RareAdvocates
#RareDC2016
*Silence your cell phones please*
Welcome & Overview

Dean Suhr
Parent Advocate
President & co-Founder, MLD Foundation
Thank You!
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 5th 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
Progress?

- 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
The EveryLife Foundation

We are unique – we are both a

- Public Policy Organization

and a

- Patient Organization
Mission

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
Core Principals

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
Public Policy & Patients

1) Community Programs
2) Promote Awareness
3) Public & Scientific Policy
4) Grassroots Advocacy
Community Programs

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

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.

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 & industry to ensure we receive community input about policy needs.
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

200+ patient advocates learning how policy impacts access to treatments and meeting with Congress

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

A permanent rare disease voice on capitol Hill through regular briefings to educate Congress

Honoring Advocates who give patients a policy voice

5th annual Rare Voice Awards are in DC
Meet the Team!

JULIA JENKINS
EXECUTIVE DIRECTOR
JJENKINS@EVERYLIFEFOUNDATION.ORG

CAROL KENNEDY
CHIEF DEVELOPMENT OFFICER
CKENNEDY@EVERYLIFEFOUNDATION.ORG

MAX BRONSTEIN
 SENIOR DIRECTOR, ADVOCACY & SCIENCE POLICY
MBRONSTEIN@EVERYLIFEFOUNDATION.ORG

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

GRANT KERBER
DEPUTY DIRECTOR OF COMMUNICATIONS & PATIENT PROGRAMS
GKERBER@EVERYLIFEFOUNDATION.ORG

VIGNESH GANAPATHY
ASSOCIATE DIRECTOR OF ADVOCACY AND GOVERNMENT RELATIONS
VGANAPATHY@EVERYLIFEFOUNDATION.ORG

SARAH MILLUS
OFFICE AND DEVELOPMENT MANAGER
SMILLUS@EVERYLIFEFOUNDATION.ORG

LISA SCHILL
RDLA SPECIAL EVENTS COORDINATOR
LSCHILL@EVERYLIFEFOUNDATION.ORG
We are proud that this year Rare Disease Week is patient led.

THANK YOU!

Stephanie Fischer
Patient Advocate & Stroke Survivor

Lisa Schill
RASopathies Parent Advocate
RARE DISEASE WEEK
ON CAPITOL HILL
130 Organizations, 330 Registered Advocates

Our 5th Year on the Hill together!
Our 5th Year!

- Over 330 patient advocates registered
- 130 patient organizations represented
- 17 biopharmaceutical companies represented
- Leaders from the Food and Drug Administration (FDA) & National Institutes of Health (NIH)
Nametag Stickers for Lobby Day Participants

- State Sticker (American Flag for District of Columbia)
- Sticker for your lobby day team
Your Conference Booklet

- Overview of the Week’s Events
- Conference Agenda
- Speaker Bio’s
- Maps – Metro, Capitol Hill & Event Locations (*end of book*)
- Overview of each Bill/Legislative Ask (*pgs 31–36*)
  - Talking Points & Advocacy Tips for your Hill Meetings
Participate to win a $50 Amazon Gift Card!
This Week’s Remaining Events

Wednesday, March 2\textsuperscript{nd}

- 7:30 am – Breakfast at the Capitol Hill Club \textit{(30 min by Metro)}
- 9:00 am – Meetings on Capitol Hill

Thursday, March 3\textsuperscript{rd}

- 12:00 pm – Rare Disease Congressional Caucus Briefing
- 5:00 pm – Rare Artist Reception
The closest Metro stop is Capitol South on the blue, orange and gray lines.
It’s a 15 minute walk plus security between Senators and Representatives.
Wednesday: **Lobby Day Breakfast**

Capitol Hill Club – 300 First Street SE

- **7:30 Breakfast** – please be prompt!
- **Speakers**
  - Dr. Emil Kakkis
  - Fred Upton, House Energy and Commerce Chairman (R-MI)
  - Dr. Robert Califf, FDA Commissioner
  - Representative Gus Bilirakis (R-FL)

The closest Metro stop is **Capitol South** on the blue, orange and gray lines (toward New Carrollton or Largo)
<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
</tr>
</thead>
<tbody>
<tr>
<td>9:15 am</td>
<td>Election Madness: Campaign Promises and Legislative Realities</td>
</tr>
<tr>
<td>10:00 am</td>
<td>Break</td>
</tr>
<tr>
<td>10:15 am</td>
<td>The Promise of Agency Partnership</td>
</tr>
<tr>
<td>11:15 am</td>
<td>Rare Disease Legislation: A Historical Primer</td>
</tr>
<tr>
<td>11:45 am</td>
<td>Networking Lunch Break</td>
</tr>
<tr>
<td>12:30 pm</td>
<td>What Comes First? Priorities for Future Legislative Efforts</td>
</tr>
<tr>
<td>1:30 pm</td>
<td>Putting Your Best Foot Forward: Tips &amp; Tricks for a Successful Meeting</td>
</tr>
<tr>
<td>2:10 pm</td>
<td>Scenes from the Hill: A Mock Legislative Meeting</td>
</tr>
<tr>
<td>2:30 pm</td>
<td>Next Steps: Your Path to Success</td>
</tr>
<tr>
<td>2:55 pm</td>
<td>Break: Pick-up packets with Lobby Day schedule</td>
</tr>
</tbody>
</table>
3:15pm  Breakout 1: Multiple Paths to Success: Working with RD Partners
- How to Work with Industry – Vista Room
- Advocacy on Social Media – Angle Room
- Understanding FDA Review Pathways – Main Room

4:05pm  Breakout 2: Rare Disease Issues, State by State
- Modernizing Step Therapy – Angle Room
- Rare Disease State Advisory Council – Vista Room
- Newborn Screening – Main Room

4:55pm  State Meet and Greet

5:20pm  Closing Remarks
State Meet and Greet

- Meet your fellow advocates
- Review your meeting schedule
- Strategize your meetings
Overwhelmed, Scared, Confused ... ?
or
Informed, Confident, Empowered ... ?

Dean Suhr
Parent Advocate
Lindy & Darcee’s Dad
President & co-Founder, MLD Foundation
Teryn

- Her story
- Her community
- The Rare Disease community
You

- Your story
- Your community
- The Rare Disease community

Thank you – have a good week!
Thank You!!!!

@RareAdvocates
#RareDC2016