PROFILE OF A POTENTIAL "NOBEL PRIZE" WINNER FROM PASCO

-By Thomas Konda, M.D.

Kiran Musunuru, M.D., PhD, MPH, ML was born in New York City in 1976, while his father was undergoing postgraduate training in internal medicine and cardiology. He moved to west Pasco (FL) in 1981, where his father started a cardiology practice.

While in school, he was one of the top three national winners of "Mathcounts" (akin to spelling bee) in Washington, D.C. He wrote a computer program in genetics. Also he co-authored scientific publications about the mechanisms of actions of insulin (protein-kinase, etc.). He used to read EKG's with his father after making patient rounds. He also volunteered at the hospital fixing computers and teaching calculus to hospital employees. He also won national "Latin" essay writing competition.

At the celebration of Kiran's high school graduation arranged by his parents in an auditorium attended by family members and friends with their children, the usually shy Kiran astonished everybody with his an hour long program of music and magic (piano and advanced magic including Houdini's metamorphosis). That was the beginning of his public scientific presentations at national and international levels. He has become a star with his excellent command of language in addition to a commanding voice.

While at Harvard, as an undergraduate he started and edited "Journal of Undergraduate Sciences." Even though his major was biological sciences, he took "Advanced Engineering Calculus, Crystallography and Buddhism" as

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electives. He also co-authored a book "Cell-Cycle Regulators in Cancer" while undergraduate at Harvard.

While doing combined M.D., PhD program (on full merit scholarship) at Cornell and Rockefeller, (his thesis was in neurosciences at a molecular level) after a selected scientific presentation in California, he earned a glorified editorial in a prestigious journal "Nature Medicine" which ended with the sentence "The presentation marked Musunuru out as a future star in biomedicine". A lot of Nobel Prize winners were among the speakers and audience at that meeting. His presentation was sandwiched at the opening session between two presentations from two Nobel laureates.

While doing 2 years (instead of 3) internal medicine residency at Harvard (Brigham) he authored "Pocket Books" in internal medicine and critical care for the rest of the house staff. He received "Best Outgoing Resident Award" which is usually reserved for 3rd year resident. The director of the program described him as a "National Treasure" in writing. Also while doing residency, he worked as a consultant for a pharmaceutical company and guided them to produce new cardiovascular medicines (RNA based).

Kiran has been volunteering for American Heart Association (AHA) for decades. He has served on the leading roles for its scientific councils like clinical cardiology and functional genomics. He received national awards from AHA for his service in science; he also finds time to work with "needy students" in the inner cities.

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While doing first 2 years of interventional cardiology fellowship at Johns Hopkins, he simultaneously finished masters in public health (MPH) from Johns Hopkins School of Public Health. He later acquired masters in law (ML) covering patent, business and administration and also masters in regulatory affairs (MRA) covering drugs, devices and development, from University of Pennsylvania.

While continuing his cardiology research fellowship back at Harvard (Massachusetts General) he advanced the knowledge in 'stem cells and regenerative medicine'. He also earned "Excellence in Science Teaching" award among Harvard faculty. He was honored at white house by president Obama in person for "Presidential Early Career Award for Scientists and Engineers". He was also bestowed the most prestigious American Philosophical Society's (started by Benjamin Franklin)"Judson Daland prize for Outstanding Achievement in Clinical Investigation" at its 275th anniversary in Philadelphia, in recognition of his work discovering and therapeutically targeting cardiovascular disease genes. It carried a $50,000 prize. Kiran also collaborated with MIT and Broad Institute.

Shortly afterwards, Kiran was recruited by University of Pennsylvania in Philadelphia to become a tenured professor at a young age with his own research lab leading to many advances in gene editing. He voluntarily teaches undergraduates (biochemistry), medical students (genetics)and cardiology fellows-in-training (staying with them day and night when he is on call, developing and implementing treatment strategies for treatment of sick

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cardiac patients transferred from other hospitals. He earned "Excellence in Teaching Award" at University of Pennsylvania also.

He authored publications in many prestigious scientific journals over decades. He also served as the editor of International Circulation Journal: Genomic and Precision Medicine. He also contributed chapters in many cardiology books, including "Braunwald's Text Book of Cardiology." His latest books include "Crisper Generation" and "Genome Editing- A Practical Guide to Research and Clinical Applications", for scientists and researchers to learn the art. He conducts boot camps at national AHA meetings and he constantly preaches ethics.

At this point with all his extreme knowledge in various areas and aspects of physics, mathematics, biochemistry, computer literacy, clinical medicine, and research in genetics (well planned since his school time, as you can see) he began specializing in "gene editing" to create cures for diseases that did not have any until now, not only cardiovascular but also some metabolic (e.g.: Phenylketonuria) diseases. His latest endeavor is intrauterine gene editing to prevent damage from bad genes while baby is still in uterus.

He currently serves on NHLBI council. Collaborating with universities, NIH and pharmaceutical industry, Kiran is going to make a world of difference for the humanity all over the world.

Kiran frequently associates with Nobel Prize winners worldwide. He receives a lot of attention, appreciation and admiration from them. For example, recent 'Nobel prize winner in science' Jennifer Doudna published a book in 2017-"A crack in Creation." In the first two pages of the fourth chapter she described

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about her excitement about meeting Kiran at his lab at Harvard and marvels about his work and she writes “Kiran was already one step ahead of me about applications of “CRISPR” as a therapeutic tool” Without any doubt, he will earn a Nobel Prize for himself in the future.

Kiran dedicated his life to science and he is the self-proclaimed “Pope” for religion of “discovering cures for all kinds of diseases”. He is a down to earth humble person, very respectful to everyone, irrespective of their age or status. He is very kind to his students and researchers always giving them credit for all his own ideas, work and publications.

He is a person of many skills and talents. He is a package of brilliance, selflessness, generosity, and dedication. He encourages his parents to donate his inheritance to help the needy in addition to investing his own for the advancement of science.

Let us thank God for this gift to humanity!

Let us pray to God to bless Kiran and his parents for long healthy, happy and productive lives.

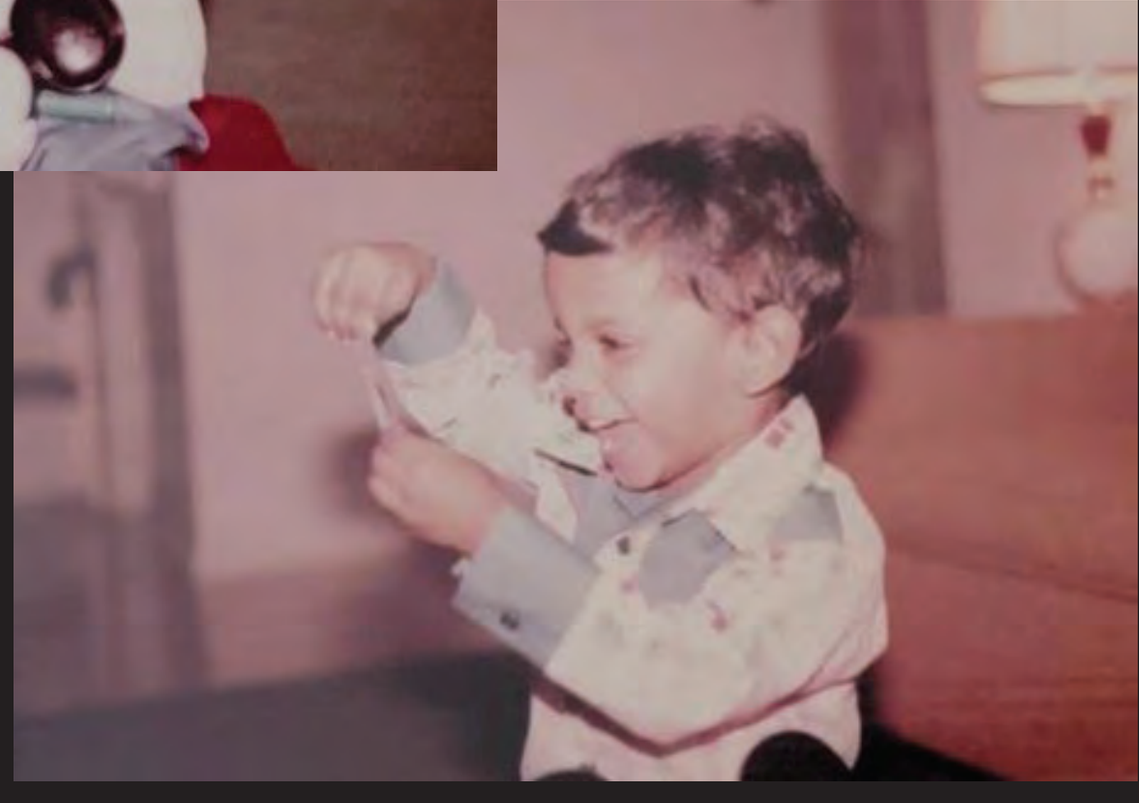
P.S. Dr. Thomas Suman Konda is a retired endocrinologist with a keen interest in academic and research medicine all his life. He has known Kiran, since Dr. T.S. Konda moved to Pasco County decades ago to support his wife (Nirmala), an excellent practicing cardiac anesthesiologist. He is also a successful stock market investor. He has always admired, enjoyed and encouraged Kiran, sharing their mutual enthusiasm and interest in medical research.

From there, "wherever you go
Go with all your heart."

Kiran as an infant.

Happy young family in
New York City.
Kiran at eight months.

Kiran growing up.



A happy boy.


KIRAN

Always happy, happy.

