Community Congress
Annual Meeting

November 16, 2017
Thank you Member Sponsors
Today’s Agenda

- **Foundation Overview**: Julia Jenkins, Executive Director, EveryLife Foundation
- **Special Message**: Emil Kakkis, Founder, EveryLife Foundation
- **Address from New Board Chair**: Mark Dant, Executive Director, Ryan Foundation
- **Rare Disease Center of Excellence**: James Valentine, Hyman, Phelps & McNamara P.C
- **Capitol Hill Update**: Rachel Klein, Senior Director of Advocacy & Strategy, EveryLife Foundation
- **Working Group Co-Chair Updates**:
  - **Public Policy**: Cristina Might, Executive Director, NGLY1.org
  - **Regulatory**: Isabelle Lousada, President & CEO, Amyloidosis Research Consortium & Lynne McGrath, Vice-President Regulatory Affairs, RegenxBio
  - **Newborn Screening**: Elisa Seeger, Founder, Aidan Jack Seeger Foundation & Jennifer Helfer, Patient Advocacy, bluebird bio
- **Networking Lunch**
- **12:30 – 2:30 Working Group Breakout Sessions**
  
  *For Community Congress Members Only*
Mission, Milestones & Goals: The Future is Collaboration

Julia Jenkins
Executive Director
Mission and Core Principles

Accelerating biotech innovation through science-driven public policy

What We Believe:
- No disease is too rare not to deserve treatment
- Rare disease therapies should be safe and effective
- We could do more with the science we already have

What We Do:
- Advocate for evidence-based public policy and regulatory reform

How We Get it Done:
- Grassroots action
- Scientific and policy expertise
Our Team

- Julia Jenkins, Executive Director*
- Rachel Klein, Senior Director, Advocacy & Strategy
- Hannah Clauson, Regulations and Policy Fellow
- Stephanie Fischer, Chief Patient Engagement and Communications Officer*
- Grant Kerber, Deputy Director of Communications & Patient Programs
- Lindsey Cundiff, Associate Director of Patient Engagement*
- Sabah Bhatnagar, RDLA Program Director*
- Lisa Schill Event Development Consultant (part-time)*
- Carol Kennedy, Chief Development Officer
- Ted Brasfield, Director of Development
- John Lally, Operations Director
- Erin Garcia, Office Manager and Administrative Support*
- Deborah Walter, Manager of Finance & Human Resources (part-time)

*6 staff members are rare disease patients or have family members affected

No Disease Is Too Rare to Deserve Treatment
Our Board of Directors

• Chair, Mark Dant, Executive Director, Ryan Foundation
• Co-Chair, Frank Sasinowski, Director, Hyman, Phelps & McNamara, P.C.
• Secretary Julia Jenkins, Executive Director, EveryLife Foundation
• Treasurer, Vicky Seyfert-Margolis, PhD, Founder and CEO, MyOwnMed
• Founder, Emil D. Kakkis, MD, PhD, President/CEO, Ultragenyx
• Ritu Baral, Managing Director/Senior Biotechnology Analyst, Cowen & Company
• Mike Astrue, Former Commissioner, Social Security Administration
• *3 former FDA - 4 with family affected by rare disease
We Succeed by Giving Rare Disease Patients a Voice

- We do not speak on behalf of patients
- Our programs seek to
  - Educate patients about the challenges of drug development and the legislative and regulatory process
  - Train advocates on how to tell their stories to affect policy change
  - Create opportunities to allow patients to be heard by policy makers and to build relationships with elected officials
  - Provide financial recourses to ensure patients can travel to policy events
- Patients will be the key to fighting for any innovation policies in the next Congress - We must double down to support of our patient communities
Community Support & Outreach

**RareGiving** gives $120,000 to the community in grants and scholarships to ensure FDA & Congress hears from patients.

**Rare Artist** promotes awareness of rare diseases & highlights the talents of our community.

**Rare Affair** promotes investment in rare disease treatments during the JP Morgan Health Care Conference in San Francisco, CA.

