



Meeting Program

Friday, September 22nd, 2017

UC San Diego



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

INVOLVED INSTITUTIONS







ORGANIZATIONAL AND CORPORATE SPONSORS















PPMD - Carol Gregorio, Patricia Furlong
ASCB - Heather Smith
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StemoniX - Ryan Gordon, David Buche, Fabian Zanella
ACEA - Leyna Zhao
IONIS - Tamar Grossman
MyoKardia - Sadie Ingle
The Company of Biologists

WELCOME NOTE

Thank you for attending the Alternative Muscle Club 2017!

The meeting organizers welcome you to the 5th American Alternative Muscle Club meeting. This year we are again in San Diego at the Sanford Consortium for Regenerative Medicine.

With more than 100 attendees we are certain that this years AMC meeting will be another great success. *Genea Biocells* has kindly agreed to sponsor this years **Young Investigator Awards**, and the **poster prizes**. This years meeting also features the '**Jean Hanson Award**' to promote diversity in science that is kindly sponsored by *StemoniX*.

We would also like to extend our gratitude to the institutional support we receive, and to our corporate sponsors as well as the charitable organizations to hep us organize this meeting!

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* and the *Eureka Institute*. We also added two more career events: the 'Interviewing for Industry' workshop and a 'Funding Opportunities Panel' 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.

Constanza Cortes
Yoshitake Cho

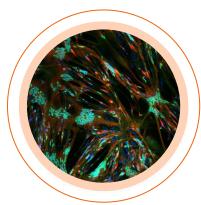
Jordan Blondelle Julius Bogomolovas Stephan Lange Anastasia Gromova Marissa Lopez-Pier



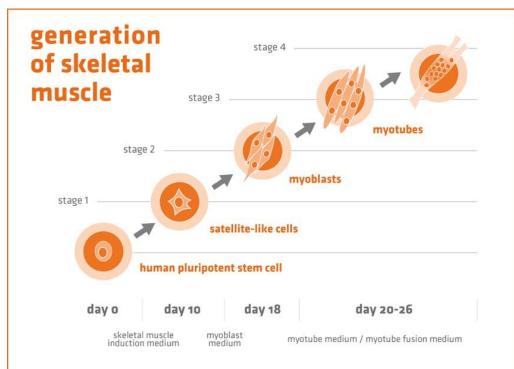
SKELETAL MUSCLE MEDIA SUITE



HUMANLY POSSIBLE



Genea Biocells developed the first robust, high-yield protocol to differentiate human pluripotent stem cells (hPSCs) to functional skeletal muscle (Caron et al. 2016. Stem Cells Translational Medicine 5: 1145–61) without cell sorting or overexpression of myogenic transcription factors. We and our collaborators successfully differentiated over 50 human embryonic and induced pluripotent stem cell lines. The third generation of our media suite is now available and offers further improved yields, robustness and enhanced myotube formation.



Genea Biocells myogenesis protocol directs feeder-free, serum-free human pluripotent stem cells through PAX3/7⁺ myogenic progenitor (satellite-like cells), MYOD+ myoblast, and MHC⁺ (Myosin Heavy Chain) skeletal muscle myotube stages.

hPSC are dissociated to single cells and plated in Myogenic Induction Medium. After approx. 10 days, cells are passaged and plated in Myoblasts medium. Once they reach confluence, after approx. 8 days, they are switched to Myotube or Myotube Fusion Medium to generate myotubes.

Genea Biocells focuses on therapeutics discovery for neuromuscular diseases using proprietary human pluripotent stem cell technologies. We also pursue discovery partnerships with pharma and supply reagents to academic researchers. 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.

Ordering information

Our skeletal muscle differentiation media can be ordered directly from us (North America and rest of world). Contact us for a free trial kit.

- 250 ml Myogenic Induction Medium
- 250 ml Myoblast Medium
- 250 ml Myotube Medium
- 250 ml Myotube Fusion Medium

For industry or customers requiring larger volumes and continuous supply, we set up supply agreements. All sales are subject to our terms and conditions.

