl June’s CAR T leukemia clinical trial in December 2016 and January 2017. She was first diagnosed with acute lymphoblastic leukemia (ALL) in June 2008 when she was just 11 years old. She followed the standard two-year protocol of chemotherapy and entered high school in 2010 in remission. In March 2015, Rachel’s leukemia returned during her freshman year at the College of Charleston. This

time, she experienced cytokine release syndrome due to the chemotherapy treatments and was in a medically-induced coma for 90 days and needed a tracheotomy. She spent 100 days in the hospital to help rebuild her immune system, followed by four months of intense physical therapy. In the fall of 2016, Rachel transferred to Virginia Commonwealth University, which was closer to home and her oncologist. After she relapsed again that October, she was committed to not going through the rigors of chemotherapy and the horrible effects she had previously experienced. Rachel was given two options: go home and go into hospice or take part in the CAR T trial developed by Dr. Carl June at the Children's Hospital in Philadelphia (CHOP). That December, Rachel went to CHOP for the clinical trial. Before the end of January, she returned home cancer-free without any severe side effects. Rachel resumed college classes and has remained cancer free ever since.

### **About Alliance for Cancer Gene Therapy (ACGT)**

Established in 2001, ACGT is the nation's only non-profit dedicated exclusively to cell and gene therapy treatments for all types of cancer. One hundred percent of contributions go directly to research. Since its inception, ACGT has funded some of the underlying science that has resulted in the formation of either licensing agreements or biotech companies including Novartis, Ziopharm, Juno, Tmunity, Turnstone Biologics, all of which are in various stages of bringing new treatments to patients. ACGT has funded 55 grants in the U.S. and Canada to conduct and accelerate critically needed innovative research. 36 of those grants have gone to Young Investigators and 19 grants to Clinical Investigators, totaling more than \$28 million in funding. ACGT is located at 96 Cummings Point Road, Stamford, Connecticut 06902; 203-358-5055. To learn more, visit [acgtfoundation.org](http://acgtfoundation.org) or join the ACGT community on [Facebook](#), [Twitter](#) and [YouTube](#) at [@acgtfoundation](#).

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