RISING TO NEW HEIGHTS

IN CELL AND GENE THERAPY

Gates Institute

UNIVERSITY OF COLORADO
ANSCHUTZ MEDICAL CAMPUS

2024
ANNUAL REPORT

Gates Institute is a world-class translational research institute with biomanufacturing capabilities.

WHO WE ARE

Based at the University of Colorado Anschutz Medical Campus, we are part of a rich biomedical ecosystem, enabling the development of life-saving treatments for a host of diseases at unmatched speed and scope. We bring together and support researchers and clinicians who specialize in regenerative, cell and gene therapies, accelerating their discoveries from concepts to cures.

OUR MISSION

We positively transform patient lives by advancing science through discovery, development, and delivery of biologic, cellular and gene-based therapies.

OUR VALUES

Patient Driven
We prioritize patients, especially those with rare or difficult-to-treat conditions, through a commitment to develop, manufacture, and deliver innovative cell and gene therapies.

Find a Way
We overcome challenges with resilience and a solution-oriented mindset, turning setbacks into opportunities to advance science and change the practice of medicine.

"No one does their best work alone." —Charles C. Gates
Teamwork leads to excellence, where diverse skills and perspectives come together to achieve success.

Gates Institute
UNIVERSITY OF COLORADO
ANSCHUTZ MEDICAL CAMPUS

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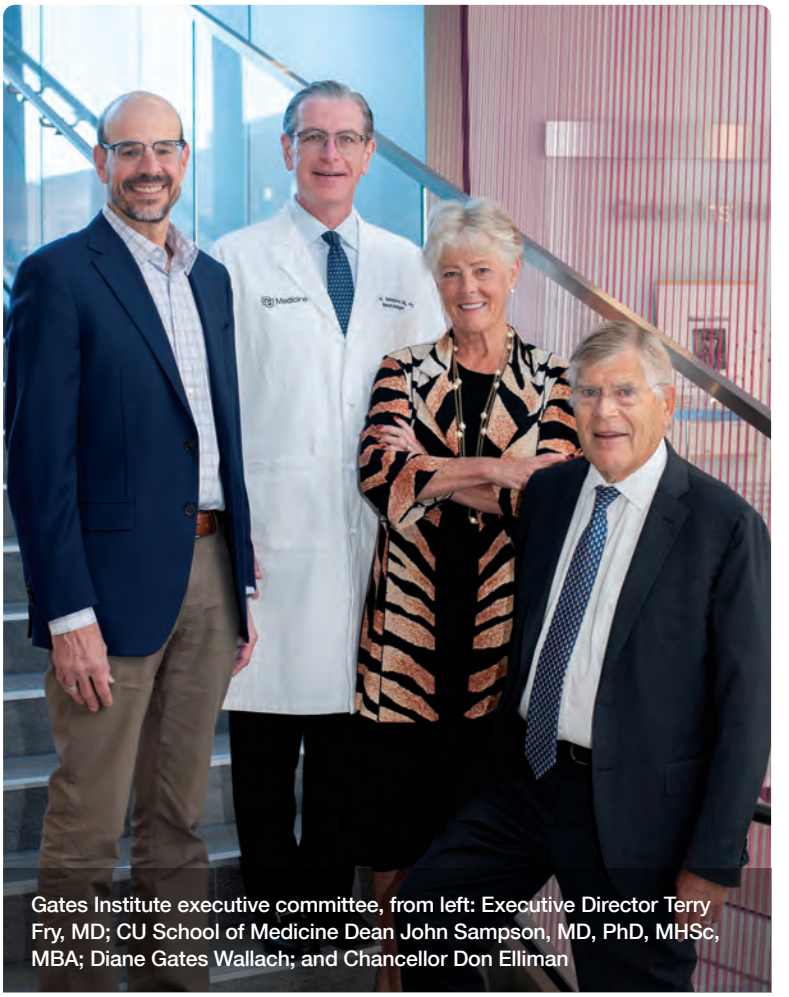
RISING TO NEW HEIGHTS



Gates Institute executive committee, from left: Executive Director Terry Fry, MD; CU School of Medicine Dean John Sampson, MD, PhD, MHSc, MBA; Diane Gates Wallach; and Chancellor Don Elliman

As 2024 was winding down, I had the opportunity to attend the annual meeting of the American Society of Hematology, an event I've been attending since I began my career. What was particularly noteworthy was the prominence given to chimeric antigen receptor (CAR) T-cell therapies at this event; 20 years ago, CARs were something of a side show. Now cell and gene therapies (CGTs) are front and center because of their transformative impact on patient care. Nowhere is this more evident than at University of Colorado Anschutz Medical Campus, where cell and gene therapies are being advanced across a broad range of areas including oncology, autoimmune disorders, ophthalmology and orthopedics.

Now in year three as an institute, we can see our efforts taking off. We are supporting many new achievements at CU Anschutz, which you can read about in the pages of this report. We are wholeheartedly embracing Dean John Sampson's initiative to propel the CU School of Medicine into the top 10 in 10 years, knowing that advancing cell and gene therapy will be key to achieving this goal.

We are marking a few important milestones in 2025, which were made possible by the groundwork already laid. In the coming year, we expect to start a new CAR T-cell trial for a therapy developed entirely through collaborative research on this campus (see page 9). We will open a new laboratory dedicated to translational research, to more effectively steer therapies from bench to bedside. Finally, this will be the 10th year for the Gates Grubstake Fund and the 10th anniversary of the Gates Biomanufacturing Facility – both of which have had impact across and beyond this campus.

Groundbreaking work doesn't happen in a vacuum. We're engaged with other entities across the CU ecosystem and that's what is lifting us higher. How we orient ourselves now will have impact for years to come.

Terry Fry, MD

Terry Fry, MD

With Dean John Sampson, MD, PhD, MHSc, MBA • Diane Gates Wallach • Chancellor Don Elliman

LEADING BIOTECH EXPERTS
FORM NEW SCIENTIFIC ADVISORY BOARD

Five individuals with biotechnology expertise have been recruited by Gates Institute to form a new scientific advisory board. They will provide strategic and scientific input to guide specific cell and gene therapy programs, with a focus on increasing patient impact, prioritizing near- and long-term goals, and exploring new platforms, said Gates Institute Executive Director Terry Fry, MD.

This board will supplement the scientific expertise of the larger Gates Institute Advisory Board, which advises Fry and other members of the executive committee on matters ranging from marketing to intellectual property.

Members of both boards will serve as ambassadors for the institute in their own professional networks, promoting the Institute's work and helping to identify opportunities for collaboration and partnership, said Fry.

The scientific advisory board members, who will serve three-year terms, may already be familiar to those in the Gates community. They represent a variety of backgrounds in academia and industry and are recognized for their expertise in cell and gene therapies, rare diseases, clinical research, regulatory, and health policy/advocacy.

The inaugural advisory board members are:

Kristi S. Anseth, PhD
Kristi Anseth is Distinguished Professor, Tisone Professor of Chemical and Biological Engineering, and Head of Academic Leadership of the BioFrontiers Institute at the University of Colorado Boulder. Her research interests lie in the interface between biology and engineering for design of new biomaterials applied in drug delivery and regenerative medicine. She is an elected member of the National Academies of Medicine, Engineering, and Science.

Laurie Goodrich, DVM, PhD
Laurie Goodrich is professor of Orthopedics in the Department of Clinical Sciences and Director of the Orthopedic Research Center at Colorado State University's C. Wayne McIlwraith Translational Medicine Institute. Her research employs new approaches to bone and joint healing in equine athletes using both gene therapy and stem cell therapy. Ongoing studies include using adeno-associated viral vectors to deliver growth factors and anti-inflammatory molecules important in cartilage and bone healing.

Jay Hesselberth, PhD
Jay Hesselberth is professor of Biochemistry and Molecular Genetics and co-director of the CU School of Medicine RNA Bioscience Initiative (RBI). His research involves RNA damage and repair, technology development focused on single-cell and spatial transcriptomic measurements, and the collaborative development and application of RNA vaccines and therapies.

Paul Rothman, MD
Paul Rothman is the former dean of Medical Faculty at Johns Hopkins University School of Medicine and CEO, Johns Hopkins Medicine. A rheumatologist and molecular immunologist, his research interest involves immune system molecules known as cytokines and the role they play in the normal development of blood cells, as well as the abnormal development of these blood cell that leads to leukemia. He is on the boards of Merck and Labcorp.


Top Row: Paul Rothman, MD, Dan Welch, MBA
Bottom Row: Kristi S. Anseth, PhD, Laurie Goodrich, DVM, PhD, Jay Hesselberth, PhD

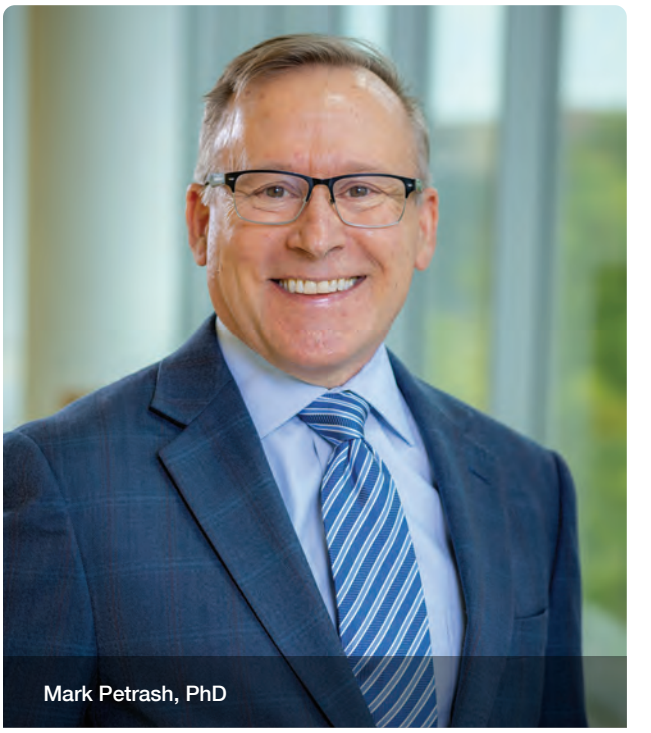
Dan Welch, MBA
Dan has over 40 years of experience developing medicines in entrepreneurial biotech and multinational pharmaceutical companies. He has had roles in bringing over a dozen breakthrough new medicines to patients with HIV, multiple sclerosis, cardiovascular disease, cancer, infectious disease and several rare diseases. The technologies behind these new medicines include adeno-associated virus vector-based gene therapies, monoclonal antibodies, mRNA and small molecules. Welch currently serves as the board chair or board director of several public and private companies that are developing medicines and gene therapies to treat various forms of cancer, pediatric and adult rare diseases and cardiovascular disease and diabetes/obesity.

LASER FOCUSED ON *CELLSIGHT*

In Advisory Role, Former Gates Center Associate Director Brings Decades of Expertise to Help Ready iPSC Research Program for Clinical Trials

By Addie Netsanet

Not long after the technology to produce induced pluripotent stem cells (iPSCs) from adult cells was developed in 2007, Mark Petrash, PhD, then-professor and vice chair of research in the Department of Ophthalmology at the University of Colorado Anschutz School of Medicine, recognized how this breakthrough would impact ophthalmology research and innovation. After joining CU Anschutz in 2008, Petrash became involved with the recently formed Gates Center for Regenerative Medicine and advocated for the goal to find a stem cell-based cure for age-related macular degeneration (AMD). The Gates Center (forerunner of Gates Institute) was also following the discoveries around iPSCs, cells that can generate all the different cell types found in the body (such as retinal cells).


