Science AMA Series: We're reporters for the news team at Nature. From GM crops to customized pets, patent disputes to CRISPR babies, we're here to answer your questions on genome editing. Ask us anythi

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Hi guys! As a biotech grad, I have had the opportunity to hear (at length) the dangers and frustrations relating to the patent system as it works and could work in the biotech industry. My questions to you are:

What are your general views on the current state of the patent system in regards to genomics and gene technologies?

And, in regards to the patent system, what issues to we need to get ahead of in order to prevent potential future problems?

jourdan442

I'd say the current state of the patent system with respect to genomics is in flux. As several people have commented here, there have been major changes in how genes and genetic tests can/cannot be patented. Patent attorneys (and the USPTO) are still working their way through the implications of all of that. For CRISPR specifically, the big, ongoing patent interference fight is a remnant of an old system that has since been replaced in the US. And as for problems with the patent system more broadly, I often think a lot of it could be cleared up if it were only easier to figure out who has licenses to what patent, what the patent claims actually claim, and so on. But I suppose that's what patent attorneys are for. – Heidi

My question is in the foreseeable future will genome editing be widely available to remove genetic disorders or mutations from babies as a prevention from genes that cause health problems from being passed down. My husband has an autoimmune kidney disease that was inherited and I have Factor V Leiden blood mutation, so I'm curious if it will ever be something insurance would cover in the future or if it's too expensive and impractical to use widespread to prevent health defects.

Acrophobe

Based on what we know now, researchers are by-and-large saying there are actually very few diseases they would want to treat using CRISPR-Cas9 editing in embryos. Instead, they generally prefer

treatments that would be administered later in life. Tinkering with the genome of an embryo poses a few unique technical and ethical challenges, including the fear of unintended consequences that could be inherited by generations-to-come. As for how patients and their advocates feel about that, I really loved a story that my colleague Erika Check Hayden recently wrote about the nuances that could shape their decisions. And as for how insurance companies feel about it… phew. I haven't a clue. --Heidi

Firstly, I want to say thank you for doing this AMA. It's got to be quite hectic working for Nature *and* taking time from that schedule to answer a slew of laypeople's questions about a complex gene editing technique.

My question to you is this: how effective can CRISPR be in vivo (if at all? Is it a technique that needs to be engaged at the embryonic stage of an organism's life, or can we edit phenotypes in children and adults?

Ungodlydemon

Possibly very effective, but it hasn't been tested in clinical trials yet. Editas Medicine, one of the CRISPR companies that has popped up recently, plans to test it next year in humans with a retinal disease. But there is plenty of evidence in animal studies that injecting CRISPR, or replacing cells with CRISPRd ones, could treat adult diseases. For instance, injecting CRISPR into the muscles of mice models of Duchenne muscular dystrophy and editing the protein responsible for the disease appears to have helped with the disease's symptoms. Of course, there are still safety issues to be worked out – we don't know whether the human immune system could attack Cas9 enzyme, for instance. -Sara

What are the biggest faults and inadequacies of Crispr? I understand it is not any kind of perfect tool, for all the benefits it brings.

Whats the next model of the "tool" going to be? Anyone working on better versions already?

SurfaceReflection

People I've spoken to have coalesced around three major beefs with CRISPR-Cas9: 1) it's easy to make a deletion in a gene; not-so-easy to insert a new DNA sequence of your desire into the genome; 2) sometimes it makes mistakes; and 3) it can be tough to deliver into mature human cells (for therapies). The field is moving quickly to make improvements on all three fronts, and new-and-improved CRISPR-Cas9 techniques are emerging at a breakneck pace: molecular tricks to make it easier to insert sequences; less error-prone Cas9 enzymes; smaller Cas9 enzymes or novel delivery methods to make it easier to cram it all into a human cell; and more. --Heidi

Hi Heidi and Sara, thanks for the AMA!

My question: With CRISPR democratizing the access to gene editing, are there concerns that the technology is now accessible to those who might use it irresponsibly? How do we make sure a would-be Dr. Moreau can't get carried away and start doing unethical work without any of the checks and balances that exist in the formal research environment?

superhelical

A lot of people share your concern. We recently had a goodcomment piece from Todd Kuiken at the Wilson Center who took a look at risks posed or not posed by the DIY Bio community. (I do think that creating Moreau-ean creatures, at least the ones that I remember, would still pose a significant

challenge, even with CRISPR-Cas9.) From a biosecurity standpoint, I think a lot of these worries aren't new – anthrax was weaponized pre-CRISPR, scientists engineered a more dangerous avian flu without CRISPR. But the CRISPR discussion is bringing some of these concerns out again, and it's probably a good reminder to revisit the topic. One concern that is somewhat unique to CRISPR is the issue of 'gene drives' – engineering an organism so that it can spread a certain mutation throughout a population much more rapidly than usual. That was a much more difficult thing to do pre-CRISPR and the topic is getting a lot of attention now, both from individual scientists and the National Academies, which are looking into the risks and benefits posed by the approach. -- Heidi

I'm a college student, and I recently did a presentation on genome modification for a class. I read that Chinese scientists had used CRISPR and other techniques to modify human embryos. Are there any plans to conduct similar experiments in the U.S., or would there be too many ethical laws to work around?

