



Gates Center for Regenerative Medicine

UNIVERSITY OF COLORADO **ANSCHUTZ MEDICAL CAMPUS**

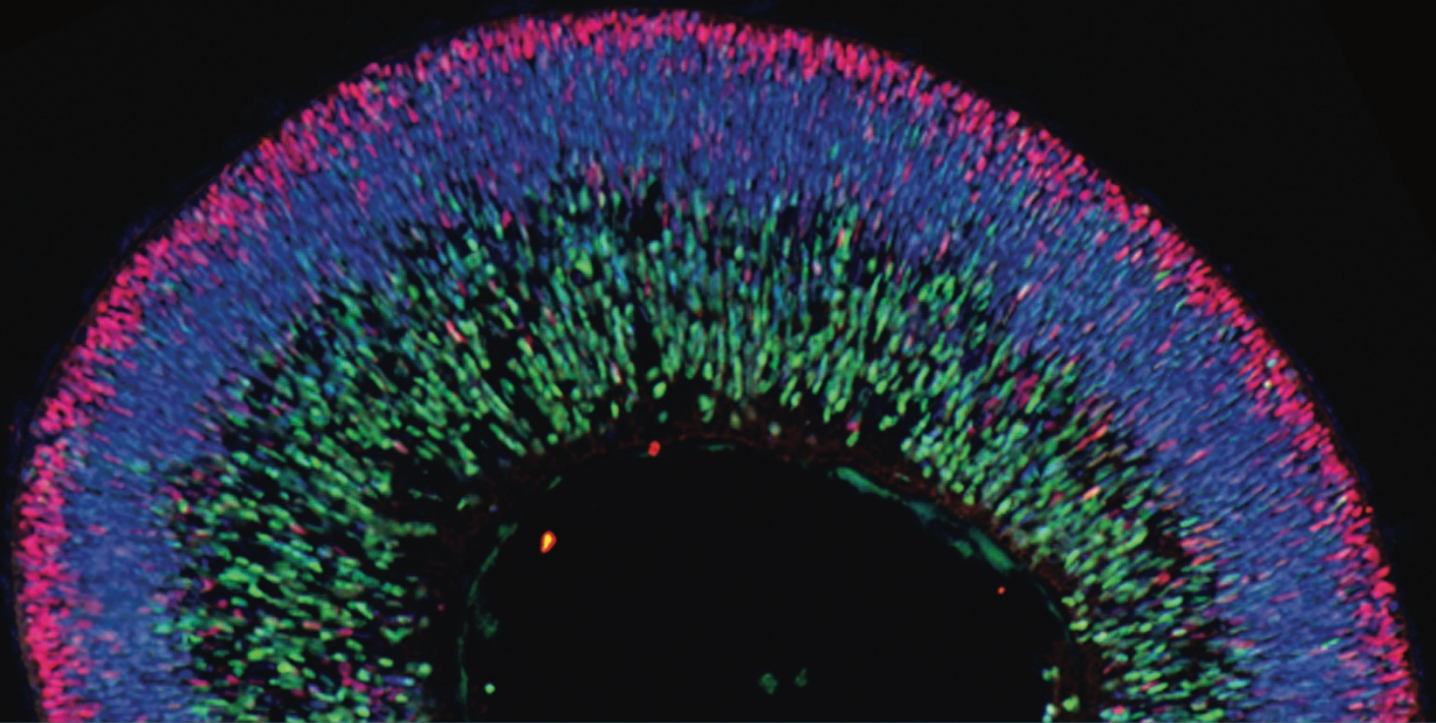


# 2016

## *Annual Report*

The Gates Center





***Generating light-sensitive human retinas in a dish*** (Courtesy of Valeria Canto-Soler, Ph.D., director of the Ocular Stem Cell and Regeneration Program (CellSight) effective July 1, 2017): Human induced pluripotent stem cells recapitulate in a laboratory dish the developmental steps that lead to the formation of the retina in the human embryo, culminating with the generation of 3D retinal tissue. These “3D mini retinas” recreate the cellular composition of the native human retina, including ganglion cells (green) and photoreceptors (red) with the capacity to sense light. Human 3D mini retinas are being used to develop novel treatments for retinal degenerative diseases such as age-related macular degeneration and retinitis pigmentosa.







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Charles C. Gates  
May 27, 1921, to August 28, 2005



# THE VISION



When wagon trains headed west and came to a challenging creek, pioneers threw their hats to the other side. Since no one in their right mind would leave a hat behind, throwing it across committed them to ford the creek, retrieve it and carry on.

To entrepreneur and philanthropist Charles C. Gates, throwing the hat symbolized a commitment to take the risks necessary to keep moving toward a goal.

Toward the end of his life, he foresaw the future in regenerative medicine and exhorted his children, Diane Gates Wallach and John Gates, and others to bring it to fruition. We are grateful for the legacy of his vision that lives on at the Gates Center for Regenerative Medicine and within its Charles C. Gates Biomanufacturing Facility, which is dedicated to enabling the safe and expedited translation of discovery into human therapies for people worldwide.





# WHO WE ARE

Established in 2006 with a generous gift in Charles Gates' memory, the Gates Center for Regenerative Medicine is a world-class center headquartered on the University of Colorado Anschutz Medical Campus, the only comprehensive academic health sciences center in Colorado, the largest academic health center in the Rocky Mountain region and one of the newest education, research and patient care facilities in the world.

Home to 21,500 employees, more than 4,400 degree-seeking students and two nationally recognized hospitals that treat 1.7 million patients each year, CU Anschutz trains the health sciences workforce of the future and fuels the economy.

Operating within this interconnected campus, the Gates Center brings together its medical research and clinician members to utilize the center's state-of-the-art laboratory facilities and technology. Members also benefit from the collaboration the center fosters and the services it provides in the areas of marketing and philanthropy, education and outreach, and commercialization support designed to accelerate discoveries into clinical practice as quickly as possible.

From the beginning, the major focus of the Gates Center has been adult stem cells. Recent research has proven that adult stem cells, also referred to as differentiated stem cells, can be reprogrammed into embryonic-like stem cells, referred to as induced Pluripotent Stem Cells (iPS cells), and then differentiated into virtually any cell type in the body. For example, Gates Center researchers have biopsied skin cells from individual patients, reprogrammed those cells into iPS cells and corrected their underlying genetic defect.

The long-term goal is to return genetically corrected iPS cell-derived adult stem cells to the patient from whom they were derived. In this way, the Gates Center's clinical pathway is leading toward a new paradigm of personalized medicine in

which an individual's own cells can be used to cure a number of diseases and conditions.

Professor Dennis Roop, Ph.D., was recruited from the Baylor College of Medicine in January 2007 to lead the Gates Center by establishing a critical mass of faculty, clinicians, students, research staff and administrators to execute its mission. The Gates Center is a multi-institutional center currently comprised of 92 members from the University of Colorado Anschutz Medical Campus and CU Boulder, Colorado State University, Colorado School of Mines and private industry.

In addition to being multi-institutional, the Gates Center is also multidisciplinary, with members investigating regenerative therapies and stem cell treatments in the areas of cardiology, dermatology, endocrinology, immunology, neonatology, neurology, oncology, ophthalmology, orthopedics, otolaryngology, pathology and pulmonology, among others.

Therapies under development include immunotherapies for cancer and viruses, stem cell therapies for inherited skin diseases, macular degeneration, Parkinson's disease and cardiovascular disease, and stem cell therapies to repair bone and cartilage.

The Gates Center for Regenerative Medicine is helping bolster the Anschutz Medical Campus's growing reputation as a global medical destination. Among other efforts, the Gates Center launched its fourth core facility—the Gates Biomanufacturing Facility—in spring 2015, in which future cellular therapies and protein-based biologics are being manufactured for human trials under the highest FDA standards.

The Gates Biomanufacturing Facility is one of six combined cell therapy and protein manufacturing facilities in the United States and the only one of its kind within an 800-mile radius.





**2016 GATES  
CENTER  
ADVISORY  
BOARD**

Diane Gates Wallach, Co-Chair  
Daniel Ritchie, Co-Chair  
Donald Elliman, Co-Chair  
Dori Biester, Ph.D., FAAN

Janelle Blessing  
Marilyn Coors, Ph.D.  
Yvette Pita Frampton  
William Hiatt, M.D.

Wayne Hutchens  
Kevin Reidy  
Dennis Roop, Ph.D.  
Wagner Schorr, M.D.

Geoff Solich  
Ann Sperling  
Rick Stoddard



# WHAT WE DO

## MISSION

We conduct leading-edge research in stem cell biology and regenerative medicine, accelerating discoveries from the lab through clinical trials leading to effective cures and therapies for patients.

RECRUIT &  
RETAIN  
TOP TALENT

SECURE  
FUNDING

SUPPORT  
RESEARCH  
& INNOVATION

FACILITATE  
CLINICAL  
TRIALS

BENEFIT  
PATIENTS

## GATES CENTER

92 MEDICAL RESEARCH AND CLINICIAN MEMBERS



University of Colorado  
Anschutz Medical Campus



Boulder

uchealth



Children's Hospital Colorado



Colorado  
State  
University



PRIVATE INDUSTRY

## PROVIDE ACCESS TO GATES CENTER'S STATE-OF-THE-ART LABORATORY FACILITIES:

GATES  
BIOMANUFACTURING  
FACILITY

FLOW  
CYTOMETRY  
CORE

MORPHOLOGY  
AND PHENOTYPING  
CORE

BIOENGINEERING  
CORE

## SUPPORT MEMBERS THROUGH THE GATES CENTER INFRASTRUCTURE:

MARKETING AND PHILANTHROPY

EDUCATION AND OUTREACH

COMMERCIALIZATION SUPPORT

# LIST OF MEMBERS

NAME	DEGREE(S)	POSITION	ACADEMIC INSTITUTION/AFFILIATES	PRIMARY (AND SECONDARY) APPOINTMENT(S)
Appel, Bruce	PhD	Professor Diane G. Wallach Chair in Pediatric Stem Cell Biology	University of Colorado Denver Anschutz Medical Campus	Department of Pediatrics/ Developmental Biology
Artinger, Kristin	Ph.D.	Associate Professor	University of Colorado Denver Anschutz Medical Campus	Department of Craniofacial Biology, School of Dental Medicine
Ayers, Reed	Ph.D.	Professor	Colorado School of Mines	Metallurgical and Material Engineering
Bailey, Susan	Ph.D.	Professor	Colorado State University	Department of Environmental and Radiological Health Sciences
Baker, Christopher	M.D.	Associate Professor	University of Colorado Denver Anschutz Medical Campus	Department of Pediatrics/ Developmental Biology
Bamburg, James	Ph.D.	Professor	Colorado State University	Department of Biochemistry and Molecular Biology
Barlow, Linda	Ph.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Cell and Developmental Biology
Bilousova, Ganna	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Dermatology
Birlea, Stanca	M.D., Ph.D.	Associate Professor	University of Colorado Denver Anschutz Medical Campus	Department of Dermatology
Box, Neil	Ph.D.	Associate Professor	University of Colorado Denver Anschutz Medical Campus	Department of Dermatology
Boyle, Kristen	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Pediatrics
Bruckner, Anna	M.D.	Associate Professor	University of Colorado Denver Anschutz Medical Campus	Department of Dermatology
Brzezinski, Joseph	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Ophthalmology
Burnham, Ellen	M.D., M.S.	Associate Professor	University of Colorado Denver Anschutz Medical Campus	Department of Medicine/ Pulmonary Sciences and Critical Care
Chan, Sanny	M.D., Ph.D.	Assistant Professor	National Jewish Health	Department of Pediatrics/ Division of Allergy and Immunology
Chick, Wallace	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Cell and Developmental Biology
De Langhe, Stijn	Ph.D.	Associate Professor	National Jewish Health	Department of Pediatrics
DeGregori, James	Ph.D.	Professor Courtenay C. and Lucy Patten Davis Endowed Chair in Lung Cancer Research	University of Colorado Denver Anschutz Medical Campus	Department of Biochemistry and Molecular Genetics
Dempsey, Peter	Ph.D.	Associate Professor	University of Colorado Denver Anschutz Medical Campus	Department of Pediatrics
Dow, Steven	DVM, Ph.D.	Professor	Colorado State University	Department of Clinical Sciences
Ehrhart, Nicole	VMD, Diplomate, ACVS	Professor	Colorado State University	Department of Surgical Oncology
Ford, Heide	Ph.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Pharmacology
Franco, Santos	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Pediatrics/ Developmental Biology
Freed, Brian	Ph.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Medicine/ Allergy and Clinical Immunology
Freed, Curt	M.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Medicine/ Clinical Pharmacology and Toxicology
Friedman, Jed	Ph.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Pediatrics
Fujita, Mayumi	M.D., Ph.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Dermatology
Goodrich, Laurie	DVM, M.S., Ph.D.	Associate Professor	Colorado State University	College of Veterinary Medicine
Gore, Lia	M.D.	Professor The Robert J. and Kathleen A. Clark and Ergen Family Endowed Chairs	University of Colorado Denver Anschutz Medical Campus	Department of Pediatrics/Hematology, Oncology and Bone Marrow Transplantation

