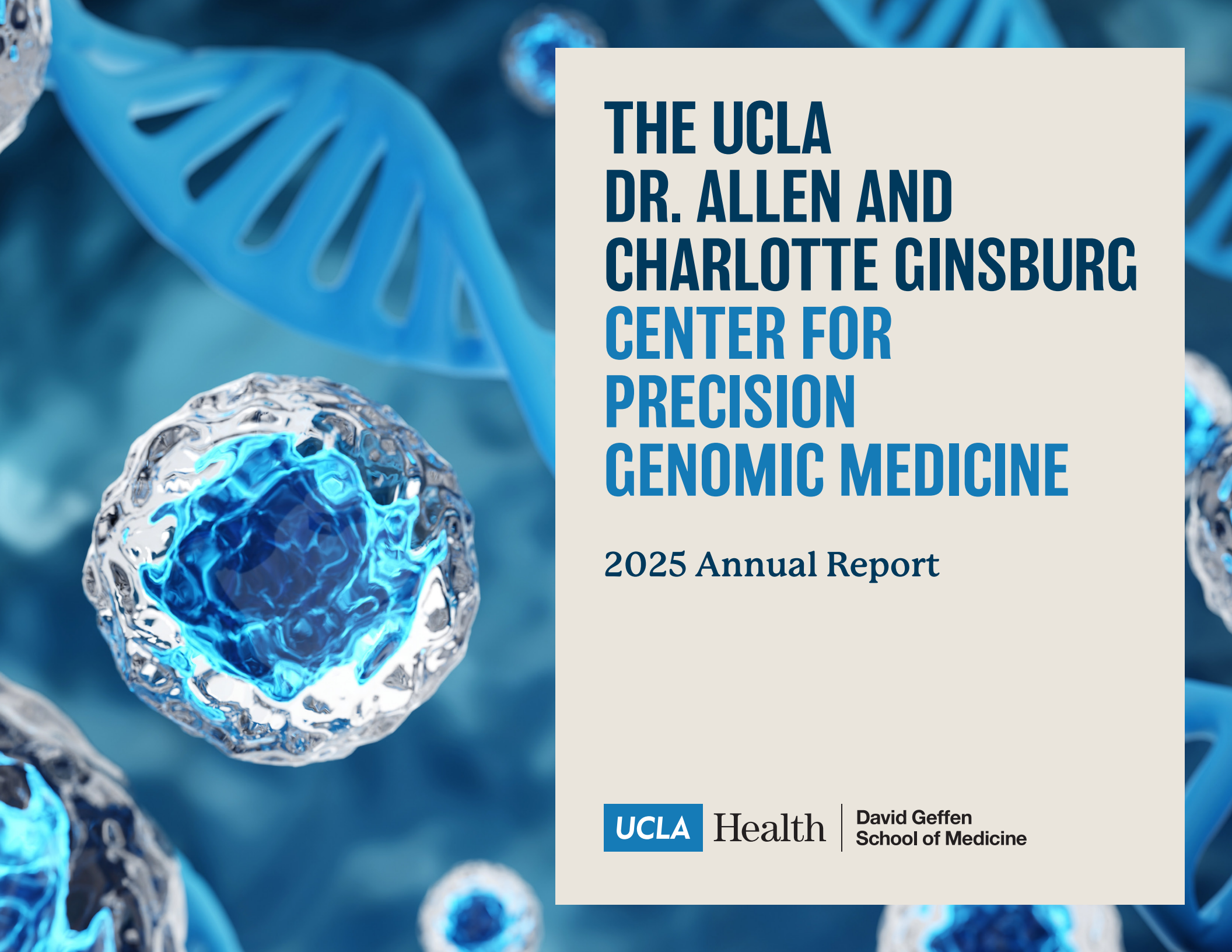




THE UCLA DR. ALLEN AND CHARLOTTE GINSBURG CENTER FOR PRECISION GENOMIC MEDICINE

2025 Annual Report

UCLA

Health

**David Geffen
School of Medicine**



INTRODUCTION

The emerging field of precision health is poised to transform modern medicine, and the UCLA Dr. Allen and Charlotte Ginsburg Center for Precision Genomic Medicine sits at the forefront of this oncoming revolution. By harnessing the power of precision genomic medicine, researchers and clinicians will not only improve the diagnosis and treatment of disease but also anticipate and prevent it, ultimately extending longevity and quality of life. When genomic data is combined with insights into a person's lifestyle and environment, it becomes a robust tool for predicting treatment responses and identifying potential health risks. This gives patients greater knowledge about their own health and the opportunity to take proactive, preventive steps.

Already a leader in this area of medicine, the center is driving the creation of advanced platforms for big-data integration, clinical diagnostics and patient biobanking, expanding the reach and impact of precision health. The generosity of Dr. Allen and Charlotte Ginsburg has been essential in propelling the center's mission and increasing its ability to serve patients and researchers alike.

ENDOWED CHAIRS

Chairs endowed by the Dr. Allen and Charlotte Ginsburg Research Foundation are key in UCLA's ability to attract and retain exceptional physician-scientists working at the vanguard of precision genomic medicine. The annual endowment payout supplies flexible resources that fuel scholarly and scientific progress, supporting new research directions, postdoctoral training, professional development and other academic pursuits. These chairs strengthen UCLA's prominence in precision health while creating a lasting connection between the Ginsburg family and the scientific advances their philanthropy makes possible.

**Dr. Allen and Charlotte Ginsburg
Endowed Chair in Precision Genomic Medicine**



The Dr. Allen and Charlotte Ginsburg Endowed Chair in Precision Genomic Medicine helped to recruit **Nicola Longo, MD, PhD**, a globally recognized metabolic geneticist and

