@RareAdvocates

*Silence your cell phones please*
Thank You!

STEVESMITHPLANS - LLC
Welcome

Steve Smith
CEO and Management Consultant
SteveSmithPlans LLC

SteveSmithPlans.com
Overview

Carol Kennedy, CFRE
Chief Development Officer
EveryLife Foundation for Rare Diseases
The Need

- 1 in 10 ... 30 million Americans affected
- Over 7,000 different rare diseases
  - 83% of rare diseases have less than 6,000 US patients
- Over 50% of the diseases affect children
  - 30% of children will not live to see their 5\textsuperscript{th} birthday
- Average diagnostic odyssey is 7 years
The Medical Science

• **Only 289 of the 7,000 rare diseases have an FDA approved treatment**
  • That’s less than 5%

  ▸ The science exists to treat many of these diseases

  ▸ Development of treatments is challenging
    ◦ Difficult to get investment with small populations
    ◦ Complicated regulatory environment
Founded by Dr. Emil Kakkis, MD, PhD in 2009, researcher at UCLA

Approached by a father of a child with MPS 1 who had raised money for a treatment

Dr. Kakkis saved Ryan Dant’s life by developing an enzyme replacement therapy (Aldurazyme)

Worked at BioMarin for several years developing rare disease therapies

After leaving BioMarin, decided to start the Foundation
Accelerating Biotech Innovation through science-driven public policy

We Believe:

- No disease is too rare to deserve treatment
- All new drugs for rare diseases should be safe and effective
- We could be doing more with the science we already have
We are unique – we are both a

- Public Policy Organization

and a

- Patient Organization
Core Principles

*We seek to achieve our goals* ...

... by advocating practical and scientifically sound policies

... to increase the predictability of the regulatory process

through scientific analysis and dialogue, grassroots support, and expert-led workshops
Over 500 new rare disease treatments developed since the passage of the Orphan Drug Act 30 years ago
  ◦ 19 new rare disease drugs were approved in 2015

Innovation Gap:
  ◦ Many of the new treatments are for diseases that already have treatments available
  ◦ Only 7 of the 15 drugs approved in 2015 were for diseases that had no other approved treatment

WE MUST DO BETTER
  ◦ Public policy is needed to close the innovation gap
Public Policy & Patients

1) Community Programs
2) Promote Awareness
3) Public & Scientific Policy
4) Grassroots Advocacy
COMMUNITY SUPPORT!
Over $100,000 in stipends to ensure FDA & Congress hears directly from patients

AWARENESS!
RareArtist shares rare disease and celebrates the talents of our community. 8th annual contest opens on Facebook on July 31st closes on December 19th

INVESTMENT!
Rare Affair informs investors about the impact policy changes have on investment potential

PHYSICIAN TRAINING!
Fund North American Metabolic Academy to recruit & train the next generation of rare disease physicians & scientists
Public & Scientific Policy

POLICY SOLUTIONS!
Science-driven policy changes to improve drug development process.

REGULATORY SCIENCE!
Annual workshop to build the science needed to implement policy solutions. Next workshop on September 13th in Washington, DC

EARLY DIAGNOSIS!
Speeding the implementation of new newborn screens as they are added to the RUSP. CA SB 1095 is model legislation.

COLLABORATION!
Formalize relationships with patient orgs and industry to ensure we receive community input about policy needs.
Grassroots Advocacy

RDLA – Rare Disease Legislative Advocates is a program of EveryLife Foundation
Rare Disease Legislative Advocates

- Educate patient advocates about how policy impacts the availability and access to treatments
- Provide resources to be successful legislative advocates
- Build awareness on Capitol Hill and ensure Congress hears directly from patients
- RareAdvocates.org … an advocacy training center and legislative clearinghouse for all rare disease policy issues
Advocacy Events

330+ patient advocates learning how policy impacts access to treatments and meeting with their Members of Congress

Meeting with your Members during August recess, Regional Conferences train advocates

A permanent rare disease voice on Capitol Hill through regular briefings to inform Congress/Bicameral

Honoring advocates who give patients a policy voice

*all events are free to patients
Please Register by July 5th for In-District Lobby Days

Get the opportunity to meet your Members of Congress locally!
Honors Advocates who give patients a voice on Capitol Hill – 2016 will celebrate 5 years
Nominations are open now and will close on July 31st. Nominate someone today!
www.rareadvocates.org
Nominees attend the event in Washington, DC.

