

ALTERNATIVE MUSCLE CLUB 2016

SAN DIEGO

Meeting Program



THE SANFORD
CONSORTIUM
FOR REGENERATIVE
MEDICINE

Friday, September 30th, 2016







We gratefully acknowledge the support of the following organizations and people, who helped to make this meeting happen

INVOLVED INSTITUTIONS











ORGANIZATIONAL AND CORPORATE SPONSORS

















SDMRC/IEM - Lorenzo Puri, Alessandra Sacco, Velia Fowler, Sam Ward, Ju Chen, Ken Tomory

PPMD - Carol Gregorio, Patricia Furlong

Genea Biocells - Heather Main, Debra Bressaw

Stemonix - Fabian Zanella

ACEA - Levna Zhao

VWR - Jilan Knoblauch

Eppendorf - Michael Sutton, Melody Wilkinson

Aurora Scientific - Matthew Borkowski

Credits 2

PREFACE

Thank you for attending the Alternative Muscle Club 2016!

Welcome to the 4th American Alternative Muscle Club meeting. This year we are back in San Diego at the Sanford Consortium for Regenerative Medicine.

With close to 90 attendees and over 60 abstracts we are certain that this years AMC meeting will be another great success. Genea Biocells has kindly agreed to sponsor this years Young Investigator Award, and the poster prizes. We would also like to extend our gratitude to the institutional support we receive, and to our corporate sponsors!

The AMC has always been a meeting for young scientists with a strong emphasis on career support and networking. Therefore, we are continuing the translational medicine workshop that is run in collaboration with the Parent Project Muscular Dystrophy. We also added a panel on 'transitioning to industry' to this years meeting, which may be of interest to a lot of graduate students and postdoctoral fellows.

Most importantly we hope that you enjoy this years AMC meeting. We are sure that the scientific breadth and quality of the research and its participants will make for an exciting meeting!

With kind regards, The meeting organizers.

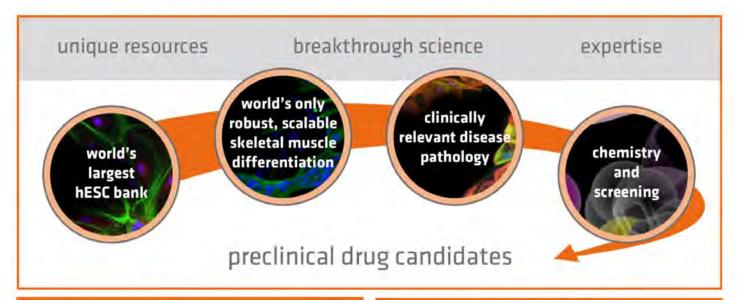
Abby Buchwalter Jordan Blondelle Sabine van Dijk
Stephan Lange Julius Bogomolovas Stefanie Novak

Matthew Stroud

Preface 3



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Genea Biocells is a neuromuscular disease-focused discovery stage company using proprietary human pluripotent stem cell technologies. Genea Biocells also provides contract research services to pharma and supplies reagents to strategic academic collaborators. Genea Biocells has one of the world's largest banks of pluripotent human embryonic stem cells and developed the world's first consistent, scalable and high-yield differentiation process for functional skeletal muscle cells.

For more information email info@geneabiocells.com or visit geneabiocells.com

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MEETING OUTLINE

Тіме	TOPIC	WHERE?
7:45am-8:25am	Registration, Breakfast, Poster hanging	Bella Vista Caffé & Terrace Sanford Lobby
8:25am-8:30am	Welcome Address	Duane J Roth Auditorium
8:30am-9:45am	Podium Session 1	Duane J Roth Auditorium
9:45am-10:00am	Coffee Break	Bella Vista Caffé & Terrace
10:00am-11:15am	Podium Session 2	Duane J Roth Auditorium
11:15am-11:50am	Eureka Network-Duchenne (END) Translational Medicine Workshop	Duane J Roth Auditorium
11:50am-1:45pm	Lunch break & Poster session	Bella Vista Caffé & Terrace Sanford Lobby
12:15pm-1:45pm	Poster Session odd numbered posters: 12:15pm-1pm even numbered posters: 1pm-1:45pm	Sanford Lobby
1:45pm-2:30pm	Transition to industry panel	Duane J Roth Auditorium
2:30pm-3:45pm	Podium Session 3	Duane J Roth Auditorium
3:45pm-4pm	Coffee Break	Bella Vista Caffé & Terrace
4pm-5:30pm	Podium Session 4	Duane J Roth Auditorium
5:30pm-5:45pm	Award Ceremony	Duane J Roth Auditorium
from 5:45pm	Reception / Networking / Social Mixer	Bella Vista Caffé & Terrace