\* New members in 2016



NAME	DEGREE(S)	POSITION	ACADEMIC INSTITUTION/AFFILIATES	PRIMARY (AND SECONDARY) APPOINTMENT(S)
Gubbels, Samuel*	M.D.	Associate Professor	University of Colorado Denver Anschutz Medical Campus	Department of Otolaryngology
Hagman, James	Ph.D.	Professor	National Jewish Medical Research Center	Department of Immunology
Herson, Paco*	Ph.D.	Principal Investigator	University of Colorado Denver Anschutz Medical Campus	Department of Anesthesiology, Neuronal Injury Program
Hiatt, William	M.D.	Professor Novartis Endowed Chair in Cardiovascular Research	University of Colorado Denver Anschutz Medical Campus	Department of Medicine/Cardiology
Huang, Hua	M.D., Ph.D.	Professor	National Jewish Medical Research Center	Integrated Department of Immunology
Jacot, Jeffrey*	Ph.D.	Associate Professor	University of Colorado Denver Anschutz Medical Campus	Department of Bioengineering
Janssen, William	M.D.	Associate Professor	National Jewish Medical Research Center	Division of Pulmonary, Critical Care and Sleep Medicine
Jimeno, Antonio	M.D., Ph.D.	Professor Daniel and Janet Mordecai Chair in Cancer Stem Cell Biology	University of Colorado Denver Anschutz Medical Campus	Department of Medicine/Medical Oncology
Kahook, Malik	M.D.	Professor Slater Family Endowed Chair in Ophthalmology	University of Colorado Denver Anschutz Medical Campus	Department of Ophthalmology
King, Karen	Ph.D.	Associate Professor	University of Colorado Denver Anschutz Medical Campus	Department of Orthopedics
Klemm, Dwight	Ph.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Medicine/Pulmonary Sciences
Koch, Peter	Ph.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Dermatology
Kogut, Igor	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Dermatology
Königshoff, Melanie	M.D., Ph.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Division of Pulmonary, Critical Care Medicine
Koster, Maranke	Ph.D.	Associate Professor	University of Colorado Denver Anschutz Medical Campus	Department of Dermatology
Krebs, Melissa	Ph.D.	Assistant Professor	Colorado School of Mines	Chemical and Biological Engineering
Kumar, Raj*	Ph.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Obstetrics and Gynecology
Lee, Katherine	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Pediatrics/Infectious Disease
Liechty, Kenneth	M.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Surgery/Pediatric Surgery
Long, Carlin	M.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Medicine/Cardiology
Lu, Shi-Long	M.D., Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Otolaryngology
Lyons, Traci	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Medicine/Medical Oncology
Magin, Chelsea	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Division of Pulmonary/Department of Medicine
Malkoski, Stephen	M.D., Ph.D.	Associate Professor	University of Colorado Denver Anschutz Medical Campus	Department of Medicine/ Pulmonary Sciences and Critical Care
Mandava, Naresh	M.D.	Professor Sue Anschutz-Rodgers Endowed Chair in Retinal Diseases	University of Colorado Denver Anschutz Medical Campus	Department of Ophthalmology
Masterson, Joanne	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Pediatrics
McCurdy, Carrie	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Pediatrics/Neonatology
Meng, Xianzhong	M.D., Ph.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Medicine/Cardiology
Niswander, Lee	Ph.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Pediatrics/ Developmental Biology
Norris, David	M.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Dermatology
Olwin, Bradley	Ph.D.	Professor	University of Colorado Boulder	Department of Molecular, Cellular and Developmental Biology



NAME	DEGREE(S)	POSITION	ACADEMIC INSTITUTION/AFFILIATES	PRIMARY (AND SECONDARY) APPOINTMENT(S)
Ormond, David Ryan	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Neurosurgery/ Translational Clinical Research
Patel, Vikas	M.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Orthopedics
Payne, Karin	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Orthopedics
Payne, Thomas	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Dermatology
Perraud, Anne-Laure	Ph.D.	Assistant Professor	National Jewish Health, University of Colorado Denver Anschutz Medical Campus	Department of Biomedical Research/ Immunology and Microbiology
Phiel, Christopher	Ph.D.	Assistant Professor	University of Colorado Denver	Department of Integrative Biology
Pietras, Eric	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Medicine/Hematology
Petrash, Mark, J.	Ph.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Ophthalmology
Porter, Christopher	M.D.	Associate Professor	University of Colorado Denver Anschutz Medical Campus	Department of Pediatrics/Hematology, Oncology and Bone Marrow Transplantation
Purev, Enkhee*	M.D., Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Division of Hematology
Refaeli, Yosef	Ph.D.	Associate Professor	University of Colorado Denver Anschutz Medical Campus	Department of Dermatology
Ribera, Angeles	Ph.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Physiology and Biophysics
Roop, Dennis	Ph.D.	Professor Charles C. Gates Endowed Chair in Regenerative Medicine and Stem Cell Biology	University of Colorado Denver Anschutz Medical Campus	Department of Dermatology
Russ, Holger*	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Barbara Davis Center for Childhood Diabetes
Sartorius, Carol	Ph.D.	Associate Professor	University of Colorado Denver Anschutz Medical Campus	Department of Pathology
Sclafani, Robert	Ph.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Biochemistry and Molecular Genetics
Sherk, Vanessa	Ph.D.	Instructor	University of Colorado Denver Anschutz Medical Campus	Department of Medicine/Endocrinology
Song, Kunhua	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Medicine/Cardiology
Soranno, Danielle	M.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Pediatrics
Stenmark, Kurt	M.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Pediatrics/Critical Care
Terzian, Tamara	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Dermatology
Thamm, Douglas	VMD	Assistant Professor	Colorado State University	Department of Clinical Sciences
Torchia, Enrique	Ph.D.	Assistant Professor	University of Colorado Denver Anschutz Medical Campus	Department of Dermatology
Wagner, David	Ph.D.	Associate Professor	University of Colorado Denver Anschutz Medical Campus	Department of Neurology
Walker, Lori	Ph.D.	Associate Professor	University of Colorado Denver Anschutz Medical Campus	Department of Medicine/Cardiology
Wang, Xiao-Jing	M.D., Ph.D.	Professor John S. Gates Endowed Chair in Stem Cell Biology	University of Colorado Denver Anschutz Medical Campus	Department of Pathology
Wang, Zhijie	Ph.D.	Assistant Professor	Colorado State University	Department of Mechanical Engineering
Williams, Trevor	Ph.D.	Professor	University of Colorado Denver Anschutz Medical Campus	Department of Craniofacial Biology and Cell and Structural Biology, School of Dental Medicine
Wilusz, Carol	Ph.D.	Professor	Colorado State University	Department of Microbiology, Immunology and Pathology
Wilusz, Jeff	Ph.D.	Professor	Colorado State University	Department of Microbiology, Immunology and Pathology
Yi, Rui	Ph.D.	Associate Professor	University of Colorado Boulder	Department of Molecular, Cellular and Developmental Biology
Zhou, Wenbo	Ph.D.	Associate Professor	University of Colorado Denver Anschutz Medical Campus	Department of Medicine/ Clinical Pharmacology and Toxicology

\* New members in 2016





# Gates Center for Regenerative Medicine

UNIVERSITY OF COLORADO **ANSCHUTZ MEDICAL CAMPUS**



The end of 2016 marked the tenth anniversary of my arrival on campus to lead the Gates Center in January 2007. It also marked the conclusion of an extraordinary year in which our collaborations and the Gates Biomanufacturing Facility helped us make outstanding progress. As director of the Gates Center, I am proud to share some of the year's highlights.

In January 2016, the Gates Center convened a group of 30 top-tier volunteers to work on our 2020 strategic plan. Committees populated by institutional leadership from the University of Colorado Anschutz Medical Campus, UCHealth, Children's Hospital Colorado, the University of Colorado Foundation, the Gates Center Advisory Board, clinicians, faculty and members of the community met regularly throughout the spring and subsequently made recommendations to the Gates Advisory Board in the areas of recruitment, campus alignment, messaging, patient outcomes and funding.

Dori Biester, Gates Center Advisory Board member and former president and CEO of Children's Hospital Colorado, commented so aptly last spring, "The engagement and enthusiasm shown by this group, and their serious consideration of issues that could be addressed with regard to the Gates Center, was a true gift." These superb volunteers and others who have generously shared their time, treasure and talent are listed at the end of this report.

Key to the Gates Center's mission—accelerating discoveries from lab to clinical trials to effective therapies and cures for

patients—is the acquisition and retention of accomplished, passionate and innovative change agents that include both clinicians and basic scientists. In 2016 the campus recruited several world-class individuals who hope to work in collaboration with the Gates Center and the Gates Biomanufacturing Facility to advance their discoveries into the clinic and to patients.

Among these individuals is Valeria Canto-Soler, Ph.D., whose recruitment to lead the Ocular Stem Cell and Regeneration Program (CellSight) effective July 1, 2017, is due to a successful partnership between the Department of Ophthalmology and the Gates Center, and a number of benefactors passionate about the promise of stem cells for patients battling age-related macular degeneration. Dr. Canto-Soler and other new recruits working in the area of regenerative medicine are highlighted in the Recruiting Top Talent section on page 14. We are delighted to welcome them to campus.

The University of Colorado, Stanford University and Columbia University also came together in 2016 to create a privately funded, unique research consortium to fight the rare and debilitating genetic skin blistering disease epidermolysis bullosa.

Throughout the year, Gates Center members published and received grant awards; commercialization efforts progressed as 2015 Gates Grubstake Fund awardees advanced their research and the selection of three new Grubstake awardees neared completion. Our three core facilities continued to serve the campus, and the Gates Biomanufacturing Facility dramatically ramped up both its number of employees and business.

As always we worked hard to inspire the next generation through our educational and outreach initiatives, and to promote the inclusion of regenerative medicine in the 21st Century Cures Act that was signed into law in December. Notably, we also hosted our second summer of the Gates Summer Internship Program. Thanks to a number of



impassioned donors, 18 exceptional students from across the country spent 11 weeks working in Gates Center members' labs and our final day featured the students' projects and guest speaker CBS medical correspondent Max Gomez, Ph.D. Among the accolades the program received, one student described it as "a fantastic program," and added, "One of the best decisions I've made in college was to join."

Above all, we are grateful for the many people who believe in Charles Gates' vision of a future in regenerative medicine and who are working to achieve it. This includes our members, our staff, the Gates Center Advisory Board (which added Yvette Pita Frampton and Geoff "Duffy" Solich to its midst in 2016), our campus leadership and colleagues, and our donors, volunteers and friends. It was our great pleasure to host the first annual "Charlie's Picnic" in August to thank them.

As I look back over the first ten years of the Gates Center and my tenure on the Anschutz Medical Campus, I must thank Diane Gates Wallach, whom we were so proud to nominate for an honorary degree in recognition of her unsurpassed commitment to the Gates Center and the Anschutz Medical Campus. Her father liked to say "no one does their best work alone." I want her to know that we could never have come so far without Charlie's vision and her unwavering commitment and support for our cause.

Sincerely,



Dennis R. Roop, Ph.D.

Director, Gates Center for Regenerative Medicine  
Charles C. Gates Endowed Chair in Regenerative Medicine  
and Stem Cell Biology

May 28, 2016

*Dennis and the entire Gates Center team:*

*I want to thank you for your kind nomination of me for an honorary doctorate. It was a surprise and quite an honor, and I don't believe I ever expressed my thanks.*

*Since I chose the latest possible date to receive the degree—yesterday—almost two years have passed. Time does fly, but it has been a pleasure and inspiration to see how far the center and collaboration across campus have come, even in the last year alone. You are an amazing group!*

*The future is bright and there is no rest for the weary. All ahead full as we start delivering real outcomes for patients and building on what is now a very solid foundation from the bench to bedside.*

*Thank you for all your positive energy and hard work. Charlie is definitely happy, looking down from above. Interestingly, yesterday was his birthday.*

*Best,  
Diane*



On May 27, 2016, CU President Bruce Benson awarded Diane Gates Wallach the Honorary Doctor of Humane Letters degree in recognition of her unsurpassed commitment to the Gates Center and the Anschutz Medical Campus.



# RECRUITING TOP TALENT

One of the absolute highlights of 2016 was welcoming extraordinary recruits working in the area of regenerative medicine to the Anschutz Medical Campus. We asked them to expound upon their recruitment and to share their hopes for the future.