Winners receive the Abbey Award. The “Abbey” represents the “rare voice” speaking on behalf of patients, especially children, who might not otherwise be heard.

The award, with her blessing, was named for Abbey Meyers, founder of the National Organization for Rare Disorders (NORD).
RARE DISEASE WEEK ON CAPITOL HILL
February 27th – March 2nd
Monday, Feb. 27th: Reception and Documentary Film Screening
Tuesday, Feb. 28th: Legislative Conference
Wednesday, March 1st: Lobby Day Breakfast, and scheduled visits on Capitol Hill for Lobby Day
Thursday, March 2nd: Rare Disease Caucus Briefing and RareArtist Reception on Capitol Hill
2016 was our fifth and most successful year!

- 330+ patient advocates registered
- 130+ patient organizations were represented
- We scheduled 228 Hill meetings for advocates
Social Media

494 people tweeted nearly 1900 times about Rare Disease Week on Capitol Hill with our hashtag (#RareDC2016) including Members of Congress and the House Energy and Commerce Committee.
Please join us!
Save the date:
February 27th–March 2nd
Meet the Team!

JULIA JENKINS
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JJENKINS@EVERYLIFEFOUNDATION.ORG

CAROL KENNEDY
CHIEF DEVELOPMENT OFFICER
CKENNEDY@EVERYLIFEFOUNDATION.ORG

MAX BRONSTEIN
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STEPHANIE FISCHER
SENIOR DIRECTOR, PATIENT ENGAGEMENT & COMMUNICATIONS
SFISCHER@EVERYLIFEFOUNDATION.ORG
Meet the Team!

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DEPUTY DIRECTOR OF COMMUNICATIONS & PATIENT PROGRAMS
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LISA SCHILL
RDLA SPECIAL EVENTS COORDINATOR
LSCHILL@EVERYLIFEFOUNDATION.ORG

SUE COLTON
DIRECTOR OF OPERATIONS & DEVELOPMENT
SCOLTON@EVERYLIFEFOUNDATION.ORG
Messages from Members of Congress

Ed Hynes
Legislative Correspondent
U.S. Senator Richard Durbin (D–IL)
U.S. Senator Mark Kirk (R–IL)

Please vote for CURES

Max
Messages from Members of Congress

Cindy Wellwood – Burke
Director of Constituent Services and Advocacy
Senator Mark Kirk (R-IL)
Why Public Policy Should Be a Top Priority for Rare Disease Patients

Moderator: **Marilyn Vetter**, Vice President, Government and Public Affairs, Horizon Pharma

**Dorelia Rivera**, Parent Advocate

**Tracy VanHoutan**, President, Noah’s Hope, and Vice President, Batten Disease Support and Research Organization

**Clyde W. Yancy, MD, MSc, MACC, FAHA, MACP**, Vice Dean, Diversity and Inclusion, Northwestern University, Feinberg School of Medicine
Marilyn Vetter
Vice President
Government and Public Affairs
Horizon Pharma
Dorelia Rivera
Parent Advocate
Tracy VanHoutan
President
Noah’s Hope
Vice President
Batten Disease Support and Research Organization
Tracy VanHoutan

- Father of 4 children; 2 affected by Late Infantile Neuronal Ceroid Lipofuscinosis (CLN2 disease) - more commonly known as Batten Disease
- Noah- Age 11, Laine- Age 10
- Noah lost his fight with Batten this past March.
- VP Board of Directors - Batten Disease Support and Research Association (BDSRA)
- President & Founder: Noah’s Hope--Hope4Briget Foundation
What is Batten Disease?

- Neuronal Ceroid Lipofuscinosis (NCL)
- Autosomal recessive
- Ultra Rare - affecting 2-4 births out of 100,000
- 12 different disorders with different defective genes
- Children develop normally until onset age, then regress
- Accumulation of waste material in the brain
- Vision loss, ataxia, seizures, loss of motor function
- Always fatal

There is no FDA approved therapy for Batten Disease……YET!
Noah & Laine went from being happy, energetic children....