innovator in genomics. Dr. Longo was also named director of the UCLA Dr. Allen and Charlotte Ginsburg Center for Precision Genomic Medicine and chief of the UCLA Division of Clinical Genetics.

In his first year as the inaugural chair holder, Dr. Longo's overarching goal was to establish the Division of Clinical Genetics as the premier multidisciplinary hub for translational genomics research and clinical care in the David Geffen School of Medicine at UCLA. To this end, the division has expanded its outreach activity to engage new geneticists, resulting in three genetics residents at UCLA Health. On July 1, 2026, the division will add a biochemical genetics fellow.

Dr. Longo has also launched clinical investigations to bring new therapies to patients with genetic diseases. He is starting a trial for gene therapy targeting Fabry disease, a rare lysosomal storage disorder characterized by severe pain, eye problems and other distressing symptoms. Another clinical trial is testing small molecules to treat phenylketonuria, a rare metabolic disease, which can lead to brain damage and intellectual disability. Additional trials are slated to start later this year.

Dr. Longo continues to research brain creatine deficiencies. When diagnosed and treated early, seizures and developmental delays associated with the deficiencies can be prevented. Dr. Longo is working with UCLA colleagues to establish gene therapy for one such condition, creatine transporter deficiency.

Another of Dr. Longo's primary projects focuses on creating a dynamic medical genetics clinic in the Dr. Allen and Charlotte Ginsburg Clinical Research Suite, where basic scientists and clinicians can

integrate their work. He and Deborah Krakow, MD, chief genomics officer, are partnering to incorporate genetic testing as a standard offering for all UCLA Health patients. Dr. Krakow is building a precision prevention clinic in the center to counsel and care for those with genetic diseases.

The suite houses the Division of Clinical Genetics, which, in addition to its research efforts, provides comprehensive evaluations and genetic counseling for individuals with genetic disorders and congenital abnormalities. Its expert team is uniquely equipped to diagnose a broad spectrum of conditions, including rare diseases.