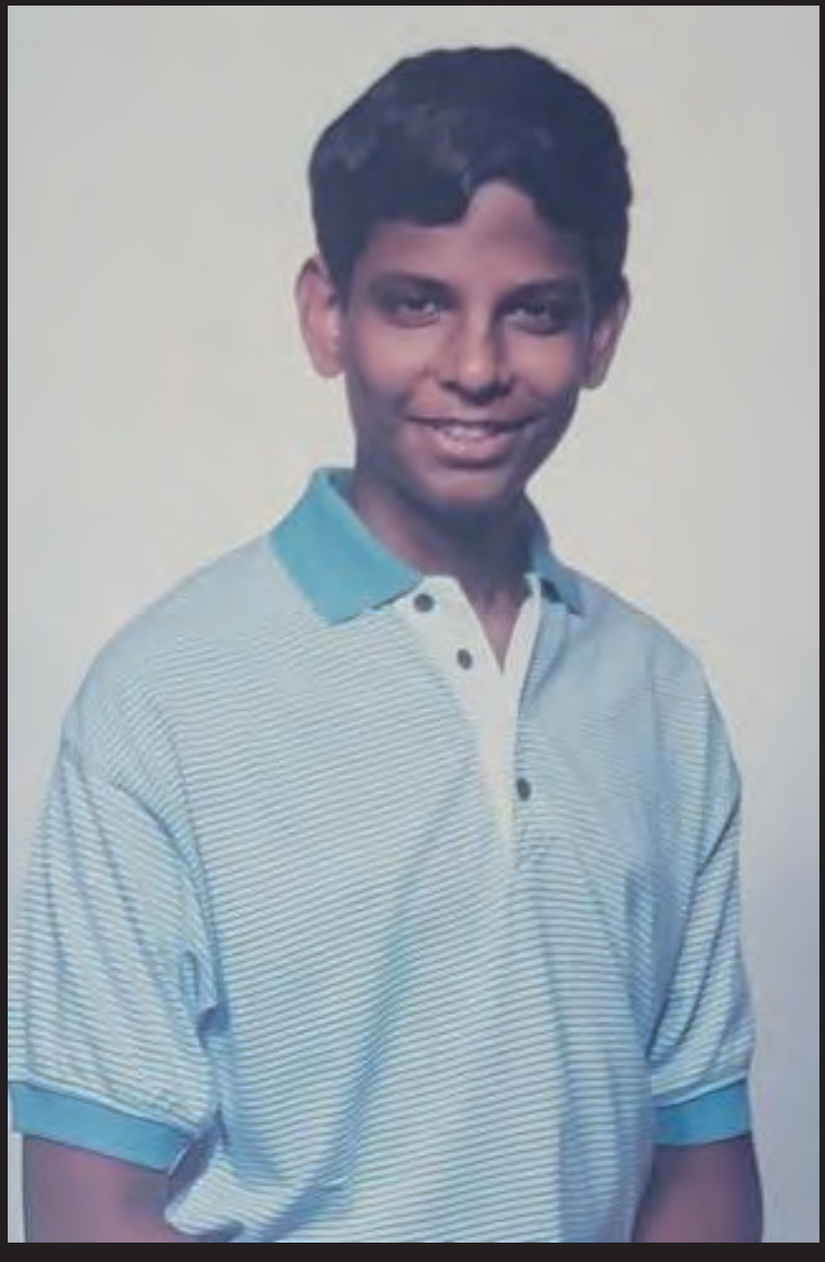
Kiran at work,
learning himself
and from his Dad.

Kiran, future teacher.

Kiran at piano.

Feeding the body,
not only the mind.

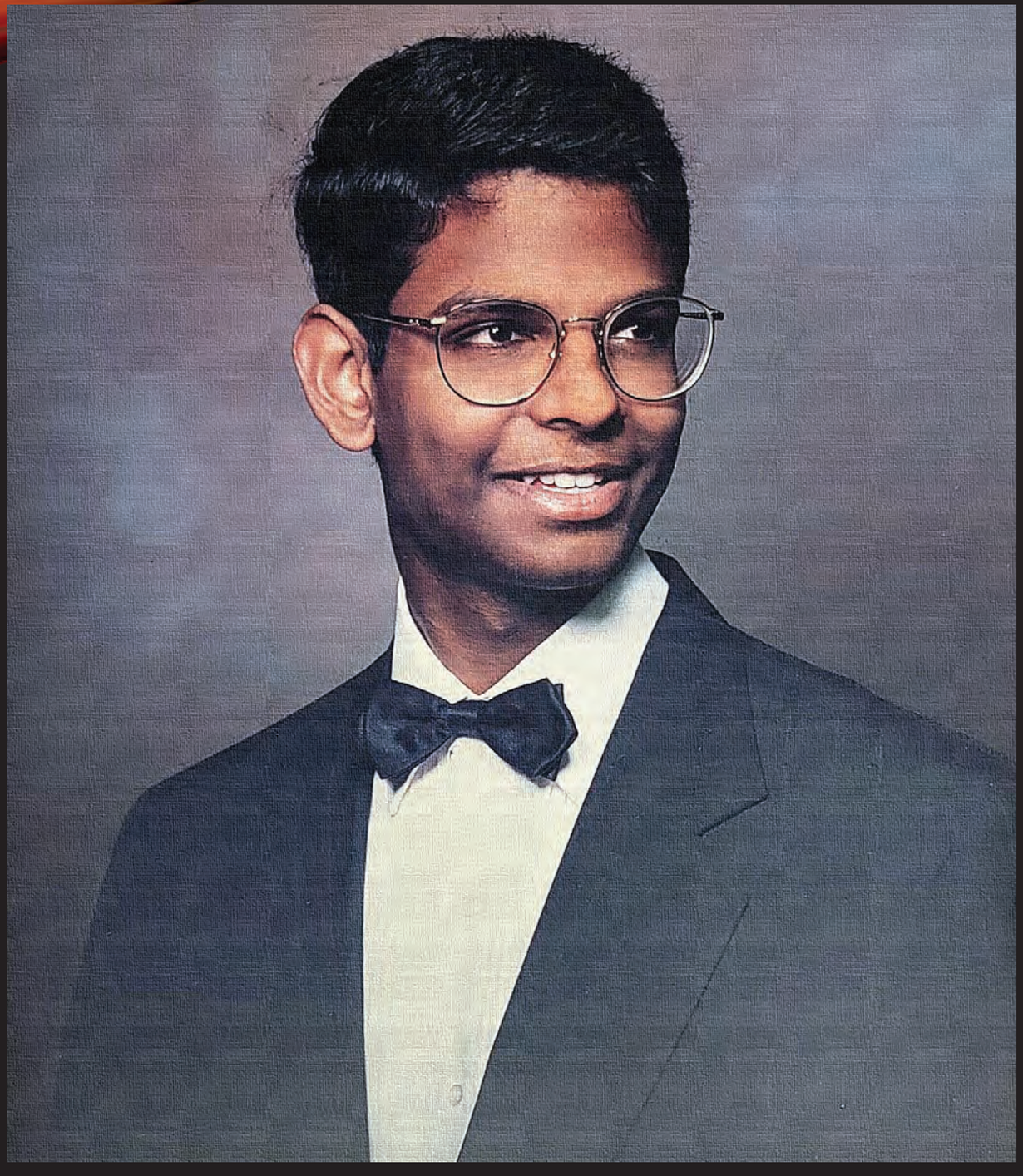
Innocence at its best.



Kiran growing up.

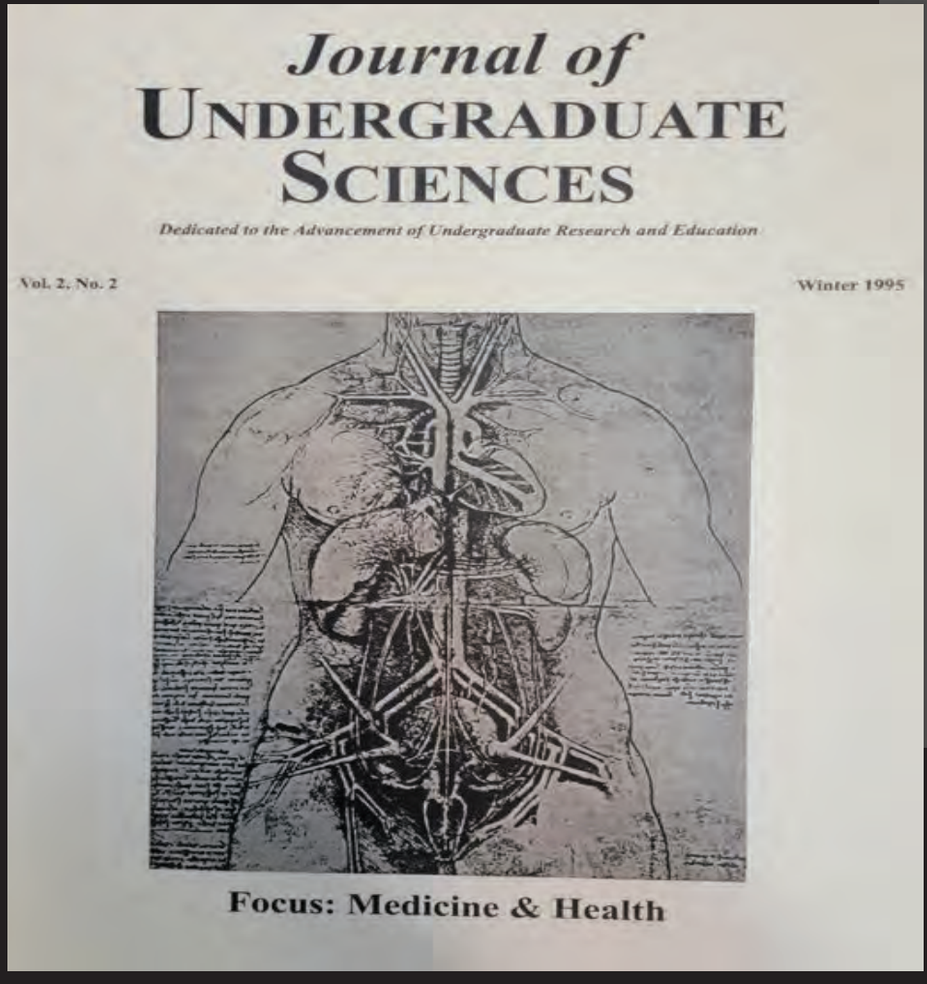
Kiran's personal book library during his school years. *(Part 1)*