**Italian Street Painting Marin** is a local festival in that provides us an opportunity to educate the general public in our local community about the Foundation and Rare Diseases.
Advocacy - Rare Disease Legislative Advocates

Brings 350 patients to Washington DC to learn about how legislation impacts access to treatments & to meet with Congress

Allows advocates who cannot come to DC to meet with their Members during August Recess, Regional Conferences train advocates

Ensures the Rare Disease Community has a permanent voice on Capitol Hill through regular briefings to educate Congress

Honors Advocates who give patients a voice in state & federal government

www.RareAdvocates.org
Public Policy Objectives

The Foundation seeks practical policy solutions that will:

- Close the innovation gap for the 95% of rare diseases that have no FDA-approved treatment
- Ensure patients receive earliest access to diagnostic and treatment opportunities
- Improve the regulatory process and advance regulatory science for rare disease therapies
- Enhance the patient voice in policymaking, drug development and regulatory decision-making
Science & Public Policy

Brings together FDA, NIH, industry and patients to address urgent regulatory challenges through case examples and expert led discussion.

Bipartisan legislation granting 6 months of market exclusivity for repurposing a drug for a rare disease.

Seeks to improve newborn screening policies at the State and Federal levels to ensure earliest access to treatment.

Establishing an FDA Center of Excellence for Rare Diseases to improve the expertise of FDA reviewers and improve access to accelerated approval pathway.
Collaboration is our Future

Community Congress

• Program created to foster COLLABORATION between industry and patient organizations to seek policy solutions

• Working groups create a formal way to discuss policy issues facing our community and create opportunities to work together on issues important to all stakeholders

• Free for patient orgs to join, open to all patient orgs
  • Not just rare disease focused

• Industry Membership fees help support the important work

When we work together, we win
Our First Success in Collaboration

- Nov 15, 2016, we partnered with NORD & Global Genes on a first ever **United Day of Action** to pass the 21\textsuperscript{st} Century Cures Act which had been stalled in the Senate for more than a year

- December 7\textsuperscript{th} the Senate passed 21\textsuperscript{st} Century Cures 94 to 5
Strategic Planning with Global Genes

June 2017 – 2 Day Collaboration

We asked ourselves how we can work better together to meet the needs of the rare disease community.
This is a first of its kind collaboration harnessing the core competencies of Global Genes and EveryLife with the goal to bring increasing value, insights and knowledge to patients and advocates challenged by rare disease.

2018 Dates & Locations
Saturday, June 9\textsuperscript{th}  Houston, TX
Saturday, June 30\textsuperscript{th}  Salt Lake City, UT
Saturday, July 21\textsuperscript{st}  Nashville, TN
Collaboration – Promoting Collaboration Across Disease

- Helped Launch CAL Rare and the California Rare Disease Caucus to organize the community at the state level

- Through Rare Giving we provide financial sponsorship & promotion of the State House Events for Rare Disease Day
RDLA is expanding it’s influence

Director of RDLA was hired to manage the program

Rare Disease Congressional Caucus

- 127 Members
- 67 Democrats
- 40 States
- 1 Senate HELP
- (+11) Members from In District Lobby Days in August

RDLA Monthly Meetings/Webinars

- 150-180 people participate monthly

Rare Disease Week on Capitol Hill

- 600 people attending one of the 6 events
- 325 patient advocates from 48 different states participated
- 152 patient organizations were represented
- New Venue in 2018 to hold 400 Advocates
OPEN ACT Advocacy Grows

• **286** Advocacy Organization have signed on to support the OPEN ACT

• Launched a campaign earlier this month to get **26,000** emails to Congress in 4 weeks
  • 13,500 have already been sent & we are on track to meet our goals
Foundation Founder
Emil Kakkis,
President & CEO of Ultragenyx
Special Presentation

No Disease Is Too Rare to Deserve Treatment
New Foundation Board Chair
Mark Dant,
Executive Director, Ryan Foundation

No Disease Is Too Rare to Deserve Treatment
Future Directions for Rare Disease Advocacy
Building an FDA Center of Excellence (COE) for Rare Diseases

James E. Valentine, J.D., M.H.S.
2017 Annual Meeting
Statutory requirement to establish COEs

21st Century Cures Act, Section 3037: Establishment of FDA Intercenter Institutes

- …shall establish one or more Intercenter Institutes within FDA for a major disease area or areas
- …shall establish at least one Institute within 1 year
- Friends of Cancer Research proposed COEs at FDA with pilot in oncology
- 6/29/2016: VP Biden announced FDA Oncology COE as part of Cancer Moonshot
- 12/13/16: 21st Century Cures Act enacted
- 7/27/17: FDA issued notice in *Federal Register* establishing new organizational structure for Oncology COE
First COE Established
What is a COE?

