STAT

The breakthroughs that are revolutionizing health care

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TABLE OF CONTENTS

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The breakthroughs that are revolutionizing health care

Every major scientific advance might have sounded like science fiction before it happened. Gene editing technology being deployed to treat devastating and deadly diseases in children. Viruses being harnessed to deliver one-time treatments for rare diseases. Vaccines developed at record speed to combat a deadly virus.

The field of science has seen unprecedented advances in the past year. And those breakthroughs were center stage at the 2021 STAT Breakthrough Science Summit, which explored the biggest advances in science and medicine and looked ahead to the breakthroughs that have the potential to revolutionize medicine in the years to come.

These stories demonstrate the impact those breakthroughs have had on health care, the painstaking research that went into making them a reality, and the challenges ahead to ensure that science's biggest advances are accessible for everyone.

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HEALTH

The world will not exit Covid-19 pandemic without booster shots, vaccine developer says

By Helen Branswell | JULY 13, 2021

The world will not get the Covid-19 pandemic under control without using booster shots for messenger RNA vaccines, one of the key figures involved in the development of the Pfizer and BioNTech vaccine said Tuesday.

Ugur Sahin, co-founder and CEO of BioNTech, insisted booster shots are going to be necessary, despite caution from some experts.

"At the end of the day it really matters that we get this pandemic under control. And we will not get it under control without boosting. That's my strong opinion," Sahin said.

Sahin made the remarks during a panel session at the STAT Breakthrough Science Summit, where he was joined by Kathrin Jansen, head of vaccine research and development at Pfizer. Jansen said Pfizer's decision to develop and test Covid-19 vaccine booster shots is being driven by data. "The booster situation is not about making money," she said.

A large study of booster shots developed by the company will read out soon, Jansen added.

Pfizer triggered controversy last week — National Institutes of Health Director Francis Collins referred to it as "a dust-up" — when the company issued a statement suggesting booster shots will be needed to keep protection against the virus at high levels. The statement referred to data that have not yet been published.

The director-general of the World Health Organization, Tedros Adhanom Ghebreyesus, criticized the notion of giving residents of wealthy countries a third shot before health workers and older adults in many countries get their first Covid-19 jab. And Anthony Fauci, director of the National Institute of Allergy and Infectious Diseases, said people are "jumping the gun" if they think booster shots are on the verge of being authorized.

Neither Jansen nor Sahin elaborated on the data that the companies say support their view that booster shots will be needed. Sahin said there is evidence of a slight decline in antibody levels in people four to six months after they were vaccinated. To date, there hasn't been an indication that people who have been vaccinated are developing severe Covid infections, but Sahin said "we expect that we will also see some drop of protection against severe disease … a small drop."

"Therefore our position is … that a booster shot could be helpful to restore immunity, full immunity, and thereby ensure we have a winter season which is not complicated by true infections in vaccinated people," he said.

There is currently no sign that people in the United States who were vaccinated early in the country's vaccine rollout are seeing their protection wane, Jay Butler, the deputy director for infectious diseases at the Centers for Disease Protection and Control, said earlier Tuesday.

"We're not seeing evidence at this point in time that waning immunity is occurring among people who were vaccinated back last December or January and that they are at higher risk of breakthrough infections," Butler said during a media briefing organized by the Infectious Diseases Society of America.

Jansen said part of the goal of the companies' research on boosters — which includes studying the impact of giving people a third dose of vaccine that protects against one of the variants of concern — is to create a regulatory pathway for updating Covid vaccines if that becomes necessary.

Though she did not mention it, there is a regulatory pathway for updating influenza vaccines. Rather than requiring manufacturers to run large-scale clinical trials every time flu vaccines are tweaked to try to keep up with ever-changing viruses, the Food and Drug Administration allows manufacturers to update the viral targets through an expedited process referred to as a strain change. As long as the production process remains unaltered, manufacturers can swap out an old version of H1N1 flu, for instance, and include a strain that is currently causing disease.

In other remarks, Jansen voiced concern about Covid-19 vaccine uptake, saying the misinformation about vaccines that is prevalent on the internet is impeding progress toward getting enough people vaccinated to control the virus.

"I think it's a huge issue for us, because we always will be behind the virus," she said. "We cannot get ahead of it if large fractions of the population refuse to be vaccinated."