A new state-of-the-art gene therapy infusion suite is being built within the larger research suite. The hospital-licensed, three-room gene therapy infusion suite will be alongside the clinic, enabling patients to receive truly integrated care in one location.

While renovations to the suite are underway, patients continue to receive vital services. Once completed, faculty and staff will more easily administer gene

therapy and other advanced treatments for genetic conditions.

Dr. Allen and Charlotte Ginsburg Endowed Chair in Translational Genomics



Stanley F. Nelson, MD, distinguished professor of human genetics, neurology, pediatrics, psychiatry, and pathology and laboratory medicine, is the inaugural holder

of the Dr. Allen and Charlotte Ginsburg Endowed Chair in Translational Genomics. He directs the UCLA Clinical Genomics Center, which offers clinical exome sequencing; the UCLA Center for Duchenne Muscular Dystrophy; and the California Center for Rare Diseases (CCRD).

Dr. Nelson's laboratory is building the world's most comprehensive single-cell map of Duchenne muscular dystrophy (DMD) skeletal muscle. This resource is revealing new therapeutic targets and clarifying how muscle responds to gene therapy, which was first approved for DMD in 2023. Current DMD gene therapy delivers a viral vector to all

muscle cells to replace the missing DMD gene. Dr. Nelson is identifying strategies to enhance the therapy's effectiveness, with the goal of slowing — and ultimately reversing — progressive muscle loss characteristic of DMD. A series of publications detailing these groundbreaking advances is in preparation and being presented at national scientific meetings.

Families travel from around the world to UCLA, where they can receive DMD care while participating in translational research. Dr. Nelson's team pioneered minimally invasive methods to repeatedly sample muscle, enabling unprecedented insight into the long-term effects of one-time gene therapy. This work is creating a blueprint for personalized medicine, informing individualized pre- and post-treatment care to optimize artificial gene expression and support muscle regeneration. His efforts are also accelerating the development and clinical adoption of future gene therapies for other rare, life-threatening genetic diseases.

In the past year, Dr. Nelson's group secured major new funding from the California Institute of Regenerative Medicine and the United States Department of Defense to investigate the durability of gene therapy, immune responses that limit its benefit, and whether muscle scarring can be reversed to improve outcomes.

Like the Ginsburg Center, the CCRD remains a priority for the UCLA Institute for Precision Health, and the two entities complement and leverage each other. The CCRD seeks to solve the mainly genetic rare diseases affecting 1 in 20 Americans. Dr. Nelson and colleagues use team-based clinical evaluation and sophisticated genomic analyses invented at UCLA in coordination with the Undiagnosed Diseases Network and the National Organization for Rare Disorders to identify the causal mutation; therapy can then begin. The team's work has led to the discovery of dozens of previously unknown genetic disorders. Much of the patient-facing work occurs in the Ginsburg Clinical Research Suite.

MATERNAL AND FETAL MEDICINE/CLINICAL GENETICS

Chief genomics officer



Dr. Krakow, who joined the Ginsburg Center this past year, is assistant dean of interdepartmental genetics and genomics; professor of human genetics, obstetrics and gynecology, and orthopedic surgery; and chief genomics officer for UCLA Health. She also holds the Joshua S. and Beth C. Friedman Chair for Women's Genetic Research. Dr. Krakow is board certified in clinical genetics and genomics, maternal and fetal medicine, and obstetrics and gynecology. In addition, she is co-director of the UCLA International Skeletal Dysplasia Registry, a research database of rare genetic skeletal disorders that helps to facilitate diagnosis, the identification of causative genes and biological pathways involved, and the development of potential treatments.