Gadgetman914

The laws on human embryo modification are complicated and vary from country to country. The experiment in China was perfectly legal there, but may not have been in some European countries. Heidi did a great roundup here. In the US, human embryo modification is legal, but the federal government isn't allowed to fund it. And a researcher in the UK recently got permission from the government to modify human embryos in her lab. -Sara

Hi, thank you for the AMA.

I'm a Biology undergrad student with great interest in scientific journalism, and I'd like to know if you have any career tips for me. Should I do Journalism after I graduate, or jump right into a Masters and then PhD in Biology? How was you guys' career paths? How do you evaluate the current panorama for scientific journalism in the US, specifically compared to academia? Can you recommend me websites and discussion groups to stay updated on the field?

Thank you!

jmneri

Oh, I wouldn't get a PhD unless I really wanted to be a scientist! (That's what I wanted to do when I started mine.) It's a long, tough slog if you don't intrinsically enjoy the work. I stuck it out to the end because I did enjoy some aspects of it and adored the people I worked with. But I suspect that if you want to be a science journalist, you probably already have enough of a science background to do a great job. (At least one of the best science reporters I've known had no science degree at all.) Just my opinion. Here's a good blog post with more opinions. I also really like this book and this blog. -- Heidi

Hello Heidi and Sara!

I have a question regarding CRISPR. A MD friend of mine who works in the field stated to me that CRISPR most likely will not be a viable way to successfully edit genes to give someone blue eyes, dark hair, etc. for another 40-50 years most likely. Is that time frame something that you agree with?

orlyfactor

I'm going to wimp out on estimating a time frame, but I can say that many of the traits people talk about altering — like hair color, height, or a lot of really important common diseases like schizophrenia or diabetes — will be pretty tough to tinker with. Often they are governed by more than one gene; often

we don't yet fully understand which/how many genes are involved. The medical applications will likely start with simpler fare. -- Heidi

What's the most impressive successful trial that has occurred with the help of CRISPR? How helpful will it be for editing out diseases that have already taken place, such as schizophrenia?

anonymouse0_0

You'd never guess it from the way investors have flocked to the technology, but to my knowledge there have been no clinical trials of CRISPR-Cas9. Editas Medicine, a CRISPR-Cas9 company in Cambridge, MA, has said that it hopes to begin trials in 2017. CRISPR Therapeutics (Basel, Swizterland) has estimated it'll be about 2018 when it begins its trials. There have been clinical trials of gene-editing via different methods (called 'zinc finger nucleases' and TALENs). Sara wrote about a few of those here. As for schizophrenia, that's going to be a tough one. We're only just beginning to understand the genetic links to schizophrenia, and it's likely to be a very complicated picture. -- Heidi

Is there the possibility of accessing genes from previous evolutions to create a predecessor to the animal that was being edited? Aurochs for instance.

Would gene editing, in effect, force evolution to move at a faster pace provided the offspring weren't sterilized?

weatherseed

"If I understand your question correctly, then yes. I'm not aware of anyone trying to modify aurochs but at least one group is using CRISPR to swap wooly mammoth genes into elephant genomes in order to make hairy, cold-resistant elephants Another is trying to "de-extinct" the passenger pigeon by putting its genes into modern pigeons. The question about evolution is a good one. Here's an example: researchers have engineered CRISPR mosquitoes that don't transmit malaria. A synthetic biological system called a gene drive ensures that the malaria-resistance gene is passed on to all of their offspring. The goal is to quickly breed the normal mosquitoes out of the population. And there are any number of other ways that a animal or plant could be genetically edited so that it outcompetes the natural population. So yes, we could in a way direct evolution. - Sara

How did CRISPR become such a controversial topic? Did the tabloids blow this new technique out of proportion? And who do you believe deserves the acknowledgement/award for the creation of the technique?

