EXPANDING OUR PATIENT IMPACT

2023 ANNUAL REPORT

Gates Institute
UNIVERSITY OF COLORADO
ANSCHUTZ MEDICAL CAMPUS

WHO WE ARE

Gates Institute is a world-class translational research institute with biomanufacturing capabilities. 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 cell and gene therapies.

OUR VALUES

- *"Set the bar high; clear it; and raise it further."* - Charles C. Gates
- *"No one does their best work alone."* - Charles C. Gates
- Be relentless in the pursuit of improving outcomes for patients.
- Stay curious about the ideas of others.
- Innovate, embrace change, and be nimble.

Gates Institute
UNIVERSITY OF COLORADO
ANSCHUTZ MEDICAL CAMPUS

TABLE OF CONTENTS

MESSAGES FROM LEADERSHIP 2–3

ARTICLES

- **Advancing Science Through Funding**
  - Gates Grubstake Fund 4–5
  - Startup Toolbox 6
- **Q&A with Grubstake Recipient Josh Thurman, MD** 7
- **Groundbreaking CAR T-Cell Trial Launched at CU Anschutz** 8–9
- **A 'Mighty' Therapy for Solid Tumors** 10–11
- ***CellSight*: Retinal Transplant Project at an Inflection Point** 12–13
- **The Gates Biomanufacturing Facility**
  - Technology Transfer 14–15
  - Frozen Apheresis 16–17

HIGHLIGHTS

- **Select Member Publications** 18–19
- **Honors, Awards, Recognitions** 20
- **John S. Gates Seminar Series** 21
- **New Staff** 21

EDUCATION

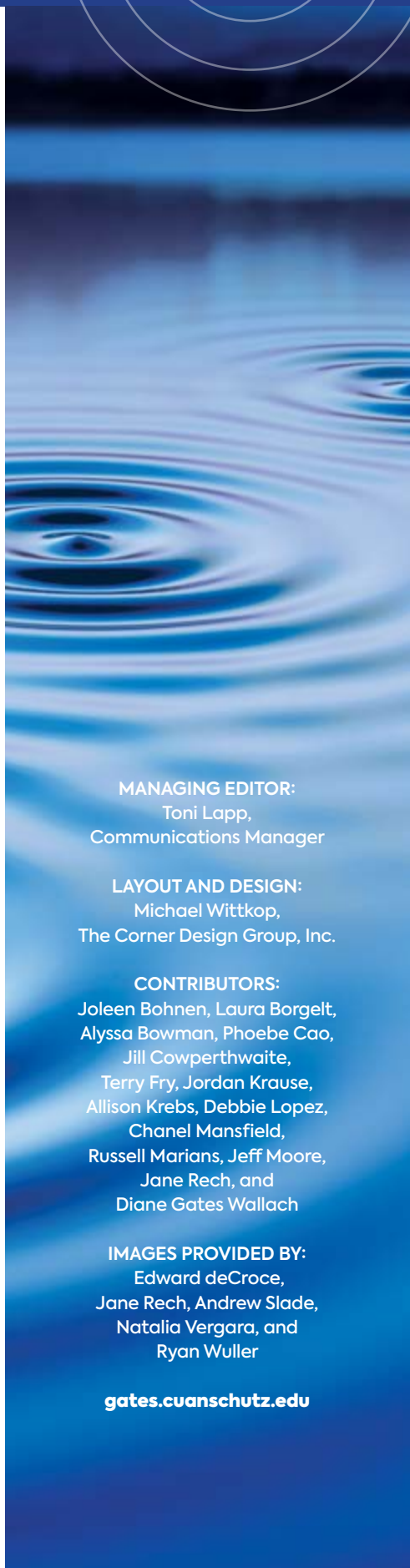
- **Gates Summer Internship Program** 22–25
- **Graduate Program in Cell Biology, Stem Cells and Development** 26

GATES INSTITUTE MEMBERSHIP BENEFITS 27

MEMBERSHIP DIRECTORY 28–31

ACKNOWLEDGEMENTS

- **Financial Overview** 32
- **Donors** 33
- **The Year in Photos: Charlie's Picnic and more!** 34–35
- **Gates Institute Advisory Board** 36
- **Our Team** 37
- **Contacts** 37



MANAGING EDITOR:
Toni Lapp,
Communications Manager

LAYOUT AND DESIGN:
Michael Wittkop,
The Corner Design Group, Inc.

CONTRIBUTORS:
Joleen Bohnen, Laura Borgelt, Alyssa Bowman, Phoebe Cao, Jill Cowperthwaite, Terry Fry, Jordan Krause, Allison Krebs, Debbie Lopez, Chanel Mansfield, Russell Marians, Jeff Moore, Jane Rech, and Diane Gates Wallach

IMAGES PROVIDED BY:
Edward deCroce, Jane Rech, Andrew Slade, Natalia Vergara, and Ryan Wuller

gates.cuanschutz.edu

A NEW FRONTIER AWAITS

From left: Chancellor Don Elliman, Diane Gates Wallach, and Dean John Reilly

A plaque posted by the door states: "The Gates Institute represents a shared commitment to achieve the vast potential of regenerative, cell and gene therapies. This historic philanthropic partnership between CU Anschutz, the Gates Frontiers Fund and many others is accelerating the development of next-generation treatments and the delivery of hope and cures to patients and their families." Chancellor Elliman's words inscribed on the panel capture our mission, and the collision of opportunity, Western spirit and serious talent are what is making it happen.

There have been many references to new frontiers and entrepreneurial spirit since the family of Charlie Gates decided to take a chance on a new frontier in medicine in 2007 – all with the hope of cures and therapies derived from our own bodies using a deeper understanding of cells and genes, and how they can be harnessed to heal. These types of therapies are still young in their development and exciting beyond belief.

Opportunity knocks. The expanded partnership between CU Anschutz and the Gates Frontiers Fund anchors a rapidly growing enterprise, and many others are joining the challenge with generous gifts and collaboration. There is an urgency as the Institute tackles the hard work from basic research through phase I (first in human) clinical trials. We thrive on the pressure while driving towards the highest standards and aspirational goals.

Charlie Gates often said "Riding for the brand" was a positive way of embracing and living what you care about, how you do business, and what you are proud to be associated with. The Gates Institute is building its brand every day, which is an activist thought – deliberate and exciting. We want to do things a bit differently with an entrepreneurial flair. We want to crack the code and deliver results.

Our engine is our people. Talent galore. We aren't everything to everyone; we focus; we go deep; and we take the long view. We are in it for the long haul, and as everyone in this space knows, it can be daunting with ups and downs, but it keeps us on our toes.

Thank you for joining us on this journey and as we share highlights in the coming pages. Hop on and enjoy the ride.

Chancellor Don Elliman
Diane Gates Wallach
Dean John Reilly

LETTER FROM OUR EXECUTIVE DIRECTOR

BUILDING THE GATES BRAND

It's been a pivotal year since I wrote to you from the pages of our inaugural annual report. As we begin our second year, it's helpful to look back at what we have achieved so far.

We're now a team of more than 75. We added several talented individuals, including Navin Pinto, MD, who joined as medical lead (page 21). We also recruited Veena Krishnamoorthy, PhD, an accomplished scientist with extensive cell and gene therapy expertise. Having key roles filled by highly skilled people has enabled us to support researchers at CU Anschutz. Our staff served on two projects of recipients of the Anschutz Accelerator Initiative, which you'll read about on pages 10 and 12.

We launched a new CAR T-cell trial (page 8), our first since the formation of Gates Institute. Our Scientific Investment Advisory Committee named four new Grubstake winners. All of the projects were focused on novel CAR T cells with high potential to enter the clinic over the next five years.

We're still getting our sea legs, so to speak, in a nascent field that has had a year of triumphs, but also trials and tribulations. This was exemplified in December at the annual meeting of the American Society of Hematology, a premier conference dedicated to CAR research. The event was abuzz with news that five-year data were being released for the earliest CAR study participants, showing 30–40% remained in remission. There was also tremendous enthusiasm for the transformative potential of stem cell gene therapy for sickle cell anemia and thalassemia. At the same time, the FDA reported on secondary cancers in recipients of CAR T-cells, emphasizing the work that needs to be done.

Developing novel cell and gene therapies is hard work, but we didn't choose this field because it was easy. To the people who enroll in trials, these treatments are often their last option – a moonshot to treat a potentially fatal diagnosis. Unpredictability and uncertainty are inherent in these types of clinical trials. Thus, we embark on this journey with humility and recognition of the bravery of the patients who enter these trials.

It is with this grounding that we build the Institute with this vision of Charlie Gates. It's in our core values to innovate, embrace change and be nimble, all to benefit patients. That is our brand. I'm excited about what we have accomplished and about our future – to launch additional therapeutic T-cell trials for refractory cancer and autoimmune diseases and to expand our patient impact into new areas such as skin and retinal regeneration.

When you have an ambitious objective – something never done before – there is no blueprint. Making progress is important, and so is the learning that comes from it.

Gratefully,

Terry Fry MD

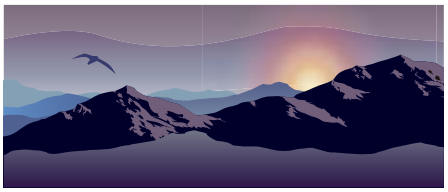
Terry Fry, MD
Executive Director, Gates Institute
Charles C. Gates Endowed Chair in Regenerative Medicine

Would you like updates from Gates Institute? Scan this QR code to sign up for our newsletter!

ADVANCING SCIENCE THROUGH FUNDING

$1.5 MILLION AWARDED TO CU RESEARCHERS IN 2023

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 FUND

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 2023, four awards were announced to four principal investigators (PIs) immersed in chimeric antigen receptor (CAR) T-cell research, each PI representing a different department in the University of Colorado Anschutz School of Medicine. In addition, a second-tranche award was made to a project originally funded in 2015.

2023 GRUBSTAKE AWARDEES

Mayumi Fujita, MD, PhD

Professor of Dermatology: Modified CD4+ T Cells Expressing IL-37

There are no curative treatments for autoimmune diseases and although much progress has been made to manage symptoms clinically, many patients still fail to respond. One promising and potentially curative therapeutic strategy is infusing patients with regulatory T cells (Tregs) to reduce immunogenicity. However, Treg therapies have had poor efficacy due to a lack of persistence, loss of function, and limited expansion capacity. CU Anschutz Professor Mayumi Fujita, MD, PhD, and her team have identified a gene that when expressed in conventional CD4 T cells, can polarize them to become Tregs. Furthermore, these Tregs retain their immunosuppressive function, have improved expansion, and persist in various in-vivo disease models. With the Grubstake Award, Fujita will conduct investigational new drug (IND)-enabling studies to bring the technology closer to clinical trials.

Jordan Jacobelli, PhD

Associate Professor of Immunology and Microbiology: Engineered CAR T Cells for Improved Solid Tumor Rejection

One of the limiting factors for effective CAR T-cell therapy against solid tumors is the lack of T-cell infiltration into the solid tumor. Jordan Jacobelli, PhD, associate professor at CU Anschutz, has identified a novel gene that when overexpressed in T cells, improves T-cell infiltration into the tumor by up to five times. The Grubstake Award will enable Jacobelli to move his technology through the proof-of-concept phase.

Joshua Thurman, MD

Professor of Medicine: C3d Targeted CAR T Cells for the Treatment of Solid Tumors

The treatment of solid tumors using CAR T cells has been limited by poor efficacy, which is in part due to the lack of suitable cancer-associated antigens. One mechanism that cancer cells use to evade CAR T-cells is downregulating the target antigen. CU Anschutz Professor Joshua Thurman, MD, and his team have found that the immune system tags tumor cells with C3d via the complement system, and this process does not depend on a single tumor antigen. As such, the Thurman lab is developing a C3d targeting CAR T cell to treat a variety of solid tumors. The Grubstake Award will allow the team to perform their initial proof-of-concept studies.

