



University of Colorado **Anschutz Medical Campus**

# The Gift of Sight

Your Impact on the *CellSight* Ocular Stem  
Cell and Regeneration Research Program

2023

Sue Anschutz-Rodgers Eye Center  
Department of Ophthalmology  
University of Colorado School of Medicine





# Our Heartfelt Gratitude



On behalf of the CU Anschutz Medical Campus, thank you for your investments in a shared vision to bring sight back to life. Without philanthropic support, the *CellSight* program would not exist. Incredible things happen when communities come together in pursuit of a shared goal, and your collective philanthropic support has been integral to the success of *CellSight* and our impact on novel stem cell-based therapeutics.

Since *CellSight* launched in 2017, the program has revolutionized ocular stem cell-based technologies, exceeding its own goals and becoming a global leader in the fight to prevent blindness in diseases such as age-related macular degeneration (AMD). Our researchers have dreamt of developing a retinal transplant containing photoreceptors and retinal pigment epithelium cells to start transplanting it in animal models. Today, that dream is well underway with very promising, preliminary results.

In this report, we highlight some of the ways your philanthropy has kept us on the forefront of innovation and discovery. One significant milestone is the completion of our proof-of-concept studies toward the development of a stem cell-based therapeutic approach to dry AMD. Reaching this important inflection point would not have been possible without dedicated philanthropic support from friends like you.

Our groundbreaking research and innovation have earned our team the top two prizes in the National Eye Institute's 2022 3D Retinal Organoid Challenge (NEI 3D ROC). I am immensely proud of the work we've been doing for several years to bring these organoid technologies to the next level. It is through teamwork that we can achieve such excellence. Because of your collective generosity and trust in our work, we continue to succeed.

Thank you for standing with us in the fight to save and restore sight in patients with blinding diseases. I look forward to our continued partnership and all that we will accomplish together.

A handwritten signature in black ink, reading "Valeria Canto-Soler".

**Valeria Canto-Soler, PhD**

Director, *CellSight* — Ocular Stem Cell and Regeneration Research Program  
Doni Solich Family Chair in Ocular Stem Cell Research



# Our Journey Together

## 2023

- ✓ **CellSight prepares for meetings with the FDA**
- ✓ **Inflection point for transitioning to pre-clinical studies achieved**

## 2022

- ✓ **AMD transplant proof-of-concept studies completed**
- ✓ **AMD animal model established**
- ✓ **CellSight wins the top 2 awards of the NEI 3D Retinal Organoid Challenge (3D ROC) sponsored by the National Eye Institute**
- ✓ **Establishment of the ExoSight Lab under Dr. Flores-Bellver's leadership**



**COMPLETED**

## 2021

- NEI 3D ROC Prize
- Growth and expansion of *CellSight* teams

## 2020

- Colorado OEDIT Advanced Industries Accelerator grant
- Department of Defense grant
- *CellSight* 2020 Initiative successfully accomplished

## 2019

- Phase 2 launched ahead of schedule
- First programmatic goal officially launched
- *CellSight* 2020 Initiative kicked-off

## 2018

- Grubstake Award
- SPARK program
- Preliminary studies toward first programmatic goal

## 2017

- *CellSight* officially launched
- Phase I launched
- Cell culture facility fully operational
- *CellSight* founding teams recruited and established

## 2016

- Dr. Canto-Soler recruited to lead *CellSight*

# Our Progress to Date

## ACCOMPLISHMENTS



### PATENTS

**1**

Granted

**13**

Under  
Review



### PUBLICATIONS

**38**

Published



### PARTNERSHIPS

**9**

Academic  
Partnerships

**4**

Industry  
Partners



### TRAINEES

**48**

Total

## ACCOLADES AND AWARDS



Received top two awards in Phase 3 of the NEI contest to develop retina organoids (2022)

Received award in Phase 2 of the NEI contest for the research team's work to create better models to accelerate the development of new therapies for retinal diseases (2021)

# Our Launchpad to Success

Your generous support and belief in the importance of our work is crucial to our success. Without your invaluable philanthropy, *CellSight* would not have come to be, and without *CellSight*, there would be no dream of restoring vision in patients. Prior to 2021, your support provided ...



