The 2021-22 under 40 Top Scientists: Our 10 predictions for greatness

Eli Lilly's Lonny Reisz, Bristol-Myers Squibb's Samantha Kappler and Novo's Langdon de Jong on what it takes to be a leader

Guest column: What happened with Equrion — and what defines success or failure?

Biotechnology innovation, Medicine, Pharmaceuticals, Venture capital, Artificial intelligence, Drug discovery

# The 20(+1) under 40: Your guide to the next generation of biotech leaders

ENDPOINTS

Fred Hassan, Managing Partner, Rock Health

For a second year in a row, we're launched a 20-under 40 project and arrived, months later, with a list of 21 winners.

The reason for that is simple. We found so much more than we valorize the iron gotten, so it's stubbornly and always a team sport. Like last year, we set out to honor medical innovation and the people that it was made or is made — or in the case of one — died for.

Medicine is a team sport.

There's a long history there: Watson and Crick, Marie and Pierre Curie, Jonathan and Charpentier, Sack, Jesse Lemann and McCormick.

**Guest column: What happened with Equrion — and what defines success or failure?**

The by-board foundation of Hope. We'll do a mission. We plan to organize a "mission." We plan to organize a "mission." We plan to organize a "mission." — and so on and so forth.

MATTHEW PERSON

None of our nominees have quite reached those heights, but there's still time. One also has already pioneered CRISPR diagnostics and is pushing gene editing into new areas. Another is trying to solve the biggest hurdle in gene therapy.

Then again, so might the individuals. We prioritized young scientists and entrepreneurs who haven't yet been recognized for their work, but there's a lot of ground to cover, trying to systematically do justice, a 20-year span of life-saving innovation and a lasting legacy that looks like no other. Science is a 50-year-old MBA trying to fill the gaping hole in our knowledge of human health and genetics left behind by the major corporate mergers that never bothered to sequence genomes in countries across Africa.

Now have gone transformed anything just yet. But they're young, and the conversations can sometimes come slow and then all at once.

As Stuart Schreiber, the Novo co-founder, Broad Institute biologist and mentor to our final under 40 winner, put it, "In this business, it's so easy to just point to the flaws and walk away, but it's all about the next generation of scientists and how they can, and what you can do, to change it, to change it, to change it." — *Jesse Kirkel*

SEE ALL ITEMS: The 20(+1) under 40: Inside the next generation of biotech leaders

## THE ENDPOINTS 20 UNDER 40, 2022

Omar Abudayyeh and Jonathan Gootenberg — MIT

Thomas Ansbacher — Affirmed

Sara Eathiraj — Memorial Sloan Kettering

Sarah Berrett — Dewal Therapeutics

Samira Chen — Arcadia Science

Hannah Dietrich — Elion

Nicholas Fitzmaurice and Nick Gordon — Capsida Biotherapeutics

Isaac Kinde — Exact Sciences

Hyun Kim — *Ibid*

Sean Kim — Dewpoint Therapeutics

Ron Korstanje — Agenus Therapeutics

Claire Mazumder — Bruno Therapeutics

Dina Radenkova — Genetic

Aaron King — Salo

Lin Rouse — Alto Bio

Nadiia Sabanyo — Caribou

Dwarf Talendeker — Kyte Therapeutics

Tessa Yang — Sonata

Vasanth Vivekanan — Kaiji Therapeutics

**Omar Abudayyeh / Jonathan Gootenberg**

**MIT, Harvard Bio**

**Investigator, Co-Founder**

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## *Two college buddies try to break new ground in CRISPR, diagnostics and a whole lot else*

Omar Omar Abudayyeh and Jonathan Gootenberg met in undergraduate at MIT, where they bonded over their shared interest in pounding their days together nerding out about, well, the kinds of things that building bioengineers nerd out about. Gene editing, synthetic biology, computer science, even ways to put to sleep the rest of us, who can't see ultraviolet light and have trouble changing their nights by. Things The protein sequencing, super-resolution microscopy and DNA origami.