Allowing the virus to continue to spread unchecked will give rise to additional variants, some which may be able to evade the protection of vaccines, Sahin and Jansen warned.

"As long as we allow the virus to have a breeding ground in unvaccinated people, we will force the virus to adapt, to mutate, to change, and it will be a disaster because we will not be able to get ahead of it," Jansen said.

The two also talked about the challenges of developing a new vaccine in under a year. "In every vaccine development program you have enormous challenges. And the difference is that those challenges are over 10 years. Here we have the same challenges over nine months," Jansen said with a laugh.

Asked what lies ahead for mRNA technology, after its enormously successful testing in the Covid pandemic, she said Pfizer wants to turn its focus to flu.

"We have aspirations to come up with a truly game-changing influenza vaccine that could actually far surpass the efficacy, potentially, of current vaccines," she said. Existing flu vaccines aren't adequately effective in elderly people, who are at high risk of developing severe illness if they contract the flu. But the Covid mRNA vaccines are highly effective in the elderly, she noted, which suggests the platform could be very useful for flu shots.

"It opens up a lot of possibilities in the infectious diseases space," Jansen said.

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POLITICS

FDA chief Janet Woodcock acknowledges agency may have misstepped in process leading up to Alzheimer's drug approval

By Rachel Cohrs | JULY 14, 2021

WASHINGTON — Acting Food and Drug Administration Commissioner Janet Woodcock acknowledged on Wednesday her agency may have misstepped in its handling of its polarizing approval of a new Alzheimer's drug.

She was emphatic in her defense of the therapy and the agency's approval decision in an interview at STAT's Breakthrough Science Summit, but said "it's possible that the process could have been handled in a way that would have decreased the amount of controversy involved."

"Was the process done exactly the best that it could be?" she said. "Possibly not."

The FDA's approval of the drug, Aduhelm, has been mired in controversy after controversy since its approval last month. Already, the agency has had to narrow the patient population for whom the drug is approved; late last week, Woodcock also called for an independent watchdog to investigate the actions that led to the drug's approval, after a STAT investigation revealed close coordination between Biogen, the drug maker, and FDA regulators.

In the interview Wednesday, Woodcock was clear in her support for the drug approval itself.

"The accelerated approval was based on very solid grounds," she said. "I do believe that will play out over time, as people see that was a very appropriate use of that authority and the right thing to do for patients."

But she also deflected questions about whether she was aware of a May 2019 off-the-record meeting between an FDA regulator she supervised and Biogen, which STAT first reported.

"I was working in Operation Warp Speed all of last year, all right, as the therapeutic lead. And I'm not going to comment on my awareness or elsewise," Woodcock said. She pushed back on a follow up question, too, saying "I think we ought to move on," and, later, "This is an interrogation right now."

Operation Warp Speed was launched to expedite development of products related to Covid-19 in May 2020, after the off-the-books meeting on Aduhelm occurred.

The acting commissioner also defended the FDA's decision to approve Biogen's controversial Alzheimer's drug for a wide range of patients outside of those studied during clinical trials. The decision surprised even some senior FDA officials, STAT reported last week.

It's more common for the FDA to approve a drug for a broader patient population for a drug in the neurodegenerative disease space, Woodcock contended.

"In this case I think after getting feedback, they realize they should be more descriptive about who was in the trial and what was known about those not in the trial," Woodcock said.

Asked more broadly about how the FDA could address the pharmaceutical industry's efforts to influence its regulatory decisions, Woodcock said that while the agency was well aware of that push, it is focused on the data.

"Drug makers are very eager to get their drugs approved, and they have various strategies that they pursue and the FDA is quite aware of this," she said. "We look at the data that's before us and we verify those data, and that's been our long standing procedure and process. … We believe our decisions are based on the data in front of us and the standards that we have."

ON COVID-19 AND THE POTENTIAL NEED FOR BOOSTER SHOTS

Pfizer has suggested that its own, unpublished data shows waning immunity to the Covid-19 vaccine — and therefore supports the controversial idea of a third "booster" shot. It and Moderna are both studying the option right now.

Woodcock, however, was firm, saying that vaccinated Americans shouldn't worry about the need for a booster right now.

"We don't think that boosters are needed at the current time, and we will follow the data, the data on waning of immunity over time and the data on acquisition of inspections by the vaccinated, to see if at some point it appears that a booster would be warranted."