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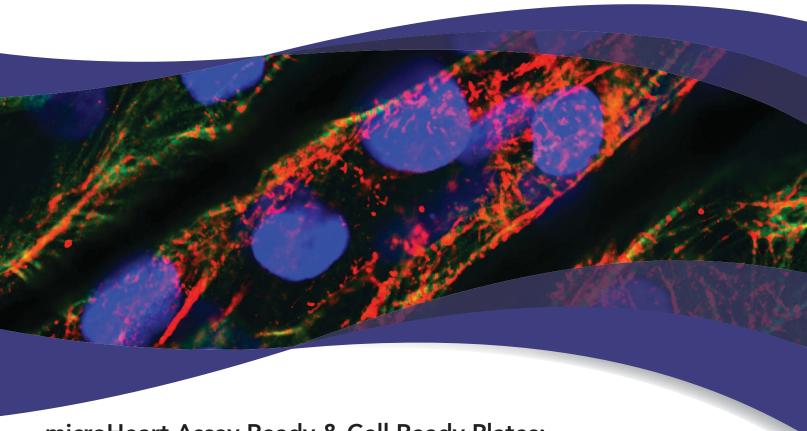
MEETING OUTLINE

TIME	TOPIC	WHERE?
8:30am-9:00am	Registration, Breakfast, Poster hanging	Bella Vista Caffé & Terrace Sanford Lobby
9:00am-9:05am	Welcome Address	Duane J Roth Auditorium
9:05am-10:05am	Podium Session 1	Duane J Roth Auditorium
10:05am-10:20am	Coffee Break	Bella Vista Caffé & Terrace
10:20am-11:20am	Podium Session 2	Duane J Roth Auditorium
11:20am-12:00pm	Eureka Network-Duchenne (END) Translational Medicine Workshop	Duane J Roth Auditorium
	Successful Interviewing for Industry Workshop	Meeting room 1013
12pm-2:00pm	Lunch break & Poster session	Bella Vista Caffé & Terrace Sanford Lobby
	Poster Session odd numbered posters:12:30pm-1:15pm even numbered posters: 1:15pm-2pm	Sanford Lobby
2:00pm-2:55pm	Funding Opportunities panel: Funding - Where and How to Get It	Duane J Roth Auditorium
2:55pm-3:55pm	Podium Session 3	Duane J Roth Auditorium
3:55pm-4:10pm	Coffee Break / poster removal	
4:10pm-5:10pm	Podium Session 4	Duane J Roth Auditorium
5:10pm-5:30pm	Jean Hanson Award	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

StemoniX

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Contact StemoniX Customer Service:









morning

SCIENTIFIC PODIUM SESSIONS

REGISTRATION

BELLA VISTA CAFFÉ & TERRACE

8:30am-9:00am Registration / Coffee / Social

Poster presenters: Please hang up your posters!

Presenters for Session 1, please set up your computers.

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PODIUM SESSION 1

Duane J Roth Auditorium

Session Chairs: Jordan Blondelle & Constanza Cortes

9:00am-9:05am Welcome address

9:05am-9:20am Daniel Smith

Identifying Novel Interacting Partners for the UNC-45 Chaperone in

Drosophila melanogaster.

9:20am-9:35am Mai Tran

The muscle-less jerboa foot as a novel system to understand muscle

degeneration.

9:35am-9:50am Michael Stec

The E3 ligase Fbxw7 regulates postnatal skeletal muscle growth.

9:50am-10:05am Xi Fang

Loss-of-function mutations in co-chaperone BAG3 destabilize small HSPs and cause cardiomyopathy.

15 minutes break

PODIUM SESSION 2

DUANE J ROTH AUDITORIUM

Session Chairs: Stephan Lange & Julius Bogomolovas

10:20am-10:35am Haibin Xi

Single Cell RNA-Sequencing Reveals Distinct Skeletal Muscle Progenitor

Populations across Human Development.