Mark Petrash, PhD

"Leadership in ophthalmology saw the unique opportunity to build an ocular stem cell and regeneration program at the Anschutz campus," Petrash recalls. "We saw so much synergy with the mission of the Gates Center, so the partnership we created was an outgrowth of that commitment to bring together leading laboratory research and clinical expertise that would be needed to bring this dream closer to reality."

Naresh Mandava, MD, the Sue Anschutz-Rodgers Professor and Chair in Retinal Diseases and chair of the Department of

Ophthalmology, worked with generous donors to raise $5 million, which was matched by the Gates Frontiers Fund, to fund the *CellSight* Ocular Stem Cell and Regeneration Research Program. They knew they would need a world-renowned researcher to lead the program, so Petrash then played a prominent role in recruiting Valeria Canto-Soler, PhD, who had led her team to be the first to grow light-sensitive human retinal tissue from iPSCs in their lab at Johns Hopkins in 2014. In July 2017, Canto-Soler came to CU Anschutz as associate professor of ophthalmology, the Doni Solich Family Chair in Ocular Stem Cell Research, and the inaugural director of the *CellSight* program.

"Dr. Petrash and Dr. Mandava acted as visionaries to bring all of the key players together to make *CellSight* a reality: the Gates Institute, the Department of Ophthalmology, my research team, and the Anschutz Medical Campus overall," Canto-Soler recounts. "This partnership and the enormous support from the leadership has allowed our team to be at the forefront of this research producing innovative breakthrough technology."

The quest to bring sight-saving treatments to patients

Canto-Soler and her research team grow functioning, multi-layered, light-sensing retinas in-vitro, starting from iPSCs. Canto-Soler's team is developing a retinal implant and the surgical procedures that they hope will one day be used to correct vision loss in humans stemming from common conditions such as macular degeneration.

Although Petrash retired from his faculty position in July 2024, he will continue to support the *CellSight* program by advising on the translation of its research with Gates Institute. It comes at a pivotal time: In 2024, *CellSight* was selected for an Anschutz Acceleration Initiative award for research innovations that are poised to impact patients within the next three to five years, and will receive over $9 million in funding for two innovative products. The first is a transplant designed to reverse vision loss in patients with dry AMD with developing geographic atrophy (GA), and the second is a first-of-its-kind transplant designed to restore vision in dry AMD patients affected by late-GA. The *CellSight* team is also contributing with their expertise to the Anschutz Medical Campus-led consortium working on the development of whole eye transplants to restore vision through a $46 million initiative funded by Advanced Research Projects Agency for Health (ARPA-H).

What started as a groundbreaking idea has evolved into an innovative research and scientific development program that has the capacity to reverse vision loss in millions of patients. As Petrash looks back on his career, he is gratified to have been a part of this remarkable program."Seeing the successful launch of *CellSight* and its great progress so far, together with new collaborations with translational scientists and regulatory experts at the Gates Institute, I am confident our team has the potential to make the key research discoveries and innovations required to bring sight-saving treatments to patients with AMD," he says. "I can't wait to see products of the *CellSight* program continue to develop and make their way to human clinical trials and ultimately to patients."


Valeria Canto-Soler, PhD

"Dr. Petrash and Dr. Mandava acted as visionaries to bring all of the key players together to make CellSight a reality: the Gates Institute, the Department of Ophthalmology, my research team, and the Anschutz Medical Campus overall."

– Valeria Canto-Soler, PhD

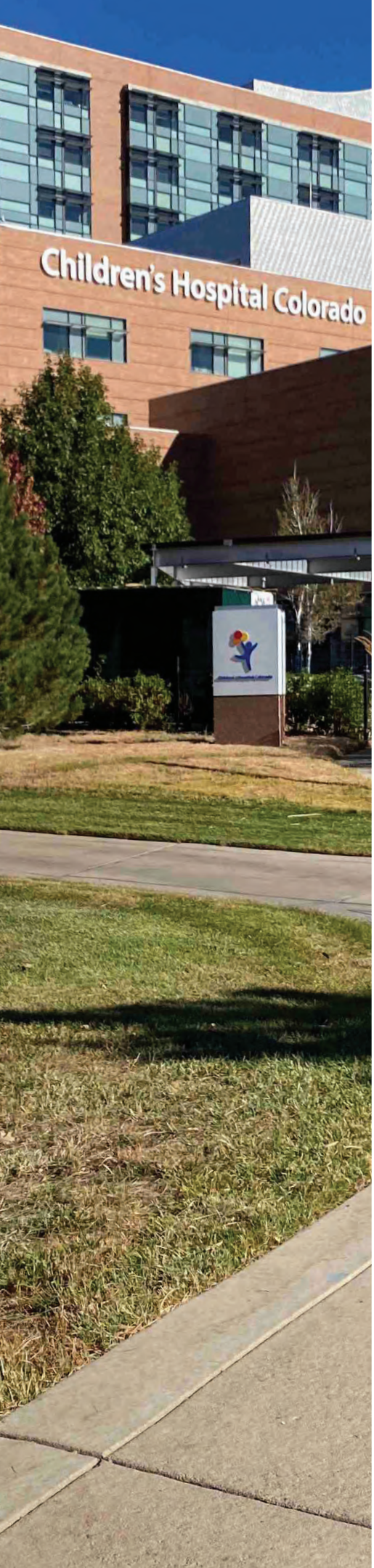
TWO CARs IN ONE

In 2024, a first-in-human study began enrolling pediatric and young adult patients with a subtype of leukemia at Children's Hospital Colorado to test an engineered white blood cell that targets two markers commonly found on leukemia cell surfaces.

By Toni Lapp

A new phase 1 study of a chimeric antigen receptor (CAR) T-cell therapy at Children's Hospital Colorado for pediatric patients with relapsed or refractory pre B-cell acute lymphoblastic leukemia (B-ALL) enrolled its first patient in late 2024. The trial is the latest launched through Gates Institute, and is the fifth for which the Gates Biomanufacturing Facility produces the CAR T-cell product for research at the University of Colorado Anschutz Medical Campus.

B-ALL is the most common malignancy seen in children, says principal investigator (PI) Vanessa Fabrizio, MD, MS, assistant professor of pediatric hematology/oncology and bone marrow transplantation in the CU Anschutz School of Medicine. While B-ALL patient outcomes are generally positive, that is not the case for the subset of patients who relapse. The first patient enrolled in the trial is a young adult who had relapsed several times after starting treatment for the disease as a teenager. (Patients from 3 months to 30 years of age are eligible for the trial.) He



received the treatment in late October and has so far tolerated the treatment with the new CAR T cell well, says Fabrizio, who is a member of Gates Institute and the CU Cancer Center.


Vanessa Fabrizio, MD, MS

Packaging two CARs in one

Since the first CAR T-cell treatment for pre B-ALL was approved by the FDA in 2017, it has shown remarkable success as a treatment in patients with multiple relapsed and refractory pre B-ALL, with over three-quarters of patients experiencing remission. However, about half of these patients eventually relapse, and this trial will help researchers explore a new variation of the novel therapy.

The CAR T cell now being tested is called CD19x22, so named because it targets two proteins, CD19 and CD22, which are frequently found on a specific leukemia cell surface. The technology was initially developed over the course of 2016-2018 by Gates Institute Executive Director Terry Fry, MD, during his tenure as an investigator with the Pediatric Oncology Group at the National Cancer Institute (NCI).

Fry worked on some of the earliest CAR-T clinical trials at the NCI

"Initially, we built a CAR that targeted the CD19 receptor, but about half of patients who had achieved remission eventually relapsed, and we found the leukemia had lost the CD19 protein," he said. "When we developed a CAR that targeted CD22, the same thing happened."

Although the Fry laboratory had previously engineered CAR T cells that target both CD19 and CD22 using a single CAR, they found it wasn't as effective at targeting both using two separate CARs introduced in the same T cell. Fry surmised it was due to the architecture of the CAR T cell, and devised a CAR T cell that placed the targets in separate places on the cell surface. This CAR T-cell is also currently being used in a clinical trial at UCHealth University of Colorado Hospital for adults with B-cell lymphomas – the first patients in the world to receive the dual-antigen CD19x22 T cells in this new format. Results from that trial were reported by PI Manali Kamdar, MD, at an American Society of Hematology meeting in December 2024.

Specialized expertise

Patients undergoing CAR T-cell therapy at CU Anschutz begin by having their blood drawn in a process called apheresis. Their T cells are isolated from other components of their blood and sent to the Gates Biomanufacturing Facility (GBF) where they are engineered to recognize cancer cells. These engineered T cells multiply over the course of several days before being injected back into the patient's body, where they continue to multiply and attack cancer cells.

The complexity of a CAR-T clinical trial requires specialty expertise. The Gates

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Institute houses a team of cell and gene therapy clinical trial experts who support the development and design of the clinical trial through scientific writing, project management, and development of regulatory strategy and communications with the FDA. The team also supports the execution of the clinical trial once open by overseeing patient safety and supporting trial operations. Coupled with the biomanufacturing by the GBF, a pillar of Gates Institute, the Gates Institute has led the charge in getting this groundbreaking trial open for enrollment.

The FDA requires the CAR T cells be created in a Good Manufacturing Practice (GMP)-compliant facility. The GBF is the only GMP facility at an academic medical campus equipped to produce CAR T cells in the Rocky Mountain region. Before the GBF had opened its doors, research like this would have required sending cells across the country for the biomanufacturing process.

Customized treatments offer new approach to cancer therapy

CAR T-cell therapy is considered a form of immunotherapy because it leverages the immune system to fight disease by targeting specific antigens on cancer cells. In contrast, chemotherapy kills both cancer cells and healthy cells indiscriminately. CAR T-cell therapy is an autologous treatment, meaning the product is manufactured from cells provided by the patient. Fabrizio plans to infuse 21 patients over a 36-month period. This phase I study will determine the optimal dose of CAR T cells. The patients receive one infusion of CAR T cells and are then followed for the next 12 months to monitor for safety and side effects. Because it's a "living drug," patients continue to be followed for 15 years per FDA requirements to monitor for safety and side effects.

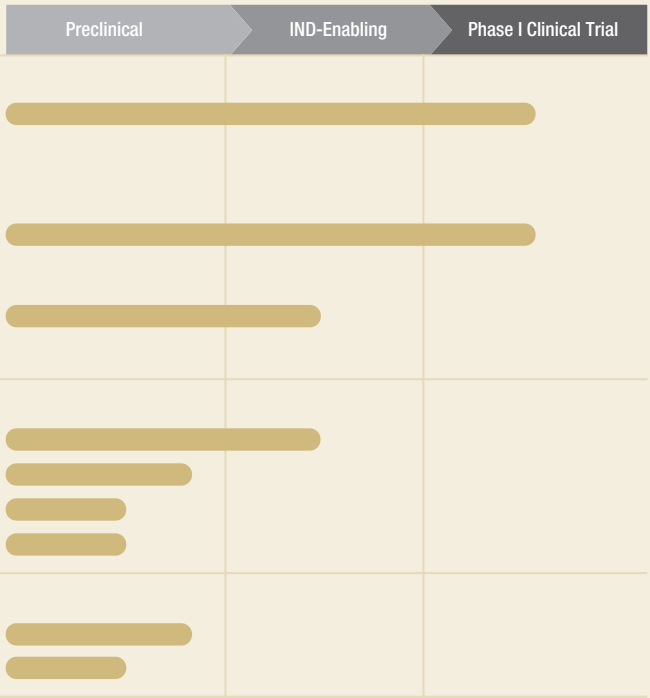
The treatment can cause severe side effects; for example, when T cells multiply in the patient, it can induce an inflammatory reaction by releasing too many cytokines causing cytokine release syndrome (CRS). This can cause high fevers, requiring oxygen support, and/or blood pressure support.