Asked, as a follow-up, whether the FDA can keep working as hard and as fast as it did during the Covid-19 pandemic, Woodcock was clear: No.

"People were working 24-7. We can't work for people until they drop, we won't have any staff. So that is not sustainable. Obviously with greatly increased resources we could probably move somewhat faster, but there is a limit," she said.

"Much of the speed, remember, of drug development and so forth was within the execution of the clinical trials, and that had to do with really putting a lot of resources against getting those things done, particularly the vaccines."

She added, however, that she'd like to see some of the flexibilities of teleworking remain, even after the pandemic.

ON WHAT'S NEXT FOR HER

Woodcock, a longtime veteran of the FDA, is a rumored contender for the nomination to full commissioner of the agency. But if she isn't nominated by November, she'll have to step down, she acknowledged Wednesday.

"Well, of course, there's a goal of having good continuity," she said. "Some of the work that I'm working on now, which include the really, instantiation of the modernization plans for both infrastructure and data, will, will be long term projects that will need to go on."

STAT

With fewer than 400 progeria patients worldwide, testing a CRISPR cure will be challenging

By Eric Boodman | JULY 14, 2021

When Sammy Basso was diagnosed with progeria at 2, it seemed likely that the disease's hyper-accelerated aging would kill him before he graduated high school.

"There was nothing. Nothing. No cure, of course, no treatments, no information. It was catastrophic," said Basso, spokesperson for the Progeria Research Foundation, at STAT's Breakthrough Science Summit on Wednesday. "Nobody could believe that we will be able to arrive at this point. But here we are."

What he meant was that the last two decades have seen a dramatic surge in scientists' understanding of progeria. It has brought not only an approved drug for the disease — one that's helped Basso live to 25 — but also a potential cure, which has so far worked in mice, and for which researchers hope to start clinical trials soon.

Designing a human study for such an ultra-rare and deadly disease poses a challenge, though. With only 400 progeria patients worldwide, there aren't enough of them for traditional trials — and the disease is so deadly that to give them a placebo is viewed as unethical.

Plus, they don't have much time. Progeria is a genetic illness that seems to speed up time. A baby will seem healthy at birth, but then, at some point in the next year or so, proves not to be growing at a normal rate, loses fat under the skin, and starts prematurely showing the health problems of old age. On average, patients with untreated progeria live to the age of 14 and a half, their deaths often caused by cardiovascular disease or stroke.

The biology behind this first emerged in 2003. During his medical training nearly 20 years earlier, National Institutes of Health Director Francis Collins had seen a patient with progeria, and when advocates said they were looking for researchers to help, he felt invested and began asking around. "After I tried to get other people interested in working on this to find the cause, I decided, 'Well I've got a lab, and my postdoc's looking for a project, and so Maria, why don't you see if you can figure this out?'" Collins said during the panel.

It was a challenge, but she did — with help from many colleagues. (Her name is Maria Eriksson, and she's now a professor at Sweden's Karolinska Institute.) "Almost all of the individuals with progeria had a single letter of the genome out of 3 billion that was misspelled," Collins explained — and that tiny typo was giving rise to a toxic protein that shortens the life spans of cells. "We thought, 'Gosh, maybe there's some way — somehow, someday — this could lead to a benefit,'" he said.

The benefit arrived, in a way, because David Liu, vice-chair and core faculty member of the Broad Institute of MIT and Harvard, saw an interview about progeria on TV. That put the disease on his radar screen, and years later, when his lab was working on a more precise way to edit DNA than your run-of-the-mill CRISPR Cas-9 system, his team saw that the technique could change that particular progeria-causing typo.

"It does so not by cutting and messing up the gene as CRISPR as [it] evolved in nature, does," Liu explained. Rather, it uses CRISPR's ability as a kind of guide, homing in on the genetic bit that needs to be changed, but instead of slicing into the double helix and potentially screwing up genes, it rearranges the atoms in one letter — known to biologists as a base — so that it becomes a different one. Hence the name base editor.

Working with Collins, Liu found that this editing tool helped patients' cells in a dish, but also allowed mice with progeria to survive longer — results they published in January 2021.

Now, the researchers are trying to assemble all the data they need to apply for regulatory permission to start a clinical trial. That's hard for most diseases, but even harder for progeria. "Time is not on our side," said Leslie Gordon, co-founder and medical director of the Progeria Research Foundation, and professor of pediatrics at Brown University.

