Future of Medicine: Body Heal Thyself

A panel of Hutch scientists talked gene therapy, big data and financial toxicity to reporters at the annual AAAS meeting.

April 13, 2020 By Sabrina Richards

We're on the cusp of a new era in disease treatment. We have the unprecedented ability to alter the human genome and harness our immune systems to fight cancer and other diseases. But with the promise of a new future come pitfalls, including innovative, expensive new treatments beyond the reach of many, and the potential for unethical use of genome-modifying techniques. Hutch scientists are leading the charge into this new future while balancing — and researching — its potentials and perils.

At a Hutch-sponsored breakfast for news media at the 2020 AAAS conference held in Seattle, Washington, they spoke about leading the charge into this new future with enthusiasm without ignoring potential perils. Increasing scientific literacy and diversity in science will be part of the solution, they said.

The panel, made up of Veena Shankaran, MD, a physician-scientist and expert on the financial toxicity of cancer treatment as well as co-director of the Hutchinson Institute for Cancer Outcomes Research, or HICOR; gene therapist Jen Adair, PhD, who holds the Fleischauer Family Endowed Chair in Gene Therapy Translation; and biostatistician Raphael Gottardo, PhD, who directs the Translational Data Science Integrated Research Center, or TDS IRC, and holds the J. Orin Edson Endowed Chair, convened to discuss the next horizons for cancer research. Jeanne Chowning, PhD, who directs the Hutch's long-running Science Education Partnership, moderated the panel.

Potential and pitfalls of new technologies

The researchers are excited about what's coming in research and what's already arrived in the clinic.

In the last 10 years, "There has been tremendous progress in oncology in terms of what we're able to offer patients. Patients are living longer," said Shankaran, who treats patients with gastrointestinal cancer at Seattle Cancer Care Alliance, the Hutch's clinical care partner. Limited to just one treatment option in 2009, patients with colorectal cancer now have several, including immunotherapies, she said.

More may well be on the horizon. New technologies are making it possible for scientists to examine our biology, including during treatment with experimental therapies, in unprecedented

detail. This holds enormous potential for helping us understand why some therapies only benefit some patients, and what to do about it, Gottardo said.

Adair came to the Hutch with the goal of turning gene therapy from a complicated, expensive undertaking limited to the research setting into a simple, cheap technology that could be provided to anyone, anywhere, she said. This would make it possible to tackle diseases, like HIV and sickle-cell disease, that affect tens of millions of people in some of the world's most resource-poor settings.

But her optimism and enthusiasm hasn't blinded her to the potential pitfalls of genome editing, said Adair, who gave a flash talk on the use of CRISPR in gene editing during the Human Development and Innovation session. Hutch colleague Mark Roth, PhD, spoke during the same set of flash talks on the potential of iodide as a trauma treatment.

"Basically if it has DNA, somebody is probably trying to CRISPR it already," Adair said. This includes human embryos. Last year, He Jiankui was sentenced to three years in jail for using CRISPR to edit the DNA of two human infants prior to their birth.

A big issue is "how we're going to manage ethical concerns around editing DNA, something that I think about pretty much daily," she said. "The upside of creating simpler technologies is accessibility and affordability. The downside is making the ease of use so easy that it gets misused. Trying to balance that in our work is something we think about a lot."

Opportunity for everyone

Accessibility isn't just a problem in resource-poor countries, Adair acknowledged — patients in the U.S. also struggle to gain access to, and pay for, lifesaving cancer care. As exciting as many new technologies and therapies are, they're not accessible to many patients. Many people who should have a hand in developing them struggle to get a foothold in the scientific community. The panelists spoke about research and initiatives at the Hutch that aim to address this.

Shankaran touched on the financial hurdles to effective cancer treatment faced by many patients. For example, she cited work from HICOR showing that cancer patients are 2.6 times more likely to file bankruptcy than average, and that cancer patients who file for bankruptcy are more likely to die from their disease than those who don't file.

"Our ultimate goal, I would say, is really that all patients, regardless of their financial status or insurance type, have the opportunity to receive the highest quality of care and access to the most promising therapies," Shankaran said.

Making therapy work for every patient

The scientists also highlighted the lifesaving potential of expanding our therapeutic options.

"There's not going to be one answer that works for every patient or every disease type. We need a

big toolbox to be able to pull from and be able to treat lots of patients," Adair said.

Another pressing need is to make sure that the treatments already in the clinic work for everyone. Even when patients have access to the newest and most innovative therapies, they don't always benefit. The mandate of Gottardo and scientists within the TDS IRC is to figure out why. He believes that big data, driven by new single-cell technologies, will be the answer.

Single-cell technologies, in which scientists learn about the molecular characteristics of individual cells in a blood sample or tumor, is revolutionizing the knowledge they can work with. Researchers can generate more data from a single patient sample than they can feed onto a personal computer. Big data is here, and it's ballooning.

Soon scientists will add three-dimensional information on top of their single-cell analyses, in what Gottardo referred to as "multi-modal datasets." This will give them critical information about which cells are interacting — or not — with tumor cells.

"We'll have a large amount of data soon enough, whether that's from a lot of patients or going very, very deep on selected patients, we'll have the ability to learn a lot," he said. "And this will allow us to move forward and ultimately come up with amazing, amazing therapies and cure cancer."

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