

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.

A circular frame with a dark border contains a repeating gold-colored floral and leaf pattern. In the center of this frame is a rectangular, parchment-like area with a dark border. Inside this rectangle, the text "HOME is where your story begins." is written in a black, serif font. The word "HOME" is in all caps, while the rest of the text is in lowercase. The background of the entire slide is dark grey, with a colorful, wavy, rainbow-like gradient at the top right corner.

*HOME is
where your
story
begins.*

**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.





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.

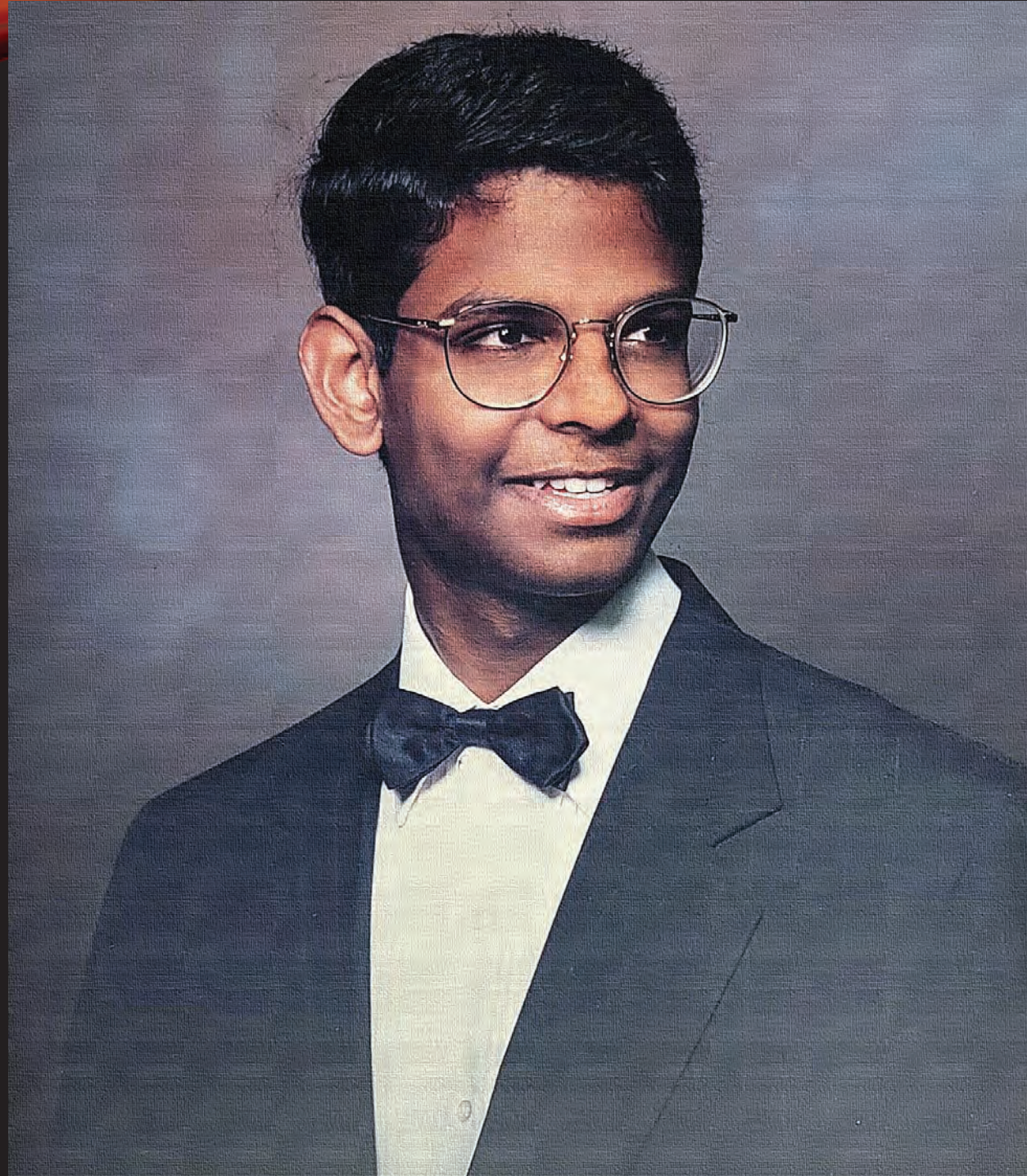




Kiran's personal book library during his school years. (Part 1)



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



**Kiran,
the
Performer**



College graduation at Harvard with dad.

