



**Global Genes™**  
Allies in Rare Disease

**2014**  
Year In Review

**Global Genes** is a leading rare disease advocacy organization with global reach to the worldwide rare community of patients, caregivers, and clinical partners. Each day, we promote the needs of our community under the unifying symbol of hope- the Blue Denim Genes Ribbon™.

**Our mission** is to eliminate the challenges of rare disease. We build awareness, educate the global community, and provide critical connections and resources that equip advocates to become activists for their disease.



## EDUCATIONAL PROGRAMS

### RARE WEBINARS

#### RARE Webinars

Online learning to inform and share resources for rare disease advocates and supporters. These 60 to 90 minute sessions bring together multidisciplinary expert panels that educate, inform and offer access to the latest information on a broad range of rare disease topics. In 2014, four webinars were hosted by Global Genes and reached over 10,000 people in the rare community.

#### Webinar Topics

- Newborn Screening
- Essential Health Benefits
- Understanding Rare Disease Registries
- Caregiver Resources (In association with National Caregivers Month)
- World Rare Disease Day
- Navigating Health Insurance Issues
- Using Successful Online Fundraising Strategies



hope  
it's in our

### RARE TOOLKITS

#### RARE Toolkits™

Print and online collaborative resources that educate rare disease advocates about issues critical to their journey. Each toolkit includes a resource section, FAQ section, video tutorials and patient advocate/expert testimonials. In 2014, seven Toolkits were produced and published through sponsorships with **Alexion**, **Genzyme**, and **Ambry Genetics**.

#### Toolkit Topics

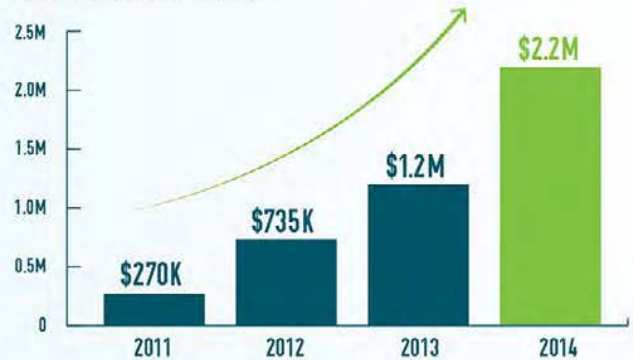
- Starting A Nonprofit: The First Steps
- Bringing Rare Disease to Capitol Hill
- Parenting A Child With A Life-Limiting Illness
- Genetic Testing
- Access to Unapproved Medicine
- Becoming an Empowered Patient
- Exploring Treatment Options for Rare Disease

## FINANCIALS

Even more important to our organization than the funds we raise, is how we utilize them. We are extremely conscientious with the donations you have entrusted to our care.

In addition to our programs and annual events, funds raised help maintain the **RARE Daily**, **RARECast** Podcast Series, scholarships and stipends, social media awareness and marketing & communications efforts for the rare community.

#### REVENUE GROWTH BY YEAR



## RARE FACTS AND STATS



Rare diseases affect approximately **30 million Americans** or **1 in 10 people**.



It is estimated that **350 million people worldwide** suffer from rare diseases.



Approximately **30% of children** with these debilitating diseases **will not live** to see their 5th birthday.

**7000**

Over **7,000 distinct types of rare diseases** exist with more being discovered every day.



Approximately **80%** of rare diseases are genetic.



Rare diseases **impact more people** than AIDS and Cancer combined.



**95% of rare diseases** do not have a single FDA approved drug treatment.

## ANNUAL EVENTS



### Tribute of Champions of Hope™

Each year, Global Genes hosts its annual Tribute to Champions of Hope Gala, where we have the good fortune of hearing from rare disease champions from around the globe who are proving that great things can be accomplished by dreaming, planning, taking action, and believing. The results of which, are quite inspiring.

In 2014, the 3rd Annual Tribute to Champions of Hope was host to over 500 attendees at the Hyatt Regency in Huntington Beach, California. Guests enjoyed performances from LA dance crew Culture Shock, up and coming singer/song writer Jessi Malay, and platinum recording artist Kris Allen.



## COLLABORATIVE PARTNERSHIP PROGRAMS



### RARE Corporate Alliance™

The mission of the RARE Corporate Alliance™ is to actively combine the resources and capabilities of industry leaders and patient communities to improve health and expedite access to effective therapies for all patients with rare diseases. In 2014, membership to the RARE Corporate Alliance increased 30%.

Specific initiatives of the Corporate Alliance include:

- Health Care Practitioner (HCP) Education
- Patient Education
- Patient Access



### RARE Meetups™

Patients and families fighting different rare diseases connect in their local communities to share experiences, knowledge, and gain support. In 2014, five aHUS Meetups and one Cystinosis Camp were hosted. Plans for 2015 include at least fifteen aHUS Meetups, one aHUS Pediatric Conference, one Narcolepsy Meetup, and one Cystinosis Camp.