UC San Diego Institute of Engineering in Medicine



The San Diego Muscle Research Center (SDMRC) is one of the IEM centers of excellence. Its mission is to provide investigators with the infrastructure and environment to accelerate their cutting-edge muscle research in an efficient and cost-effective manner.

http://sdmrc.ucsd.edu

UC San Diego

Institute of

Engineering in Medicine

The Institute of Engineering in Medicine (IEM) was established in 2008 at UC San Diego to bring together outstanding faculty in its Jacobs School of Engineering, the School of Medicine and other units, for interdisciplinary research and education.

Our mission is to accelerate discoveries of innovative science and technology through teamwork and facilitate their translation to improve healthcare delivery.

The IEM is composed of seven Centers focusing on diseases and systems and nine Centers for technologies, education and industry collaboration.

http://iem.ucsd.edu

SCIENTIFIC PODIUM SESSIONS

REGISTRATION

BELLA VISTA CAFFÉ & TERRACE

7:45am-8:25am Registration / Coffee / Social

Poster presenters: Please hang up your posters!

Presenters for Session 1, please set up your computers.

PODIUM SESSION 1

Duane J Roth Auditorium

Session Chairs: Jordan Blondelle & Abby Buchwalter

8:25am-8:30am Welcome address

8:30am-8:45am Jason Pellman:

A Novel Role for SNARE Proteins in Cardiac Desmosomal Biology and

Disease.

8:45am-9am Miensheng Chu

Fragile-X related protein 1 regulates gap junction remodeling in

cardiomyocytes.

9am-9:15am Adrian Arrieta

MANF, a Structurally Unique ER stress-inducible Chaperone, Restores

ER-protein Folding in ER Stressed Cardiac Myocytes & in the Ischemic

Heart.

9:15am-9:30am **Daniel Smith**

Identifying Novel Interacting Partners for the UNC-45 Chaperone in

Drosophila melanogaster.

9:30am-9:45am Yoshitake Cho

Perm1 (PGC-1 and ERR-induced Regulator, Muscle 1) is required for

exercise-induced mitochondrial biogenesis and enhances oxidative

capacity in skeletal muscle.

15 minutes break

PODIUM SESSION 2

DUANE J ROTH AUDITORIUM

Session Chairs: Stephan Lange & Julius Bogomolovas

10am-10:15am Francesca Boscolo Sesillo

p53 Balances Self-Renewal and Myogenic Commitment of Muscle Stem

Cells Upon Activation.

10:15am-10:30am Michael Yu

Engineering Multipotent Cardiogenic Progenitors and Cardiomyocytes for

Regenerative Medicine and Disease Modeling Purposes.

10:30am-10:45am Erik Blackwood

ATF6 Is Required For ANP Secretion From The Heart.

10:45am-11am Sadie Ingle

Generation of a minipig model for genetic hypertrophic cardiomyopathy.

11am-11:15am Anthony Hessel

Optimal muscle length is the same for twitch and tetanic contractions in muscles from mdm mice: a role for titin in isometric force production?

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EUREKA NETWORK-DUCHENNE (END) TRANSLATIONAL MEDICINE WORKSHOP

DUANE J ROTH AUDITORIUM

Session Chairs: Brett Colson and Leonela Amoasii

11:15am-11:50am The Eureka Network-Duchenne (END) Translational Medicine Workshop.

LUNCH & SCIENTIFIC POSTER SESSION

BELLA VISTA CAFFÉ & TERRACE, SANFORD LOBBY

11:50am-1:45pm Lunch

12:15pm-1:45pm Poster Session

Odd numbered posters: 12:15pm-1pm Even numbered posters: 1pm-1:45pm

TRANSITION TO INDUSTRY PANEL

DUANE J ROTH AUDITORIUM

1:45pm-2:30pm Transition to Industry Panel.

Fabian Zanella - Stemonix

David Gokhin - Carling Communications

Indroneal Banerjee - Abbvie Heather Main - Genea Biocells

.....

