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The Genetic Goad: Gene Therapy Research has Exploded, but What Separates Good Knowledge from Bad, and How Scientists Should Negotiate that Line, is a Critical Question

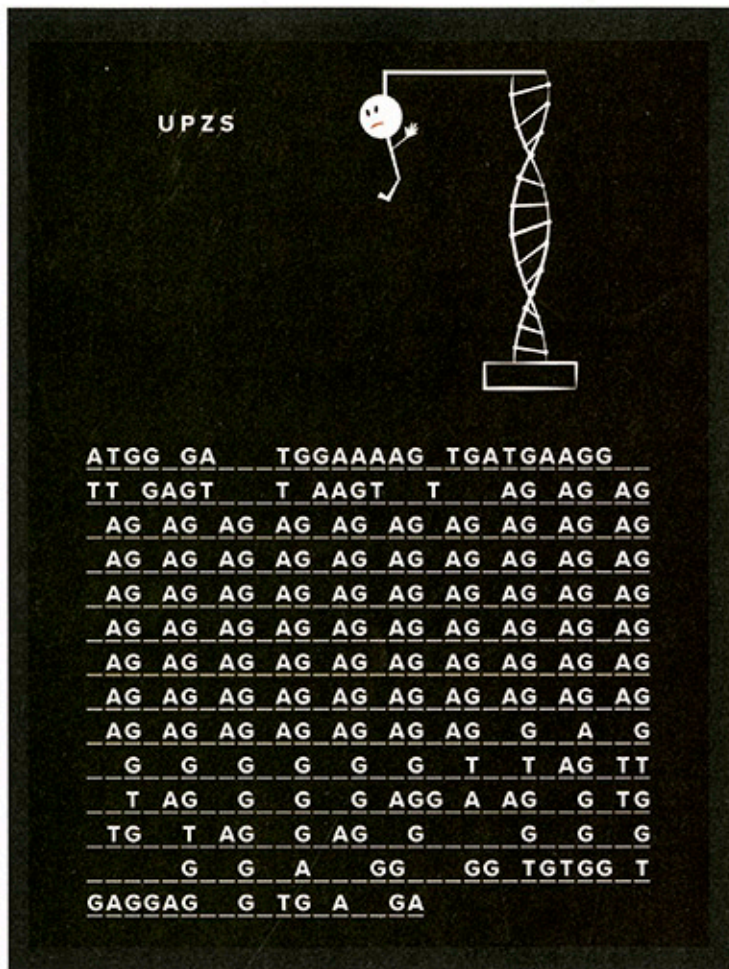
Lindsay Niedringhaus '07
Furman University

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The Genetic Goad

Gene therapy research has exploded, but what separates good knowledge from bad, and how scientists should negotiate that line, is a critical question.

BY LINDSAY NIEDRINGHAUS '07

It's the Monday before Thanksgiving break, and the anticipation of a few days off is counterbalanced with the dread of exams that will follow soon after students have returned. To abate the strange dynamic, Renee Chosed, professor of today's genetics class, has baked cookies. Students settle in to

seats, cookie in one hand while shuffling through notes with the other.

"All right, Jacqueline, you're up first," says Chosed. "You'll each have six minutes to present on the disease you chose to research. I'll start the timer when you're ready."

Jacqueline begins her PowerPoint. "I chose Severe

Combined Immunodeficiency, or SCID. Also known as the 'Bubble Boy Disease.' It's caused by a buildup of lympho-specific toxins. The disease has genetic origins but no specific mutation."

Jacqueline goes on to explain how gene therapy, administered through a bone marrow transplant, has become a way to treat SCID. However, studies show that after this therapy, four out of 10 patients developed leukemia because of the activation of oncogenes—genes that can cause cells to transform into tumor cells. "So, there is no easy treatment or cure at this point," she concludes.

Next up is Kennedy, who presents on Batten Disease, a common form of a group of disorders called neuronal ceroid lipofuscinoses. Symptoms of the disease include problems with balance, seizures, trouble seeing at night, and speech difficulties. "At this time, there's no known treatment," she says at the end of her presentation.

Then Jonah, who speaks about Huntington's Disease. "Treatments help with the symptoms, but they don't cure the disease. Scientists haven't found a cure as of yet."

The next couple of hours follow a similar pattern, with the students presenting on a variety of genetic diseases, the majority of which are incurable. To those accustomed to seeing headlines in science that promise healing at every turn, today's class is sobering. However, it paints a relevant picture of the medical field's current relationship with genomic research.

"We live in a post-genomic era," says Chosed. "We know the architecture of genomes, what happens with mutations, and how these mutations cause diseases. All of this is great, but it's still not enough. In many

cases, we don't know yet what to do with this knowledge—how to fix these mutations and, in essence, cure the diseases."

It's as though we are aware of all the ingredients to make a recipe, but we don't know the steps to put them together. The result is a patient—and a medical field—that is knowledgeable about a condition, but not always equipped to take informed and definitive actions based on that knowledge.