"The worst thing that could happen in a clinical trial is you design it so that you don't really know the answer," said Gordon, who lost her son Sam to progeria. A traditional study would recruit patients, give some of them the drug, some a placebo, and then compare how they do. But that approach doesn't work for progeria — and so in trials for the currently approved treatment, researchers compared participant results to data on untreated progeria from past studies. On average, that treatment added two and a half years to patients' lives.

The trial that the panelists are now working toward has to account for the fact that participants are so few and far between, and may not have long to live. "We're changing the traditional paradigm of what has to be there in order to run a clinical trial that cannot be there for rare diseases," said Gordon.

"We don't have 100, 200, 1,000 children. We have to design something where you at least can get a hint, you know, a symbol… We have to design something that works for this population, for these kids."

It isn't easy, by any means. The progeria community is one that's plagued by loss. Every time a patient passes away, Basso said, "it is to lose a brother, a sister." But he's also watched his disease go from being unexplained to having a potential cure — a remarkable biological feat: "Every scientific discovery is like a party. It's a party for these families."

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New modalities, technology and computational tools speed innovation

The life science field is emerging with renewed optimism in the fight against some of the world's most serious diseases, including neurodegenerative diseases. These are some of the most complicated and difficult diseases to treat, and the development of medicines in neuroscience has been marked more by failure than success over many years.

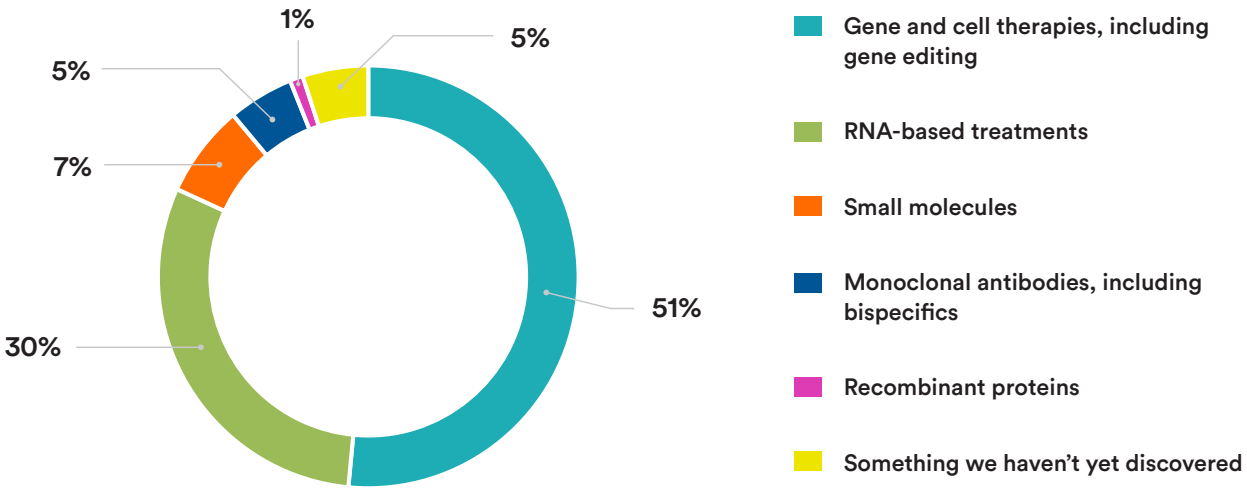
A poll of attendees of the STAT Breakthrough Science Summit showed there's real hope for key biological and medical breakthroughs in the next 10 years. New modalities, increasingly sophisticated computational tools and technological advances are speeding innovation. A growing convergence of computational biology and vast volumes of data is bringing hope to patients by opening new opportunities to treat neurodegenerative diseases and other serious conditions.

Moreover, the pandemic prompted the life science industry to quickly adapt and adopt new ways of working, collaborating and researching. Whether directly battling COVID or navigating around its many obstacles to continuing research and development - responding to the pandemic required speed, creativity and persistence. Unlikely partners worked together to produce COVID vaccines and medicines that may not have been developed as quickly with traditional research approaches.