Fabrizio, who began her career when CAR therapies were just beginning to receive FDA approval, was drawn to conduct clinical trials to offer more choices to patients who have few options. It's an investigator-initiated trial with the CAR T cells being manufactured at the GBF, which presents the opportunity to put CU Anschutz on the map in the cell therapy world, she says.

"This is an opportunity to really push medicine forward," she says. "We are finding better ways to treat patients with therapies that weren't available 20 years ago."

"A lot of that starts with bench research, but clinical trials are what inform the future and how we are going to integrate these therapies into patient care."

GATES INSTITUTE CELL AND GENE THERAPY PRODUCT PIPELINE



CAR = chimeric antigen receptor; iPSC = induced pluripotent stem cell; IND = investigational new drug

EARLY-CAREER INVESTIGATOR TAKES AIM AT LEUKEMIA

Through a collaborative role with Gates Institute, Mathew Angelos, MD, PhD, is pioneering CU Anschutz's first entirely home-grown chimeric antigen receptor (CAR) T-cell trial.

By Addie Netsanet

Recruited to the CU School of Medicine as assistant professor of hematology in 2024, Mathew Angelos, MD, PhD, joins a wave of promising early-career physician-investigators on campus. Angelos is exploring the potential of CAR T-cell therapy to revolutionize the treatment of acute myeloid leukemia (AML) and other hematologic cancers. His research using CD64 as a target in CAR T-cell therapy will be the first CAR T-cell therapy created from inception at CU Anschutz.

Angelos' passion for hematology and oncology began during his post-baccalaureate research program at the National Cancer Institute, an experience that set him on the path toward a dual MD/PhD degree at the University of Minnesota. During his PhD, Angelos focused on hematopoietic stem cells. He went on to complete a residency and fellowship at the University of Pennsylvania, where the burgeoning field of cellular therapies solidified his interest in leukemia research. He found the rapid progression of leukemia cases to be both a challenge and an opportunity for innovative therapies like CAR T cells to make a significant impact.

"Unlike other blood cancers, AML presents unique challenges for CAR T-cell therapy," he said. "The biggest issue is finding a suitable leukemia target that doesn't cause toxicity to normal blood cells and other organ systems."

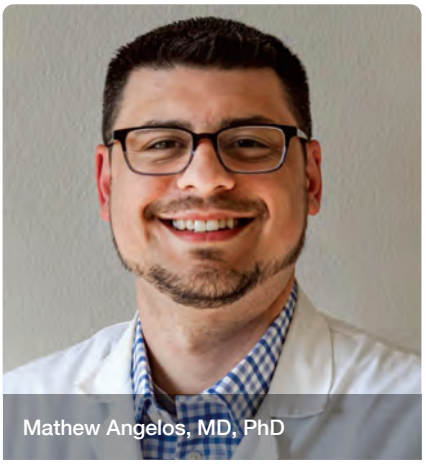
Angelos' research focuses on overcoming these challenges by developing rationally designed CAR T-cell therapies that are tailored to each patient's unique AML biology.

Teamwork and Translational Research

Angelos credits robust research at CU Anschutz for laying the groundwork for clinical trials, including that of leukemia expert Craig Jordan, PhD. Jordan and his laboratory identified CD64, a marker robustly expressed on leukemic stem cells in AML patients who had relapsed after standard treatment. This discovery led Eric Kohler, MD, PhD, and Haley Simpson, MD, PhD, to engineer and validate a highly active CAR T-cell therapy that targets CD64 in both in vitro and in vivo models.

"Drs. Kohler's and Simpson's preclinical work has really laid the foundation to bring this to CAR T-cell product to the clinic," Angelos said. "This will be a first-in-human study, and it's incredibly exciting. There isn't even a pharmaceutical drug targeting CD64 right now, so this is truly novel work. We're creating something from scratch here in Colorado that could fundamentally change how we treat myeloid diseases."

As he begins preparations for the first phase of the phase 1 clinical trial in mid-2025, Angelos reflects on the supportive environment he's found.

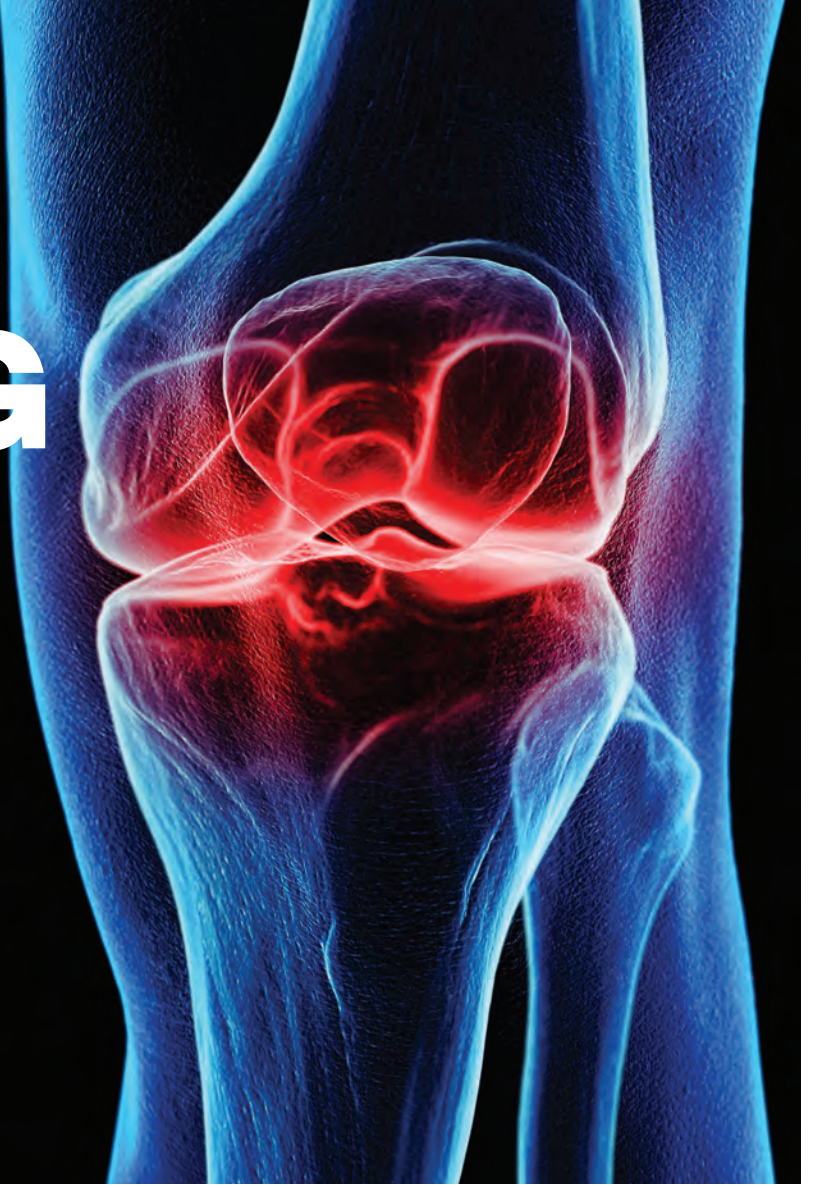


Mathew Angelos, MD, PhD

> ***"Unlike other blood cancers, AML presents unique challenges for CAR T-cell therapy."***
>
> – Mathew Angelos, MD, PhD

"Through CU Anschutz and the Gates Institute, I've found a community that's dedicated to translational science, and I'm excited to be part of these efforts," he said. "I was impressed by the comprehensive resources available at the Gates Institute to facilitate the regulatory and biomanufacturing processes needed to realize our research goals. This really is a rare find and establishes CU Anschutz as a leader among academic medical centers in the cellular therapies field."

MAJOR FUNDING FOR OSTEOARTHRITIS CURES

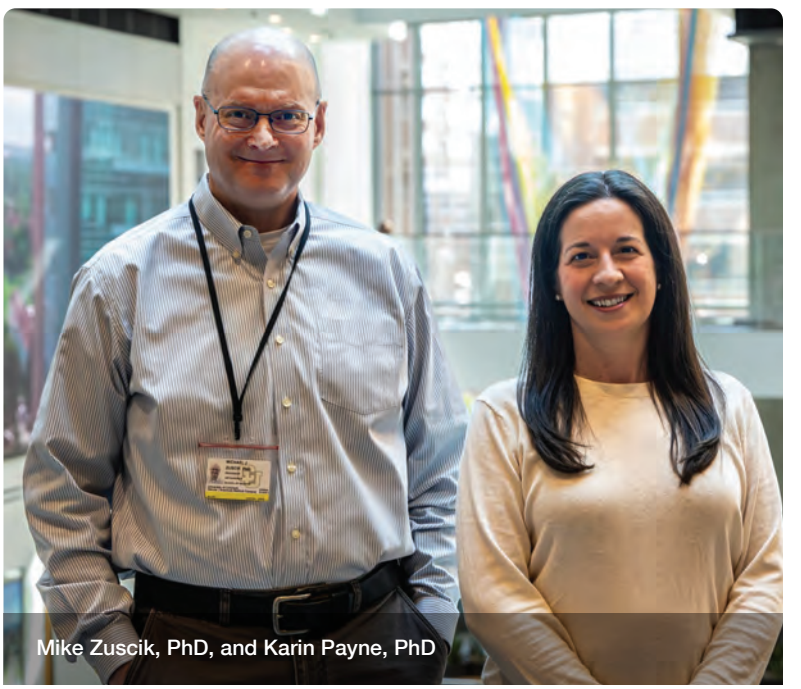


Four previous Gates Grubstake Fund awardees received an infusion of up to $39 million to develop a suite of products to reverse osteoarthritis. The award was made in March 2024 through the Advanced Research Projects Agency for Health (ARPA-H) Novel Innovations for Tissue Regeneration in Osteoarthritis (NITRO) program.

By Toni Lapp

When Gates Institute and CU Anschutz Department of Orthopedics faculty members Karin Payne, PhD, associate professor, and Mike Zuscik, PhD, professor and Orthopedics Research Vice Chair, were notified that their team's research to develop therapies to reverse osteoarthritis had been awarded major funding from the Advanced Research Projects Agency for Health (ARPA-H), they reflected on the years of collaborative work that had led them and their colleagues to this point.

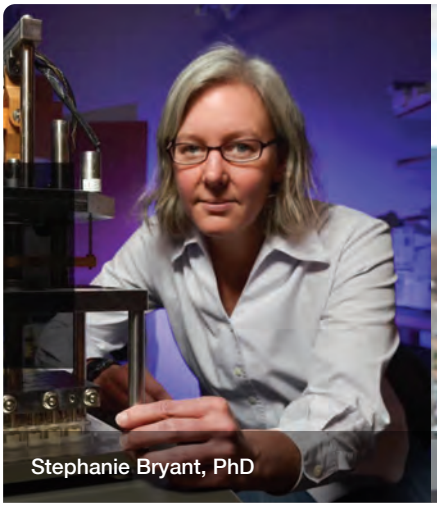
"We immediately thought of the Gates Grubstake Fund," Zuscik said. The early-stage funding was awarded in 2017 to Payne and her co-principal investigator Stephanie Bryant, PhD, professor in the Department of Chemical and Biological Engineering, Materials Science and Engineering, and the BioFrontiers Institute at CU Boulder.



