FUTURE BIOTECH SUMMIT

2014

FEBRUARY 20-21, 2014 • SAN DIEGO, CA

Includes the three co-located conferences:

- 4TH UBIQUITIN RESEARCH & DRUG DISCOVERY
- 2ND ORPHAN DRUGS RESEARCH & COMMERCIALIZATION
- CELL & GENE THERAPY

Corporate Sponsors:  Media Partners:

Visit www.gtcbio.com for more information!
BENEFITS

GTC’s Future Biotech Summit will bring together an exciting balance of industry and academia, so that delegates have the unique opportunity to network with colleagues from different sectors and gain fresh perspective on the various research happening. The Summit includes three concurrent conferences, all of which will talk about complementary areas of the latest breakthroughs in the biotechnology field:

4th Ubiquitin Research & Drug Discovery
This premier conference will host an exciting group of professionals from the academic, biotech, pharmaceutical, and business communities, and will focus on drug discovery aspects within the ubiquitin pathway, and a balance of both academic and industry experts will facilitate discussion.

2nd Orphan Drugs Research & Commercialization
This meeting provides a forum for all stakeholders, from researchers, industry leaders, patient advocacy organizations, and regulatory experts to gather and share different perspectives on how to best guide the field forward. The program will include presentations on the latest scientific research, such as RNAi therapeutics and gene therapy; pricing and reimbursement challenges; clinical endpoints; and much more.

Inaugural Cell & Gene Therapy
Treatment of disease using cell and gene therapy is now a reality. With emerging technologies and new clinical trials being developed and launched at a growing rate, this meeting will discuss the evolving regulatory challenges and pre-clinical requirements, the development and applications of cell and gene therapy, immune responses, as well as business model and commercial milestones.

ABOUT

NETWORKING OPPORTUNITIES
Connect with high level professionals and renowned scientists in academia within the field in an intimate and highly interactive environment. We are expecting over 300 attendees for the entire Future Biotech Summit 2014.

SPONSORSHIP OPPORTUNITIES
- Sponsors/exhibitors will receive the delegate list a week before and a week after the conference.
- Exhibit space is shared between all conferences, providing you access to delegates at all conferences.
- Exhibitors are located in the same room where all networking functions take place. The coffee, snack stations, breakfast, and reception all occur in this room.
- Your corporate logo will be placed on the cover page of the conference brochures, the USB proceedings given to all conference delegates, and on the presentation screen during all breaks to enhance your company’s visibility at the event. Your logo will also be placed on the conference website agenda linked to your website.

For sponsorship and exhibit opportunities, please contact:
Amber Kempf
Account Executive
amber.kempf@gtcbio.com
Proteostasis Therapeutics is developing novel disease-modifying therapeutics that modulate protein chaperone and proteasomal degradation pathways within the cell to enhance the function and capacity of the protein homeostasis or Proteostasis Network. Our lead programs are in cystic fibrosis and protein aggregation diseases such as Parkinson’s disease.

BioSpherix will be exhibiting the Xvivo System, world’s first and only barrier isolator optimized for cells. Economical and practical alternative to cleanrooms for cGMP compliant production of human cells for clinical use. Get better contamination control, better process control, better quality control, better pocketbook control. Stop by the BioSpherix booth.
### AGENDA AT A GLANCE