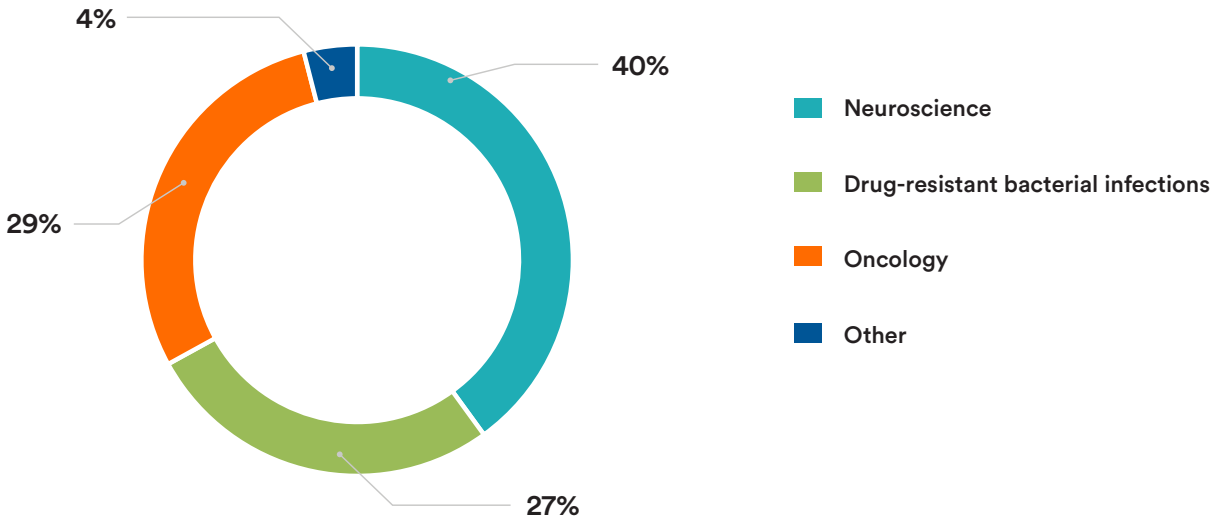
These advances, of course, are welcomed by those they help. But the pandemic has also starkly shown that not all patients are treated the same. There is an urgent need to attack deep-rooted healthcare inequities with the same rigor, creativity and passion brought to the cutting-edge drug discovery process.

At Genentech, we believe that starts with asking the boldest and biggest question: How can we ensure that ALL patients benefit from medical innovation? From there, we will follow the science and data to bring life-changing medicines to all patients. To learn more about Genentech and our work, please visit gene.com/askbiggerquestions.

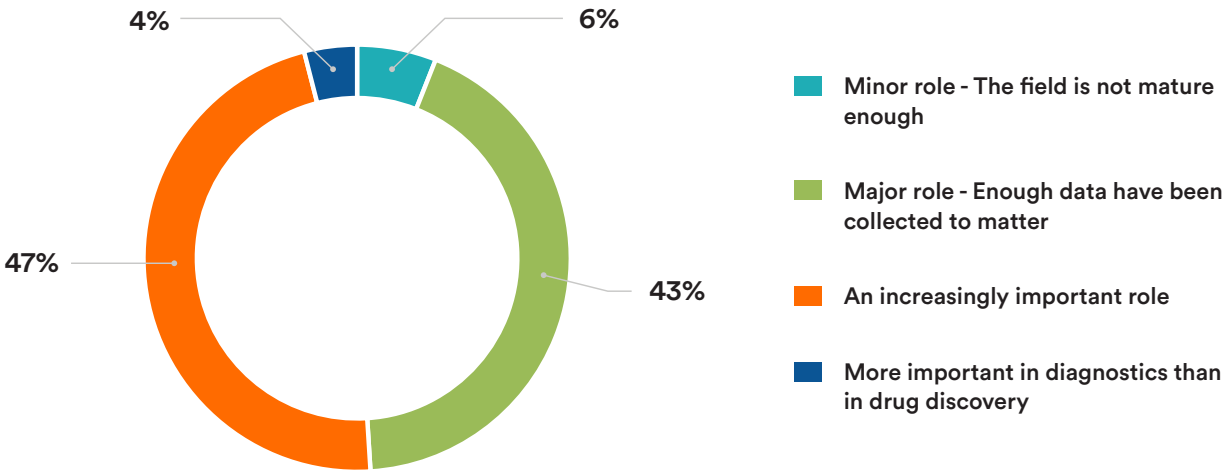
Which modalities are poised to make the largest impact in medicine over the next ten years?