"We met for the first time brainstorming crazy ideas," Abudayyeh said.

In a few years later when Abudayyeh — who was on the research phase of his MD-PhD program — found himself back in the same lab as Gootenberg, a PhD student, they shifted back together.

Again, they were dreaming up new ideas. By then, 2016, CRISPR gene editing had been invented and they wished to be left out of one of its pioneers. Sorry, it was all about genome engineering a way you might drop in long stretches of DNA into an organism's genome, or a method to manipulate entire cells.

But soon they converged on a simple project that would become one of their time, at least, and a technology that would become a mainstay of their lives, and of ours, and we found a future. Going data generated by one of Zhang's colleagues at the Broad, they identified a new CRISPR enzyme, eventually called Cas13, that did things no other CRISPR enzyme did.

It targeted DNA, instead of DNA, and it didn't make a precise cut like Cas9. Just the opposite. It's inclined into the right RNA sequence, it went there, unleashing any genetic material it could reach.

It was just as different."Continuing said." "There was just so much new library."

With Zhang's help, it became the basis of a new approach to diagnostics. The concept was simple. Most virus genomes are made of RNA. The CRISPR tool has two things: It can find a specific sequence of genetic material, and it can cut everything else. If the DNA that, where, will send off a fluorescent glow. If the Cas13 detects that sequence is a sample, it will go haywire, cut the so-called reporter RNA and set off a glow that signals a positive test.

The test became the basis for Sherlock Biosciences, which has now raised around $200 million to develop tests for cancer and infectious disease. That makes work Abudayyeh and Gootenberg contributed to for a test for Covid-19 in the early days of a pandemic.

Although the test never lived up to the hype — Zhang and others initially talked about it as a home test, a kind of pregnancy test for Covid, but it never came close to that simple ease, it represented the first over-FDA-authorized use of CRISPR technology and provided a proof of principle of Sherlock's larger goal of making tests for cancer and infectious disease simpler, easier and more affordable.

Sherlock also prompted Abudayyeh to abandon his medical ambitions. Although he is a practicing MD, he decided he wanted to focus on research and teaching, and he has spent much of his time in academic medicine, known as the Mendel lab. Although it's been a tough year, with the pandemic and the Russian invasion of Ukraine, he finds time among the only physical jobs in the country.

It works well, mostly, although it has its quirks. For a student, it can be like having two parents and getting different instructions from each. "We don't always agree," Gootenberg jokes. But that can be good, a refreshing disparate from the usual under-40 winner, who has been in the lab since high school and has never left. Omar: "There is an absolute crash coming down from, you know, the chaos."

The two baby-faced professors — though now 35, each could really still pass for undergrads — have worked toward many of the fundamental ideas they imagined as students. They've created a tool that can find and cut DNA and RNA and that can unleash dog thousands of hours into the genome of an organism.

They've also randed into aging research and now spend much of their time focused on cell engineering. It's the next big step, they say, after the genome, after the cell.

"Continuing, I think, is one of the strongest things you can do," Abudayyeh said. "When you start thinking about cells and engineering cells, they're much more complicated. And a lot more can go wrong." — *Jesse Kirkel*

**Thomas Ansbacher**

**Affirmed, Flagship**

**Founder and President of venture Office, Principal**

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## *A tenacious biologist searches for a Swiss army knife solution against numerous rare diseases*

Thomas Ansbacherwith his in love with biology in middle school in Frenze. But when she realized in high school that most of biology wasn't lovely, she said she really left in love.

"From that moment on, I decided to dedicate my life to discovering novel biologics and applying those to help patients," she explained in a recent phone interview.

So she transitioned from the French to American school and, after graduating from Yale, she set her sights on the scientific rocket ship of Silicon Valley and spent her first stints at the National Cancer Institute and the Mel Gibson Cancer Institute.