10:35am-10:50am Christa Trexler

Biological Sex Influences Cardiomyocyte Function and Gene Expression.

10:50am-11:05am Elaheh Karbassi

Global Chromatin Structural Changes Mediate Pathological

Transcriptome Remodeling during Heart Failure.

11:05am-11:20am Usue Etxaniz

Fibro-Adipogenic Progenitors (FAPs) are composed of discrete

subpopulations with distinct temporal dynamics, specific transcriptional

profiles and biological activities.

.....

11:20am-12:00am

EUREKA NETWORK-DUCHENNE (END) TRANSLATIONAL MEDICINE WORKSHOP

DUANE J ROTH AUDITORIUM

Session Chairs: Elizabeth Gibbs (UC Los Angeles), Brett Colson (U of Arizona)

INTERVIEWING FOR INDUSTRY WORKSHOP

MEETING ROOM 1013

Debra Bressaw (Genea Biocells)

LUNCH & SCIENTIFIC POSTER SESSION

BELLA VISTA CAFFÉ & TERRACE, SANFORD LOBBY

12:00am-2:00pm Lunch

PARALLEL SESSIONS

12:30pm-2:00pm Poster Session

Odd numbered posters: 12:30pm-1:15pm Even numbered posters: 1:15pm-2pm

Scientific Podium Sessions

FUNDING OPPORTUNITIES PANEL: FUNDING - WHERE AND HOW TO GET IT

DUANE J ROTH AUDITORIUM

2:00pm-2:55pm Funding Opportunities Panelists:

Yoshitake Cho - American Heart Association

Jordan Blondelle - Muscular Dystrophy Association Anastasia Gromova - National Science Foundation

Rachel Cook - UC San Diego Pre-Award Research Administrator
Health Sciences Sponsored Project Pre-Award Office

PODIUM SESSION 3

DUANE J ROTH AUDITORIUM

Session Chairs: Anastasia Gromova and Yoshitake Cho

2:55pm-3:10pm Florian Barthelemy

Identification and evaluation of drugs targeting the RyR pathway for combination therapy to boost exon skipping in human DMD patient

derived culture models.

3:10pm-3:25pm Simone Bersini

Engineering 3D human vascularized muscle environments for the study

of fibrosis.

3:25pm-3:40pm Vitor Martins

p300 is essential for the full effect of calorie restriction to enhance skeletal

muscle insulin sensitivity.

3:40pm-3:55pm Annunziata Crupi

CCL11 (Eotaxin-1) Contribution to Skeletal Muscle Aging (Sarcopenia).

15 minutes break

Please remember to take down your posters!

PODIUM SESSION 4

DUANE J ROTH AUDITORIUM

Session Chairs: Julius Bogomolovas and Marissa Lopez-Pier

4:10pm-4:25pm Yan Liang

A Newly Identified Interaction Between Desmoplakin and COP9

Signalosome Subunit 6 Reveals A New Mechanism Underlying Sudden

Death.

4:25pm-4:40pm Valeria Marrocco

Carp1-mediated signaling in dilated cardiomyopathy.

4:40pm-4:55pm Brian Cohn

Extracting muscle-to-force properties across arbitrary tendon-driven

limbs.

Biotech industry highlight:

4:55pm-5:10pm Riccardo Contu

Characterization of hiPSC-derived Cardiomyocytes on a micro-

engineered High Throughput Screening Platform.

.....

AMC AWARD SESSION AND CEREMONY

DUANE J ROTH AUDITORIUM

5:10pm-5:30pm **JEAN HANSON AWARD** FOR DIVERSITY IN SCIENCE AWARD

SUPPORTED BY STEMONIX

Helen Miranda

Using human stem cells to dissect the contribution of multiple cell types in

motor neuron disease.