Sujatha Venkataraman, PhD

Associate Research Professor of Pediatrics: Next-Generation CAR T-Cell Therapy for Diffuse Intrinsic Pontine Glioma

For the last five decades, there has been a lack of significant advancement in the development of a treatment for diffuse intrinsic pontine glioma (DIPG), an aggressive brain tumor in children with a median survival of only 11 months. Sujatha Venkataraman, PhD, associate professor at CU Anschutz, has identified an antigen prominently expressed in DIPG tumors and engineered a novel CAR T-cell product that has effectively eradicated the tumor in diverse DIPG mouse models, quadrupling their life expectancy. Through the Grubstake Award, the Venkataraman lab will perform the necessary preclinical IND-enabling studies to move this technology to clinical trials, and ultimately, provide hope to individuals diagnosed with DIPG.

continued on next page >

SECOND-TRANCHE AWARD

Ganna Bilousova, PhD, and **Igor Kogut, PhD**
Associate Professors of Dermatology: Induced Pluripotent Stem Cell Services as a Platform for Clinical Research

No cure exists for recessive dystrophic epidermolysis bullosa (RDEB), a genetic disease that causes severe skin blistering and scarring. CU Anschutz Associate Professors Ganna Bilousova, PhD, and Igor Kogut, PhD, are developing a stem cell-based therapy for RDEB using autologous induced pluripotent stem cells (iPSCs) derived from skin cells. One obstacle to the strategy is the inefficient reprogramming of adult somatic cells into iPSCs. To overcome this obstacle, the team has applied their patented RNA-based method to the skin cells and successfully improved iPSC reprogramming efficiency by close to 20-fold. The team intends to leverage their technology's potential to improve reprogramming efficiency to treat a variety of diseases. As such, the second-tranche funding, which extends awards made in 2015 and 2022, will enable Bilousova and Kogut to perform the necessary proof-of-concept.

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**
- **Mark Lupa, PhD**
- **Kimberly Muller, JD**
- **Matt Seefeldt, PhD**
- **Ann Sperling, MBA**
- **Sibylle Hauser**
- **David L. Lacey, MD**
- **Mani Mohindru, PhD**
- **Mark Petrash, PhD**
- **Geoff "Duffy" Solich**
- **Robert Traver, PhD, JD**

IMPORTANT DATES

AUGUST:
Applications due

SEPTEMBER:
Select investigators notified

NOVEMBER:
Presentations made to advisory committee

DECEMBER:
Awardees notified

STARTUP TOOLBOX

A microgrant program lowering barriers to commercialization

Developing a cutting-edge therapeutic or novel healthcare technology is challenging and requires a different skillset from academic research. To address this need, the Startup Toolbox was conceived to support Grubstake awardees and encourage entrepreneurship. The Startup Toolbox is jointly managed by Gates Institute and its partners at CU Innovations to provide guidance, services and resources to help entrepreneurs develop their discoveries and improve patients' lives. Scan the QR code to view a complete list of preferred services providers or seek staff guidance online at **cuanschutz.edu/cuinnovations/startup-toolbox**

EXCITING NEWS
JUNE 10-12

Join Startup Toolbox for a 3-Day Innovation Forum at CU Anschutz, hosted in collaboration with CU Innovations and Academia Industry Alliance

Discover the latest in innovation, network with industry professionals, and explore opportunities to win additional funding for your start-up. Don't miss out on insightful sessions covering technology transfer, IP protection, team building, and more. Plus, witness the success stories of Anschutz start-ups and connect with peers on campus. Mark your calendars and stay tuned for updates on CU Innovations website and social media channels! #CUInnovations #InnovationForum
Details to follow online: cuanschutz.edu/cu-innovations, twitter.com/CUInnovations, linkedin.com/company/cu-innovations

Q&A WITH GRUBSTAKE RECIPIENT JOSHUA THURMAN

Chimeric antigen receptor (CAR) T-cell therapy has shown great promise in the treatment of blood cancers, but results in solid tumors have not been as impressive. This is due, in part, to the lack of suitable cancer-associated antigens. Joshua Thurman, MD, a professor in the division of renal diseases in the CU Anschutz School of Medicine, and his team have found that the immune system tags tumor cells with a novel molecule, which could lead the way to creating a CAR to target solid tumors. He was recently awarded Gates Grubstake funding to continue this research.

How did your career path lead you to CAR research?

As a nephrologist, my research has focused on inflammatory kidney diseases for more than 20 years. While researching causes of kidney disease, recent findings have piqued my interest in cancer biology. Several years ago, a publication reported that the same inflammatory pathways that can damage the kidney also promote cancer growth. This was unexpected, but subsequent research by my team and others confirmed it. We realized that many of the tools that we were already using to study kidney disease could also be applied to the study of cancer.

What led you to study the complement system?

The complement system, which is a cascade of plasma proteins, is involved in many different kidney diseases, and most nephrologists measure complement proteins as part of their routine analysis of patients with autoimmune disease. Kidney biopsies are also routinely stained for these proteins, so the role of the complement system in disease is well established. Nevertheless, the past 15 or 20 years have seen major advances in our understanding of complement in disease, and several new drugs that target the complement cascade have entered the clinic. These drugs have many beneficial effects that are not achieved with standard immunosuppressive medications. That's why I've been very excited about the role of complement inhibitors in nephrology. As we learn more about tumor growth, it has become clear that the drugs to manipulate complement activation may also provide a novel means of slowing cancer growth.

Your Grubstake project was "adopted" by a group of philanthropists (see sidebar below). What has philanthropy meant for your research?

Organizations like the National Institutes of Health provide incredible resources for biomedical researchers. But it is a truism that you already need to have some results in hand to get funding. This is where philanthropy and awards like Grubstake are invaluable. It gives a researcher the opportunity to test out a high-risk/high-reward concept. It really is the first, most important step on the journey.

Note: This is excerpted from an article appearing at news.cuanschutz.edu/gates.

Three Families, Connected by Philanthropy

In 2021, the Gates Grubstake Fund expanded from three to four annual awards of up to $350,000 for researchers in the regenerative space. The fund was able to do so thanks to the generosity of three families who came together to create the **ABN Ohana Grubstake Award**. The acronym ABN represents the Atherton, Brooks and Newell families, and ohana is the Hawaiian word for family, a nod to the families' roots on the islands. In 2023, the ABN Ohana group funded Dr. Thurman's project.

GROUNDBREAKING CAR T-CELL TRIAL LAUNCHED AT CU ANSCHUTZ

In 2023, the first trial opened through the newly formed Gates Institute began enrolling patients

By Toni Lapp

In 2023, enrollment began for a new phase 1 study of chimeric antigen receptor (CAR) T-cell therapy at UCHealth University of Colorado Hospital for adults with B-cell acute lymphoblastic leukemia (B-ALL). The trial will be the fourth for which the Gates Biomanufacturing Facility produces the CAR T product for research at CU Anschutz Medical Campus (see related table).

Currently, CAR T-cell therapy is approved by the Food and Drug Administration (FDA) for patients with B-ALL who have relapsed after receiving other treatments such as chemotherapy or a stem cell transplant. But that's beginning to change, and this trial will put CU Anschutz on the forefront of new research.

Marc Schwartz, MD
Assistant professor of medicine-hematology at the University of Colorado School of Medicine

"With this study, we're looking at patients who are in their first remission, but they're also at high risk for relapse," said principal investigator Marc Schwartz, MD, assistant professor of medicine-hematology at the University of Colorado School of Medicine.

The trial participants will be in remission but have evidence of "minimal residual disease," says Schwartz, meaning leukemia cells are still present in bone marrow but below the threshold of what can be detected by a microscope.

GATES INSTITUTE PROVIDES EXPERTISE FOR RESEARCH

Patients undergoing CAR T-cell therapy at CU Anschutz begin by having their blood drawn in a process called apheresis. Their T cells, a subtype of white blood cells, are isolated from other components of their blood at the Gates Biomanufacturing Facility (GBF) and engineered to recognize cancer cells. These newly engineered T cells multiply over the course of several days before being injected back in the patient's body, where they continue to multiply and attack cancer cells. This is why it's referred to as a "living drug," said Michael Verneris, MD, translational sciences lead at Gates Institute. "It's an entirely new approach to cancer therapy."

The complexity of a cell therapy clinical trial requires specialty expertise. The Gates 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 (pharmacovigilance) and supporting trial operations. Coupled with the biomanufacturing by the GBF, which operates as a business unit under Gates Institute, the Gates Institute has led the charge in getting this groundbreaking trial open for enrollment.

CUSTOMIZED TREATMENTS OFFER NEW APPROACH TO CANCER THERAPY

The trial being led by Schwartz will enroll 10 patients over a 24-month period. The patients receive just one infusion, and are then followed for the next 24 months to monitor for safety and side effects, and to determine optimal dosage of CAR-T cells.

The treatment can cause severe side effects; when the T cells multiply in the patient, it can induce an inflammatory reaction known as cytokine release syndrome (CRS). "These effects are more common in patients with larger burden of disease," said Schwartz. "By design, we're treating patients with a lower disease burden, so we expect to see less CRS in this study group."

CAR T-cell therapy could significantly shorten the total duration and intensity of therapy needed for cure and thereby lead to better quality of life, Schwartz added. "I knew pretty early on in my medical training that I wanted to be a leukemia physician," Schwartz said. "I knew I'd be helping patients with a disease that has been historically very difficult to treat. But in just the last five years, we have many new treatment options. We hope to continue making progress by offering patients an innovative therapy that has the potential to cure this devastating disease while still preserving quality of life."

THE PATIENT IMPACT OF CLINICAL TRIALS

	HOSPITAL PARTNER	INDICATION	STATUS	NUMBER ENROLLED	NUMBER TREATED
UCD19					
Phase I Study of Feasibility and Safety of UCD19 CAR T Cells in Adult Subjects with Relapsed/Refractory (R/R) B-Cell Non-Hodgkin's Lymphoma (B-NHL)	UCHealth	B-NHL	Closed to accrual, publication pending	10	10
Phase I/II Dose Escalation and Preliminary Efficacy of CD19 Directed CAR-T Cells Generated Using the Miltenyi CliniMACs Prodigy System (UCD19 CAR-T) in Pediatric Patients with Relapsed and/or Refractory B-Cell Acute Lymphoblastic Leukemia (B-ALL) and B-Cell Non-Hodgkin Lymphoma (B-NHL)	Children's Hospital Colorado	B-ALL, B-NHL Expanding to include ALL patients in first relapse Q1 2024	Open	8	8
Phase I Safety and Tolerability Trial of CD19 Directed CAR T Cells in Adult Patients with B-Cell Acute Lymphoblastic Leukemia (B-ALL) with Minimal Residual Disease (MRD) Positivity at First Complete Remission	UCHealth	B-ALL	Open	6	4 treated 2 awaiting treatment
CD19x22					
Phase 1/1b Study of Bispecific CD19 and CD22 Chimeric Antigen Receptor Co-Expressing T Cells (CD19x22 CAR T) in Adolescent and Adult Patients with Relapsed and/or Refractory B-Non-Hodgkin's Lymphoma (B-NHL)	UCHealth	B-NHL Expanded to include Mantle Cell Lymphoma sub-type Q4 2023	Open	7	7
Phase I Dose Escalation and Preliminary Efficacy Study of Bispecific CD19 and CD22 Chimeric Antigen Receptor Co-Expressing T Cells (CD19x22 CAR T) in Pediatric Patients with Relapsed and/or Refractory B-Cell Acute Lymphoblastic Leukemia (B-ALL)	Children's Hospital Colorado	B-ALL	Expected opening Q2 2024	–	–

NOTE: FIGURES CURRENT AS OF MARCH 22, 2024. CAR–CHIMERIC ANTIGEN RECEPTOR.

A 'MIGHTY' THERAPY FOR SOLID TUMORS

For decades, Gates Institute member Eduardo Davila, PhD, has been studying tumor-infiltrating lymphocytes, a type of white blood cell found in tumors. A team of researchers, clinicians and biomanufacturing experts have joined Davila's effort to better understand these cells and use them effectively to fight cancer.

By Toni Lapp

Like many other forms of immunotherapy, tumor-infiltrating lymphocyte (TIL) therapy uses a person's own cells to treat their condition. TILs are a type of immune cell that moves from the blood into a tumor and can recognize and kill cancer cells.

At least that's what they're supposed to do, says Eduardo Davila, PhD, a professor of oncology in the University of Colorado School of Medicine. Davila began studying TILs early in his research career. "Often, for a number of reasons, they're exhausted, they're tired," he says. "As we age, or as the T cell is prematurely aged in a tumor environment, it loses all those functions."

In 2020 and again in 2022, Davila received Gates Grubstake funding to research and manufacture genetically engineered TILs. The technology targets a gene called MyD88, which plays a role in programming immune cells and is designed to "rejuvenate" these cancer-fighting cells. "They'll live longer, kill better, run faster," he says. In other words, they'll be mighty.

The project recently received additional momentum – major funding from the Anschutz Acceleration Initiative. Leadership within Gates – Terry Fry, MD, Michael Verneris, MD, and Matt Seefeldt, PhD – consulted on the AAI application. Davila's growing team is now setting their sights on filing an investigational new drug (IND) in 2025 with clinical trials ensuing upon authorization by the U.S. Food and Drug Administration.