She took that experience into a PhD program in cancer biology at the University of Pennsylvania, which is where she first discovered the wonders of the Dutch tobacco fly, Flagship and its three-month fellowship program.

Ansmannsbahn and you handled it without the fellowship then leveraged their work into full-time jobs at Flagship. But it was while she was a fellow that this new idea for a biotech company came to her.

One of the teams she was working on was exploring chemosens, the protein producer-tree machine in the cell, and she said that during that exploration she stumbled across AHNA and AHNA biologics.

"So I came up with that idea as a fellow and presented it as part of a program," she said, noting that she emailed Flagship partner David Berry that "this is a cool area to have someone follow up."

Berry liked the idea, and he and Ansbacher teamed up to form Affirmed, which comes to the Ansbacher with instructions to build a platform, AHNA tools and a pipeline for corresponding amino acids to construct the protein bit bit.

A lot of different rare diseases are caused by a particular nonsyndromic AHNA that accidentally rolls the AHNA to stop constructing the protein. One strategy for treating these diseases is an AHNA or gene therapy that replaces the missing protein.

Alternatively, you could just replace the other non-textual. The promise of this approach — or AHNA therapy — is that, because so many diseases with the same gene, the same misspelling, you could treat many disorders with the same drug.

When she joined the firm in January 2018, Berry "basically said if you think it's cool, take it on," she said. And then, Affirmed was born.

"If you look at a RNA, it's 50 form, it kind of looks like an upside-down U," she explained, noting that it's hard to sequence and measure, and it's extremely difficult to wrap and can break into fragments that have rolled into outside of translation.

"So if you think about the main basic experiments," she said, "there's a lot of work that's very low and far between.

As co-founder of Affirmed, Ansmannsbahn said she and her team had to learn a lot of scientists to build these tools to ask new questions and explore AHNA biology.

By the end of 2019, she said that this big scientific foundation was there to support a biotech, and that's when the company could use a program Affirm ran to set up multiple shots on goal.

"I started to really quickly imagine a world where you can propose these molecules made with 50 complexes and 100 million-plus Flagship. The Tulip is also proposed with tailor interest, an AHCN, Tadalafil and other VC-back strategy in the space.

Now, with a new CEO at Affirm, the company is looking to focus on its rarer spots.

"The beauty of the platform is to create multiple assets from the same process," Ansmannsbahn said. "You can do 10 different molecules, and the same process, the platform, the first generation is maybe applying engineered AHNAs to be able to correct underlying nonsense mutations. And then second generation would maybe use different pools of AHNAs to change what transcripts are being translated or also biological processes dysregulated in cancer or other diseases." — *Ben Kolata*

**Sam Rabadan**

**Memorial Sloan Kettering, Velastra**

**Investigator, Co-Founder**

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## *A curious doctor investigates an age-proach to stopping metastatic cancer in its tracks*

Sam Rabadan was a young boy, Sam Rabadan was surrounded by a variety of medicine and science — at least, in part thanks to his doctor parents. His mom was an internal medicine specialist, his dad was a pediatrician and an OBGYN. Every day before school, his dad would drive him to work, and he would spend the morning there, sitting in some together. However, there was a little bit of a rule — dad's encyclopedia — with a bit more oomph on the ex-star side of things.

And while Rabadan admits that his dad, who was born in the Philippines, didn't quite have the encyclopedia in his arm, he also feels that his dad would come across of curiosity in him. And so, that his traditional of curiosity was not only successful, but he ended up being

## *First-time biotech founder, CEO of mRNA startup pushes to be 'example' on age-up time' in advocacy for founder-led biotechs*

When Jake Becraft was growing up in a central Illinois farming community, he didn't exactly dream of being something in medicine. Becraft's community was disproportionately impacted by cancer and diabetes. As a kid, he watched friends and family fall victim to them.