According to Chosed, more advanced knowledge will come with time, as more testing in labs is needed to fully understand how to manipulate and correct mutations. In the interim, patients must be responsible for how we react to the information given to us.

Currently in the field are a variety of genomic testing companies—some offer actionable results (you are at risk for diabetes, so perhaps you should change your lifestyle) while others offer diagnoses (you have a mutation in the Huntington gene, so you could have Huntington's Disease sometime in your life). For uninformed patients, Chosed sees problems in the second category.

Take 23andMe, for example, a company founded in 2006 in Mountain View, CA, that claims to "help people access, understand, and benefit from the human genome." For \$199 and a test-tube full of saliva, you'll get a "complete" genetic report. You'll learn if you're a carrier for any condition that may be passed down to your children, as well as information about your ancestry. You'll also learn about traits that are controlled by your DNA, such as hair loss and your preference for sweet or salty foods.

More than one million people have opted in to the company's service—because

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surely information about your genetic makeup is helpful in planning for the future. Except it may not be. Critics such as Chosed argue that some of this information, taken out of context and without proper counseling, could be dangerous.

“Your report could say, for example, that you could possibly develop leukemia later in life. It won’t say, however, that this will only happen if the gene mutates, and that will only happen if specific circumstances occur. It’s important that these results are complemented with proper counseling and advising in order for the patient to fully understand the implications.”

In December of 2013, 23andMe faced a class action \$5 million lawsuit less than a week after the Federal Drug Administration ordered the company to stop sales. The FDA accused the company of false advertising, stating that 23andMe claimed to provide “health reports” to customers,

yet they had not provided the FDA with any proof of clinical validation. Further, the company was taking the information received from customers to create a database of information that was then marketed to other companies in the scientific community. Only after changing the way they presented results to customers was 23andMe allowed to resume sales.

Medical advances in the last century have brought a host of new information about how our bodies work and respond to treatments. The result has been a proliferation of treatments and cures. Heart rate quickening? There’s a pill for that. Need to lose weight? There’s a surgery for that. In the process, we have come to a point where we are afflicted with a new, overarching disease: our need to feel in complete control of our health—and, more broadly speaking—our lives.

Chosed’s class, however, reminds us that grasping for ultimate control is, at this point, still a futile effort. To be sure, we are much more informed patients than we were 25 or even five years ago, but there is still much more to be discovered. In the meantime, we should remain contemplative and engaged, not only about the current state of our health, but also about how we approach our lives.

“Science is moving very fast, and medicine just has to catch up at this point,” she says. “It’s my hope that my students leave this class with a basic understanding of genetics, and refer back to this knowledge should certain medical situations arise. More importantly, I want them to be knowledgeable medical consumers who feel confident in questioning and pushing for the truth.” It’s in the questioning where that truth emerges. ●



NOW

Reflection on the Importance of Furman as It Is Now

In a world that is concerned about achievement and recognition, Furman does more than hold its own. If one takes the time to read about our alumni and faculty, their accomplishments can be discovered far and wide. On an almost daily basis, current students are presented with opportunities that shape the kind of leaders the world is looking for. Greek life, for example, has given me lifelong friends; the Business Block course the confidence that I can succeed; and Furman’s culture has rekindled in me a drive for excellence.

When I got into college, that was an achievement, but at Furman, it’s not a laurel to rest upon. Just skating by is not what we strive for here. Very rarely have I come across someone who isn’t driven to succeed or make a difference. The more I talk to and interact with these people, the more I feel my own drive reappear. This may seem uniform across college campuses, but I would argue it’s not. So many college students are focused on doing just enough to graduate.

“Work ethic” is a major part of the Furman DNA. You don’t survive or succeed here if you fail to adopt or incorporate this. Of course there is an emphasis on academic success, but what Furman does is emphasize the bigger picture, forcing students to pick our heads up from the narrow tunnel we often build.

Cross-disciplinary studies, CLPs, activities that help us to do a better job of not becoming pure bookworms—I can almost guarantee that if I was at Southern Cal or Baylor (my other choices),

my course load would not include classes on Islamic Studies or Arthur Conan Doyle. Nor would I have attended lectures on the impacts of drone warfare or a critical analysis of the Affordable Care Act. Fulfilling those requirements demands a work ethic, time management, general curiosity, and characteristics that drive the success of leaders.

When I was asked to contribute to this series, I first thought about ideas related to improvement—concerning Furman’s reputation, Furman’s athletics, Furman’s overall appeal. But the more I thought, the more I realized improvement isn’t my main concern.

Rather, I don’t want our efforts to meet the benchmarks of “prestige” to mean we forget the real opportunities here. Because, while a Furman education is tremendous, what really sets us up for success is the experience we undergo here as a whole. We can so easily overlook that in the face of desiring recognition and awareness.

ABOUT THE AUTHOR

Samuel Cubeiro '16, born and raised in California, traveled across the country in hope of escaping the bubble he had grown accustomed to. A business and political science double major, he will leave Furman with invaluable experiences that have charted a course much different than what he imagined when he arrived. He plans to apply his education toward a career with a Major League Baseball organization.