5:30pm-5:45pm Young Investigator Awards &

POSTER AWARDS

SUPPORTED BY GENEA BIOCELLS

RECEPTION / NETWORKING / SOCIAL EVENT

BELLA VISTA CAFFÉ & TERRACE

starting at 5:45pm Reception

Please remember to take down your posters!

Scientific Podium Sessions

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 **12:30pm-2pm**, although AMC attendees are welcome to browse and discuss posters whenever they wish. Posters must be taken down <u>before</u> 5pm. *Please note that we cannot save any posters that remain hung up after that time*.

POSTER AWARDS



An independent jury of senior scientists selects the four best poster presentations during our poster session for the "AMC 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, CARDIAC FIBROBLASTS

NAME / TITLE Roberto Alvarez Jr Cardiac Cell Cycle Revealed by FUCCI. Chen Gao Cytosolic RBFox1 In Cardiac Fibrosis Regulation. Jessica Wang Lineage Contribution of Adult c-Kit+ Cardiac Progenitor Cells in Embryonic and Neonatal Development. Lizhu Lin

autonomous defect in cell fate determination and migration.

- 5. Mikella Robinson
 - Right Ventricle Fibroblasts Upregulate alpha-SMA signaling in a highthroughput platform

Loss of ETS-1 causes conotruncal defects through a neural crest cell

6. Lu Wang

Secreted miR-27a Induced by Cyclic Stretch Modulates the Proliferation of Endothelial Cells in Hypertension

7. Matthew Koppinger

The Effects of Pre-treatment with Lactobacillus reuteri on Cardiac Damage following a Myocardial Infarction

8. Jaimie Mayner

Genetic Heterogeneity Associated with the 9p21 Gene Locus in Vascular Smooth Muscle Cells

SECTION 2 - MUSCLE STRUCTURE, FUNCTION & SIGNALING

9. Manuel Rosa Garrido

Changes in cardiomyocyte 3D genome structure during heart failure.

10. Alice Zemljic-Harpf

Cardiac-specific overexpression of caveolin-3 expedites cardiac relaxation after adrenergic stimulation

11. Winston Stauffer

Endogenous Activating Transcription Factor 6 Preserves Heart Structure and Function in a Mouse Model of Myocardial Infarction-Induced Heart Failure

12. Stefanie Novak & Miensheng Chu (shared poster)

Increased cardiac arrhythmogenesis associated with gap junction remodeling with upregulation of RNA binding protein FXR1

13. Rafael Shimkunas

Mechano-chemo-transduction through nitric oxide pathway enhances contractility in cardiomyocytes

14. 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

Patrick Desmond

Novel Roles for Obscurin Proteins in Cardiac Muscle

16. Andrew D'Lugos

Acetaminophen (paracetamol) consumption alters the signaling and intracellular localization of mTOR in human skeletal muscle following resistance exercise

17. Suraj Chakravarthi Raja

The need for a pluralistic neuromorphic approach to neuromuscular modeling

18. Shivani Lakkaraju

Investigating the role of increased autophagy in Spinal and Bulbar Muscular Atrophy in vitro

19. Jianlin Zhang

Cardiomyocyte loss of mouse ZO-1 causes abnormal conduction but is dispensable for contractile function

20. Julius Bogomolovas

Regulatory events in the titin kinase signaling node hint at a pathological potential for the rare SNP D24728V

21. Anup Sarakki

Age-related Decline of the Adaptive Proteostasis Gene Program in Cardiac Myocytes

22. Simone Spinozzi

Exercise-induced alterations and loss of sarcomeric M-line organization in the diaphragm muscle of obscurin knockout mice

SECTION 3 - MUSCLE REGENERATION

Jenna Kastenschmidt

A potential role for group 2 innate lymphoid cells in muscular dystrophy

24. Francesca Boscolo

Muscle stem cells induce the development of rhabdomyosarcomas in dystrophic mice

25. Erik Blackwood

A small molecular activator of the ER proteostasis regulator, ATF6, Compound 147 is beneficial in the ischemic heart