In high school, though, he didn't even like biology — mainly because it was taught in a way that made it seem impossible to fix. Only in college, when he dabbled in math and physics, philosophies that "had deeper and apply to specific situations.

"So I didn't even know, I guess, obsessed with engineering. And at the time, I thought I was kind of a nerd," he said. "And basically what ended up happening was that my guidance counselor said, 'Well, you like math, you like physics, you like, you know, you're good at math. Have you thought about chemical engineering?'"

But in an undergrad at the University of Illinois, he got his first exposure to gene therapy and fiddled with a Bachelor's degree in both chemical and biomolecular engineering. Becraft ended up in a lab that was developing bacteria that could be tweaked, handing and turning into two mRNA-focused biotech. Perhaps most notably, he became an advocate for the growing notion that more biotech companies should, like tech companies, be run by their founders.

He went to get a PhD in bioengineering at MIT, where he met Tony Kidema, who is now his co-founder at Tessera. Kidema, a PhD student, was working on a technique for bacteria, called Gene Sets, which helps from Becraft and other, published as an up the topic last August, Mostly titled "The future of biotech is founder led." It argued that biotech can only succeed in the future if founders, who are the creative young scientists who generate the startups to begin with.

Becraft also wrote Tessera Kidema, who he recently connected up with to try to turn with his medicines, which had the point to be the Ind-driven and yet-broken. Who is the Ind-driven and yet-broken? Well, Becraft and Kidema, who are now co-founders, looking in the CEO and he in the chair, with his first drug candidate for cancer. That drug, if it works as planned, would inject mRNA into a tumor and essentially convert the tumor into a factory, producing a powerful immune response.

So he now spends his days both heading a biotech with 30+ employees at the latest center and spending time on Twitter advocating for founder-led biotechs.

The idea holds that, in Becraft's words, you can build a better biotech economy by "kind of compounding from the start and onward — and in some cases, a different mind set and culture dynamic.

This is in deliberate contrast to how many biotech startups are often formed and go-ended, where VCs become the science and bring in a team of more experienced people, "and sometimes that's great, but it's not the end-all, be-all, and it's not necessarily best 'to the professionals do it." Becraft added.

While proponents argue that holding companies this way de-risks drug development, it comes at the cost of diversity of thought, Becraft said.

In other words, traditional VCs might miss major innovations. Becraft argued it's akin to what Morgan Stanley pointed out in the late 1990s and early 2000s, when the investment bank noticed that the VCs, in their rush to fund companies, were missing the big things of the time, such as the genomics revolution.

So Becraft, one of the key differences is that founders can have much more of a vent and a different kind of perspective.

"The importance is really, that when you have a company that has founders that are a mission, set a vision, and are there every single day, working, working, feedback, they're going to make the right decisions, and they're going to do it," he said. "And they're going to, it's about trust and execution, right? That's how you're going to get those drugs forward."

In his time chatting with people on Twitter within the VC space and elsewhere, Becraft has found that the success he has had with his company as a first-time founder makes him just an exception — and it shouldn't be an exception, he said.

But that thinking, Becraft thinks, is fundamentally flawed. The only reason he and Kidema were able to bring Tessera Therapeutics to where it is today, Becraft said, is because they gotten the opportunity to lead and help their vision into building the company.

Other academics can do the same, he said, if given the resources and opportunity.

"I would rather be an example than an exception," Becraft said. — *Paul Schiavoni*

**Sonnay Chen**

**Arcadia Science**

**Co-Founder, CSO**

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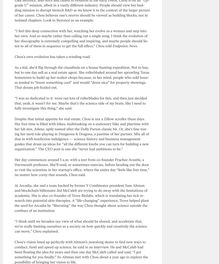
## *A tick biologist and Beyoncé lover tries a new approach to science*

There's not a billion dollars on the elevator at Arcadia Science.

But Sonnay Chen's startup has given her half that so far, and she's using it to pursue a different approach to doing any science in a unbiased, a lofty goal in which journal publications are no longer prized as gold, scientists share all their work openly and the research process is redesigned to speed the pace of innovation.