PODIUM SESSION 3

DUANE J ROTH AUDITORIUM

Session Chairs: Sabine van Dijk and Jordan Blondelle

2:30pm-2:45pm Joanna Palade

Identification of satellite cells from anole lizard muscle and demonstration

of increased musculoskeletal potential.

2:45pm-3pm Constanza Cortes

Skeletal muscle control of systemic metabolism: a role for Transcription

Factor E-B (TFEB) signaling.

3pm-3:15pm Valeria Marrocco

Pharmacological inhibition of PKCθ in vivo improves healing in mdx mice.

3:15pm-3:30pm Juliane Campos

β2-adrenoreceptor agonist improves autophagy in skeletal muscle

weakness/wasting.

3:30pm-3:45pm Patrick Desmond

Identification of Small Ankyrin 1 as a novel SERCA1 regulatory protein in

Skeletal Muscle.

15 minutes break

Please remember to take down your posters!

PODIUM SESSION 4

DUANE J ROTH AUDITORIUM

Session Chairs: Stefanie Novak & Stephan Lange

4pm-4:15pm Tzu-Han Lin

Role of Rab35 and Endosomal Trafficking in T-tubule Remodeling.

4:15pm-4:30pm Michael Hicks

Human developmental myogenesis identifies enrichment and maturation

strategies for hPSC-derived skeletal muscle.

4:30pm-4:45pm Cassandra Happe

Mechanical Patterning Improves Neuromuscular Junction-in-a-dish

Modeling.

4:45pm-5pm Michael Stec

Fbxw7 is a novel regulator of skeletal muscle stem cell expansion.

5pm-5:15pm Bradley Nelson

Adeno-associated virus serotype 9 gene therapy for the treatment of

Danon disease.

Industry special - novel applications

5:15pm-5:30pm Fabian Zanella

microHeart: A screening-ready, physiologically relevant human iPSC-

derived cardiomyocyte platform.

POSTER AND YOUNG INVESTIGATOR AWARD CEREMONY

DUANE J ROTH AUDITORIUM

5:30pm-5:40pm AMC Genea Biocells Poster Awards

AMC Genea Biocells Young Investigator Awards

RECEPTION / NETWORKING / SOCIAL EVENT

BELLA VISTA CAFFÉ & TERRACE

starting at 5:45pm Reception

Please remember to take down your posters!

SCIENTIFIC POSTER SESSION

Posters should be mounted on the poster boards in the Sanford Consortium Lobby before 10am. There will be a formal poster session after lunch, from 12:15pm-1:45pm, although AMC attendees are welcome to browse and discuss posters whenever they wish. Posters must be taken down before 6pm. Please note that we cannot save any posters that remain hung up after that time.

GENEA BIOCELLS POSTER AWARDS



POSTER NO.

An independent jury of senior scientists selects the six best poster presentations during our poster session for the "Genea Biocells Poster Award". The winners will be announced at the end of the Scientific Sessions at 5:30pm.

We gratefully acknowledge Genea Biocells for sponsoring the poster and young investigator awards this year.

SECTION 1 - MUSCLE/CARDIAC VASCULATURE

NAME / TITLE

1. Chao Chen Novel Variants in VINCULIN and TROPOMYOSIN1 Combinatorially Predispose Patients to Dilated Cardiomyopathy

- 2. Ramon Diaz Trelles

 Hypoxia tolerance and cardioprotection in the adult heart
- 3. Marcy Martin
 SREBP2-Induced EndoMT Contributes to Pulmonary Fibrosis
- 4. Mei Methawasin

 Upregulating compliant titin in the heart attenuates left ventricular stiffness in a mouse model with diastolic dysfunction.
- 5. Gerburg Schwaerzer
 "Role of protein kinase G signaling in aortic wall maintenance and repair"

SECTION 2 - MUSCLE STRUCTURE, FUNCTION & SIGNALING

Development of MRI Methods Towards Evaluating Cardiomyocyte Performance in Duchenne Muscular Dystrophy

7. Matthew Bills

Development of an in vitro assay for assessing cardiomyocyte function

8. Eric Carruth

Regional Gradients in Tissue Anisotropy During Pressure Overload Hypertrophy Reflect Endogenous Heterogeneity in the Rat Ventricles