Kiran's personal library during his school years. *(Part 2)*

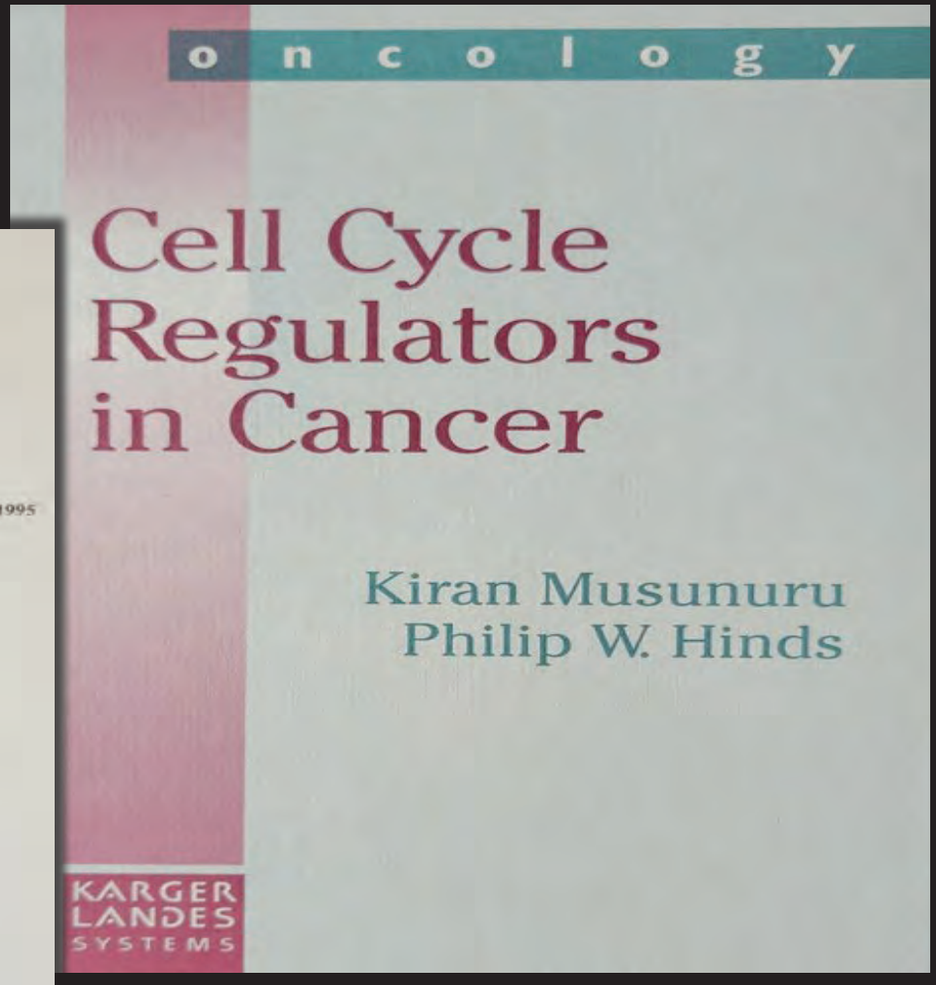


Kiran,
the
Performer

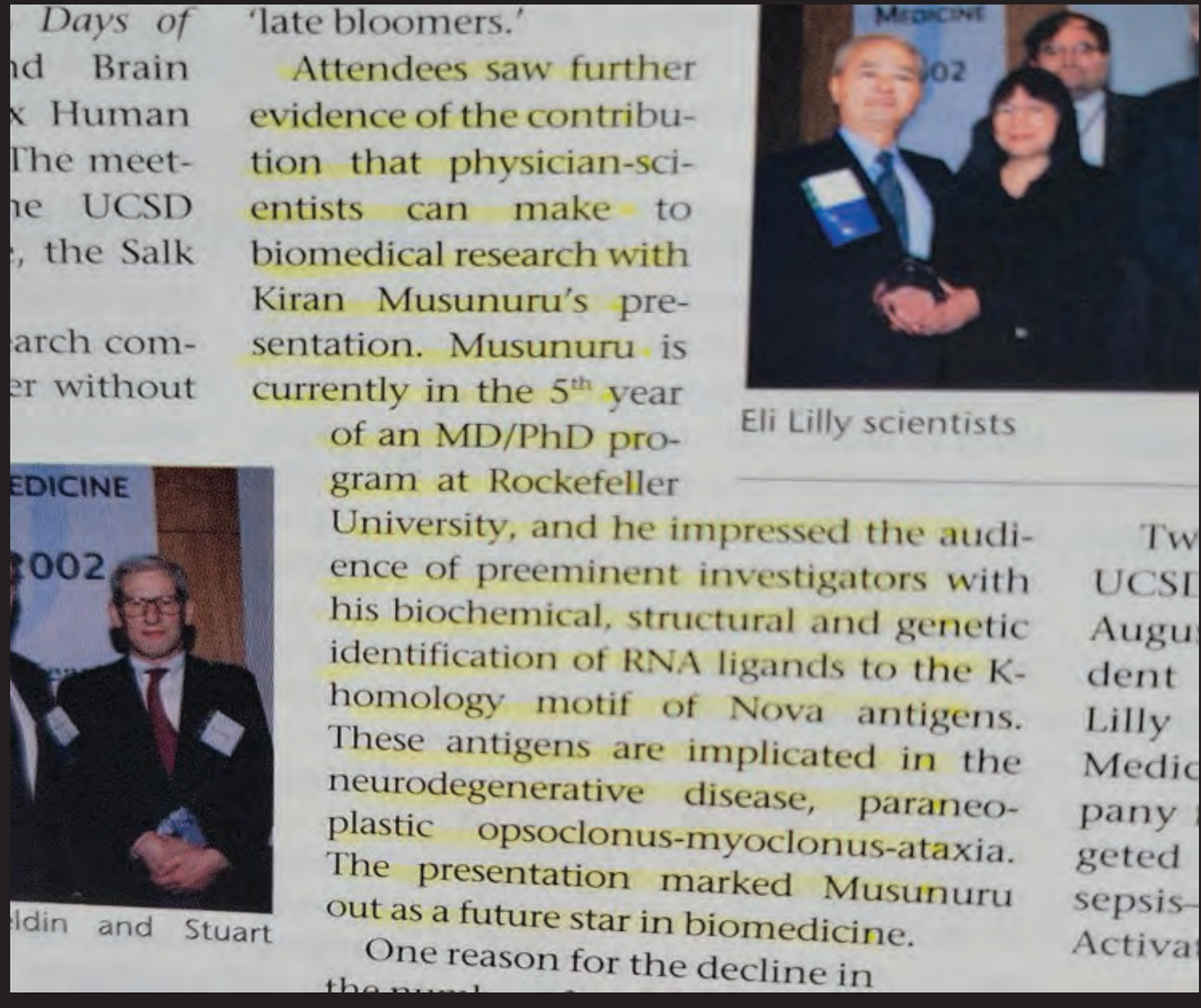
College graduation at Harvard with dad.



Kiran was the founder and first editor of this journal at Harvard while he was an undergraduate.



Kiran was the co-author for this medical book, written at age 20, while he was an undergraduate.



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Attendees saw further evidence of the contribution that physician-scientists can make to biomedical research with Kiran Musunuru's presentation. Musunuru is currently in the 5th year of an MD/PhD program at Rockefeller University, and he impressed the audience of preeminent investigators with his biochemical, structural and genetic identification of RNA ligands to the K-homology motif of Nova antigens. These antigens are implicated in the neurodegenerative disease, paraneoplastic opsoclonus-myoclonus-ataxia. The presentation marked Musunuru out as a future star in biomedicine.

