

University of California San Francisco



## F Innovation UCSF: Unique Within University of California System

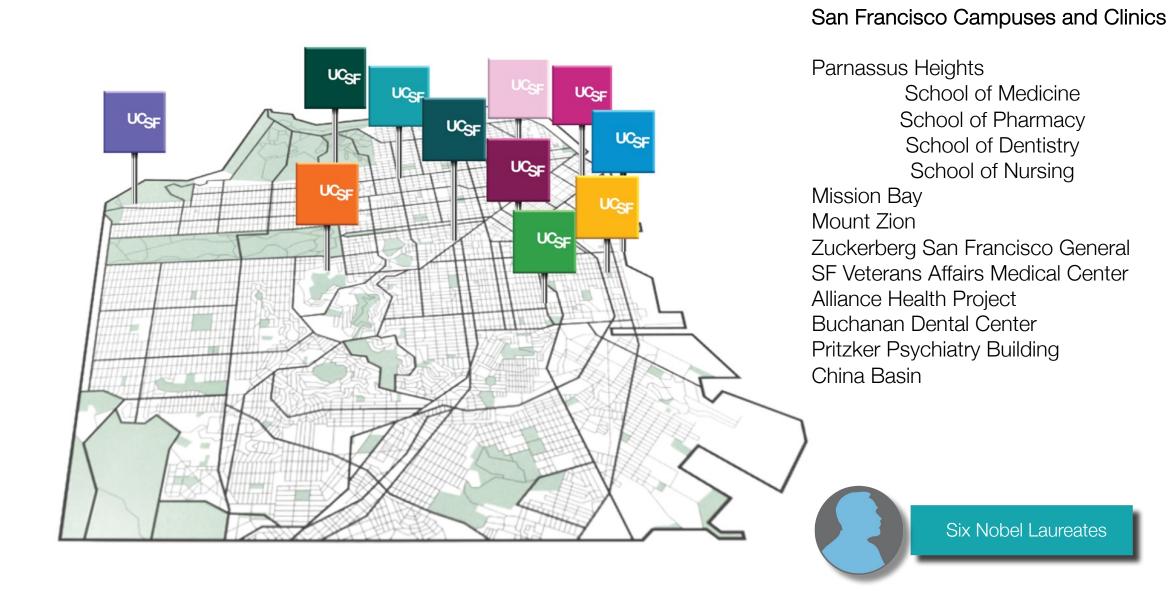
# UCSF is a purely a health sciences university

- 4 professional schools: medicine (MD), dentistry (DDS), nursing (RN) and pharmacy (PharmD)
- Graduate Division has 20 PhD programs and 12 Master's programs
- ~3000 faculty
- ~3000 students (professional and graduate)
- ~3000 trainees (residents and postdoctoral fellows)
- UCSF Health: 18,000 staff and physicians, 1,290 beds, admits 41,000 patients and has over 2.5 million outpatient visits per year, annual revenue of \$5B



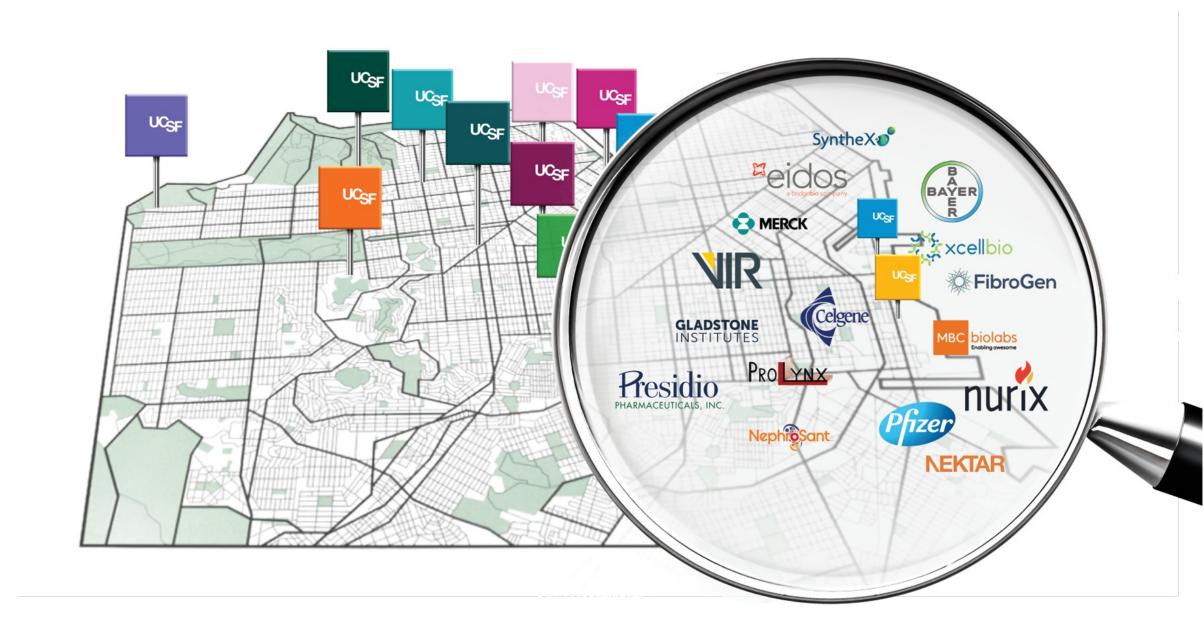


#### University of California San Francisco





## Innovation Ecosystem at UCSF Mission Bay





#### UCSF in the Al era



Sam Hawgood, MBBS

Chancellor, Office of the Chancellor Arthur and Toni Rembe Rock Distinguished Professor

# UCSF Leading Health Care Al's Revolution

UCSF has several campus-wide Al initiatives to become **THE** Al-enabled academic life sciences university





## UCSF Leading in Health Care Al's Revolution



Atul Butte, MD, PhD

Bakar Institute Director; Priscilla Chan & Mark Zuckerberg Distinguished Prof; Chief Data Scientist for UC Health

"It's fair to say UCSF has always been in the business of building tools that don't exist today. It's going to be no different for artificial intelligence."





#### UCSF is a Leading Academic Medical Center



Catherine Lucey, MD, MACP Executive Vice Chancellor and Provost, UCSF

"UCSF is a leading center of biomedical education, clinical and basic science research. We need to be thinking across all of our mission areas and schools to meet the health care challenges of our time."





#### UCSF Innovation in Numbers



200+ startups

\$594M raised in 2023

191 Licensing deals (2023)

2970 Patents

2133 Active Inventions

100+ Products to Market

\$50M+ in Licensing Revenue

\$823M in NIH funding





#### Industry Contracts Division



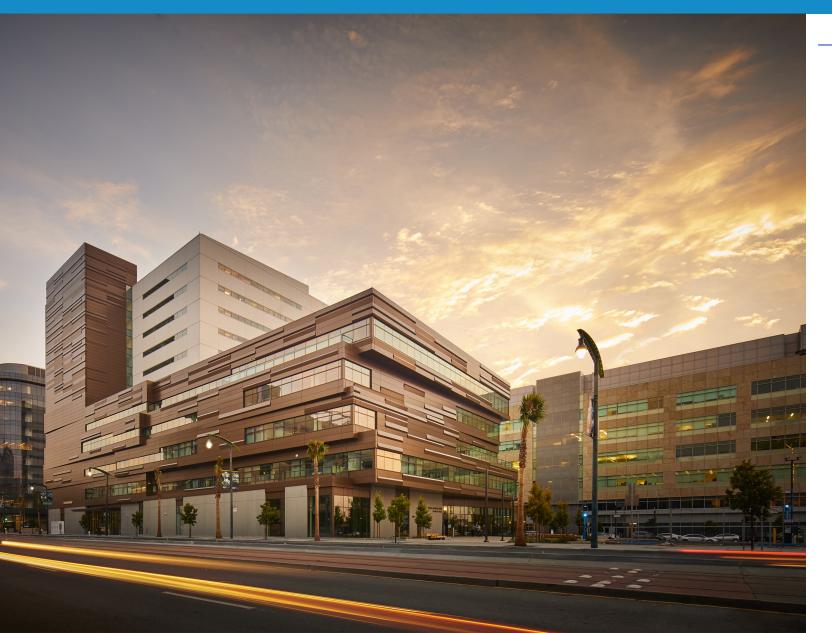
Our experienced team provides comprehensive guidance during contract negotiation and throughout project lifecycles... allowing you to focus on what you do best

Groundbreaking Research





#### UCSF Innovation Ventures



## We help with

Entrepreneurial Education

Technology Advancement

Transformational funding

Licensing

Strategic Partnerships

Startup Creation





#### UCSF Innovation Ventures



Peter Kotsonis, PhD
Interim Vice Chancellor of Business Development

Dedicated to helping the UCSF community navigate the complexities of translational technology development by working up-close with our research faculty, promising students, and industry partners to really understand the science and how it can be applied to healing.