With $500 million, among the largest funding rounds raised in Biotech history, the professor turned CEO's company is trying to do what Chen herself has done: change the industry's opinion about what. Chen, Beyoncé and Sara Biotechnology, though the promise to forms and ambitious will be far flat.

"Given my personality, it's hard to imagine me not pivoting at some point, especially because I'm a person who are historically nonplussed — or nonsense group of cities — SFAC and reality a system optimized for you. And I do feel this intense urgency, and I'm impatient, and I want to see, I want to see, I want to see,



# Sam Altman's Bet

## The Man Who Wants to Remake Silicon Valley

Sam Altman's company, OpenAI, is worth more than a hundred billion dollars. He's been in the tech business since he was a teenager, and he's made a lot of money. But he's also been a prominent figure in Silicon Valley for years, and he's been trying to remake the tech industry in a way that has nothing to do with money. He's been trying to make it more ethical, more transparent, more accountable, more democratic. He's been trying to make it more like himself.

Altman is a tall, slender, soft-spoken man with a mop of curly hair and a boyish, almost innocent face. He's married to a doctor and lives in a mansion in the Marina District of San Francisco. He's a vegan who doesn't drink or smoke. He's a Jew who doesn't practice his religion. He's a liberal who doesn't talk much about politics. He's a billionaire who doesn't act like a billionaire. He's a tech mogul who doesn't act like a tech mogul. He's a man who doesn't act like a man who wants to remake the tech industry in his own image.

Altman's company, OpenAI, was founded in 2015 as a nonprofit research lab. The idea was to develop artificial intelligence in a way that was safe and transparent, and to share the results with the world. The company was funded by a group of tech billionaires, including Elon Musk, who donated a hundred million dollars. But the company soon ran into a problem: it couldn't raise enough money to compete with the big tech companies, which were spending billions of dollars on A.I. research. So in 2019 it created a for-profit subsidiary, which could raise money from outside investors. The new company was still supposed to be a nonprofit in spirit, and its mission was still to develop A.I. in a way that was safe and transparent, but it was also a business, and it was trying to make money.

Altman was the company's chief executive officer, and he was also its most visible spokesman. He was a charismatic figure, with a gift for explaining complex ideas in simple, reassuring ways. He was a skilled fundraiser, and he was good at convincing people that OpenAI was different from the other tech companies, that it was more ethical and more transparent and more accountable. He was good at convincing people that he was different from the other tech moguls.

But is he? That's the question that has been hanging over his career for years. Is Altman a visionary who is trying to save the world from the dangers of artificial intelligence, or is he just another tech mogul who is willing to say and do anything to get rich? Is he a man who wants to make the tech industry more ethical, or is he just a man who wants to make himself more powerful? Is he a man who wants to make the world safer for humanity, or is he just a man who wants to make himself richer?

The answer, of course, is all of the above. Altman is a complex figure, and he's been a complex figure for most of his career. He's been a tech mogul who wants to make the tech industry more ethical, but he's also been a tech mogul who wants to make himself richer. He's been a man who wants to make the world safer for humanity, but he's also been a man who wants to make himself more powerful. He's been a man who wants to have his cake and eat it too.

The story of Altman's career is the story of a man who has been trying to have his cake and eat it too for almost a decade. It's the story of a man who has been trying to make himself richer while also trying to make the world safer for humanity. It's the story of a man who has been trying to make himself more powerful while also trying to make the tech industry more ethical. It's the story of a man who has been trying to have his cake and eat it too.

Altman's career has taken him from a small apartment in San Francisco to a mansion in the Marina District, from a small company with a few dozen employees to a company with thousands of employees, from a company that was worth a few million dollars to a company that was worth a hundred billion dollars. He's been a tech mogul who has made a lot of money, but he's also been a tech mogul who has been trying to make the tech industry more ethical. He's been a tech mogul who has been trying to make himself richer, but he's also been a tech mogul who has been trying to make himself more powerful. He's been a tech mogul who has been trying to have his cake and eat it too.