One reason for the decline in

Eli Lilly scientists

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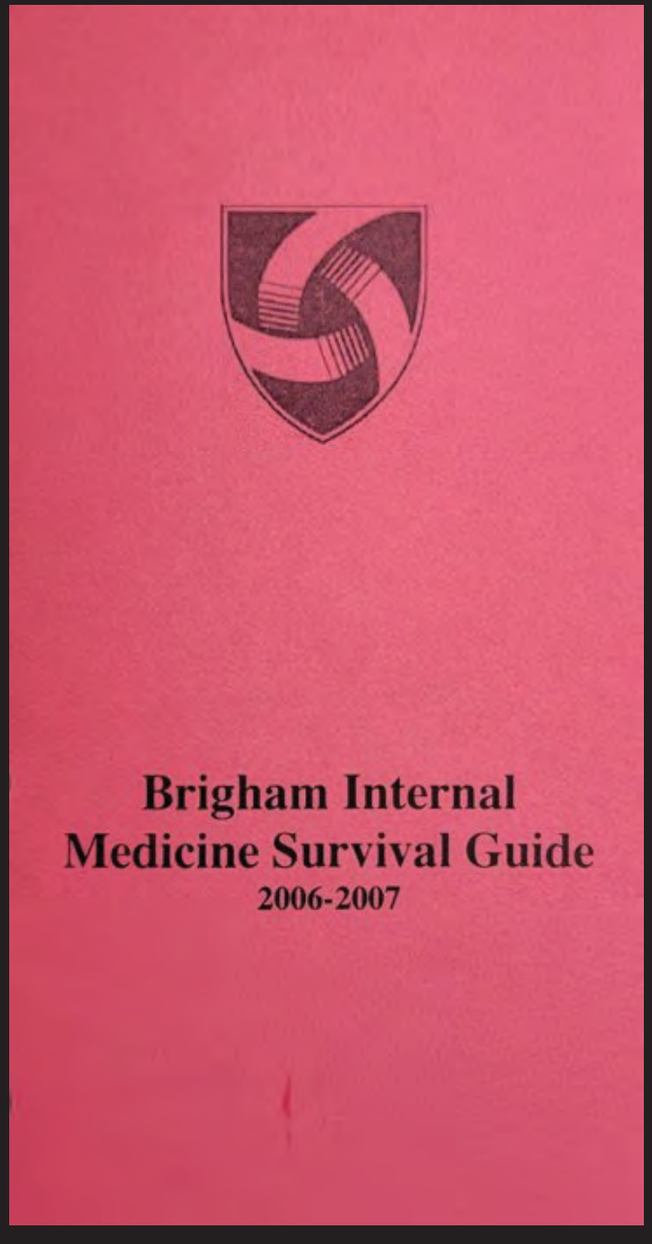
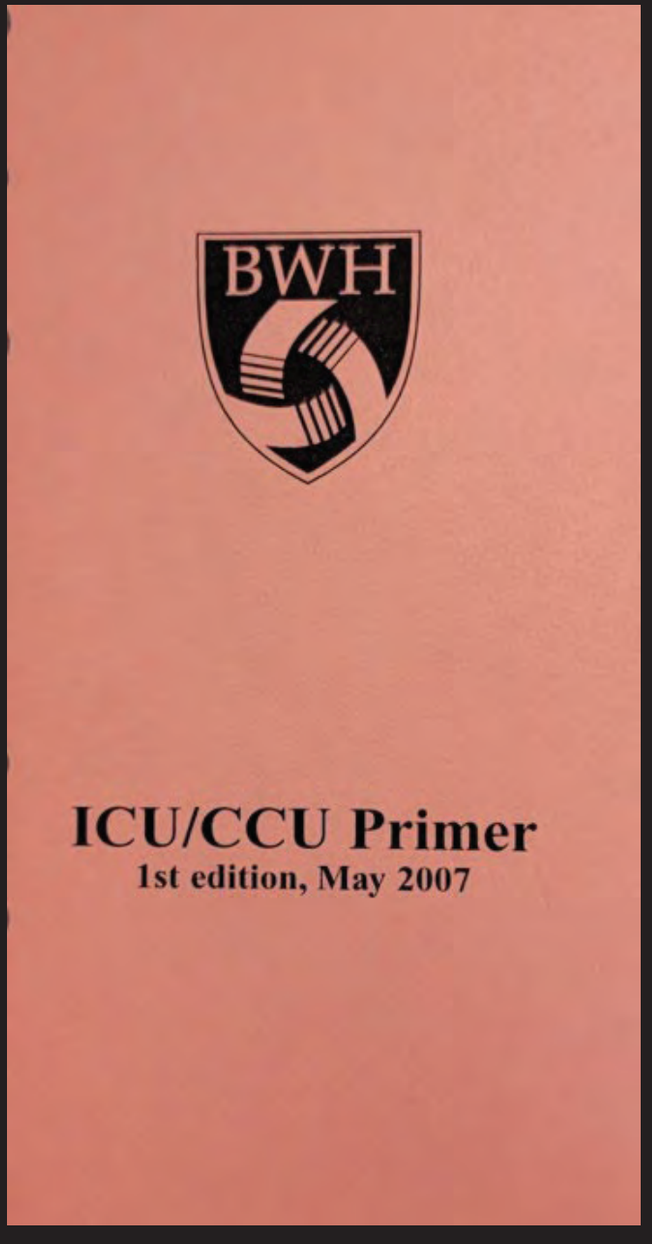
Kiran was described as a "future star" in bio-medicine during his M.D./Ph.D. program.

Primer written for colleagues by Kiran as a first-year resident at Harvard.





Primer written for fellows by Kiran as a second-year resident at Harvard.

suncoastnews.com ~ WEDNESDAY, MARCH 9, 2016 ~ THE SUNCOAST NEWS/PASCO WEST

Researcher son of cardiologist to get presidential honor

Kiran Musunuru, a Harvard biomedical researcher receives congratulations from his father, cardiologist Rao Musunuru of the Heart Institute in Hudson. Kiran Musunuru will receive the Presidential Early Career Award for scientists and engineers.

Harvard's Kiran Musunuru studies heart disease

Suncoast News staff report

HUDSON — The White House staff announced that physician and biomedical researcher Kiran Musunuru will receive the Presidential Early Career Award for scientists and engineers.

Both Kiran Musunuru, 40, and his father, cardiologist Rao Musunuru of the Heart Institute at Regional Medical Center Bayonet Point, are board-certified cardiologists.

Kiran Musunuru, an assistant professor at Harvard, will get the highest honor bestowed by the United States Government on science and engineering professionals in the early stages of their independent research careers. An event this spring in Washington, D.C., will celebrate his accomplishment.

He was also selected to receive another national award for his scientific and volunteer work from the American Heart Association this summer in Dallas.

Rao Musunuru has been practicing cardiology, serving residents of Pasco and Hernando counties, since 1981. He has been instrumental in establishing the nationally recognized Heart Institute. Both father and son have received national awards and recognitions over the years.

Kiran grew up in West Pasco. He received his master's degree from Cornell University, a doctorate degree from Rockefeller University and a master's in public health degree from Johns Hopkins University.

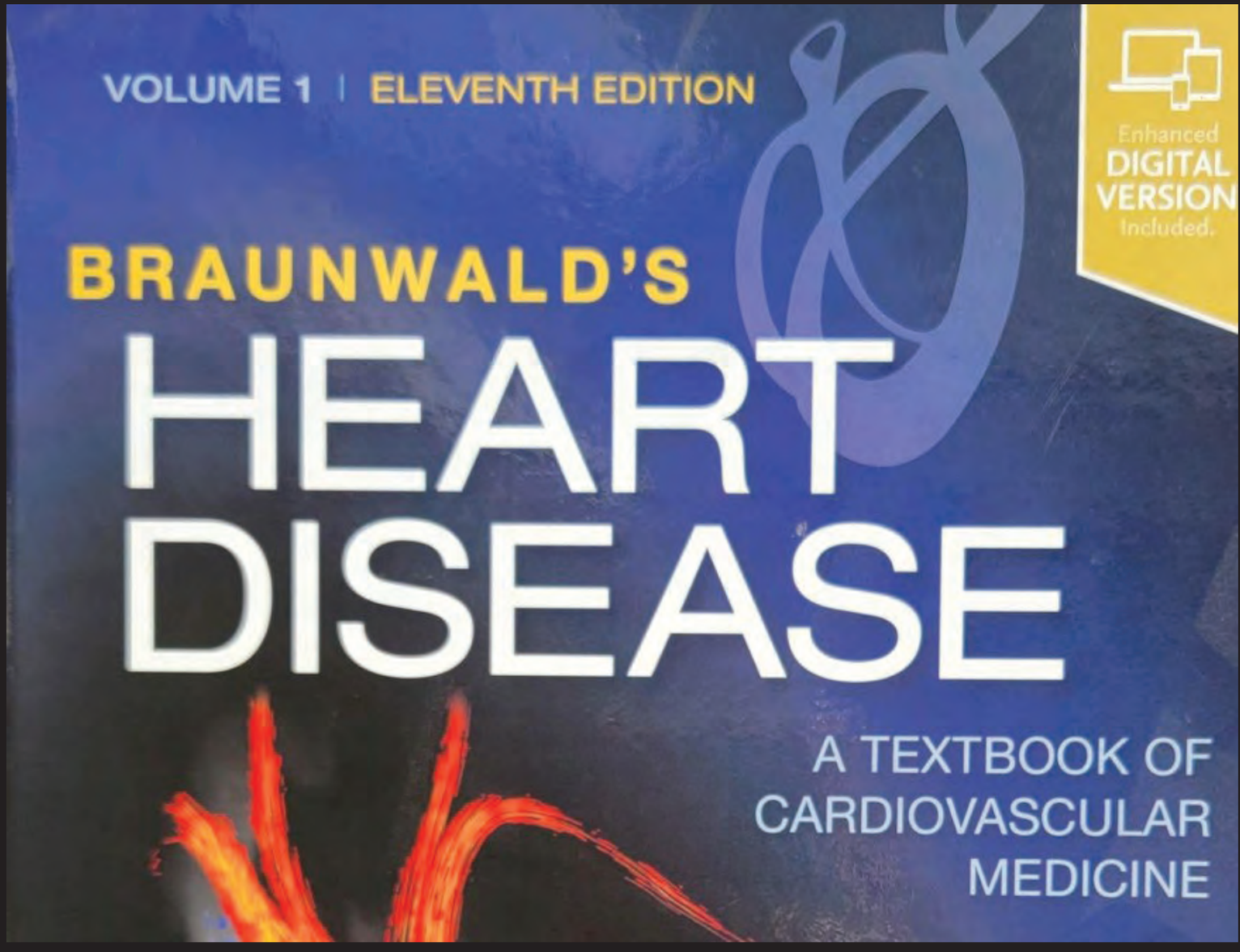
He published his first medical book, "Cell Cycle Regulators In Cancer," at age 19 as an undergraduate at Harvard.

He did his cardiology fellowship at Johns Hopkins and Massachusetts General Hospital.

He published extensively in many prestigious scientific journals. He wrote a chapter on Principles of Cardiovascular Genetics in the latest edition of Braunwald's Textbook of Cardiology.

He teaches undergraduates at Harvard, medical students at Harvard Medical School and patient care to the medical residents at Brigham and Women's Hospital, where he is an associate physician. He runs his own research lab on the Harvard campus in the Department of Stem Cell and Regenerative Biology. He lectures extensively all over the country.

Kiran Musunuru is pursuing genomic research on heart disease. He is striving to develop once-in-a-lifetime vaccine for prevention of coronary heart disease, thus preventing heart attacks. Many experts in his field describe him as a future prospect for a Nobel Prize.



Kiran wrote three chapters in this textbook of cardiovascular medicine.

INNOVATION NEEDS RESPONSIBILITY

In January 2009, I wrote a guest column in the *Tampa Tribune* titled "Unraveling Genome Has Great Potential, But We Are Not There Quite Yet."

Little did I know then that, by 2015, we would be there!

We have known for a long time that in humans, life is transmitted through 23 pairs of chromosomes in each cell, half of each pair contributed by each parent. The chromosomes are made of DNA, which is encoded into various genes.

In 1953, James Watson and Francis Crick unveiled the structure of DNA, cracking the code of life. Decades later, the entire human genome (all of the DNA sequences in a single cell) was mapped, made of billions of nucleotides (the chemical alphabet). The scientists then focused on identifying the tiny portions of the gene that are responsible for various functions and dysfunctions.