9. Brett Colson

Cardiac Myosin-Binding Protein C Structural Dynamics - a Time-Resolved FRET Biosensor for Enhanced Contractility in Heart Failure Therapeutic Discovery

10. Argus Sun

Force Thresholds: Modeling Subcellular Myocyte Mechanotransduction

11. James Caldwell

X-ray crystallography structures of Drosophila striated muscle myosin II

12. Andrew D'Lugos

High Intensity Exercise Preserves Myocellular Size and mTOR Signaling During Doxorubicin Treatment

13. Michael Gibbons

Histological Assessment of Chronically Torn Human Rotator Cuff Muscles: Evidence of Degeneration, Regeneration and Remodeling

14. Charles Grav

CaMKIIδ subtypes differentially regulate infarct formation following ex vivo myocardial ischemia/reperfusion through NF-κB and TNF-α

15. Sherin Hashem

LAMP-2 deficiency impairs mitophagy and promotes mitochondrial damage in models of Danon disease

16. Valeria Marrocco

CARP1-mediated signaling in dilated cardiomyopathy

17. Stefanie Novak

Elucidating the role of phosphorylation on thin filament length regulation in cardiomyocytes

Kristoffer Svensson

Markers of mitochondrial biogenesis are not altered by overexpression of SIRT1 in skeletal muscle of adult mice

19. Sabine van Dijk

Binding of the M-domain of Cardiac Myosin Binding Protein C to Actin Contributes to the Regulation of Cardiac Contraction

20. Robbert van der Pijl

Diaphragm hypertrophy following passive stretch: a role for titin-based mechanosensing?

21. Jianlin Zhang

Physiological function of prion protein in the heart.

SECTION 3 - MUSCLE REGENERATION

22. Alessandra Castaldi

C-kit+ cells resident in adult heart are susceptible to aging

23. Melissa Hernandez

Injectable Skeletal Muscle Matrix in Aged Mouse Model of Peripheral Artery

Disease

24. Jessica Ungerleider

Extracellular Matrix Hydrogel Promotes Tissue Remodeling, Arteriogenesis,

and Perfusion in a Rat Hindlimb Ischemia Model

25. Barbora Malecova

Single cell analysis reveals dynamic transitions within distinct subpopulations

of Fibro-Adipogenic Progenitors (FAPs) implicated in skeletal muscle

regeneration or fibrosis.

26. Christopher Penton

Maintaining the differentiation potential of muscle stem cells in vitro in order to

support therapeutic discovery for muscular dystrophy diseases

27. David Sala Cano

Identification of STAT3 downstream effectors regulating muscle stem cell

function

SECTION 4 - MUSCLE DEVELOPMENT

28. Cherie Alissa Lynch

Mohawk Regulation of Macrophage Polarity During Muscle Repair

29. Gaurav Agrawal

Engineered 3D Skeletal Muscle-on-a-Chip as an In Vitro Tool

30. Amanda Rickard

A Pluripotent Cell Based DUX4 Reporter for Modeling FSHD in skeletal

muscle

31. Tian Wang

Regulation of T-tubule membrane remodeling in Drosophila muscle

Jordan Blondelle 32.

Role of Cullin-RING ligase activity in skeletal muscle development

33. Anabel de la Garza

Multinucleated myotube formation from human pluripotent stem cells

34. Sole Gatto

Epigenomic mechanisms driving hESC conversion into skeletal muscle cells

35. Vic Keschrumrus

Drug Screening and Therapy Development Using Engineered Heart Tissues

36. Heather Main

Skeletal Muscle modeling of Myotonic Dystrophy from human pluripotent cells

37. Geo Vogler

Identification of Disease-relevant Genetic Variants using the Drosophila Heart

Model

SECTION 5 - MUSCLE BIOPHYSICS & BIOMECHANICS

38. Ivan Tomasic

2-deoxy-ATP enhances multiple kinetic parameters to improve cardiac

function

39. Xiaoyu Zhang

Assessment of Positive and Negative Inotropic Compounds Using an

Impedance-based System with human iPSC-derived Cardiomyocytes under

Controlled Pacing Conditions

40. Johan Lindqvist

Mechanical ventilation reduces the optimal length for force production in the

diaphragm

41. **Ayla Sessions**

Extracellular Matrix Downregulation in the Drosophila Heart Preserves

Contractile Function and Improves Lifespan

SECTION 6 - LATE BREAKING

42. Ike Chinyere

Feasibility and Testing of a Human Induced Pluripotent Stem Cell Derived

Cardiac Graft in a Pre-Clinical Swine Model of CHF



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- Bioprocessing
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- And more!