"We're just incredibly grateful for the support," says Davila. "Being able to conduct these types of studies helps us pioneer the next generation of immunotherapy, specifically for cancer patients that have in all reality exhausted all therapeutic options. They come to us as a last resort."

SIMILARITIES TO CAR T-CELL THERAPY

TILs have been studied in clinical trials since the 1990s, but TIL technology has lagged the progress seen with chimeric antigen receptor (CAR) T cells, which first received Food and Drug Administration approval in 2017. In early 2024, the TIL Amtagvi became the first FDA-approved tumor-derived T-cell immunotherapy and will be used for inoperable or metastatic melanoma. Davila expects there will be many more TILs approved in the coming years.

While there are similarities between CAR T-cell and TIL therapies, there are important differences, Davila notes. CAR T cells are engineered to recognize one or two antigens on the surface of a compromised cell, hence labels like CD19 or CD19x22. CAR T-cell therapy has shown more success in blood cancers than in solid tumors; the reverse is true for TIL therapy.

TILs can recognize a broad array and combination of antigens on the cancer cell surface; thus they can more effectively target rogue cells. TILs have shown success in solid tumors; Davila's MyD88 TIL therapy will be used in clinical trials for three indications: head and neck tumors, lung cancer, and sarcoma.

Another difference: CAR T cells can be manufactured from a patient's source cells in about two weeks; it takes about four weeks to bioengineer TILs therapy from a surgically extracted tumor.

A TEAM EFFORT

While creating a therapy from a solid tumor sounds simple, it is anything but. There are many steps in the process – from surgery to remove the tumor, to extracting and growing cells, to delivery of the product and infusion – where failure can occur. "The treatment is like one shot on goal," says Davila.

Collaborations have been key to moving the project forward, says Davila. The philanthropy of the Gates Grubstake Fund and the Anschutz Acceleration Initiative have been key to meeting milestones, but Davila notes that the partnerships forged across the University of Colorado Anschutz Medical Campus have also been paramount. Thanks to this support, the possibility of treating patients is coming to fruition.

"This is truly a team effort," he says. "Administering the therapy and caring for patients [in a clinical trial] requires an army of individuals, from the oncologist to the nursing staff to physician assistants. It will require round-the-clock monitoring for the first few weeks of therapy."

Oncologist Breelyn Wilky, MD, will serve as medical director and clinical lead on the trial, bringing a depth of clinical experience.

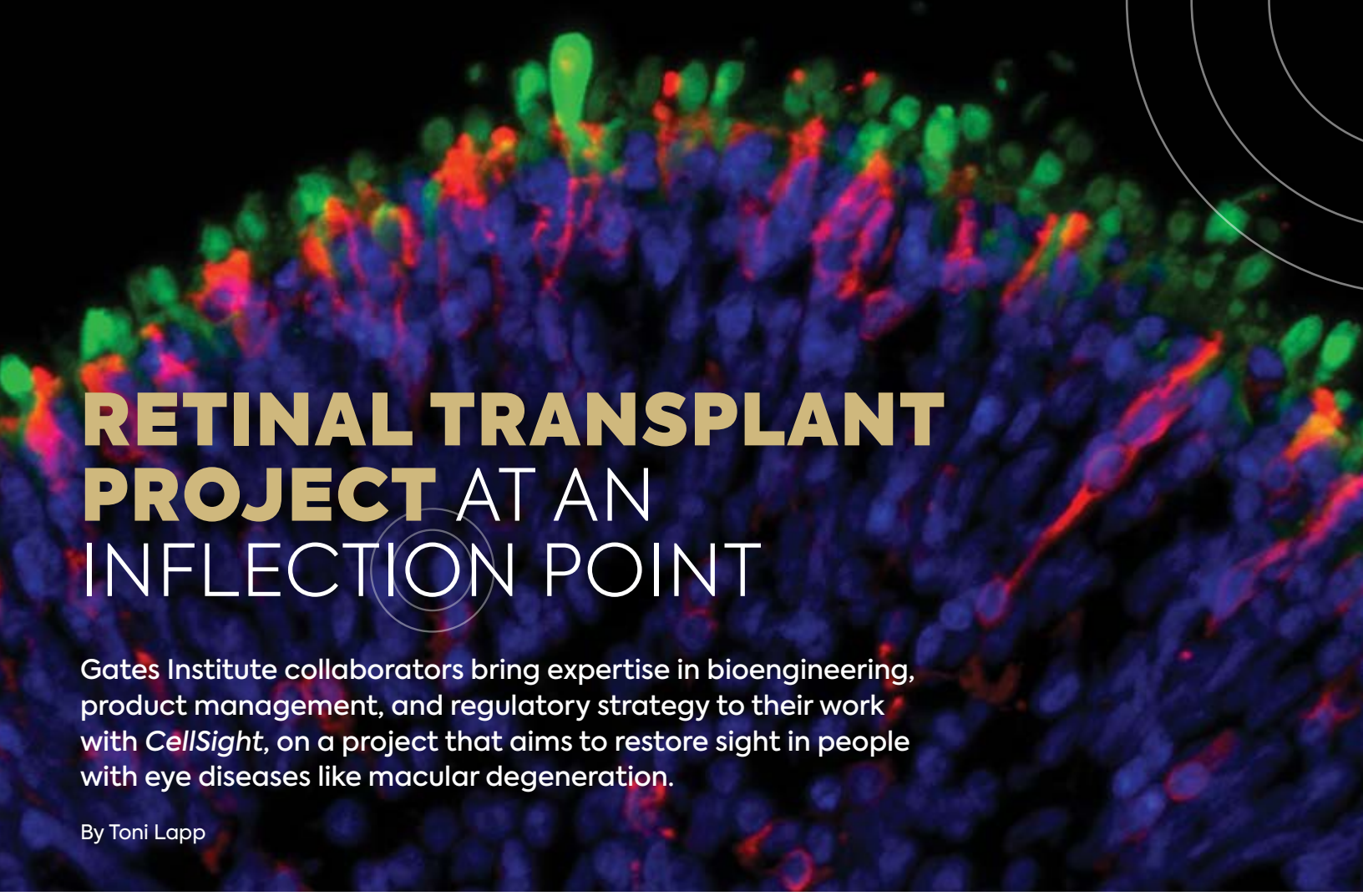
Eduardo Davila, PhD

And the addition of Liz Franks, PhD, a translational scientist with cell therapy expertise, "synergized our activities," says Davila.

Franks, who works at Gates Institute in cell therapy, joined the project after completing a postdoctoral fellowship at the National Cancer Institute. She is tasked with working on the preclinical requirements to file an IND application in 2025.

"It's such an incredibly strong team effort and we're just over the moon about it," says Davila. "Being able to impact a patient's life, and their family and friends, is just beyond words."

"It's such an incredibly strong team effort," says Eduardo Davila, center, of the work being done to bring tumor-infiltrating lymphocytes to clinical trials.



RETINAL TRANSPLANT PROJECT AT AN INFLECTION POINT

Gates Institute collaborators bring expertise in bioengineering, product management, and regulatory strategy to their work with *CellSight*, on a project that aims to restore sight in people with eye diseases like macular degeneration.

By Toni Lapp

When Valeria Canto-Soler, PhD, was recently named a recipient of the inaugural Anschutz Acceleration Initiative (AAI) for the *CellSight* project "Retinal Transplant to Restore Vision in Patients with Macular Degeneration," Gates Institute staff were celebrating behind the scenes. Canto-Soler, an associate professor in the Department of Ophthalmology and founding director of the ocular stem cell and regeneration research program, has been a Gates member since 2017, and several members of the Gates team (see list below) consulted with *CellSight* on the AAI application.

CellSight reflects the strong partnership between Gates Institute and the Department of Ophthalmology at the University of Colorado Anschutz School of Medicine. The program was founded on the belief that regenerative therapies could cure the most difficult-to-treat eye diseases, so there are many synergies with the Gates community.

"I think the AAI application was successful in large part because we had already been working with Gates on regulatory strategies and technology transfer to Gates Biomanufacturing Facility," Canto-Soler said following the AAI announcement. "We received critical feedback from Gates during the process. The success belongs to all of us."

GROUNDBREAKING DISCOVERY

Like many scientists in the cell and gene therapy sphere, Canto-Soler was inspired by the discovery in 2007 that human adult cells such as skin and blood cells, could be reprogrammed into embryonic-like cells, the basis for induced pluripotent stem cell (iPSC) technology. These iPSCs could theoretically develop into any cell type found in the human body. In 2014, Canto-Soler became the first scientist to demonstrate that iPSCs could generate light-sensitive retinal cells. However, retinal tissue is complex, made up of different cell types that are neatly organized in layers according to their function.

"My dream was to do more than just generate cells that mimic retinal cells," says Canto-Soler. "The idea was to create a 3D (three-dimensional) system that re-creates the cellular structure, physiology and function of the retinal tissue." In their 2014 publication, Canto-Soler and her team showed that

CellSight has rapidly expanded from six founding members when it launched in 2017.

iPSCs are capable of doing so. This groundbreaking technology is the foundation of the retinal transplant they are now developing.

The *CellSight* program has five research groups led by Canto-Soler, Natalia Vergara, PhD, Joseph Brzezinski, PhD, Miguel Flores-Bellver, PhD, and Marc Mathias, MD. From its six founding members, the team has rapidly expanded, now comprising scientific and strategic advisors Naresh Mandava, MD, Mark Petrash, PhD, Alan Palestine, MD, Project Manager Silvia Aparicio-Domingo, PhD, and over 20 internationally recruited staff and trainee members.

CellSight's success has been built from the ground up, just like its custom-built laboratory, because such retinal transplants have never been attempted in humans.

They've created several novel innovations to improve the chance of success:

- Collaborated with a private company to develop a hydrogel to improve transplant stability
- Collaborated with an ophthalmic imaging company to develop non-invasive technology to evaluate function of retinal cells
- Designed surgical instruments specifically for retinal transplants
- Tested immune suppressant drugs to avoid transplant rejection

"We're at an inflection point," says Canto-Soler. "We demonstrated proof-of-concept in 2022-2023. Our intention is to initiate preclinical studies in 2024 as a step toward filing an investigational new-drug application (IND), which is a requirement to initiate studies in patients."

TEAMWORK MAKES THE DREAM WORK

Jordan Krause, assistant director of product management at Gates Institute, has been collaborating with the *CellSight* team on the project. Having begun her career in the Gates Biomanufacturing Facility in 2015, she knows these next milestones will be a heavy lift.

"Transfer of any process from an academic lab to a GMP (Good Manufacturing Practice)-compliant format can be challenging," says Krause. "This process has been demonstrated to be robust and consistent in *CellSight* labs, but there are many complexities involved."

Handling iPSCs requires extensive training and expertise, she says. It's a complex process, with donor cells reprogrammed back to stem cells. Growing retinal cells in a petri dish takes about as long as it takes in a living being – five to six months. A duration of that length poses additional risks, adds Krause.

But perseverance is a hallmark of the *CellSight* team and collaborators at Gates Institute. Although the horizon for achieving the next milestones may be long, the researchers know what's at stake – this could someday restore sight and prevent blindness from macular degeneration and other eye diseases impacting millions of people.

"We are committed to finding a cure for blindness," says Canto-Soler. "Even though we cannot promise we will achieve this, we are giving our best to get as close as possible. Our dream of one day giving sight back to people is what drives us to continue forward no matter the challenges ahead."

Note: Photo on opposite page shows rod and cone photo receptors in retinal tissue derived from induced pluripotent stem cells.

TECHNOLOGY TRANSFER: THE KEY TO MOVING SCIENTIFIC DISCOVERIES FORWARD

By Russell C. Marians, PhD

All medicines produced in the USA must meet strict quality and manufacturing standards set forth by the FDA. Those standards, put in place to ensure safe and effective medicines are available to the public, are collectively known as the current Good Manufacturing Practices, or cGMPs. Both drug development and manufacturing require specialized teams of dedicated, intelligent people, but moving from development to manufacturing can be challenging because it necessitates transitioning between related yet disparate skillsets. Technology transfer is the process of forging the secret decoder ring that enables the development and manufacturing teams to effectively communicate with each other.

At the surface, technology transfer is exactly what it sounds like; the transfer of technology from one entity to another. When working with your friendly neighborhood cGMP facility (the Gates Biomanufacturing Facility, or GBF), technology transfer should take on a whole new meaning.