For a while, scientists toyed with the idea of altering DNA (the basic units of the gene), finally resulting in genetic engineering with recombinant DNA (cut and paste - cut the nucleotides from the genes of one organism and paste them into the genes of another) to introduce desired traits.

For example, by altering the genes of bacteria, large quantities of hormones (insulin, for instance), antibiotics and clot-busting medications were manufactured for human medical treatment, saving millions of lives. Similar benefits were achieved from transgenic animals.

For the past couple of decades, the technique was also used to produce transgenic crops called GMOs (genetically modified organisms) by splicing genes from one species into a different species to improve productivity and to enrich their quality.

For the past few years, scientists have embarked on the idea of "gene editing," a process that nature does all by itself to protect bacteria from viruses. The scientists observed it, learned from it and duplicated it in animals and humans.

When a bacterium is invaded by a virus, it keeps a genetic record of the virus in memory and when re-invaded by the same virus, it produces a powerful enzyme that effectively snips the virus out (a molecular scissor). Scientists named the process "CRISPER" and the enzyme "Cas9."

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This process of gene editing, which can be done on any living organism, has enormous potential in infinite ways to improve on nature. The applications can range from cancer research to curing diseases, production of vaccines to eliminating mosquito-borne illnesses, production of fuel and electricity to disposal of plastic and production of super crops to save endangered species.

This new genome-editing technique (deleting, altering or rearranging DNA) is precise, relatively quick and easy, and inexpensive. A CRISPER kit can be commercially bought for $130.

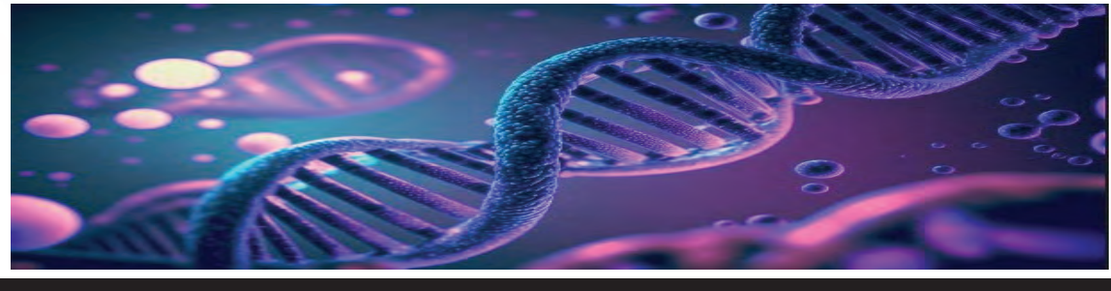
The story is all rosy, until one thinks of using this technique to edit germ-line cells (sperm, egg, embryo). The resulting change can be passed on to future generations forever. What might be the long-term unintended consequences of altering the genes permanently? A rogue scientist could go beyond the ethical, moral and legal limitations, producing designer babies, superior humans and super bugs.

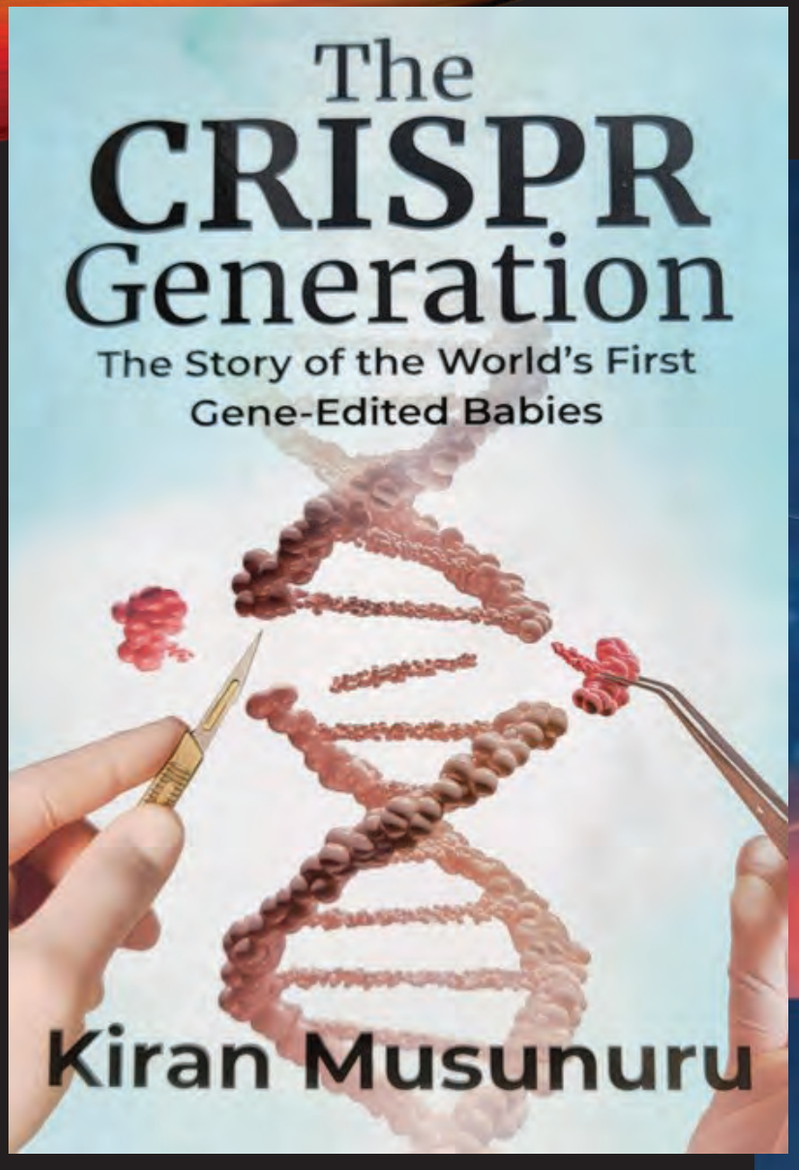
Are we trying to play God? I wouldn't even venture to go there in this column. However, I cannot help but wonder why the creator is empowering humans with super-intelligence to decode all the secrets of creation and the means to alter it in no small measure. What is the creator's ultimate plan? Only God knows.

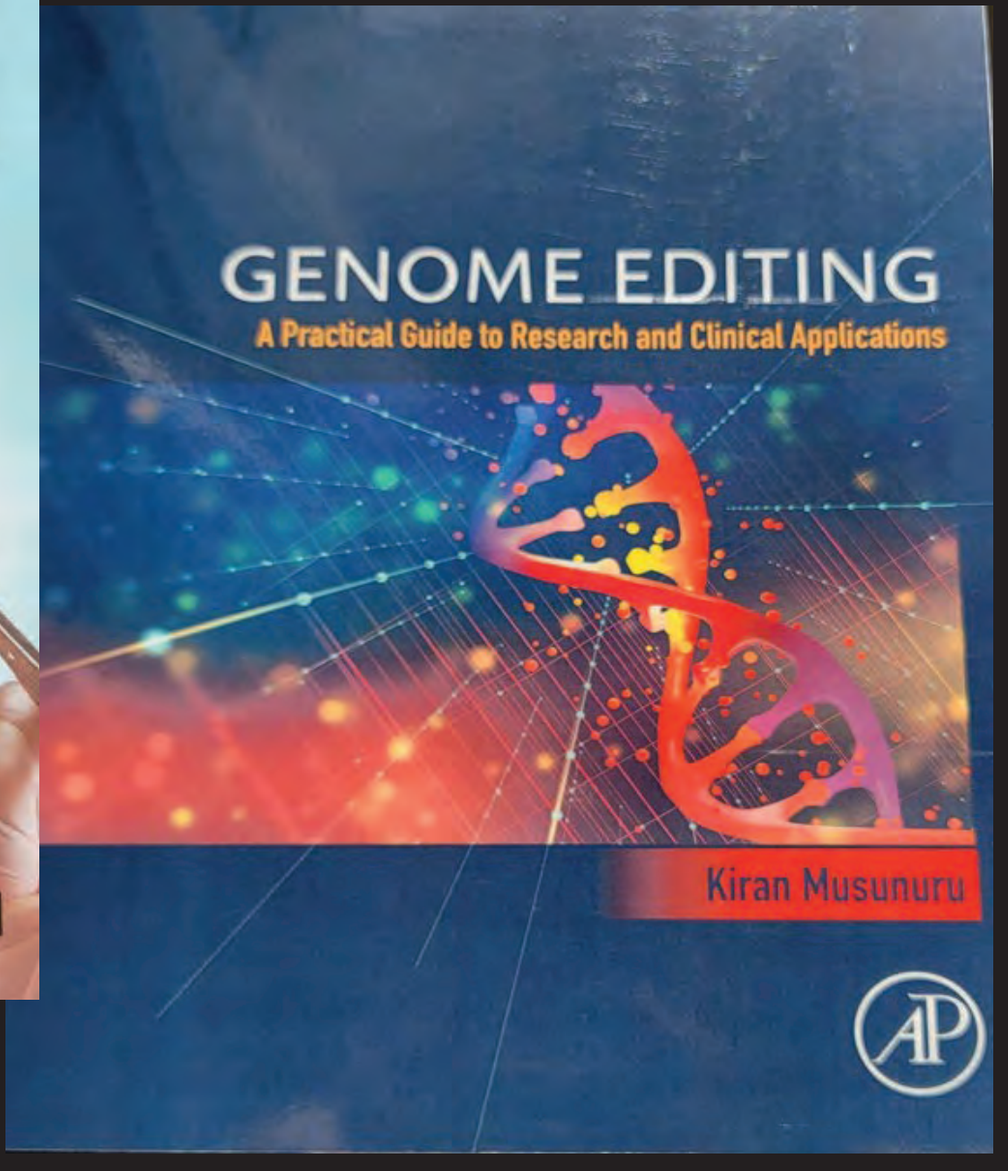
But the genie is out of the bottle, and gene editing is here to stay. This technology is one of the biggest things since nuclear energy. We can only hope that it will be put to good use.