### Strategic Alliances at UCSF



Peter Kotsonis, PhD
Interim Vice Chancellor of
Business Development

#### A partner of choice

- Identify and build strategic partnerships between UCSF and industry
- Develop contractual and business models
- Incentivized with a shared risk/reward structure
- Provide alliance management expertise





## Strategic Alliances Team at UCSF



Jon Brown, PhD Alliance Development Manager



Amy Gryshuk, PhD Associate Director



Gabriela Fernández-Cuervo, PhD Alliance Development Manager



Olivia Roberson, PhD Senior Alliance Manager



Aleksandra Kijac, PhD Alliance Development Manager



Aenor Sawyer, MD Senior Alliance Development



Inder Takhar Project Coordinator



Sean Karlin
Senior Brand and
Communications





## Catalyzing Innovation at UCSF



Charles Hart, PhD
Executive Director, Catalyst Program



Roopa Ramamoorthi, PhD Director, Catalyst Program & InVent Fund



Nathaniel Prorok, MHA
Director, LaunchPad &
Senior Program Manager, Catalyst



Sohela Shah Program Director Catalyst & Invent Fund

- Seed funding up to \$100k through Catalyst Program
  - Up to \$1M per project through InVent Fund
    - Expert Industry Advisors
    - NewCo Mentorship and Guidance
    - 'Entrepreneur in Residence' Program





## Technology Management and Advancement at UCSF



Todd Pazdera, PhD
Director, Business Development,
EOD & Licensing



Gonzalo Barrera-Hernandez, PhD Director, Business Affairs & Strategic Partnerships





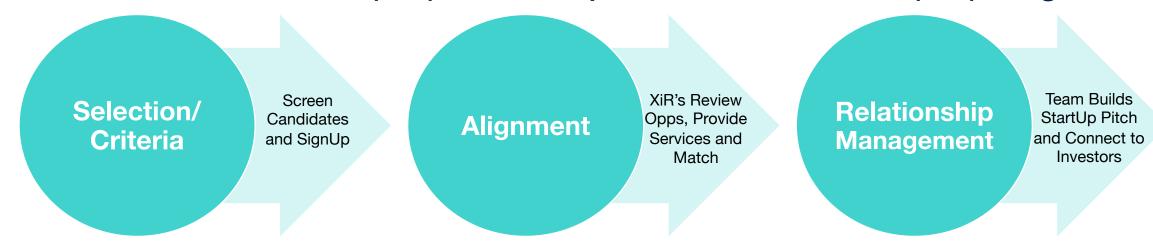
#### Technology Management and Advancement at UCSF

# Translating UCSF ideas into new companies and commercial products to benefit patients

- Evaluate and grow new ideas
- Assess market, develop business models
- Connect with industry and KOLs

- Build startups and pitch to investors
- Develop Intellectual Property Strategy
- Value technology
- Negotiate licensing deals

#### Executive in Residence (XiR) and Entrepreneur in Residence (EiR) Programs





## Entrepreneurship at UCSF



# Stephanie Marrus, MBA, MA Managing Director of Entrepreneurship

Managing Director of Entrepreneurship Innovation Ventures

# Exposing academics to entrepreneurial experience

- Exploring the commercial potential of your ideas
- Translating them into a business plan
- Connecting to accelerators and funding sources
- Global Startup Course in 36 countries





### UCSF partners with Autobahn Labs



Pamela England, PhD UCSF Professor



#### Drug Discovery Accelerator Bringing

- Expertise
- Global drug discovery technology and capabilities
- Financial capital
- > 3 Options to license from UCSF
- > 1 Licensed technology from UCSF







Jon Brown, PhD
UCSF Business Development and Alliance Manager











#### UCSF as a founding academic collaborator of PICI



Kole Roybal, PhD
Director of UCSF PICI



Cancer research and breakthrough immune therapies accelerator

- Brings top researchers together
- Provides resources
- Eliminates barriers







Olivia Roberson, PhD
UCSF Sr. Alliance and Business
Development Manager











### UCSF partners with Bristol Myers Squibb





Industry and Academia Collaboration focused on development of next-generation, recombinant antibody-based cancer therapies





### UCSF partners with Bristol Myers Squibb



Jim Wells, PhD
UCSF Co-founder of RAN

# Bristol Myers Squibb





"This is a spectacular example of how industry and academia can work hand-in-hand to discover new medicines"

> Jim Wells, PhD UCSF RAN Lead







Gabriela Fernández-Cuervo, PhD

UCSF Alliance and Business Development Manager









## UCSF partners with Foundery Innovations



Max Krummel, PhD UCSF Professor



# Accelerator for transformative immunotherapies

- Immunotherapy venture studio
- Focus on company creation
- Efficient validation and translation of early drug concepts







Aleksandra Kijac, PhD
UCSF Business Development and
Strategic Alliance Manager









## UCSF partners with Eli Lilly and Company



Max Krummel, PhD UCSF Professor



# Working toward new insights into autoimmune diseases

- Proteomic, transcriptomic, epigenomic and structural data
- Freshly collected tissue
- Matched peripheral blood samples
- Clinically well-annotated patients







Gabriela Fernández-Cuervo, PhD UCSF Alliance and Business Development Manager

Lilly









#### UCSF & HS Progress



Haley Naik, MD, MHSc, FAAD UCSF Co-founder

## HS PROGRESS

The <u>Hidradenitis Suppurativa</u>

PRospective Observational REgistry

and bioSpecimen repoSitory

- Multicenter
- Longitudinal

Mission to improve the lives of people living with Hidradenitis Suppurativa







Peter Kotsonis, PhD
UCSF Interim Vice Chancellor of Business Development

## HS PROGRESS

Mission to improve the lives of people living with Hidradenitis Suppurativa





#### The Weill Neurohub



University of California San Francisco





## Weill Neurohub

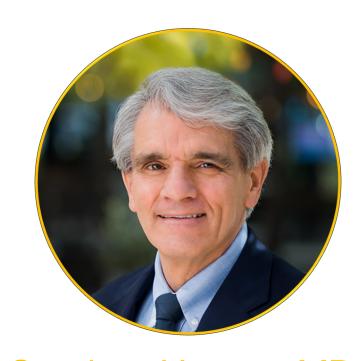
Accelerating the development of new treatments for neurological and psychiatric disease

- Seed funding for novel research ideas
- Interdisciplinary and collaborative projects
- Focus on near-term transformational potential





#### The Alliance for Therapies in Neuroscience



Stephen Hauser, MD

Director of UCSF Weill Institute for Neuroscience





## Weill Neurohub

Accelerating the development of new therapeutics

- Long-term research partnership
- Centered on brain diseases and disorders of the central nervous system (CNS)







Jon Brown, PhD
UCSF Business Development and Alliance Manager







Weill Neurohub





## Arc Institute Accelerating Research



Silvana Konermann

Executive Director and Core Investigator



Patrick Hsu

Co-Founder and Core Investigator

## **Arc Institute**

#### Nonprofit research organization

- Curiosity-driven and goal-oriented research with a focus on complex diseases, including neurodegeneration, cancer and immune dysfunction.
- Operates in collaboration with Stanford University, the University of California, Berkeley, and the University of California, San Francisco.





Amy Gryshuk, PhD

Associate Director, Strategic Alliances UCSF Innovation Ventures



Gemma Rooney, PhD

Assistant Director, Strategic Partnerships & Licensing, UCSF Innovation Ventures

## **Arc Institute**









# Chan Zuckerberg Biohub Network Driving Disruptive Innovation



Steve Quake, D.PHIL.

Head of Science, Chan Zuckerberg Initiative



Joe Derisi, PhD
President,
CZ Biohub San Francisco



Group of nonprofit research institutes bringing together scientists, engineers, and physicians

- Goal of pursuing grand scientific challenges over a 10-15 year timeframe.
- Focuses on understanding the mysteries of the cell and how cells interact within systems as well as developing new technologies leading to diagnostics and therapies.