“

*The Gates Center and the Gates Biomanufacturing Facility have become an indispensable tool for the recruitment of top talent to both Children's Hospital Colorado and the greater Anschutz Medical Campus. Time after time, physicians and researchers have said, 'I can't believe you have this resource on campus.'*

*—Rick Stoddard, chair of the Children's Hospital Colorado Foundation Board of Trustees and member of the Gates Center Advisory Board, Children's Hospital Colorado Board and the Health System Board*

”



## **Valeria Canto-Soler, Ph.D.**

*In 2014, Valeria Canto-Soler, Ph.D., of Johns Hopkins University transformed the scientific community when she published evidence*

*showing how to induce stem cells in the laboratory to organize themselves into mini retinas while growing in a petri dish. Following an international recruitment, she was selected as director of the Ocular Stem Cell and Regeneration Program (CellSight), effective July 1, 2017, and will hold the Doni Solich Family Endowed Chair in Ocular Stem Cell Research.*

*CellSight is a partnership between the Department of Ophthalmology and the Gates Center, established thanks to benefactors passionate about the promise of stem cells for patients battling age-related macular degeneration.*

*An ambitious \$10 million initiative to set up the ocular stem cell and regeneration program and recruit its director began with a generous \$5 million challenge grant from the Gates*

*Frontiers Fund and quickly gathered momentum among friends and benefactors passionate about the future of eye care. Additional support from the Solich Fund, the Glendorn Foundation, LGA Foundation, members of the Gates family and others will fuel research into the promise of stem cells for patients with age-related macular degeneration—the leading cause of blindness among Americans age 50 and older.*

*“When I first visited the Anschutz Medical Campus, I was struck by the vision, the commitment, and the vibrant and collegial environment I found. I would even venture to say that I felt I could almost physically touch these qualities. I felt immersed in them from the first moment I visited the Department of Ophthalmology, and that perception kept increasing as I interacted with members of the Gates Center, faculty from different departments and the university leadership.*

*“Long before this opportunity came my way, I had envisioned a research program focused on developing stem cell-based technologies for treating blindness, but I could not see how it could become a reality. Here I found a leadership team that shares my vision, along with the drive and the commitment necessary to bring it to life.*



"As importantly, I also found a unique commitment from private and philanthropic foundations that I have never encountered before. All this gives you that special strength that comes from knowing you are not alone in the fight, and the conviction that together we can make it happen.

"The Gates Center definitely played a pivotal role in my recruitment process. The evolution of the Gates Center since its launch in 2006 to its current state, and the actual establishment of the Gates Biomanufacturing Facility, represented to me the materialization of that vision, drive and commitment I had sensed. In addition to this, it meant that a key element of the infrastructure needed for delivering the therapies we expect to develop at CellSight to patients was already in place.

"Our work will entail a joint effort between the clinicians at the Department of Ophthalmology, the researchers at

CellSight and the Gates Center. Clinicians will play a key role in the diagnosis and management of diseases, as well as in the creation of patient registries including cell banking.

"The cells obtained from the patients, such as skin or blood cells, will then be reprogrammed into stem cells at the Gates Center. At CellSight we will use these patient-derived stem cells to look for new drugs that could prevent and treat different eye diseases, and to develop stem cell-based therapies involving cell transplantation.

"These novel pharmacological and cellular therapeutic products will then be manufactured according to clinical grade standards at the Gates Biomanufacturing Facility and taken back to the clinic to treat the patients.

"I dream of the day when all this becomes a reality!"



**Oliver Eickelberg,  
M.D., FERS**

*In December 2016, Oliver Eickelberg, M.D., FERS, became the new head of the Division of Pulmonary and Critical Care Medicine*

*at the University of Colorado School of Medicine.*

*Originally from Germany, Dr. Eickelberg has always had a reputation as an innovator, earning numerous accolades in respiratory medicine and rising to the top to lead several institutions. He chose medicine over the family business of engineering and studied in Switzerland, Austria and the United States of America, which provided him with a varied perspective of medical training. Dr. Eickelberg later returned to Germany to create The Comprehensive Pneumology Center (CPC) in order to develop the next generation of scientists.*

"I was specifically attracted to the outstanding talent in the Division of Respiratory Sciences and Critical Care Medicine (PSCCM) in the Department of Medicine on the Anschutz Medical Campus. PSCCM is one of the leading divisions in respiratory medicine in the world, and I didn't need too much time to realize that leading this division would be a one-of-a-kind opportunity to work with fantastic faculty and staff in the future. I loved the idea of being part of one of the most exciting pulmonary programs of the world.

"I also immediately realized the excellent opportunities on the campus, including scientific interaction with leading basic science departments and interdisciplinary centers such as the Cancer Center and the Gates Center. The Gates Biomanufacturing Facility provides a unique chance to bring the next generation of cell therapy approaches to chronic lung disease, the third largest cause of death of all diseases to date. Modifying therapeutic cell preparations, such as mesenchymal stem cells, will transform our treatment of lung diseases, such as chronic obstructive lung disease or idiopathic pulmonary fibrosis, in the near future and significantly benefit our patients' quality of life."





## **Jeffrey Jacot, Ph.D.**

*Jeffrey Jacot, Ph.D., an associate professor in bioengineering, was recruited by the departments of bioengineering and*

*pediatrics in 2016 from Rice University to further his research on developing biomaterials designed to fix heart defects in infants, eliminating the need for heart transplants or multiple and complex surgeries. Jacot received a B.S. in chemical engineering from the University of Colorado Boulder, followed by six years of industry experience in the design and development of devices for heart surgeries. He received a Ph.D. in biomedical engineering from Boston University, did postdoctoral work in the Cardiac Mechanics Research Group at the University of California, San Diego, and joined Rice University in 2008.*

"We work to understand the clinical needs in congenital heart defect management and repair, analyze the mechanical and biological processes in heart tissue development, and develop novel biomaterials for tissue-engineered heart muscle. Our research therefore involves bridging basic biological research and translational clinical therapies; active collaborations with surgeons, clinicians, radiologists and biologists are critical to our success. The Anschutz Medical Campus allows close contact with basic science biologists from a top medical school and clinicians from a top children's hospital.

"Additionally, the Gates Center provides a structure to bring scientists, engineers and clinicians together for translational regenerative medicine with both financial support and professional expertise. The Gates Biomanufacturing Facility is unique in its ability to work through the development of cell processing and protein manufacturing processes in a good manufacturing facility. I have met with many members of the Gates Center, and I plan to use the facility for cell processing to move my research forward through preclinical trials and into early clinical trials."



## **Melanie Königshoff, M.D./Ph.D.**

*Melanie Königshoff, M.D., Ph.D., joined the University of Colorado in November 2016 as a professor of medicine in the Division*

*of Pulmonary Sciences and Critical Care Medicine. Her multidisciplinary research team—representing clinical medicine, molecular and cell biology, pharmacology, and bioengineering—aims to understand how the lung repairs itself, and to create new approaches to repairing and regenerating lung tissue.*

In addition to her own research, Königshoff will also work to consolidate CU's significant research portfolio in lung damage and fibrosis into a new fibrosis and regeneration program. Having been recruited from the Helmholtz Comprehensive Pneumology Center in Munich, Königshoff speaks of her

commitment to teaching and mentoring in the future: "In Germany, there is no general structured program giving M.D.'s dedicated time to perform research. The U.S. fellowship program represents an important basis and structure to promote translational science, and to train future leaders in respiratory medicine."

"One major aim in respiratory medicine is to be able to rebuild a diseased lung. To successfully achieve this aim, we need to approach it in an interdisciplinary and integrated approach. I am very attracted by the breadth of expertise and scientific excellence present in the pulmonary division, as well as on the entire Anschutz Medical Campus, which includes a unique composition of basic, translational and clinical science investigators. The collaborative spirit across centers and institutions on campus, which I have experienced from the beginning, presents an invaluable basis to achieve our aim and to advance our knowledge in regenerative medicine approaches to respiratory disease.



"The Gates Center is a unique example of an interdisciplinary center that represents an essential pillar for anyone at Anschutz Medical Campus to better understand and apply regenerative approaches to medicine. It provides state-of-

the-art stem cell expertise, facilities and resources such as iPS cell generation, which we aim to apply for modelling and therapy of chronic lung disease."



## **Holger Russ, Ph.D.**

*Holger Russ, Ph.D., was the first recruit to the Barbara Davis Center for Diabetes in 2016 under the new leadership of Director of Basic and Translational*

*Research Lori Sussel, Ph.D. He received his Ph.D. from Tel Aviv University and did his postdoctoral training at the University of California, San Francisco (UCSF).*

"I have been studying the underlying molecular mechanisms of diabetes for the last decade with a focus on the insulin-producing beta cell located in the pancreas. The Barbara Davis Center for Diabetes (BDC), located on the Anschutz Medical Campus, has been on the forefront of diabetes care and research for as long as I have been working in the field.

"After concluding my postdoctoral work with professor Matthias Hebrok, director of the Diabetes Center at UCSF, I looked at diabetes centers worldwide to identify the ideal conditions in which to establish my independent research program. Indeed, the BDC hosts an outstanding array of basic and clinical researchers with complementary expertise working on finding a cure for diabetes, and also provides an excellent infrastructure and work environment.

"However, key for my decision to move my family to Colorado and to join the BDC, was the recruitment of the new research director, professor Lori Sussel from Columbia University, and her vision to further accelerate the ongoing research activities

with the goal of establishing the BDC as one of the foremost diabetes centers worldwide. I wholly share this vision and am thankful and proud to be part of the BDC now.

"In addition, the Anschutz Medical Campus is one of the fastest growing medical campuses in the nation. It already is host to state-of-the-art technologies, boasts a wide array of specialized core facilities and provides a research hub for an impressive sum of renowned experts in biological sciences. I anticipate that this only will get better in the coming years.

"During my visits to Denver, prior to my decision to join here, I had the chance to meet with several researchers within the Gates Center and the Gates Biomanufacturing Facility, including Gates Center director and professor Dennis Roop. The overall enthusiasm and clear path to move leading-edge technologies from the bench to bedside was a critical signal to me.

"Previously, I have successfully generated glucose responsive beta-like cells and functional thymic cells from human pluripotent stem cells. These findings have tremendous translational potential, and we are actively working on further refining our approaches to be able to move them into a clinical setting. Having the combined expertise of the Gates Center and the Gates Biomanufacturing Facility has helped me develop novel strategies for creating functional cell types for cell therapy purposes, and we are currently testing these in my laboratory.

"Looking forward, I am excited to be part of the Gates Center and to take the next step in my research, further advancing our findings to improve human health."



## **Michael Verneris, M.D., Ph.D.**

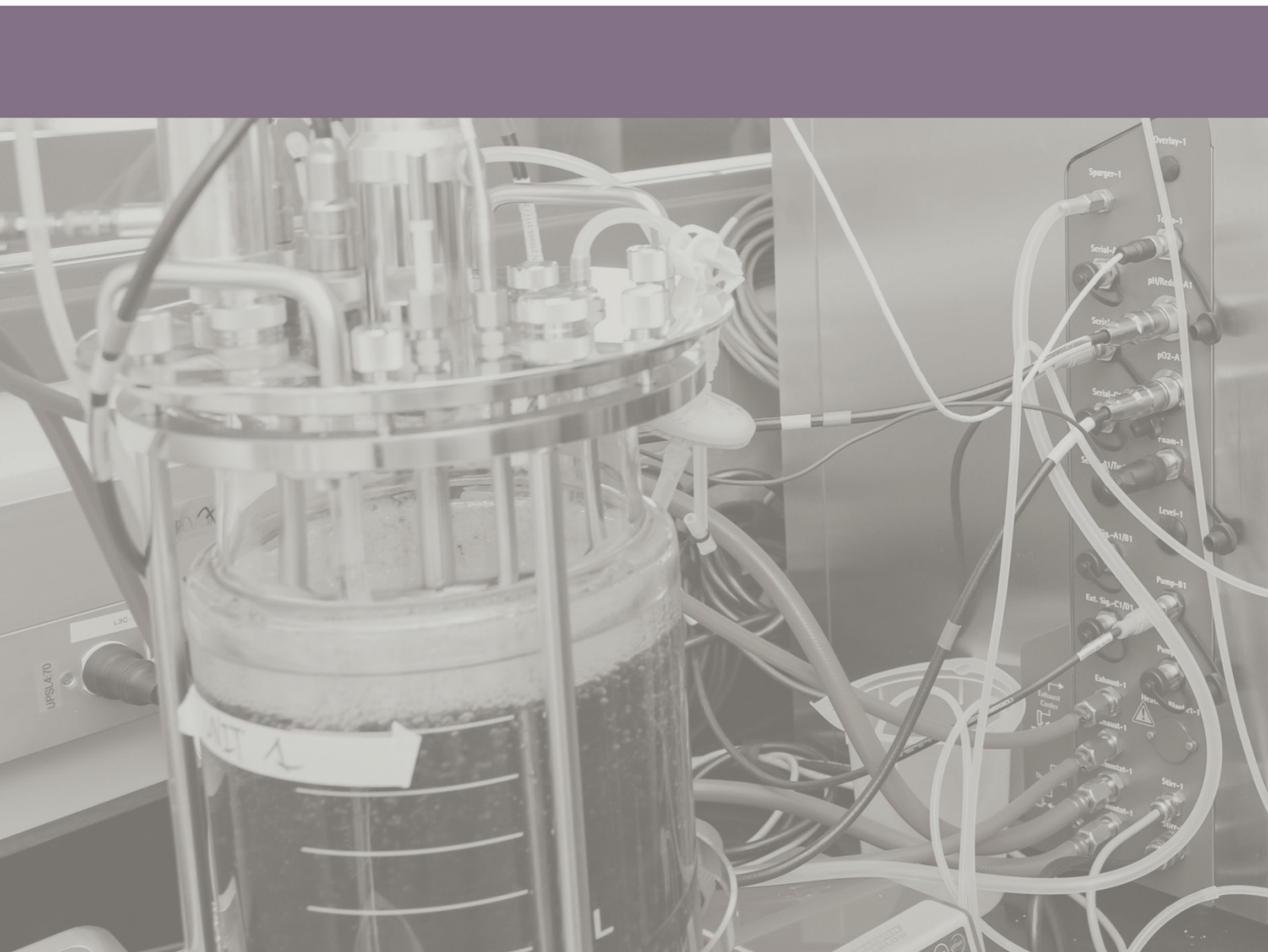
*In October 2016, Michael Verneris, M.D., Ph.D., was named director of Children's Hospital Colorado's Bone Marrow Transplant and*