## Surgical delivery instruments

*CellSight* collaborated with medical device companies to design the best surgical instruments for delivering retinal transplant in human patients.



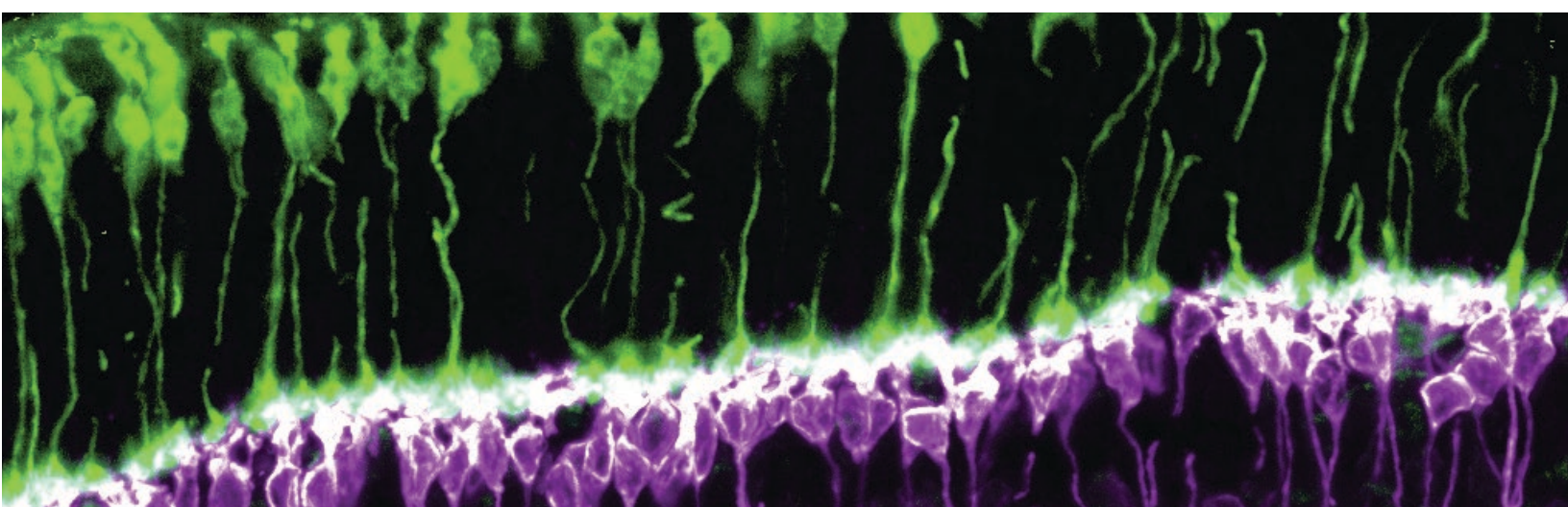
## Imaging technologies

*CellSight* also worked with ophthalmic imaging companies to create non-invasive technologies for evaluating how the eye with transplanted retinas is progressing and to test the ability of the transplant to transmit signals to other cells in the host retina.



## Immune-suppression regimen

For successful human implantation, the team must be sure that the host's immune system does not reject transplanted retinal tissue. For that, they need to be able to fine-tune the immune response. The team tested several combinations of immune suppressant drugs in its animal models, with positive results.



