eSequence

ACGT National grants for cell and gene therapy cancer research
ALLIANCE FOR CANCER GENE THERAPY

November 6, 2013

A new era for cancer treatment



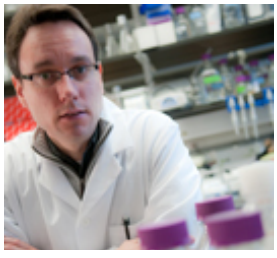
Drs. Cooper, Woo, ACGT President Barbara Netter, Drs. June and Sadelain Photo credit: E. Ubiña

An ACGT educational forum in October brought together three of the best medical researchers in the world to discuss tremendous strides being made to combat cancer using cell and gene therapies. Donors, sponsors and friends enjoyed a reception at the Harvard Club in Manhattan, followed by a panel discussion facilitated by ACGT Scientific Advisory Council Founder and Chair, Dr. Savio Woo, Professor of Hematology and Oncology at Mt. Sinai School of Medicine. In a lively discussion, award-winning research physicians Dr. Laurence Cooper, M.D. Anderson, Dr. Carl June, University of Pennsylvania, and Dr. Michel Sadelain, Memorial Sloan-Kettering, discussed the targeted therapies that trigger the body's natural defense system to destroy and prevent cancer. This form of molecular medicine, which has few or no side effects, has shown remarkable promise in laboratory research and has been proven in clinical trials. Also in attendance was Connie Burnett-West, a clinical trial participant who defeated end-stage lung cancer after all other treatments had failed. To learn more about immunotherapy or about medical research breaking new ground, contact ACGT Executive Director, Margaret Cianci.

"After only a sixth-month course of gene therapy, I've been in remission for over ten years, and I could not have imagined a treatment so easy and effective."

Connie Burnett-West, Cancer Survivor

In the News



Science Translational Medicine journal reported October 30th that a laboratory treatment at Northwestern University successfully killed a deadly gene in brain cancer. ACGT Research Fellow, Dr. Alexander Stegh, is the study's co-investigator. This nanotechnology makes it possible to cross the blood-brain barrier to get to the tumor and turns



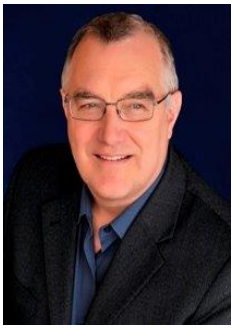
off a specific cancer-causing gene in the cells. "The beauty of the gene we silenced in this study is that it plays many different roles in therapy resistance. Taking the gene out of the picture should allow conventional therapies to be more effective." On average, 16,000 new cases of glioblastoma are diagnosed every year with only a 14 - 16 month survival rate. Read the full report.

Pharmaceuticals Focus on Immunotherapy

Five major drug companies, including Bristol-Myers Squibb, Merck and Roche, are developing drugs that stimulate the immune system to recognize and destroy tumors. These treatments derive from cell and gene therapy research like the 42 studies funded by ACGT. Bristol-Myers has already introduced Yervoy for skin cancer, which also has the potential to treat other cancers. Thus far, among 1800 advanced melanoma patients treated with the drug, 22% were living three years later after a single 12-week course. Now that these studies have caught the attention of pharmaceutical giants, Wall Street will be watching, which will hopefully ensure further funding for development. Read more about it.

THE WALL STREET JOURNAL.

Grant Awarded to Canadian Scientist, Dr. John Bell



Dr. John Bell, PhD, is the recipient of the 2013 ACGT Investigator Award for clinical translation for a novel treatment for brain cancer. A Senior Research Scientist and Professor of Medicine at Ottawa Hospital Research Institute, Dr. Bell deploys an oncolytic virus to destroy brain cancer, among the most difficult to treat. Oncolytic viruses have proven an effective gene therapy in late-stage clinical trials for other forms of cancer; however the treatment has been less safe for the brain, and also more difficult to produce on a large scale. This research will aid in the design of a manufacturing process for broader use and to establish new efficacy benchmarks for treatment. Read more about it.

Rallying for Research

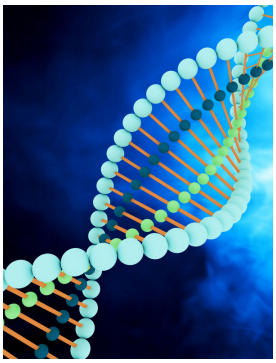
In September, ACGT joined the Rally for Medical Research in Washington, DC, a coalition of 172 organizations and 60 support groups established to advocate for increased spending on medical research. In total, 246 research agencies and universities organized nearly 300 meetings on the





hill, and 72 members of Congress engaged personally in the dialogue. Many individual participants were survivors and patients, joining forces with clinicians and researchers to voice their concerns. The rally was especially important this year, as NIH research spending has been threatened with significant cuts in the Sequester budget. Breakthrough research, particularly in early discovery, continues to be underfunded, and budget cuts slash not only spending, but hope for advancement. Read more about it.

Progress and Promise



ACGT is still the only organization in the nation dedicated exclusively to cell and gene therapy research for cancer. Over the years, we have awarded $23 million to 42 research projects, split almost evenly between discovery and clinical translation. To date, funded studies have led to 11 clinical trials that validate early findings, test safety and efficacy, and save lives. 100% of donations go directly to research, as funding for administrative and fundraising costs is provided separately.

"*We are on the cusp of a golden age of cancer prevention and treatment, thanks to the promise of recent discoveries*."

Dr. Michel Sadelain, MD, PhD, ACGT Research Fellow
Memorial-Sloan Kettering Cancer Center

Help us support breakthrough research that is changing the way cancer is treated and defeated. Donate in honor of a loved one, give a holiday or memorial gift, host an event, tell your friends, and keep posted on progress at www.acgtfoundation.org, on Facebook and Twitter@acgtfoundation.

Donate now!

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