Rare Disease Legislative Advocates
March Monthly Meeting

March 21, 2019
Welcome!

- If you are joining us via WebEx, please also call into the conference line at:
  - Call-in number: 1-650-479-3208
  - Access code: 660 485 944

- For those joining us on the phone, the conference line will remain muted due to the large volume of callers. If you would like to ask a question, please send via the chat function and a staff person will direct them to the speakers.

- Reminder for media: We ask that media formally announce their participation and refrain from quoting any of the discussion during the meeting. We encourage media to follow up with participants after the meeting for direct quotes.
Agenda

- FDA Update: Public Meeting on Rare Diseases, Dr. Janet Maynard, Acting Director, Office of Orphan Products Development, FDA
- Rare Disease Burden Study Update, Deanna Portero, Executive Director, Fibrous Dysplasia Foundation
- President's Budget for FY 2020, Steve Silvestri, Policy Director, EveryLife Foundation for Rare Diseases
- Lymphedema Treatment Act Update, Heather Ferguson, Executive Director, Lymphedema Advocacy Group
- Medical Nutrition Equity Act, Camille Bonta, Summit Health Care Consulting
- Rare Disease Legislative Advocates Update, Shannon von Felden, Program Manager, RDLA, EveryLife Foundation
FDA Update: Public Meeting on Rare Diseases

Dr. Janet Maynard, Acting Director, Office of Orphan Products Development, FDA
FDA Update: Public Meeting on Rare Diseases

Rare Disease Legislative Advocates (RDLA)
Monthly Legislative Webinar
March 21, 2019

Janet Maynard, MD, MHS
Director, Office of Orphan Products Development, FDA
Rare Disease Product Development

• Developing a treatment for a rare disease can present unique challenges

• Involvement of patients and patient input is critical in addressing the challenges of developing a treatment for rare diseases

• Goal of this meeting is to identify common issues across rare diseases to help address some of these challenges
Patient Perspectives on the Impact of Rare Diseases: Bridging the Commonalities

- Public meeting and opportunity for public comment

- Intended to obtain patients’ and caregivers’ perspectives on impacts of rare diseases on daily life and to assess commonalities

- **Meeting webpage:**
  - https://www.fda.gov/NewsEvents/MeetingsConferencesWorkshops/ucm628352.htm
Meeting Details

• Date
  – April 29, 2019

• Time
  – 1pm-5pm

• Location
  – FDA White Oak Campus (Silver Spring, Maryland)
  – If you are unable to attend in person, you can view a live webcast of the meeting
Meeting Overview

• Two sessions
  – Each will include a panel discussion and a facilitated group discussion
• Open public comment
• Opening and closing remarks from FDA

• Contact
  – Eleanor Dixon-Terry (301-796-7634 or OOPDOrphanEvents@fda.hhs.gov)
Rare Disease Burden Study Update

- Deanna Portero, Executive Director, Fibrous Dysplasia Foundation
Deanna Portero

Executive Director
Fibrous Dysplasia Foundation

Public Policy Working Group Co-Chair
EveryLife Foundation Community Congress

dportero@fibrousdysplasia.org
Rare Diseases Are Expensive.

- Undiagnosed and untreated rare diseases probably cost the United States hundreds of billions of dollars a year or more.
  - The diagnostic odyssey
  - Inpatient and outpatient costs of uncontrolled disease progression
  - Costs of palliative care for symptoms
  - Lost economic productivity by patients and caregivers
  - Other private costs like power wheelchairs, vans, home adaptation
  - Other social costs like in home nursing, group homes, special education
Let’s Bring Data to a Data Fight

• The solutions we need (biomedical research and more rare disease treatments) are big ticket items, and those costs can result in sticker shock.

• The humanitarian case for rare disease research and therapeutic development is undeniable. We’re all prepared to speak to that. But that argument isn’t a silver bullet for making change.

• We’re not prepared to talk about the costs of undiagnosed, untreated rare diseases in an empirically-supported way. We’re not prepared to demonstrate what we already know (but that lawmakers may not fully appreciate) that inaction/the status quo is also tremendously expensive.