...to children needing 100% care within a few short years.
Drug Development Timeline

- **Discovery**
  - FDA License
  - Small Molecule & Peptide
  - Protein-Based Drug

- **Preclinical**
  - "NCE"
  - "NBE"

- **Ph I-III**
  - "NME"*
  - "New BLA"

- **Approval**
  - NDA
  - BLA

- **Market**
  - "Generic"
  - "Biosimilar"
  - "Follow-on biologic"

- **Generic Approval**
  - ANDA

- **Begin to Market Branded Drug**

- **Begin to Market Generic Drug**
But THIS is what time looked like to us ...
Connecting with rare disease umbrella groups was critical for us to achieve our scientific, organizational & legislative goals.

- EveryLife Foundation
- Rare Disease Legislative Advocates
- Global Genes
- NORD
- Genetic Alliance
- Faster Cures
Our kids are literally drowning in the middle of the ocean!
We are looking for something like THIS!
Intracerebroventricular Cerliponase Alfa (BMN 190) in Children with CLN2 Disease: Results from a Phase 1/2, Open-Label, Dose-Escalation Study

Angela Schulz, MD, PhD
Children's Hospital
NCL Specialty Clinic
International Centre of Lysosomal Storage Disorders (ICLD)
University Medical Center Hamburg-Eppendorf
Hamburg, Germany
After clinical trial data released a few months ago...we may actually have THIS!
Fighting Against #BattenDisease #RareDisease

A Fifth Season.....Brightening the landscape of Batten Disease Nov. 15, 2014
Clyde W. Yancy, MD, MSc, MACC, FAHA, MACP
Vice Dean
Diversity and Inclusion
Northwestern University
Feinberg School of Medicine
@RareAdvocates
Rare Disease Legislation in the Queue

Jennifer Bernstein
Executive Vice President
Horizon Government Affairs

Lisa Schill
RDLA Special Events Program Coordinator
Vice President
RASopathies Network USA
My Journey into the Rare Disease World
Our mission is to advance research of the RASopathies by bringing together families clinicians and scientists.

www.RASopathiesnet.org
Did you know??

11–14 years to develop a new treatment!!

Up to 2.6 BILLION dollars!!

That is A LOT of Bake Sales!!
Drive LEGISLATION to Create Change
&
CURE THE PROCESS
- 7,000 diseases, 95% of which have no FDA-approved tx
- Average 7+ year diagnostic journey, wasting health care dollars and time, meanwhile disease may progress unchecked
- Patients often prescribed medicines off-label:
  - Poor Data
  - Limited efficacy
  - Difficult to get reimbursement
De-risking Rare Disease Drug Development since 1983: The Orphan Drug Act

- INCENTIVES!!!
  - Exclusivity
  - Tax Credits
  - User fee exemptions
  - Eligible for fast review at FDA
500+ Rare Disease Drugs in Development

Medicines in Development
FOR RARE DISEASES

- Autoimmune Diseases: 25
- Blood Cancer: 82
- Blood Disorders: 9
- Cancer: 151
- Cardiomyopathy: 12
- Digestive Disorders: 10
- Eye Disorders: 10
- Genetic Disorders: 148
- Growth Disorders: 7
- Infectious Diseases: 31
- Neurologic Disorders: 38
- Respiratory Diseases: 11
- Transplantation: 13
- Other: 49

Note: Some medicines may be in more than one category.

www.PhRMA.org
Problem: 100s of safe and effective medicines, but very few had ever been tested in neonates, infants, and children

BPCA offered an incentive for conducting clinical trials in children
- Gain SIX months of exclusivity

Impact: 426 label changes
A six-month exclusivity extension for therapies repurposed for a rare disease indication

- Common→Rare or Rare → Rare
- Compound is only eligible for 1 extension
- Would stack with BPCA
- Could double the number of rare disease treatments available to patients
Zometa
- FDA indication for hypercalcemia of malignancy (higher levels in calcium in blood due to cancer)
- Off-label use for Giant Cell Lesions of the Jaw
OPEN ACT – Champions

Sen. Hatch (R–UT)
Sen. Klobuchar (D–MN)

Rep. Bilirakis (R–FL)
Rep. Butterfield (D–NC)

+ 5 Bipartisan Cosponsors!
+ 19 Bipartisan Cosponsors!
Support for the OPEN ACT

- Overwhelming support from patient community:

166 National Patient Orgs and Growing!

Still time to sign–on
The ASK

- For House & Senate:
  - PLEASE Co–Sponsor the OPEN ACT (HR 971/S 1421)
  - See Sample Scripts & One Sheets
  - Questions welcome!
It started with a visit from two little princesses.
CURES New Jersey Roundtable