Before we get to the thesis, I want to introduce you to GBF. Our doors opened in 2015 as the current Good Manufacturing Practices (cGMP) arm of the University of Colorado Anschutz Medical Campus. Our mission is to feed the vibrant ecosystem of Anschutz, including the research labs and hospitals, by producing both biologics and cell therapies for phase 1 clinical trials. Our first cGMP protein was manufactured in early 2018, followed quickly by our first cGMP cell therapy run that same year. With over 130 cGMP runs under our belt, we do our best to partner with researchers in bringing innovative new therapies to patients.

Technology transfer centers on transferring technology from your facility to ours. The more we know about the technology, the easier it

Listening and collaboration are key to a smooth technology transfer process.

will be to manufacture. We're talking about materials lists, protocols, and analytical methods—everything. And the more detail, the better. The less we have to guess, the higher the likelihood of success. The more complete the data package, the better. But don't worry if the lab notebooks are not as thorough as you think they could be. Don't be alarmed if we ask about the precision of an assay and you don't know the answer. We will work together to figure out those things. At its most rudimentary level, this aspect of tech transfer can be a box-checking exercise. Protocol write-up: check. Materials list: check. Identity assay: check. But there's little to be gained from such a transactional relationship.

Both parties bring different skillsets and perspectives to the table. You, the technology developer, understand the technology inside and out, while we, the manufacturer, understand how to make medicine in a cGMP-compliant manner. There can be a big gap between those two knowledge bases and disparate opinions often result. That's because manufacturing has a different set of roles and responsibilities than does the research lab. The focus of the research lab is usually to develop the drug and demonstrate proof of concept the drug will elicit a certain response in a specific disease model. In cGMP, the goal is to produce the drug that meets FDA standards for identity, purity, and safety. As the technology holder and inventor, the principal investigator has committed years to understanding every nuance of that potentially therapeutic agent. The manufacturer has accumulated years of experience in addressing the FDA, enabling them to adapt and put forth solid manufacturing and analytical plans even if they have no direct experience with your particular therapeutic agent. In the research lab, the emphasis can be on getting things to work, because the process is often less important than the outcome. But in cGMP, it is critical to remember somewhere out there is a patient waiting for their medicine, so redos are not taken lightly. Questions will be asked and changes will be proposed. Not because we don't approve of the work that has been performed, but because cGMP has constraints unknown to the research lab. Jane may add cytokines one at a time to cells while John first makes a cytokine cocktail before moving on. A seemingly simple change in the order of operations can add variability to a process and must be locked down before moving to cGMP. The high-performance liquid chromatography purification method painstakingly developed over five months by a dedicated graduate student is no doubt useful in the academic lab but might not satisfy all the characteristics needed to successfully release a product by the quality control team. Even sourcing reagents can be a challenge. Commonly used reagents such as bovine serum and penicillin are frowned upon by FDA because they can transmit disease and pose a safety risk in certain individuals, details that may be unknown to the research lab. The FDA demands that level of control on behalf of the patients we're trying to serve. Ultimately, a therapeutic candidate will not make it to the patient if it cannot be manufactured. That is why manufacturability is almost always a gating item to venture capital investment. However, changes proposed by GBF may not be feasible in the context of the specific agent in question. Then it is your job to explain why and help find alternatives. Productive discourse is the key to success. Working together, we will move that potentially therapeutic medicine from the bench to the bedside and improve patient's lives.

GBF DEPLOYS CAR T-CELL PROCESS IMPROVEMENT

Using a frozen apheresis as the starting point for a CAR T product can provide benefits to clinical teams and increase treatment options for patients.

Benjamin Skinner began testing frozen leukopaks as a source of cells for the biomanufacturing process in 2022.

Since receiving FDA approval in 2017, chimeric antigen receptor (CAR) T-cell therapy has shown remarkable success in treating patients with certain blood cancers such as lymphoma and leukemia. A complex biomanufacturing process is required to produce the therapy, which is performed for each individual patient. The process begins with apheresis, generating a leukopak containing the patient's white blood cells. From there, T cells are isolated, genetically engineered to create CAR proteins that enable T cells to attack cancer cells, and multiplied in a laboratory. They are then reintroduced to the patient.

Until recently, manufacturing at Gates Biomanufacturing Facility (GBF) began with a patient's freshly drawn leukopak, typically received within 24 hours of apheresis, as a source of cells for the manufacturing process. However, the GBF has successfully tested techniques using cryopreserved leukopaks as a starting point, which could have far-reaching implications – for researchers, clinicians, and patients. Because CAR T-cell therapy is typically implemented after a patient has relapsed following chemotherapy, being able to cryopreserve healthy white blood cells before chemotherapy could improve outcomes. In addition, using cryopreserved cells could ease some of the logistical challenges of scheduling, shipping, and biomanufacturing processes, says GBF Executive Director Matt Seefeldt, PhD.

"Starting from cryopreserved starting material allows the clinic to 'archive' the blood prior to any chemotherapy for the patient, so the cells will be in a healthier state," says Seefeldt. "In addition, the fresh blood expires 48 hours after the patient apheresis, so starting from cryopreserved cells allows the GBF and the clinic to have flexibility in its scheduling."

Seefeldt and cell process engineer Benjamin Skinner began testing cryopreserved leukopaks donated by healthy individuals in 2022.

"The thaw process was designed to overcome two different mechanisms of poor cell recovery: coagulation and thaw-induced cell death," says Skinner. "Coagulation would cause cells to be trapped as part of the normal clotting cascade, which initiates as the product thaws. Immediately after thaw, the cells are fragile and sensitive to osmotic stress. Moving cells from the solution they're frozen into, and then transferred into the solution needed for manufacturing is enough to kill a large portion of the cells unless that process is performed carefully." Regulatory approval was required before using CAR T cells made from frozen apheresis in an actual patient, says Cheri Adams, MSHS, program director of Regulatory Strategy at Gates Institute. "This meant amending the investigational new drug application to include the use of cryopreserved cells," she says. "To satisfy the Food and Drug Administration, the GBF had to show that the finished product had a comparable risk-to-benefit profile as a therapy produced with fresh apheresis."

The first products were administered in September 2023 to pediatric patients with B-cell lymphoblastic leukemia enrolled in a phase 1/2 trial of a CD19 CAR T cell at Children's Hospital Colorado.

"A lot of work has been put into it making sure the cells are viable," says Seefeldt. "This is a significant manufacturing improvement, which will enhance cell and gene therapy research at CU Anschutz and benefit our pediatric patients who require manufacturing flexibility."

An operator prepares a nutrient mixture for growing CAR T cells in the process development lab.

HIGHLIGHTS

SELECT MEMBER PUBLICATIONS

A Case of Fetal-Induced Graft-versus-Host Disease. New England Journal of Medicine

Michael Alberti, MD, PhD, Department of Pathology, CU Anschutz

Transfusion-associated graft-versus-host disease is a rare complication following blood transfusions, resulting from donor T cells reacting against patient skin, liver, gastrointestinal tract, or hematopoietic stem cells. In a letter to the editor, Alberti and coauthors note that pregnancy creates a unique immunologic challenge requiring adaptation for a fetus to survive in utero. Although a two-way transfer of nucleated cells occurs between the mother and fetus during pregnancy, and small numbers of fetal cells may survive and integrate into maternal tissues, studies have shown rapid clearance of fetal DNA from maternal circulation shortly after birth.

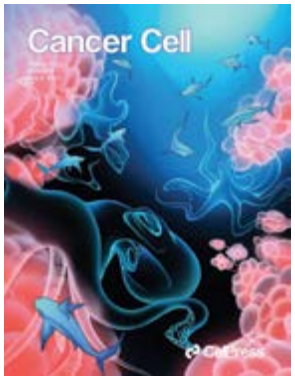
LTP induction by structural rather than enzymatic functions of CaMKII. Nature

Ulli Bayer, PhD, Department of Pharmacology, CU Anschutz

New research challenges long-standing dogma about the molecular mechanisms underlying learning and memory. A team of researchers led by Ulli Bayer report that long-term potentiation, which is crucial for learning and memory, requires structural rather than enzymatic functions by an enzyme known as CaMKII. Ulli and his colleagues write in this Aug. 30 article that "it has not escaped our notice that the specific findings we have reported immediately suggest possible therapeutic applications for chronic CaMKII inhibition."

Simultaneous targeting of PD-1 and IL-2R bg with radiation therapy inhibits pancreatic cancer growth and metastasis. Cancer Cell

Angelo D'Alessandro, PhD, Department of Biochemistry and Molecular Genetics, CU Anschutz.



In pancreatic ductal adenocarcinoma (PDAC) patients, D'Allesandro and his coauthors show that response to radiation therapy (RT) is characterized by increased IL-2Rb and IL-2Rg along with decreased IL-2Ra expression. Using a murine PD-1-targeted IL-2 variant antibody complex (PD1-IL2v) composed of a high-affinity anti-PD-1 antibody fused to an IL-2v with abolished binding to CD25 (IL-2Ra) allowed for the expansion of tumor-antigen-specific T cells and their differentiation toward functional immune effectors. In combination with single-dose RT, PD1-IL2v treatment resulted in reduced tumor growth and metastasis, reduced tumoral Tregs while increasing systemic NK cell frequency, induced a durable memory response with tumor eradication, and showed evidence that human PDAC patients upregulate molecular targets of PD1-IL2v post-RT. These data show that PD1-IL2v leads to profound local and distant response in PDAC.

Combination Early-Phase Trials of Anticancer Agents in Children and Adolescents. Journal of Clinical Oncology

Lia Gore, MD, Department of Pediatrics/Hematology, Oncology and Bone Marrow Transplantation, CU Anschutz

There is an urgent need to develop new therapies for children and adolescents with cancer, to improve outcome for poor prognosis malignancies and to reduce acute and long-term adverse effects of current treatments. Strong biological rationale and clinical experience suggest that multiple agents will be more efficacious than monotherapy for most diseases and may overcome resistance mechanisms and increase synergy. Therefore, there is an increasing need to evaluate innovative drugs for childhood cancer using combinatorial strategies. The general principle for combination early-phase studies is that, when possible, clinical trials should be dose- and schedule-confirmatory rather than dose-exploratory, and every effort should be made to optimize doses early. An optimized, agreed approach to the design and evaluation of early-

phase pediatric combination trials will accelerate drug development and benefit all stakeholders, most importantly children and adolescents with cancer.

Phase 1 dose escalation study of DSP107, a first-in-class CD47 and 4-1BB targeting fusion protein, in combination with atezolizumab in patients with advanced solid tumors. Journal of Clinical Oncology

Antonio Jimeno, MD, PhD, Department of Medicine/ Medical Oncology, CU Anschutz

DSP107 is a novel, CD47 and 4-1BB targeting fusion protein with a differentiated safety, binding and pharmacodynamic profile compared to other CD47 and 4-1BB targeting agents. This paper describes results from a cohort of patients with solid tumors that completed a DSP107 plus atezolizumab combination dose escalation portion of study. In 19 patients, DSP107 with atezolizumab was well tolerated with no dose limiting toxicities. Grade 1-2 treatment related adverse events (AEs) were observed in 68% of patients (13/19). The most frequent AEs included diarrhea (21%), fatigue (21%), infusion related reaction (IRR; 16%), nausea, decreased appetite, arthralgia, myalgia, and Grade 1 anemia (11% each). These phase 1 data also suggested that the combination of DSP107 with atezolizumab may have anti-tumor activity in cold tumors such as microsatellite stable-colorectal cancer.

Molecular Function and Contribution of TBX4 in Development and Disease. American Journal of Respiratory and Critical Care Medicine

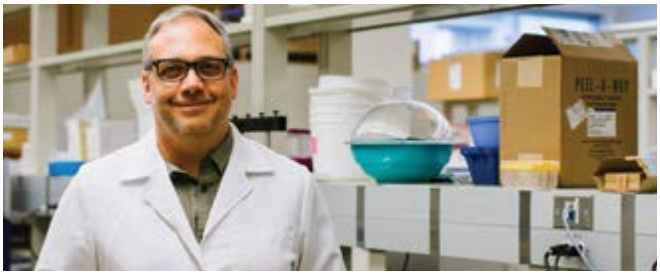
Christian Mosimann, PhD, Department of Pediatrics/ Section of Developmental Biology, CU Anschutz

Over the past decade, recognition of the profound impact of the TBX4 (T-box 4) gene, which encodes a member of the evolutionarily conserved family of T-box–containing transcription factors, on respiratory diseases has emerged. The developmental importance of TBX4 is emphasized by the association of TBX4 variants with congenital disorders involving respiratory and skeletal structures; however, the exact role of TBX4 in human development remains incompletely understood. Here, we discuss the developmental, tissue-specific, and pathological TBX4 functions identified through human and animal studies and review the published TBX4 variants resulting in variable disease phenotypes. We also outline future research directions to fill the gaps in our understanding of TBX4 function and of how TBX4 disruption affects development.