• If we were, we could make a better case for the need for rare disease research, diagnostics, and treatments. We might even be able to make a purely economic case for cures.
“If you think research is expensive, try disease!”

-Mary Lasker (1900-1994)
Community Congress Timeline

July 2018
- Idea introduced in annual survey. Survey finds 90% of Public Policy Working Group members support a “burden study”

October 2018
- Follow-up survey confirms interest
- Consensus is also apparent at an in-person meeting

Today
- Preliminary, privately-funded, pilot study in progress
- EveryLife Foundation researching and coordinating community action steps for stakeholders to encourage Congress to fund a NASEM study
Lewin Group Study

- Study to be completed in 2019
- Analytical approach will estimate direct medical cost and lifetime medical costs of 5 ICD 10 codes:
  - Lysosomal storage diseases E75, E76, E77
  - Congenital malformation syndromes predominantly associated with short stature Q87.1
  - Genetic susceptibility to other disease Z15.89
  - Mitochondrial metabolism disorder unspecified E88.40
  - Metabolic disorder E88.9
- Goal is to measure economic impact of major rare disease on US economy, including direct medical costs, non-medical costs, & indirect costs
- How you can help: Help EveryLife understand the non-medical and indirect costs that most frequently impact your disease community.
NASEM Study

• National Academies of Science, Engineering & Medicine has resources to perform a comprehensive burden study
• Timeline for this would be several years
• Would let nation at large understand true economic and societal burden rare diseases, and resource needs
• Study would build on foundation laid by Lewin Report
• $1.5 million appropriation in the Fiscal Year 2020 Labor, Health and Human Services, Education Appropriations bill and direction in report language for the National Academies of Sciences, Engineering and Medicine to conduct a study on the burden of rare disease.
Appropriations Timeline

Legislative Process:

• March: President released Budget Proposal
• March/April: Appropriations Committee hearings
  • Deadline to submit appropriations requests
• May: Appropriations bills released and marked up by Committee
• June: Bills considered, amended, voted on by full House of Representatives and Senate
• September: Final appropriations bills signed into law by the President

What this means for you:

• Now: Spread the word with policy makers and your community! (Esp. L-HHS SubCom)
• Now: Tell your story and why the burden study matters
• Now: Submit request to Members of Congress
• Soon: Request Members of Congress sign on to Dear Colleague Letter led by Rare Disease Caucus
• Soon: Sign on to stakeholder letter to Congress
President's Budget for FY 2020

Steve Silvestri, Policy Director, EveryLife Foundation for Rare Diseases
President’s FY20 Budget Request

https://www.whitehouse.gov/omb/budget/

No Disease Is Too Rare to Deserve Treatment
“No money shall be drawn from the Treasury but in consequence of appropriations made by law”
- U.S. Constitution Article 1, Section 9, Clause 7
National Institutes of Health

- FY2019 Enacted Level: $38 billion
- FY2020 Requested Level: $33.4 billion
- Difference: reduction of $4.6 billion
- NIH is the world’s leading funder of basic biomedical research, supporting breakthroughs in how we understand and treat deadly and debilitating diseases.
Food and Drug Administration

- FY2019 Enacted: $2.9 billion (Discretionary Budget Authority)
- FY2020 Requested: $3.3 billion (Discretionary Budget Authority)
- Difference: Increase of $362 million
  *Total request including user fees is $6.1 billion

- The FDA plays a critical role in putting cures into the hands of patients through the review and approval of innovative treatments and devices. Increased funding supports the infrastructure and staff that move products through the review pipeline.
Health Resources and Services Administration

- FY2019 Enacted: $10.7 billion
- FY2020 Requested: $9.7 million
- Difference: reduction of $981 million
- HRSA is the primary agency that carries out newborn screening programs along with CDC.
- This budget recommends zero funding for HRSA’s Heritable Disorders program and recommends unspecified reductions for the Newborn Screening Quality Assurance Program at the Centers for Disease Control and Prevention (CDC).
Congressional Appropriations

- The Labor, Health and Human Services, Education Appropriations Subcommittee has jurisdiction over most health funding.