21st Century Cures Roundtable
August 4, 2019 • 10:00AM
Celgene • Summit, NJ

Agenda:

- Introduction & Welcome Remarks – Rich Bagger, Senior Vice President, Corporate Affairs and Strategic Market Access, Celgene
- Panelist Introductions
- Moderated Discussion
- Audience Q&A

Panelists:

- Mark Alles
  COO and President, Celgene
- Ricki Fairly
  President & Thought Leader, DOVE Marketing
- Scott Mellis, M.D., Ph.D.
  Vice President, Translational Medicine, Regeneron
- Dr. Fernando Muzzio
  Distinguished Professor, Chemical and Biochemical Engineering, Rutgers University
- Dr. Paul Reider
  Pharmaceutical Specialist and Lecturer, Princeton University
- Brian Rosen
  Leukemia & Lymphoma Society
- John E. Runnels
  General Partner, The Vertical Group
- Debra L. Weitz, Ph.D.
  CEO, New Jersey Association of Mental Health and Addiction Agencies
Bipartisan Bill

The Committee spent over 1 year gathering input from stakeholders and released 4 draft bills

Includes a number of important provisions for rare disease patients
Rare Disease Community Priorities:

- Orphan Product Extensions Now, Accelerating Cures & Treatments (OPEN ACT)
- Improve biomarker qualification
- Improve FDA’s ability to recruit & retain staff & keep up on the latest science
- Billions in NIH & hundreds of millions in FDA Funding
- Expanding Hope Act (Priority Review Vouchers)
- Neurological Disease Surveillance
- Compassionate Use Reform & Enhancement Act
- Patient Focused Drug Development
Dear Congressman,

My name is Max. I am 12 years old and live in [City, State]. I am writing to you because I want you to know how important the opening of the [Clinic/Store/Project] is to me and my family.

The [Clinic/Store/Project] will provide vital services to our community and will help many families like mine. It is important to us that the location of this clinic is near our homes. We have been waiting for this to happen for many years.

I encourage you to support the opening of this clinic and to help us in any way you can. This is an important issue for our community and it will make a real difference in the lives of many people.

Thank you for your time and effort in this matter.

Love,
Max

Dear US Senator,

My name is Max. I am 12 years old and live in [City, State]. I am writing to you because I want you to know how important the [Clinic/Store/Project] is to me and my family.

The [Clinic/Store/Project] will provide vital services to our community and will help many families like mine. It is important to us that the location of this clinic is near our homes. We have been waiting for this to happen for many years.

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Love,
Max

Dear Congresswoman,

My name is Max. I am 12 years old and live in [City, State]. I am writing to you because I want you to know how important the [Clinic/Store/Project] is to me and my family.

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Thank you for your time and effort in this matter.

Love,
Max

H.R. 971
swimming
biking
playing
Smiling
#curesin4words

OPEN your heart to #curesnow

Dear Santa,
1. Curses
2. Skylanders
3. Star Wars
4. LEGO

Love, Max
Create Relationships with Staff
Who are the KOL’s?
21st Century Cures in Committee
51–0 Vote
21st Century Cures Floor Vote
Onto the Senate!
NOT SURE IF SHOULD DO
*Cough Cough*
OR CRICKETS CHIRPING
01.19.16

Chairman Alexander Announces Committee Schedule for “Step by Step” Consideration of Biomedical Innovation Bills

Feb. 9 meeting will be 1st of 3 to produce companion legislation to 21st Century Cures Act already passed by House

“The House has completed its work on the 21st Century Cures Act. The president has announced his support for a precision medicine initiative and a cancer ‘moonshot.’ It is urgent that the Senate finish its work and turn into law these ideas that will help virtually every American.”

WASHINGTON, D.C., Jan. 19 – Chairman Lamar Alexander (R-Tenn.) today announced the Senate health committee will hold its first executive session considering bills on biomedical innovation on Tuesday, Feb. 9. At that committee
Advancing Targeted Therapies for Rare Diseases Act of 2015 (Sens. Bennet, Burr, Warren, and Hatch)
- A bill to allow the sponsor of an application for the approval of a targeted drug to rely upon data and information with respect to such sponsor's previously approved targeted drugs.

Advancing Research for Neurological Diseases Act of 2015 (Sens. Isakson & Murphy)
- To amend the Public Health Service Act to provide for systematic data collection and analysis and epidemiological research regarding Multiple Sclerosis (MS), Parkinson’s disease, and other neurological diseases.