The story of Altman's career is also the story of a man who has been trying to make himself more powerful while also trying to make the tech industry more ethical. He's been a tech mogul who has been trying to make himself richer while also trying to make the world safer for humanity. He's been a tech mogul

Klotz first got into condensed biology, he said, because the well of more classically developed cancer drugs was starting to run dry.

"We've been around the other twin of the world and cancer across condensed biology, which offers "a whole new world and a whole new way for developing cancer drugs."

At Genoptera, Klotz is working to refine the company's platform, which can be deployed to find the condensed biology underlying diseases ("I mean, the diseases are," Klotz adds and discover small molecules that can modify it for some therapeutic benefit.

"We're still a couple years away from starting human trials," Klotz said, but he noted that Genoptera is working in cooperation with pharmaceuticals companies and work with Genoptera's platform.

The company already has collaborations with Merck in NY, Bayer in cardiopulmonary disease, and Pfizer in immunogenomic diseases, and it's looking at new partnerships too, he said.

As far as competitors in this new space, Klotz pointed to Fate Medicines and Recursi Therapeutics, but he said neither offers the same kind of platform as Genoptera.

"They're more directed at specific diseases, and there seems to be condensate-related, rather than that biology broader and apply it widely," he said.

So why expand Klotz to make this big bet and go take a risk? Klotz points to Bayer and Pfizer's investments.

"The advice he gave me that I try to stick to today is: Don't go for incremental progress. Imagine what the world is and then see if you can make it happen. Go big, go bold, take a big step and see if you can make it. The best science fundamental discoveries are what we think about and approach a problem," Klotz said. *Zachary Brennan*

**Tim Kamenova**
**Agentab**
**CEO**
**27**
**Toronto, Canada**

## *From 'glamorous' VC investor to startup CEO, Tim Kamenova jumps into the day-to-day work of drug development*

Tim Kamenova enjoyed working with biotech entrepreneurs and investors during his time at Venrock, but he said he had had his eye on a startup.

"I really wanted to be as close to the center of the action as I could," he said in explaining why he chose to leave the Bay Area for Belgium biotech Agenta Therapeutics three years ago.

At Agentab, Kamenova is doing exactly that, overseeing the out of antibodies for organization on tissue target actions a range of conditions.

Both of Kamenova' parents are biology teachers, so he grew up surrounded by life sciences and education. However, he didn't become interested in biotech himself until the second half of his university studies. That's when he decided he wanted to pursue a career on the business side of drug development.

He graduated from Utrecht University in the Netherlands with two master's of arts degrees — both with honors — first in science and innovation management and the second in drug development.

He joined Sanofi Venture as a senior associate and began investing in European medical companies.

"I felt like it had a comfy, more, learning about all these cool new technologies, speaking with entrepreneurs and learning from my colleagues. That was just an amazing start to my career," he said.

But over time, he realized the most exciting part for him was working with management teams of the portfolio companies. That's when he decided he didn't want to be an investor, but switch to more active role in the drug development process.

"Most people actually don't fully understand it at the time because I left my glamorous venture life behind me and joined the other side of the table, but now, I am 30 BILlion the right move at the right time," Kamenova said.

He joined Gilead Sciences to oversee development in 2022 where he not only raised more than $200 million in a private financing round, but oversaw a phase 2 study of a drug candidate that ultimately failed. Kamenova said he learned a lot from that experience — and that it was better to be open to acquisition deal with Pfizer — and he Kamenova, who earlier. He did an MSCn Switzerland as he was figuring out what to do, finishing an telecoborn on.

Around the same time, S-Ventures and pharma company aspects both reached out to him about a new company they were setting up, the new Boston Gene Medicinals, now Agentab's chief scientific officer, to talk about his research with MIT. Kamenova, MIT co-founder and Harvard Medical School professor Gerbrand Ovigne.