- The Agriculture Subcommittee has jurisdiction over FDA funding.

- With release of the President’s budget request, these subcommittees will now be writing their FY2020 appropriations bills.
Lymphedema Treatment Act Update

Heather Ferguson, Executive Director, Lymphedema Advocacy Group
Heather Ferguson
Founder & Executive Director
Lymphedema Advocacy Group
# Lymphedema Treatment Act - 116th Congress

### Senate Bill – S.518

**Lead sponsor:**
Maria Cantwell (D-WA)

**Co-lead:**
Todd Young (R-IN)

**Introduced**
February 14, 2019
w/ 32 Original Cosponsors

**Current Cosponsors 45**

### House Bill - H.R. XXXX

**Lead sponsor:**
Jan Schakowsky (D-IL)

**Co-leads:**
Buddy Carter (R-GA)
Earl Blumenauer (D-OR)
Mike Kelly (R-PA)

**Introduction will occur the week of March 25th,**
currently gathering Originals
Please contact your members of Congress using the advocacy tools at LymphedemaTreatmentAct.org

How you can help

#1 PRIORITY – Email Congress
#2 – Call Your Senators
#3 – Call Your Representative
#4 – Congress via Social Media
#5 – Attend a District Meeting
Advocacy Training Webinars
Advocacy Handbook & Tools
Join Your State’s Team
Share Your Lymphedema Story
Tools to Increase Awareness
About our Lobby Days in DC
Act Now – Donate Today
2019 Lymphedema Lobby Days
May 5th-7th

Everyone is welcome, including children.

Registration deadline is April 14th.

Discounted hotel rooms until April 4th or sold out.
Please visit our website or contact me for more information:

LymphedemaTreatmentAct.org
Heather@LymphedemaTreatmentAct.org
Medical Nutrition Equity Act

Camille Bonta, Summit Health Care Consulting
Capitol Hill Day
May 6-7, 2019
**Why:** To advocate for the Medical Nutrition Equity Act

**What:** Meet with congressional offices — Meetings scheduled for you!

**Where:** Washington, D.C. — Capitol Skyline Hotel

**When:** May 6-7, 2019 — Event starts 3pm on May 6
How:
Register online at https://nutritionequity.org
Deadline to register is April 5!

Spread the Word:
Facebook: https://www.facebook.com/nutritionequity
Twitter: @nutritionequity

Share Your Story:
https://nutritionequity.org/share-your-story/
Rare Disease Legislative Advocates Update

- Shannon von Felden, Program Manager, RDLA, EveryLife Foundation
Rare Disease Caucus

House Co-Chairs

Representative G.K. Butterfield (NC)

Representative Gus Bilirakis (FL)

Senate Co-Chairs

Senator Roger Wicker (MS)

Senator Amy Klobuchar (MN)
Rare Disease Caucus

12 new Members joined since Rare Disease Week on Capitol Hill!

**New in the House:** Representatives Jackie Speier (CA-14), Sanford Bishop (GA-2), Steve Cohen (TN-9), Nydia Velazquez (NY-7), Greg Walden (OR-2), Abby Finkenauer (IA-01), Peter Visclosky (IN-1), Andy Kim (NJ-03), C.A. Dutch Ruppersberger (MD-2)

**New in the Senate:** Senators Cindy Hyde-Smith (MS), Maria Cantwell (WA), John Kennedy (LA)

**House Caucus Members:** 112 Members

**Senate Caucus Members:** 17 Senators

Members are listed at rareadvocates.org/rarecaucus
Over 800 people participated in at least one event during Rare Disease Week on Capitol Hill

Over 425 advocates participated in 298 meetings on the Hill

Thank you to everyone who attended and advocated on the Hill!!!!
Thank you to Rare Disease Week volunteers!
Our next RDLA meeting will take place on Thursday, April 11th, 2019 at noon ET.

If you would like to speak or for a particular topic presented at the next RDLA meeting, please email Shannon at svonfelden@everylifefoundation.org.
Thank You!