Advancing Hope Act of 2015 (Sens. Casey, Isakson, Brown & Kirk)
- Amends the Federal Food, Drug, and Cosmetic Act to expand (and make permanent) the priority review voucher program for rare pediatric diseases to include treatments for sickle cell disease and pediatric cancers.

Advancing Precision Medicine Act of 2016
- Encourages the Secretary of Health and Human Services (HHS) to establish a Precision Medicine Initiative (PMI) in collaboration with the National Institutes of Health (NIH), the Food and Drug Administration (FDA), and the Office of the National Coordinator for Health Information Technology.

Patient Focused Impact Assessment (PFIA) Act (Sens. Wicker, Klobuchar, Isakson, Bennet, Donnelly)
- Urges greater transparency of product approval at FDA.

FDA & NIH Workforce Authorities Modernization Act
No Agreement (yet) on NIH/FDA Funding

OPEN ACT
  - Hopeful that OPEN ACT will be included when package goes to Senate floor or in conference.
Innovation is needed in the fight against rare diseases

Congress should encourage companies to repurpose existing drugs for rare diseases, potentially doubling the number of treatment options for patients.

By Klane K. White and David Fajgenbaum
Senate do you hear us??
#CuresNow not tomorrow!

Time is running out!

#CuresNow Max
Challenges Remain in the Senate

- FUNDING for NIH, FDA, and the OPEN ACT
- Cures package could get bundled with opioid legislation
- Clock is ticking: District Work Period, gun control, election–year politics, appropriations bills
- TIME IS RUNNING OUT

Path Forward:
The ASK

- House of Representatives: Please encourage your colleagues in the Senate to *bring 21st Century Cures to a floor vote!*
- For the Senate: Please *bring 21st Century Cures to a floor vote!*
- See One Sheets & Script
- Questions Welcome!
FEDERAL APPROPRIATIONS
ADVOCATING FOR NIH & FDA

National Institutes of Health

U.S. Food and Drug Administration
The Appropriations Process
The Appropriations Cycle—How it Should Work

The Annual Federal Budget Process

1. Federal agencies submit budgets for review
2. President submits budget request to Congress
3. House Appropriations subcommittee markups & votes
4. Senate Appropriations Committee markup
5. Conference Committee
6. President vetoes or signs into law
In An Ideal World, This Happens:

**Annual Budget Process - Timeline**

- **February**
  - President submits budget proposal to Congress

- **April 15**
  - Congress adopts a budget resolution

- **May/June**
  - Appropriations Committees Begin Reporting Bills

- **Late July**
  - House and Senate finish considering appropriations bills

- **Fall/Winter**
  - House and Senate reconcile, send bills to President

- **October 1**
  - New Fiscal Year Begins
In Reality...

KICKS CAN DOWN THE ROAD
TAKES VACATION
NIH estimates that it invests $3.7B in rare disease research annually!
The BRDPI measures changes in the weighted average of the prices of all the inputs (personnel, supplies, equipment) purchased with NIH budget to support research.

What does this mean?
- Level funding from one fiscal year to the next is technically a large decrease, due to the decrease in purchasing power.

The following pattern of growth in the BRDPI is projected through 2020:
- 2.2 percent for FY 2015
- 2.4 percent for FY 2016
- 2.7 percent for FY 2017
- 2.9 percent for FY 2018
- 3.1 percent for FY 2019
- 3.2 for FY 2020
NIH helps the RASopathies

Use of Rapamycin for the Treatment of Hypertrophic Cardiomyopathy in Patients with LEOPARD Syndrome

LEOPARD syndrome (LS) is a rare genetic disease affecting only about 200 patients worldwide. Nearly all cases of LS result from mutations in a single gene, PTPN11. In the heart, the most common manifestation of LS is hypertrophic cardiomyopathy (HCM), a thickening of the walls of the heart. There is no existing treatment for LS patients who have HCM, and end-stage heart failure can lead to early death. The lead collaborator has shown that rapamycin can prevent and reverse HCM in animal models of LS. The purpose of this project is to develop rapamycin or similar compounds as effective HCM therapy for LS patients.
FDA Funding

FDA regulates 25% of US economy and all rare disease therapies!
the **FDA BUDGET** in context