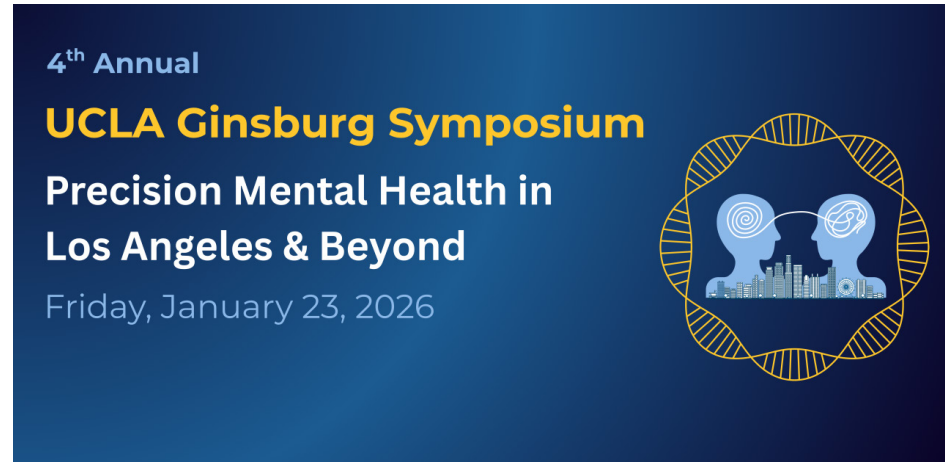
Dr. Krakow's interests align closely with the mission of the Ginsburg Center, and she is eager to make UCLA the world's leader in personalized medicine.

Her research focuses on:

- Prenatal ultrasound parameters for osteochondrodysplasias (a rare, diverse set of inherited conditions caused by genetic mutations affecting cartilage and bone development)
- The molecular basis of musculoskeletal disorders
- Gene expression patterns in the skeleton
- Disorders of joint development
- The role of cilia in skeletal dysplasias
- Osteogenesis imperfecta (brittle bone disease)
- Preclinical models of human disease

She has completed one clinical trial in rare diseases and has two ongoing.

UCLA GINSBURG SYMPOSIUM



The UCLA Institute for Precision Health hosted the fourth annual Dr. Allen and Charlotte Ginsburg Symposium on precision mental health on January 23, 2026, at the UCLA Meyer and Renee Luskin Conference Center.



The full-day event showcased numerous innovations in precision mental health, including early detection of depression, personalized therapies, and the integration of digital tools and wearable technologies in research.

Guillermo Sapiro, PhD, delivered the keynote speech. Dr. Sapiro is the Augustine Family Professor in

Engineering at Princeton University and a professor of electrical and computer engineering. He is also a distinguished engineer with Apple, Inc., where he heads a team on health artificial intelligence. His work focuses on theory and applications in computer vision, computer graphics, medical imaging, image analysis and machine learning. Dr. Sapiro has authored and co-authored over 500 papers and a book on these topics. He is the only scientist to have received the prestigious Test-of-Time Award at both the International Conference on Computer Vision and the International Conference on Machine Learning.

Nine additional trailblazers in the field covered exciting topics, such as the use of electronic health records in precision psychiatry and digital phenotyping.

All UCLA graduate students and postdoctoral fellows were encouraged to submit an abstract for the poster session. Following the presentations, panel discussions allowed speakers to delve deeper into their respective topics.

UCLA DR. ALLEN AND CHARLOTTE GINSBURG FELLOWSHIP IN PRECISION GENOMIC MEDICINE

2025-26 fellows

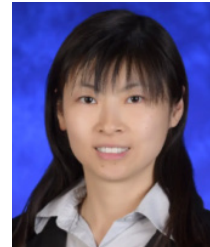
Two fellows were selected for 2025-26, expanding the center's expertise and advancing research in translational precision genomic medicine.



Jennifer Chia, MD, PhD, is working on a project, “Comparative Multiomics to Dissect Heterogeneity in Inflammation and Aging of the Bone Marrow Niche.” She is a health sciences clinical

instructor of pathology and laboratory medicine at the David Geffen School of Medicine. Dr. Chia graduated from the Weill Cornell / Rockefeller / Sloan Kettering Tri-Institutional MD-PhD Program, concentrating on immunology and microbial pathogenesis for her PhD. She then completed residency in anatomic pathology and a fellowship in hematopathology at UCLA. As a postdoctoral fellow in the lab of Alexander Hoffmann, PhD, her research is centered on understanding

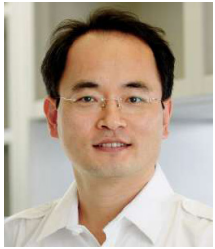
the interplay between immune dysregulation and hematologic disorders across the age span.



