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Making science serve humanity: Jennifer Doudna, PhD, says CRISPR gene-editing technology should be accessible to all

The co-winner of the 2020 Nobel Prize in chemistry shared insights into the clinical potential of gene editing, the importance of equity, and how CRISPR could help fight future pandemics at Learn Serve Lead 2021, the AAMC's annual meeting.

By Bridget Balch, Staff Writer

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Jennifer Doudna, PhD, Nobel Prize co-winner for developing CRISPR gene-editing technology, speaks with session moderator, J. Larry Jameson, MD, PhD, chair of the AAMC Board of Directors, at Learn Serve Lead 2021: The Virtual Experience on Nov. 8.

Photo by Laura Zelaya, AAMC

The path that led Jennifer Doudna, PhD, and her colleagues to the development of CRISPR, the gene-editing tool that has revolutionized science and earned her a Nobel Prize, started with their deep curiosity and drive to understand how the most basic building blocks of life function.

When Doudna first decided to investigate precisely what systems bacteria use to adapt their immune systems to fight off viral infections, she had little expectation that the findings would ultimately provide the key to technology that could be used to safely alter genetic code.

“All of us [on the research team] realized that what had started as a fundamental research question was morphing into a very different kind of project; namely, one with enormous technical potential and also risks and opportunities that we had not appreciated when we started the work,” Doudna explained during a conversation with J. Larry Jameson, MD, PhD, chair of the AAMC Board of Directors and executive vice president of the University of Pennsylvania Health System, at the opening plenary of Learn Serve Lead 2021: The Virtual Experience, on Monday, Nov. 8.

Doudna, who runs a laboratory at the University of California, Berkeley, and founded the Innovative Genomics Institute, which works to advance genome research in a way that benefits humanity equitably, gave a presentation explaining the science behind CRISPR technology before engaging in a discussion with Jameson and attendees about the technology's potential opportunities and risks.

"It's been extraordinary to see how fast CRISPR has taken off in labs around the world," Doudna said, listing examples of clinical trials already underway to use CRISPR to treat illnesses such as sickle cell disease, congenital blindness, and cancer. She added that, within the next two decades, CRISPR could even be used to treat more common ailments, such as dementia and cardiovascular disease, and to bolster immunity against pathogens that might cause future pandemics.

Still, despite reveling in the great power of the technology, Doudna emphasized the responsibility she feels to advocate for its ethical use.

"Like any new technology, CRISPR comes with risks," she said. "It was clear early on that there were going to be some real ethical challenges."

Those challenges include ensuring there are internationally recognized regulations for the use of CRISPR — including addressing the idea of creating "designer babies" — and the need to ensure the treatments derived from it are affordable and accessible to people from all socioeconomic groups across the globe.

"We have to be cognizant of how to be sure that everyone who can benefit from this technology has access to it," Doudna said. "That's something I'm very deeply committed to."

An important key to making progress in science is by embracing collaboration, especially by including people from diverse backgrounds in the lab, she added.

"There's nothing better than having people from different parts of the world, different cultural backgrounds, [and] different educational backgrounds who converge on a question or a problem," Doudna said. "In my experience, that type of collaboration often leads to the very best outcomes in science."

She also acknowledged the research and discoveries that are yet to come that will continue to propel CRISPR and science into a better future. Doudna feels that mentoring the next generation of scientists is among her most important responsibilities.

In a comment directed to students, she said, “It’s an extraordinary time to be getting into science. I’m really excited to see what you discover.”

In advance of Learn Serve Lead, Doudna spoke with *AAMCNews* about her hopes for the future of CRISPR, the importance of representation in science, and how academic medicine can improve opportunities for innovation moving forward.

How has this pandemic impacted your work — and how did you shift your own work to respond to the pandemic?

Well, like for many people — maybe everybody — the pandemic really changed the way we're doing research in the laboratory: initially because we were basically prevented from coming into the lab, and then primarily because we decided to focus our efforts on addressing the pandemic by setting up a clinical testing laboratory institute and by working to develop new types of COVID diagnostics. So that was work that started in March of 2020. As a result, we have a CLIA [Clinical Laboratory Improvement Amendments] certified laboratory running on our campus. We run about 15,000-20,000 COVID-19 tests a week for the university community and for a number of our health care partners in the San Francisco and East Bay area. And that's been just amazing. It's been a wonderful opportunity to engage with our community — but [it] also has allowed us to begin accessing and reporting on clinical results and working directly with physicians.

And having this clinical lab has also allowed us to work together with health care partners on advancing the development of CRISPR-based diagnostics. This has led to three new types of diagnostic chemistries that all rely on the CRISPR technology. And we hope that one or more of those will, in fact, have applications for point of care testing. That’s something that we're actually exploring currently with a couple of our partners.