- Organizational unit within the Office of Medical Products and Tobacco
- Leverages the combined skills of regulatory scientists and reviewers with expertise in major disease areas in drugs, biologics, and devices (including diagnostics)
- Helps expedite the development of a medical products and support an integrated approach in the clinical evaluation of drugs, biologics, and devices for the treatment of major disease areas
- Works with CDER, CBER, and CDRH, as well as other offices across FDA (e.g., OPT)
How a COE works?

- In accordance with an Inter-Center Agreement, is responsible for sign off on clinical portion of medical applications within its major disease area
- Other functions:
  - Harmonization of disease area-specific regulatory approaches
  - Coordination of disease area-specific regulatory science initiatives and outreach
  - Implementation of cross-Center disease area-focused meetings
  - Stakeholder engagement to the external community & international regulatory agencies
Why a Rare Disease COE?

- Exciting new therapies are changing the way we prevent, diagnose, and treat rare diseases
- Traditional regulatory processes have become more complicated with the reliance on combinations of therapies, genomics, diagnostic tests, and precision medicine
- Numerous parts of the regulatory system need to work together
- Myriad of challenges in rare disease medical product development remain
- Navigating these issues requires its own set of expertise (inconsistently distributed within review divisions)
THANKS!

Any questions?
You can find me at
jvalentine@hpm.com
Rachel Klein, Senior Director, Advocacy and Strategy, EveryLife Foundation for Rare Diseases

Capitol Hill Update

No Disease Is Too Rare to Deserve Treatment
Capitol Hill Update 2017

• Healthcare was a key issue on Capitol Hill in 2017, but little has been enacted in Congress.
• CHIP Reauthorization is still pending in both Houses, must be passed before the end of the year.
• Tax Reform is another big, complex bill with any number of challenges to enactment.
  • Includes ODTC & Medical expense deduction
• Omnibus budget bill must pass before the end of the year – could include health initiatives.
Looking Ahead to 2018

- Healthcare is likely to be high on the 2018 agenda.
  - Likely more ACA changes ahead.
- ODTC debate is indicative of interest in healthcare cost containment.
  - Drug prices are likely to be among the first issues.
- Speculation that more Orphan Drug Act changes may be sought in 2018.
- State healthcare cuts likely to impact rare disease community.
Administrative Activity

- Opportunities to make change administratively
  - FDA Administrator Scott Gottlieb intent on incorporating more patient engagement.
  - FDA Center of Excellence
  - Community Congress presents an opportunity for patient groups to collectively approach FDA for improvements.
2018 Annual Community Congress Schedule

Calls & Meetings

• January – Call with working group co-chairs
• February – Working group call update
• April – Call with working group co-chairs
• May – All Community Congress Member Update Webinar
• July – Call with working group co-chairs
• August – Working group call update
• October – Call with working group co-chairs
• November – Annual in person meeting in Washington DC

*Working group chairs may schedule additional calls to discuss urgent action items

*Actual dates are listed in your materials
Public Policy Working Group
2017 Co-Chairs

Cristina Might, Executive Director
NGLY1.org
Cristina@ngly1.org

Looking to elect new Chairs for 2018

No Disease Is Too Rare to Deserve Treatment
New Chairs Needed for 2018

Patient

Cristina Might
co-chair since 2015

Industry

2018
These could be YOU!
What we do

• Close the innovation gap for the 95% of rare diseases that have no FDA-approved treatment
  • Support robust funding for the National Institutes of Health (NIH)
  • Seek policies that incentivize the creation of novel or repurposed therapies for rare diseases
  • Protect and expand incentives that foster innovation and investment in R&D
  • Support initiatives and new technologies that foster novel and innovative treatments such as personalized and precision medicine