Ting Zhang, MD, PhD, is furthering her project, “Identification of Molecular-Neuropathological Signatures of Sporadic AD and FTLD.” She received her medical degree from

Shanghai Jiao Tong University School of Medicine and PhD in molecular, cellular and integrative physiology from UCLA. Dr. Zhang also completed her residency in pathology and laboratory medicine at UCLA and is currently enrolled in a neuropathology fellowship. Her research focuses on single-cell spatial multiomic analysis of multiple brain regions from individuals affected by Alzheimer's disease, frontotemporal lobar degeneration and COVID-19.

GENOMIC TECHNOLOGY DEVELOPMENT FUND



The CRISPR core was launched four years ago with the hiring of **Kitai Kim, PhD**, as director of the Dr. Allen and Charlotte Ginsburg Human Stem Cell and Genome Engineering Center. Dr. Kim has hired and trained three additional scientists to provide induced pluripotent stem cell and gene editing services, using CRISPR and Talen, to the broader UCLA community. Within a short period of time, UCLA investigators have created several dozen pluripotent human stem cell lines and conducted numerous gene-editing projects.

In mid-October, the team launched a targeted marketing initiative aimed at 105 stem cell and genome engineering cores across the U.S. and Canada, offering access to the Ginsburg Center's CRISPR core services. Institutions responded enthusiastically, as outsourcing to UCLA enables them to broaden their service capabilities without

making their own capital investments. To date, interested institutions include the Georgia Institute of Technology, Indiana University, Johns Hopkins University, Sanford Burnham Prebys Medical Discovery Institute, Scripps Health, Stanford University, Tulane University, the University of Alabama, the University of California at Berkeley, the University of California at Santa Cruz, the University of Colorado Boulder, the University of Maryland and the University of Southern California. This model, combined with grant funding, will sustain the core long term.

THE RESEARCH ACCELERATION FUND

This past year, the Research Acceleration Fund supported the development of the Biomedical Data Commons (BDC), expected to launch in October 2026. The BDC is designed to transform how clinical and genomic information is used in research and patient care at UCLA. The team has secured a contract with DNAnexus, a leading, user-friendly platform for genetic analysis, cohort identification and data sharing.

UCLA has built the most diverse patient biobank in the nation, enrolling more than 250,000 participants through the ATLAS Community Health Initiative. To date, approximately 155,000 specimens have been collected, yielding over 125,000 DNA samples and more than 117,000 whole-exome sequencing results available for studies. Insights from this unprecedented dataset are already helping predict, prevent and treat disease with greater precision than ever before.

Unlocking the full research and clinical potential of this vast resource requires pairing of advanced data science tools, such as artificial intelligence and machine learning, with clinical and genetic data. This is not a task for a single laboratory; it demands an institution-wide effort and a new generation of computational infrastructure capable of securely housing, analyzing and sharing data at scale.

The UCLA Institute for Precision Health and the Ginsburg Center are meeting this challenge by launching the BDC. The BDC is positioning UCLA

to fuel progress for decades, accelerating the evolution of new diagnostic tools and treatment strategies.

The BDC will shape the future of medicine in multiple ways. By making genetic and clinical data readily accessible, it will empower researchers to uncover lifesaving breakthroughs more rapidly — from novel cancer therapies to treatments for rare diseases. It will also enable clinician-scientists to translate data into personalized care, allowing physicians to anticipate disease before symptoms appear, tailor therapies to individual patients, and reduce trial-and-error approaches.