#### Day 1 - Thursday, February 20, 2014

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Joint Session</th>
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<tbody>
<tr>
<td>7:00</td>
<td>Registration &amp; Continental Breakfast</td>
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<tr>
<td>7:55</td>
<td>Welcome &amp; Opening Remarks</td>
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<tr>
<td>8:00</td>
<td><strong>4th Ubiquitin Research &amp; Drug Discovery</strong></td>
<td><strong>2nd Orphan Drugs Research &amp; Commercialization</strong></td>
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<td></td>
<td><strong>Session I:</strong> Ubiquitin-like Modifications – New Opportunities</td>
<td><strong>Joint Session:</strong> Cell and Gene Therapy for Rare Diseases: From Research to Regulatory</td>
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<td><strong>Morning Networking &amp; Refreshment Break</strong></td>
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<tr>
<td>10:30</td>
<td><strong>Session II:</strong> Ubiquitin Proteasome System and Oncology</td>
<td><strong>Joint Session:</strong> Cell and Gene Therapy for Rare Diseases: From Research to Regulatory</td>
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<td>12:30</td>
<td><strong>Lunch on Your Own</strong></td>
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<tr>
<td>1:00</td>
<td><strong>Session III:</strong> Deubiquitinating Enzymes as Drug Targets</td>
<td><strong>Session II:</strong> Emerging Therapeutics in Rare Diseases</td>
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<td><strong>Afternoon Networking &amp; Refreshment Break</strong></td>
<td><strong>Session II:</strong> Emerging Technologies</td>
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<td>4:35</td>
<td><strong>Session III:</strong> Deubiquitinating Enzymes as Drug Targets</td>
<td><strong>Session III:</strong> Forming Cross-Stakeholder Partnerships</td>
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<td>5:40</td>
<td><strong>Networking Reception &amp; Poster Session</strong></td>
<td><strong>Session II:</strong> Emerging Technologies</td>
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#### Day 2 - Friday, February 21, 2014

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<tr>
<td>8:00</td>
<td><strong>4th Ubiquitin Research &amp; Drug Discovery</strong></td>
<td><strong>2nd Orphan Drugs Research &amp; Commercialization</strong></td>
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<td><strong>Session IV:</strong> Novel Drug Targets in the Ubiquitin System</td>
<td><strong>Session II:</strong> Emerging Technologies</td>
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<td><strong>Session III:</strong> Clinical Trials &amp; Research</td>
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<td>10:05</td>
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<tr>
<td>10:30</td>
<td><strong>Session IV:</strong> Novel Drug Targets in the Ubiquitin System</td>
<td><strong>Session V:</strong> Reaching the Markets &amp; Commercialization Challenges</td>
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<td>12:15</td>
<td><strong>Lunch Provided by GTC</strong></td>
<td><strong>Session III:</strong> Clinical Trials &amp; Research</td>
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<td>1:15</td>
<td>Session V: Ubiquitin Pathways in Neurobiology</td>
<td><strong>Session IV:</strong> Strategies for Commercialization</td>
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<tr>
<td>3:00</td>
<td><strong>Conferences Conclude</strong></td>
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*All times are approximate and subject to changes without notice*
4th Ubiquitin Research & Drug Discovery Conference - February 20-21, 2014

KEYNOTE SPEAKER

Tumor Suppression by Protein Complex Quality Control
Michael Rape
Howard Hughes Medical Institute
UC Berkeley

DISTINGUISHED SPEAKERS

Identification of a New Functional Motif in the P97 ATPase Cofactor FAF1
Gabriela Alexandru
University of Dundee

Identification of Selective DUB Inhibitors Targeting DDR Deficient Tumours
Xavier Jacq
Mission Therapeutics

Probing the Response to Protein Homeostasis Stress Using Quantitative Ubiquitin
Eric J. Bennett
University of California-San Diego

Co-translational Protein Quality Control in Normal Cells and in Disease
Claudio Joazeiro
The Scripps Research Institute

Surviving Proteasome Inhibition
Anne Bertolotti
MRC Laboratory of Molecular Biology

Studying Structure Function-relationships of 20S Proteasomes using Active Site Specific Inhibitors
Peter-Michael Kloetzel
Charité-Universitätsmedizin, Berlin

The Role of SUMOylation in Expression and Localization of the c-Myc Oncogene
Yuan Chen
Beckman Research Institute of the City of Hope

Function and Regulation of Cullin-Ring E3 Ligases
Thimo Kurz
University of Dundee

A Deubiquitinase Involved in Parkinson's Disease
Jacob Corn
Genentech

Inhibition of Proteasome Deubiquitinating Activity as a Novel Cancer Therapy
Stig Linder
Karolinska Institute; Uppsala University

Targeting Ubiquitin-like Protein Activation for Oncology Drug Discovery
Lawrence R. Dick
Takeda Pharmaceuticals International Company

NextGen Proteomics: A Cellular Snapshot of Enzymatic Activities +/- Compound Treatment
Christian Loch

For an updated speaker list visit: www.gtcbio.com/ubiquitin/speakers
4th Ubiquitin Research & Drug Discovery Conference - February 20-21, 2014

DISTINGUISHED SPEAKERS (cont.)