## Want to know more?



Gemma Rooney, PhD
Assistant Director, Strategic Partnerships &
Licensing, UCSF Innovation Ventures











## UCSF & Laboratory for Genomics Research (LGR)



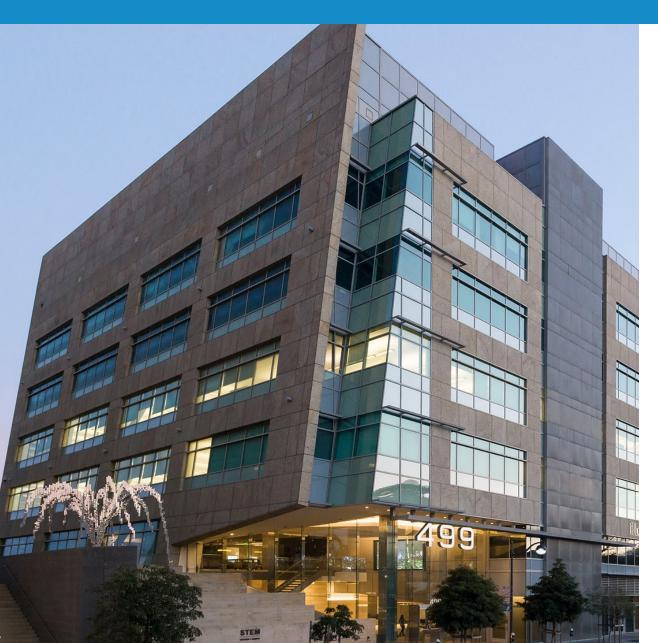


The aim is to deepen our understanding of genetics, discover new targets, and create next-generation technologies at scale that will become future standard practice for the pharmaceutical industry.





## UCSF partners with GSK





Next-generation CRISPR-based technologies at scale

- Develop and optimize new CRISPR technologies
- Invest in automation platforms for large-scale CRISPR screens
- Deepen our understanding of genetics and discover new targets





## Want to know more?



Amy Gryshuk, PhD
UCSF LGR Alliance Manager
UCSF Associate Director, Innovation Ventures













## About SOM Tech



## **BROAD TECHNOLOGY EXPERTISE**



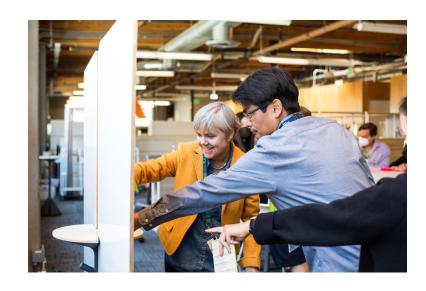
DEEP KNOWLEDGE OF THE UCSF ECOSYSTEM



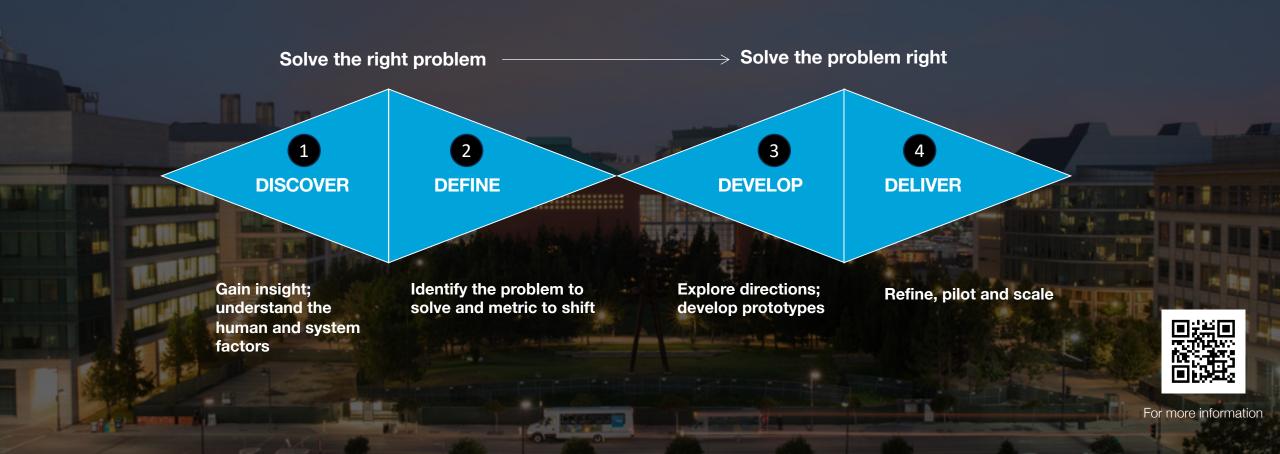
HUMAN-CENTERED APPROACH



SOM Tech provides leadership and advocacy across the School of Medicine's technology spectrum, from research to program incubation to data security.



# The UCSF Clinical Innovation Center aims to accelerate innovations to solve the most critical care delivery issues.





Departments of Surgery and Bioengineering & Therapeutic Sciences

## The Mission

To accelerate the translation of pioneering medical devices to improve patient care by lowering the barriers for surgeon-innovators. Focusing on value-based solutions with market viability, and Educating trainees in interdisciplinary collaboration and translation

## The Team



Hanmin Lee, MD Clinical Lead



Shuvo Roy, PhD **Engineering Lead** 



Usha Thekkedath, MD Admin Director

## The Programs

- Weekly Innovators Forum
- Biodevice Innovation Fellowship Program
  - Two-year mentored research experience for surgical residents
- UCSF/UCB Masters of Translational Medicine
- Project Consultation for Faculty, Trainees, and **UCSF-Affiliated Companies**



**Learn More and Contact Us Here:** 

# MASTER OF TRANSLATIONAL MEDICINE

Translating biomedical discoveries into clinical reality.

The MTM program is a joint program between UC Berkeley and UCSF.













We make getting to market easier for entrepreneurs by offering insider knowledge, mentorship, connections and access to funding







Join Our 8th Annual

## Rosenman Symposium

Innovating Healthcare Access: A New Era for the Future

Attend



Keynote speaker:

**Nick Kristof** 

Two-time Pulitzer
Prize-Winning
Journalist, Author,
& Opinion
Columnist for The
New York Times





May 21-22, 2024 | Mission Bay

## **AW24**

UCSF ALUMNI WEEKEND | APRIL 12-13, 2024

## Celebrate with us at UCSF's Mission Bay campus

Join fellow UCSF alumni at Alumni Weekend 2024



Point your mobile device camera at the QR code to visit alumni.ucsf.edu/aw.



Health Innovation via Engineering

engineering.ucsf.edu

## **Completing the Circle of Innovation: Biology for Engineering**

Robotics for High Throughput Science



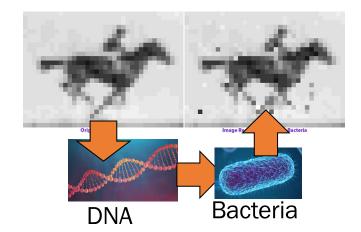




Medical Implants



Recording Video in DNA



**Living Building Materials** 





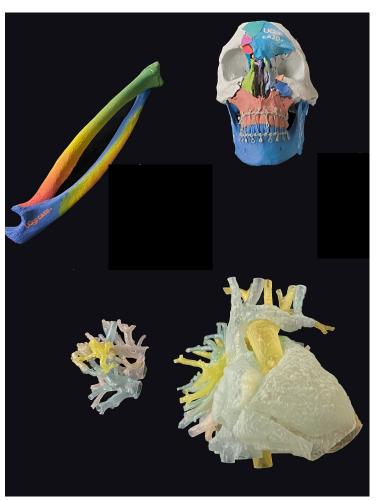




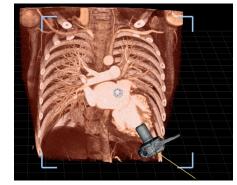
## UCSF Center for Advanced 3D+ Technologies (CA3D+)



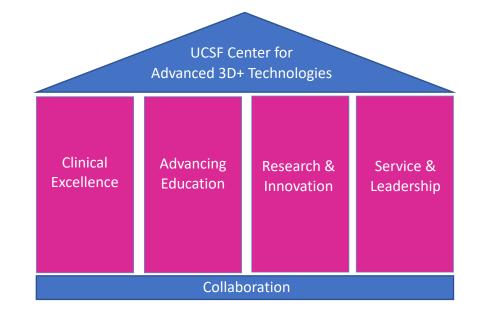




Virtual surgical planning



Advanced visualization
Augmented/Virtual/Mixed Reality





https://ca3dplus.ucsf.edu/



## The Mission

To improve the health, safety, and quality of life of pediatric patients by accelerating high-value, high impact pediatric device solutions at all stages of the total product lifecycle towards commercialization.