### RARE Foundation Alliance™

Brings together rare disease foundations with a shared commitment to advocacy in order to build a stronger collective voice for the rare disease community. In 2014, the RARE Foundation Alliance multiplied from 130 rare disease foundations to 241, which is an increase of 85%.



### Undiagnosed Patient Program

Supports the start of the rare disease journey for undiagnosed patients through connections, resources and direct funding for complex diagnostics. In 2014, a pilot program to fund exome sequencing for 30+ patients was launched in partnership with clinical sequencing providers **UCLA Clinical Genomics Center** and **Parabase Genomics**.

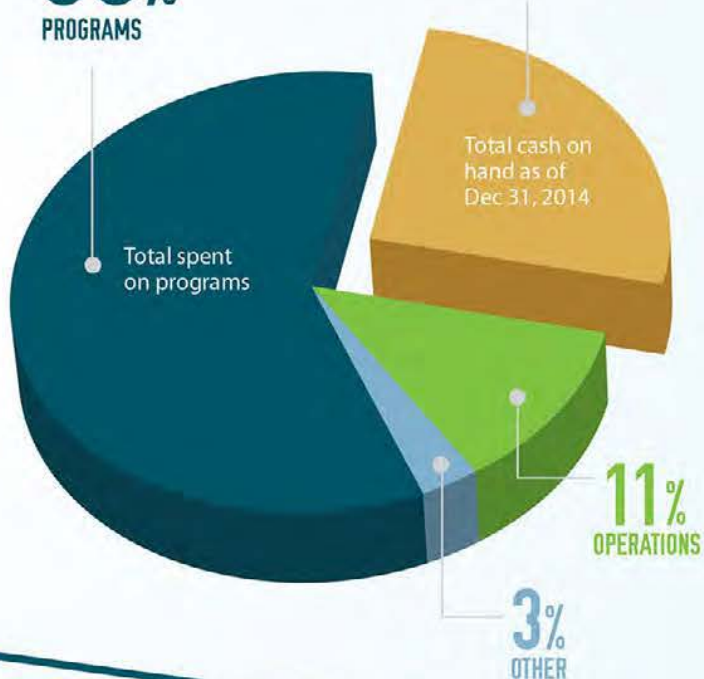


### RARE Concierge™

Assists patients and advocates faced with the challenges of locating resources and tools for reaching a rare disease diagnosis or potential treatments. In 2014, the RARE Concierge program provided information and support to over 500 inquiring members of the rare community.

60%  
PROGRAMS

26%  
TOTAL CASH



### RARE Patient Advocacy Summit™

The goal of the Summit is to have patient advocates and other rare disease stakeholders, become inspired, as well as walk away with new information and new connections. The world of rare disease is always changing, providing the community with new opportunities and new challenges; this is why empowering patient advocates to become successful activists is so important. In 2014, the 3rd Annual RARE Patient Advocacy Summit was host to over 350 in person attendees and over 1,000 who participated via LiveStream.



### World RARE Disease Day

World RARE Disease Day is the last day in February, and is an internationally recognized day of advocacy to call attention to rare conditions both in the United States and around the world. This day is celebrated annually on the last day of February each year. Global Genes participates in a wide variety of awareness activities leading up to the event and has helped catalyze numerous events around the world.



# COMMUNITY GROWTH



**2011** 5,000  
community members



**2014** 80,000+  
community members  
with reach to over 3 million

# JOIN THE MOVEMENT

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@GlobalGenes

Pinterest.com/GlobalGenes

## MEET THE TEAM AND BOARD

### TEAM



**Nicole Boice**  
Founder & CEO



**Andrea Epstein**  
Executive Director



**Barbara Lavery**  
VP, Strategic Alliances



**Amy Grover**  
Director, Operations



**Christina Fumia**  
Director, Marketing & Communications



**Lisa James**  
Manager, Technology



**Kathy O'Connor**  
Manager, Strategic Alliances



**Carrie Ostrea**  
Manager, Advocacy



**Katie Mastro**  
Manager, Programs



**Ashley Girtman**  
Manager, Events



**Jaymie Rodems**  
Coordinator, Marketing & Communications



**Ilana Jacqueline**  
Managing Editor, RARE Daily



**Nikki Berry**  
Coordinator, Web Design



**Brynn Dickson**  
Administrative Assistant

### BOARD



**Nicole Boice**  
Founder & CEO, Global Genes



**John Crowley**  
CEO, Amicus Therapeutics, Parent Advocate



**Natalie Douglas**  
CEO, Healthcare at Home



**Hugh Hempel**  
Founder, Solution Therapeutics, Parent Advocate



**Caroline Loewy**  
Biotechnology Executive/Consultant, Co-Founder, KCNQ2 Cure Alliance, Parent Advocate



**Brad Margus**  
Co-Founder & CEO, Exigence Neurosciences, Parent Advocate



**Peter Pellizzon**  
Founder, Hollencrest Capital



**Wendy White**  
Senior VP, Dohmen Life Science Services, Parent Advocate



**Matt Wilsey**  
President, Grace Wilsey Foundation, Parent Advocate



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