



Thursday, September 24 – Friday, September 25, 2015
Livestream Agenda
Huntington Ballroom

Summit Day One

8:00 – **Welcome & Meeting Kick Off- Summit Advisory Board Introduction**
Nicole Boice, Founder & CEO, Global Genes

8:15 – 8:55 **Opening Keynote**

Bonner Paddock - Ironman Competitor, Marathoner, Extreme Climber, Author, Patient Advocate, Cerebral Palsy

9:00 – 9:45 **Presentation**

Building a Game Plan - Beginning with the end in mind, otherwise the journey looks impossible
Presenter: Pat Furlong, President & CEO, Parent Project Muscular Dystrophy

9:35- 9:45 **Begin Q&A**

Tweet @GlobalGenes using #RARESummitQA with your questions

9:45 – Break

Sponsored by Aegerion Pharmaceuticals

10:00 – 10:55 **Fireside Chat**

The 10 things you need to do once you have a diagnosis – What are my next steps?
Discussion with Matt Wilsey, Parent Advocate, NGLY1, President, Grace Wilsey Foundation
Interviewed by Daniel Levine, Host, Global Genes' RARECast, Principal, Levine Media Group

10:45- 10:55 **Begin Q&A**

Tweet @GlobalGenes using #RARESummitQA with your questions

11:00 – 11:55 **Interactive Session**

The 10 things you need to pursue research for your disease. What are the options for research and why is it so hard? *Presenter: Brad Margus, Parent Advocate, Founder and Chairman, A-T Children's Project, Founder and CEO, Exigence Neurosciences*

12:00 – 1:00 No Session - Lunch Break

Sponsored by Alexion Pharmaceuticals

1:15 – 2:15 **Panel Presentation**

Sequencing, Data Sharing & Consent: Understanding your genetic information, owning your data and sharing with others to drive research and drug development

Moderator: Peter Goodhand, Executive Director, The Global Alliance for Genomics and Health

Panelists: Denise Perry, Senior Genetic Counselor, Illumina Clinical Services Laboratory (Sequencing 101), Megan Doerr, MS, LGC Principal Scientist, Governance Sage Bionetworks (Consent), David Glazer, Director of Engineering, Google Genomics

2:05- 2:15 **Begin Q&A**

Tweet @GlobalGenes using #RARESummitQA with your questions

2:15 – 2:30 Transition to Breakouts

2:30 - 3:20 **Clinical Trial Training – Pre-Drug Development**

When and how patients should become engaged with industry. Tools and strategies to take back to your community. *Host: Bray Patrick-Lake, BS, MFS, Director, Stakeholder Engagement, Clinical Trials Transformation Initiative, Duke University*

3:10- 3:20 **Begin Q&A**

Tweet @GlobalGenes using #RARESummitQA with your questions

3:20 - 3:40 **Grab & Go Session**

SBIR Grants and interesting funding partnerships

Host: Tim McDaniel, PhD, Senior Vice President, Emerging Opportunities, Translational Genomics Research Institute

3:40 - 4:30 **PCORI**

Host: Suzanne Schrandt, Deputy Director Patient Engagement, Patient-Powered Research Networks

4:20- 4:30 **Begin Q&A**

Tweet @GlobalGenes using #RARESummitQA with your questions

4:40 - 5:30 **Access to testing:** Insurance, Billing, Reimbursement

Host: Shannon Stasi, Licensed Genetic Counselor, Department of Laboratories and Pediatric Laboratory Utilization Guidance Services, Seattle Children's Hospital.

5:20- 5:30 **Begin Q&A**

Tweet @GlobalGenes using #RARESummitQA with your questions

5:30 – End of Day One

Summit Day Two

8:30 – 8:45 **Welcome and Day 2 Kickoff-** 21st Century Cures

Lisa Schill, Vice President, RASophies Network USA, and Max Bronstein, Senior Director, Public and Government Affairs, Everylife Foundation

8:45 – 9:45 **Presentation**

Mapping Drug Development: The Role Patients Play

Presented by Gayatri Rao, Director, Office of Orphan Products Development, FDA

9:35- 9:45 **Begin Q&A**

Tweet @GlobalGenes using #RARESummitQA with your questions

9:45 – 10:00 Break and Transition to Tracks

Sponsored by Retrophin

10:00 - 10:50 **Caregiving in the US:** Landmark Study Results & introduction to Rare Disease Caregiving Study

Host: Grace Whiting, Director, Strategic Partnerships, National Alliance for Caregiving

10:40- 10:50 **Begin Q&A**

Tweet @GlobalGenes using #RARESummitQA with your questions

11:00 - 11:50 **Clinical Care – Patients deserve the best care:** Building Comprehensive Care Centers

Hosts: Kelly Ranallo , Founder & President, Turner Syndrome Alliance, Dina Matos, Executive Director, CARES Foundation, and Stephanie Okey, Former Head of North America & SVP, Rare Diseases Business, General Manager US, Genzyme

11:40- 11:50 **Begin Q&A**

Tweet @GlobalGenes using #RARESummitQA with your questions

12:00 - 1:00 **No Session : Lunch Break**

Sponsored by Shire

1:10 – 2:05 **Show Me The Money** - Where to ask and when is appropriate.

Host: Deb Geraghty, VP Commercial Strategy, Dimension Tx

Alison Silva, Executive Vice President, Chief Operating Officer, Co-Founder, The Orphan Group

1:55- 2:05 **Begin Q&A**

Tweet @GlobalGenes using #RARESummitQA with your questions

2:10 – 3:05 **No Session - We will resume at 3:40 pm PST for the RARE Tribute to Champions of Hope- Friday Awards**

RARE Tribute to Champions of Hope- Friday Awards

Sponsored by Horizon Pharmaceuticals

3:40 – Event Kick off and Toast

Nicole Boice, Founder & CEO, Global Genes

3:45 – Awards Presentation Master of Ceremony & Welcome

Gail Moore, Patient Outreach Manager, Horizon Pharma

3:55 - Special Video Message

Chandra Wilson, Grey's Anatomy, Parent Advocate

4:00 – Champion of Hope – Science

Honoree: **Dr. David Fajgenbaum, Co-Founder, Executive Director, Castleman Disease Collaborative Network**

4:10 - Champion of Hope – Advocacy

Honoree: **Alison Rockett Frase, Founder and Vice President, Joshua Frase Foundation**

4:20 - Champion of Hope – Medical Care and Treatment *sponsored by SERMO*

Honoree: **Maria Escolar, SMD, MS, Director, Study of Neurodevelopment in Rare Disorders Program, Associate Professor, Pediatrics, University of Pittsburgh School of Medicine**

4:40 – David R. Cox Prize for Rare Compassion

Finalists Announced

Presented by: Kym Kilbourne, VP Advocacy - Global Genes

4:50 – Champion of Hope – International Teen Advocacy

Honoree: **Madi Vanstone, Patient Advocate, Cystic Fibrosis**

5:00 – Champion of Hope – Collaborations in Advocacy

Honorees: **Bill and Victoria Strong, Parent Advocates, Spinal Muscular Atrophy (SMA), Founders, Gwendolyn Strong Foundation**

5:10 - Champion of Hope – Collaborations in Science

Honorees: **Ben Lenail, Business Strategy Officer, ALD Connect and Advocate
Florian Eichler, MD, Board Member, ALD Connect and ALD/AMN Clinician, Associate Professor of Neurology, Harvard Medical School and Massachusetts General Hospital
Kathleen O'Sullivan, Board Member, ALD Connect, and ALD/AMN Advocate**