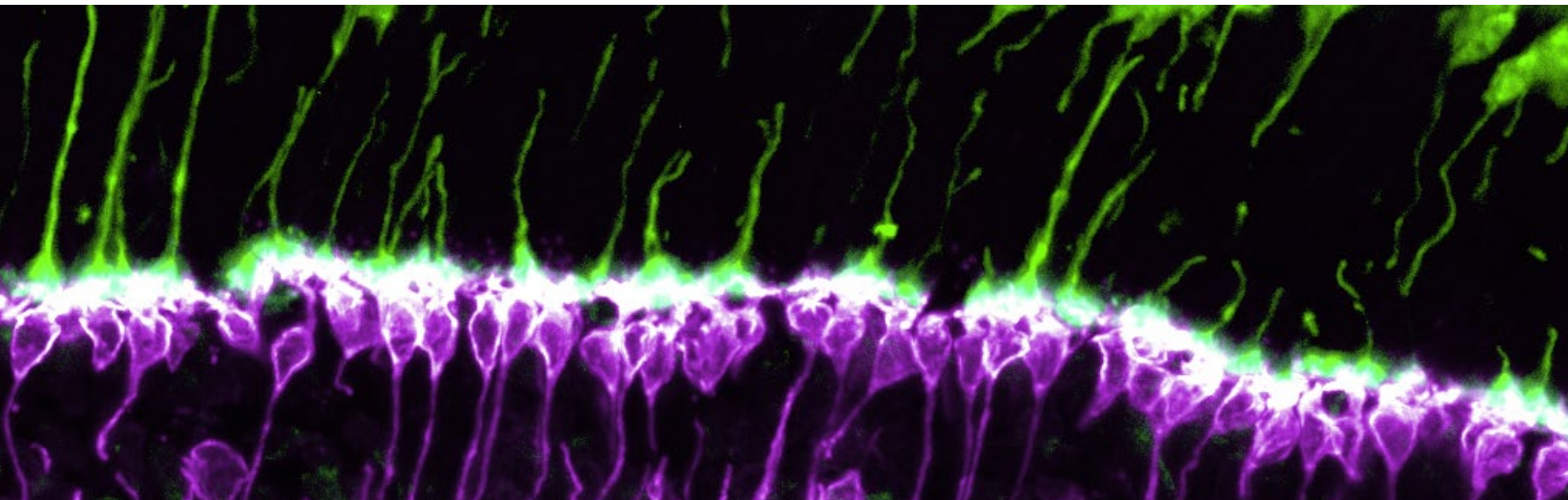
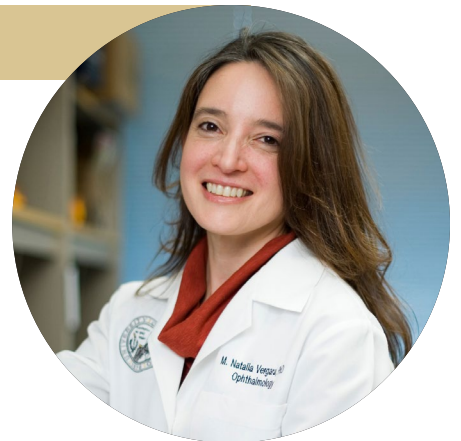
# 2021-2022

In 2021-2022, we competed in the NEI Retina Organoid Challenge and took home the top two prizes. This is the second time the *CellSight* team has placed in NEI's 3D Retinal Organoid Challenge in recognition of their work to develop stem-cell technologies to cure retinal diseases.

- *CellSight* Director **Valeria Canto-Soler, PhD**, led the team that won the NEI 3D ROC's disease modeling category, earning \$500,000. Canto-Soler's research group created a three-dimensional retinal model that recreates pathological features of age-related macular degeneration, with the ultimate goal of discovering new treatments for this blinding disease.

## Natalia Vergara, PhD

Natalia Vergara, PhD, director of *CellSight*'s Ocular Development and Translational Technologies Laboratory, led the research group that won the drug-screening category, earning \$250,000. Vergara and her team expanded their work with their organoid model to evaluate the effects of drug toxicities on the retina and developed a first-of-its-kind organoid model of Alzheimer's disease retinopathy.