*Cellular Therapy for the hospital's Center for Cancer and Blood Disorders. Dr. Verneris received a medical degree from the Dartmouth-Brown Program in Medicine and completed a pediatric internship and residency at Children's National Medical Center, followed by a pediatric hematology/oncology fellowship at Stanford University and a post-doctoral fellowship at the Stanford University Division of Bone Marrow Transplant. Before joining Children's Colorado, Dr. Verneris served as vice*

*chair for research and was a tenured professor at the University of Minnesota Medical School, Department of Pediatrics.*

"There were many factors that attracted me to Anschutz Medical Campus, including a fantastic children's hospital, a stellar research community in immunology and a large clinical volume that will allow us to translate our laboratory findings into patient care.

"The presence of the Gates Center played a critical role in my recruitment to Children's Hospital Colorado and the University of Colorado. To be honest, I looked at a number of jobs and those that did not have a good manufacturing practice facility were nonstarters for me. It was absolutely necessary to have a facility such as the Gates Biomanufacturing Facility, which was already established and functioning."





# RESEARCH COLLABORATION SPOTLIGHT



EB iPS Cell Consortium funders and members (left to right): Dr. Angela Christian from Columbia University Medical Center, EBRP co-founder Heather Fullmer, EB Medical Research Foundation CFO Paul Joseph, Dr. Anthony Oro from Stanford University School of Medicine, Dr. Dennis Roop from the University of Colorado and EBRP co-founder Alex Silver

The Epidermolysis Bullosa (EB) iPS Cell Consortium was established on May 1, 2016, to fight the rare and debilitating genetic skin blistering disease. The consortium includes research teams from the University of Colorado Anschutz Medical Campus, Stanford University School of Medicine and Columbia University Medical Center.

EB affects thousands across the U.S. and worldwide, and is characterized by chronic skin wounds similar in property to thermal burns and indistinguishable from burns induced by chemical agents such as mustard gas. Many children afflicted with recessive dystrophic EB, one of the most painful and disfiguring forms of the condition, do not survive their teens after lives compromised by chronic, debilitating pain.

The consortium is jointly funded by the EB Research Partnership (EBRP) and the EB Medical Research Foundation (EBMRF). EBRP was cofounded by Jamie and Alex Silver and Heather and Ryan Fullmer, who have children diagnosed with EB, and by Jill and Eddie Vedder of Pearl Jam.

EBRP is the largest nonprofit dedicated to funding research aimed at treating and ultimately curing the disease. Using a venture philanthropy model, EBRP takes concepts from venture capital investing and applies them toward achieving philanthropic goals. Notably, when the partnership makes a traditional donation to a research project, they retain the added upside of generating a recurring donation stream if the therapy or product is commercially successful. This revenue is then used to fund additional EB research. EBMRF is currently directed by Paul and Andrea Joseph, who also have a son with EB.



Led by Gates Center of Regenerative Medicine Director Dennis Roop, Ph.D., the Colorado team includes Ganna Bilousova, Ph.D., and Igor Kogut, Ph.D., both assistant professors of dermatology; Anna Bruckner, M.D., chief of Pediatric Dermatology and director of the Epidermolysis Bullosa Center of Excellence at Children's Hospital Colorado; and Thomas Payne, Ph.D., director of Cellular Therapies at the Gates Biomanufacturing Facility.

In the fall of 2015, Dr. Roop and his team, as well as research teams led by Dr. Angela Christian from Columbia University Medical Center and Dr. Anthony Oro from Stanford University School of Medicine, applied to EBRP and EBMRF for funding. Rather than being awarded individual grants, they were challenged as a group to form a consortium with a collaborative team approach meant to produce faster results through partnership and shared resources. After approval from each of the institutions, the newly formed EB iPS Cell Consortium kicked off with its first meeting on the Anschutz Medical Campus on July 22, 2016.

The initial goal for the consortium was to spend the first year comparing and contrasting approaches and sharing techniques and ideas to settle on a validated approach with which to move forward in clinical development of an iPS cell therapy for EB. The great news is that the consortium accomplished these year-one milestones in only six months, prior to the end of 2016.

The group agreed to adopt Colorado's reprogramming technology for generating patient-specific iPS cells. A renewal application for the consortium was recently awarded and provides supplemental funds for the Colorado team to supply reprogramming reagents for the Stanford and Columbia teams. Colorado also received an additional award to develop an iPS cell biobank for EB patients.

“EBRP was founded on the principle that partnership is the cornerstone to success in healing and curing EB. This first-ever EB iPS Cell consortium has allowed three global leading EB research teams to come together to find a treatment or cure faster than any individual research approach we've seen.

When time is the most precious resource of any child or adult living with EB, progressive collaboration like this is increasingly important. We are extremely encouraged by the consortium's accomplishment of its year-one milestones in just six months and are more hopeful than ever about its ability to change the lives of those living with EB.

—Alex Silver, EBRP co-founder

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UNIVERSITY OF COLORADO  
ANSCHUTZ MEDICAL CAMPUS

Members of the EB Research Consortium attended the kick-off meeting on the Anschutz Medical Campus on July 22, 2016, which included a tour of the Gates Biomanufacturing Facility.



# SELECT MEMBER PUBLICATIONS\*

**Bruce Appel, Ph.D., Professor, Pediatrics**, published a paper in *Development* that provides new evidence for how neural progenitor cells stop dividing and produce neurons and glia. In particular, the manuscript shows how microRNA mir-219 modulates the response of neural progenitors to a key molecular signal called Sonic Hedgehog that promotes progenitor cell division.

**Steven Dow, DVM, Ph.D., Professor, Clinical Sciences, Colorado State University**, published two papers in *Stem Cells and Development* investigating mechanisms by which canine mesenchymal stem cells (MSCs) suppress T cell activation and dendritic cells maturation and activation. These studies discovered that adipose and bone marrow derived MSCs utilize overlapping as well as distinct biochemical pathways to suppress T cell activation and cytokine production. A key role for PD-L1 in suppression of dendritic cell function by MSCs was also discovered.

**Curt Freed, M.D., Professor, Medicine, Division of Clinical Pharmacology, and Wenbo Zhou, Ph.D., Assistant Professor, Medicine, Division of Clinical Pharmacology**, published a paper in the journal *PLoS One* that describes their creation of a cell line that will help research on Parkinson's disease. The cells make the chemical dopamine, which is essential for normal movement. In Parkinson's disease, the brain cells that make dopamine die off. The authentic dopamine neurons were selected by isolating single cells and allowing clones to grow. This new cell line is being requested by labs around the world to help with research on Parkinson's.

**Mayumi Fujita, M.D., Ph.D., Professor, Dermatology**, has a paper accepted for publication in *Oncogene* identifying the mechanism by which NLRP1 (NACHT, LRR and PYD domains containing protein 1) promotes the growth of human melanoma by enhancing inflammasome activation and suppressing apoptosis.

**James Hagman, Ph.D., Professor, Immunology and Microbiology**, has a paper accepted for publication in the *American Journal of Human Genetics* identifying de novo mutations in the atypical transcription factor EBF3 as the cause of a neurodevelopmental syndrome.

**Jeffrey Jacot, Ph.D., Associate Professor/Bioengineering**, published a paper in *Cellular and Molecular Bioengineering* reporting that stem cells isolated from human amniotic fluid have the potential to differentiate into heart muscle cells. His long-term goal is to use tissue engineering to create a living cardiac patch from amniotic fluid stem cells isolated from a fetus with a congenital heart defect that fetal surgeons would use to repair the heart defect in utero.

**Antonio Jimeno, M.D., Ph.D., Professor of Medicine, Division of Medical Oncology**, published a paper in the *Journal of the National Cancer Institute* describing how cancer stem cells resist therapy and are a major cause of relapse, long after the bulk of a tumor has been killed. The study was the result of seven years of research and innovation, including the development of novel techniques that allowed Dr. Jimeno's team to identify, harvest and grow these elusive cancer stem cells into populations large enough to study. This major body of work provides specific targets for the development of new cancer therapeutics.

**Melissa Krebs, Ph.D., Assistant Professor, Chemical and Biological Engineering, Colorado School of Mines and Karin Payne, Ph.D., Assistant Professor, Orthopedics** published a scientific video in the *Journal of Visualized Experiments* demonstrating a rat tibial growth plate injury model that they have developed to characterize repair mechanisms and evaluate growth plate regeneration strategies. They also published a paper in *Carbohydrate Polymers* reporting the development of an injectable microgel that can deliver biological factors to a site of injury in the growth plate to promote repair.

**Kenneth Liechty, M.D., Professor of Surgery, Division of Pediatric Surgery**, published a paper in *Wound Repair Regeneration* suggesting that the decline in biomechanical properties of diabetic skin during the progression of the diabetic phenotype is due to the dysregulation of the microRNA, miR-29a, resulting in decreased collagen content. Dr. Liechty and his team also showed that the diabetic phenotype could be reversed by treating diabetic mice with mesenchymal stem cells or by directly inhibiting miR-29a.

**Traci R. Lyons, Ph.D., Assistant Professor of Medicine, Division of Medical Oncology**, published a report in *Oncogene* describing a novel role for a neuronal guidance protein, semaphorin 7a, in promotion of breast cancer progression. Specifically, her studies showed that semaphorin 7a is associated with increased metastasis and breast cancer-related deaths in clinical cohorts and that semaphorin 7a drives breast cancer progression by promoting growth, invasion into the surrounding breast tissue and invasion into the lymphatic vasculature, which can ultimately lead to lymph node metastasis. These preclinical results provide impetus for exploring semaphorin 7a as a novel target for therapeutics and/or as a marker for determining the likelihood of metastatic progression in breast cancer patients.

**Chelsea Magin, Ph.D., Assistant Professor, Division of Pulmonary, Department of Medicine** published a paper in *Experimental Biology and Medicine* describing the development of a new wound treatment device that results in faster healing of both dermal and epidermal tissue. This device consists of a bi-layered, biodegradable hydrogel dressing that uses microarchitecture to guide two key steps in the proliferative phase of wound healing, re-epithelialization and revascularization. The results reported demonstrate a high potential for this new dressing to effectively accelerate wound healing.

**Enkhee Purev, M.D., Ph.D., Assistant Professor, Medicine, Division of Hematology**, published a paper in the *British Journal of Hematology* describing the development of a novel partial T-cell depleted transplant procedure that infuses high numbers of granulocyte colony-stimulating factor-mobilized CD34+ selected peripheral blood stem cell combined with a bone marrow transplantation-equivalent dose of nonmobilized donor T-cells, which achieved excellent engraftment and survival, and dramatically reduced the incidence of both acute and chronic graft-versus-host disease in patients with severe aplastic anemia.



Rui Yi, Ph.D., *Associate Professor of Molecular, Cellular and Developmental Biology, University of Colorado Boulder*, published a paper in *Science* describing the discovery of a new mechanism that activates the quiescent cellular state by a transcription factor, Foxc1, when hair follicle stem cells undergo self-renewal. A perspective article that accompanied this paper stated the following: “the findings push our understanding of hair cycle control from the morphogen level to an epigenetic level.” An image from this paper was also highlighted on the cover of the same issue of *Science*.

\* This list highlights only a few of numerous Gates Center members' publications.