CD4+ CAR T cells — more than helpers. Nature Cancer

M. Eric Kohler, MD, PhD, Department of Pediatrics-Hematology/Oncology, CU Anschutz, and Terry Fry, MD, executive director, Gates Institute

The coauthors note in their May 2023 article that therapeutic products containing CD8+ and CD4+ T cells expressing CARs effectively induce remission in patients with cancer. How CD4+ CAR T cells contribute to the anti-tumor response has not been well established. This commentary highlights a study that used syngeneic models and in vivo imaging to glean mechanistic insights into how CD4+ T cells target tumors.



An in vitro characterization of a PCL-fibrin scaffold for myocardial repair. Materials Today.

Jeffrey G. Jacot, PhD, Department of Bioengineering, CU Denver

In a December 2023 article, Jacot and his coauthor describe work to create a biodegradable patch that could be used to correct congenital heart defects in infants. Currently, patch materials are exclusively non-living and non-degradable. As a result, they do not grow with the patient and are prone to fail. Jeffrey's work seeks to develop a full-thickness, tissue-engineered myocardial patch from biodegradable components.

HIGHLIGHTS

GRANTS AND AWARDS

Angelo D'Alessandro, PhD, professor of biochemistry and molecular genetics, was awarded funding with colleagues at the University of Pittsburgh by the Biomedical Advanced Research and Development Authority of the U.S. Department of Health and Human Services, to test interventions for life-threatening bleeding in traumatically injured children.

M. Eric Kohler, MD, PhD, received a $150,000 Scholar Award from the American Society of Hematology (ASH) to investigate a method to make CAR T cells function even better. His research, which also received Gates Grubstake funding in 2022, resulted in the creation of a new type of CAR cell that is able to more effectively go after tumor cells with lowered levels of the target protein.

Tânia Reis, PhD, is one of eight recipients of the National Institutes of Health's prestigious Director's Pioneer Award for 2023. The grant provides Reis with $3.5 million over the next five years to continue her research into ways that organs communicate with one another to maintain energy balance. She is the first researcher from the CU Anschutz Medical Campus to win the Director's Pioneer Award.

Dennis Roop, PhD, Gates Institute associate director and professor of dermatology, with **Anya Bilousova, PhD**, and **Igor Kogut, PhD**, associate professors of dermatology, received Department of Defense funding of $4.6 million for the project, "Stem Cell-Based Therapy for Recessive Dystrophic Epidermolysis Bullosa Delivered with a Spray-On Skin Device."

The EB Research Partnership provided $999,724 in new funding to Roop, Bilousova and Kogut for their project on adapting the induced pluripotent stem cell-based therapy for recessive dystrophic epidermolysis bullosa to an automated robotic platform to facilitate clinical translation in the Gates Biomanufacturing Facility (GBF). Roop with **Anna Bruckner, MD**, professor of dermatology and director of the EB Center of Excellence at Children's Hospital Colorado, received $1,076,400 in funding from NIAMS (National Institute of Arthritis and Musculoskeletal and Skin Diseases). This award was received in response to an NIH award mechanism for the 21st Century Cures Act for the Regenerative Medicine Innovation Project (RMIP). This award mechanism requires 1 to 1 matching funds from non-federal funds, and EB Charities (New York-based EB Research Partnership, Los Angeles-based EB Medical Research Foundation and London-based Cure EB) provided the matching funds. The University of Colorado (Roop, Bilousova, Kogut and Bruckner) is the only institution to receive three rounds of funding from the RMIP. The UG3 phase of this award provides one year of funding to plan a clinical trial. A Study of the Safety, Tolerability and Efficacy of an iPS Cell-based Therapy for Recessive Dystrophic Epidermolysis Bullosa Delivered with a Spray on Skin Device. The UH3 phase ($4,305,600) of the award is contingent on the Colorado team receiving investigational new drug (IND) approval from the FDA.

HONORS

Manali Kamdar, MD, associate professor of medicine and clinical director of lymphoma services, has been named the Morton and Sandra Saffer Endowed Chair, which was created by the Saffer family in gratitude for care they received after a lymphoma diagnosis. She also received the School of Medicine's Distinguished Clinician Award.

Heartfelt appreciation goes to **David Norris, MD**, chair of dermatology, for his dedicated service to the CU Anschutz campus and his impressive achievements in medical science. David joined the faculty in 1977 and was appointed department chair in 2001. He is an internationally renowned investigator in cutaneous immunology and cell biology, and melanoma research. **Maryam Asgari, MD, MPH**, was named the inaugural University of Colorado Medicine Endowed Chair of the Department of Dermatology for the CU School of Medicine, effective May 1, 2023.

Traci Lyons PhD, associate professor of medicine at University of Colorado Anschutz Medical Campus, Grubstake recipient, SPARK awardee, and founder of Pearl Scientific, has been accepted into the first cohort of the prestigious BrightEdge - American Cancer Society Program. BrightEdge propels groundbreaking patient-centric solutions by investing in and supporting the most innovative startup companies to advance science, reduce disparities, and promote healthcare sustainability.

SEMINARS RENAMED IN HONOR OF JOHN S. GATES

An architect and lifelong Coloradan, **John S. Gates**, co-trustee of the Gates Frontiers Fund (pictured above), was a prominent supporter of Gates Institute. As the son of our namesake Charles C. Gates, he shared his father's passion for translating research for the benefit of patients. He passed away in 2023 at the age of 66.

Gates Institute renamed its signature seminar series in his honor. The John S. Gates Seminars are hybrid presentations on the fourth Tuesday of the month feature leading investigators involved in cell and gene therapy and regenerative medicine research. Scan the QR code at right for the John S. Gates Seminar Series Schedule.

2024 Presenters

- **Michael Verneris, MD**, Translational Sciences Lead, Gates Institute
- **Eric Kohler, MD, PhD**, Department of Pediatric Hematology/Oncology/BMT, CU Anschutz School of Medicine
- **Josephine Lembong, PhD**, and **Laura Stringham**, Alliance for Regenerative Medicine
- **Daniel LaBarbera, PhD**, CU Anschutz School of Pharmacy
- **Jordan Jacobelli, PhD**, Department of Immunology and Microbiology, CU Anschutz School of Medicine
- **Kristin Watt, PhD**, Department of Craniofacial Biology
- **Sujatha Venkataraman, PhD**, Department of Pediatric Hematology-Oncology, CU Anschutz School of Medicine
- **Miguel Flores-Belver, PhD**, Department of Ophthalmology, CU Anschutz School of Medicine
- **Mi-Hyun Nam, PhD**, Department of Ophthalmology, CU Anschutz School of Medicine
- **Dan Sherbenou, MD, PhD**, Department of Medicine-Hematology, CU Anschutz School of Medicine
- **Hatim Sabaawy, MD, PhD, MS**, Department of Medicine-Medical Oncology, CU Anschutz School of Medicine

NEW STAFF IN 2023

Science/research/clinical staff

Chris Garbe, MS, MBA, joined Gates Institute as Technical Operations Director. In this role, he provides research leadership, designing process, analytical, product development, quality, and regulatory strategies to advance biological and cell/gene therapies. He previously worked at Gates Biomanufacturing Facility as Director of Quality.

Veena Krishnamoorthy, PhD, joined Gates Institute as Head of Preclinical Development. She will lead a team of scientists and researchers who are transforming cell therapy for cancer and other indications.

Navin Pinto, MD, a professor in the Department of Pediatrics at University of Colorado Anschutz School of Medicine, was named Medical Lead at Gates Institute. He will work in partnership with the Investigational New Drug and Device (IND/IDE) Office to oversee Gates Institute-supported clinical trials.

Gina Vanderveen joined Gates as the Clinical Trial Operations Manager on the Clinical Investigation and Regulatory Sciences (CELLS) team. She is responsible for ensuring that day-to-day trial activities run efficiently and compliantly with all stakeholders.

Administrative staff

Alyssa Bowman, Business Services Professional
Toni Lapp, Communications Manager
Ronan Witte, Senior Financial Analyst

Biomanufacturing staff

Robert "Ked" Byrd, BIO Process Development/Mfg. Associate III
Michael Caffrey, Assistant Director, Facilities
Jason Hopkins, Senior Accountant
Erling Howard, Facilities Maintenance Associate III
Chris Petrelli, Project Manager II
Michael Yarnell, CT Process Engineer II

GATES SUMMER INTERNSHIP PROGRAM 2023

By Jill Cowperthwaite

GSIP CLASS OF 2023

FRONT ROW From left: Jennan (Jeny) Lahamer, Kanita Hrustanovic, Leia Jiang, Aiman Sabaawy, Taylor LaValley, Catherine Bates, Ilana Baum, Emily Xie, Amanda (Manna) Morris, Anna Gartner, Amelia Grosskopf

MID ROW From left: Haley Sax, Anna Pocsi, Zebulon (Zeb) Trovinger

BACK ROW From left: Blake Barron, Oreoluwa Ladele, McKinley (Mac) Rump, Aikhan Tugambekova, Andrew Lu, Mary (Claire) Howell, Ananya Talanki, Julia Johnson, Bailey Leaphart, Jeevan Mann, Yifei Chen

The 23 members of the Gates Summer Internship Program (GSIP) Class of 2023 became the first GSIP cohort under Gates Institute.

Every year, we greet the arrival of our Gates Summer Internship Program interns with joy and enthusiasm, but the Class of 2023 interns must have felt a little more fanfare than typical. They arrived in late May, after a month of events marking the launch of Gates Institute.

Over the next 11 weeks, the GSIP students, along with two additional interns from Berea College, with which GSIP enjoys an ongoing summer internship arrangement, experienced a variety of opportunities and challenges working in Gates Institute members' labs and developing their projects. They attended an array of seminars and events designed to expose them to high-impact opportunities and career paths. Without exception, these talented students excelled in their laboratory placements and developed friendships and connections with their mentors, fellow lab members, Gates Institute staff and our seminar speakers.

The summer program culminated in the final day poster presentation. As always, it is immensely gratifying to see these young minds gain confidence as public speakers in this setting, displaying the knowledge they've acquired from hands-on lab experience and interactions with their mentors and peers.

Their departure, as usual, was somewhat bittersweet for us as we reflected on the summer. We welcomed fresh-faced undergraduates in May, but bade farewell to seasoned researchers in August.

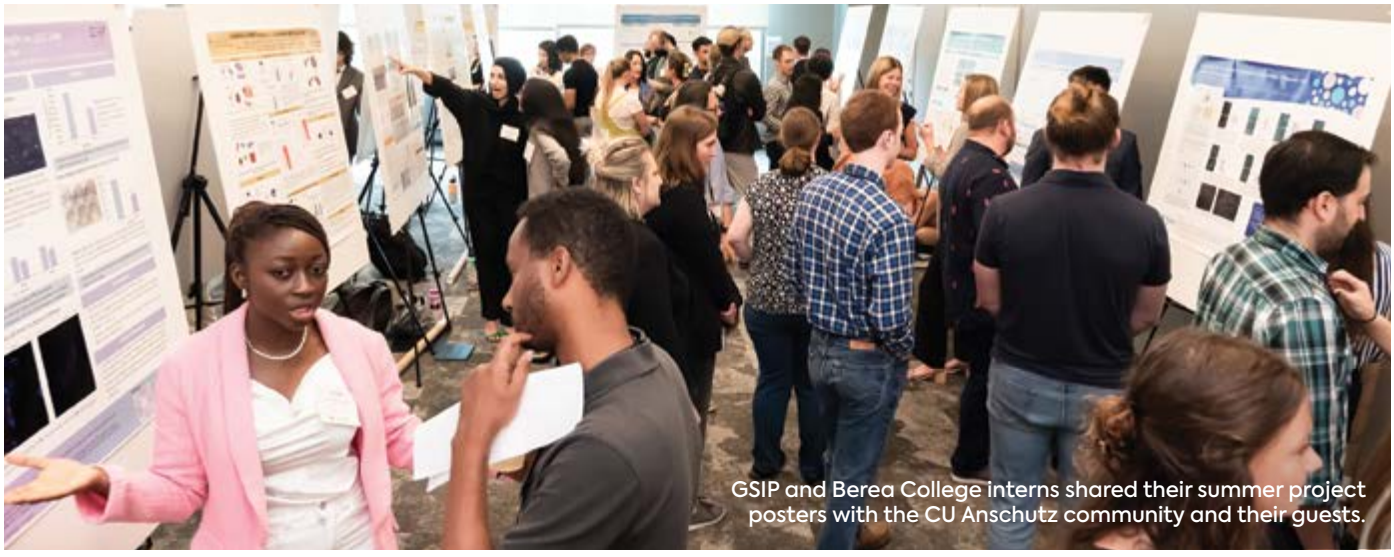
Ultimately, their contributions will advance medical research on the Anschutz Medical Campus. This is what Gates members Neal Box, PhD, Tamara Terzian, PhD, and Enrique Torchia, PhD, had in mind when they recommended launching an internship program focused on regenerative medicine over a decade ago. We're so grateful for the generosity of the late Peter Grant, and his widow, Rhondda, who continues to support the program – as their gift enabled us to take a great idea and bring it to fruition.