Each year, Americans spend …

- **$17 Billion**
  on video games

- **$9.1 Billion**
  on potato chips

- **$3.36 Billion**
  on baggage fees

- **$2.4 Billion**
  on FDA appropriations
Relevant House Appropriations Subcommittees

LABOR, HEALTH AND HUMAN SERVICES, EDUCATION, AND RELATED AGENCIES
Labor, Health and Human Services, Education, and Related Agencies

2358-B Rayburn House Office Building
(202) 225-3508
Chairman: Rep. Tom Cole, Oklahoma

Agriculture, Rural Development, Food and Drug Administration, and Related Agencies

2362-A Rayburn House Office Building
(202) 225-2638
Chairman: Rep. Robert Aderholt, Alabama
Chairman Thad Cochran (R–MS)

Ranking Member Barbara Mikulski (D–MD)
Relevant Senate Appropriations Subcommittees

Agriculture, Rural Development, Food and Drug Administration, and Related Agencies

Chairman: Jerry Moran
Ranking Member: Jeff Merkley

Labor, Health and Human Services, Education, and Related Agencies

Chairman: Roy Blunt
Ranking Member: Patty Murray
Time for Advocacy is NOW!!
The ASKS:

- Please support robust and mandatory funding for the National Institutes of Health (NIH) and the Food & Drug Administration (FDA)
- See One Sheets & Sample Scripts
- Questions Welcome!
Thank You
In-District Lobby Days

Stephanie Fischer
Senior Director of Patient Engagement and Communications
EveryLife Foundation for Rare Diseases
RARE DISEASE LEGISLATIVE ADVOCATES
IN-DISTRICT LOBBY DAYS
Goals of In-District Lobby Days

- Advance legislation that would benefit the rare disease community
- Strengthen your relationships with Members of Congress and staff
  - View meetings as opportunities to build or sustain a meaningful relationship with your Representative and Senator.

A best practice of effective advocacy is to make your friends before you need them! We NEED to gain allies who will champion rare disease causes.
Why Relationships Matter

If your Member/Senator has not already arrived at a firm decision on an issue, how much influence might the following advocacy strategies directed to the Washington office have on his/her decision?

- In-Person Issue Visits from Constituents: 46% A Lot of Positive Influence, 51% Some Influence
- Contact from a Constituent Who Represents Other Constituents: 36% A Lot of Positive Influence, 60% Some Influence
- Individualized Postal Letters: 20% A Lot of Positive Influence, 70% Some Influence
- Individualized Email Messages: 19% A Lot of Positive Influence, 69% Some Influence
- Phone Calls: 14% A Lot of Positive Influence, 72% Some Influence
- Comments During a Telephone Town Hall: 17% A Lot of Positive Influence, 68% Some Influence
- Visit From a Lobbyist: 8% A Lot of Positive Influence, 74% Some Influence
- News Editorial Endorsement of an Issue: 10% A Lot of Positive Influence, 63% Some Influence
- Individualized Faxes: 8% A Lot of Positive Influence, 62% Some Influence
- Form Postal Letters: 1% A Lot of Positive Influence, 53% Some Influence
- Form Email Messages: 1% A Lot of Positive Influence, 50% Some Influence
- Postcards: 1% A Lot of Positive Influence, 44% Some Influence
- Comments on Social Media Sites: 1% A Lot of Positive Influence, 41% Some Influence
- Form Faxes: 0% A Lot of Positive Influence, 39% Some Influence
Meetings will take place during the summer Congressional recess from July 18th and September 5th.

The meetings will be in the local offices of your federal legislators.

We retained Advocacy Associates to schedule the meetings and contact advocates with their schedules.

YOU MUST REGISTER BY JULY 5th.
In-District Lobby Days 2016

The 3rd annual In-District Lobby Days is fast approaching. From July 18th to September 5th, rare disease advocates from across the country will surge into the district offices of their Members of Congress to advocate for legislation benefiting the rare disease community.