# GRANT AWARDS

PRINCIPAL INVESTIGATOR(S)	SPONSOR	AMOUNT AWARDED	TITLE	FUNDING PERIOD
Artinger, Kristin/ Niswander, Lee	National Institutes of Health	\$2,519,234.00	Function of chromatin modifiers in cranial neural crest development	6/01/2016– 5/31/2020
Artinger, Kristin/ Heide, Ford	National Institutes of Health	\$372,034.00	Using zebrafish to study the role of six1a/b and microRNA-30a in rhabdomyosarcoma (RMS) initiation and progression. This project also provides funding for the Ford lab.	7/01/2016– 6/30/2018
Artinger, Kristin	Skin Disease Research Center	\$20,000.00	Identification of epigenetic inheritance in melanoma initiation, progression and susceptibility	9/01/2016– 8/31/2017
Bilousova, Ganna/ Kogut, Igor	Gates Grubstake Fund	\$325,000.00	Induced pluripotent stem cell services as a platform for clinical research	2/01/2016– 1/31/2019
Bilousova, Ganna	Linda Crnic Institute for Down Syndrome	\$100,000.00	Study of stem cell defects in Down syndrome	4/29/2016– 04/29/2017
Bruckner, Anna	Epidermolysis Bullosa Research Partnership	\$83,664.00	Epidermolysis Bullosa Clinical Research Consortium	7/01/2016– 6/30/2017
Dellavalle, Robert	Pfizer Independent Grants for Learning & Change	\$332,010.00	Comprehensive Decision Aids for Psoriasis and Acne Patients	5/23/2016– 6/01/2017
Dellavalle, Robert	National Institutes of Health NIAMS	\$15,000.00	Conference Grant for the 7th International Congress of Dermatoepidemiology	9/01/2016– 8/31/2017
Dow, Steven	Shipley Foundation	\$500,000.00	Program in applied regenerative medicine	2/01/2016– 2/01/2018
Dow, Steven	Gates Grubstake Fund	\$312,000.00	Antimicrobial stem cell therapy for infected diabetic foot ulcers	6/01/2016– 6/01/2018
Freed, Curt	LaGuardia Foundation	\$20,000.00	Improving human iPS cell derived dopamine neurons for transplant into animal model of Parkinson's disease	8/01/2016– 7/31/2017
Fujita, Mayumi	National Institutes of Health	\$1,778,531.00	Modulation of inflammasome-mediated cytokine activation by EGCG in human melanoma	3/01/2016– 2/28/2021
Fujita, Mayumi	Veterans Administration	\$20,000.00	Supplement to 5I01BX001228 (autoinflammation in human melanoma)	10/01/2016– 9/30/2017
Hiatt, Will	National Institutes of Health	\$1,931,868.00	Multicenter clinical trial of antithrombotic therapy in children	9/15/2016– 6/30/2021
Kogut, Igor	Skin Disease Research Center	\$20,000.00	Develop a new technology for stem cell gene correction	8/01/2016– 7/31/2017
Krebs, Melissa	Boettcher Foundation	\$5,000.00	Development of synthetic bone grafts and evaluation of regenerative potential	1/01/2016– 12/31/2016
Krebs, Melissa	Colorado Office of Economic Development and International Trade	\$39,000.00	Fluorapatite-containing hydrogels as novel dental filling matrices to repair cavities	4/01/2016– 3/31/2017
Liechty, Kenneth	National Institutes of Health	\$1,170,000.00	Identifying CXCR4 receptor agonists to improve diabetic wound healing	7/01/2016– 6/30/2019
Lyons, Traci/Serkova, Natalie	Colorado Clinical & Translational Sciences Institute, University of Colorado Denver	\$25,000.00	Creating an imaging technique to correlate inflamed breast cancer and lymph node metastasis	6/01/2016– 5/31/2017
Lyons, Traci/Hansen, Kirk	Golfers Against Cancer	\$50,000.00	Pilot funding to decipher the tumor extrinsic role of SEMA7A in breast cancer progression	7/01/2016– 6/30/2017
Magin, Chelsea	Skin Disease Research Center	\$20,000.00	Bio-inspired 3D microenvironments for improved patient-specific keratinocyte differentiation	4/17/2016– 7/31/2017




PRINCIPAL INVESTIGATOR(S)	SPONSOR	AMOUNT AWARDED	TITLE	FUNDING PERIOD
Magin, Chelsea	National Institutes of Health	\$1,500,000.00	Advanced, micropatterned wound dressings for enhanced epithelialization	7/12/2016–7/31/2018
Payne, Karin/Krebs, Melissa	National Institutes of Health	\$150,000.00	Endogenous repair of growth plate injuries by local and sequential delivery of factors that inhibit osteogenesis and promote chondrogenesis	3/01/2016–2/28/2019
Payne, Thomas	Colorado Office of Economic Development and International Trade	\$225,342.00	Support for the production of cGMP-grade induced pluripotent stem cells	7/18/2016–12/31/2017
Roop, Dennis/Bilousova, Ganna	EB Research Partnership	\$249,867.00	EB Research Partnership Consortium grant to support the development of novel therapies for epidermolysis bullosa	5/01/2016–4/30/2017
Roop, Dennis/Bilousova, Ganna	DEBRA International and King Baudouin Foundation	\$168,000.00	A stem cell-based therapy for patients with epidermolysis bullosa simplex	6/15/2016–6/14/2018
Sartorius, Carol/Kabos, Peter	National Institutes of Health	\$229,464.00	Cancer-associated fibroblasts in estrogen receptor positive breast cancer	1/01/2016–12/31/2020
Sartorius, Carol/Horwitz, Kathryn	Breast Cancer Research Foundation	\$208,000.00	Metastasis initiating cells of luminal breast cancers	10/01/2016–9/30/2017
Sartorius, Carol/Kabos, Peter	RNA Biosciences Center, University of Colorado Anschutz Medical Campus	\$50,000.00	Single cell analysis of organ specific metastasizing cells in estrogen receptor positive breast cancer	10/01/2016–9/30/2017
Sherk, Vanessa	CCTSI Child and Maternal Health Mentored Pilot Award	\$20,000.00	Metabolic phenotype, insulin signaling and lipotoxicity in osteoblasts from lean and overweight adolescent girls	7/20/2016–7/19/2017
Song, Kunhua	National Institutes of Health	\$1,943,750.00	Mechanisms for cell signaling in the control of cardiomyogenesis	8/01/2016–6/30/2021
Terzian, Tamara	Skin Cancer Foundation	\$25,000.00	Targeting p53 for skin cancer prevention	4/01/2016–3/31/2017
Terzian, Tamara	Cancer League of Colorado	\$30,000.00	p53 in melanoma	7/01/2016–6/30/2017
Terzian, Tamara	National Institutes of Health	\$155,355.00	p53 in melanomagenesis	1/01/2016–12/31/2018
Terzian, Tamara	University of Colorado Dean's Academic Enrichment Fund	\$50,000.00	p53 in skin cancers	1/01/2016–6/31/2016
Torchia, Enrique	Skin Disease Research Center	\$20,000.00	Exploring the role of lipid signaling and metabolism in hyperproliferative skin conditions	8/01/2016–7/31/2016
Wang, Xiao-Jing	Veterans Administration	\$1,028,472.00	Mechanisms of breaking indolence in squamous cell carcinoma; This proposal will study how indolence is maintained and exited using a genetically engineered mouse model.	10/03/2016–10/02/2020
Yi, Rui	National Institutes of Health	\$1,888,687.00	MicroRNA-mediated regulation in mammalian skin	4/01/2016–3/31/2021
Total Awards =		\$17,600,278		

Traci Lyons, Ph.D., and husband John



# COMMERCIALIZATION



2015 Gates Grubstake Fund recipients: Ganna Bilousova, Ph.D., and Igor Kogut, Ph.D.

The commercialization efforts of the Gates Center are led by the Gates Grubstake Fund, which was incorporated as a private 501(c)(3) foundation in August 2015 with the mission of providing translational research funding for projects and teams affiliated with the Gates Center for Regenerative Medicine. Investment decisions are made on a competitive basis by the Scientific Investment Advisory Committee (SIAC), which is comprised of subject matter experts and institutional investors with a focus on biotechnology:

- William Hiatt, M.D., professor, Division of Cardiology, University of Colorado Anschutz Medical Campus; president, CPC Clinical Research
- Ryan Kirkpatrick, partner, Colorado Impact Fund
- David L. Lacey, M.D., biopharmaceutical consultant, former SVP, head of research, Amgen
- Kyle Lefkoff, founder and general partner, Boulder Ventures
- Kimberly Muller, J.D., director of technology licensing, University of Colorado
- Michael Perry, DVM, Ph.D., FRCVPT, senior vice president and chief scientific officer, Global Business Development & Licensing, Novartis AG
- Mark Petrash, professor and vice chair, Department of Ophthalmology, University of Colorado Anschutz Medical Campus
- Ann Sperling, senior director, Trammell Crow Company; member, Gates Advisory Board Translational Research



# PROGRESS REPORTS FROM 2015 GRUBSTAKE FUND RECIPIENTS

**Ganna Bilousova, Ph.D., and Igor Kogut, Ph.D.,**

*Department of Dermatology and Gates Center for Regenerative Medicine*

Ganna Bilousova, Ph.D., and Igor Kogut, Ph.D., have developed a proprietary method of producing induced Pluripotent Stem Cells (iPS cells) that is safer than most current methods because it does not use viral vectors, and it is more efficient. This year they also developed a new cell source for iPS cell generation that is easier to obtain than fibroblasts isolated from skin biopsies. Noninvasive urine samples can now be used as a source of somatic cells for reprogramming iPS cells. Notably, Drs. Bilousova and Kogut and their team successfully generated an iPS cell line from renal epithelial cells, which were isolated from just 30 milliliters of urine.

A sign of the effectiveness and efficiency of this technology is the number of programs that have chosen to adopt their technology over the past year. The Linda Crnic Institute for Down Syndrome at the University of Colorado at Anschutz Medical Center plans to generate a large biorepository of iPS cells from Down syndrome patients, and they have asked Drs. Bilousova and Kogut to produce patient-specific iPS cell lines for them.

Additionally, the EB iPS Cell Consortium, which is comprised of research teams from the University of Colorado, Stanford University and Columbia University and was established to develop a clinically relevant treatment strategy for patients with inherited skin blistering diseases, has also chosen this reprogramming technology as the preferred method for the production of iPS cells for clinical trials (see Research Collaboration). Finally, the iPS cell core facility at Stanford University has expressed interest in using this reprogramming technology for all of its iPS cell projects outside of the consortium.

As a way to broaden the use of this safe and efficient technology within the research community, the Gates Center is developing a new Stem Cell Biobank and Disease Modeling core that will be using this method. Several research groups on the Anschutz Medical Campus have already expressed an interest in using this core facility for the generation of iPS cell biorepositories for a variety of diseases, including epilepsy, cardiovascular disease, chronic lung diseases, type 1 diabetes and age-related macular degeneration.

**Steven Dow, DVM, Ph.D., and Mary Ann DeGroote, M.D.,**

*Department of Clinical Sciences, College of Veterinary Medicine, Colorado State University, in collaboration with the Gates Center for Regenerative Medicine*

Steven Dow, DVM, Ph.D., and Mary Ann DeGroote, M.D., hope to improve healing of chronic drug-resistant infections in humans through the use of activated, allogeneic mesenchymal stem cells (MSCs) as a new therapeutic for treatment of chronic wounds.

This past year the team created cultures of human MSCs from bone marrow of healthy human volunteers purchased from Lonza (Boston, Massachusetts). The human MSCs were successfully expanded in vitro (passaged) and then frozen at low passage for subsequent assays. These studies revealed that human MSCs exhibit high levels of spontaneous bacterial-killing activity (higher than mouse or canine MSCs against MRSA strains of *S. aureus*) and that much of this activity is mediated by factors secreted by MSCs. Importantly, the level of antimicrobial activity released by human MSCs appears to increase as they expand in culture, which is important from the standpoint of in vitro expansion and retention of biological activity.

In an effort to see if their technology could enhance the effectiveness of existing antibiotics, the team screened six representative antibiotics widely used to treat wound infections for additive or synergistic activity for killing of MRSA when combined with conditioned medium from human MSCs. These studies revealed that four of the six major antibiotic classes exhibited synergy with MSC conditioned medium for bacterial killing, and the other two classes exhibited additive activity.

These findings suggest that activated human MSCs can be used broadly with essentially any antibiotic for treatment of chronic wound infections. Dr. Dow has chosen patients with chronic orthopedic implant infections as the optimal patient population to target for the first human clinical trial based on results of preclinical studies of this technology in dogs with similar joint infections.

In the area of corporate development, Dr. Bill Quirk remains committed to actively moving the anti-infective stem cell technology forward as the CEO of a to-be-formed company. He and Drs. Dow and DeGroote are working with CU Innovations to finalize details of this startup company and to begin plans for capital fundraising and pursuit of nondilutive funding, including Small Business Innovation Research proposals.