We look forward to celebrating the tenth year of the program next summer when we welcome another group of talented interns to conduct research in Gates laboratories and become engaged with the transformative science that occurs at CU Anschutz.

Final Day Grant Family Distinguished Speaker, Michael Holers, MD, shared career advice with our interns and the community.

GSIP Co-Director Jill Cowperthwaite welcomed the crowd to our Final Day Program.

> **Our interns are bright and highly committed to their research projects; they perform at the level of PhD students!**
>
> – M. Valeria Canto-Soler, PhD
> GSIP Mentor



GSIP and Berea College interns shared their summer project posters with the CU Anschutz community and their guests.

EDUCATION

GSIP STUDENTS AND MENTORS

Blake Barron
Davidson College
Mentor: Ram Nagaraj, PhD
Professor, Ophthalmology

Catherine Bates
University of Colorado Boulder
Mentor: Russell Marians, PhD
Associate Director, Gates Biomanufacturing Facility - Cellular Therapies

Ilana Baum
Occidental College
Mentor: Michael Alberti, MD, PhD
Assistant Professor, Pathology

Yifei Chen
Johns Hopkins University
Mentor: Ronald Vagnozzi, PhD
Assistant Professor, Cardiology

Anna Gartner
Wake Forest University
Mentor: Nicholas Jacobson, MDes
3D/Bioprinting/Cardiology

Amelia Grosskopf
Middlebury College
Mentor: Maria Natalia Vergara, PhD
Assistant Professor, Ophthalmology

Mary (Claire) Howell
Davidson College
Mentor: Lori Sussel, PhD
Professor, Cell and Developmental Biology

Kanita Hrustanovic
Colorado State University
Mentor: Mi-Hyun Nam, PhD
Research Instructor, Ophthalmology

Leia Jiang
University of Texas at Austin
Mentor: Miguel Flores-Bellver, PhD
Assistant Professor, Ophthalmology

Julia Johnson
Middlebury College
Mentor: Daniel Sherbenou, MD, PhD
Associate Professor, Hematology

Oreoluwa Ladele
Berea College
Mentor: Xiying Fan, PhD
Assistant Professor, Dermatology

Taylor LaValley
Miami University
Mentor: Kristen Boyle, PhD
Associate Professor, Pediatrics

Bailey Leaphart
Davidson College
Mentor: Lori Walker, PhD
Associate Professor, Cardiology

Andrew Lu
Washington University in St. Louis
Mentor: Chelsea Magin, PhD
Assistant Professor, Bioengineering, Pediatrics

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

Amanda (Manna) Morris
University of Colorado Denver
Mentor: Bruce Appel, PhD
Professor, Pediatrics

Anna Pocsi
University of Colorado Boulder
Mentor: Jeffrey Jacot, PhD
Associate Professor, Bioengineering

McKinley (Mac) Rump
University of Colorado Boulder
Mentor: Eric Pietras, PhD
Associate Professor, Hematology

Aiman Sabaawy
Cornell University
Mentor: Santos Franco, PhD
Assistant Professor, Pediatrics

Haley Sax
Hobart and William Smith Colleges
Mentor: Jennifer Richer, PhD
Professor, Pathology

Ananya Talanki
Emory University
Mentor: Gana (Dash) Batt, PhD
Associate Director, Biomanufacturing – Protein Development

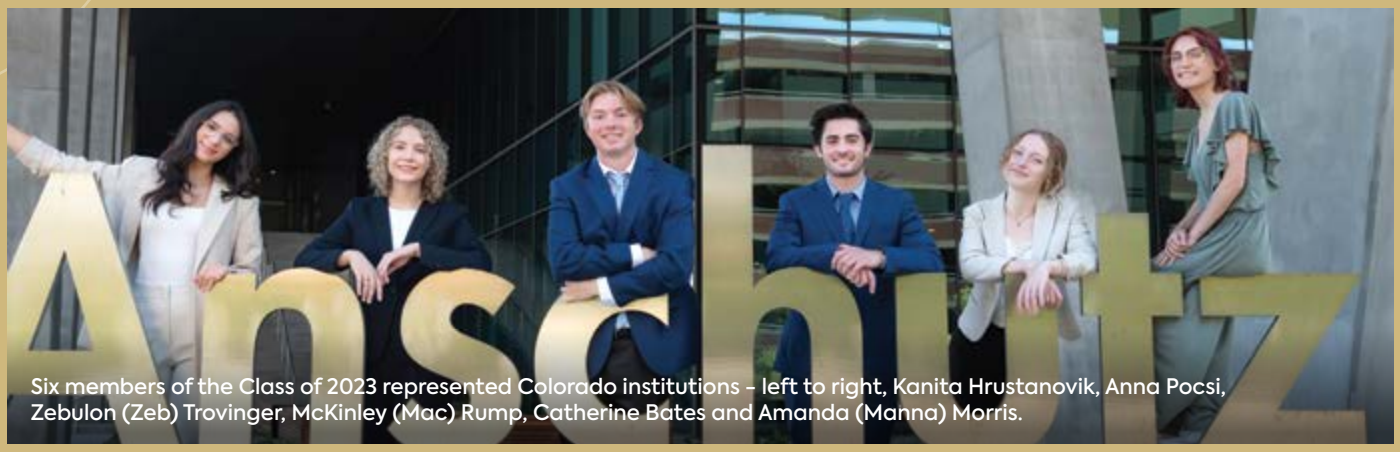
Zebulon (Zeb) Trovinger
University of Colorado Boulder
Mentor: Joseph Brzezinski, PhD
Associate Professor, Ophthalmology

Emily Xie
University of California, Berkeley
Mentor: Karin Payne, PhD
Associate Professor, Orthopedics

BEREA INTERNS

Jennan (Jeny) Lahamer
Berea College
Mentor: Xiying Fan, PhD
Assistant Professor, Dermatology

Aikhan Tugambekova
Berea College
Mentor: Igor Kogut, PhD
Associate Professor, Dermatology



Six members of the Class of 2023 represented Colorado institutions – left to right, Kanita Hrustanovik, Anna Pocsi, Zebulon (Zeb) Trovinger, McKinley (Mac) Rump, Catherine Bates and Amanda (Manna) Morris.


Cellular Therapies Associate Director Russell Marians, on left, tours interns through the Gates Biomanufacturing Facility.

"

This internship solidified my career path to include research into clinical medicine. The research allowed the interns to see not only the research side of medicine but also the translation to the clinic. The seminar speakers also gave us an opportunity to connect with people in industry and academia to determine the work-life balances that accompany each career.

– GSIP Intern


All CU Anschutz summer internship program interns got together for a pizza party in June.

"

This was truly an amazing summer, and I am so grateful for all the work that was put in behind the scenes for us. Every lecture I attended, every skill I acquired, and every opportunity that came my way through GSIP is a testament of this program's belief in education and commitment to our futures.

– GSIP Intern


A glorious day in Rocky Mountain National Park hosted by Rhondda and Liza Grant.

"

My mentor and team were by far the best I have ever had! They supported me throughout, worked beside me on the bench, and dedicated so much of their time and energy to helping me succeed.

– GSIP Intern

AT THE INTERSECTION OF CELL BIOLOGY, STEM CELLS, AND DEVELOPMENT

Gates Institute provides financial support for the CSD PhD program at CU Anschutz

By Jeff Moore

One of 12 graduate training programs on the University of Colorado Anschutz Medical Campus, this program is distinguished by a specific focus at the intersection of Cell biology, Stem cell biology and Developmental biology (CSD). Our goal is to train the next generation of leaders in understanding the origins of human disease and discovering new therapeutic avenues. Gates Institute continues the support, begun in 2015 by the Gates Center, for the growth and vibrance of the CSD.

Support from the Gates Institute allows CSD students to travel to national and international conferences, where they present their work and build relationships with leading researchers in their fields. In 2023, Gates Institute supported 16 CSD students for travel to conferences across the US and abroad, including Sophia Kim, who won a presentation award at the 2023 Brain Barriers meeting at Cold Spring Harbor Laboratory for her work on meningeal layer loss during neonatal meningitis infections.

After graduation, CSD alumni go on to pursue careers in academia, science communication, nonprofit organizations, the biotech industry, and more. Every year, one of our fabulous alumni returns to the Anschutz Campus for a homecoming visit to present a seminar on their recent science endeavors and meet with current CSD students to discuss their career path. Support from the Gates Institute helps fund these visits. This year, we were thrilled to welcome back David Castillo Azofeifa, PhD. David completed his thesis in the lab of Dr. Linda Barlow and now leads his own research group at Genentech. In his seminar, David described how his training in CSD propelled him to a postdoc at University of California San Francisco, where he became an expert in intestinal stem cell models, and ultimately to his position at Genentech, where he studies the regeneration of intestinal epithelia and other epithelial tissues.

David Castillo Azofeifa, PhD

Looking ahead, CSD is expanding access to research training to prepare the future leaders of biomedicine. The Gates Institute partners in that effort. In 2023, support from the Gates Institute helped continue the Developing Scholars Program (see photo below), which provides students biomedical training opportunities for students from undergraduate-serving institutions in Colorado that have limited facilities and resources for research training. These scholars come to the Anschutz campus for nine weeks in the summer, working under the mentorship of PhD students in CSD. This is a first step in their science journey.

Members of the 2023 Developing Scholars Program.
Top row, left to right: Ian Purvis, Sylvia Nunez, Elliott Brooks, McKenna Casey, Jeff Moore
Middle row: Olivia Clise, Ayaka Schmitz, Braelyn Cayaban, Sophia Schmidt, Andrew Han
Front row: Emma Thomure, Chris Schaaf, Tina Piarowski, Trevor Isner, Bryan Johnson

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 providing similar services.

- 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.

Its presence at our campus has been a key element in the recruitment and retention of leading cell and gene therapy researchers at the CU Anschutz Medical Campus.

NETWORKING OPPORTUNITIES

Gates Institute serves as a networking hub for its membership with events throughout the year to foster collaboration and provides an opportunity to conduct essential aspects of research cost effectively. 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.