Journal of
**UNDERGRADUATE
SCIENCES**

Dedicated to the Advancement of Undergraduate Research and Education

Vol. 2, No. 2

Winter 1995



Focus: Medicine & Health

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

o n c o l o g y

Cell Cycle Regulators in Cancer

Kiran Musunuru
Philip W. Hinds

KARGER
LANDES
SYSTEMS

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

Days of
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The meet-
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arch com-
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'late bloomers.'

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.



**Brigham Internal
Medicine Survival Guide**
2006-2007



ICU/CCU Primer
1st edition, May 2007

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

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.

VOLUME 1 | ELEVENTH EDITION

BRAUNWALD'S

HEART DISEASE

A TEXTBOOK OF
CARDIOVASCULAR
MEDICINE



Enhanced
DIGITAL
VERSION
Included.

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.

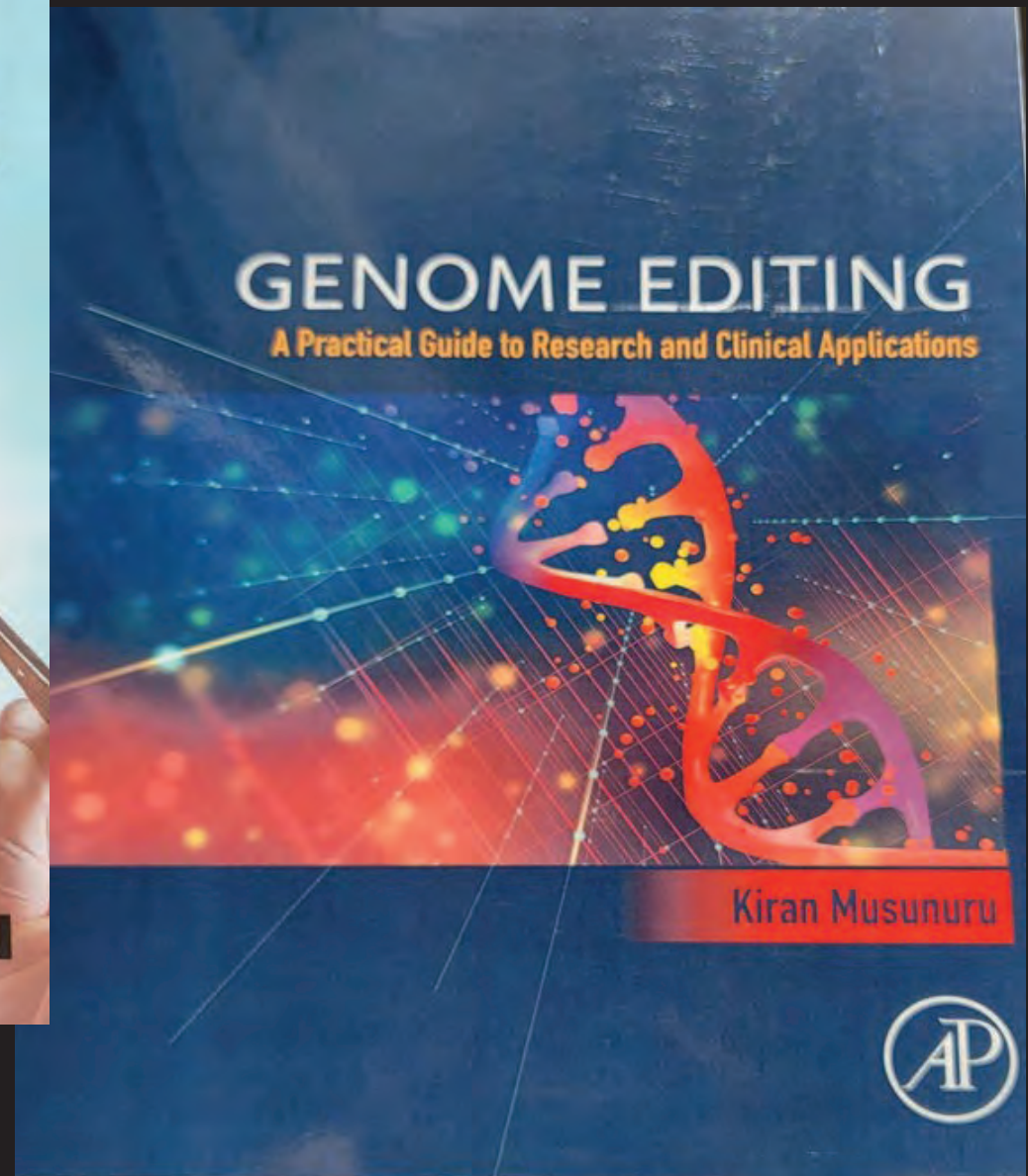
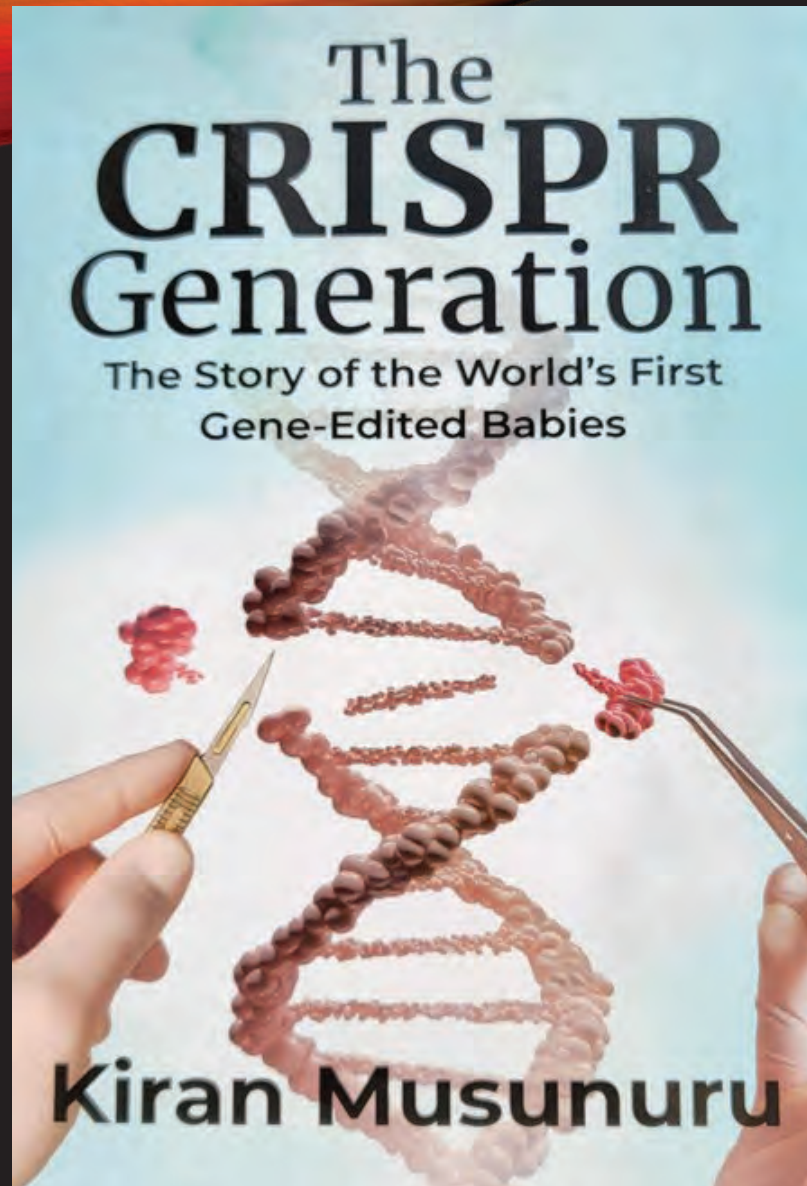
Are we trying to play God? I wouldn't even venture to go there in this column. However, I cannot help but wonder why the creator is empowering humans with super-intelligence to decode all the secrets of creation and the means to alter it in no small measure. What is the creator's ultimate plan? Only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