26. Levna Zhao

Functional Maturation of Human iPSC-derived Cardiomyocytes and Assessment of Inotropic Compounds

27. David Sala Cano

The STAT3-Fam3a axis promotes muscle stem cell myogenic lineage progression

28. Jing Zhang

Human Induced pluripotent stem cells recapitulate arrhythmogenic right ventricular cardiomyopathy in a donor-reflective manner

29. Marissa Pier

Activation of Non-canonical Estrgoen-dependent Pathways to Mitigate Pathological Cardiac Remodeling

SECTION 4 - MUSCLE DEVELOPMENT

30. Katelyn Busse

An Improved Method to Differentiate Human Pluripotent Stem Cells to

Skeletal Muscle that Resembles Adult Myotubes

31. Analyne Schroeder

Cardiac Reprogramming: translational regulation of Hox genes by Nascent

polypeptide Associated Complex-Alpha (NAC)

32. Karissa Jade Muñoz

tbd

33. Kohei Miyata

Molecular function of Rp58 in de novo DNA methylation during myogenic

differentiation

34. Jordan Blondelle

Role of Cullin activities in skeletal muscle development

SECTION 5 - LATE BREAKING POSTERS

35. Ali Hussaini

Cardiac myosin-binding protein C structural dynamics-a time-resolved FRET biosensor for enhanced contractility in heart failure therapeutic discovery.

CAREER DEVELOPMENT / MEETING BENEFITS

EUREKA NETWORK-DUCHENNE 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 hosted by the Eureka Institute.

This years workshop will be chaired by



Elizabeth Gibbs UC Los Angeles



Brett Colson University of Arizona

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



"The workshop catalyzed new collaborations in translational medicine and illuminated the complex and interdisciplinary challenges of bringing a basic science discovery to patients."

Brett Colson, UofA

"The Eureka workshop gave me valuable insights into the components of successful academic/industry partnerships, and helped me identify ways to maximize the translational potential of my research."

Elizabeth Gibbs, UCLA

SUCCESSFUL INTERVIEWING FOR INDUSTRY WORKSHOP



Congratulations! You made it through the first round and on to an interview for what could be your dream job. *Now what?*

A successful interview will be key in obtaining that job offer. This workshop will focus on three key areas:

- · Preparation,
- Presence and
- Follow-up.

We will discuss how you can manage the process while highlighting what you can bring to the table and impressing the interviewer(s) enough to land that awesome job!

Debra Bressaw is currently the Business Coordinator & Marketing Director at *Genea Biocells*, a biotech whose purpose is to accelerate the development of therapies for patients with neuromuscular diseases by utilizing clinically relevant, stem cell-based discovery platforms.

Debra is Principal of Global Edge Strategies, a leadership development company focused on working with professionals & teams to reach their career and business goals faster and achieve greater fulfillment in their work and lives through individualized coaching and training programs.

Debra has over 20 years of business development, marketing & leadership experience working in for-profit and not-for-profit organizations.

Debra earned both her BA in Economics and M.Ed. from Rutgers University as well as Leadership and Coaching Certifications from Cornell and NYU.



Debra Bressaw

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www.linkedin.com/in/debrabressaw

FUNDING OPPORTUNITIES PANEL

Funding - Where and How to get it...

This panel session features career-stage dependent funding mechanisms. Recipients of Fellowships and Grants from federal and charitable organizations will outline their successful application strategy, and are available to answer questions from the audience. A Pre-Award Administrator from UCSD will highlight institutionally required steps to submit grant applications.

Yoshitake Cho

Dr. Cho is an Assistant Project Scientist at UC San Diego. He is currently a recipient of an American Heart Association (AHA) Scientist Development Grant.



Jordan Blondelle

Dr. Blondelle works as a Postdoctoral Scholar at UC San Diego. He recently received a Development Grant from the Muscular Dystrophy Association (MDA).