MEMBER DIRECTORY

Michael Alberti, MD, PhD
Assistant Professor, Pathology
CU Anschutz

Bruce Appel, PhD
Professor, Pediatrics, Cell and Developmental Biology
Diane G. Wallach Chair in Pediatric Stem Cell Biology
CU Anschutz

Kristin Artinger, PhD
Professor, Department of Diagnostic and Biological Sciences
University of Minnesota School of Dentistry

Reed Ayers, PhD
Assistant Research Professor, Orthopedics
CU Anschutz

Susan Bailey, PhD
Professor, Environmental and Radiological Health Sciences
Colorado State University

Christopher Baker, MD
Associate Professor, Pediatrics
CU Anschutz

James Bamburg, PhD
Professor, Biochemistry and Molecular Biology
Colorado State University

Linda Barlow, PhD
Professor, Cell and Developmental Biology
CU Anschutz

William Barrett, JD*
Partner, Wilson Sonsini Goodrich & Rosati

Ulli Bayer, PhD
Professor, Pharmacology
CU Anschutz

Jaime Belkind-Gerson, MD, MSc
Associate Professor, Pediatrics, Digestive Health Institute
CU Anschutz

Leslie Berg, PhD*
Professor & Chair, Immunology & Microbiology
CU Anschutz

Ganna Bilousova, PhD
Associate Professor, Dermatology
CU Anschutz

Stanca Birlea, MD, PhD
Associate Professor, Dermatology
CU Anschutz

Petter Bjornstad, MD
Assistant Professor, Pediatric Endocrinology and Nephrology
Children's Hospital Colorado
CU Anschutz

Andrea Bonetto, PhD*
Associate Professor, Department of Pathology
CU Anschutz

Neil Box, PhD
Associate Professor, Dermatology
CU Anschutz

Kristen Boyle, PhD
Associate Professor, Pediatrics
CU Anschutz
National Jewish Health

Anna Bruckner, MD, MSCS
Professor, Dermatology, Pediatrics
CU Anschutz
Children's Hospital Colorado

Justin Brumbaugh, PhD
Assistant Professor, Molecular, Cellular and Developmental Biology
CU Boulder

Stephanie Bryant, PhD*
Professor, Department of Chemical and Biological Engineering
CU Boulder

Joseph Brzezinski, PhD
Associate Professor, Ophthalmology
CU Anschutz

Jason Burdick, PhD*
Professor, Department of Chemical and Biological Engineering
BioFrontiers Institute
CU Boulder

Ellen Burnham, MD, MS
Associate Professor, Pulmonary Sciences and Critical Care
CU Anschutz

Valeria Canto-Soler, PhD
Associate Professor, Ophthalmology
Doni Solich Family Chair in Ocular Stem Cell Research
CU Anschutz

Christina Couglan, PhD*
Assistant Professor, Neurology
CU Anschutz

Ryan Crisman, PhD
Co-Founder and Chief Technical Officer
Umoja Biopharma

Angelo D'Alessandro, PhD
Professor, Biochemistry and Molecular Genetics, Division of Hematology
CU Anschutz

Eduardo Davila, PhD
Professor, Medical Oncology, Immunology
Amy Davis Chair in Basic Immunology Research
CU Anschutz
VA Medical Center

James DeGregori, PhD
Professor, Biochemistry and Molecular Genetics
Courtenay C. and Lucy Patten Davis Endowed Chair in Lung Cancer Research
CU Anschutz
VA Medical Center

Peter Dempsey, PhD
Associate Professor, Pediatrics
CU Anschutz

Steven Dow, DVM, PhD
Professor, Clinical Sciences
Colorado State University

Jason Dragoo, MD
Professor, Orthopedics, and Vice Chair of Academic Affairs
Endowed Chair of Regenerative Medicine
CU Anschutz

Nicole Ehrhart, VMD, MS, Diplomate ACVS
Professor, Clinical Sciences, School of Biomedical Engineering
Colorado State University

Patricia Ernst, PhD
Professor, Pediatrics
Postle Family Chair in Pediatric Cancer and Blood Disorders
CU Anschutz

Xiying Fan, PhD
Assistant Professor, Dermatology
CU Anschutz

Michael Flores-Bellver, PhD*
Assistant Professor, Ophthalmology
CU Anschutz

Terri Foote, MBA
Director, Program Management and Supply Chain
Gates Biomanufacturing Facility
CU Anschutz

Heide Ford, PhD
Professor and Chair, Pharmacology
University of Colorado Medicine Endowed Chair in Pharmacology
CU Anschutz

Santos Franco, PhD
Assistant Professor, Pediatrics, Developmental Biology
CU Anschutz

Brian Freed, PhD
Professor, Allergy and Clinical Immunology
CU Anschutz

Curt Freed, MD
Professor, Clinical Pharmacology and Toxicology
Leopold Korn and Michael Korn Chair in Parkinson's Disease
CU Anschutz

Jed Friedman, PhD
Professor, Pediatrics
CU Anschutz

Terry Fry, MD
Professor, Pediatrics, Hematology and Immunology
Charles C. Gates Endowed Chair at the Gates Institute
Executive Director, Gates Institute
CU Anschutz

Mayumi Fujita, MD, PhD
Professor, Dermatology, Immunology and Microbiology
CU Anschutz
VA Medical Center

Robert Fuhlbrigge, MD, PhD*
Professor, Children's Hospital–Rheumatology
CU Anschutz

Christopher Garbe, MBA
Senior Director, CMC and Allogeneic Cell Therapy Program
Technical Operations Director, Gates Institute
Umoja Biopharma

Kathleen Gavin, PhD
Assistant Professor, Division of Geriatric Medicine
CU Anschutz

Moumita Ghosh, PhD
Assistant Professor, Division of Pulmonary, Critical Care and Sleep Medicine
National Jewish Health

Laurie Goodrich, DVM, MS, PhD
Associate Professor, College of Veterinary Medicine
Colorado State University

Lia Gore, MD
Professor, Pediatrics, Hematology/Oncology and Bone Marrow Transplantation
Ergen Family Endowed Chair in Pediatric Oncology
CU Anschutz

Ankush Gosain, MD, PhD*
Professor & Endowed Chair, Surgery
CU Anschutz

Samuel Gubbels, MD
Associate Professor, Otolaryngology
CU Anschutz

James Hagman, PhD
Professor, Immunology and Microbiology
CU Anschutz
Immunology and Genomic Medicine Program in Molecular Biology
National Jewish Health

Kirk Hansen, PhD
Associate Professor, Biochemistry and Molecular Genetics
CU Anschutz

Paco Herson, PhD
Associate Professor, Anesthesiology, Neuronal Injury Program
CU Anschutz

Jay Hesselberth, PhD
Associate Professor, Biochemistry and Molecular Genetics
CU Anschutz

Christene Huang, PhD
Professor, Surgery
Division of Plastic & Reconstructive Surgery
CU Anschutz

Hua Huang, MD, PhD
Professor, Integrated Department of Immunology
National Jewish Health

Srividhya Iyer, PhD
Assistant Professor, Orthopedics
CU Anschutz

Jordan Jacobelli, PhD*
Associate Professor, Immunology & Microbiology
CU Anschutz

Nicholas Jacobson, MDes
Research Faculty
CU Anschutz
Inworks Innovation Initiative

Jeffrey Jacot, PhD
Associate Professor, Bioengineering
CU Anschutz

Sujatha Jagannathan, PhD
Assistant Professor, Biochemistry and Molecular Genetics
CU Anschutz

William Janssen, MD
Associate Professor, Division of Pulmonary, Critical Care and Sleep Medicine
National Jewish Health

Antonio Jimeno, MD, PhD
Professor, Medical Oncology
Daniel and Janet Mordecai Chair in Cancer Stem Cell Biology
CU Anschutz

Malik Kahook, MD
Professor, Ophthalmology
Slater Family Endowed Chair in Ophthalmology
CU Anschutz

Manali Kamdar, MD*
Associate Professor, Division of Blood and Marrow Transplant
CU Anschutz

Dwight Klemm, PhD
Professor, Pulmonary Sciences
CU Anschutz

Igor Kogut, PhD
Associate Professor, Dermatology
CU Anschutz

M. Eric Kohler, MD, PhD
Assistant Professor, Pediatric Hematology, Oncology, BMT and Cellular Therapy
CU Anschutz
Children's Hospital Colorado

Elizabeth Kovacs, PhD
Professor, Surgery, Immunology and Molecular Biology
CU Anschutz

Melissa Krebs, PhD
Assistant Professor, Chemical and Biological Engineering
Colorado School of Mines

T. Rajendra Kumar, PhD
Professor, Obstetrics and Gynecology
Edgar L. and Patricia M. Makowski Family Endowed Chair
CU Anschutz

Daniel LaBarbera, PhD*
Professor, Pharmacy and Pharmaceutical Sciences/Ophthalmology
CU Anschutz

Kenneth Liechty, MD
Pediatric Surgery Division Chief
Department of Surgery
University of Arizona Tuscon College of Medicine

Carlin Long, MD
Professor, Cardiology
CU Anschutz

Shi-Long Lu, MD, PhD
Professor, Otolaryngology
CU Anschutz

* New member

MEMBER DIRECTORY

Traci Lyons, PhD
Associate Professor, Medical Oncology
CU Anschutz

Chelsea Magin, PhD
Assistant Professor, Bioengineering, Pediatrics
CU Denver
CU Anschutz

Susan Majka, PhD
Professor, Medicine
National Jewish Health
CU Anschutz

Naresh Mandava, MD
Professor, Ophthalmology
Sue Anschutz-Rodgers Endowed Chair in Retinal Diseases
CU Anschutz

Joanne Masterson, PhD
Assistant Professor, Pediatrics
CU Anschutz

Xianzhong Meng, MD, PhD
Professor, Cardiology
CU Anschutz

Christian Mosimann, PhD
Associate Professor, Pediatrics, Section of Developmental Biology
The Helen and Arthur E. Johnson Chair for Cardiac Research Director
CU Anschutz

Ram Nagaraj, PhD
Professor, Ophthalmology
School of Pharmacy
CU Anschutz

Devatha Nair, PhD
Assistant Professor, School of Dental Medicine
CU Anschutz
CU Boulder

Mi-hyun Nam, PhD
Research Instructor, Ophthalmology
CU Anschutz

Corey Neu, PhD
Associate Professor, Mechanical Engineering
Donnelly Family Endowed Professor
CU Boulder

Lee Niswander, PhD
Professor, Molecular, Cellular and Developmental Biology
CU Boulder

David Norris, MD
Professor, Dermatology
CU Anschutz

Jeffrey Olson, MD
Associate Professor, Ophthalmology
CU Anschutz

Bradley Olwin, PhD
Professor, Molecular, Cellular and Developmental Biology
CU Boulder

David Ryan Ormond, MD, PhD
Assistant Professor, Neurosurgery, Translational Clinical Research
CU Anschutz
VA Medical Center

Vikas Patel, MD
Professor, Orthopedics
Endowed Chair in Orthopedic Spinal Innovation
Executive Vice Chair, Surgery
CU Anschutz
CU Denver

Karin Payne, PhD
Associate Professor, Orthopedics
CU Anschutz

Mark Petrash, PhD
Professor, Ophthalmology
School of Pharmacy and Pharmaceutical Science
CU Anschutz

Christopher Phiel, PhD
Associate Professor, Integrative Biology, Pharmacology
CU Denver

Eric Pietras, PhD
Associate Professor, Hematology
Cleo Meador and George Ryland Scott Endowed Chair in Hematology
CU Anschutz

Robert Plenter
Senior Professional Research Assistant, Division of Pulmonary Sciences
CU Anschutz

Christopher Porter, MD
Associate Professor, Pediatrics, Hematology/Oncology and Bone Marrow Transplantation
CU Anschutz
VA Medical Center

Huntington Potter, PhD
Professor, Neurology
Director, CU Alzheimer's and Cognition Center
CU Anschutz

Chaitanya Puranik, BDS, MS, MDentSci, PhD
Assistant Professor, School of Dental Medicine
Children's Hospital Colorado
CU Anschutz

Nidia Quillinan, PhD
Associate Professor, Anesthesiology
CU Anschutz

Yosef Refaeli, PhD
Associate Professor, Dermatology
CU Anschutz

Tânia, PhD
Associate Professor, Endocrinology/ Metabolism Diabetes
CU Anschutz

Mary Reyland, PhD
Professor, Craniofacial Biology, Cell Developmental Biology, Pathology
CU Anschutz

Jennifer Richer, PhD
Professor, Pathology
Dean, Graduate School
CU Anschutz

Mercedes Rincon, PhD*
Professor, Immunology Microbiology
CU Anschutz

James Roede, PhD
Associate Professor, Pharmaceutical Sciences
CU Anschutz

Dennis Roop, PhD
Professor, Dermatology
John S. Gates Endowed Chair in Stem Cell Biology
CU Anschutz

Paul Rozance, MD
Professor, Pediatrics and Integrative Physiology
CU Anschutz

Andrii Rozhok, PhD
Research Instructor, Dermatology
CU Anschutz

Holger Andreas Russ, PhD
Associate Professor, MD-Phamacology/Therapeutics
University of Florida

Hatim Sabaawy, MD, PhD*
Professor, Medical Oncology
Associate Director of Translational Research
CU Anschutz

Branden Salinas, PhD
Senior Director, Manufacturing Science and Technology
Umoja Biopharma

Carol Sartorius, PhD
Associate Professor, Pathology
CU Anschutz

Marc Schwartz, MD*
Assistant Professor, Division of Hematology, Department of Medicine
CU Anschutz

Matthew Seefeldt, PhD
Executive Director, Gates Biomanufacturing Facility
CU Anschutz

Karina Serban, MD
Assistant Professor, Division of Pulmonary and Critical Care Medicine
National Jewish Health

Kartik Shankar, PhD
Professor, Pediatrics, Section of Nutrition
CU Anschutz

Yiqun Shellman, PhD
Associate Professor, Dermatology
CU Anschutz

Daniel Sherbenou, MD, PhD
Associate Professor, Medicine
CU Anschutz

Vanessa Sherk, PhD
Instructor, Endocrinology
CU Anschutz

Kunhua Song, PhD
Associate Professor, Cardiology
CU Anschutz

Danielle Soranno, MD
Assistant Professor, Pediatrics
CU Anschutz

Balaji Sridhar, MD, PhD
Resident
CU Anschutz
CU Boulder

Kurt Stenmark, MD
Professor, Pediatrics, Critical Care
La Cache Endowed Chair in Pediatric Critical Care
CU Anschutz