CAREER DEVELOPMENT / MEETING BENEFITS



One of the goals for this meeting is to give you a head-start into the career as a scientist in academia or industry.

EUREKA NETWORK-DUCHENNE (END) TRANSLATIONAL MEDICINE WORKSHOP

This workshop is specifically designed to provide a learning environment where early career scientists (graduate students, postdoctoral fellows, assistant professors) working on various aspects of muscular dystrophies (neuromuscular, cardiac or skeletal) can learn concepts on taking their science from

'Bench To Bedside'

The workshop session before lunch will provide a general overview on translational medicine to all AMC attendees. We will also offer information on how to apply for a follow-up workshop taking place in Italy later this year.



Parent Project Muscular Dystrophy LEADING THE FIGHT TO END DUCHENNE







TRANSITION TO INDUSTRY PANEL

This panel session will feature postdoctoral fellows from UC San Diego, The Scripps Research Institute and the University of Sydney that made the successful transition into an industry position. Each panelist will showcase his career path and experiences. The panelists will be available for a Q&A during the career development session, and may also attend the networking event at the end of the day.

Dr. Heather Main

worked in academia for 11 years across 3 continents, including a PhD in Sweden and post doc in Australia. In 2014 she joined Genea Biocells, a neuromuscular disease company, which utilizes pluripotent stem cell technologies towards disease modeling, drug discovery and stem cell therapies.

Dr. Indroneal Banerjee

was a Postdoctoral Fellow at UC San Diego. His current position at the pharmaceutical company Abbvie sits at the interface between research and drug applications for clinical use.

Dr. David Gokhin

was a Postdoctoral Fellow at the Scripps Research Institute. He currently works as a medical writer for Carling Communications, a professional services firm that specializes in fullservice marketing, advertising, and physician communications within pharmaceutical, medical device, and specialty biotechnology industries.

Dr. Fabian Zanella

was a Postdoctoral Fellow at UC San Diego. He is now working for the local biotech company Stemonix that sets a new economic paradigm around stem cell technologies to meet the demands of drug discovery and personalized medicine.









AMC POSTER & YOUNG INVESTIGATOR AWARDS

In addition to presenting your work in a talk or poster presentation, we aim to add another line to your *CV or resumé*.



Especially if your poster or podium presentation receives one of the Genea Biocells sponsored **Young Investigator or Poster Awards**.

The first prize in the **Young Investigator Award** category is a Nespresso coffee machine. We also offer all four young investigator awardees an opportunity to run an experiment on the IN Cell Analyser 6000 at Genea Biocells.

The six **Poster Awards** receive a goodie bag containing a mug, candy, pen as well as Myotube Mike (a crusader for awareness of neuromuscular diseases), and a lab-coat.

RECEPTION / NETWORKING / SOCIAL HOUR

Connect with other scientists and attendees from local biotech companies or universities. Our networking event is your chance to quiz people on how to transition into industry, network, establish new collaborations, or simply make new friends with similar scientific interests.



VENUE



SANFORD CONSORTIUM FOR REGENERATIVE MEDICINE

The AMC meeting will be held at the **Sanford Consortium for Regenerative Medicine**, adjacent to the Salk Institute and the University of California San Diego campus in La Jolla, CA.

The **Duane J. Roth Auditorium** will host the scientific podium sessions. The poster sessions will be held in the **lobby** of the Sanford Consortium building.

For the location of the Sanford Consortium Building on a map and directions, please see the following: LINK.