## The Team @ UCSF



Hanmin Lee, MD Clinical Lead

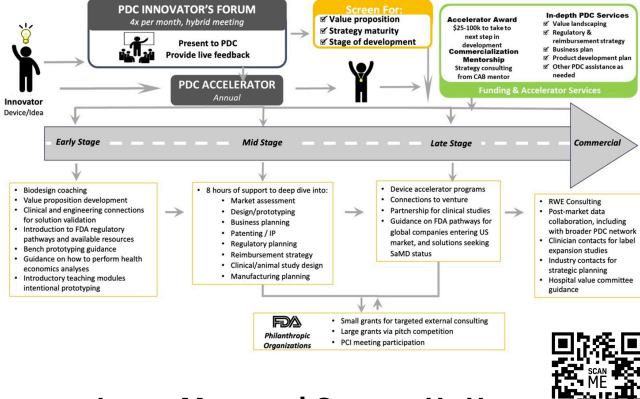


Shuvo Roy, PhD Engineering Lead



Usha Thekkedath, MD Admin Director

## The Process



**Learn More and Contact Us Here:** 



## Targeting Mucus Plugs to Improve Lung Health





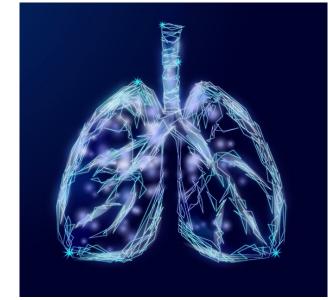
John Fahy, MD, MS
Co-founder, Aer
Therapeutics
UCSF Pulmonologist and
Innovator

## PROBLEM:

- Between 30 and 50% of COPD patients with severe and very severe COPD suffer from airway obstruction caused by mucus plugs.
- Mucus plugs reduce lung function and diminish quality of life.
- There are no drugs approved to effectively liquify mucus plugs (mucolytics) in patients with COPD.

## **SOLUTION:**

 AER-01 is a novel inhaled best-in-class therapeutic candidate designed to improve lung health by liquifying mucus plugs.



 AER-01 is a thiol-modified carbohydrate ("thiol-saccharide") which cleaves mucine disulfide bridges to liquefy ("lyse") mucus plugs.

- >\$18M in NIH funding
- \$36M Series A





We drive innovation in oncology and solid organ diseases through development of organ selective therapy for early interception and treatment of the prostate disease

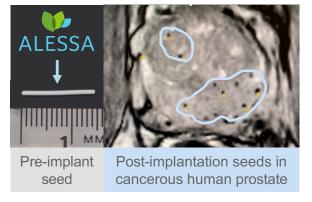


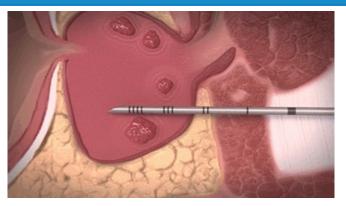


Pamela Munster, MD CEO and Co-founder, Alessa Therapeutics UCSF Professor of Medicine and Innovator

## PROBLEM:

- Some 1 in 6 men will be afflicted with prostate cancer during their lifetimes, 30k will die every year.
- 12M men in the US seek treatment for benign prostate hyperplasia every year.
- Current therapies mainly centered around systemic testosterone ablation.





## **SOLUTION:**

- Implant and delivery systems for localized, sustained drug delivery without systemic side effects.
- Focused on treatment of localized prostate cancer and BPH.
- Robust pipeline of target specific organ selective strategies.

## TRACTION:

- Alessa Therapeutics continues patient Enrollment in Biolen®+RT Study with National Cancer Institute
- Alessa Therapeutics Announces Research Collaboration with Janssen
  - Alessa about to start clinical trial with Enolen in Q1 2024

LEARN MORE:





## Programmable Cell Therapy to Defeat Solid Tumors



## **UCSF Co-founders**



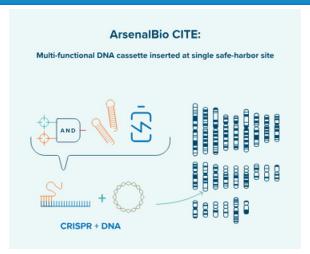


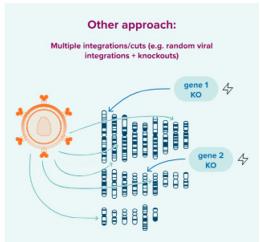
Alexander Marson, MD, PhD Co-founder, ArsenalBio UCSF Professor and Innovator

Kole Roybal, PhD Co-founder, ArsenalBio UCSF Professor and Innovator

## PROBLEM:

 Solid tumors are complex and refractory to most treatment regimens.





## **SOLUTION:**

 Deploying the combination of CITE editing, a toolkit of synthetic receptors for tumor recognition and a combination of T cell enhancements to improve therapeutic activity.

## TRACTION:

- Dose escalation underway with AB-1015 in Phase 1 clinical trial in ovarian cancer. Second program in kidney cancer, AB-2100, enrolling
- Collaborations with BMS and Genentech
- >\$500M in Equity Funding and Revenues



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## BRAYVE<sup>TM</sup>: The Smartphone Technology Platform for Radiation Oncology





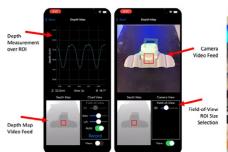
Tomi F. Nano, PhD, DABR Co-founder, Bravye<sup>TM</sup> UCSF Assistant Professor of Radiation Oncology, Medical Physicist

Dante Pl. Capaldi, PhD, DABR Co-founder, Bravye<sup>TM</sup> UCSF Assistant Professor of Radiation Oncology, Medical

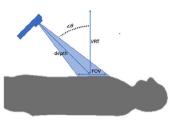
## PROBLEM:

- Only 30% of clinics have access to surface breath-hold systems and patients have no effective way of practicing their breath-holds.
- This more than doubles the chances of cardiac toxicity during radiation treatment.

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## **SOLUTION:**

• BRAYVE is a smartphone technology platform for radiation oncology and radiology that empowers clinics to more effectively treat cancer patients undergoing breath-hold, while also giving patients access to the worlds-first at-home device that will improve their breath-hold consistency.

- Provisional patent approved, full patent pending
- MVP 80% complete and technology validated against current systems







## Reimaging Genome Regulation





Luke Gilbert, PhD Co-founder, Chroma Medicine UCSF Professor and Innovator

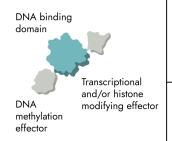
## PROBLEM:

 To build single dose therapeutics that durably control expression of human genes.

## **SOLUTION:**

- Single-dose genomic medicines that harness epigenetics for durable and heritable gene silencing.
- A modular platform for epigenetic editing to address a wide range of complex diseases.

## Chroma Epigenetic Editor



### Gene Silencing

Methylate to silence gene expression

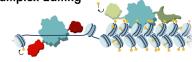


### **Gene Activation**

Demethylate to activate gene expression



### Multiplex Editing



Modify multiple genes simultaneously

- Chroma Medicine Presents Preclinical In Vivo Data Showing Durable Cholesterol Reduction with a PCSK9-Targeted Epigenetic Editor at the 2023 AHA Scientific Sessions
- >\$250M in Funding



## Curaidh Bio

## Targeting Tumor Immortality by Degrading the Cancer-Specific Regulator of TERT Expression

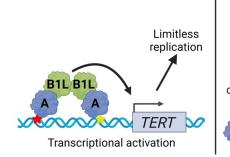


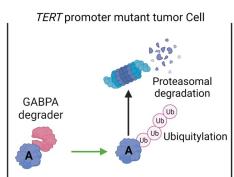


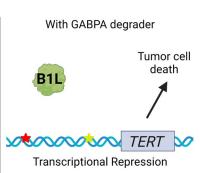
Joseph F. Costello, PhD Co-founder, Curaidh Bio UCSF Professor and Karen Osney Brownstein Endowed Chair in Molecular Neuro-Oncology

## PROBLEM:

- Mutation in the TERT promoter enables unlimited cell proliferation for over 50 cancer types, including glioblastoma.
- The SOC for glioblastoma has remained unchanged for decades and the average survival rate is 15 months.







## **SOLUTION:**

- GABP degrader reduces TERT expression in tumor cells harboring the TERT promoter mutation.
- Reduced TERT causes a shortening of telomeres in cancer cells and improves survival in an orthotopic xenograft mouse model of GBM.
- Replicating retroviral delivery system only infects dividing cells and will be used for cancer specific delivery of the degrader.

## TRACTION:

Provisional patent filed. Publication under revision at Nature Genetics.







## Immune Therapy to Extend Healthspan

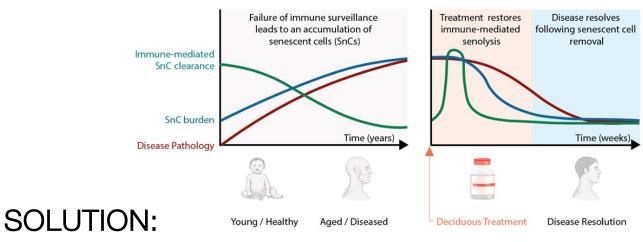




Anil Bhushan, PhD Scientific Co-founder, Deciduous Therapeutics UCSF Professor and Innovator

## PROBLEM:

- Killing pathologic senescent cells improves many preclinical agerelated disease models.
- Identifying a target that is safe for systemic administration remains a challenge.