The Cop9 Signalosome Complex, A Potential Therapeutic Target
Aude Echalier-Glazer
Centre de Biochimie Structurale - CNRS - INSERM

Mechanisms of H2A Ubiquitination in Response to DNA Damage
Francesca Mattiroli
HHMI @ Colorado State University

Immunomodulatory Agents Lenalidomide and Pomalidomide Inducing Degradation of Ikaros and Aiolos via Modulation of the CRL4CRBN E3 Ubiquitin Ligase Complex
Anita Gandhi
Celgene Corporation

Ben Nicholson
Progenra, Inc

USP44 is a Multi-functional TumorSuppressor in Mice and Humans
Paul Galardy
Mayo Clinic

Targeting Siah Ligases: Implications for UPR and Cancer
Ze'ev Ronai
Sanford-Burnham Medical Research Institute

New Insights into ProteasomeMechanisms in Normal and Disease States
Alfred Lewis Goldberg
Harvard Medical School

Small Molecule Inhibitors of the Deubiquitinase USP7 Interfere with Ubiquitin Binding
Ingrid E. Wertz
Genentech

The Development of Usp14 Inhibitors as Disease-Modifying Therapeutics for Protein Aggregation Diseases
Markus Haeberlein
Proteostasis Therapeutics

Control of Cellular Dynamics and Signalling by Deubiquitinases
Sylvie Urbe
University of Liverpool

Uncleavable Ubiquitin as a Tool to Survey Ubiquitination Switches in Mammalian Cells
Tony T. Huang
NYU School of Medicine

The Making and reaking of Cullin-RING Ubiquitin Ligase Complexes
Deiter Wolf
Sanford-Burnham Medical Research Institute

Novel Drug Targets in the UPS: Potent and Selective P97 Inhibitors with in Vivo Activity
David Wustrow
Cleave Biosciences

For an updated speaker list visit: www.gtcbio.com/ubiquitin/speakers
KEYNOTE SPEAKERS

Stephen Groft
Director
Office of Rare Diseases, NIH

Harald Petry
Chief Scientific Officer
uniQure B.V.

DISTINGUISHED SPEAKERS & PANELISTS

PANEL DISCUSSION: Forming Strategic Partnerships
Robi Blumenstein
CHDI Foundation

Elizabeth McNeil
National Institute of Neurological Disorders and Stroke (NINDS)

PANEL DISCUSSION: Navigating the Regulatory Landscape
Carrie Burke
Shire HGT

Vanessa Rangel Miller
Patient Crossroads

PANEL DISCUSSION: Advocating For & Engaging With Patients
Jean Campbell
JF Campbell Consultants

Carl Morris
Pfizer

PANEL DISCUSSION: Patient Registries in Orphan Drug Development
Ron Christensen
Mapi Group

Alysson Muotri
University of California, San Diego

PANEL DISCUSSION: Patient Registries in Orphan Drug Development
Kathleen Coolidge
Genzyme