Writ3rs

I'd say it was the news that researchers had used CRISPR-Cas9 to tweak the genome of a human embryo that did the trick. Scientists had been happily using CRISPR for a while before that, and other gene-editing technologies had been around for years without creating such a stir. (CRISPR also gets more attention than those older methods because it is far easier and cheaper to use, making it more widely accessible.) Are tabloids blowing it out of proportion? Probably some of them, sure. But I'm glad it brought out a wider discussion on how these technologies should be deployed in medicine, agriculture, and so on. Who deserves credit? A whole bunch of people. CRISPR is a beautiful illustration of the value of basic research: from the microbiologists who first saw these odd repeated DNA sequences and said "hmmm, wonder what those do?" to all the people who went into turning that observation into a powerful research tool – and maybe, someday, a way to treat disease. -- Heidi

Hey guys, thanks for doing this AMA.

What is your take on the recent developments in using CRISPR as a potential treatment for Duchenne Muscular Dystrophy? I know some clinical trials are occurring right now using antisense oligos for exon skipping, but CRISPR/Cas9 exon 45-55 deletions seem to be producing promising results.

bagwatchfruit

It's been great to see so much activity around DMD lately, using a variety of approaches. (For those who aren't familiar with DMD, here's a primer.) I recently spoke with Charles Gersbach (bioengineer, Duke, author on some of the recent CRISPR/DMD papers, and he said the hope is to move their work into larger animals (pub'd results were in mice) and then, eventually, phase 1 clinical trials. But he was also realistic about all the work that has to be done before then. He noted that their approach relies on long-term expression of the Cas9 protein, which raises concerns about triggering an immune response, and could increase the chance of off-target cutting. He said it would certainly be years before they can move it into the clinic. But given the recent clinical trial/regulatory disappointments in DMD, I think it's heartening to see that there are more approaches working their way up the pipeline. -Heidi

Hello and thank you for doing this AMA!

What are the most compelling advances utilizing this technology that we'll see in the next 10 years in the way of combatting diseases such as alzheimers and cancer? More specifically, will these advances address an individual's genetic predisposition to getting these conditions? Which corporations are the most advanced/best positioned to make these breakthroughs?

Thanks

M-D-J-D

With that time frame and those diseases, I suspect the bulk of the most compelling advances using CRISPR-Cas9 will still be in basic research, especially when it comes to addressing an individual's genetic predisposition. Often we still need to figure out what those predispositions are and how they work. Researchers are using CRISPR-Cas9 to improve their drug screens, and to model human mutations in cell cultures and animal models. That said, there is a lot of interest in using CRISPR-Cas9 to engineer immune system cells called T-cells to attack some cancers, like leukemias and lymphomas, and I can imagine it won't take too terribly long to get that into the clinic. There is already some precedent: a clinical trial involving an older gene-editing technology reported some success against leukemia last year. Also, the technique doesn't pose as many safety concerns as some applications of CRISPR-Cas9 because the editing would be done outside of the body (then the edited T cells would be infused back in). -- Heidi

Hello researchers. Recently, many prominent scientists met at a biotech conference where they decided to put a moratorium on genome editing. While there are many pros and cons to whole genomic editing in humans, do you think this moratorium does more harm than good while it stints research or is it better to fully understand the consequences of genome editing prior to experimentation? Thanks!

Speedy26xc

edited to include links I want to clarify this a bit. The meeting was a great opportunity for scientists,

government, advocacy groups, and other stakeholder groups to discuss the many aspects of CRISPR. But the conclusions they reached don't have any regulatory authority that could stint research. Rather, it simply stated what most people are thinking anyway: we should not implant human embryos with transmittable genetic modifications - yet. It's more of a gentlemen's (and –women's) agreement among scientists. Anyway, even if someone wanted to do that – and it would be semi-legal in many countries – there are a lot of technical hurdles that still need to be overcome. And the meeting did support research on human embryos, as long as they stay in the lab and not in the clinic.

Is there any research into using CRISPR to improve the efficiency of photosynthesis? It seems like it would be a great way to develop, for example, lipid fuel manufacture in bioreactors.

elucify

Absolutely. It's still relatively early days, but lots of plant biologists are using gene-editing techniques like CRISPR-Cas9, and the approach gets a mention in this review on redesigning photosynthesis. --Heidi

What do you mean by customize your own pets? Are we talking cats that live forever, or mini great white sharks I can keep in my fish tank?

mybustersword

Aren't nine lives enough for a cat? Now you're just getting greedy. :)Mini-critters are definitely do-able – lots of labs are using CRISPR to modify genes involved in growth. Keep an eye on the genomics company BGI in China, which is already making micropigs and koi carp intended to be sold as pets. They say they'll eventually take orders for customized colours and patterns. We've also got CRISPR ferrets and dogs, but they're not being sold as pets (yet), and CRISPR birds should be available in the near future. It's only a matter of time before other CRISPR-pet companies spring up – watch this space! --Sara