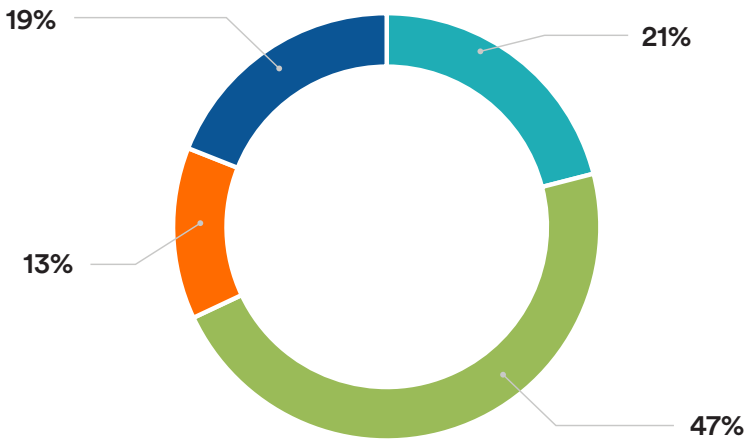
Which tough-to-treat disease area will have the biggest breakthrough in the next ten years?



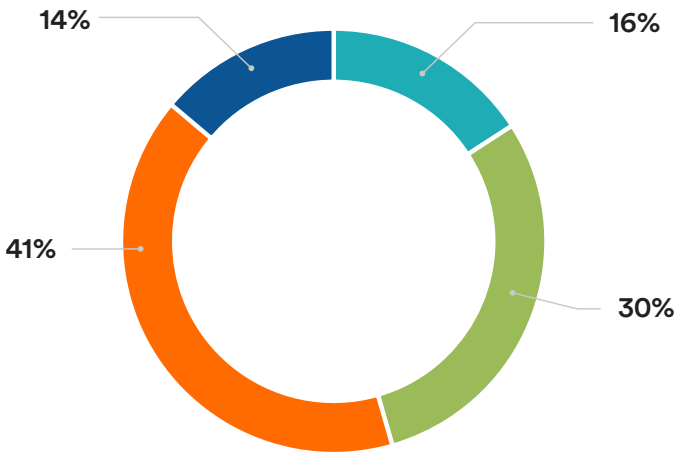
What role will computational biology/big data play in discovery and innovation?



Which pandemic-related trend will have the biggest and most lasting impact on scientific innovation and drug development?



Which of the following is most important to ensuring all patients can access and benefit from medical innovation?



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BIOTECH

Messenger RNA vaccine pioneer Katalin Karikó shares her long journey to Covid-19 vaccines

By Claudia López Lloreda | JULY 19, 2021

In the span of the Covid-19 pandemic, and thanks to the success of two of the currently available vaccines for SARS-CoV-2, messenger RNA, or mRNA, went from being an obscure cell biology concept understood and mentioned only by scientists to being a household term.

But the technology behind the mRNA vaccines from Moderna and Pfizer and BioNTech is anything but new. Developed over an arduous 40 years, it was the result of an unlikely success story. One of the key figures behind this achievement was Katalin Karikó, senior vice president of the German biotech company BioNTech and adjunct associate professor at the University of Pennsylvania.

Speaking at the 2021 STAT Breakthrough Science Summit Wednesday, Karikó shared how despite many, many failures — including demotions, grant rejections, and more — she was clear in her focus. "I always looked to RNA [as a way] to develop therapeutics," she said, and shared details about her journey to BioNTech as well as her unyielding faith in the technology she was developing.

AROUND THE WORLD FOR MRNA

Karikó grew up in Hungary, where at 16 she already knew she wanted to be a scientist, and her dedication to mRNA took her around the world. She wanted to go wherever the best mRNA science was, if that meant academia or biotech, Japan or Pennsylvania, where she worked at Arbutus Biopharma, previously Tekmira. "I was just so determined to go somewhere, do something" with RNA, she said. In 1990, she ended up in Philadelphia, studying the mechanisms of mRNA biology at Penn.

ON PERSEVERING AGAINST THE ODDS

Even though Karikó's breakthrough research has brought her recognition, and with it grant money, her trajectory was not without its low points. For the first 40 years of her research career, she did not receive a single R01 grant, the main way the National Institutes of Health funds scientists. "There were low points, but every time something went wrong, I tried to focus on the things I could change," she said. Seeing progress, no matter how gradual, kept her going. "Whether we got more protein, better delivery, or any kind of data, that gave us the push when we were deep in the problems," she recalled.