• Ensure patients receive earliest access to diagnostic and treatment opportunities
  • Support effective policies that encourage expanded access programs allowing patients earliest access to lifesaving medicines
  • Support policies that facilitate patient access and choice to the best available FDA-approved therapies
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*No Disease Is Too Rare to Deserve Treatment*
Past Projects

- **September 13th 2016 – Evaluating Models to Expand Access for Rare Disease Patients Workshop in Washington, DC**
  - The workshop featured presentations from and participation by experts in Industry, FDA, and academia and focused on highlighting case studies for companies seeking to provide early access to experimental therapies for patients. ([Click here to access the agenda.](#))
  - Event reached maximum capacity with 120 participants.

- **February 24th 2017 – 146 Advocacy Organizations Join Foundation to Urge President to End to Hiring Freeze at FDA and NIH**
Working Group Survey Results

- We had 21 of the 33 patient organizations & 8 of the 13 industry members take the survey.
- Overall there was strong support in advocating for the Orphan Drug Tax Credit and educating patients about Medicaid and advocating for its protection.
- While there was strong support from the patient community about advocating to expand Medicaid for genetic counseling and sequencing, Industry respondents did not have strong opinions on the issue, most needing more information.
- While there was no opposition to SCHIP, there lacked a 50% consensus to support the issue.
Next Steps from Survey

• When Congress brings up tax reform, the working group will consider a patient organization sign on letter in support of protecting this incentive and will assess the need to for additional advocacy efforts if real threats emerge.

• The working group will begin planning for an education webinar as well as a resource webpage to help the rare disease community better understand Medicaid as a policy issue.
  • Topics to highlighting issues like SCHIP, coverage of genetic testing and genetic counseling in those educational resources as appropriate.

• If new threats to Medicaid emerge, the working group will draft and circulate a patient organization sign on letter urging for its protection and will assess the need for additional educational and advocacy efforts.

• The working group may convene an additional call in the fall if an urgency policy issue emerges that the working group should consider addressing.
Today’s Agenda

1. Introductions
2. Discuss New Working Group Co-Chairs
3. Orphan Drug Tax Credit
4. Education Resources for Medicaid/Medicare
   A. Webinar
   B. Webpage
5. Other projects to consider
6. Next Steps

Foundation Staff Support:
Rachel Klein, Senior Director, Advocacy & Strategy
Ted Brasfield, Director of Development
Regulatory Working Group

2017 Co-Chairs

Isabelle Lousada, President & CEO
Amyloidosis Research Consortium
ILousada@arci.org

Lynne McGrath, Vice-President
Regulatory Affairs, RegenxBio
LMcgrath@regenxbio.com
Purpose of working group

- To partner with advocacy groups to facilitate understanding of regulatory issues
- Key topics
  - Understanding regulatory pathways for rare diseases
  - Expanded access regulations for unapproved drugs
  - Role of patients in drug development
  - Role of advocates at FDA meetings and advisory committees
  - Facilitate input into relevant guidance
- The regulatory environment is rich with opportunities for patients to provide their voice for rare disease development
  - 21 Century Cure
  - Prescription Drug User Fee Act 2017 Reauthorization
Jurisdiction: Improve the regulatory process and advance regulatory science for rare diseases

• Support robust funding for the Food and Drug Administration (FDA) drug review

• Encourage a more specialized FDA drug review, which includes:
  • Allowing reviewers to become experts in diseases;
  • Having the ability to keep up on the latest science;
  • Improving the specialization of the drug review divisions;
  • Allowing the FDA to recruit and retain the best staff

• Allow for rare disease therapies to have access to the accelerated approval pathway through the use of biomarkers and surrogate endpoints

• Ensure the FDA maintains rational and flexible regulatory approaches and performance parity with international counterparts

• Encourage policies that allow for alternative clinical study designs and analysis

• Encourage patient-focused drug development for regulators and drug developers

*No Disease Is Too Rare to Deserve Treatment*
Working Group Members

Bristol-Myers Squibb Company
Hyman, Phelps & McNamara, P.C.
Mallinckrodt Pharmaceuticals
OneVoice for the Rare Disease Network