CRISPR has had so many research implications. What has it been used for that you find most fascinating or most inspiring so far?

A couple of things come to mind. One is that it's been extraordinary how fast the CRISPR technology has moved into the clinic. We've seen, in less than 10 years, the technology has already gone into Phase 1 trials for a number of different types of diseases, including cancer and disorders of the liver, the eye, and the blood. So far — knock on wood — those trials are going well, and I think there's a lot of momentum in the field right now. I think [there’s] a lot of expectation that, within the next five to 10 years, we will see — we hope — approved therapies that rely on fundamentally correcting the disease-causing mutation at the source — you know, in the DNA. It's a different way of thinking about disease. Rather than mitigating the consequences of disease, [scientists are] trying to prevent disease in the first place or cure it, so that patients don't have to deal with the consequences of genetic disease in the future. I think that's really very exciting.

Is that something that you had anticipated coming together as quickly as it has? And what are your long-term hopes for the future of medicine, especially with genetic diseases?

I think it's always hard, in my experience, to predict how fast things will progress when you go into the clinic with human patients. It's really wonderful to see how fast the technology has progressed, but I certainly couldn't have predicted it. The technology works great in the laboratory, but that doesn't necessarily speak to how effective or safe or efficacious it would be in a human. One of the things that I think about the most right now is affordability and accessibility of the technology. It certainly looks right now that, in the future, we will have the ability to use CRISPR to cure genetic diseases. The question will be: Who gets to use [it]? Who can afford those treatments? I think this is so important in terms of thinking about — as the technology advances — how do we, as scientists, make sure that we're addressing some of the technical and scientific issues that could otherwise be barriers to affordability? One example of that is the way that the CRISPR technology is delivered into patients. There's a wide range of methods that could or should or might be used, and they have different costs and timelines associated with them. I think that's one thing that will be very important to address going forward.

You've spoken very publicly about the potential ethical implications of gene editing and how that might be used to make enhanced humans in the future. What are your concerns about the ethics, and how do you think the scientific world and society in general should be talking about this to ensure that this technology is not abused?

Well, first and foremost, I think it's critical to have transparency around the use of the technology. That's one of the reasons that I've been involved, for the past several years, in organizing meetings — both locally and internationally — on the subject. And I think just inviting the scientific community and other stakeholders to engage together on thinking about how this technology is to be used in the future is so important. It's an extraordinary opportunity that we have — but [it] also comes with a lot of responsibility.

You've talked about how, when you were in high school, seeing a woman who was a scientist helped put you on your career path. Why do you think that was important to you to see a woman who was in science?

When I was growing up, my image of a scientist was the classic photograph of Albert Einstein or cartoons of mostly older White men who were in lab coats with black-rimmed glasses and crazy hair. That was the media caricature of a scientist, but I think without even realizing that, I had really internalized that as what a scientist looks like. By definition, a scientist is a man. And for me, being in high school and realizing that I was interested in mathematics and chemistry and biology — and then having the opportunity to see a wonderful presentation from a scientist who happened to be a woman who was working on cancer biology — I just thought that was amazing. And when I saw that talk, I still remember the way I felt. I just thought that “that's what I want to do. I want to be her.” And I think it's really important for students to have mentors that they can identify with — people who look like them in some way or make them think that there's a connection between where they are in their life at an early stage of their education and where they could be someday.

What still needs to change in the scientific community to make it more accessible to women — for them to see themselves in that career?

Certainly, having more diversity in science is a good thing. I think that's very important. I think that having opportunities for students in the early part of their training and their education, giving students opportunities to work in labs or hear about research that's going on from different types of scientists — a diverse set of scientists, diverse in all ways, not only in terms of the scientists themselves but also the types of work that they're doing — I think that's critical because, in my experience, kids are natural scientists. They're curious about their world and, unless somebody squelches that, they're interested in the world that we inhabit. Why do we look the way we do? Why does our world work the way it does? All those types of questions. Finding ways to encourage that natural curiosity is so important.

What do you see as the most pressing issues facing the academic medical community now and what message would you want to give its leaders?

Given where things stand today with the challenge of the ongoing pandemic and what science has already achieved, having scientists continue to work together to collaborate — to the extent possible — to be adaptive is critical. We've seen this in real time with the development of vaccines for COVID-19, for example, as well as other advances that have happened so quickly over the last 18 months due to teamwork and collaboration in academic groups and companies and in partnerships between them. The other thing I would say is that I think there needs to be a balance between fundamental curiosity-driven science and applied science, because we need each other. The fundamental science often goes in unexpected directions and leads to advances that wouldn't have been made otherwise, and CRISPR is certainly in that category. But we also need to have ways to quickly bring those ideas and discoveries to real-world solutions to problems. That's an important challenge for all of us — to figure out how to best achieve that balance.

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