Mike Zuscik, PhD, and Karin Payne, PhD

The award for their project to develop a bioresorbable 3-D printed implant for cartilage regeneration "really sparked the momentum for work on technologies that became pivotal for this new project."

Then, in 2020, Zuscik and Payne partnered on a follow-up Grubstake-funded project to further develop an injectable drug to target osteoarthritis that Zuscik has been working with for over 15 years. "The goal of both projects was to regenerate cartilage," said Payne. "The Grubstake funding helped us develop the technologies and test them in small animal models, and we can now keep building on the technologies through the ARPA-H funding."


Stephanie Bryant, PhD


Laurie Goodrich, PhD, DVM

The Gates Grubstake Fund, which was established in 2015, provides awards of up to $350,000 to investigators researching and developing regenerative medicine-related technologies; this focus is synergistic with the Novel Innovations for Tissue Regeneration in Osteoarthritis (NITRO) program, offered through ARPA-H, an agency within the Department of Health and Human Services.

These technologies will be leveraged to create a "suite of noninvasive therapies," said Bryant, project lead for the multidisciplinary team, which also includes Laurie Goodrich, PhD, DVM, professor and director of the Orthopaedic Research Center in the C. Wayne McIlwraith Translational Medicine Institute at Colorado State University.

But the support from Gates Institute, of which all four researchers are members, goes beyond the seed money received to develop cartilage-regenerating technologies.

> ***"The Grubstake funding helped us develop the technologies and test them in small animal models, and we can now keep building on the technologies through the ARPA-H funding."***
>
> – Karin Payne, PhD

"The complexity of developing these technologies to translate to humans cannot be overstated," said Zuscik. "We would need to have GMP (good manufacturing practice) grade materials so we can embark on human studies. That's no small feat when you think of the chemistries."

Matt Seefeldt, PhD, then-executive director of the Gates Biomanufacturing Facility (GBF), wrote a letter of collaboration in support of the project. He concurred that the chemistries involved in the suite of products are complicated.

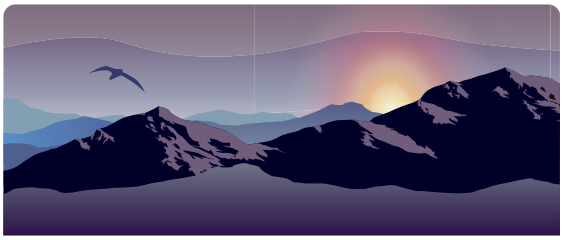
"The GBF is particularly suited to develop the processes to support this product as we have both microbial-based clean suites for protein production as well as cell therapy suites that can accommodate gene-modifying viruses with appropriate cross contamination controls," he said.

The team also conferred with Gates Institute Executive Director Terry Fry, MD, who wrote a letter of collaboration and readily offered the institute's support, beginning with process/analytical development and clinical trial protocol establishment to patient safety oversight and manufacture and release of an investigational product for the proposed studies.

Fry, a pediatric immunologist immersed in chimeric antigen receptor (CAR) T-cell therapy, brings a wealth of experience in the clinical translation of cell- and gene-based therapies into clinical trials. The institute he now leads has been conducting cell therapy trials for cancer with products manufactured at the Gates Biomanufacturing Facility since 2018.

This research was funded, in part, by the Advanced Research Projects Agency for Health (ARPA-H). The views and conclusions contained in this document are those of the authors and should not be interpreted as representing the official policies, either expressed or implied, of the U.S. Government.

ADVANCING SCIENCE THROUGH FUNDING



GATES GRUBSTAKE FUND

$1.5 MILLION
AWARDED TO CU RESEARCHERS IN 2024

Gates Institute promotes researchers dedicated to cell and gene therapy by investing in funding programs such as the Gates Grubstake Fund and Startup Toolbox.

Gates Grubstake awards have been granted annually since 2014 to advance innovative ideas with the potential for commercial application. The fund's name comes from the Gold Rush, when investors would give prospectors seed money known as "grubstakes" to buy food and supplies. At CU Anschutz, the Gates Grubstake Fund fuels innovation that may lead to new therapies or devices and helps fund what may become commercialized products or even spin-off companies. The program uses a competitive grant-making process, facilitated collaboratively by Gates Institute and CU Innovations.

In 2024, five awards were announced to principal investigators (PIs) immersed in cell and gene therapy research….

2024 GRUBSTAKE AWARDEES

Ram Nagaraj, PhD
Gene Therapy for Diabetic Retinopathy

Diabetic retinopathy (DR) is a significant complication of diabetes and accounts for half of all global blindness cases due to vascular damage marked by the breakdown of the blood-retinal barrier. However, there are no treatments that specifically target capillary cell death to prevent vison loss in these patients. Ram Nagaraj PhD, professor of Ophthalmology at CU Anschutz, has identified a gene that preserves the function of these retinal endothelial cells that can be intravitreally administered as a single dose gene therapy for protection against DR. The Grubstake Award will enable Nagaraj to optimize this therapeutic for DR and to conduct investigational new drug (IND)-enabling studies to bring this technology to clinical trials.

Mike Verneris, MD, and Traci Lyons, PhD
Sema7a Targeted CAR T-cells for Women's Cancer

Breast and ovarian cancers propose a significant global health challenge due to their high incidence, poor outcomes and limited options following relapse. CU Anschutz Professor Tracy Lyons, PhD, demonstrated that semaphorin 7a (Sema7a) is highly expressed on both breast and ovarian cancers and its expression is associated with aggressive disease, immune suppression and chemotherapy resistance. Her group demonstrated that targeting Sema7a with a neutralizing monoclonal antibody can regulate tumor cell growth. Building on this, CU Anschutz Professor Michael Verneris, MD, and his team designed a chimeric antigen receptor (CAR) construct targeting Sema7a using the DNA sequence from Lyons' group and demonstrated that anti-Sema7a CAR T cells possess superior efficacy. The Grubstake Award will fund experiments optimizing Sema7a CAR T-cell constructs as well as provide support for safety and toxicology studies enabling this team to move forward with the FDA IND application process.

Zhirui Wang, DVM, PhD
CCR4 Immunotoxin for Breast Cancer Immunotherapy via Treg Depletion

Triple negative breast cancer (TNBC) represents 15% of all breast cancer incidence and 40% of all breast cancer-related mortality. TNBC responds well to chemotherapy initially but tends to recur more frequently than other breast cancer subtypes. Recently, the use of immunotherapies inhibiting immune checkpoints have been used to treat TNBC with limited efficacy due to the infiltration of CCR4+ regulatory T cells (Tregs) causing an immunosuppressive phenotype. CU Anschutz Professor of Surgery Zhirui Wang DVM, PhD, and his team have developed a diphtheria-based immunotoxin that targets and depletes CCR4+ Tregs in the tumor microenvironment for increased efficacy of immunotherapies. The Grubstake Award will fund pre-clinical efficacy studies to prepare for National Cancer Institute Small Business Technology Transfer (STTR) Fast Track grant for IND-enabling studies.

Christian Young, PhD
First-In-Class Topical Biologic to Treat Atopic Dermatitis

Atopic dermatitis (AD), the most common eczema type, affects over 25 million in the U.S. Unlike allergic dermatitis and chronic hives, AD lacks effective long-term treatment. Newly FDA-approved systemic drugs help control moderate-to-severe cases, however, not all patients respond, resistance can develop, and severe side effects may occur. Christian Young, PhD, assistant professor in the Department of Pathology, and his team have developed Tat-PYC-Smad7, a protein therapeutic featuring a truncated human Smad7 that rapidly penetrates cells upon contact. This first-in-class topical biologic combats inflammation in AD patients while promoting epithelial healing. The Grubstake award will support pivotal nonclinical trials in representative models with atopic dermatitis, providing proof of efficacy and dose determination to mitigate risks for future human trials.

continued on next page >

2024 GRUBSTAKE AWARDEES *continued*

Yuwen Zhu, PhD
Developing a Humanized GPR182 Blocking mAb for Immunotherapy
Immunotherapy and adaptive cellular therapies (ACTs), like chimeric antigen receptor (CAR) T cells, have provided opportunities for treatment and prolonged survival in patients with malignancies that historically have limited or no options. However, only 20%-30% of patients benefit from these treatment modalities because of the immunosuppressive tumor microenvironments limiting the infiltration of CD8+ T-cells, immunotherapies and ACT. Yuwen Zhu, PhD, associate professor in the Department of Surgery, has shown that blocking GPR182 can increase intratumoral immune effector cells infiltration, thereby sensitizing immunologically cold tumors to existing immunotherapies. The Grubstake Award will fund experiments to further characterize the monoclonal antibody and perform safety and toxicity studies in preparation for IND filing and clinical trials.

ACKNOWLEDGMENTS

Gates Grubstake Fund investment decisions are made on a competitive basis by a Scientific Investment Advisory Committee. The committee comprises a diverse group of subject matter experts and institutional investors with a focus on biotechnology — all dedicated to moving the needle toward commercialization and patient treatment. We thank these individuals for their work on the committee.

- **Mark Brunvand, MD**
- **Ryan Kirkpatrick, MBA**
- **Doreen Molk, MS**
- **Mark Petrash, PhD**
- **Ann Sperling, MBA**
- **Sibylle Hauser**
- **Mani Mohindru, PhD**
- **Kimberly Muller, JD**
- **Matt Seefeldt, PhD**
- **Robert Traver, PhD, JD**

IMPORTANT DATES

AUGUST:
Applications due

SEPTEMBER:
Select investigators notified

NOVEMBER:
Presentations made to advisory committee

DECEMBER:
Awardees notified

A MICROGRANT PROGRAM LOWERING BARRIERS TO COMMERCIALIZATION

Developing a cutting-edge therapeutic or novel healthcare technology is challenging and requires a different skill set from academic research. To address this need, the Startup Toolbox was conceived to support Grubstake awardees and encourage entrepreneurship. It is jointly managed by the Gates Institute and its partners at CU Innovations to provide guidance, services and resources to help entrepreneurs advance their discoveries and improve patients' lives.

Educational Resources Available: The Startup Toolbox offers a range of educational resources to support early-stage entrepreneurs. These resources include workshops, mentorship programs and access to a network of industry experts to help you navigate the complexities of commercialization. Click or scan the QR code to read about additional Startup Toolbox resources, view a complete list of preferred service providers or seek staff guidance online at https://cuanschutz.edu/cu-innovations/startup-toolbox.

Supporting Paths to Commercialization: CU Innovations supports campus research from idea through execution, with experts available to guide you through your commercialization journey shown here: https://www.cuanschutz.edu/cu-innovations/campus-innovator/faculty-or-researcher/roadmap Contact CU Innovations: for more ways to bring your technology to fruition: https://cuanschutz.edu/cu-innovations/about-us/contact.

RECOGNITIONS

GRANTS, HONORS AND AWARDS

Gates Institute Scientific Advisory Board member **Kristi Anseth, PhD,** was awarded the VinFuture Special Prize for Women Innovators in recognition of her pioneering research in tissue engineering. Anseth, Tisone Professor in the Department of Chemical and Biological Engineering at CU Boulder and the associate faculty director of CU Boulder's BioFrontiers Institute, designs biomaterials that interact with living tissues to promote repair and regeneration, aiding in healing injuries and diseases.