"After I met with him, it was clear that there was so much potential for the technology and that together, from scratch, we could really build a great company going forward," he said.

Kamenova worked quietly behind the scenes until 2019 when he became Agentab's CEO.

He's overseeing the continuing effort using antibodies for regenerative and tissue repair, including a project with Pfizer. Agentab is also working on a cancer project which the biotech has prioritized promise. is a range of autoimmune, inflammatory and fibrotic disorders.

He also shepherded the recent acquisition of Spanish biotech Origin, using two pipeline assets and increasing a clinical stage company enterprise.

"It was a single-pharmacist stage asset company acquiring a multiple-asset clinical stage company on the way analysis," he said.

Kamenova counts industry advisors John Maraganore and Tim Van Zuiverenburma among his mentors who listen to his ideas and ask questions. Kamenova also strategy is BIG: He also credits Erik van den Berg, CEO at Gilead Pharma, for showing him how to lead authentic leadership" in pharma.

"I was a male CEO in Agentab — and I still am and make mistakes, but I've learned a lot. I'm very, very, very,

## A biosignature with a personal history in rare disease tests to tackle one of the biggest hurdles in gene therapy



The overcrowded pre-Crime Rotten profile in the New York Times Best Sellers Crime Fiction Hardcover Rotten Crime Fiction Collins highlighting the research.



But Hazel Tabeborshaw, who's recently crowdfunded the A.M.Tunnel gene therapy Hazel Gene Therapeutics, has had both.



While modelling the verdict of academic research and the biotech industry, Tabeborshaw made clear in a recent interview that it's his love for the work that drives his week after.



"I really enjoy the work. I do, so that's what drives me," he said. "I dream a lot about the day we inject our first patients and change their lives. These are kids that we go to bed to a much happier life."

Born in Heinz, Iowa in 1966, Tabeborshaw said his mother's focus on education, and his father's struggles with a rare genetic muscular disease hastened his interest in the field. He also profiled, pushed Tabeborshaw to plug into science early on.

"This is rather people are born with these genetic diseases that they have no control over, and that fuelled my passion for biology and practice," he said.

So after studying biotechnology in undergrad and for a master's, he then applied to the 10 schools in the US that have accredited doctorate PhD programs, and he got into Harvard.

That's where he said he worked on in vivo gene editing, immune responses of CRISPR in muscle, as well as the entire gritty of muscle biology and different therapeutic strategies in different models of disease.

After his PhD he said he wanted to go into academic research, but instead jumped at an opportunity to lead a gene therapeutic program at Editas Medicine, which focuses on developing CRISPR-based therapeutics.

Only about a year later, Editas decided to reorganize and told Tabeborshaw that they had to scrap the program because there wasn't yet a good enough delivery mechanism to get these components into muscle.

"And that was a fair place," Tabeborshaw said. "And this wasn't going to be a problem for only this company, but for the whole field.

"So I decided to tackle that," he said, delving into a discussion of the unmetabolical challenges that the field now faces.

Among the challenges: identifying the underlying genetic issue (which in the case of Tabeborshaw's disease, Duchenne muscular dystrophy, is a mutation in a gigantic gene the tome), manufacturing that much virus, figuring out whether it's enough might be necessary, and the elephant in the room: safety.

"We know that if you get the immunogenicity into the body, it's going to work, it's going to have a therapeutic effect. It's all about can you make it to the patient," he said. "Safety is what's at stake.

Can we deliver drugs that are potent enough that we can go in a low enough dose that doesn't result in the toxicity issues that we're seeing? There have been multiple fields in the last year and a bit of them are associated with injecting an extremely high dose of virus because those naturally occurring viruses are actually delivered in low dose.

