

Legislative Conference
February 26, 2013

About RDLA

- ▶ Designed to be a clearinghouse & advocacy center for all Rare Disease Legislation
- ▶ Provides resources & tools for individuals & organizations
- ▶ All events & resources are free & open to the entire Rare Disease Community
- ▶ Program of the EveryLife Foundation for Rare Diseases (501 c3 public charity)
 - All contributions to RDLA go directly to RDLA events

www.RareAdvocates.org



RDLA Resources:

- ▶ DC Office with workstation & meeting space for organizations to use when working in DC
- ▶ Consulting on legislative and grassroots strategies
- ▶ Online advocacy tools to contact Members of Congress
- ▶ Assistance with coalition building and support for legislation
- ▶ Coordination of Lobby Days & Congressional Caucus Briefings
- ▶ Conference calls/meetings to learn about legislation
- ▶ Clearinghouse of all legislation that affects rare disease patients



RARE VOICE

Awards Gala



Rare Disease Lobby Day

- ▶ Only Lobby Day that invites all rare diseases organizations & patients to participate together
- ▶ 2012 – 1st Lobby Day
 - 70 patient advocates on the Hill
 - Supported the ULTRA/FAST Legislation & Creating Hope Act
 - **both were included in FDASIA**
- ▶ 2013 – Legislative Conference & Lobby Day
 - 150 patient advocates on the Hill
 - Granted travel 75 scholarships



Goals for Conference & Lobby Day

- ▶ Educate patient advocates about the legislative and appropriations process
- ▶ Provide advocates with the tools to be successful in introducing and passing legislation
- ▶ Inform Congress about rare diseases and the many different needs of the patient community
- ▶ Build lasting relationships with Members of Congress and their staff
- ▶ **Advocate for NIH & FDA Funding**
- ▶ **Build the Rare Disease Congressional Caucus**



Who's attending today

- ▶ 160 Patient Advocates Registered
- ▶ 20 Industry Representatives
- ▶ **More than 50 different patient organizations/diseases represented**
- ▶ Thank you to our event partners:
 - Global Genes Project
 - The Sarcoma Foundation of America
 - The Rare Disease Report
- ▶ Industry Sponsors:

genzyme
A SANOFI COMPANY

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New Medicines. New Hope.®

ALEXION
pharmaceuticals

Shire

Pfizer

Bio BIOMARIN
BIOTECHNOLOGY
INDUSTRY ORGANIZATION

VIROPHARMA
INCORPORATED

Amicus
Therapeutics

VERTEX

- ▶ Adrenal Insufficiency United
- ▶ Alagille Syndrome Alliance
- ▶ ARPKD/CHF Alliance
- ▶ Association for Frontotemporal Degeneration
- ▶ BDSRA – Batten's Disease
- ▶ CADASIL Association
- ▶ CRB1 Fund
- ▶ Curing Retinal Blindness Foundation
- ▶ Desmoid Tumor Research Foundation
- ▶ EDSers United
- ▶ Fragile Skin Foundation
- ▶ Global Genes Project
- ▶ Hereditary Disease Circle
- ▶ Histiocytosis Association
- ▶ Hope for Trisomy 13 and 18
- ▶ Klippel Feil Syndrome Alliance
- ▶ Lam Foundation
- ▶ Lymphangiomyomatosis
- ▶ Lipodystrophy United

- MAGIC Foundation
- Mastocytosis Society
- MLD Foundation
- MPS Society
- Multiple System Atrophy Coalition
- Myocarditis Foundation
- National Patient Advocate Foundation
- National PKU Alliance
- NOMID Alliance
- Oxalosis & Hyperoxaluria Foundation
- PANDAS Resource Network
- Pediatric Hydrocephalus Foundation
- Phelan–McDermid Syndrome Foundation
- Sarcoma Foundation of America
- Sickle Cell Disease Association of America
- Syndromes Without a Name
- Undiagnosed Disease Network
- VT Coalition for Disability Rights
- Williams Syndrome Family of Hope

The Power of the United Voice of the Rare Disease Community

- ▶ **On July 9, 2012** President Obama signed the Food & Drug Administration Safety & Innovation Act (FDASIA)
 - landmark legislation that will encourage the development of new treatments for patients with rare diseases
- ▶ More than 10,000 emails were sent from constituents to Congress from RDLA's Congressional action center to support the legislation
- ▶ During the negotiations to reconcile the House and Senate bills, 121 organizations signed onto a letter in 24 hours championing the inclusion of the strongest rare disease provisions in the final bill.
- ▶ **All five provisions supported in the letter were included in the final bill**