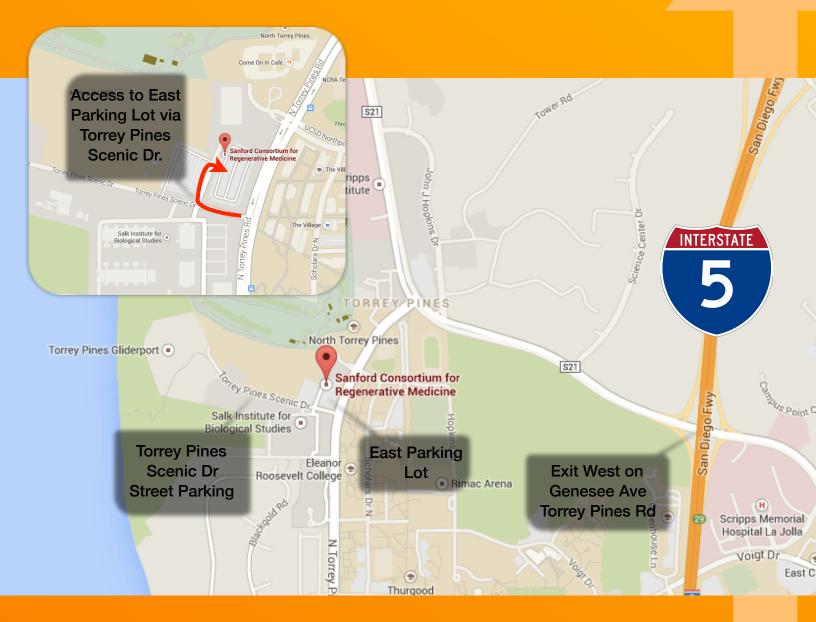
BELLA VISTA SOCIAL CLUB AND CAFFÉ

All catering will be provided by the <u>Bella Vista Social Club and Caffé</u>, located on the grounds of the Sanford Consortium Building. **The Bella Vista Social Club and Caffé** is renowned for its mix of art and culture. The Caffé provides delicious cheese and meat platters, and a good selection of micro-brewed beer and wine.

A networking event concludes this years AMC, and will also be held at the Bella Vista Caffé. Wind down and enjoy a relaxing networking social hour after the meeting with us, while the sun sets in the Pacific Ocean!

Los Angeles

PARKING AND DIRECTIONS



There is limited space available for street parking along Torrey Pines Scenic Drive (free) and in the East Parking Lot adjacent to the Sanford Consortium building (\$8 for daylong parking, get your ticket at the machine in the beginning of the day). Please arrive early to get your parking spot, breeze through the registration, hang up your poster and grab a bite before the start of the scientific sessions.

Street address for GPS/Google Maps: 2880 Torrey Pines Scenic Dr., La Jolla, CA-92037

Please use Google Maps (LINK) to see the location.

Los Angeles

PUBLIC TRANSPORT



Although a bit more cumbersome, but the Sanford Consortium Building is also reachable through public transport.

You can also use the trip planner offered by the Metropolitan Transit Service (MTS) -

the destination address is: 2880 Torrey Pines Scenic Dr. La Jolla, CA

http://www.sdmts.com/Planning/googleTP.asp

LODGING

For those coming from out of town, please use the following link for a list of local hotels and lodging options: http://amcsd.ucsd.edu/directions.html

CONTACT

If you have questions about the meeting, you may contact us by email.