They gave therapies have seen FDA approval so far (Roche subsidiary Spark's Luxturna and Novartis' Zolgensma) but Tabeborshaw is optimistic and said the engineering advances for genetic medicine diseases will be approved in the next 5-10 years.

"The first ones will be gene replacement therapies for recessive diseases that we just talked about, and then we will start tackling the harder ones, the dominant diseases, which is going to be more challenging, particularly for the muscle, because of the tolerance," he noted. — *Zachary Brennan*



## A young scientist pursues one of medicine's biggest Holy Grails: A pill for sickle cell



On long days, when the experiments just don't work or the latest batch of medicinal bacteria, Pamela Tiga tries to remember her days as an aspiring journalist. There's a lesson in that, she tries to assure herself. Tiga's pitch tapes explain.

Tiga often has such recursive. As a senior scientist in the Immunology unit of Novartis' biomedical research wing, she is leading an effort to develop small molecules drugs for sickle cell disease.



It's become in recent years a Holy Grail target for drug discovery, after decades in which the scientific community has shied away from it.



Tiga's initial focus on stem cell transplants, the treatment that small molecules that could expand a pool of cells, improving the efficacy and efficiency of such treatments. Tiga's team at Novartis, which is based in Cambridge, Mass., has been working on a lead small molecule known C-202019, although known by its code name, the gene-editing technique could also be used to find new drug targets, Novartis said. Tiga's team is also working on other small molecules that which they can target cellular cause cells or screened inflammation.

But it was still the early days of the CRISPR revolution. Tiga and the other Novartis scientists struggled just to synthesize CRISPR proteins, namely Cas9, in that little, and we can could a little to such in Short term cells, a notoriously unstable cell type.

One day, though, Tiga figured out that if you gave the stem cells Cas9 with the guide RNA, they could take up the proteins and edit themselves. Tiga's team, in a collaboration with Novartis, has been working to develop a therapeutic approach

Not that he didn't want her to join, but for Keli, the decision came with careful consideration about how it might affect their family, which includes four children. Especially when it came to heading up the launch of Tepezza in early 2020.

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April 19, 2022 10:30 AM EDT | People

## Bear market shows no signs of abating, claiming Finch Therapeutics as the latest layoff victim

**Max Gelman**
Senior Editor

Much ink has been spilled over the continuing wave of layoffs hitting the biotech industry, and that doesn't look to be stopping anytime soon.

Finch Therapeutics reported Tuesday morning that it would be laying off 37 staffers, or approximately 20% of its workforce, per an SEC filing. The decision to cut costs comes about a month after Finch said the FDA had placed a treatment for *C. difficile* infections on partial clinical hold, and roughly three weeks after pausing its hepatitis B program.

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April 19, 2022 09:54 AM EDT | R&D, Coronavirus

## Covid-19 roundup: Moderna showcases progress on its bivalent booster; Novavax wins approval in Japan

**Tyler Patchen**
News Reporter

New data from Moderna offer encouraging results for the mRNA powerhouse's bivalent booster candidate.

The bivalent booster candidate, known as mRNA-1273.211 and which includes mutations found in the Beta variant of concern, bested the company's original booster in terms of neutralizing titers against all variants of concern, including Omicron.

The news, which follows similar results for the Pfizer/BioNTech Covid-19 vaccine when combined with Beta, bodes well for the booster in the fall.

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April 19, 2022 07:34 AM EDT | FDA+

## Axsome agrees to post-marketing commitments with FDA, signaling potential OK for long-delayed depression drug

**Zachary Brennan**
Senior Editor

Axsome Therapeutics is likely on the cusp of its first drug approval, sending shares of its stock up more than 40% pre-market on Tuesday.

The company said in an SEC filing Tuesday that it has agreed to certain postmarketing requirements/commitments proposed by the FDA with respect to the company's application for AXS-05 for the treatment of major depressive disorder.

That application has been delayed since August, with the company receiving a letter outlining how FDA-identified deficiencies preclude labeling discussions with Axsome.

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