**REGENXBIO, Inc**
Sobi, Inc
Stealth BioTherapeutics
SteveSmithPlans, LLC
Teva Pharmaceuticals
Alagille Syndrome Alliance
American Porphyria Foundation
Amyloidosis Foundation

**Amyloidosis Research Consortium**
Dyskeratosis Congenita Outreach, Inc
EDSers United
Fibrous Dysplasia Foundation

FOP Association
International Alliance of Dermatology Patient Organizations
Jett Foundation
Joshua Frase Foundation
Lupus Foundation of America
Lymphangiomatosis and Gorham's Disease Alliance
National Leiomyosarcoma Foundation
Parent Project Muscular Dystrophy
Pityriasis Rubra Pilaris Alliance, Inc.
PRP Alliance, Inc
RASOPATHIES Network
Sickle Cell Consortium
The Bluefield Project to Cure Frontotemporal Dementia
United Mitochondrial Diseases Foundation
Past Projects:

• October 2015, working group collaborated on comments on the FDA’s "Draft Guidance on Rare Diseases: Common Issues in Drug Development"

• March 2017, Clinical Development for Rare Diseases: A Primer for Rare Disease Patients and Advocates (302 registrants)
  • Topics included: Importance of Natural History Studies and Patient Registries, Overview of Rare Disease Clinical Development Process, Expedited Approval Pathways and Incentives, Early Access and Right to Try

• October 2016, Navigating the FDA Accelerated Approval Process: Rare Disease Case Studies (212 registrants)
  • Topics included: Accelerated Approval Pathways for Rare Disease Therapies, Industry and Patient Organization Case Studies for the Accelerated Approval Pathway

Survey Results

• We had 7 of the 9 industry/consultant working group members vote & 15 of the 20 patient organizations, 76% of our members

• All stakeholders - Patient Organizations, Industry and Consultants voted the same on the issues.

• By a landslide – with 86% of respondents giving it a 1 or 2, the webinar that most people thought was most important was: “Best practices for collaboration between patient organizations and biopharmaceutical companies through the entire life cycle of drug development including, clinical trial design, legislative advocacy, Ad Coms, sponsorship, patient support, etc”

• 73% felt strongly that working group should consider advocacy and educational efforts to make sure insurers cover new therapies that are approved via expedited review pathways such as Accelerated Approval via surrogate endpoint to ensure patients have access to innovated new therapies.

• 68% of respondents strongly agreed that the working group should create a survey to assess patient advocates level under of understanding and preparation to engage with the FDA and industry on each stage of the drug development and approval life cycle.
Next Steps from Survey

• **Webinar Series:** Best practices for collaboration between patient organizations and biopharmaceutical companies throughout the entire life cycle of drug development. *(E.g. clinical trial design, legislative advocacy, Ad Coms, sponsorship, patient support, etc.)*

• **Dec 7th Part 1:** Opportunities and Challenges for Patient and Industry Collaboration: Case Examples & Best Practices

• **Jan 18th Part 2:** Risk/Benefits of Engagement: Formalizing your Policy for Partnership with Patient Organizations & Industry
Today’s Agenda

• Introductions
• Patient Focused Drug Development Projects
  • Upcoming Webinars
  • Resources to be created
  • Survey to assess patient advocates level under of understanding and preparation to engage with the FDA and industry
• Other Issues to Address
  • Advocate for insurers to cover new therapies that are approved via expedited review pathways

Foundation Support Staff
Stephanie Fischer, Chief Patient Engagement and Communications Officer
Lindsey Cundiff, Associate Director of Patient Engagement
Newborn Screening Working Group

2017 Co-Chairs

Elisa Seeger, Founder
Aidan Jack Seeger Foundation
ElisaSeeger721@gmail.com

Jennifer Helfer, Patient Advocacy
bluebird bio
JHelfer@bluebirdbio.com
The *Mission* of the **Aidan Jack Seeger Foundation**:

- Advocate for ALD newborn Screening Nationally
- To provide financial support for families
- To raise awareness for ALD
- Dissemination of information in regard to ALD