Email address: amclub.us@gmail.com

ORGANIZING COMMITTEE

Stephan Lange

UC San Diego, School of Medicine

email: slange@ucsd.edu

Julius Bogomolovas

UC San Diego, School of Medicine email: jboqomolovas@ucsd.edu

Jordan Blondelle

UC San Diego, School of Medicine

email: jblondelle@ucsd.edu

Abby Buchwalter

The Salk Institute

email: abuchwalter@salk.edu

Sabine van Dijk

University of Arizona

email: sjvandijk@email.arizona.edu

Stefanie Novak

University of Arizona

email: smares@email.arizona.edu

Matthew Stroud

UC San Diego, School of Medicine

email: mstroud@ucsd.edu





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PARTICIPANTS

Name	email	Institute/Company
Gaurav Agrawal	gagrawal@eng.ucsd.edu	UC San Diego
Sonia Albini	salbini@sanfordburnham.org	Sanford Burnham Prebys Medical Discovery Institute
Angels Almenar	aalmenar@ucsd.edu	UC San Diego
Leonela Amoasii	leonela.amoasii@utsouthwestern.edu	UT Southwestern
Adrian Arrieta	aarrieta1335@gmail.com	San Diego State University
Paul August	Paugust@icagen.com	Icagen
Indroneal Banerjee	indroneal.banerjee@abbvie.com	Abbvie
Monica Bennett	monica.bennett@geneabiocells.com	Genea Biocells
Matthew Bills	billsm@email.arizona.edu	University of Arizona
Erik Blackwood	eblackwo@alumni.nd.edu	San Diego State University Heart Institute
Jordan Blondelle	jblondelle@ucsd.edu	UC San Diego
Rolf Bodmer	rolf@sbpdiscovery.org	Sanford Burnham Prebys Medical Discovery Institute
Julius Bogomolovas	jbogomolovas@ucsd.edu	UC San Diego
Francesca Boscolo Sesillo	fboscolo@sbpdiscovery.org	Sanford Burnham Prebys Medical Discovery Institute
Abby Buchwalter	abuchwalter@salk.edu	Salk Institute
Katelyn Busse	katelyn.busse@geneabiocells.com	Genea Biocells
James Caldwell	jamestorecaldwell@gmail.com	San Diego State University Research Foundation

Name	email	Institute/Company
Juliane Campos	Juliane.Campos@cshs.org	Cedars-Sinai Medical Center ~ Heart Institute
Sara Carbajo	scarbajo@scripps.edu	The Scripps Research Institute
Eric Carruth	ecarruth@ucsd.edu	UC San Diego
Alessandra Castaldi	acastaldi@ucsd.edu	UC San Diego
Chao Chen	chaochen@ucsd.edu	UC San Diego
Ike Chinyere	ichinyere@email.arizona.edu	University of Arizona
Yoshitake Cho	cyoshitake@ucsd.edu	UC San Diego
Miensheng Chu	mchu@email.arizona.edu	University of Arizona
Mert Colpan	colpanmert@gmail.com	University of Arizona
Brett Colson	bcolson@email.arizona.edu	University of Arizona
Riccardo Contu	riccardo.contu@stemonix.com	StemoniX
Constanza Cortes	cjcortes@ucsd.edu	UC San Diego
Andrew D'Lugos	andrew.dlugos@asu.edu	Arizona State University
Usue Etxaniz	uetxaniz@sbpdiscovery.org	Sanford Burnham Prebys Medical Discovery Institute
Elie Farah	efarah@ucsd.edu	UC San Diego
Anabel de la Garza	anabel.delagarza@geneabiocells.com	GeneaBiocells
Patrick Desmond	pdesmond@ucsd.edu	UC San Diego
Ramon Diaz Trelles	trelles@sbpdiscovery.org	Sanford Burnham Prebys Medical Discovery Institute

Name	email	Institute/Company
Jone Lopez Erauskin	jerauskin@ucsd.edu	Ludwig Institute
Alison Fanton	alison.fanton@stemonix.com	Stemonix
Qingfen Gan	fgan@myokardia.com	Myokarida
Sole Gatto	sgatto@sbpdiscovery.org	Sanford Burnham Prebys Medical Discovery Institute
Michael Gibbons	mgibbons@ucsd.edu	UC San Diego
David Gokhin	David.Gokhin@carlingcom.com	Carling Communications
Charles Gray	cgray@ucsd.edu	San Diego State University
Cassandra Happe	chappe@ucsd.edu	UC San Diego
Sherin Hashem	s1hashem@ucsd.edu	UC San Diego
Melissa Hernandez	mjh003@eng.ucsd.edu	UC San Diego
Anthony Hessel	alh385@nau.edu	Northern Arizona University
Michael Hicks	Michaelhicks@ucla.edu	UC Los Angeles
Karen Hsu	karenhsuu@gmail.com	San Diego State University
Ali Hussaini	hussaini@email.arizona.edu	University of Arizona
Sadie Ingle	sringle@myokardia.com	MyoKardia
Vic Keschrumrus	vkeschrumrus@email.arizona.edu	University of Arizona
Jilan Knoblauch	Jilan.Knoblauch@vwr.com	VWR International
Shivani Lakkaraju	slakkara@ucsd.edu	UC San Diego