## 2016 GATES GRUBSTAKE FUND UPDATE

In its second year of operation, the Gates Grubstake Fund continued to focus on accelerating basic, translational and clinical research related to the field of regenerative medicine through the award of three annual grants of approximately \$350,000 each. This year the Gates Center partnered on the Grubstake awards process with the new office of CU Innovations. Led by Kimberly Muller, who joined the campus in June 2015 from the Yale Entrepreneurial Institute, CU Innovations provides the Anschutz Medical Campus with intellectual property management services and connections to a variety of commercialization programs in the university and the community.

Along with Gates Center Entrepreneur in Residence Heather Callahan, Kimberly's office collaborated with the Gates Center to communicate availability of Grubstake funding across campus and to add structure to the evaluation and selection process. As a result of this increased exposure, we received 24 proposals.

After careful review of the written proposals, the Scientific Investment Advisory Committee invited eight applicants to make personal presentations. This competitive selection process resulted in the following three awards in early 2017:

- Jeffrey Olson, M.D.: Intraocular device to potentiate retinal stem cell transplantation in macular degeneration
- Kenneth Liechty, M.D.: A platform technology useful for treating a variety of diseases where inflammation and oxidative stress are pathognomonic in the progression of the disease; initial focus is treatment of diabetic ulcers, where compelling in vitro and animal model data exists showing that treatment results in closure rates of diabetic wounds equal to nondiabetic wounds
- David Wagner, Ph.D.: A 15-amino acid peptide to potentially stop demyelination and restore myelin production in multiple sclerosis

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*Getting great ideas out of the lab and off the shelf is critical to realizing future therapies. In a world of shrinking public funding, we need new solutions like venture philanthropy, forward-thinking business/investor partnerships, and competitive 'valley of death' funding to drive innovation and keep brilliant investigators in the game.*

*—Diane Gates Wallach*

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# CORE FACILITIES

Since January 2011, the Gates Center has established and operated three core facilities that provide members with access to expert advice and state-of-the-art equipment and technologies at discount rates. The three core facilities are the Flow Cytometry Core, Morphology and Phenotyping Core and Bioengineering Core. These core facilities have been partially established and operated with funding from the Gates Frontiers Fund, Gates Frontiers Fund/CU Foundation matching funds, a Skin Diseases Research Core Center grant from the National Institute of Arthritis and Musculoskeletal and Skin Diseases awarded to Drs. David Norris and Dennis Roop, and Academic Enrichment Funds provided by the dean of the CU School of Medicine.

The success of these cores in providing quality service with a quick turnaround time is further illustrated by the fact that in addition to being utilized by Gates Center members, they are utilized by investigators in 15 different departments, divisions or centers within the School of Medicine and in the

following: the schools of pharmacy and dentistry, National Jewish Health, CU Boulder and Colorado State University. In addition, the Bioengineering Core has users from outside Colorado that include the University of Alaska Fairbanks, the University of Alabama at Birmingham, Thomas Jefferson University, the University of Lausanne (Switzerland) and Stony Brook University.

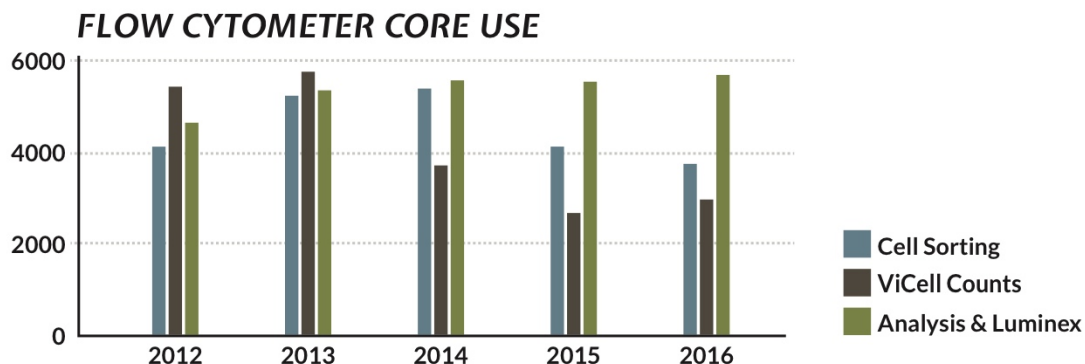
The five-year Skin Diseases Research Core Center grant to Drs. Norris and Roop, which was renewed in September 2014, provides \$400,000/year in direct costs to support four research core facilities, three of which (the Flow Cytometry Core, Morphology and Phenotyping Core and Bioengineering Core) are all located in the Gates Center. This grant provides partial salary support for the directors of these cores, and subsidizes the costs of the cores to keep usage fees low for Gates Center members. This grant also provides partial salary support for an administrative assistant, who additionally serves as the administrative assistant for the center.

A brief description of these core facilities and graphic summaries illustrating their use by both Gates Center members and nonmembers follows.

# 1

## FLOW CYTOMETRY CORE

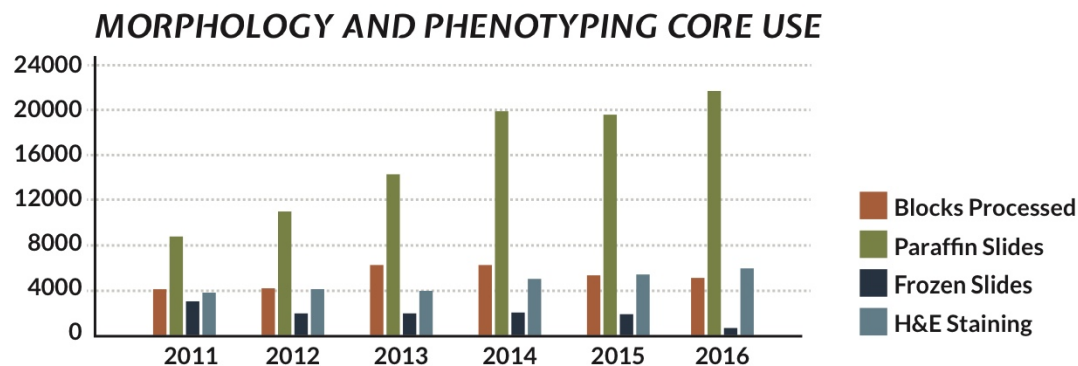
Flow cytometry (FC) is an essential tool for stem cell research. FC uses fluorescent tags in a laser-based fluidic instrument to quickly examine and collect cells at the single-cell level. By using multiple different fluorescent tags, the FC Core can identify the type of cells present in a sample and the biological status of these cells. The FC Core provides cutting-edge equipment at a cost-effective price, a highly enthusiastic and skilled staff, experiment design consultation, data analysis assistance and instrument training services to Gates members.



# 2

## MORPHOLOGY AND PHENOTYPING CORE

The ability of clinicians and scientists to analyze tissues at the microscopic level is essential for understanding disease mechanisms. To facilitate this, the Morphology and Phenotyping Core provides a full set of histology services. Upon consultations with the core's histotechnicians, users submit tissue specimens that are processed according to the investigator's needs. Tissue sections are prepared from the specimens to allow for a qualitative and quantitative analysis of the tissues. Analysis of these tissue sections using various staining procedures further contributes to understanding disease pathology.





# 3

## BIOENGINEERING CORE

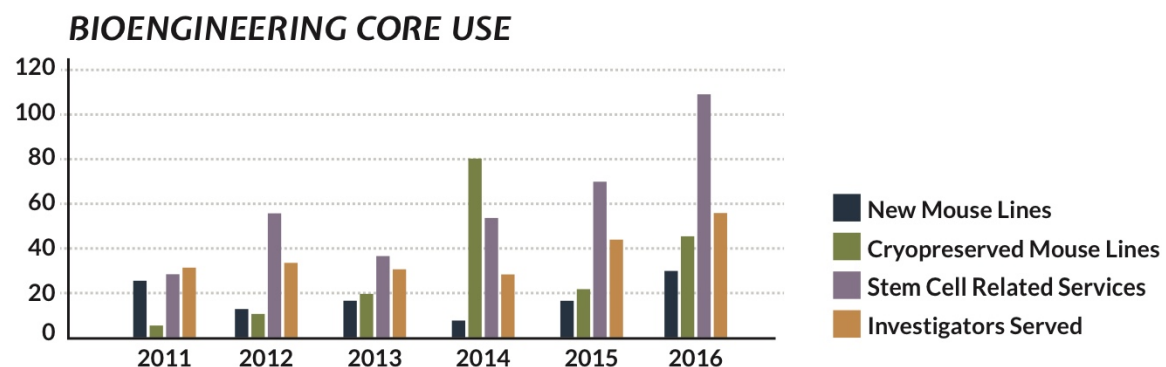
The generation of genetically modified animals is a powerful tool for understanding the role of genes and gene mutations in normal tissue and organ development as well as in diseases. The Bioengineering Core provides pronuclear injections to produce conventional and bacterial artificial chromosome (BAC) transgenic mice, mouse embryonic stem cell (ES) cell injections to produce mice with modified genes (e.g., knockout), cryo-preservation of embryos and embryo re-derivation. We also conduct “gene targeting” experiments in ES cells and induced iPSCs that allow the introduction of a deletion or a targeted mutation into a disease-promoting gene.

With the advent of induced iPSC technology, researchers can now reprogram adult tissue-derived cells into stem cells that can differentiate into any type of cell in the adult body. This approach has tremendous potential, not only for designing new therapies, but also for the understanding of complex human diseases. The Bioengineering Core generates iPSC from various species used in biomedical research (e.g., human, mouse, dog, horse) and conducts genome editing experiments in these cells using the CRISPR/CAS system. This approach enables researchers to specifically change gene sequences in these cells (e.g., create or eliminate disease-causing mutations).

In 2016, the Bioengineering Core supported the research projects of 56 investigators. Our services included the generation of 30 genetically engineered mouse lines, the cryo-preservation of 46 mouse lines and 109 stem cell-related services (e.g., gene targeting in mouse embryonic stem cells, iPSC generation, iPSC differentiation into defined cell types and gene editing in iPSC).

The Bioengineering Core serves investigators from the University of Colorado Denver as well as investigators from other Colorado universities (e.g., Colorado State University, University of Colorado Boulder) and universities across the United States and abroad (e.g., Switzerland, Australia).

Financial support of our facility is provided by grant funding from the NIH (SDRC, CCTSI), funding from the CU School of Medicine (dean’s funding) to Dr. Peter J Koch and user fees. The Gates Center for Regenerative Medicine has provided space and equipment for our stem cell operations.



# GATES BIOMANUFACTURING FACILITY



Gates Biomanufacturing Facility staff (BACK ROW UPPER STAIRS, left to right) Jamie Bunker, Jordan Krause, Tom Street, Gabe Orosco, Frances Brostrom, Matt Brenton, Jackie Nelson (FRONT ROW, left to right) Dante Merrill, Mitchell Fraller, Sonja Giguere, Tom Payne, Dr. Roop, Sadhna Mehra, Brittany Robinson, Abitha Jacobs, Deepa Vadakan, Paul Lemaire, Tim Gardner, Jessica Freeman, Matt Seefeldt, and Laura Titter



## Charles C. Gates Biomanufacturing Facility

UNIVERSITY OF COLORADO **ANSCHUTZ MEDICAL CAMPUS**

This year was the first full year of operations for the Gates Biomanufacturing Facility. With over \$1.3 million in service revenues generated from six commercial customers and three Anschutz Medical Campus laboratories, we exceeded our revenue forecast. In order to meet the expanding needs of our clients, we also increased our team of experienced laboratory technicians and quality managers from 10 at the beginning of the year to 21 by December 2016.

In August 2016, we delivered our first clinical grade material to an early-stage company that is developing a unique protein-based biologic, which enhances the engraftment of hematopoietic stem cells during transplantation. The company expects to use the material we produced for a human clinical trial in 2017.



At the request of one of our customers, we invested in the purchase of a CliniMACS Prodigy® system in early 2016. The CliniMACS Prodigy® is an integrated and enclosed solution for streamlining cell-processing operations—from cell separation through cell culture to formulation of the final product.

During the year, our staff completed the required training and used the system to develop a key analytical assay for one of our clients. By the third quarter of 2016, we had begun process development work for three different cell therapy clients from around the country based in large part on the fact that we have the experience and ability to use the CliniMACS Prodigy® system to develop cell therapies.

At this point, we are one of only a handful of academic manufacturing facilities nationwide with the ability to process cells for CAR-T cell-based immunotherapies for cancer. We purchased a second CliniMACS Prodigy® unit later in 2016 to support growing customer demand for cell therapies and anticipated cGMP production in 2017.

One of the primary justifications for building the Gates Biomanufacturing Facility was to retain and recruit leading principal investigators and clinicians interested in developing cell therapies for clinical trials. In 2016, our recruiting objective became reality as the facility played a crucial role in attracting several high profile Anschutz campus recruits who are introduced in the Top Talent Recruitment section on page 14.