REIMAGINING THE FUTURE OF HEALTHCARE

PRESENTING PARTNER



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

BY CHANTELE 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.

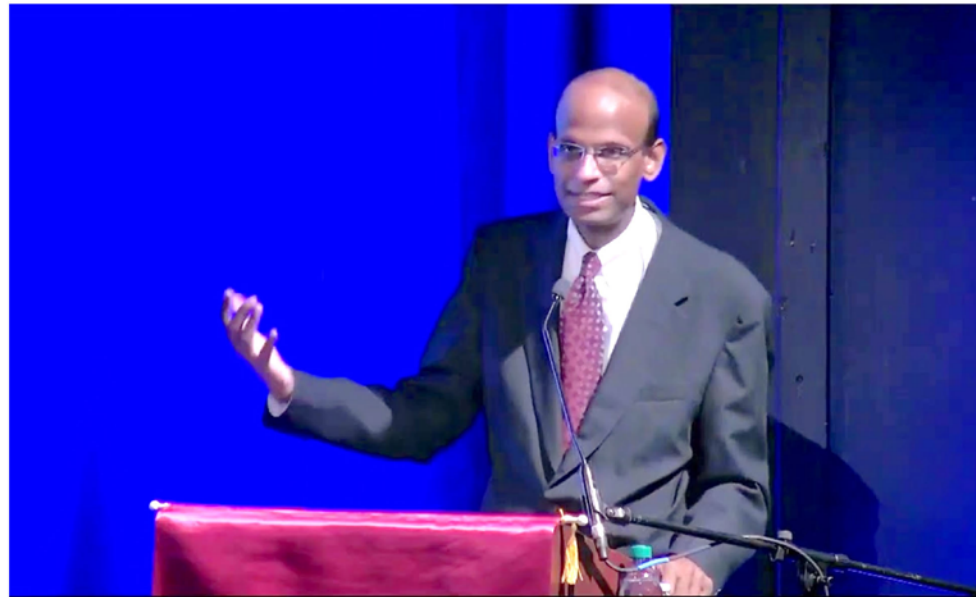
AMERICAN KAHANI

[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.

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](https://www.nejm.org).

DOI: 10.1056/NEJMoa2504747

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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 editing⁵] or adenine-to-guanine changes [adenine base editing⁶]) and prime editing⁷ (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



BUSINESS A15 SHAPIRO PUSHES FOR LIMITS ON PRIVATE EQUITY IN HEALTHCARE

SPORTS C1 WHAT A RELIEF! INSIDE JORDAN ROMANO'S REBOUND

SPORTS C7 PREAKNESS STAKES ODDS AND PICKS

LIFE & CULTURE B8 TREASURE HUNTING FOR \$4,000 IN REGIONAL PARKS

FRIDAY, MAY 16, 2025 VOL. 166, NO. 200 ESTABLISHED IN 1879 CITY & SUBURBS | C | \$2.95

The Philadelphia Inquirer



KJ Muldoon (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

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 signify, 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 that apply only to the

that lower, federal district court injunctions can apply only to the

AN EDITION OF Single copy price may be higher in nonpaying areas \$2.95

The Philadelphia Inquirer

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
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