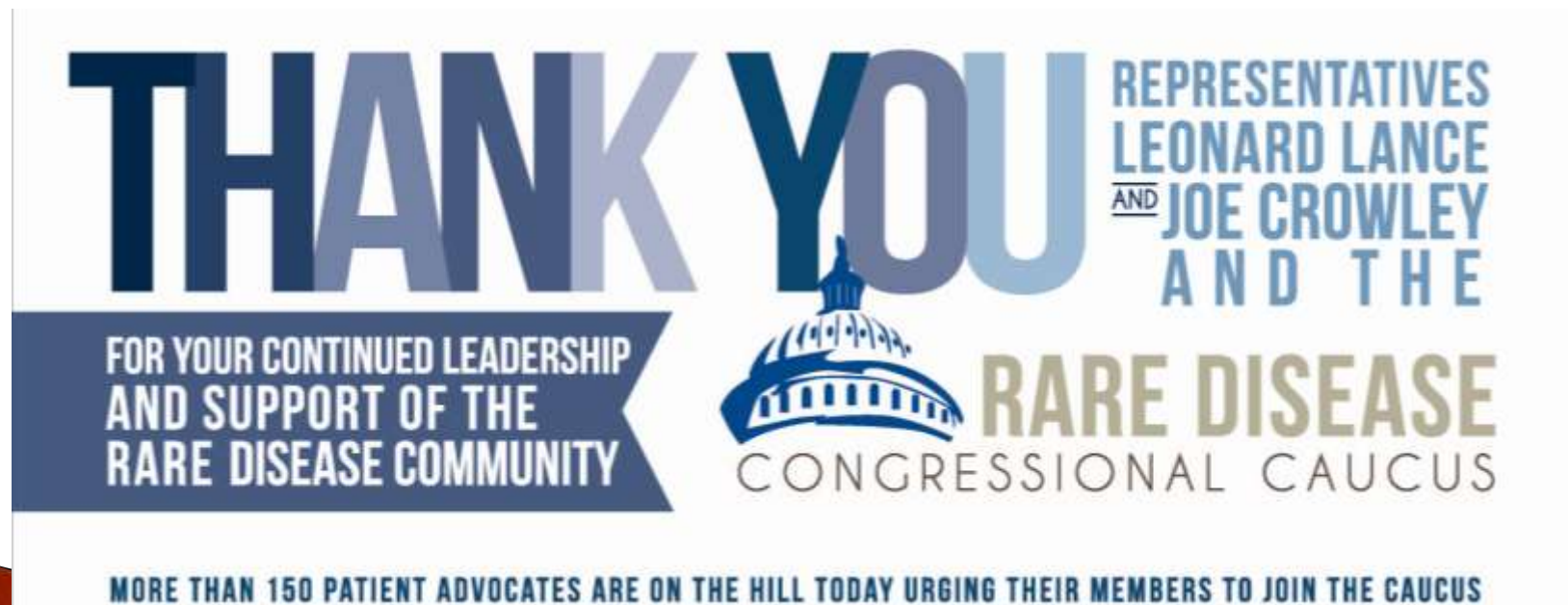
Logistics for Today

- ▶ Morning Sessions are series of presentations about successful advocacy
- ▶ Take a short lunch break to grab a boxed lunch
- ▶ Afternoon Sessions address some specific policy issues that will be affecting rare disease patients
- ▶ Afternoon break – ends the day for those who are not going to the Lobby Day
- ▶ Come back from break & sit with your state & regional Lobby Day teams
- ▶ Today's Presentations will be filmed & posted online for reference



Bonus Stuff

- ▶ Rare Disease Report
 - Is willing to take 5 minutes with you to record your story this morning
- ▶ Roll Call & Politico Full Page Ads



THANK YOU REPRESENTATIVES
LEONARD LANCE
AND JOE CROWLEY
AND THE
RARE DISEASE
CONGRESSIONAL CAUCUS

FOR YOUR CONTINUED LEADERSHIP
AND SUPPORT OF THE
RARE DISEASE COMMUNITY

MORE THAN 150 PATIENT ADVOCATES ARE ON THE HILL TODAY URGING THEIR MEMBERS TO JOIN THE CAUCUS

This is your day

- ▶ Ask questions to speakers
- ▶ Pose potential policy solutions
- ▶ Network & make new friends
- ▶ There would be no lobby day without all of you
- ▶ Thank you for being here today

You are the voices of the rare disease community!