Elisa Seeger, Founder
elisa@aidanjackseegerfoundation.org
Working Group Members

Aeglea BioTherapeutics
Alexion
Amicus Therapeutics
Audentes Therapeutics
Avexis
Biogen
Bluebirdbio
GlaxoSmithKline
LapidusData
PTCBio
Retrophin
Sanofi Genzyme
Shire
Acid Maltase Deficiency Association
Adrenal Insufficiency United
Aidan Jack Seeger Foundation
Amour Fund of Alpha Epsilon Omega Foundation
Association for Creatine Deficiencies
BORN A HERO
Cure Sanfilippo Foundation
Friedreich’s Ataxia Research Alliance
Global Genes
HCU Network America
Hope For Dante’
Hunter Syndrome Foundation
Jansen’s Foundation
Jonah’s Just Begun
Joshua Frase Foundation
Little Miss Hannah Foundation
MLD Foundation
MPS Society
National Niemann-Pick Disease Foundation
Pediatric Hydrocephalus Foundation
RareConnect
Sanfilippo Foundation for Children
SCID, Angels for Life Foundation
Sickle Cell Support Services
SSADH Association
STXBP1 Foundation
Taylor’s Tale
The Julius Louis Happy Little Guy Foundation
The Myelin Project
Zeqing for a Cure

No Disease Is Too Rare to Deserve Treatment
1. Close the innovation gap for the 95% of rare diseases that have no FDA-approved treatment
2. Ensure patients receive earliest access to diagnostic and treatment opportunities
   ✔ Enhance newborn screening policies at both the state and federal levels to expand the number of diseases screened at birth
3. Improve the regulatory process and advance regulatory science for rare disease therapies
4. Enhance the patient voice in policymaking, drug development, and regulatory decision-making
Past Webinars

March 16th, 2016
*Newborn Screening in California and Beyond*
• Featured policy experts on the legislative process in California (Registrants: 91, on website)

September 23rd, 2015
*Exploring Proposals to Advance Newborn Screening*
• Featured patient advocacy community and policy experts (Registrants: 60, on website)
November 15, Rare Disease Caucus Briefing
Diagnostic Challenges for Rare Disease Patients

- Moderator: Elisa Seeger, Founder, Aidan Jack Seeger Foundation
- Newborn Screening and the Federal Government
  - Rebecca Abbott, Deputy Director of Federal Affairs for Public Health, March of Dimes
- The Role of Genetic Sequencing in Diagnosis
  - Cristina Might, Founder and President, NGLY1.org
- Whole Genome Sequencing for Children with Rare and Undiagnosed Genetic Disease
  - Ryan Taft, Senior Director, Scientific Research, Illumina
- The Challenge of Access to Genetic Testing
  - Chris and Erin Lee, Founders, Piper's Kidney Beans
- The Future of Diagnostics
  - Cynthia Tifft, Deputy Clinical Director, National Human Genome Research Institute and Director, Pediatric Undiagnosed Diseases Program, National Institutes of Health
- The MSSNG Program
  - Stuart Spielman, Senior Policy Advisor and Counsel, Autism Speaks

Was live streamed & will be available on the web
November 30th Webinar: How to Add Your Disease to the RUSP

- Overview of the EveryLife Foundation Newborn Screening Program, Julia Jenkins, Executive Director, EveryLife Foundation

- Newborn Screening as a Public Health Program, Natasha Bonhomme, Chief Strategy Officer, Genetic Alliance

- Criteria & Considerations for the Federal Recommended Screening Panel, Joseph A. Bocchini, Jr., M.D, Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC) Chairperson, Professor and Chairman, Department of Pediatrics, Louisiana State University

- Successful Addition by a Patient Organization - Case Study on ALD, Elisa Seeger, Founder, The Aidan Jack Seeger Foundation

- Successful Addition by an Industry Member - Case Study on Pompe & MPS I, Joan Kuetzer, Vice President and Head, Global Strategic Services, Rare Diseases at Genzyme, a Sanofi company

- Patient Organization Currently Engaged - Case Study on SMA, Jackie Glascock, PhD, Scientific Program Manager, CureSMA

- Patient Organization Currently Engaged - Case Study on GAMT, Kim Tuminello, Director of Advocacy, Association for Creatine Deficiencies

- Q&A
Legislative Success: CA

California Legislation (**S.B. 1095**) passed unanimously.