Guest Column, Published Tampa Bay Times, September 23, 2016.

P.S. My son Kiran Musunuru, a nationally and internationally celebrated cardiologist from Harvard and University of Pennsylvania, is currently working with gene-editing techniques to find cures for several diseases that do not have any treatment or cure at this time.







More books written by Dr. Kiran Musunuru

Indeed, not too far in the future, the standard of cardiovascular care may look quite different from current practices. Patients would undergo whole-genome sequencing at birth, thereby allowing so-called primordial prevention by assessing the genetic determinants of an individual's lifetime risk for cardiovascular disease and institution of appropriate counseling—starting with lifelong exercise and dietary habits and, as the patient advances in age, individually tailored preventive medications and therapies that address all the individual's various validated, causal genetic risk factors for disease. If cardiovascular disease should nevertheless emerge at some point in the patient's life, he or she would receive the specific therapies that have been demonstrated to be most efficacious and safest for individuals with that genetic profile, both in the acute setting and in the long term for secondary prevention. This standard of care would represent an important step toward ensuring that people everywhere enjoy longer lives free of cardiovascular disease.

Kiran's remarks from Braunwald's textbook of cardiology.

Kiran Musunuru-Wikipedia

Kiran Musunuru is an American cardiologist who is a Professor of Medicine at the University of Pennsylvania Perelman School of Medicine. He researches the genetics and genomics of cardiovascular and metabolic diseases. Musunuru is a leading expert in the field of gene-editing.

Early life and education

Musunuru is the son of Rao and Prameela Musunuru; he was born in New York City and grew up in Florida. His father is a renowned cardiologist who moved to the US from India in 1976.

Musunuru obtained a degree in Biochemical Sciences from Harvard College in 1997. He later obtained a PhD in Biomedical Sciences from Rockefeller University in 2003, and an MD from Weill-Cornell Medical College in 2004. Musunuru also graduated with a Masters of Public Health (MPH) in Epidemiology from the Johns Hopkins Bloomberg School of Public Health in 2009, and an ML in Law from the University of Pennsylvania Law School in 2019.

Musunuru was interested in heart disease early in his medical career, first training in Internal Medicine at Brigham and Women's Hospital and then in Cardiovascular Medicine at Johns Hopkins Hospital. He also undertook postdoctoral work at the Massachusetts General Hospital, as well as the Broad Institute.

Research and career, Awards and honors, References- see-
https://en.wikipedia.org/wiki/Kiran_Musunuru


TIME 100 TALKS
PRESENTING PARTNER
American Heart Association.
100 YEARS
Bold Hearts

‘80% of Cardiovascular Disease Is Preventable’: Health Experts Reimagine Heart Care

BY **CHANTELLE LEE** JUNE 25, 2024

More than 184 million people—about 61% of U.S. adults—are likely to have some type of cardiovascular disease by 2050, the American Heart Association (AHA) reported earlier this month. That will lead to a tripling in the costs related to heart disease. It’s a statistic that TIME senior health correspondent Alice Park cited to begin her discussion about the future of healthcare with AHA CEO Nancy Brown; cardiologist Kiran Musunuru; and Andres Acosta, associate professor of medicine at Mayo Clinic, for a TIME100 Health panel in New York on Tuesday.

The event was sponsored by AHA and is part of the TIME100 Talks series. The TIME100 Health list includes the most influential people in the health industry around the world.

Heart disease has been the leading killer of Americans since 1950. Brown, who has been CEO of the AHA since 2008, said

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the number of people in the U.S. living with the risk of heart disease—and the resulting cost—is "staggering." Part of the issue, she said, is the lack of equal access to healthcare and to social determinants of health, such as healthy food and a living wage. But another issue is the way the U.S. healthcare system approaches these types of medical conditions.

"I think that this country focuses a lot on treating conditions," Brown said. "But we're not focusing enough on prevention and helping people earlier in their lives understand the power of things that make a difference in their life. You know, 80% of cardiovascular disease is preventable."

Musunuru, a professor of cardiovascular medicine and genetics in the Perelman School of Medicine at the University of Pennsylvania, said cardiovascular disease can be attributed to about half genetics and about half environment or lifestyle. There are ways to reduce risk factors for developing cardiovascular disease, such as cholesterol levels, blood pressure, and even obesity. The challenge, he said, is that these risk factors develop over time. And the country's current healthcare system attempts to cope with

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chronic disease with chronic treatment. While there can be merits to that approach, Musunuru said, it also puts "an outsized burden" on patients.

He suggested the healthcare system shift its focus to preventing chronic diseases, starting at an early age—like we do with vaccines to prevent infectious diseases.

"You're not going to eliminate heart disease, but can you push off heart attack and stroke by decades?" Musunuru said. "Instead of suffering a bad heart attack at age 60, maybe dying from it, it happens at age 100 and you enjoy 40 years of life you might not have otherwise had."

Acosta, who codirects the Nutrition Obesity Research Program and directs the Precision Medicine for Obesity Program at Mayo Clinic, discussed how some treatments can also help with reducing the risk of other diseases. Obesity, for instance, is one of the major risk factors for heart disease, and weight loss drugs like Wegovy and Zepbound are having a significant impact on treating it. AHA previously reported that people taking Wegovy decreased their risk of heart attack, stroke, or death from cardiovascular issues by

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20%, compared to those taking a placebo. Acosta said this data was a “game changer” and marked a “new era” in the management of obesity and cardiovascular disease.

The panelists also highlighted the importance of genetic testing. Few people have their genetics tested, Brown said, and a priority for the AHA is encouraging people to do so.

Musunuru researches the genetics of heart disease and aims to identify genetic factors that protect against disease. Having genetic information, he said, can help medical practitioners know early on what patients’ risks are for developing certain diseases and can allow patients to take a “proactive” approach to their health.

“Your genes are the same on the day you’re born as the day you die,” Musunuru said. “If you know what’s in your genes at the time you’re born, that gives you a forecast of what your life will look like as it unfolds.”

TIME100 Talks: Reimagining the Future of Healthcare was presented by the American Heart Association.

AMERICAN KAHANI

COMMUNITY, LEAD STORIES

The Gene Editor-in-Chief: Dr. Kiran Musunuru's Race to Save a Baby With a Rare Genetic Disorder Affecting One in a Million

May 16, 2025

The Indian American researcher's race against time is said to stand as a testament to what becomes possible when scientific innovation meets human compassion.

In the predawn hours of a February morning in 2025, Dr. Kiran Musunuru stood anxiously in a hospital room at Children's Hospital of Philadelphia. Before him lay six-month-old KJ Muldoon, sleeping peacefully in the same crib that had been his home since birth. As a clear liquid flowed through an IV into the infant's tiny veins, Dr. Musunuru felt a conflicting surge of emotions.

The NEW ENGLAND JOURNAL of MEDICINE

BRIEF REPORT

Patient-Specific In Vivo Gene Editing to Treat a Rare Genetic Disease

K. Musunuru,[1,2] S.A. Grandinette,[2] X. Wang,[2] T.R. Hudson,[3] K. Briseno,[3] A.M. Berry,[2] J.L. Hacker,[2] A. Hsu,[4] R.A. Silverstein,[5] L.T. Hille,[5] A.N. Ogul,[3] N.A. Robinson-Garvin,[1] J.C. Small,[1] S. McCague,[1] S.M. Burke,[1] C.M. Wright,[1] S. Bick,[1] V. Indurthi,[6] S. Sharma,[6] M. Jepperson,[6] C.A. Vakulskas,[7] M. Collingwood,[7] K. Keogh,[7] A. Jacobi,[7] M. Sturgeon,[7] C. Brommel,[7] E. Schmaljohn,[7] G. Kurgan,[7] T. Osborne,[7] H. Zhang,[7] K. Kinney,[7] G. Rettig,[7] C.J. Barbosa,[8] S.C. Semple,[8] Y.K. Tam,[8] C. Lutz,[9] L.A. George,[1,2] B.P. Kleinstiver,[5] D.R. Liu,[4] K. Ng,[1] S.H. Kassim,[10] P. Giannikopoulos,[3,11] M.-G. Alameh,[1,2] F.D. Urnov,[3] and R.C. Ahrens-Nicklas[1,2]

SUMMARY

Base editors can correct disease-causing genetic variants. After a neonate had received a diagnosis of severe carbamoyl-phosphate synthetase 1 deficiency, a disease with an estimated 50% mortality in early infancy, we immediately began to develop a customized lipid nanoparticle–delivered base-editing therapy. After regulatory approval had been obtained for the therapy, the patient received two infusions at approximately 7 and 8 months of age. In the 7 weeks after the initial infusion, the patient was able to receive an increased amount of dietary protein and a reduced dose of a nitrogen-scavenger medication to half the starting dose, without unacceptable adverse events and despite viral illnesses. No serious adverse events occurred. Longer follow-up is warranted to assess safety and efficacy. (Funded by the National Institutes of Health and others.)

The authors' full names, academic degrees, and affiliations are listed at the end of the article. Dr. Ahrens-Nicklas can be contacted at ahrensnicklasr@chop.edu. Dr. Musunuru can be contacted at kiranmusunuru@gmail.com.

Dr. Musunuru and Ms. Grandinette contributed equally to this article.

This article was published on May 15, 2025, at NEJM.org.

DOI: 10.1056/NEJMoa2504747

Programmable gene-editing technology based on clustered regularly interspaced short palindromic repeats (CRISPR)–CRISPR-associated protein 9 (Cas9)[1] has matured into therapeutic approaches that are improving the lives of patients with various diseases, such as sickle cell disease, β-thalassemia, and hereditary angioedema.[2-4] Precise, corrective CRISPR-Cas9 technology — namely, base editing (which can effect cytosine-to-thymine changes [cytosine base editing[5]] or adenine-to-guanine changes [adenine base editing[6]]) and prime editing[7] (which can produce any single-nucleotide change or small insertion or deletion) — can potentially address more than 90% of pathogenic variants in genetic diseases that, although rare individually, collectively affect hundreds of millions of people worldwide.[8] However, drug-development efforts have largely focused on recurrent variants in a few relatively common genetic diseases on account of the extensive resources needed to develop and bring to market any given therapy.[9]

We developed a workflow for the rapid development of customized, corrective gene-editing therapies for patients with ultrarare or unique "N-of-1" variants (Fig. 1). More specifically, we developed a base-editing therapy, delivered in vivo to hepatocytes through lipid nanoparticles, for a single patient who at birth received

Baby Is Healed With World's First Personalized Gene-Editing Treatment

The New York Times

05-15-2025

KJ Muldoon was born with a rare genetic disorder, CPS1 deficiency, that affects just one in 1.3 million babies. Credit...Muldoon Family

The technique used on a 9½-month-old boy with a rare condition has the potential to help people with thousands of other uncommon genetic diseases.