- Deciduous eliminates senescent cells by re-activating the failed immune system's surveillance mechanism in diseased patients.
- A single systemic dose improves endpoints in a pulmonary fibrosis preclinical model, as well as a diet-induced obesity metabolic disease model in under two weeks.

- >\$18M in funding
- Mechanism discovery published in *Med* titled,
   "Invariant natural killer T cells coordinate removal of senescent cells"







## Rapid Pathogen Detection by Metagenomic Next-Generation Sequencing



### **UCSF Co-founders**



Joe DeRisi, PhD UCSF Professor



Michael Wilson, MD UCSF Professor



Charles Chiu, MD, PhD UCSF Professor

## **SOLUTION:**

- Agnostic, broad-based detection of pathogens that cause neurologic infections from cerebrospinal fluid.
- Proprietary bioinformatics analysis platform.

## PROBLEM:

- 60% of Meningitis and Encephalitis (ME) cases are due to infection.
- 50% of ME cases are undiagnosed.
- Conventional testing is limited, targeting only a handful of suspected pathogens at a time and delaying effective treatment in critically ill patients.

## TRACTION:

- \$35M Series A funding
- Charles Chiu receives Research or Leadership in Clinical Microbiology Award from the American Society for Microbiology
- Delve partners with US CDC in a Nationwide Fungal Meningitis Outbreak Investigation





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## DIATIRO

## Improving patient outcomes, increasing utilizable organs, and extending transplant longevity





Keith Hansen, MD Co-founder and CEO, Diatiro UCSF General Surgery Resident and Innovator





## PROBLEM:

- 30% of patients with a kidney transplant go back on dialysis after transplant.
- Thousands of marginal donor kidneys are discarded each year.
- Warming injury to the kidney during sew-in drives these poor outcomes, and the standard of care (ice and gauze) is ineffective.

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## **SOLUTION:**



- The Kidney Pod keeps the donated kidney at the optimal temperature during sew-in, **eliminating warming injury.**
- Gives surgeons the confidence to **Say Yes** to more marginal kidneys.
- Platform cooling technology applicable to cardiac surgery and all organ transplants.

- \$1.6M in pre-seed funding (UCSF Catalyst, UCSF PDC Stanford Medicine Catalyst)
- National Kidney Foundation Innovation Fund Investment
- FDA Breakthrough Device Designation (2022)







## Novel Target Platform for Metabolic Inflammation, Fibrosis and Inflammasome Pathologies





Adam Renslo, PhD
Co-founder, Elgia Therapeutics
UCSF Professor and Associate
Dean for Entrepreneurship





## PROBLEM:

 Dramatic rise in the incident of chronic inflammatory diseases presents a global health burden.



Extraordinary Biology Insight

Intersection of untapped biology, unique MOA, efficacy, and safety



Disease and Drug
Discovery Depth

Exceptional foundation for drug discovery from combined experience of leadership



Highly Enabled
Drug Development

Advanced chemical matter, SBDD and clinical know-how to move assets quickly



Expansive Pipeline Opportunities

Multiple clinical applications for metabolic, inflammatory and fibrotic diseases

## **SOLUTION:**

- Elgia Therapeutics targets caspase-1 for hidradenitis suppurativa as initial indication.
- Our novel active-site, targeted covalent and allosteric inhibitors disrupt key cellular processes involved in metabolic, inflammatory, and fibrotic diseases.

## TRACTION:

• ~\$5M in seed funding to date.





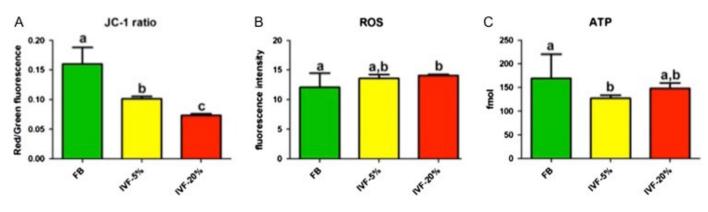


## Improving IVF Success with Advanced Embryo Selection Technology





Paolo Rinaudo, MD, PhD Cofounder, EmbryoDx Solutions Obstetrics/Gynecology and Reproductive Endocrinologist at UCSF



## PROBLEM:

 Inability to identify the healthiest embryos to transfer, leading to low success rates of IVF and need for multiple IVF cycles.

## **SOLUTION:**

- Novel biomarkers indicative of embryonic health, laying the foundation for a safe and reliable device tailored for embryo selection.
- State-of-the-art technology that has the potential to dramatically increase IVF success rates.

## STATUS:

Spinning out



## EN CELL IN

## Advancing next generation therapies, starting with endocrine disorders.





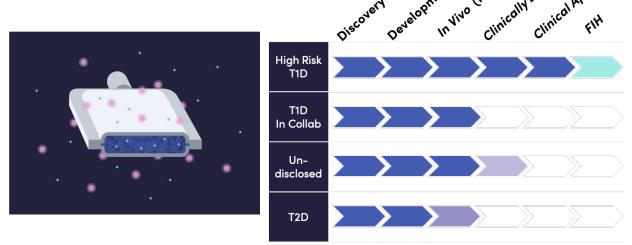
Crystal Nyitray, PhD
Co-Founder Encellin
Founder, Inventor
UCSF, Sanofi, YCombinator

## SOLUTION:

- Encapsulated Cell Replacement Therapy (EnCRT).
- Encellin's EnCRT allows enclosed cells to function like smart molecular factories, releasing therapeutics when needed.

## TRACTION:

• ~\$10M in funding 2023



peutics LEARN MORE:



## PROBLEM:

- Next generation therapies to help patients with chronic unmet clinical needs.
- Starting with endocrine disorders.



## Pioneering Covalent Biologics

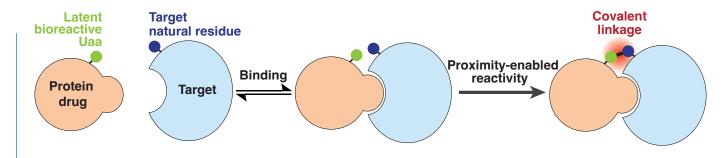




Lei Wang, PhD Inventor, Enlaza Therapeutics UCSF Professor and Innovator

## PROBLEM:

- Proteins bind target reversibly.
- Most therapeutics still suffer from low tumor retention and high off-target toxicity.



## **SOLUTION:**

- New generation of covalent 'War-Lock<sup>TM</sup>' biologics
- Proprietary unnatural amino acids.
- Protein drugs derived from the platform can be modified to incorporate various payloads, creating antibody-drug conjugates (ADCs) or radioligand therapies (RLTs) with specific target tissue delivery, without the need for half-life extension engineering.<sup>1</sup>

## TRACTION:

\$61M in Funding







## Expanding Protein Degradation to Membrane and Extracellular Targets

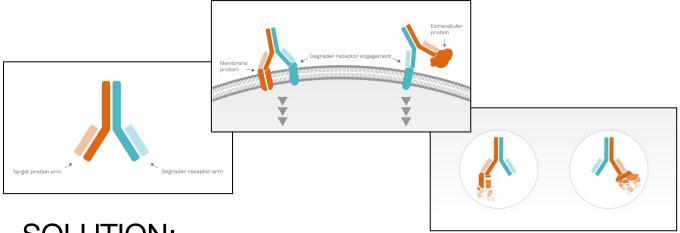




Jim Wells, PhD
Co-Founder,
EpiBiologics
Director, Small Molecule
Discovery Center (SMDC)
Director, Antibiome Center
UCSF Innovator

## PROBLEM:

- First generation protein degradation approaches target intracellular proteins only.
- 40% of the proteome is unaccounted for.
- Better targeted therapies are still needed.



## **SOLUTION:**

- EpiTAC platform enables the development of targeted therapies.
- Targeted degradation of disease-driving membrane and extracellular proteins.
- Solution has applications in cancer, immunology and neurological diseases.

## **TRACTION:**

>\$70M in funding 2023

LEARN MORE:





## Redefining Drug Discovery for Rare Genetic Epilepsies





Scott C. Baraban, PhD Co-founder, Epygenix Therapeutics Professor, William K. Bowes Jr. Endowed Chair in Neuroscience Research UCSF Innovator

## **PROBLEM**

- 30-40% of epilepsy is caused by genetic mutation.
- Most genetic epilepsies are pharmacoresistant, emerge early in life & are lifethreatening.
- Existing antiepileptic medications were not identified using genetic epilepsy models.





## SOLUTION

 'Aquarium-to-Bedside' drug discovery using genetically modified zebrafish models in highthroughput phenotype-based drug screening.