- Supported by 120+ patient organizations
- Key provision: must screen for any disease added to the RUSP within two years
- August 2018, CA will begin screening for MPS I and Pompe
Legislative Success: FL

Florida Legislation (S.B. 1124) passed unanimously.

- Supported by 86+ patient organizations
- Key provisions:
  - State Advisory Council must consider within one year of RUSP addition
  - DOH must request appropriation
  - State must implement within 18 months of Advisory Council decision
  - Secured appropriations to implementation of ALD & increased funds for genetic centers
  - Allows for use of non-FDA approved tests
- August 25th, The Florida Advisory Council meeting will consider Pompe and MPS I
Passed Legislation: GA, MO, NE and WA

Georgia: **Krabbe** (May, 2017)

Missouri: **SMA** and **MPS II** (July, 2017)

Nebraska: Introduced Pompe, MPS I and X-ALD legislation (L. 401)

Washington: **Pompe** and **MPS I** (August, 2017) (Done via regulatory decision)

*Missouri is the first state to screen for SMA!*
Pending or Stalled Legislation: NC, MA, OR and TX

North Carolina: Referred to Operations of the Senate Committee Pompe, MPS I, and X-ALD legislation (S. 190)

Massachusetts: Referred to Joint Committee on Public Health Krabbe, Fabry, Gaucher, Pompe, MPS I, and Niemann Pick A/B legislation (S. 1197)

Oregon: Stalled “May screen for conditions on RUSP” legislation (S.B. 808)

Texas: Pending in Committee X-ALD legislation (H.B. 1067)
Pilot Programs: MA and NC

Massachusetts: SMA (Fall 2018)
  • This is in addition to the current MET Pilot Study

  • The program is called EarlyCheck
  • Program collaboration: North Carolina State Laboratory of Public Health, RTI International, University of North Carolina at Chapel Hill, Duke University and Wake Forest Baptist Medical Center
Updates on the RUSP

• Under consideration: SMA and GAMT

• Meeting on November 8-9 delayed decision until upcoming meeting on February 8-9.
Federal Issues

- Trump Administration (FY) 2018 Budget Proposal completely eliminates Health Resources and Services Administration (HRSA)
  - HRSA houses the Heritable Disorders program and the Secretary’s Advisory Committee that establishes the RUSP
  - Also reduces funding for CDC’s Newborn Screening Quality Assurance Program (NSQAP)
- March of Dimes Appropriations Requests (Testimony)
  - $29.8 million for CDC’s NSQAP
  - $19.9 million for the HRSA’s Heritable Disorders program
  - Deadline of FY2019
  - 2014 opposition and negotiations
  - Establishes a parental consent requirement before residual newborn blood spots (NBS) can be used in federally-funded research.
    - Huge loss for medical research -- patients where not mobilized to take action
Online Action Center: Map & Toolkit

• Map
  • Status of NBS policies
  • Conditions on the RUSP that are not screened for
  • State Legislature and/or committee session dates
  • Link to NBS statute
  • Key decision maker contacts

• Toolkit
  • Step-by-step guide for navigating the state legislative process
  • Key components needed to pass legislation (E.g. stakeholder assessment, supporter coalition, draft legislation, committee review)
  • Case studies of states with successful implementation (CA, FL)
  • Sample testimony, support letters, etc
Tool Kit Outline

• Interactive map
• Step-by-step guide
  • Navigating the state legislative process
  • Key components needed to pass legislation (E.g. stakeholder assessment, supporter coalition, draft legislation, committee review)
• Case studies of states with successful implementation (CA, FL)
  • Sample testimony, support letters, etc
• Reference list
• Calendar
• Government and non-government resources
• Research/Publications
Today’s Agenda

1. Introductions
2. Feedback on NBS Advocacy Center
3. States for consideration for Foundation led legislative advocacy
4. Updates on NBS State & Federal Advocacy Efforts
5. Updates from the RUSP
6. 2018 NBS Awareness Month Sept
7. Other Projects/Discussion
8. Next Steps

Foundation Staff Support:
Julia Jenkins, Executive Director
Hannah Clauson, Regulations and Policy Fellow
Lunch & Networking Break
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