By Gina Kolata

- May 15, 2025

Something was very wrong with Kyle and Nicole Muldoon's baby.

The doctors speculated. Maybe it was meningitis? Maybe sepsis?

They got an answer when KJ was only a week old. He had a rare genetic disorder, CPS1 deficiency, that affects just one in 1.3 million babies. If he survived, he would have severe mental and developmental delays and would eventually need a liver transplant. But half of all babies with the disorder die in the first week of life.

Doctors at Children's Hospital of Philadelphia offered the Muldoons comfort care for their baby, a chance to forgo aggressive treatments in the face of a grim prognosis.

"We loved him, and we didn't want him to be suffering," Ms. Muldoon said. But she and her husband decided to give KJ a chance.

IN THE LAB

CRISPR is used in landmark treatment to correct genetic misspelling of a single patient

Treatment of baby with rare disease could usher in era of personalized genome editing

Baby KJ with two of the researchers who treated him, Kiran Musunuru (left) and Rebecca Ahrens-Nicklas. Children's Hospital of Philadelphia

By Jason Mast May 15, 2025

General Assignment Reporter

STAT

FRONTIERS OF HEALTH AND MEDICINE

Kiran Musunuru, M.D., PhD, MPH, ML, MRA

Professor of Cardiology,

Professor of Pediatrics and Genetics,

At the University of Pennsylvania.

Has been selected as one of the top 50 most definitive impactful individuals in health, medicine and life sciences whose work has made headlines and will be celebrated at a gala in Boston on October 14th 2025

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The Philadelphia Inquirer

ALWAYS ASKING, ALWAYS PHILLY

New Jersey takes lead role in birthright defense

The state's solicitor general called on the Supreme Court to reject Trump's Day 1 executive order on citizenship.

By Jeff Gammage
Staff Writer

New Jersey led the legal argument against President Donald Trump's effort to end birthright citizenship on Thursday, its solicitor general urging the Supreme Court to bring clarity to the matter through a nationwide ruling.

It was not immediately evident what such a decision might say, but a majority of justices were concerned about the impact of even temporarily allowing the Trump administration to deny citizenship to children born to people who are in the country without official permission.

Trump has asked the court to rule that lower, federal district court injunctions can apply only to the

Kiran Musunuru (left), director of the Penn Cardiovascular Institute's Genetic and Epigenetic Origins of Disease Program, and Rebecca Ahrens-Nicklas, director of the Gene Therapy for Inherited Metabolic Disorders Frontier Program at Children's Hospital of Philadelphia, with KJ Muldoon at CHOP. Courtesy of Children's Hospital of Philadelphia

A baby with a rare metabolic disease

AN EDITION OF The Philadelphia Inquirer

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DAILY NEWS

FRIDAY MAY 16, 2025

LITTLE MIRACLE

THANKS TO GENETIC EDITING, CHOP AND PENN DOCTORS HAVE BABY KJ ON THE MEND PAGE 3

KJ Muldoon smiles while in treatment at Children's Hospital of Philadelphia. KJ was diagnosed with a rare genetic disorder and has been successfully treated with customized gene editing therapy. Courtesy of Children's Hospital of Philadelphia

HUNGER GAMES
FOOD-BOX PROGRAM FOR SENIORS FACING CUTS PAGE 4

UP IN SMOKE
SENATE PANEL REJECTS STATE STORE WEED PROPOSAL PAGE 6

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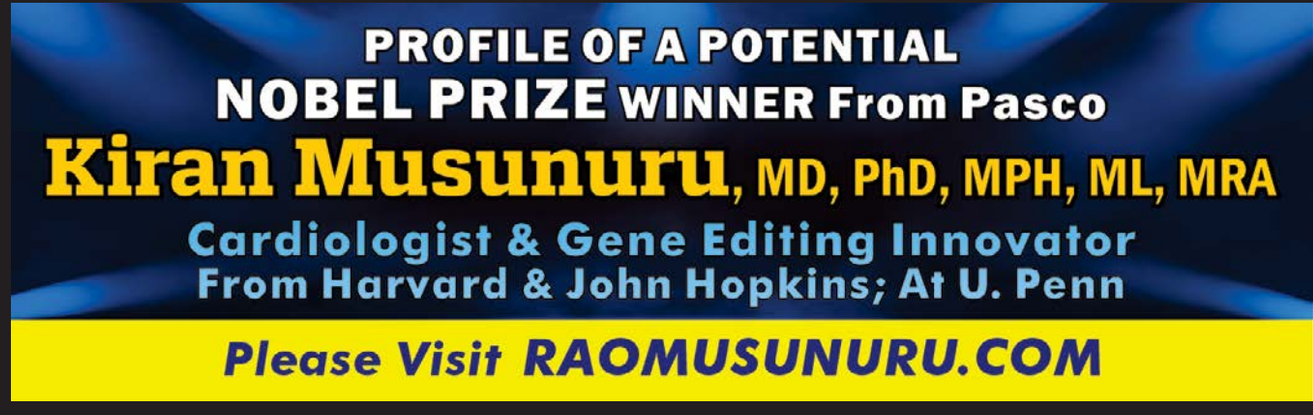
PROFILE OF A POTENTIAL
NOBEL PRIZE WINNER From Pasco
Kiran Musunuru, MD, PhD, MPH, ML, MRA
Cardiologist & Gene Editing Innovator
From Harvard & John Hopkins; At U. Penn
Please Visit RAOMUSUNURU.COM

Kiran Musunuru

M.D., PhD, MPH, ML, MRA

Recognitions of 2026

Continued...

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2026 Time -100 Invitation Letter

Dear Kiran Musunuru,

On behalf of Sam Jacobs and the editors of TIME, I'd like to let you know that you have been selected for the 2026 TIME100, our annual list of the hundred most influential people in the world.

As a member of the TIME100, you are invited to join us for the annual TIME100 Gala on Thursday, April 23, 2026 at Jazz at Lincoln Center in New York City. This intimate black tie dinner of 300 people—including many current and former TIME100 members—brings together the world's leading figures in politics, technology, science, philanthropy, media, business, and entertainment, and attracts worldwide media coverage.

Recent galas have featured musical performances from artists such as Taylor Swift, Jennifer Lopez, Dua Lipa, Mary J. Blige, Ed Sheeran, and John Legend. Previous guests include Michelle Obama, Nicole Kidman, Serena Williams, Michael B. Jordan, Ryan Reynolds, Patrick Mahomes, Emma Watson, Oprah Winfrey, Steven Spielberg, Simone

Continued...

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Biles, Sandra Oh, Drew Barrymore, Dwyane Wade, Michael J. Fox, Demi Moore, and Zendaya, as well as activists and thinkers who are making a difference in the world—whether fighting for human rights or seeking a cure for cancer.

You are also invited to attend the sixth-annual TIME100 Summit, which will take place during the day on Wednesday, April 22 at Jazz at Lincoln Center. The full list will be revealed during the week of April 15, so please keep the news confidential until it's published.

Our communications team will be in touch closer to launch with more details, including social assets and sample language for a press release.

We look forward to revealing this year's TIME100 list in full.

Best,

Cate Matthews
Executive Editorial Director, TIME

Time-100 Pioneers

April 27,2026 Page 32

Since its advent in 2012, CRISPR genome-editing technology has held the potential to change medicine. But getting from a research breakthrough to practical impacts on patients is no simple feat. In 2025, doctors Kiran Musunuru and Rebecca Ahrens-Nicklas showed it can be done. When baby KJ was born with a rare metabolic disease, giving him a 50% chance of dying in infancy, the pair led a remarkable multi-institutional team that designed, tested, and administered a personalized gene-editing therapy in just six months. Baby KJ is now home with his family and recently took his first steps.

This achievement goes beyond one child. Musunuru and Ahrens-Nicklas brought together innovations from across biomedical science and proved that on-demand CRISPR therapies can be developed safely and quickly enough to help patients who don't have years to wait. A decade from now, their names will be in medical textbooks, not only for baby KJ, but for opening the door to personalized genetic medicine for thousands of children after him.

by

Jennifer Doudna

Doudna is a Nobel Prize–winning biochemist and founder of the Innovative Genomics Institute

Friday, May 29, 2026
Today's Paper | Newsletters

The Philadelphia Inquirer

HEALTH

Share

A gene therapy lowered cholesterol in early trial results. It has a Philly backstory.

In a recent study, Verve's gene-editing therapy reduced cholesterol levels by 62% in patients receiving the highest dose. Penn cardiologist Kiran Musunuru was central to its origin story.

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by Kayla Yup
Published May 28, 2026, 5:01 a.m. ET

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Like countless Americans urged to take medicine every day to control high cholesterol, University of Pennsylvania cardiologist Kiran Musunuru frequently forgets to take his pills.

But unlike most patients, he has dedicated his career to trying to do something about it: develop a one-and-done gene therapy that would fix the condition without the daily hassle.

The company he co-founded, Verve Therapeutics, this week announced progress in early clinical trial results showing its gene-editing therapy had within a month reduced cholesterol levels by 62% in patients receiving the highest dose.

Although the numbers treated so far are small — the clinical trial included 35 participants — the early results were considered significant enough to be published in the *New England Journal of Medicine*. Those with the strongest results were studied for up to 90 days, with evidence of durable effects, while those at the lowest dose have been studied up to 18 months.