The Advanced Research Projects Agency for Health (ARPA-H), awarded up to $39 million to four researchers working to develop a suite of products to reverse osteoarthritis. The award was made through the ARPA-H program, Novel Innovations for Tissue Regeneration in Osteoarthritis (NITRO) to project lead **Stephanie Bryant, PhD**, professor of chemical and biological engineering at CU Boulder; **Laurie Goodrich, DVM, PhD**, professor of orthopedics in the Department of Clinical Sciences at Colorado State University; **Karin Payne, PhD**, associate professor of orthopedics at CU Anschutz; and **Mike Zuscik, PhD**, professor and Orthopedics Research Vice Chair at CU Anschutz. See related article on page 10.

Miguel Flores-Bellver, PhD, assistant professor of ophthalmology at CU School of Medicine, received the Dr. Joe G. Hollyfield New Investigator Award for Macular Degeneration Research from the BrightFocus Foundation. The award was announced in May at the annual meeting of the Association for Research in Vision and Ophthalmology in Seattle.

Christene A. Huang, PhD, professor of surgery in the CU School of Medicine Division of Plastic and Reconstructive Surgery, serves as co-principal investigator for a CU Anschutz-led team awarded up to $46 million from the ARPA-H Transplantation of Human Eye Allografts (THEA) program. The award will support the work of the Total Human Eye-allotransplantation Innovation Advancement project team led by CU.

Malik Y. Kahook, MD, professor of ophthalmology, has been named to the Ophthalmologist Power List 2024, a ranking of individuals whose work and influence have earned them recognition as global leaders and highlights their groundbreaking contributions to medicine. The list is published annually by The Ophthalmologist.

Benjamin J. Kopecky, MD, PhD, assistant professor of medicine in the Division of Cardiology, was named to the 2024 Class of Boettcher Investigators in May. Each awardee receives $250,000 from the Webb-Waring Biomedical Research Program to support three years of scientific research. The award will fund Kopecky's work dissecting the smooth muscle cell pathobiology driving cardiac allograft vasculopathy.

Elizabeth Kovacs, PhD, professor of surgery in the CU School of Medicine Division of GI, Trauma, and Endocrine Surgery, has been awarded the 2024 Distinguished Service Award by the Shock Society. The award was presented at the society's annual conference in June.

Traci Lyons, PhD, associate professor of medicine, has received a $3.3 million grant from the National Institutes of Health to study how the molecule known as semaphorin 7A promotes resistance to therapies for estrogen receptor positive breast cancers, which represent about three-quarters of all breast cancer cases. Lyons will work with Virginia Borges, MD, CU professor of medicine, and Weston Porter, PhD, professor at the Texas A&M School of Veterinary Medicine & Biomedical Sciences.

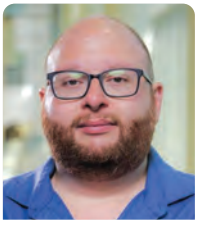
continued on next page >

RECOGNITIONS *continued*

Paul Rozance, MD, professor of pediatrics at CU School of Medicine, has been elected president-elect of the Perinatal Research Society. The society fosters scientific interchange among neonatologists, obstetricians, and basic scientists with research interests in the areas of perinatal medicine and developmental biology. Rozance's areas of interest include neonatal hypoglycemia, intrauterine growth restriction and placental nutrient supply.

Habim Sabaawy, MD, PhD, professor in the CU School of Medicine Division of Medical Oncology, was awarded $750,000 in funding from the National Cancer Institute for a project to improve models for evaluating potential cancer-fighting therapies before they go to clinical trials. The funding will support the first year of the multiyear project, which focuses on immunotherapies for non-small cell lung cancer.

Lori Sussel, PhD, professor of pediatrics and director of basic and translational research at the Barbara Davis Center of Diabetes, has been awarded Albert Renold Prize by the European Association for the Study of Diabetes (EASD). The award recognizes Sussel for outstanding contributions to better understand the function of the pancreas. The prize was awarded in September at the EASD's annual meeting in Madrid, where Sussel delivered the 18th Albert Renold Lecture.

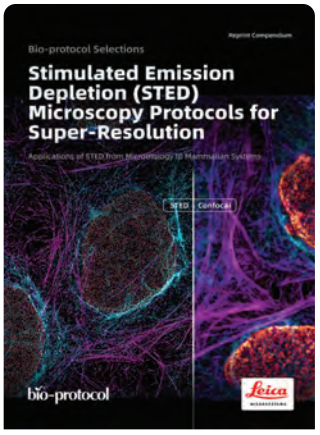


Ronald J. Vagnozzi, PhD, assistant professor of medicine, Division of Cardiology, was named a 2024 Translational Research Scholar by CU School of Medicine's Program to Advance Physician Scientists and Translational Research. The program aims to foster translational research among early career faculty who are within the first four years of their assistant professor appointment. Each scholar receives up to $300,000 over four years to support their research. Vagnozzi's funded research studies cellular mechanisms of the injury and stress response in heart failure and myocardial infarction, with emphasis on the innate immune system and fibrotic wound healing.

SELECT MEMBER PUBLICATIONS

Mathew G. Angelos, MD, PhD, assistant professor of medicine-hematology, is lead author of the article "Progress and Pitfalls of Chimeric Antigen Receptor T Cell Immunotherapy against T Cell Malignancies," in the journal Transplantation and Cellular Therapy. The article describes how CAR T-cell therapy has been groundbreaking in treating certain B cell cancers. However, applying this approach to T-cell cancers poses unique challenges. One major issue is that the markers used to identify and attack cancerous T cells are also present on healthy T cells, leading to potential self-targeting and depletion of essential immune cells.

K. Ulrich Bayer, PhD, professor of pharmacology, is corresponding author of an article in Cell Reports that explores how spatial learning and memory contribute to nighttime navigation across challenging terrains. It highlights the role of synaptic plasticity, specifically long-term potentiation, in enhancing an individual's ability to remember and adapt to spatial environments. He also photographed the issue's cover image of footprints on a glaciated Cotopaxi mountain slope. Bayer described the connection between the study and the cover photo: "Navigating crevasses at night is aided by spatial learning and memory, which requires forms of synaptic plasticity such as long-term potentiation."



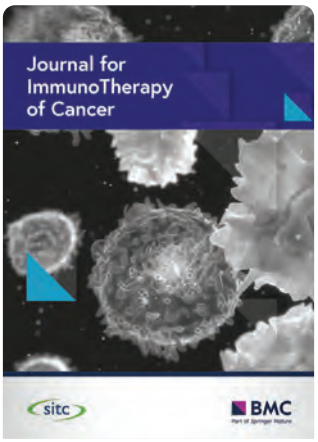
Ganna Bilousova, PhD, and **Igor Kogut, PhD**, associate professors of dermatology, and **Dennis R. Roop, PhD**, professor of dermatology, are co-authors of "The Development of an Advanced Model for Multilayer Human Skin

Reconstruction In Vivo," in the journal Bio-protocol. The article discusses a new method to create human-like skin layers on mice using small amounts of human skin cells. This approach involves first applying human skin cells called fibroblasts, followed by another type of skin cell called keratinocytes, within a special gel and silicone chamber. This technique is particularly useful when only limited patient skin cells are available, such as when studying specific skin conditions.

Peter J. Dempsey, PhD, professor of Pediatrics, and **Justin Brumbaugh, PhD**, assistant professor of Molecular, Cellular & Developmental Biology at CU Boulder, are corresponding authors of an article, "H3K36 Methylation Regulates Cell Plasticity and Regeneration in the Intestinal Epithelium," published by Nature Cell Biology. In their research of cell plasticity, they find that H3K36 methylation reinforces expression of cell-type-associated genes to maintain specialized cell identity in intestinal epithelial cells. Depleting H3K36 methylation disrupts lineage commitment and activates regenerative gene expression. Their study suggests a fundamental role for H3K36 methylation in reinforcing specialized lineages and regulating cell plasticity and regeneration. Seven colleagues from CU Anschutz are co-authors.

Miguel Flores-Bellver, PhD, and **Valeria Canto-Soler, PhD**, authored "Generation of Induced-Primary Retinal Pigment Epithelium from Human Retinal Organoids." The article, appearing in Methods in Molecular Biology, discusses a detailed method they have developed to produce retinal pigment epithelium (RPE) cells from human stem cell-derived retinal organoids. This process involves isolating and cultivating RPE tissue that closely resembles natural human RPE in structure and function. The resulting RPE monolayers are functionally mature and polarized, making them valuable for studying eye development and related diseases. This advancement offers a promising platform for exploring therapeutic approaches for retinal disorders.

Gates Institute Executive Director **Terry Fry, MD**, is corresponding author of "Antigen Experience History Directs Distinct Functional States of CD8+ CAR T Cells During the Antileukemia Response," appearing in the journal Nature Immunology. Researchers have discovered that the prior experiences of CD8+ CAR T cells with antigens influence their effectiveness against leukemia. CAR T cells that have previously encountered antigens exhibit stronger immediate responses and can eliminate leukemia cells more effectively, especially when the target antigen is scarce. However, these experienced cells tend to proliferate less, are more prone to exhaustion, and may become dysfunctional over time, particularly when fewer CAR T cells are present. This finding suggests that the history of antigen exposure in CAR T cells plays a crucial role in their performance during cancer therapy. Eight colleagues from CU Anschutz are co-authors, including **Eric Kohler, MD**.



In the July 2024 issue of Journal for ImmunoTherapy of Cancer, a new approach to enhance CAR T-cell therapy for pediatric sarcomas developed by the research team of Gates Institute translational science lead **Michael Verneris, MD**, is described. By engineering CAR T cells to express the receptor for interleukin (IL) 8, a chemical signal often elevated after treatments like radiation, these modified cells can better locate and infiltrate tumors that produce IL-8. This targeted homing significantly improved tumor reduction in preclinical models, suggesting a promising advancement for treating aggressive childhood cancers like rhabdomyosarcoma and osteosarcoma.

GATES BIOMANUFACTURING FACILITY

STRATEGIC COLLABORATION
FORGED TO MANUFACTURE LENTIVIRAL VECTOR

Lentiviral vector CDMO collaboration to support advanced cell therapies for hematological cancers.

Charles River Laboratories International Inc. and the Gates Institute announced a lentiviral vector contract development and manufacturing organization (CDMO) agreement. Gates Institute will leverage Charles River's premier cell and gene therapy CDMO expertise to develop Good Manufacturing Practice (GMP)-grade lentiviral vectors (LVVs) for use in novel chimeric antigen receptor (CAR) T-cell therapies for hematological cancers.

The Gates Institute mission at the University of Colorado Anschutz Medical Campus is to develop and deliver advanced therapies in cell and gene therapy. The Institute brings together researchers, clinicians, and a GMP biomanufacturing facility for first-in-human clinical trials.

"The cell therapy ecosystem that Gates Institute has created is exciting and we look forward to helping them advance the development of CAR-T therapies for hematological cancers," said Kerstin Dolph, Corporate Senior Vice President, Biologics Solutions, Charles River. "Our complementary strengths and concept to cure capabilities are well positioned to help move the science forward into the clinic and beyond for oncology patients."