Anastasia Gromova

Ms. Gromova is a PhD student in the biomedical graduate program at UC San Diego. She successfully applied for and received a predoctoral fellowship from the National Science Foundation (NSF).





Rachel Cook

Ms. Cook works at UC San Diego's Health Sciences Sponsored Project Pre-Award Office as Senior Grant Analyst and Supervisor.

AMC AWARDS

The AMC meeting features a range of competitive awards.

All podium presentations will compete for the **AMC Young**Investigator Awards. One of these prestigious awards will be given to the most exceptional presenters in each of the four podium sessions. The AMC meeting will also feature four **AMC**Poster Awards to highlight research excellence and outstanding posters. The Young Investigator and Poster Awards are generously supported by:



This years meeting will also feature the AMC 'Jean Hanson Award' to promote diversity in science. This award, named in honor of Jean Hanson FRS, is aimed to promote diversity of speakers and attendees at the AMC meeting. Meeting attendees from disadvantaged backgrounds and underrepresented minorities were particularly encouraged to apply for this award! The Jean Hanson Award is generously supported by:

StemoniX

Dr. Emmeline Jean Hanson was a biophysicist and zoologist that is best known for her outstanding contributions to muscle research. While working with Hugh Huxley at MIT, Jean Hanson developed the 'sliding filament theory'.

After leaving the United States, Dr. Hanson became full Professor at King's College London in 1966 and made significant strides to elucidated the role and function of the muscle thin filament. In 1970, Prof. Hanson succeeded Prof. John Randall as Director of the Biophysics unit. Her groundbreaking research sits at the core of how muscles contract on a molecular level. Jean Hanson stood out as an exceptional women-scientist and pioneer in the muscle research field. Those who knew her described Jean as generous, open-minded, and most of all eager to help and mentor young researchers.



Dr. Jean Hanson. From Peter Knight. JMRCM 2004. 25(6). pp 447–450

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.



STAY CONNECTED

The AMC is present on Facebook, LinkedIN and Twitter. Sign-up and follow us to receive the latest news and updates on this and the next AMC meeting.















VENUES





SANFORD CONSORTIUM FOR REGENERATIVE MEDICINE

The AMC meeting will again 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. **Room 1013**, which holds one of the career events can be accessed through the lobby.

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



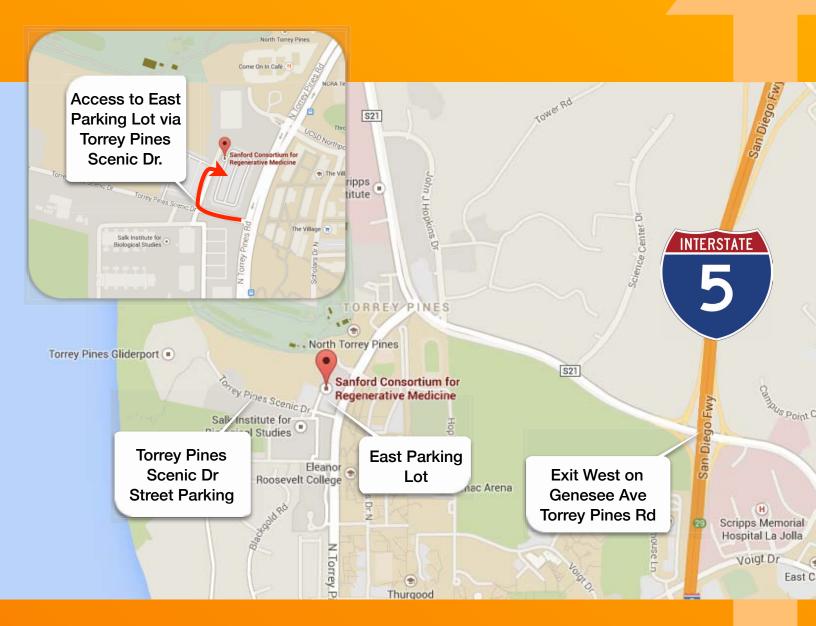
BELLA VISTA SOCIAL CLUB AND CAFFÉ

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, pay by stall-number at beginning of the day!).