The tremendous progress made at the Gates Biomanufacturing Facility in 2016 would not have been possible without the guidance and financial support of our operating partners:

GATES FRONTIERS FUND

uchealth



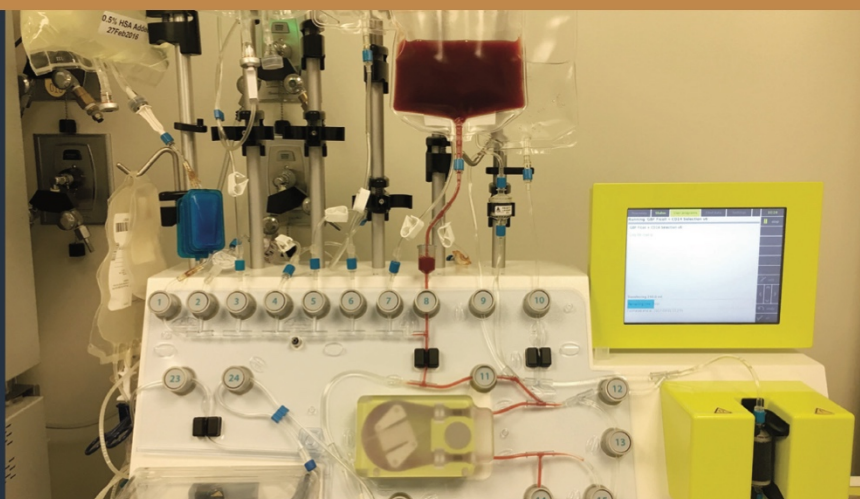
“

*I have worked for a number of laboratories over my career, including 10 years in quality assurance. I have never worked with a better team or organization. We've used cross-functional teams to resolve complex issues, creating an environment of empowerment, inclusion and engagement. I look forward to many years here improving and developing the Gates Biomanufacturing Facility to be one of the finest academic good manufacturing facilities in the US.*

**-Frances P. Brostrom, manager of quality assurance, Gates Biomanufacturing Facility**

”

Private philanthropy allowed the Gates Center to acquire a second CliniMACS Prodigy® system for use within the Gates Biomanufacturing Facility. State-of-the-art and mobile, this amazing German-made machine has attracted both current and prospective researchers, as well as commercial companies, to the facility. Many thanks to our generous benefactors who have helped us meet this demand.





# EDUCATION



2016 Gates Center Summer Internship Program (GSIP) participants and affiliated students: (BACK ROW, left to right) Andrew Ramos, Michael Wright, Smaranda Birlea, Molly Kubesh, Dante Merrill, Drew Dyson, Jackson Knappen, Sadie Doran, Ian Minzer, George Lampe (FRONT ROW, left to right) Basel Tamimi, Anas Atassi, Ryo Iwata (kneeling), Daniel Kim (sitting), Andrew Strosnider, Paige Ostwald, Andrew Parker, Aastha Dhakal, Emma Office, Christopher Chow (not shown: GSIP intern Stephanie Fukui, and AI Faisal student Ismat Alkhani)

“

My most sincere and kind thank you to the people who made it possible for me to be part of this program and to the members of my lab who supported my project and taught me not only the techniques necessary for the project but also about respecting the work of others. This experience was truly valuable for the research skills it taught me and for introducing me to the life of a biomedical researcher.

”



2016 was another year in which the Gates Center worked hard to inspire and train the next generation of scientists and clinicians.

Summer 2016 marked the second successful year of the Gates Center Summer Internship Program (GSIP). The GSIP directors Drs. Neil Box, Tamara Terzian and Enrique Torchia, and coordinator Charlie Wall, recruited 19 college undergraduates from across the country competitively chosen for the program. The directors also established partnerships with Middlebury College and Alfaisal University in Saudi Arabia, from which we had four affiliated students. Two undergraduates from Berea College in Kentucky also shadowed the program.

Each of the interns was assigned to a mentor's lab for the 11-week program and attended a Gates Center orientation, a tour of the Gates Biomanufacturing Facility and weekly lectures from various researchers and clinicians including Gates Advisory Board members Marilyn Coors, Ph.D., and Wag Schorr, M.D. The program culminated on the afternoon of Friday, August 12, with award-winning CBS medical broadcaster Dr. Max Gomez as guest speaker, a poster session showing the interns' work and a reception for family, friends, donors and campus attendees.

Follow-up surveys showed that we are meeting our goal of encouraging outstanding undergraduates to consider careers in biomedical research. We were also pleased to hear that 100 percent of this year's mentors would like to take another intern in summer 2017, and 100 percent would recommend the program to others. Thanks to Jeff Siebert of Banana Bones Media, we have a video describing and commemorating the 2017 program. See: <https://vimeo.com/bananabones/review/181954533/a052b8c0fc>.

By fall 2016, the program had received additional funding that enabled the dedicated program directors to institute several changes to improve the program overall. Having done a survey of other similar programs in an effort to attract the most competitive group of students, we decided to increase the summer stipend from \$3,000 to \$3,500.

Thanks to a lovely gift from Rhondda and Peter Grant in December 2014, we inaugurated the Gates Summer Internship Program (GSIP) in the summer of 2015. Gifts received during the 2016 calendar year enabled the Gates Center to host 18 college undergraduates in the GSIP and subsequently to plan for an expanded summer 2017 program.

Specifically, we received generous gifts from Rhondda and Peter Grant, Monty and Frank Kugeler, the Walter S. Rosenberry, III, Trust, Penny Lewis (in honor of her grandson and 2016 GSIP alumnus Drew Dyson) and others. This additional funding allowed the program directors to also address the only suggestion from 2016 program participants for improving the program by way of providing outside-of-work activities and housing opportunities.

Additionally, we were able to enhance the program's mission by offering two fully funded summer training positions that include lodging, transport and food to highly qualified undergraduates from rural Colorado who are in financial need.



Rhondda and Peter Grant helped the Gates Center launch its Gates Summer Internship Program (GSIP) in the summer of 2015.



*I appreciate what the program has provided for my fellow peers and me. Over the months, I have gotten a better glimpse at the world of research. My aspirations were strengthened, and along the way I have gained valuable experience. I look forward to seeing this program continue to grow and how I can apply everything that I have learned to my own future. I would like to thank my mentor, the coordinators and all those who helped make this opportunity possible.*



Additionally, given that the only suggestions for improving the program centered around providing additional outside-of-work activities and housing opportunities for 2017, the GSIP initiated a partnership with the Colorado Leaders, Interns, Mentors in Business (CLIMB) program. This local and successfully established program directed by Suzanne McKenzie sponsors weekly lectures and dinners and occasional weekend events for summer interns as well as housing spots on the CU Denver campus.

We will be including an events fee in our budget so all our interns can participate in the CLIMB program activities—even students living at home. Moreover, thanks to the efforts of Christian Valtierra, assistant director of the Office of Inclusion and Outreach on the Anschutz Medical Campus, GSIP established a partnership with the office in conjunction with the CCTSI Clinical and Translational Science Institute. The two offices will jointly provide a Career Development Lunch Series in 2017 for our GSIP students that focuses on networking and professionalism, the stages of the academic scientific path, opportunities in the private and public sector and applying to graduate school. These are all auspicious developments for our program, and we look forward to the summer ahead.

Following the recommendations made by the Gates Center self-study reviewers in spring 2015, the Gates Center also continued its support of and integration with the Graduate Program in Cell Biology, Stem Cells and Development Graduate Program (CSD), directed by Bruce Appel, Ph.D., Gates Center member and Diane G. Wallach Chair in Pediatric Stem Cell Biology. This combined program is based on the premise that although medical use of stem cells holds great promise for treatment of human diseases and birth defects, to advance the use of stem cells in the clinic scientists must continue to pursue

fundamental discoveries as to how cells function and how cells in embryos form the different tissues of the body.

This year the Gates Center funded graduate student fellowships that will go toward two outstanding candidates in fall 2017. Gates Center funding also enabled 12 students to attend national and international scientific conferences and one student to travel to the University of California, Los Angeles, to receive specialized training in induced pluripotent stem cell production and differentiation to support a collaborative study in neural tube formation and developmental defects.

The Gates Center helped support classroom activities, publicity and boot camp training taught by graduate students for approximately 30 students in summer 2016. Dr. Appel is also serving on a subcommittee of training program directors that is renewing the core curriculum for first-year graduate students in the fall semester. As part of this new curriculum, starting in fall 2017, the CSD plans to provide two three-week intensive courses: Stem Cells to Regenerative Medicine and Introduction to Experimental Models and Approaches in Developmental Biology, which will include stem cell models. The Gates Center hopes its ongoing support of the CSD will result in significant impact to this crucial, combined program.

Finally, Gates Center members Ganna Bilousova, Ph.D., and Karin Payne, Ph.D., expanded their course, Special Topics in Bioengineering, to include more in-depth discussion of the topics. Taught in fall 2016 with visiting instructors Igor Kogut, Ph.D., Danielle Sorzano, M.D., Thomas Payne, Ph.D., Jeffrey Jacot, Ph.D., and Tristan McClure-Begley, Ph.D., the course covered current advances in bioengineering of human tissues and organs, and attracted bioengineering graduate students. Given the wide interest on campus, Drs. Bilousova and Payne look forward to expanding the course's credit hours to include more topics in 2017.





Gates Center Lab Manager Charlie Wall prepares to remove a vial of induced Pluripotent Stem Cells for cryopreservation in liquid nitrogen. This is one of six photos of the Gates Center and Gates Biomanufacturing Center taken in 2016 by award-winning photographer Carol M. Highsmith for inclusion in her stunning collection of images of Colorado and other states preserved in the Library of Congress.



# OUTREACH



Gates Center Executive Director Patrick Gaines welcomed the very first group of students to visit the Gates Biomanufacturing Facility.

Spurred by recommendations from our strategic planning process last spring, 2016 was filled with significant outreach opportunities: ongoing requests and invitations from school groups, organizations and individuals; and legislative progress in the area of regenerative medicine in Washington, D.C.

By virtue of our strategic planning process, we were encouraged not just to focus on external audiences, but also to reach out to colleagues and potential collaborators right here on the Anschutz Medical Campus who might benefit from services offered by the Gates Center and Gates Biomanufacturing Facility. Thanks to our gracious staff, whose everyday job descriptions include significant outreach, and champions such as our Gates Advisory Board member Rick Stoddard, we invited numerous on-campus

researchers and clinicians to come hear about the center and the facility—many of whom were amazed to find such resources on the Anschutz Medical Campus.

Our Gates Center lab, located in the Research Center 1 North building on campus, continues to draw numerous school groups, interested individuals and young people seeking career advice. Throughout the year, Gates Center Lab Manager Charlie Wall and others organized presentations and tours for school groups from Arapahoe High School, Aurora Public Schools, CSU Anschutz Scholars, CU Science Discovery, Denver Academy, Mackintosh Academy, Rock Canyon High School, Stanley British Primary School, the Rocky Mountain Stanford University Alumni Association and Vail Mountain School among others.



Meanwhile, as the staff at the Gates Biomanufacturing Facility across the street from campus grew from 10 at the end of 2015 to 21 by the end of 2016, individual employees took time away from their busy schedules to share their work with student groups. Gates Center Executive Director Patrick Gaines invited 30 students from Bell Middle School to be the first group of students to visit the Gates Biomanufacturing Facility and its staff. This maiden tour was later featured in the CU Anschutz Today newsletter: <http://cuanschutztoday.org/middle-school-students-see-future-gates-biomanufacturing-facility>.

In June 2016, Frances Brostrom and Jordan Krause from the facility planned and participated in the Center for Women's Health Girls' Career Day by sharing their career paths and work with a group of 50 high school girls.

Additionally, Gates Center faculty members Neil Box, Ph.D., Tamara Terzian, Ph.D., and others who founded the Colorado Melanoma Foundation in 2013 promoted skin cancer and melanoma awareness at their annual fundraisers. These fundraising events included the Mallets for Melanoma in partnership with the Denver Polo Club, Summit Melanoma at Cordillera together with the Vail, Aspen and Breckenridge Dermatology and the Club at Cordillera, and the Noir Fashion Show in collaboration with D'Lola clothing brand.

They also participated in outreach activities in schools, the Colorado Council of Medical Librarians' meeting and other community events such as the CareerX program, an ongoing partnership with the Denver Public Schools (DPS) and the Office of Inclusion and Outreach at UC Denver AMC. CareerX is a shadow program that introduces DPS students to careers in the biomedical field. Forty-five students and their teachers participated in hands-on genetics, skin cancer risk and sun safety workshops on February 9 and 25, 2016. These workshops were featured in the CU Anschutz Today and DPS newsletters: <http://>

[cuanschutztoday.org/high-school-students-get-look-at-health-care-careers/?source=todayAMC](http://cuanschutztoday.org/high-school-students-get-look-at-health-care-careers/?source=todayAMC).