# 2022-2023

In 2022-2023, we successfully completed the proof-of-concept phase, finalizing development and characterization of our stem cell-derived retinal transplants, and proving that they can be transplanted in animal models. *CellSight* is at an inflection point as we transition to preclinical studies and look to new innovations.



## **Completed 3D retinal transplant development and formulation**

*CellSight* collaborated with a private company to develop a hydrogel to improve transplant stability during the production process and surgical delivery.



## **Demonstrated a mechanism of action**

The team demonstrated that the cells within the 3D retinal transplant are functional before they are incorporated into their animal model.



## **Designed a route of administration**

The team finished designing surgical instruments that will be used in human clinical trials; these tools are currently being used in the transplant studies into animal models. The tools were developed by the *CellSight* team in conjunction with a private company, and two patent applications are currently under review.



## **Proven in-vivo potency and in-vivo efficacy**

*CellSight* confirmed the cells in the 3D retinal transplant retain their function after having been transplanted into animal models.

# Our Forward Momentum

Due in large part to your philanthropic leadership, the Department of Ophthalmology at the University of Colorado School of Medicine is leading the way in ophthalmology patient care. Without the support of generous benefactors like you, *CellSight* would still be just a dream. Thank you for your dedication to innovation.



We know the work is hard, and we are committed to finding a cure for blindness. Because of your support, we continue to deepen our understanding of ocular stem cell and regeneration research, bringing treatment an inch closer to prevention and cure.

As we look to the future of *CellSight*, we plan to initiate pre-clinical studies in 2024 as a step toward filing an investigational new-drug application (IND). This is a request from a clinical study sponsor to obtain authorization from the FDA to administer an investigational drug or biological product to humans — a major milestone for the program.

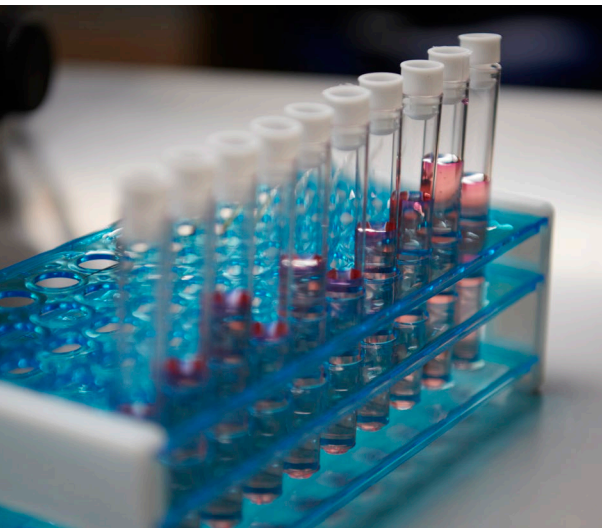
We hope you share in our pride at these incredible accomplishments and all they make possible for patients now and in the future. Thank you for your partnership in making this possible.

With gratitude,

A handwritten signature in black ink, appearing to read 'Naresh Mandava'.

**Naresh Mandava, MD**

Sue Anschutz-Rodgers Endowed Chair in Retinal Diseases  
Professor and Chair, Department of Ophthalmology  
Director, Sue Anschutz-Rodgers Eye Center







## Vision

### Bringing Sight Back to Life

*CellSight* is a cross-disciplinary team of research scientists and clinicians working in a highly collaborative environment with a common goal: to develop novel stem cell-based therapeutics to save and restore sight in patients with blinding diseases.

## Mission

### Catalyzing Stem Cell Innovations to Treat Ocular Diseases

*CellSight* integrates several independent research groups that have come together as a unified team to find solutions to complex ocular diseases. In a concerted effort, the investigators at *CellSight* work with clinicians and scientists at the Sue Anschutz-Rodgers Eye Center and the Gates Institute for Regenerative Medicine, with a goal of translating innovative treatments from the laboratory bench to the patient bedside.



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