Karikó compared the trajectory of science to rowing, the sport in which her daughter Susan Francia, won Olympic gold medals on the U.S. team in 2008 and 2012. Because rowers face away from the direction in which they're headed, "They don't see the finish line, they don't see how far it is, they just kind of sense it. Science is sometimes like that," she said.

WHEN BIOTECH CALLS

Slowly, Karikó started moving into biotech, first by founding her own company, RNARx, in 2006, and then by advancing to bigger companies.

Again, she knew exactly where to go: "I was focusing on companies that already had [mRNA] formulations in humans, because then maybe I could help." She ended up at BioNTech, where she has been since 2013.

Karikó said that biotech has a lot of upsides over academia. "We have to have a product that is functional and will cure people. It was just so much better than a paper, then another paper that maybe nobody will read."

A SHOT IN THE ARM

When Covid-19 hit at the beginning of 2020, BioNTech and Karikó switched into overdrive to develop the elements necessary for a vaccine based on their mRNA biology. After months of work, Karikó recounted how, one Sunday night in Philadelphia — her daughter's birthday, no less — she received a call saying that the vaccine had worked. She wasn't all that surprised. "I was very happy but [it was] kind of expected," she said. Seeing not only "the clinical trial data but also how well this modified RNA worked in other infectious disease vaccines was always so powerful."

Then on Dec. 18, 2020, she got the BioNTech vaccine she had a hand in developing. When she went outside, health care workers who were also getting their vaccines started clapping for her. "They were just so happy. I'm not a very emotional person, but I just cried a little."

ON HER NEWFOUND FAME

When asked her thoughts on possibly being considered for the Nobel prize, Karikó instead focused on the collaborative nature of science and how so many contributions to the mRNA vaccine by others may be overlooked. "Many scientists, just like me, work for years and years and nobody knew about them. And so, I have to represent all of them," she said.

But she is nostalgic for the days before her newfound fame and workload, she said. "Sometimes I wish I could have that [extra time] back. When I read the title of a very exciting paper, I feel that I would never have time to read that, and I want to because that's my favorite thing to do."

THE FUTURE OF MRNA

Karikó sees mRNA, either in the form of vaccines or a therapeutic, as a powerful tool to treat everything from viruses and pathogens to autoimmune diseases, she said. At the beginning of this year, her group published a mouse study showing how an mRNA vaccine could be used to prevent immune system attacks that are common in multiple sclerosis. Although this particular vaccine has a long way to reach the clinic — about two years, she predicted — she believes that her preferred molecule will continue producing new therapies: "I am very hopeful that more and more products will be reaching the market," Karikó said

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POLITICS

NIH's Francis Collins: For Biden's new research agency to succeed, it should prepare for some projects to fail

By Lev Facher | JULY 15, 2021

WASHINGTON — Francis Collins is ready for the National Institutes of Health to fail spectacularly.

At least, he's ready for a few of the agency's potential new projects to go up in flames: the high-risk, high-reward pursuits that would come out of a new research wing that President Biden has proposed. The potential for earth-shattering successes makes the proposed new agency a risk worth taking, Collins said during an interview at STAT's Breakthrough Science Summit.

"Washington is ready," he said. "They've seen the example of DARPA, which has led to some pretty amazing things, like the internet and GPS — but also had some spectacular failures. But people forgive them for that, because of the successes."

Collins also bluntly acknowledged what many of ARPA-H's biggest proponents have recently alleged: That in many cases, the NIH has grown too risk-averse.

The new agency, Collins said, could bring a heightened pace, increased ambition, and a willingness to pursue projects that don't pan out.

The potential new agency, which the Biden administration has dubbed the Advanced Research Projects Agency for Health, is central to the White House's vision for expanding government-funded research and accelerating cures for diseases including Alzheimer's, diabetes, and cancer.

Biotech investors and venture capitalists already know "that failure is an important component of anything that's really going to get you somewhere, otherwise you're just going to be doing the next, obvious thing," Collins said. "That's not what ARPA-H is designed to do. It's aimed to take on [these] really big, bold, hairy, audacious goals that are amenable to a new way of doing science, where you have very bold plans but very specific milestones."