As in the past, members of the Gates Center staff have been invited to address a variety of outside groups. In January 2016, Gates Center Director Dennis Roop, and Gates Center members Vikas Patel, M.D., and William Hiatt, M.D., joined a panel at the Vail Symposium. Later in the spring, Dr. Roop presented to the Rotary Club of Evergreen and Mountain Foothills Rotary, and we hosted a fellowship meeting group from Rotary 31 at the Gates Biomanufacturing Facility.

Another highlight of the year was the passage of the 21st Century Cures Act by the U.S. Congress, which was signed into law in December. Significantly, it will allow the FDA to grant accelerated approval to regenerative medicine products, while also providing the agency with wide discretion on creating new approaches to regenerative medicine. It includes a small set-aside of \$30 million specifically to fund regenerative medicine clinical research projects supported by the NIH and FDA. The bill also requires the FDA to work with stakeholders, including manufacturers, sponsors and the National Institute of Standards and Technology, to identify ways to develop standards to aid in product development and evaluation.

Congressman Mike Coffman represents the Anschutz Medical Campus, and he and his staff were actively involved in the inclusion of regenerative medicine in this legislation, which was not included in the original bill that passed the House in summer 2015. We greatly appreciate his support along with those of Senator Michael Bennet and others in the Colorado delegation, Kent Springfield in the University of Colorado Office of Government Relations and Research!America, the nation's largest not-for-profit public education and advocacy alliance committed to making research to improve health a higher national priority.

“

*I want to express my gratitude to you for coming up to the foothills and presenting an absolutely fantastic program. People were truly moved and excited by what you are accomplishing at the Gates Center and its implications for mankind. I have received nothing but rave reviews from everyone who was there.*

—Wayne Lundhagen, Evergreen rotary program chair

”

# NEW FACES AT THE GATES CENTER



**Carmen Garcia:** Carmen Garcia assumed the position of research administrative manager at the Gates Center in September 2016. Having begun her career at the University of California, Irvine, as an administrative analyst/system administrator, Carmen went on to work as special assistant to the vice president at the University of New Mexico and a budget analyst/administrative coordinator at Iowa State University. Subsequent to her move to the University of Colorado Anschutz Medical Campus in 2011, she worked as a grants specialist in the Department of Pediatrics and grant contract manager at the Barbara Davis Center for Childhood Diabetes.

Carmen offers years of experience in accounting, budget planning and providing administrative support derived from serving within four institutions of higher education. She also holds a bachelor's degree from Iowa State University and an MBA from the University of Phoenix. In addition to her experience and expertise, she brings dedication, tenacity and a wonderfully generous spirit to the Gates Center. She kindly volunteers her bilingual skills to help prepare some of our post docs that provide service to linguistically and culturally diverse populations at Children's Hospital.

Born in Mexico City, Carmen is proud of her heritage and her United States citizenship. She is also the proud mother of two grown children, and her interests include gardening and reading.



**Yvette Pita Frampton:** Yvette Pita Frampton, who grew up in Miami, Florida, as part of a large family of exiles, joined the Gates Center Advisory Board in January 2016. A community leader, she has served as a board member for the Denver Health Foundation (vice chair), the Denver Health Volunteer Association Board (president), the Level One Society, the Davidson College Alumni Association Board (vice president) and the Documentary Cinema Institute. She currently serves on the Davidson College Board of Trustees. Yvette also has been an active volunteer with Project C.U.R.E. and Graland Country Day School, where her children are students. She and her husband, Chris, whom she met in college, have three children.

Yvette is an award-winning filmmaker. Her work has screened on PBS and at film festivals across the world, including South by Southwest, the Full Frame Documentary Film Festival, the HBO Urbanworld Film Festival and the New York International Latino Film Festival. She has taught film theory, writing, editing and production at Hollins University, the CU Denver and the Colorado Film Video Institute at the Community College of Aurora.

She holds a BA in English from Davidson College and an MFA in Film Directing from New York University's Tisch School of the Arts.





**Geoff “Duffy” Solich:** Geoff “Duffy” Solich joined the Gates Center Advisory Board in December 2016. As executive vice president of E & P Resources LLC, Duffy serves on the management team of a newly formed upstream oil and gas company focused in the Rockies, Texas and midcontinent regions. This follows a succession of positions he held in the energy business: managing director and founding partner of SFC Energy Partners, a private equity energy fund; vice president of land and corporate development for Medicine Bow Energy Corporation; vice president of land and business development at Cody Energy, LLC; vice president of Wildhorse Energy, a gathering/processing/marketing company; and other varied positions in other companies including Tenneco Oil Company, Grace Petroleum and Snyder Oil Company.

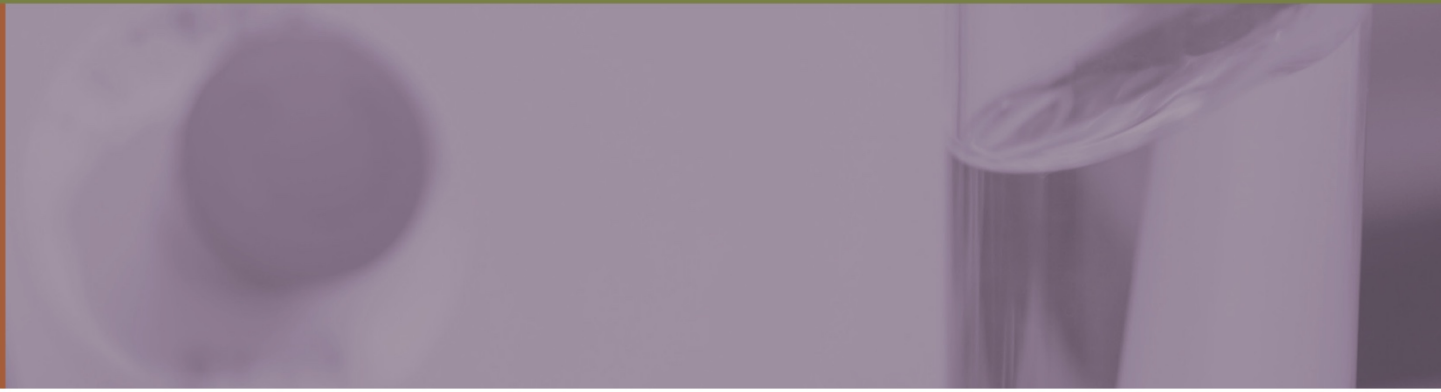
Duffy received his Bachelor of Science degree from CU Boulder in business administration for minerals land management as a four-year recipient of the Eisenhower Evans Scholarship. He now serves on the boards of the Western Energy Alliance, Coachman Energy, the Colorado Golf Foundation and the Solich Foundation (George and Carol Solich). He is also cofounder of the Solich Caddy & Leadership Academy and director and Colorado state chairman of the Western Golf Association.

Duffy is passionate about medicine and its potential for amazing new therapies and cures, having recently worked with his brother George and his foundation to endow a chair on the Anschutz Medical Campus in honor of their mother, Doni Solich. This chair will be held by Valeria Canto-Soler, Ph.D., the director of the newly formed Ocular Stem Cell and Regeneration program (CellSight), in which the Department of Ophthalmology and the Gates Center are partners in an effort to cure age-related macular degeneration, the leading cause of blindness among Americans age 50 and older.

Duffy and his wife Anita have four children.



# FINANCIAL OVERVIEW



Research performed by Gates Center members is funded directly through federal and state research grants, private foundations, individual donations and support from the Gates Center. In addition to providing research support to its members, the Gates Center operates the Gates Biomanufacturing Facility (see page 32) and three core laboratory facilities (Flow Cytometry, Morphology and Phenotyping, and Bioengineering), and it provides laboratory infrastructure to members for work done outside of the core facilities. Commercialization support, education and outreach, and marketing and development activities are also provided as part of the overall Gates Center mission.

## GATES CENTER OPERATIONS

2016 was the first year of operations under a five-year funding agreement between the Gates Frontiers Fund, the University of Colorado Foundation and the University of Colorado President's Office in which the Gates Center receives \$2.4 million per year. The Gates Center received an additional \$148,000 in new philanthropic support for key operational initiatives, including the Gates Summer Internship Program, research and equipment.

Consistent with our mission and past priorities, center program support of \$976,000 was the largest expense category, totaling 40 percent of all spending for 2016. Investments in equipment and operations of the core facilities and the Gates Center laboratories totaled \$495,000 in 2016, which was 20 percent of expenses. The balance of our expenses were for center administration totaling \$377,000 (16 percent), marketing and development totaling \$337,000 (14 percent) and education, outreach and commercialization support totaling \$255,000 (10 percent).

The Gates Center net of sources less expenditures for 2016 was \$107,154. Our combined fund balance for the Gates Center was \$24,784 as of December 31, 2016.



# GATES BIOMANUFACTURING FACILITY OPERATIONS

In its second year of operations the Gates Biomanufacturing Facility generated \$1,511,000 in revenue from a combination of outside for-profit companies and Anschutz-related laboratories. We also received an additional \$1,535,000 from our four operating partners. With cost of goods sold and operating costs totaling \$3,618,000, the Gates Biomanufacturing Facility reported a loss of \$572,254. The combined fund balance was \$192,194 as of December 31, 2016.

	2012	2013	2014	2015	2016
<b>Infrastructure and Operations Grants</b>					
Gates Frontiers Fund	\$1,000,000	\$1,000,000	\$1,000,000	\$1,000,000	\$1,200,000
University of Colorado Foundation	\$1,000,000	\$1,000,000	\$1,000,000	\$1,000,000	\$600,000
University of Colorado Presidents Office					\$600,000
Philanthropy					\$147,617
<b>Infrastructure and Operations Grants</b>	<b>\$2,000,000</b>	<b>\$2,150,000</b>	<b>\$2,003,100</b>	<b>\$2,010,330</b>	<b>\$2,547,617</b>
<b>Expenditures</b>					
Center Lab Operations	\$414,055	\$157,500	\$188,204	\$160,967	\$328,657
Center Core Facilities	\$201,777	\$197,750	\$277,826	\$458,498	\$166,039
Center Research Program Support	\$651,381	\$711,440	\$506,551	\$751,750	\$976,914
Marketing & Development	\$271,856	\$409,994	\$318,155	\$338,655	\$336,937
Center Admin/Maint/Supplies	\$243,287	\$243,107	\$317,948	\$336,792	\$376,593
Center Enrichment	\$26,165	\$28,000	\$53,198	\$18,539	\$30,375
Center Educational Activities	\$20,580	\$23,500	\$60,575	\$31,914	\$122,841
Center Extraordinary Expenses	\$37,650	\$327,866	\$18,027	\$22,000	\$-
Commercialization Support	\$-	\$151,307	\$111,224	\$62,266	\$102,433
<b>Total Expenditures</b>	<b>\$1,866,751</b>	<b>\$2,250,464</b>	<b>\$1,851,708</b>	<b>\$2,181,381</b>	<b>\$2,440,462</b>
<b>Center Sources - Expenditures</b>	<b>\$133,249</b>	<b>\$(100,485)</b>	<b>\$151,371</b>	<b>\$(171,052)</b>	<b>\$107,154</b>
<b>Gates Biomanufacturing Facility</b>					
GBF Customer Revenue				\$166,025	\$1,511,209
GBF Partner Support				\$1,376,750	\$1,535,000
GBF Income				\$1,542,775	\$3,046,209
GBF Expenditures				\$(1,762,518)	\$(3,618,463)
<b>GBF Sources-Expenditures</b>				<b>(\$219,743)</b>	<b>(\$572,254)</b>
<b>Center/GBF Combined Sources-Expenditures</b>				<b>(\$390,795)</b>	<b>(\$465,100)</b>
<b>Center/GBF Combined Fund Balance</b>					<b>\$216,978</b>



# ACKNOWLEDGEMENTS



John Gates addresses Charlie's Picnic attendees on August 24, 2016.

*Many thanks to Edward DeCroke of DeCroke Photography for the majority of the photos included in this report.*



The Gates Center gratefully acknowledges the following individuals, foundations and organizations for supporting our research and mission through their generous gifts of time, talent and treasure during the 2016 calendar year.

## DONORS

The Benevity Community Impact Fund  
Janelle and Buck Blessing  
Balbi A. Brooks  
Darlene and Arnold Brown  
Children's Hospital Colorado  
Rick and Pamela Crandall  
Marguerite Childs and Thomas Detmer, Jr.  
Beverly and Dennis Christine  
Jill Cowperthwaite and Charles Jones  
Lois Ferguson  
Lauren and Neal Finch  
Cathy and Thomas Finch  
Stephanie Foote and Lloyd Wade  
Yvette and Chris Frampton  
Faye and Michael Furuglyas  
Patrick Gaines  
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Gates Frontiers Fund  
Gates Grubstake Fund  
Mary Gearhart  
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The Glendorn Foundation

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