The patients involved either had heart disease at a younger age than usual, or a genetic disorder that increases the risk of heart disease.

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The therapy, called VERVE-102, works by introducing a specific mutation into a gene in the liver called PCSK9 — much like changing a letter in a string of words.

One to three percent of people naturally have these "good misspellings" that turn the gene off and confer "dramatic protection against heart disease," said Musunuru, who was not involved in the trial, but helped to develop the therapy's concept in its early stages.

Pharmaceutical giant Eli Lilly obtained the therapy last year in a $1 billion buyout of Boston-based Verve Therapeutics. Musunuru serves as an adviser, but is not employed by Verve and no longer has a stake in the company.

Now focused on developing personalized gene-editing therapies, Musunuru was part of the team behind last year's first-of-its-kind treatment for Baby KJ at Children's Hospital of Philadelphia. The novel therapy targeted a genetic mutation in the infant with a rare metabolic disease.

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Musunuru found that inactivating the gene in mice led to substantial drops in levels of bad cholesterol. People who naturally had the gene turned off also had these benefits with seemingly no adverse health consequences.

This made the gene "the perfect drug target," he said.

Independent researchers called the recent trial results exciting, telling STAT News and the New York Times that while they appeared to be lasting, more safety data was needed. The Food and Drug Administration generally expects patients in gene-editing trials to be followed for 15 years.

Larger trials will be needed to ensure the therapy is safe and effective, Musunuru said. He was not an author on the published study.

If it can permanently reduce cholesterol levels as hoped, "it will push off heart attacks and strokes by decades on average," he said.

One-and-done for high cholesterol?

When too much cholesterol — a fatlike, waxy substance — builds up in the body, it can clog arteries and reduce blood flow to the heart.

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Musunuru is one of millions worldwide who take daily statins to lower their cholesterol. Other options include monoclonal antibodies taken every few weeks and injections every six months.

Gene therapy potentially could mean permanent protection.

Research has found that only two in five patients who are prescribed a statin after a heart attack are still taking it as directed two years later. Others lowered or skipped doses, or stopped taking it altogether.

“Drugs don’t work if you don’t take them,” Musunuru said.

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Safety also needs to be evaluated in much larger study populations.

No major adverse events related to the therapy were reported in the latest study, but a trial for Verve's previous therapy candidate was paused after a patient developed elevated liver enzymes. This led to a safer reformulation of the delivery vehicle for the current therapy, Musunuru said.

If the therapy does prove safe and effective, he envisions a world where everyone could receive the gene therapy to prevent heart disease, not just those at high risk.

That's not to say everyone will actually want gene therapy.

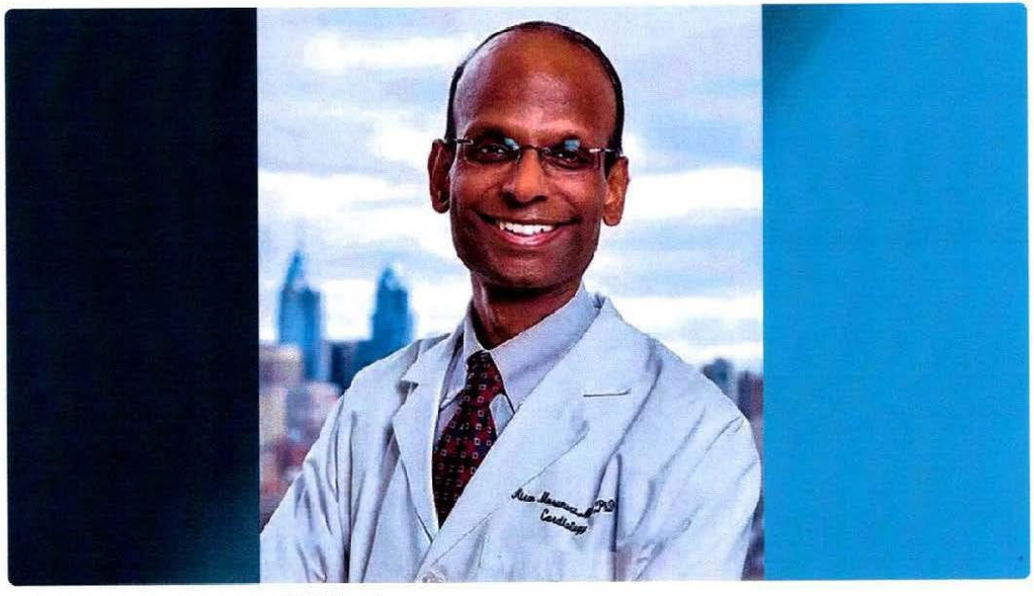
The field of gene editing is still relatively new and experimental. The first FDA-approved therapy that edited DNA using CRISPR technology was approved for sickle cell disease in 2023.

Cost and accessibility are other commonly cited challenges. Gene therapies can be prohibitively expensive, with prices reaching the millions. That's compared to statins, which are relatively cheap. Lilly's chief scientist told the New York Times they intended for a product that could be widely accessible.

"The more options you have, the better it is for everyone," Musunuru said.

2026 TIME-100 List: Telugu-Origin Cardiologist Kiran Musunuru Among World's Most Influential People

The 2026 TIME's 100 Influential People list features Google CEO Pichai, chef Vikas Khanna, actor Ranbir, YT CEO Neal, New York Mayor Mamdani among others.



Cardiologist Kiran Musunuru (ETV Bharat)

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By ETV Bharat English Team
Published : April 16, 2026 at 2:51 PM IST

Amaravati: Renowned Indian-origin cardiologist Kiran Musunuru has been named in TIME's prestigious 100 Most Influential People list for 2026, bringing global recognition to his innovative work in heart disease research. The annual list, released on Wednesday, features leaders, innovators, artists and cultural icons from around the world.

Musunuru, born in New York in 1976 to a Telugu family originally from Andhra Pradesh, was recognised for his groundbreaking contributions to cardiovascular science, particularly his pioneering use of advanced gene-editing technologies to prevent heart disease. Through his research, he has explored how genome editing can target genes involved in lipid metabolism, potentially leading to a

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'vaccine-like' preventative approach against heart attacks - a breakthrough with far-reaching implications for global health.

Musunuru is among several Indian and Person of Indian Origin (PIO) figures who featured on this year's TIME 100 list, reflecting the growing global footprint of Indian talent across sectors:

Sundar Pichai, CEO of Google, recognised for his leadership in steering the tech giant toward expanding artificial intelligence and innovation.

Vikas Khanna, the Michelin-starred chef and humanitarian, honoured for using food as a medium to connect cultures and foster empathy.

Ranbir Kapoor, Bollywood star featured for his global cultural impact and storytelling that resonates beyond Indian cinema.

Neal Mohan, CEO of YouTube, noted for leading the platform's expansion and influence in digital media.

Zohran Mamdani, New York City Mayor whose rise represents the influence of Indian-American leaders in global civic governance.

The list spans technology, culture, science, politics and global leadership, signalling a broad and diverse representation of impact. In addition to Indians and Indian-origin honourees, global figures such as US political leaders President Donald Trump, Marco Rubio who doubles as the US Secretary of State and National Security Adviser, Canadian Prime Minister Mark Carney, heads of state President Xi Jinping of China, innovators and cultural icons also made the cut.

Musunuru's Pathbreaking Work

Joining the ranks of global luminaries, Musunuru's inclusion celebrates his pioneering work in applying advanced gene-editing techniques to prevent and treat heart disease. His remarkable contribution, which involves creating a potential 'vaccine-like' approach to heart attack prevention, positions him as a key figure in the field of cardiovascular science.

Kiran Musunuru's inclusion marks a major milestone in recognising the impact of genetic research on cardiovascular health. Building on his father's legacy - his father, Koteswara Rao Musunuru, migrated from Andhra Pradesh in 1976 and established a career in cardiology - the younger Musunuru has

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focused on translating cutting-edge science into real-world solutions. His work targets the genetic basis of heart disease, aiming to intercept heart attacks before they occur.

TIME 100 is widely regarded as one of the most prestigious compilations of influential individuals worldwide. Being included on the list not only highlights personal achievement but also underscores the broader influence of Indian thought leadership and innovation on the global stage.

Invitation to Receive the Eduard Buchner Prize 78th Mosbacher Kolloquium (GBM March 2027)

Dear Professor Musunuru,

On behalf of the organizers Thomas Oellerich and myself, it is our great pleasure to invite you to the **78th Mosbacher Kolloquium**, the annual spring meeting of the German Society for Biochemistry and Molecular Biology (GBM), which will take place **March 18–20, 2027**, in Mosbach/Baden, Germany.

The Mosbacher Kolloquium is a long-standing and highly interactive meeting that brings together leaders in basic, translational, and clinical research. The 2027 meeting will focus on the theme "**Precision Medicine: The Genome and Beyond**", spanning target discovery, genome-based and molecular therapies, novel drug modalities, and their translation into clinical practice.

We are delighted to inform you that the GBM has selected you as the recipient of the **2027 Eduard Buchner Prize**, in recognition of your outstanding and influential contributions to genome editing and precision medicine. The Buchner Prize is one of the highest distinctions awarded by the GBM and we would be deeply honored if you would accept the prize and our invitation.

We would be delighted if your lecture could reflect your perspective on the development and translational potential of genome editing technologies in precision medicine.

The Mosbacher Kolloquium attracts a broad and international audience and is particularly known for its open, collegial atmosphere and lively scientific discussion, with approximately one-third of participants being early-career researchers.

The GBM will, of course, be pleased to cover your travel expenses (within the applicable regional limits; see attached overview) as well as your hotel accommodation in Mosbach, which will be arranged centrally by the GBM office. All details regarding reimbursement will be communicated to you well in advance.

We would be truly honored if you would accept the Eduard Buchner Prize and our invitation to participate in the 78th Mosbacher Kolloquium. We would be very happy to discuss any questions, preferences, or practical aspects at your convenience and sincerely hope to hear from you.

With kind regards,

Manuel Kaulich & Thomas Oellerich

2026 Articles:

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