An-Jey Su, PhD*
Assistant Professor, Surgery
CU Anschutz

Emily Su, MD, MSCI*
Professor, Obstetrics and Gynecology
CU Anschutz

Lori Sussel, PhD
Professor, Pediatrics, Cell and Developmental Biology
Sissel and Findlow Family Chair
Barbara Davis Center for Diabetes
CU Anschutz

Tamara Terzian, PhD
Assistant Professor, Dermatology
CU Anschutz

Douglas Thamm, VMD
Assistant Professor, Clinical Sciences
Colorado State University

Joshua Thurman, MD*
Professor, Nephrology / Immunology
CU Anschutz

Enrique Torchia, PhD
Assistant Professor, Dermatology
CU Anschutz

Ronald Vagnozzi, PhD
Assistant Professor, Cardiology
Consortium for Fibrosis Research and Translation (CFReT)
CU Anschutz

Sujatha Venkataraman, PhD*
Assistant Professor, Pediatrics
CU Anschutz

Maria Natalia Vergara, PhD
Assistant Professor, Ophthalmology
CU Anschutz

Michael Verneris, MD
Professor, Pediatrics, Hematology/Oncology and Bone Marrow Transplantation
The Barton Endowed Chair of Pediatric Bone Marrow Transplant
CU Anschutz
Children's Hospital Colorado

Rajeev Vibhakar, MD, PhD
Professor of Pediatrics, CU Anschutz
Chair, Cancer League of Colorado

Eszter Vladar, PhD
Assistant Professor, Cell and Developmental Biology, Division of Pulmonary Sciences
CU Anschutz

David Wagner, PhD
Associate Professor, Neurology
CU Anschutz

Lori Walker, PhD
Associate Professor, Cardiology
CU Anschutz

Xiao-Jing Wang, MD, PhD
Professor, Department of Pathology & Laboratory Medicine
UC Davis Health

Zhijie Wang, PhD
Assistant Professor, Mechanical Engineering
Colorado State University

Kristin Watt, PhD*
Assistant Professor, Craniofacial Biology
CU Anschutz

Gregory Way, PhD*
Assistant Professor, Biomedical Informatics
CU Anschutz

Mary Weiser-Evans, PhD
Professor, Medicine, Renal Diseases and Hypertension
CU Anschutz

Alicia White, MD*
Assistant Professor, Pediatrics/Neonatology
CU Anschutz

Trevor Williams, PhD
Professor, Craniofacial Biology, Cell and Developmental Biology
Timpte/Brownie Endowed Chair in Craniofacial/Molecular Biology
School of Dental Medicine
CU Anschutz

Carol Wilusz, PhD
Professor, Microbiology, Immunology and Pathology
Colorado State University

Jeff Wilusz, PhD
Professor, Microbiology, Immunology and Pathology
Colorado State University

Rui Yi, PhD
Associate Professor, Molecular, Cellular and Developmental Biology
CU Boulder

Martin Zamora, MD
Professor, Division of Pulmonary, Critical Care Medicine
CU Anschutz

Michael Zaretsky, MD
Associate Professor, Obstetrics and Gynecology, Maternal Fetal Medicine
CU Anschutz
Children's Hospital Colorado

Wenbo Zhou, PhD
Associate Professor, Clinical Pharmacology and Toxicology
CU Anschutz

Michael Zuscik, PhD
Professor and Vice Chair of Research, Orthopedics
Mack L. Clayton Endowed Chair in Orthopaedics
CU Anschutz

* New member

ACKNOWLEDGMENTS

FINANCIAL OVERVIEW

Measuring Our Impact

By Joleen Bohnen

Reflecting its commitment to accelerate advanced therapies to patients at the point of care, the Gates Institute has invested over $20 million in fiscal year 2024 to establish a comprehensive cell therapy infrastructure at the CU Anschutz Medical Campus. Our investments this year span advanced therapy programs, recruitment of top talent, and early-stage translational research funding.

Programs in Advanced Therapy
Gates Institute has invested and onboarded new platforms and capabilities:

- A tumor-infiltrating lymphocytes (TILs) program, led by Eduardo Davila, PhD, will treat patients with solid tumors and expand Gates Institute capabilities with a novel immune effector cell. Anticipated patient impact: 2025. (Related article on page 10)
- Programs involving induced pluripotent stem cells (iPSCs) led by Valeria Canto-Soler, PhD, and Dennis Roop, PhD, will treat patients with macular degeneration (related article on page 10) and epidermolysis bullosa, respectively. The technology will expand Gates Institute capabilities with a novel therapeutic platform and master cell bank. Anticipated patient impact: 2025-2028.
- A chimeric antigen receptor (CAR) T-cell program, developed by Eric Kohler, MD, PhD, building upon work by Craig Jordan, MD, and his team, will treat patients with acute myeloid leukemia (AML). This will leverage the expertise that has been gained with prior CAR T-cell programs to accelerate a new therapy option for patients with an aggressive and often difficult-to-treat cancer. Anticipated patient impact: 2025.
- Gates Biomanufacturing Facility (GBF) continues to support clinical trials with two CAR molecules and partner with industry to build additional capabilities.

Recruitment of Top Talent
We have invested in the recruitment of research and clinical faculty in advanced therapies, which is crucial for the development, advancement, and delivery of a highly specialized field. Therefore, the Gates Institute has partnered with several departments, such as pediatrics and hematology, to strategically hire and attract top talent in cell and gene therapy.

Early-Stage Discoveries
We continue to invest in early-stage discoveries to advance therapies. In December 2023, four talented faculty members were provided Grubstake Awards totaling nearly $1.5 million. Read our coverage on pages 4-5 for more details.

The Grubstake Fund honors the pioneering spirit of our namesake, Charles C. Gates, and exemplifies our commitment to advancing discovery in the cell and gene therapy space, with an emphasis on research with the potential to have the greatest patient impact.

Note: Joleen Bohnen, MBA, is the director of finance and administration at Gates Institute, where she oversees the finance and HR functions of the organization.

DONORS

We gratefully acknowledge the following individuals, foundations and organizations for supporting our research and mission during 2023.

- Anonymous
- Christine & Mohammed Attar
- Kristin Baird, MD, & Terry Fry, MD
- Jeffrey Anne & Randall Bellows
- Janelle & Buck Blessing
- Lauren & Marc Bonaca, MD
- Laura Borgelt, PharmD, & Brendan Lundy
- Balbi A. Brooks
- Ruchi & Mark Brunvand, MD
- Kathy & Michael Chevalier
- Children's Hospital Colorado
- Beverly & Dennis Christine
- Marilyn & Pete Coors
- Phyllis M. Coors Foundation/ Scott Coors
- Jill Cowperthwaite & Charles Jones
- Paula & Jack Crowley
- Melissa Crowley
- Marguerite & Thomas Detmer, Jr.
- The Dudley Family
- Cathey & Dick Finlon
- Yvette & Christopher Frampton
- Timothy Nelson Gardner Trust
- Gates Frontiers Fund
- Jill & Roger Giller, MD
- Dana Gordon
- Glendorn Foundation
- Liza Grant
- Rhondda Grant
- The Berenice Gates Hopper Family Fund
- Angie & Clay Howell
- Joyce & Wayne Hutchens
- Cynthia Koerner
- The Charlie Kurtz Family
- Mary Lanius
- Penelope Lewis
- LGA Family Foundation
- Gretchen Lobitz, PhD, & Charles Lobitz
- Sarah McGregor & Peter Naseth
- The New L Family Fund
- Dennis O'Hanlon
- ABN Ohana
- Kelly & Mark Petrash, PhD
- The Poha Fund
- Doug Pooley Lifetime Trust
- Gregg Pooley Lifetime Trust
- Deborah & Jeff Reamer
- Jane Rech, PhD, & Chris Toll
- Ann & Kevin Reidy
- Betty & Dennis Roop, PhD
- Kristine Rosen
- Legacy III Fund
- Leslie Schaeffer
- Annalee & Wagner Schorr, MD
- Sheffield Tulp
- The Solich Fund
- Ann Sperling
- The Tuesday Morning Class
- UCHealth
- University of Colorado Anschutz Medical Campus, Office of the Chancellor
- University of Colorado Anschutz Medical Campus, School of Medicine
- Diane & Marshall Wallach
- Emily Warshauer, PhD
- Marie & Daniel Welch

THE YEAR IN PHOTOS

CHARLIE'S PICNIC

Chancellor Don Elliman, Antonio Jimeno, Dean John Reilly, and Vice Chancellor for Research, Tom Flaig.

Gates Summer Internship Program (GSIP) alums surround GSIP founding donor Rhondda Grant (center). Clockwise, from bottom left: Noah Rainer ('21); Madelyn Jaeger ('22); Brett Li ('22); Vrushali Patel ('22); and Kanita Hrustanovic ('23).

From left: Jordan Krause, Chandresh Undhad, Terri Foote, Chanel Mansfield, Nidhi Kotecha and Tiffany Azamar.

From left: Doreen Molk, Laura Borgelt and Michael McGarry.

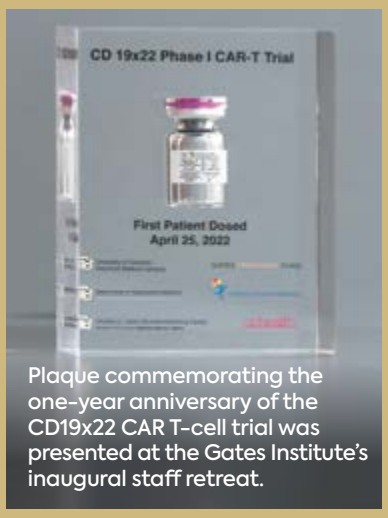
Diane Gates Wallach and a crowd of Gates Institute board, donors, members, friends and staff toast Charlie Gates at this annual celebration. **Scan the QR Code for more images from Charlie's Picnic.**

Jill Cowperthwaite and Debbie Lopez from the community engagement team challenged attendees at the CU Anschutz Block Party to a trivia contest.

CONFERENCES & MEETINGS

Anschutz Block Party attendees awarded swag after competing in gowning contest.

GBF Executive Director Matt Seefeldt traveling between conferences.



Plaque commemorating the one-year anniversary of the CD19x22 CAR T-cell trial was presented at the Gates Institute's inaugural staff retreat.

Toni Lapp, Cheri Adams, and Drew Roth representing Gates Institute at the Science Writers 2023 conference.

AT THE GATES BIOMANUFACTURING FACILITY

Matt Seefeldt leading U.S. Senator John Hickenlooper on a tour of the GBF.

Deb Froeb, wife of the late Tim Gardner, reacting to the dedication of the Tim Gardner Conference Room at the GBF.

ACKNOWLEDGMENTS

GATES INSTITUTE ADVISORY BOARD

- Janelle Blessing
- Marc Bonaca
- Don Elliman
- Yvette Pita Frampton
- Cathey Finlon
- Terry Fry, MD
- Tom Gronow
- Kevin Reidy
- John Reilly, MD
- Dennis Roop, PhD
- Wagner Schorr, MD
- Duffy Solich
- Ann Sperling, MBA
- Rick Stoddard, JD
- Diane Gates Wallach, MBA
- Dan Welch

FRONT ROW from left: Kevin Reidy, John Reilly, Wagner Schorr, Diane Gates Wallach, Ann Sperling, Janelle Blessing

BACK ROW from left: Don Elliman, Yvette Pita Frampton, Dan Welch, Cathey Finlon, Rick Stoddard, Terry Fry, Dennis Roop

NOT PICTURED: Marc Bonaca, Tom Gronow, Duffy Solich

OUR TEAM

CONTACTS

Gates Institute
Administrative office
1890 N. Revere Court
Aurora, CO 80045
303.724.1494
gates.cuanschutz.edu

Gates Biomanufacturing Facility (GBF)
12635 E. Montview Blvd.
Aurora, CO 80045

For Inquiries About the GBF
Please visit **www.gatesbiomanufacturing.com**, send email to **matthew.seefeldt@cuanschutz.edu**, or call **303.808.8436.**

For Giving Opportunities
Contact **Allison Krebs,** Office of Advancement, at **303.724.5704** or email **Allison.krebs@cuanschutz.edu**.

Gates Institute

UNIVERSITY OF COLORADO
ANSCHUTZ MEDICAL CAMPUS

1890 N. Revere Court
Aurora, CO 80045
303.724.1494

EXPANDING OUR PATIENT IMPACT

GATES INSTITUTE
2023 ANNUAL REPORT