The administration's vision for ARPA-H has come into sharper focus this week thanks to Collins' recent interviews and congressional testimony, as well as an article he and Eric Lander, the director of the White House Office of Science and Technology Policy, recently published in Science.

The new agency, however, has hit its first major snag. While the Biden administration had originally requested $6.5 billion to fund it, House Democrats this week unveiled a spending bill that would provide less than half that amount.

It's not clear whether the immense funding gap is a sign of congressional wariness. Broadly, lawmakers have appeared broadly supportive of Biden's proposal; a key bipartisan duo offered up the full $6.5 billion in a separate proposal just last month. A House Appropriations Committee spokeswoman did not immediately respond to a request for comment.

Lawmakers have also cautioned that ARPA-H should take pains to create a distinct culture from NIH. In a report accompanying the spending bill, the House Appropriations Committee even warned that the new agency "should be housed outside the NIH's main campus in Bethesda, Md."

Collins, on Wednesday, said establishing an ARPA-H headquarters far from the NIH's home base is "on the table."

"If you wanted to make the case that something of this sort, furthermore to emphasize its autonomy and its cultural difference, maybe shouldn't be in Bethesda, but maybe should be in Boston or San Diego, I'd be willing to listen to that," Collins said.

STAT

Extending the reach of gene therapies means hurdling not just scientific barriers but prices, too

By Megan Molteni | JULY 14, 2021

While Covid-19 struck a blow to a vast number of clinical trials around the world, the pandemic may turn out to be a boon for bringing gene therapies to more people in the coming years.

"Who would have predicted three years ago that we'd now be having billions of doses manufactured of mRNA packaged in a lipid nanoparticle?" Sekar Kathiresan, CEO of Verve Therapeutics said Tuesday during the STAT Breakthrough Science Summit.

Verve is currently developing a base editing therapy for a genetic form of heart disease. Similar to the vaccines produced by Pfizer and Moderna, it involves delivering mRNA enveloped in tiny balls of fat. "The manufacturing capacity for those constructs has been expanded dramatically in the last year, so a product like ours, when we get to market, will benefit a lot from that scale happening right now," said Kathiresan.

The hope is that with improved economies of scale, the next generation of gene therapies may not be as expensive as the ones currently on the market. Zolgensma, for example, a gene therapy approved in 2019, is the world's most expensive drug at $2.1 million.

But boosts to mRNA and lipid nanoparticle manufacturing won't be a magic bullet for issues of patient access. Verve's lead candidate involves "in vivo" gene editing, that is, altering the DNA of cells from inside a patient's body. (An important first test of this technique was recently achieved by Intellia Therapeutics, with its CRISPR fix for an inherited nerve disorder.)

But many gene therapies in development do something different: deliver genetic fixes to cells collected from patients before infusing them back in. Orchard Therapeutics is one of many companies working on these "ex-vivo" approaches, for patients with rare genetic diseases. A critical step in the process is conditioning, which removes a patient's defective bone marrow cells and makes space for the newly corrected cells to engraft. But because it leaves people without as many blood cells as they should have, it often relegates them to weeks in the hospital for careful monitoring. After that, they have to go to a bone marrow transplant center, with specialized physicians and staff to oversee the final step — infusing a patient's genetically corrected cells back into their bodies.

"I think that's one of the challenges we will see coming up," said Kathy High, president for therapeutics at AskBio. Multiple companies are currently testing gene therapies and CRISPR-based medicines for treating sickle cell disease, one of the most common genetic disorders. Currently, there are about 100,000 people living with sickle cell disease in the United States, said High. "We do about 25,000 bone marrow transplants a year, and those 25,000 people aren't going to stop needing them. So how is the system going to reconfigure itself to accommodate the demand in that setting?"

While that concern might only apply to the ex-vivo crowd, one issue of access is being felt by everyone in gene therapy — that of reimbursement.

"Again there, you're challenging paradigms, because here you have a one-off therapy that has the potential for lifelong change when you have reimbursement models set up for chronic therapies," said Bobby Gaspar, CEO of Orchard Therapeutics. But he remained optimistic that like every other emerging medical modality, it will get easier over time.

"As we're pushing forward the boundaries and developing new medicines, we have to cross those hurdles and sometimes it's not so easy because you've got to get it right, but the more you get it right the more you get it right further down the line. It's important to remember there's only a handful of approved gene therapies, but as more get approved we won't face these same hurdles with reimbursement and access."