Through this strategic collaboration, Gates Institute will have access to Charles River's established manufacturing platforms and dedicated viral vector CDMO center of excellence, utilizing a range of services including process development evaluation of Gates Institute's LVV backbone, phase-appropriate research grade and high–quality plasmid DNA production, and GMP LVV manufacturing. Materials produced within the collaboration will support an upcoming investigational new drug application for Phase I clinical trials.

Plasmid DNA and Viral Vector Manufacturing Services

With over 20 years of plasmid DNA and viral vector CDMO expertise and validated platform processes including eXpDNA™ and Lentivation™ with a proven track record, Charles River has standardized protocols and high-yield, optimized methods to accelerate speed to clinical and commercial manufacturing by reducing process development time and costs while ensuring premium quality production.

In recent years, Charles River has significantly broadened its cell and gene therapy portfolio with several acquisition integrations and expansions to simplify complex supply chains and meet the growing demand for plasmid DNA, viral vector, and cell therapy services. Combined with the company's legacy testing capabilities, Charles River offers a premier "concept to cure" advanced therapies solution.

"We value this new partnership with Charles River, an industry leader in biopharmaceutical services," said Terry Fry, MD, executive director, Gates Institute. "In the near term, this collaboration will accelerate the Gates Institute CAR-T programs with their plasmid and lentiviral vector production. Looking ahead to our long-term collaboration, we remain committed to advancing patient impact more broadly."

ALZHEIMER'S VACCINE HEADS TO CLINICAL TRIALS

Through a collaboration with the Institute for Molecular Medicine, Gates Biomanufacturing Facility's Biologics team successfully manufactured a novel protein-based vaccine.

By Addie Netsanet

Prevention of Alzheimer's disease presents one of the most confounding challenges in modern medicine. The disease is thought to be caused by a combination of factors, the two main mechanisms being aberrantly formed beta-amyloid proteins aggregating into harmful plaques and tau proteins becoming hyperphosphorylated, leading to oxidative stress and neuronal damage.

Past therapies focused on clearing beta-amyloid plaques and were met with limited success. Now, a team at the Gates Biomanufacturing Facility (GBF) in collaboration with California-based Institute for Molecular Medicine (IMM) is taking an innovative approach: a dual-target vaccine. The GBF's Biologics group developed two molecules, one targeting amyloid beta plaques and the other tau hyperphosphorylation, from tech transfer to process development to a large-scale current Good Manufacturing Practice (cGMP) manufacturing process for phase 1 human clinical trials. The two molecules are planned to go into clinical trials independently as well as combined to make a single drug product as a dual vaccine.

The GBF has been working for the past several years with the IMM to develop the vaccine. The two-pronged approach was developed after several prior clinical efforts stalled in later-stage clinical trials. Preclinical trials for the two molecules have been successfully completed, demonstrating efficacy, safety, and promising immunogenicity, the ability of a substance to prompt the body to make an immune response. The IMM is now working towards starting clinical trials in humans.

The Challenges of a Dual Approach

Developing a biological vaccine presents unique hurdles, including ensuring molecular stability, optimizing immunogenicity and ensuring quality control and safety measures. The development and manufacturing team, led by Gana Batt, PhD, with Supervisor of Biologics Operations Aashrit Donthi, and Process Engineer Caitlynn Pines, faced significant challenges in developing the vaccine's soluble, oligomeric (composed of more than one molecule) protein formulations. Since the two targeted molecules, AV-1959R (targeting beta-amyloid plaque formation) and AV-1980R (targeting tau hyperphosphorylation), preferred different conditions for stability, achieving the right formulations required extensive fine-tuning. Rigorous quality control standards were maintained throughout development and manufacturing to ensure compliance with FDA regulations.

"Quality control and process development go hand in hand for us," noted Donthi. "Development and scale up is performed with the safety assay limits in mind. We execute engineering runs at scale to make sure standards are met before clinical manufacture."

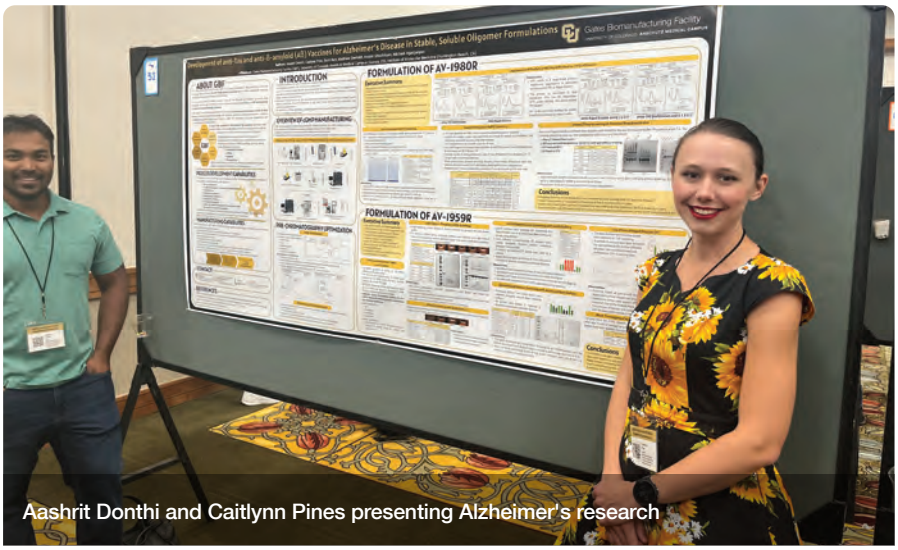
As the GBF-IMM team works to secure regulatory approval, the hope is that this innovative vaccine will not only slow disease progression but also offer preventative protection against Alzheimer's disease.

Innovation at Work

This collaborative effort highlights the cGMP expertise available at GBF, a pillar of the Gates Institute and one of the few organizations of its kind in the nation for both cell therapy and biologics manufacturing.

With the potential to revolutionize the landscape of neurodegenerative disease treatment and prevention, this novel, dual-target Alzheimer's vaccine represents a bold step forward in scientific innovation.

"We stand on the brink of a breakthrough," Batt said. "One that could redefine Alzheimer's treatment for generations to come."



Aashrit Donthi and Caitlynn Pines presenting Alzheimer's research

GSIP CLASS OF 2024

FRONT ROW FROM LEFT: Jeevan Mann, Olivia Jean Clise, Jack Moore, Holly Shankle, Edward Ning, Amelia Roselli, Tegan Wharton, Delaney Kenney, Blake Barron, Nimisha Gautam, Emma Thomure
BACK ROW FROM LEFT: Xuan An Le, Benjamin Foy, Yiran "Taylor" Han, Hannah Terry, Tvishi Yendamuri, Maydha Kumar, Julio Jaime, Oleksandra "Alex" Rachynska, Amelia Uhde, Nutsa Mdivani, Hewan Beyene

GATES SUMMER INTERNSHIP PROGRAM 2024

The 21 members of the Gates Summer Internship Program (GSIP) Class of 2024 became the first GSIP cohort under Gates Institute.

By Jill Cowperthwaite

In May 2024, 21 outstanding undergraduate students arrived on campus to participate in our unique Gates Summer Internship Program (GSIP) experience. They came from Colorado, around the country, and abroad (China, Ethiopia, Nepal, Ukraine, and Vietnam) and were joined by another exceptional student, from the country of Georgia, enrolled in Berea College's international program. These students worked in Gates Institute members' labs and formally presented their summer research to the community 11 weeks later.

While the Class of 2024 worked and attended seminars and professional development workshops, GSIP staff and alumni worked to prepare for the program's 10th Anniversary celebration on June 28. Summer interns, GSIP alumni and mentors, Gates Institute leadership, board and staff, and donors gathered to celebrate GSIP's milestone, thank those who have helped make it possible, and most importantly, highlight its impact.

The GSIP program was conceived by Gates Institute members. Tamara Terzian, PhD, Neil Box, PhD, and Enrique Torchia, PhD, with the enthusiastic support of Gates Institute Associate Director Dennis Roop, PhD, all of whom attended the 10th anniversary celebration. Critical to transforming their dream of helping train the next generation of scientific leaders into reality were Rhondda and the late Peter Grant who along with other wonderful donors and 61 mentors from a variety of medical disciplines have reveled in playing such a formative role in talented young people's lives. Highlights of this remarkable program are featured on the following pages.

206 INTERNS
have participated in GSIP since 2015, from
98 US COLLEGES AND UNIVERSITIES

20 ALUMNI
have or are pursuing degrees in the University of Colorado School of Medicine or graduate schools on the Anschutz Medical Campus

123 PROGRAM ALUMNI
are pursuing or have obtained advanced degrees (MD, PhD, MD-PhD, JD, DVM, MS, MPH)

90 RESEARCH ARTICLES AND PUBLICATIONS
have included our GSIP alums as authors

172 SCHOLARSHIPS AND AWARDS
have been received

Our GSIP team is inordinately proud of the Class of 2024, who have contributed yet another significant chapter to the program and joined the GSIP alumni network that will continue to connect and benefit our students and alumni in the years to come.

Five members of the Class of 2024 represented Colorado institutions - from left: Tvishi Yendamuri, Olivia Jean Clise, Emma Thomure, Maydha Kumar, and Julio Jaime.

Gates Institute Associate Director Dennis Roop and Diane Gates Wallach celebrate GSIP's 10th Anniversary.

Gates Institute board member, the late Wag Schorr, joined GSIP alumni attending CU School of Medicine to discuss the medical profession – left to right; Ellie Golding (GSIP 2019), Ella Annest (GSIP 2020, 2021), and Brett Li (GSIP 2022).

EDUCATION

GSIP STUDENTS AND MENTORS

Blake Barron
Davidson College
Mentor: Mi-Hyun Nam, PhD
Research Instructor, Ophthalmology

Hewan Beyene
Boston University
Mentor: Sujatha Venkataraman, PhD
Assistant Professor, Pediatrics

Olivia Clise
Regis University
Mentor:Joseph Brzezinski, PhD
Associate Professor, Ophthalmology

Benjamin Foy
Georgia Institute of Technology
Mentor: Jennifer Richer, PhD, MS
Professor, Pathology

Nimisha Gautam
Cornell University
Mentor: Ronald Vagnozzi, PhD
Assistant Professor, Cardiology

Yiran "Taylor" Han
Middlebury College
Mentors: Devatha Nair, PhD
Assistant Professor
School of Dental Medicine
Chaitanya Puranik, BDS, MS, MDent Sci, PhD
Assistant Professor
School of Dental Medicine

Julio Jaime
University of Colorado Denver
Mentor: Nidia Quillinan, PhD
Associate Professor, Anesthesiology

Delaney Kenney
Haverford College
Mentor: Ganna Bilousova, PhD
Associate Professor, Dermatology

Maydha Kumar
University of Colorado Denver
Mentor: Gana (Dash) Batt, PhD
Associate Director, Gates Biomanufacturing Facility

Xuan An Le
University of Michigan
Mentor: Maria Natalia Vergara, PhD
Assistant Professor, Ophthalmology

Jeevan Mann
University of California, Davis
Mentor: Ganna Bilousova, PhD
Associate Professor, Dermatology

Jack Moore
Vanderbilt University
Mentor: Ankush Gosain, MD, PhD
Professor, Surgery

Edward Ning
University of Illinois Chicago
Mentor: Jordan Jacobelli, PhD
Associate Professor, Immunology and Microbiology

Oleksandra "Alex" Rachynska
Dakota State University
Mentor: Miguel Flores-Bellver, PhD
Assistant Professor, Ophthalmology

Amelia Roselli
Davidson College
Mentor: Santos Franco, PhD
Assistant Professor, Pediatrics

Holly Shankle
Claremont McKenna College
Mentor: Michael Alberti, MD, PhD
Assistant Professor, Pathology

Hannah Terry
Knox College
Mentor: Eric Pietras, PhD
Associate Professor, Hematology

Emma Thomure
University of Colorado Boulder
Mentor: Jeffrey Jacot, PhD
Associate Professor, Bioengineering

Amelia Uhde
Baylor University
Mentor: Srividhya Iyer, PhD
Assistant Professor, Orthopedics

Tegan Wharton
The University of Iowa
Mentor: Michael Verneris, MD
Professor, Pediatrics, Heme/Oncology & BMT

Tvishi Yendamuri
University of Colorado Boulder
Mentor: Chelsea Magin, PhD
Assistant Professor, Bioengineering

BEREA INTERN AND MENTOR

Nutsa Mdivani
Berea College
Mentor: Xiying Fan, PhD
Assistant Professor, Dermatology

Mentors, program supporters, and Glendorn Foundation-supported GSIP interns enjoy the anniversary party - from left: Blake Barron (GSIP 2023, 2024), Frank Kugeler, Natalia Vergara, PhD, Mi-Hyun Nam, PhD, and Kanita Hrustanovic (GSIP 2023).