Using Human Neurons in a Drug-screening Platform for Autism Spectrum Disorders

Development of Cycloextrin for Treatment of Niemann-Pick C Disease

Daniel Ory
Washington University School of Medicine

For an updated speaker list visit: www.gtcbio.com/orphan/speakers
<table>
<thead>
<tr>
<th>Speaker</th>
<th>Title</th>
<th>Institution</th>
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<tbody>
<tr>
<td>Florian Eichler</td>
<td>Clinical and Biomarker Changes in Premanifest Huntington Disease: A Decade of the PREDICT-HD Study</td>
<td>Massachusetts General Hospital &amp; Harvard Medical School</td>
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<tr>
<td>Jane Paulsen</td>
<td>PANEL DISCUSSION: Navigating the Regulatory Landscape</td>
<td>University of Iowa</td>
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<tr>
<td>Mitchell H. Finer</td>
<td>PANEL DISCUSSION: Patient Registries in Orphan Drug Development</td>
<td>Bluebird Bio</td>
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<tr>
<td>Stanley Froehner</td>
<td>PANEL DISCUSSION: Advocating For &amp; Engaging With Patients</td>
<td>University of Washington, Seattle</td>
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<tr>
<td>Pat Furlong</td>
<td>Analysis of Orphan Drugs Approved Under Subpart H Accelerated Approval/ Fast Track</td>
<td>Parent Project Muscular Dystrophy (PPMD)</td>
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<tr>
<td>Brian Kaspar</td>
<td>Gene Therapy for Hemophilia A</td>
<td>Nationwide Children’s Hospital &amp; Ohio State University College of Medicine</td>
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<tr>
<td>Annie Kennedy</td>
<td>PANEL DISCUSSION: Navigating the Regulatory Landscape</td>
<td>Muscular Dystrophy Association</td>
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<tr>
<td>Karen Kozarsky</td>
<td>PANEL DISCUSSION: Forming Strategic Partnerships</td>
<td>RegenX</td>
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<tr>
<td>Trent Spencer</td>
<td>AAV Gene Therapy as a Platform for the Treatment of Rare and Inherited Disease</td>
<td>Emory University School of Medicine</td>
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<tr>
<td>Florian Eichler</td>
<td>PANEL DISCUSSION: Forming Strategic Partnerships</td>
<td>Massachusetts General Hospital &amp; Harvard Medical School</td>
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<td>Frank J. Sasinowski</td>
<td>PANEL DISCUSSION: Patient Registries in Orphan Drug Development</td>
<td>Hyman, Phelps &amp; McNamara</td>
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<td>Pat Furlong</td>
<td>PANEL DISCUSSION: Navigating the Regulatory Landscape</td>
<td>Parent Project Muscular Dystrophy (PPMD)</td>
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<td>Duane Morris LLP</td>
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<td>Adam Woodrow</td>
<td>PANEL DISCUSSION: Forming Strategic Partnerships</td>
<td>Pfizer</td>
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For an updated speaker list visit: [www.gtcbio.com/orphan/speakers](http://www.gtcbio.com/orphan/speakers)
KEYNOTE SPEAKERS

Karen S. Aboody
City of Hope National Medical Center & Beckman Research Institute

Clinical Development of an IL-12 Gene Therapeutic for the Treatment of Peritoneally Metastasized Cancers

Harald Petry
Chief Scientific Officer
uniQure B.V.

Robert Kotin
National Heart, Lung & Blood Institute / National Institutes of Health

AAV Gene Therapy as a Platform for the Treatment of Rare and Inherited Disease

Karen Kozarsky
ReGenX Biosciences

Dean Lee
University of Texas MD Anderson Cancer Center

James McLaughlin
Third Rock Ventures

The Tortoise and the Hare – Keeping Pace with the Tortoise (and Winning the Race Together)

Sean O’Bryan
Genzyme

For an updated speaker list visit: www.gctbio.com/cellandgenetherapy/speakers
DISTINGUISHED SPEAKERS (cont.)

Mitchell Finer  
BluebirdBio

Considerations for a Commercially Successful Cell Therapy Product

Timothy Fong  
Progenitor Cell Therapy

The Emergence of Gene Therapy Gene and Future Therapies

Ian Phillips  
Keck Graduate Institute of Applied Life Sciences

Lessons Learned in Initiating the Largest Gene Therapy Trial in Advanced Heart Failure

Jeff Rudy  
Celladon

HSV Vectors and the Treatment of Chronic Pain

Joseph Glorioso III  
University of Pittsburgh, School of Medicine  
NuvoVec srl.

The Emerging Technology is Intrinsic Biotechnology

Jonathan Sackner-Bernstein  
Neostem

Manufacturing and Clinical Development of MultiStem®, a Drug-like Cell Therapeutic

John Harrington  
Athersys

Harnessing the Potential of iPSC Technology

Dhruv Sareen  
Cedars Sinai Medical Center

Gene Therapy for Hemophilia A

Trent Spencer  
Emory University School of Medicine

A Small Molecule Approach for Relieving Lentiviral Vector Transduction Resistance in Human and Mouse Hematopoietic Stem Cells

Anthony Ting  
Athersys

Athersys

Noriyuki Kasahara  
University of California Los Angeles

Bruce Torbett  
The Scripps Research Institute

For an updated speaker list visit: www.gtcbio.com/cellandgenetherapy/speakers
DISTINGUISHED SPEAKERS (cont.)