Name	email	Institute/Company
Stephan Lange	slange@ucsd.edu	UC San Diego
Tania Larrinaga	tmlarrinaga@email.arizona.edu	University of Arizona
Tzu-Han Lin	tzl007@ucsd.edu	UC San Diego
Johan Lindqvist	johanlindqvist@email.arizona.edu	University of Arizona
Marissa Lopez-Pier	mal1@email.arizona.edu	University of Arizona
Cherie Alissa Lynch	cjlynch1@asu.edu	Arizona State University
Patrick Magrath	rmagrath@mednet.ucla.edu	UC Los Angeles
Heather Main	heather.main@geneabiocells.com	Genea Biocells
Barbora Malecova	bmalecova@sbpdiscovery.org	Sanford Burnham Prebys Medical Discovery Institute
Valeria Marrocco	vmarrocco@ucsd.edu	UC San Diego
Charles Martin	charles.martin@geneabiocells.com	Genea Biocells
Marcy Martin	mam085@ucsd.edu	UC San Diego
Robert McKernan	robert.mckernan@geneabiocells.com	Genea Biocells
Mei Methawasin	methajit@email.arizona.edu	University of Arizona
Shaday Michan	smichan@sbpdiscovery.org	Sanford Burnham Prebys Medical Discovery Institute
Bradley Nelson	b4nelson@ucsd.edu	UC San Diego
Vishal Nigam	vnigam@ucsd.edu	UC San Diego
Stefanie Novak	smares@email.arizona.edu	University of Arizona

Name	email	Institute/Company
Joanna Palade	opalade@asu.edu	Arizona State University
Jason Pellman	jpellman@ucsd.edu	UC San Diego
Christopher Penton	cpenton@icagen.com	Icagen
Suzanne Peterson	suzanne.peterson@geneabiocells.com	Genea Biocells
Cullen Pivaroff	cullen.pivaroff@geneabiocells.com	Genea Biocells
Jesse Placone	jplacone@ucsd.edu	UC San Diego
Amanda Rickard	amanda.rickard@geneabiocells.com	Genea Biocells
Barry Rothenberg	bri@brincubator.com	Billups-Rothenberg
Alessandra Sacco	asacco@sbpdiscovery.org	Sanford Burnham Prebys Medical Discovery Institute
David Sala Cano	dsala@sbpdiscovery.org	Sanford Burnham Prebys Medical Discovery Institute
Lauren Schultz	leschultz@email.arizona.edu	University of Arizona
Gerburg Schwaerzer	gschwaerzer@ucsd.edu	UC San Diego
Ayla Sessions	aosessio@ucsd.edu	UC San Diego
Wonjong Si	won.si@stemonix.com	StemoniX Inc.
Daniel Smith	dasmith08@gmail.com	San Diego State University
Martin T. Spang	mtspang@eng.ucsd.edu	UC San Diego
Stephan Spangenberg	stephan.spangenberg@stemonix.com	Stemonix
Michael Stec	mstec@sbpdiscovery.org	Sanford Burnham Prebys Medical Discovery Institute

Name	email	Institute/Company
Takeshi Suetomi	tsuetomi@ucsd.edu	UC San Diego
Argus Sun	argus.m.sun@ucla.edu	Sanford Consortium for Regenerative Medicine
Teri Suzuki	tsuzuki@icagen.com	Icagen
Kristoffer Svensson	kssvensson@ucsd.edu	UC San Diego
Ivan Tomasic	itomasic@myokardia.com	Myokardia Inc.
Adriana Trujillo	adrianasaratrujillo@gmail.com	San Diego State University
Jessica Ungerleider	jlungerl@eng.ucsd.edu	UC San Diego
Robbert van der Pijl	rjvanderpijl@email.arizona.edu	University of Arizona
Sabine van Dijk	sjvandijk@email.arizona.edu	University of Arizona
Jesus Villanueva	eric.villanueva@geneabiocells.com	Genea Biocells/SDSU
Geo Vogler	gvogler@sbpdiscovery.org	Sanford Burnham Prebys Medical Discovery Institute
Tian Wang	tiw081@ucsd.edu	UC San Diego
Xian Wei	swei@cibiem.com	Cibiem, Inc
Melody Wilkinson	Wilkinson.M@eppendorf.com	Eppendorf
Ming Yu	Myu@myokardia.com	Myokardia
Michael Yu	shy055@ucsd.edu	UC San Diego
Fabian Zanella	fabian.zanella@stemonix.com	StemoniX
Jianlin Zhang	jiz007@ucsd.edu	UC San Diego

Name	email	Institute/Company
Xiaoyu Zhang	Xzhang@aceabio.com	ACEA Biosciences Inc
Leyna Zhao	Izhao@aceabio.com	ACEA Biosciences Inc



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