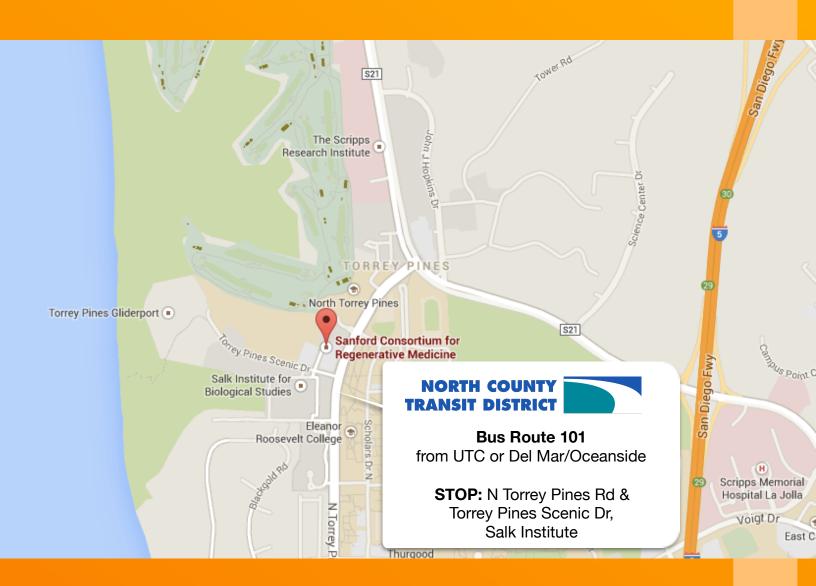
Please arrive early to get your parking spot, breeze through the registration, hang up your poster and grab a bite before the start of the scientific sessions.

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

Please use Google Maps (<u>LINK</u>) 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 US

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

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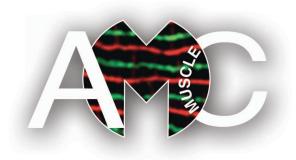
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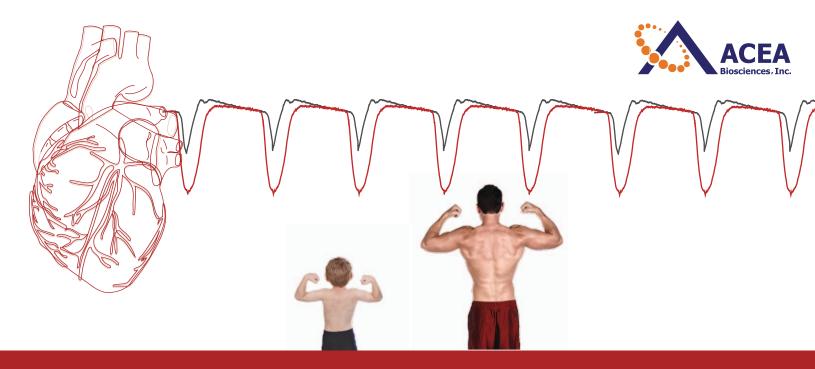
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Functional Maturation of Human iPSC-derived Cardiomyocytes Using the xCELLigence® CardioECR System

- Full Control of Beating Rate Enables Functional Maturation: Directed pacing feature improves functionality of iPSC cardiomyocytes and response to inotropic compounds.
- Powerful Multiplexing: Simultaneous readout of cardiomyocyte contractility, integrated ion channel activity, and viability.
- Easy and Flexible Work Flow: Simply plate the cells, start acquiring data, and perform combination treatments and chronic dosing.
- Superb Predictivity: Easily screen and quickly identify short-term and long-term cardiac toxicity early in drug development.



Visit our booth and poster #26 for more details.

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