- >\$35M in seed funding
- Six drug candidates licensed from UCSF w/ method-of-use and formulation IP
- Compassionate-use and OLE data
  - On-going ARGUS Phase 2 Potentially Pivotal Study of EPX-100 Treatment of Dravet Syndrome







## Using cell-free RNA and AI to reinvent liquid biopsy and improve cancer patients' lives





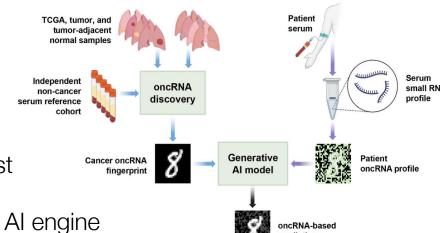
Hani Goodarzi, PhD Co-founder & Scientific Advisor, Exai Bio UCSF Associate Professor and Arc Institute Core Investigator

## PROBLEM:

- Too often, cancer is detected too late.
   Liquid Biopsy holds the promise to improve cancer screening.
- Early progress has been made using DNA biomarkers in the blood, however early stage and small tumor detection remains challenging.

## **SOLUTION:**

Novel RNA
 biomarkers actively
 shed only by living
 cancer cells. Exai
 has amassed the largest
 cfRNA database.



- Highly sophisticated AI engine reveals cancer specific patterns of RNAs in blood.
- Exai's platform can be used across the cancer diagnostics continuum including screening, monitoring and therapy selection.
- Highly sensitive detection of earliest stages of cancer and the smallest tumors.

- Exai launched in 2021 with a \$67.5M Series A funding
- Amassed the largest cfRNA dataset
- Presented data across 8 cancers





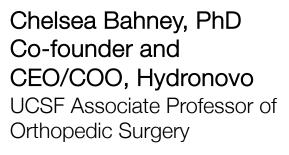


## Regenerate Tissue Through Stem Cell Activation by Restoring Tissue-Nerve Crosstalk





Sarah Knox, PhD
Co-founder and CSO,
Hydronovo
UCSF Professor of Cell and
Developmental Therapy

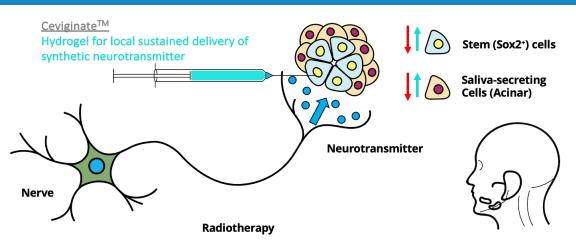




## PROBLEM:

- No regenerative therapies are available to treat xerostomia, damage incurred to salivary glands in the course of radiation treatment for head and neck cancers.
- Preventative treatments show little benefit.

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## **SOLUTION:**

 An injectable neuromimetic hydrogel to regenerate radiotherapy-damaged salivary gland tissue through stimulation of resident stem cells to overcome xerostomia, or dry mouth.

- ~\$6.3M in NON-DILUTIVE funding (NIH and CIRM)
- 505(b)2 Accelerated FDA pathway
- Pre-IND Submission Completed
- U.S.& International Patent Application filed







## Digital Health Contacts to Contracts - Accelerated!





Andrew Auerbach, MD Board Chair, Co-founder Professor of Medicine andy@kuretic.com

## PROBLEM:

- Health Systems face overwhelming risks in adopting new Digital Health tools
  - Costs ~\$80K + weeks for each vendor discovery and diligence
  - Onboarding takes forever
  - No performance monitoring

Novel platform to lower friction between health systems and digital health tool vendors, leading to faster adoption of tools with better outcomes

## **SOLUTION:**

- 100s of proprietary data points for each tool with pre-recorded demos and confidential peer feedback
   → select tools with confidence
- Automated checklists and workflow automation >
   speed up onboarding
- Performance monitoring → improve outcomes

- IP licensed from UC
- Signing up Beta customers



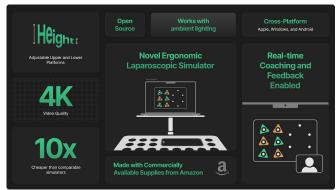
## A Novel Ergonomic Simulator for Laparoscopic Surgery





Tejas Sathe, MD
Co-inventor
UCSF Surgical Innovation
Fellow and Innovator





## PROBLEM:

- 80% of surgeons report musculoskeletal pain due to surgeries.
- Current laparoscopic surgery simulators are either too expensive, non-portable, or nonergonomic.
- Ergonomic simulation training remains inaccessible to most residents.

## **SOLUTION:**

- An affordable, high-fidelity and ergonomical simulator for laparoscopic surgery.
- Simulator utilize wireless video transmission through Apple's Continuity Camera technology that allows for real time broadcasting as the camera captures the working surface and displays real-time video on the laptop screen.
- Cost of materials <\$35 (not including laptop and iPhone).</li>

## TRACTION:

Prototype built and currently used by residents in their skills curriculum

LEARN MORE:





## From genes to phenotype in every research pipeline





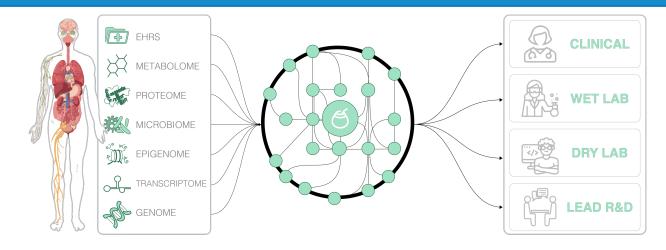
Charlotte Nelson Co-founder & CEO, Mate Bioservices UCSF BMI Alum





## PROBLEM:

- Biological complexity is resisted instead of embraced in biomedical research.
- Data and knowledge silos impede scientific breakthroughs.



## **SOLUTION:**

- Mate's core engine offers unparalleled data access harmonized, normalized, & seamlessly packaged.
- Intuitive explainable AI interfaces for wet and dry lab scientists tackle months of research in minutes.

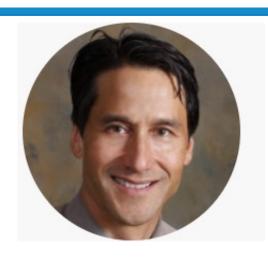
- Supported by the NSF Convergence Accelerator
- Leveraged by NASA, academic institutions, and pharma companies of all sizes, worldwide





## Novel Delivery of Ocular Therapeutics

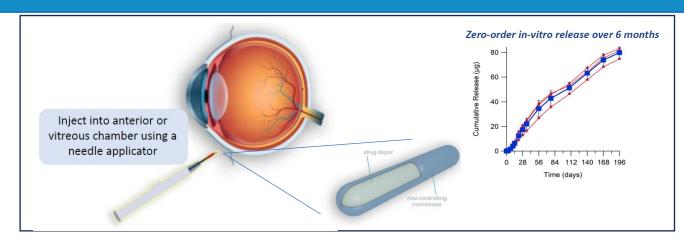




Robert Bhisitkul, MD, PhD
Co-founder, Oculinea
Inc
UCSF Professor of
Ophthalmology
and Innovator

## PROBLEM:

- Adherence with medications is a fundamental problem in the care of glaucoma patients as 24–59% fail to receive the intended treatment.
- Non-adherence to therapy leads to irreversible loss of vision.



## **SOLUTION:**

- Long acting zero-order drug delivery of small molecules & biologics for 6-months or longer. Office based procedure:
   Miniaturized Injectable Delivery System (MIDS).
- Transform treatment for glaucoma & retinal diseases with better clinical outcomes through patient compliance.

- Lead Program: Glaucoma MIDS at IND Enabling Stage
- Accelerated regulatory pathway: FDA's 505(b)(2)
- 12 Issued Patents (Domestic and International)
- Partnerships with major pharmaceutical companies





## Targeted Gene Therapy to Transform the Lives of People Living with Severe Genetic Diseases





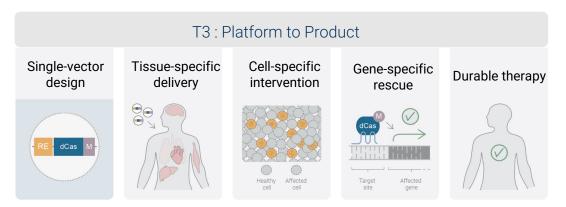
Navneet Matharu, Ph.D.
Co-founder/CSO, Regel Tx
UCSF Assistant (Adjunct)
Professor
IGI-WIES fellow





## PROBLEM:

- Disease modifying therapies for haploinsufficient disorders are lacking.
- Approx 600 such disorders have high unmet need.
- Targeted genetic therapies are needed.



## **SOLUTION:**

- Clinical vector with a dCas module and an engineered enhancer.
- Targeted delivery with a one-time injection in the affected system.
- Restricts the intervention to the affected cells
- The dCas module normalizes the level of gene expression.