Brian Kaspar
**Nationwide Children’s Hospital**

*Translational Studies in Neuromuscular Disease Using Gene Therapeutics*

Steven Kesten
**Cytori**

*Post-Approval Data Collection: A Key to Successful Commercialization*

Evan Unger
**University of Arizona Cancer Center**

*Gene Delivery with Ultrasound and Acoustically Active Carriers*

Fraser Wright
**Children’s Hospital of Philadelphia**

*CMC Supporting Early to Late Phase Clinical Development for an AAV Investigational Product*

For an updated speaker list visit: [www.gtcbio.com/cellandgenetherapy/speakers](http://www.gtcbio.com/cellandgenetherapy/speakers)
CONFERENCE DETAILS

REGISTRATION

Early Registration Discount:
20% until December 20, 2013
10% until January 20, 2014

Register for 2 the 3rd goes FREE with coupon code: RCDVB

To register online, visit: www.gtcbio.com/register/fb

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<th>Student</th>
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<td>1795.00</td>
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CALL FOR PAPERS

To be considered for a short oral presentation, please submit an abstract by January 20, 2014. Selected presentations will be based on quality of abstract and availability. Selected abstracts will be notified via email or telephone 2-3 weeks before the conference. Presentation slots fill up fast so please submit your abstract soon.

Click on the following conferences to submit an abstract:

- 4th Ubiquitin Research & Drug Discovery Conference
- 2nd Orphan Drugs Research & Commercialization Conference
- Inaugural Cell & Gene Therapy Conference

VENUE

Hyatt Regency Mission Bay San Diego
1441 Quivira Rd
San Diego, CA 92109
(619) 224-1234

Discounted Group Rate: $159.00 per night
Discount Deadline: Jan 29, 2014
Mention GTC to receive the group rate.

Online Reservations:
https://resweb.passkey.com/go/GTCFutureBiotechSummit

PROCEEDINGS

If you are unable to attend the conference you can purchase the proceedings by downloading the order form here.
Fill out the order form and fax it to 1 (626) 433-4433 or email it to proceedings@gtcbio.com.
Use this form to register up to 3 attendees – Please TYPE or PRINT your responses clearly

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**NAME OF CONFERENCE ATTENDING:**  FUTURE BIOTECH SUMMIT 2014

**PRICING OPTIONS:**

<table>
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<th>Standard Pricing per Attendee</th>
<th>Discounted Rate before 1/20/2014</th>
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<td>Student $805.50</td>
<td>Student $1,790.00</td>
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**PAYMENT METHOD (CHECK ONE) □ Check* □ Bank Transfer** □ Money Order □ Visa □ MasterCard □ American Express □ Discover**

Card #: ___________________________ Exp: Date: ________________ CSV #: ___________________________
Cardholder Name: ___________________________ Signature: ________________
Billing Address
Street: ___________________________ City, State, Zip: ___________________________

**HOW DID YOU HEAR ABOUT THIS CONFERENCE:** 2014 Brochure

**CANCELLATIONS**

All cancellations will be subject to a $195 cancellation fee. In order to receive a refund, you must submit a written notice of cancellation (by letter or fax) no later than 6 weeks prior to the conference. We regret that refunds will not be issued after this date. A conference voucher will be issued for use at any future GTC conferences within 12 months of cancellation. If you plan on sending a substitution in your place, the substitution must be from the same organization. Please notify GTC of any substitutions as soon as possible so the proper preparations can be arranged. In the event of a conference cancellation, GTC is not liable for transportation, hotel, or other costs incurred by registrants.

*Checks must be drawn on a US bank and made payable to Global Technology Community, or GTC. International money orders are also acceptable.

**Bank Transfer Information:**
Bank of America, 230 S. Myrtle Ave., Monrovia, CA 91016
ABA Routing #: 121-000-358, Account #: 0230604059
Swift Code: Bofaus6s

**BANK TRANSFER REQUIREMENTS:** When submitting a bank transfer, please add the name of the attendee on the transfer.