Interested in meeting with your U.S. Senators and Representative in their district offices during the summer Congressional recess? Rare Disease Legislative Advocates will schedule meetings for you, and help you prepare by providing key background materials. In addition, anyone registered for In-District Lobby Days will receive an invite to a preparatory webinar to be held on July 13th.
Registering

• Enter your personal info on the registration page.
• BE SPECIFIC about the dates that you are unavailable!
  • Advocacy Associates uses this to schedule your appointments.
• You can also note how far you are willing or able to travel for the meetings with your Representatives and Senators.
Once you register, we will use the information provided to:

- Match advocates to their federal legislators
- Determine (based on constituency and location) the appropriate offices to approach about scheduling
- Send meeting request letters (and initiate follow-up calls, if necessary, to schedule meetings)
- Ensure the meeting time works for both advocates and the legislator
Preparing for In-District Lobby Days

- Everyone who registers for In-District Lobby Days will be invited to a webinar on July 13\textsuperscript{th} at 2pm ET/11am PT.
  - The webinar will provide more detailed information including tips on how to make your meetings successful and what to research prior to your meetings.
  - We welcome questions during the webinar! Don’t be shy.
  - If you can’t join us, you’ll be able to access the archived webinar within a day at \url{http://rareadvocates.org/in-district-lobby-days/}. 
Preparing for In-District Lobby Days

- RDLA will provide background on key legislative issues:
  - 21st Century Cures Act
  - Appropriations for FDA and NIH
  - OPEN ACT

- Hard copies are available at the informational table at the back of this room. Please take three copies of each (one set for each meeting as leave-behinds).

- Legislative scorecards for the states represented here today are also available. Please take only one copy (from your own state).
Getting to know your Members of Congress

- Go to your legislators’ websites (which you can find at www.house.gov or www.senate.gov) to check out the bios as well as the issue and news sections.
  - Sign up for their newsletters.
- Google your legislators and read recent media coverage. What are they saying? What do they care about?
- Visit Project Vote Smart (VoteSmart.org) to look up a Member’s interest group ratings, speeches and statements, etc.
- Follow your legislators on Twitter and like their pages on Facebook.
What to bring to your meetings

- Pictures of your family member or other loved ones affected by rare disease, if they are not joining you for the meeting.
- Your business card if you have one, particularly if you are affiliated with an advocacy organization.
- Fact sheets on the key legislative issues you plan to raise.
- If you would like to leave behind information on your disease or organization for your Member of Congress, make sure that it fits on one page. It can be doublesided, but not in 4 point font!
What to **DO** in advance in order to have a successful meeting with a Member of Congress

- **DO** take the time to learn about the Member.
- **DO** learn about the bills you want to discuss and know the bill numbers.
- **DO** coordinate with your team before the meeting.
  - Determine who will make each “ask”.
- **DO** practice your two minute personal story.
- **DO** practice your ask. Why should the Member support a specific bill? Pick the talking points most relevant to you and to the Member.
What to **DO** during the meeting for it to be successful

- **DO** arrive 5–10 minutes early.
- **DO** smile and be respectful of the Member’s staff.
- **DO** thank the Member if he/she has supported rare disease legislation in the past.
- **DO** thank the Member if he/she is a member of the Rare Disease Congressional Caucus.
  - You can find a list of Caucus members on our website at http://rareadvocates.org/rarecaucus/.
- **DO** avoid talking politics if you aren’t the same party as the Member. **Rare diseases are non-partisan, and we need champions in both parties!**
- **DO** take a picture with the Member and/or staff.
What to **DO** after meeting with a Member of Congress

- **DO** send a hand-written thank you note to the district office where you had the meeting to the Member.
  - Also send a note or email to any staff who was engaged in the meeting.
  - **DO** follow-up by email with any additional information requested by the Member and/or staff.
  - Need help answering a question on 21\textsuperscript{st} Century Cures, OPEN ACT, or appropriations for FDA and NIH? Let us know!
What to **DO** after meeting with a Member of Congress

- **DO** continue to grow the relationship.
  - Look for opportunities to engage the Member in person such as town halls and even campaign debates.
  - Call the Member for relevant action alerts on legislation.
  - Thank the Member for cosponsoring or voting for legislation you asked him/her to support.
  - Stay in touch with staff.
DO thank the Member on Social Media.

- Tag the Member in a Tweet with a photo and post a thank you on the Member’s official Facebook page. Tag @RareAdvocates, too!
If Your Availability Changes

If you are not able to attend your schedule meetings, please let us know right away.

- We understand you may make last-minute vacation plans or get sick. Please contact Vignesh Ganapathy at vganapathy@everylifefoundation.org or call our office at (415) 884-0223 as soon as you realize you are no longer able to attend.
- We will let the Member’s office know to cancel the meeting, if you were the only participant, or to expect one less person, if you were attending with a group.
Questions?

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Closing Remarks

Lisa Schill
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Thank You!!!!

@RareAdvocates