### TRACTION:

- Raised 6M seed + BD partnership
- 3 programs under a Research Collaboration and Option Agreement with Sarepta Therapeutics



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# 24-Hour Intraocular Pressure (IOP) Monitoring Preventing Glaucomatous Vision Loss

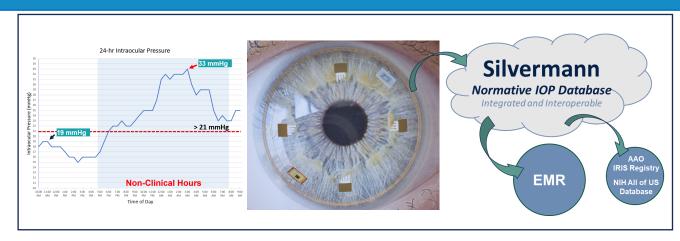




O'Rese J. Knight, MD Co-Founder, SalVista UCSF Associate Professor Ophthalmology and Innovator

## PROBLEM:

- Current methods to diagnose and manage glaucoma are inadequate and represent a significant burden on the healthcare system.
- 24-hr IOP monitoring demonstrated that nearly 80% of patients are under-treated.
- Current delays in diagnosis and under treatment yield unnecessary vision loss.



## **SOLUTION:**

- Mounting self-sensing cantilevers in soft contact lenses unlocks accurate and automated 24-hr IOP monitoring
- Normative database and algorithms enable better diagnosis and decision-making tools for treatment selection

- 3 patents filed or in process
- ~\$1M raised in non-dilutive funding
- Currently in discussions on FDA 510k De Novo





# Developing Live Biotherapeutics Targeting Core Drivers of Disease

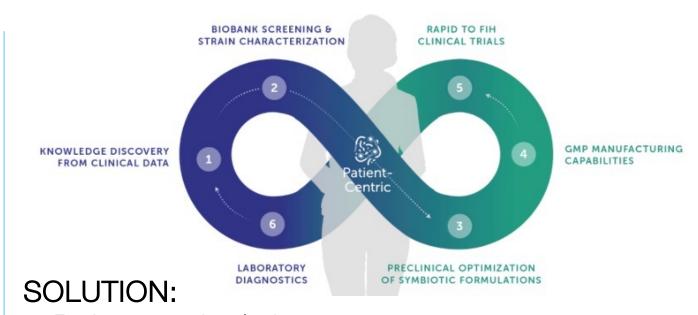




Nikole Kimes, PhD Co-founder and CEO, Siolta Therapeutics UCSF Inventor & PhD Postdoc Alum

## PROBLEM:

- Developing live biotherapeutics that target the core drivers of disease.
- Microbial therapeutics reseeding the depleted gut microbiome to prevent and treat disease.



- Patient-centric platform.
- Microbiome data analysis, machine learning, anaerobic microbiology.
- Optimizes multi-strain live biotherapeutics to prevent/treat disease.

- \$50M in funding
- Awarded multiple NIH grants





# We Bridge The Gap Between Imaging And Reality





Jesse Courtier, MD
Co-founder, Sira Medical
UCSF Chief of Pediatric
Radiology and Innovator

## **SOLUTION:**

 Augmented reality software to help with preoperative planning by providing patientspecific high fidelity 3D holograms.



### PROBLEM:

 Surgeons have difficulty translating radiology information into real world patients for preoperative planning.

### TRACTION:

- Nearly \$1M from grants, accelerators, and VC funding
- Completion of 5 pilots (presented/published)
- IP: developed proprietary software and model creation methods
- Member Microsoft Mixed Reality Partner Program
- · Finalist UCSF Digital Health Award
  - Recently submitted product for FDA 510k clearance

LEARN MORE:



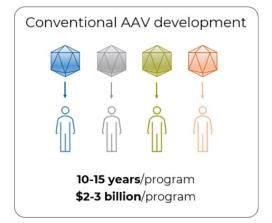


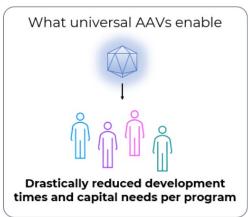
# Universal AAV Immuno-gene Therapy for Cancer





Nicole K. Paulk, PhD CEO, Founder, President Siren Biotechnology Prior UCSF Professor





## PROBLEM:

No effective therapies for brain and eye cancers.

## TRACTION:

 Awarded \$4M in Grant Funding from the California Institute for Regenerative Medicine (CIRM)

## **SOLUTION:**

- Combining AAV gene therapy and cytokine immunotherapy into a single, reimagined modality that overcomes key challenges and redefines how we destroy tumor cells and elicit anti-tumor immunity
- The first AAV gene therapy that can be made once and used in numerous indications.
- Our universal design drastically reduces clinical development times, manufacturing timelines, and capital needs for each clinical trial.
- 'Universal' means countless solid tumor cancer patients regardless of tumor type or mutations may benefit from this breakthrough approach.



# Restoring Balance is Key to Unlocking Cures

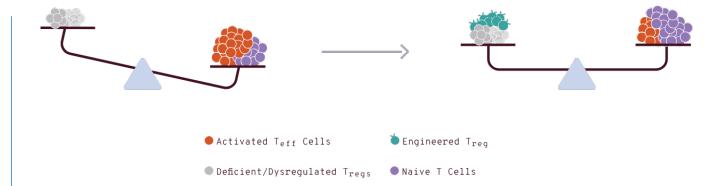




Jeffrey Bluestone, PhD
Co-founder, Sonoma
Biotherapeutics
CEO, President and Emeritus
UCSF Professor and
Innovator

## PROBLEM:

- There are many autoimmune diseases which together account for among the highest rate of medication expenditures in the US.
- RA alone contributes an estimated \$22.3B.



## **SOLUTION:**

- One time treatment focused on autoimmune and inflammatory diseases.
- A unique platform for engineering actual Treg cells and depletion and deactivated Teff cells at the site of disease.

- >\$400M in Funding
- Sonoma Biotherapeutics Presented Preclinical Data from Novel T<sub>reg</sub> Therapy for Rheumatoid Arthritis at American College of Rheumatology Convergence 2023



# SPARSA

# Unlocking the Value in the Al Stack

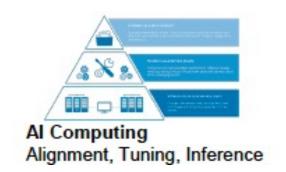




Gilmer Valdes, PhD
Co-Founder, Sparsa
Associate Professor,
Department of Radiation
Oncology and Epidemiology
and Biostatistics



Foundation Models on Edge, Mobile





## PROBLEM:

- The computational burden of Al is increasing exponentially, impacting Energy consumption and compute costs.
- Such computational burden could also worsen the climate crisis.

## **SOLUTION:**

- For any given computational budget, the SparsaAl<sup>TM</sup>algorithm delivers the optimal network architecture.
- Algorithm characterizes the optimal tradeoff between budget and architecture.

### STATUS:

Spinning out





# Unlimited, creative analytical solutions within minutes





Ralph Gonzales, MD
Chief Innovation Officer
UCSF Health
UCSF Clinical Innovation
Center

## PROBLEM:

- Timely and accurate data is a must to track, explore, and forecast clinical initiatives
- Current data collection tools may be cumbersome, delayed, or unavailable

**SOLUTION:** A self-service "value calculator" built in partnership with tag.bio analytics start-up



Avoid waiting weeks for data requests: Point-of-care use enables users to avoid waiting for data pulls



Limitless potential to explore innovative solutions: Platform draws directly from UCSF financial data



Creative cohort comparisons: Cohort feature allows inquiry of user-defined parameters



Drive real-time decision making: Queries return in seconds, allowing for rapid inquiry and iteration

## TRACTION:

 Using tag.bio platform, \$3.7M inpatient costs avoided in reducing short stay admissions from ED



# Exploiting Ferro-Addiction in Tumors with a Proprietary Platform for Iron-Activated Therapeutics



### **UCSF** Co-founders



Adam Renslo, PhD TataraTherapeutics
UCSF Professor and Associate Dean for Entrepreneurship



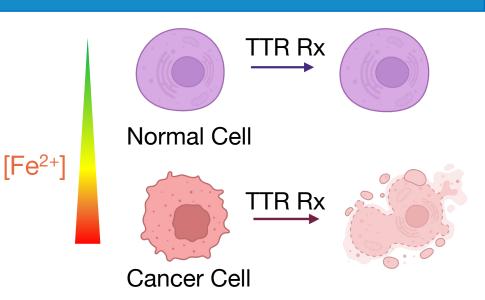
Eric Collisson, PhD
Tatara Therapeutics
UCSF Professor and
Molecular Oncologist

## PROBLEM:

 Cancer therapeutics act systemically, with systemic toxicities that reduce therapeutic index and limit efficacy.