In the lab, Morgan Adams-supported intern Hewan Beyene with her mentor, Sujatha Venkataraman, PhD.

Tegan Wharton presents her research amongst the crowd at the 2024 GSIP Symposium.

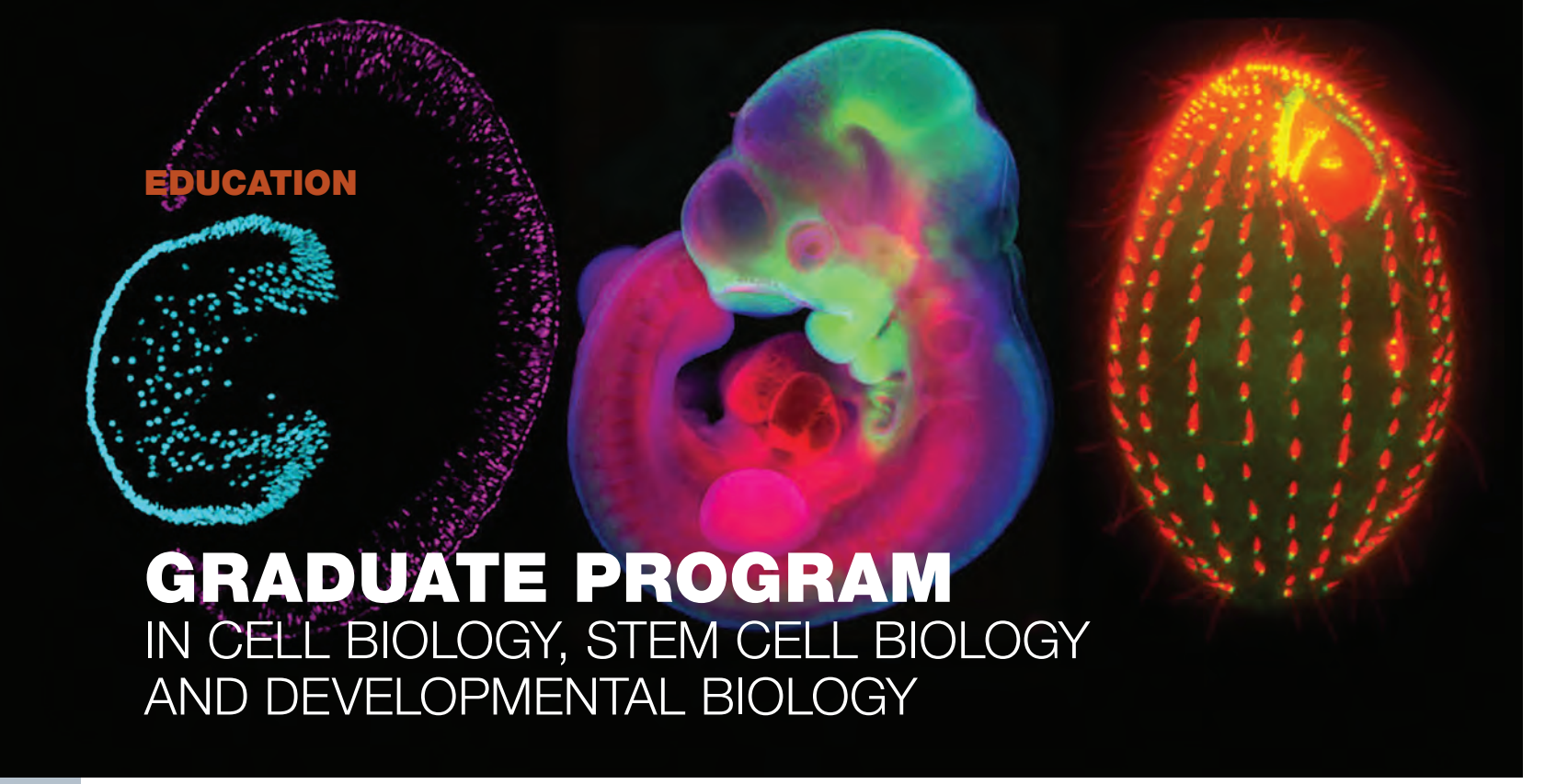
Interns taking in the spectacular view after an Estes Park climb.

Interns enjoy the Denver skyline after a fantastic afternoon at the Museum of Nature and Science.

Gates Institute Executive Director and 2024 GSIP Symposium Speaker Terry Fry, MD, enjoyed lunch and conversation with the Class of 2024.

GSIP Team left to right: Program Coordinator Jane Rech, PhD, Co-director Joseph Brzezinski, PhD, Program Assistant Debbie Lopez, and Co-director Jill Cowperthwaite.

Diane Gates Wallach and the late Gates Institute board member Rick Stoddard.



EDUCATION

GRADUATE PROGRAM
IN CELL BIOLOGY, STEM CELL BIOLOGY AND DEVELOPMENTAL BIOLOGY

The CSD Graduate Program's particular focus on understanding the origins of human disease and the creation of new therapeutic avenues makes it a natural partner with the Gates Institute.

By Jeff Moore

CSD students and program faculty at the Fall 2024 retreat in Breckenridge

The CSD Graduate Program at CU Anschutz trains the next generation of biomedical research leaders at the intersection of Cell biology, Stem cell biology and Developmental biology. Our 69 faculty represent 16 departments and divisions at University of Colorado Anschutz Medical Campus, and train 43 current PhD students. The CSD program's particular focus on understanding the origins of human disease and the creation of new therapeutic avenues makes it a natural partner with the Gates Institute. Since 2015, the Gates Institute has partnered with CSD to work toward our training goals -- increasing the number of incoming students, supporting innovative course development, hosting visiting speakers, and providing travel stipends so students can attend and present their research at scientific conferences across the globe.

In 2024, the Gates Institute supported two courses that give students unique training in cell biology, stem cells and development. The Animal Models in Developmental Biology course introduces students in their first year of study to foundational concepts in developmental biology through classic experiments using chick eggs, planaria and axolotls. The Organoids course trains advanced students on concepts and practice of using adult and pluripotent stem cell organoid culture systems to model organs and tissues, and explores bioengineering applications of these technologies. Both of these courses are special because they feature hand-on, experiential learning; using materials made possible by support from the Gates Institute.

The Gates Institute also continued to support CSD students as they traveled to scientific conferences to present their work and network with experts in their fields. In 2024, travel awards funded by the Gates Institute supported 15 CSD students for travel to conferences across the US and abroad, including Susannah Schloss, who won a presentation award at the 2024 Allied Genetics Conference for her work on the genetic pathways that underlie behavioral habituation.

GATES MEMBERSHIP BENEFITS

In our mission to advance cell and gene therapies and regenerative medicine research, we provide numerous benefits to support our diverse members as they navigate each stage of the continuum from basic science to patient care.

FUNDING OPPORTUNITIES

Research Funding

The Gates Grubstake Fund makes awards of up to $350,000 to investigators who are researching and developing regenerative medicine-related technologies including therapeutics, diagnostics, devices, manufacturing, and platforms.

Startup Toolbox

This program, jointly managed with CU Innovations, provides guidance, services and resources to help entrepreneurs develop their discoveries, lower barriers to commercialization, and improve patients' lives.

RESEARCH OPPORTUNITIES THROUGH CORE FACILITIES

Research Core Facilities provide specialized services to the Gates Institute community on a fee-for-service basis. Cores facilitate research by enabling investigators to outsource experiments that require specialized knowledge and technical abilities not found in most laboratories. Because of the nonprofit design of cores, service pricing for Gates members is significantly below that of commercial entities.

- Flow Cytometry Core
- Genomics Core
- Histology Core
- Human Immune Monitoring Shared Resource
- Organoid Core
- Stem Cell Biobank and Disease Modeling Core

Gates Biomanufacturing Facility

Gates Biomanufacturing Facility (GBF), a combined cell therapy and protein manufacturing facility, leverages its current Good Manufacturing Practice (cGMP) facility, trained personnel, qualified equipment and best practices across its service areas to work with investigators to accelerate their timeline to develop products while minimizing their overall investment.

The GBF has been a key element in the recruitment and retention of leading cell and gene therapy researchers at the CU Anschutz Medical Campus.

Translational Sciences Lab

Opening in the spring of 2025, the Translational Sciences Laboratory in the Bioscience 1 building in the Fitzsimons Innovation Community aims to bridge the gap between research and clinical application in cell and gene therapy. With its proximity to the GBF, the lab will streamline the progression of innovative therapies from the research bench to the biomanufacturing stage, expediting their journey toward clinical trials. For more information about the Translational Sciences Lab and opportunities to collaborate, please contact veena.krishnamoorthy@cuanschutz.edu.

Networking Opportunities

Gates Institute serves as a networking hub for its membership with events throughout the year to foster collaboration. On the fourth Tuesday of the month, January through November, we offer the John S. Gates Seminar Series, hourlong presentations spanning a wide range of cell and gene therapy and regenerative medicine topics. These presentations bring together a community of researchers with common interests to collaborate, learn, and advance science.

Mentorship Opportunities

The Gates Summer Internship Program (GSIP) provides paid training opportunities in Gates Institute members' laboratories at CU Anschutz. Mentors are critical to the internship experience and the success of the program.

Commercialization Opportunities

Gates Institute collaborates closely with CU Innovations to move therapeutic technologies from the laboratory into the clinic and commercialization. CU Innovations brings together industry partners, entrepreneurs, and investors to help CU researchers create biomedical technology that improves quality of life. With expertise in patents, copyrights, and licensing, CU Innovations translates discovery into impact through transparent, flexible, best practice intellectual property management services.

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CHANGES
TO OUR ADVISORY BOARD

In 2024, our Advisory Board bade farewell to John Reilly, MD, who had stepped down as dean of the University of Colorado School of Medicine. We remain grateful for his years of service on the board, and consider him to be a champion of cell and gene therapy research at CU Anschutz. The Gates organization clearly benefited from his extensive experience in creating and working in multidisciplinary clinical programs. Our staff fondly recalls that his first day as dean in April 2015 coincided with the Gates Biomanufacturing Facility's grand opening celebration, which he gamely attended!