In addition, the BDC will strengthen the integration of research and clinical practice by delivering efficient, cost-effective data solutions that dramatically shorten the time between genomic discovery and clinical implementation. Its robust infrastructure bolsters UCLA's standing as a global authority on biomedical research, attracting top scientific talent and opening new avenues for funding.

Especially important, because the ATLAS biobank is the most diverse in the nation, the BDC will help ensure that genetic insights benefit all communities, increasing health equity.

The UCLA Biomedical Data Commons is more than a research platform; it is a catalyst for a new era in how disease is diagnosed, treated and prevented. This forward-looking initiative will transform the latest science into real-world cures and create a lasting legacy for generations to come.

AN ENDURING PARTNERSHIP

With a new \$31 million commitment to the UCLA Research Park, the Ginsburgs have further solidified their standing as champions of biomedical advancement, bringing their total contributions to these university initiatives to \$60 million. The investment is intended to cultivate future leaders, accelerate discovery, and provide the laboratory resources needed to drive new breakthroughs in basic, translational and clinical science. Their philanthropy strengthens the full arc of innovation — from early

findings to real-world solutions — and deepens collaboration across disciplines. It also bolsters the work of early-career investigators, who often face steep challenges as they build teams and establish their portfolios.

The gift directs \$11 million to the Dr. Allen and Charlotte Ginsburg Fund for Graduate Student Support, which will equip graduate students, doctoral candidates and postdoctoral scholars with financial and research assistance. These awards will help cover research costs, tuition, books, housing and related expenses. This funding will give an opportunity to selected emerging scientists to train in a world-class environment and launch their careers with momentum. Recipients, chosen through a competitive review by the David Geffen School of Medicine, will be recognized as Ginsburg Scholars or Ginsburg Fellows, forming successive cohorts of highly talented researchers.

Another \$10 million will establish the Dr. Allen and Charlotte Ginsburg Biomedical Frontiers Fund.

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2025 ANNUAL REPORT

A selection committee, convened by the medical school dean, will award funding to promising investigations and teams from the school of medicine, the quantum innovation hub and the California Institute for Immunology and Immunotherapy, all of whom will conduct their work at the UCLA Research Park.

The final \$10 million will go toward the capital needs of the UCLA Research Park, including laboratories and advanced tools that will anchor research planned for the site.



Articles about the Ginsburgs' most recent philanthropy were published in *Philanthropy News Digest*, *Los Angeles Business Journal*, *Lifestyles Magazine*, the *Daily Bruin*, *Morningstar*, *StreetInsider*, *Yahoo Finance* and more.

In honor of the couple's generosity, UCLA will name the Dr. Allen and Charlotte Ginsburg Foyer, the Dr. Allen and Charlotte Ginsburg Paseo, and the Dr. Allen and Charlotte Ginsburg Innovation Bridge in the UCLA Research Park. This recognition honors the couple's significant investments in biomedical research, UCLA's dedicated scientists and the spaces that make their work possible. UCLA is proud and grateful that these spaces will bear the Ginsburg family name.

WITH DEEP GRATITUDE

UCLA Health and the Institute for Precision Health sincerely appreciate the Ginsburgs' extraordinary commitment to establishing the UCLA Dr. Allen and Charlotte Ginsburg Center for Precision Genomic Medicine, as well as the new gift to the UCLA Research Park. Such generosity sets in motion new training initiatives, novel translational genomic discovery and improvements in patient care.

The Ginsburg Center's work in human-genome sequencing and the creation of patient-specific

disease models is illuminating the genetic foundations of illness in ways that were once unimaginable. These efforts are laying the foundation for more precise diagnoses and treatments tailored to each individual — and eventually disease prevention.

The Ginsburgs' vision continues to elevate researchers at every stage of their careers, expand possibility for patients and strengthen precision genomic medicine. The impact of this philanthropy is enduring and far reaching, shaping the scientific landscape for generations to come.