## SOLUTION:

Tumor-selective activation of therapeutics based on elevated tumoral labile iron levels



- TTR Rx activated by Fenton reaction with Fe<sup>2+</sup>
- Releases Tx molecule tailored to tumor type
- Exacerbates ROS stress and promotes ferroptosis
- Spares normal cells, increasing Tx index

- \$3M seed round in 2023
- In vivo active leads with differentiated PK
- Three patent families: US 11,014,955; 11,072,594; WO 2023/049829



# tEPOR: Next Generation RBCs for Hemoglobinopathy Treatment

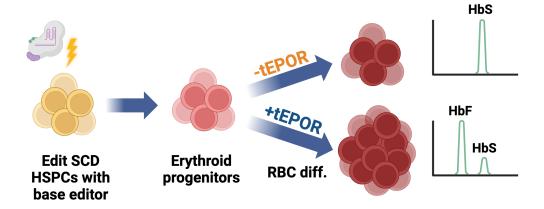




Kyle Cromer, PhD
UCSF Innovator and
Assistant Professor
Dept. of Surgery & Dept. of
Bioengineering &
Therapeutic Sciences

## PROBLEM:

- 20% of sickle cell disease patients have a matched donor, yet <1% of patients in U.S. receive a bone marrow transplant indicating a major unmet medical need.
- Ex vivo CRISPR-mediated editing allows every to patient to have a "donor", however myeloablation-associated mortality is a major barrier to safe correction of disease.
- In vivo editing has low delivery & editing frequencies.



## **SOLUTION:**

- Genome editing is used to introduce a naturally occurring truncated erythropoietin receptor (tEPOR).
- By increasing production of functional RBCs, this editing strategy *may compensate for low in vivo editing frequencies* in HSCs.
- This editing strategy *may eliminate the need for myeloablation*.

### TRACTION:

- Multiple patent disclosures filed to protect IP
- Work awarded American Society of Hematology Junior Faculty Scholar Award



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# Molecularly Targeted Radiotherapy with Conditionally Deposited Peptides





Michael J Evans, PhD Co-founder, TheraPaint Professor, Department of Radiology and Biomedical Imaging, UCSF

Charles S. Craik, PhD, Co-Founder Lawrence Fong, MD, Co-Founder

## PROBLEM:

- Current radioligand therapy (RLT) is limited by several factors leading to decreased efficacy and increased toxicity.
- Tumor responses are often transient and/or variable among patients.
- Improved and novel strategies for targeted radiotherapy are needed.

Ideal RLT therapeutics would clear rapidly while accumulating at high levels in tumors



### **SOLUTION:**

- Catalytic & renewable activation leverages the "bystander effect" to address heterogeneity.
- Platform provides specificity and payload retention with increased efficacy and reduced toxicity.
- Broad applicability to target multiple diseases beyond oncology.

- Clinical data expected in 1H 2024
- Broad IP portfolio filed





# Pioneering a Solution for Diseases of DNA Packaging Dysfunction





Geeta Narlikar, PhD Co-Founder, TippingPoint UCSF Professor, Member National Academy of Sciences

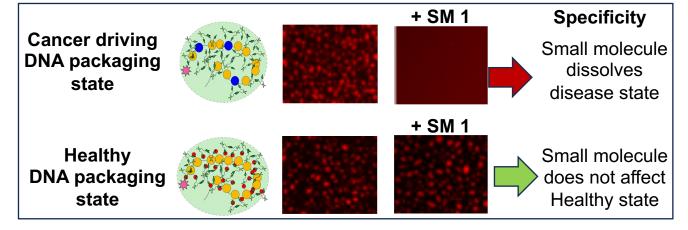




#### PROBLEM:

- Cancers arise from aberrant DNA packaging.
- Current therapeutics target *single* defective factors not the *entire* aberrantly packaged state.
- Approaches to target entire aberrantly packaged DNA states can broaden cancer treatment and reduce potential for resistance.

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#### **SOLUTION:**

- TippingPoint's platform synthetically generates disease and healthy DNA packaged states
- Readily scalable for small molecule screening
- Allows for the first time, drugging of entire disease driving DNA packaged states, with high specificity
- Applications in cancer and regenerative medicine

- \$1M in pre-seed funding from MBC Biolabs, IndieBio (SOSV), ACS BrightEdge
- Won ONO Pharma Golden Ticket & Astellas Future Innovator Award





A public benefit company with a mission to advance health equity by expanding access to cancer precision medicine





Hala Borno, MD CEO & Co-founder, Trial Library UCSF Associate Professor Medical Oncologist

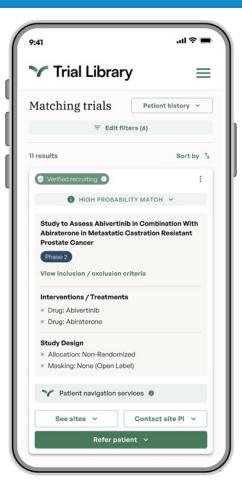
## PROBLEM:

- Oncology clinical trial recruitment remains inefficient, with only 13% of US oncologists participating in research.
- Inequities persist and <7% eligible patients enroll on trials.

## **SOLUTION:**

 Trial Library's evidence-based, provider-facing software integrated is integrated with technology-enabled patientcentered services.

- Provider network includes >250 providers across >100 clinics in the US and is rapidly growing.
- Trial Library supports several large global biopharma trials and has become the preferred recruitment vendor for a large global biopharma partner.







# Woodwinds Therapeutics

# A Drug Discovery Paradigm to Fast-Track Adaptation to Hypoxia





Lani Wu, PhD and Steven Altschuler, PhD Co-founders, Woodwinds Tx

Professor, Dept of Pharmaceutical Chemistry

Matthew Jacobson, PhD
Co-founder, Woodwinds Tx
Professor, Dept of Pharmaceutical
Chemistry

## PROBLEM:

- Drug discovery efforts for hypoxia indications have been challenging because of complex underlying mechanisms.
- Current standards of care in anemia in chronic kidney disease are effective but have significant safety concerns.

## **SOLUTION:**

- Combined Al-enabled in vivo and in vitro platform to uncover novel oxygen disease targets and therapies that fast-track adaptation.
- First clinical program: targeting anemia in CKD
- In vitro POC and pilot screen performed, and hits identified.
- Multiple promising oxygen disease targets discovered.

#### TRACTION:

- \$14M in DARPA funding
- Published tool compound for anemia in CKD target increases EPO only during hypoxia



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# Al Powered Clinical Decision Support for Rare and Genetic Disease





Kat Schmolly, MD Founder & CEO zebraMD

Affiliate researcher at the UCSF Real World Evidence Lab UCSF Innovator

## PROBLEM:

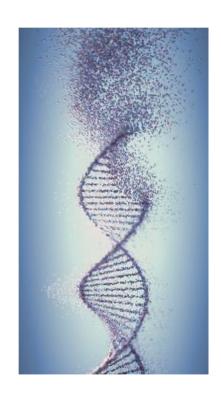
- 1 in 10 people have a rare/genetic disease 50% are children, 30% of them will die before their 5th birthday.
- Takes 12-15 years to get diagnosed
- It costs \$28k/year/patient **more** to manage an RG pt compared to an average chronic disease patient (heart disease, AD, etc).
- Lack of specialty access and knowledge of EBM care as major reasons.

## **SOLUTION:**

- An EMR integrated and solo standing app using EMR data + existing research to reduce diagnostic delays, give EBM management recs personalized to pt and department.
- Improves patient outcomes, reduces healthcare costs.

### TRACTION:

- UCSF Innovations Ventures company
- 3 patents with associated publications
- First pharma customer for \$100k/year
- Acceptance to Nucleate & Equalize



LEARN MORE:



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# Precision Targeting of the Cancer Cell Surfaceome in Acute Myeloid Leukemia (AML) and Solid Tumors: Discovering New Cancer- Specific Targets





Arun Wiita, MD, PhD
UCSF Associate Professor,
Laboratory Medicine

## PROBLEM:

- AML is a common blood cancer with poor prognosis (30.5% 5-year survival).
- There is a lack of immunotherapy targets for AML that are highly cancer-specific (i.e. not also expressed on normal tissues) resulting in significant toxicity and disappointing clinical efficacy.

## **SOLUTION:**

- Proteomic platform for novel conformational cancerspecific target discovery in AML and solid tumors
- First-in-Class CAR-T therapy for acute myeloid leukemia (AML) or any other cancer harboring the active Integrinβ2 targetable antigen
- Active Integrinβ2 scFv's developed by the PIs can be used with other immunotherapy formats like ADCs, or other cells of immune system like NK cells and macrophages.
- Combination therapy with another non-toxic CART to combat AML heterogeneity.

### TRACTION:

 Anti-active Integrinβ2 CAR-T cytotoxicity is highly specific for AML while not toxic to normal hematopoieitic cells, both in vitro and in vivo, unlike other leading AML CAR-T targets (*Nature Cancer*, 2023)