With his departure, the Advisory Board welcomes the addition of John Sampson, MD, PhD, MHSc, MBA, who is off to a great start as dean of the CU School of Medicine. He's set the goal to propel the school into the top 10 medical schools in the country within 10 years, and we are fully behind him! He was previously professor of neurosurgery and senior vice president of Duke University Health System and the Duke Health Integrated Practice, leading faculty, community physicians, psychologists, and advanced practice providers for Duke University. He is a physician-scientist and member of the National Academy of Medicine. With a career spanning over 25 years, his journey is underscored by a relentless pursuit of excellence across clinical practice, research, education, and leadership domains. We look forward with working with him on our shared goals in the years to come.

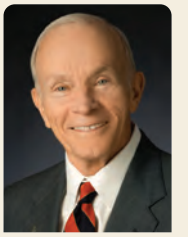

John Reilly


John Sampson

TRIBUTES

Rick Stoddard
November 23, 1950 -
November 21, 2024

Rick Stoddard joined the Gates Advisory Board in the spring of 2015. With a personal connection to Children's Hospital Colorado by way of his children's health journeys, Rick served as longtime chair of the Children's Hospital Colorado (CHC) Foundation Board of Trustees and a member of the Children's Hospital Colorado's Board. As such, he was an invaluable liaison between the Gates Center and the hospital. Similarly, Rick's remarkable legal and business acumen and service on the Gates Biomanufacturing Facility (GBF) Advisory Board was crucial to establishing and growing our GBF. His loyalty and dedication helped make the CHC and Gates Institute what they are today.



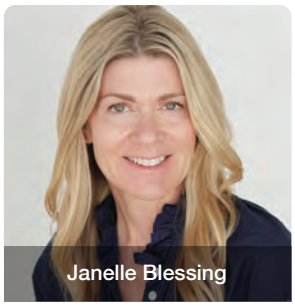
Daniel Ritchie
September 19, 1931 -
January 30, 2025

Daniel Ritchie joined the Gates Advisory Board when it first convened in 2013. As a celebrated businessman, longtime chancellor of the University of Denver, chairman of the Denver Center for the Performing Arts, and champion of numerous public service organizations, Dan served as co-chair and visionary pillar of the Gates board. He believed "we had the potential to be a leading center in the country" and that "we would see the fruits of our labor and change the world." We cherish his perennial belief in the establishment of a Rocky Mountain biomedical ecosystem, his fervent efforts to connect us well beyond the campus, and his ongoing pride in our accomplishments.

Wag Schorr
May 23, 1935 -
March 9, 2025

Wagner Schorr, MD, joined the Gates Advisory Board in 2013. A proud graduate of CU's School of Medicine, Wag trained under the legendary Thomas Starzl and pioneered work in kidney transplantation. His medical expertise and fierce determination made him both a wise counsel and successful champion of research into rare diseases like Ehlers-Danlos. A mentor to countless medical students and Gates interns, Wag's influence extended far beyond his profession. Honored with the Charlie's Angel award in 2019 for his quiet, impactful leadership, he believed passionately in the potential of cell and gene therapies as the future of medicine. His warmth, enthusiasm, generosity, and dedication to medicine and community service leave a legacy that will continue to inspire.

GATES INSTITUTE ADVISORY BOARD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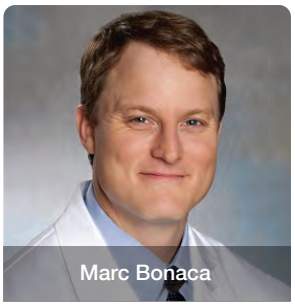
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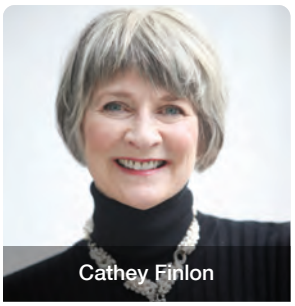
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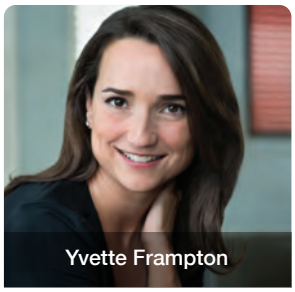
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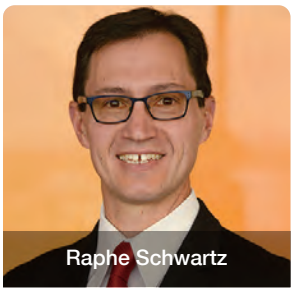
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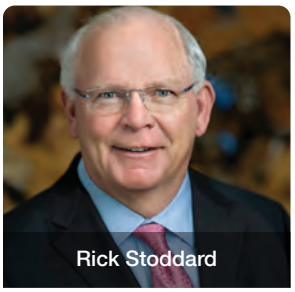
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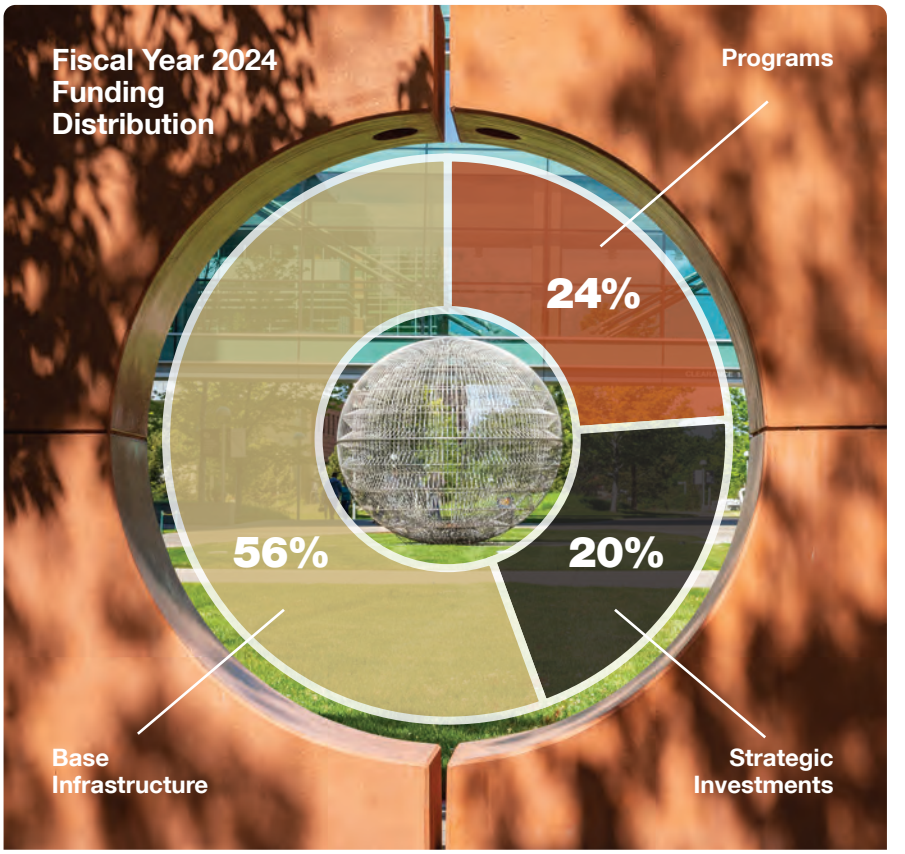
ACKNOWLEDGMENTS

FINANCIAL OVERVIEW

By Joleen Bohnen, MBA

Reaching New Heights

Since the launch of the Gates Institute in 2023, we have made significant progress in our journey to be a leader in bridging the gap between academia and industry to accelerate cell and gene therapy (CGT) development at University of Colorado Anschutz Medical Campus. We executed against our strategy to build a streamlined infrastructure, recruit top talent, expand our ecosystem of partners, and advance novel CGT programs. Today's Gates Institute is more capable than ever having established a solid foundation to support advanced therapies on campus.



Patient Impact.
Over the past five years, CU Anschutz has significantly expanded its CGT programs, increasing the number of patients treated with FDA-approved therapies and externally sponsored investigational new drug (IND) applications. Since 2020, more than 40 patients have been treated on campus with cells manufactured at the Gates Biomanufacturing Facility (GBF), meeting the increasing demand for these advanced therapies from both Colorado residents and out-of-state patients.

Our Commitment To Research.
We remain steadfast in our commitment to advancing groundbreaking research. In 2024, we proudly upheld our pledge by investing $1.5 million in early-stage research focused on advanced therapies through the Grubstake program. Additionally, we matched $10 million in promising therapies awarded through the Anschutz Accelerator Initiative.

We are deeply grateful to the Dudley Family for their generous support and partnership with the CU Cancer Center, dedicated to advancing a solid tumor cellular therapy program, for which we provided a $5 million matching contribution.

Building Infrastructure.
To accelerate the development of novel CGTs and protein-based therapies, consolidated pre-clinical research, regulatory processes, manufacturing, and clinical operations under one organization. This strategic transition includes a dedicated investment in a new Translational Science laboratory space designed to facilitate the seamless transition of research discoveries into cGMP-ready therapies for first-in-human clinical trials.

Investment in Top Talent.
Recognizing the critical role of expertise in advancing CGT therapies, the Gates Institute has invested over $2 million in recruitment packages to attract six specialized faculty members in research and clinical fields.

In addition, a $2.2 million gift in honor of the late Christine L. Honnen will create the Christine L. Honnen Endowed Chair in Regenerative Medicine and the Christine L. Honnen Fund in Regenerative Medicine, expanding the leadership of the Gates Institute to support pioneering work in cell, gene, and biologic therapies.

Strengthening Strategic Partnerships.
The Institute has collaborated with Charles River Laboratories, a premier cell and gene therapy contract development and manufacturing organization, to develop a faster and more cost-efficient process for lentiviral vector manufacturing, significantly reducing the time and cost required to bring therapies to clinical applications.

DONORS

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- Sandy Adams & Ron Sherbert
- Anonymous
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- Jean Hui Zhao

ACKNOWLEDGMENTS

CHARLIE'S PICNIC

Chancellor Don Elliman (center) confers with Huntington Potter, PhD (right)

From left: Ellie Caulkins, Diane Wallach and Laura Borgelt

From left: Joleen Bohnen, Chris Garbe, Veena Krishnamoorthy, Jordan Krause, Laura Borgelt and Jill Cowperthwaite

From left: Eileen Attar and Ganna "Anya" Bilousova

We held our 2024 Charlie's Picnic in the Leprino Family Atrium at the Denver Museum of Nature and Science.

QUIZ NIGHT AT THE MUSEUM

A Night of Trivia at the Denver Museum of Nature and Science featured "The Cells Guy," Gates Institute Executive Director Terry Fry.

The evening with quizmaster Jill Schladweiler debunked myths and explored breakthroughs in cell and gene therapy.

KICKOFF PARTY

Gates Institute staff gathered at Stanley Market to kick off the new year.

Executive Director Terry Fry, MD, delivering remarks.

Rep. Jason Crow (center) made an appearance, visiting with Terry Fry after delivering remarks.

ACKNOWLEDGMENTS

GRUBSTAKE COCKTAIL PARTY

2023 Grubstake Awardees Joshua Thurman, MD, and Sujatha Venkataraman, PhD

From left: Igor Kogut, PhD, Mike Verneris, MD, and Grubstake awardee Mayumi Fujita, MD, PhD

Gate Institute Advisory Board member Janelle Blessing and her husband Buck welcome partygoers Mike Verneris, MD, Diane Gates Wallach and others to this annual celebration.

ACKNOWLEDGMENTS

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RISING TO NEW HEIGHTS

IN CELL AND GENE THERAPY

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