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Dear Rare Disease Advocate,

Thank you for joining us for Rare Disease Week on Capitol Hill. This event is a success because of your participation and determination to be an effective advocate for yourself or loved ones. We are honored that you have chosen to advocate with us in DC.

As you know, Washington is often gridlocked with partisan bickering. However, rare diseases are not a partisan issue. Rare diseases do not discriminate; they affect Republicans and Democrats. We are not elephants or donkeys, we are zebras. We are the rare party and we are 30 million strong!

It is truly an honor to help our community by giving patients a voice on Capitol Hill. We hope this week is everything you imagined and that you continue year-round to use your voices to ensure rare disease patients are heard.

Sincerely,

The EveryLife Foundation Team

Julia Jenkins
Executive Director
jjenkins@everylifefoundation.org

Carol Kennedy
Chief Development Officer
ckennedy@everylifefoundation.org

Christina Hartman
Senior Director of Policy and Advocacy
chartman@everylifefoundation.org

Britta Vander Linden
Senior Director of Communications and Marketing
bvanderlinden@everylifefoundation.org

Ted Brasfield
Director of Development
tbrasfield@everylifefoundation.org

Steve Silvestri
Director of Public Policy
ssilvestri@everylifefoundation.org

Lindsey Cundiff
Associate Director of Patient Engagement
lcundiff@everylifefoundation.org

Erin Garcia
Special Events Manager
egarcia@everylifefoundation.org

Shannon von Felden
Rare Disease Legislative Advocates Program Manager
svonfelden@everylifefoundation.org

Lauren Grinnals
Rare Hub Office Manager
lgrinnals@everylifefoundation.org

#RareDC2019
SUNDAY, FEBRUARY 24
5:30 – 9:30 PM

- Rare Disease Documentary Screening and Cocktail Reception
  Ronald Reagan Building and International Trade Center - Amphitheater
  1300 Pennsylvania Ave NW, Washington, DC 20004
  Nearest Metro Stop: Federal Triangle
  Featuring the film “My Turn, the Film”

Presented by:
Takeda

MONDAY, FEBRUARY 25
8:00 AM – 4:45 PM

- Legislative Conference
  Ronald Reagan Building and International Trade Center - Atrium
  1300 Pennsylvania Ave NW, Washington, DC 20004
  Nearest Metro Stop: Federal Triangle

Presented by:
Mallinckrodt Pharmaceuticals

TUESDAY, FEBRUARY 26
7:00 AM – 8:30 AM

- Lobby Day Breakfast
  Washington Court Hotel on Capitol Hill
  525 New Jersey Avenue NW, Washington, DC 20001
  Nearest Metro Stop: Union Station

9:00 AM – 5:00 PM

- Scheduled Meetings with Members of the House and Senate on Capitol Hill
  10:00am – 5:00pm: Hospitality Room
  Cannon House Office Building, Room 121
  Nearest Metro Stops: Union Station (Senate side) or Capitol South (House side)

6:00 - 8:00 PM

- Young Adult Meetup
  Rare Hub, 1012 14th Street NW, Suite 500, Washington, DC 20005
  Nearest Metro Stop: McPherson Square

Scheduled by:
Prime Advocacy

Hosted by:
YARR

#RareDC2019
Rare Disease Week on Capitol Hill
2019 Events

WEDNESDAY, FEBRUARY 27

- Rare Disease Congressional Caucus Briefing
  Location and time TBD

IMMEDIATELY FOLLOWING THE CAUCUS BRIEFING

- Group Photo on the Capitol Steps
  Meetup location: Steps on the northeast corner of the Capitol Building
  *Metro Stop: Union Station or Capitol South*

5:00 – 7:00 PM

- Rare Artist Reception
  Rayburn House Office Building, Foyer, 1st Floor
  45 Independence Ave. SW, Washington, DC 20515
  *Nearest Metro Stop: Capitol South*

THURSDAY, FEBRUARY 28

8:30 AM - 4:00 PM

- Rare Disease Day at the National Institutes of Health
  National Institutes of Health, Main Auditorium, Natcher Conference Center, Building 45
  9000 Rockville Pike, Bethesda, MD 20892
  *Nearest Metro Stop: Medical Center Station*
SUNDAY, FEBRUARY 24
5:30 – 9:30 PM

- Ronald Reagan Building and International Trade Center Amphitheater
  1300 Pennsylvania Ave NW
  Washington, DC 20004

- My Turn, the Film tells the story of Scott Matzka, a former professional hockey player, and his first-hand perspective on how his life has changed since being diagnosed with amyotrophic lateral sclerosis, or ALS.

GUEST PANEL
- Moderator: Jean Campbell, Founder, JF Campbell Consultants
- Catie Matzka, ALS Advocate
- Brooke Peters, Cinematographer and Editor, Rhino, A Story Company
- Kevin Romeo, Co-Founder and CEO, Rhino, a Story Company
- C. Grace Whiting, J.D., President and C.E.O., National Alliance for Caregiving
MONDAY, FEBRUARY 25
8:00 AM – 4:45 PM
• Ronald Reagan Building and International Trade Center - Atrium
  1300 Pennsylvania Ave NW, Washington, DC 20004

REGISTRATION AND BREAKFAST
8:00 – 8:45AM
• Ronald Reagan Building Atrium

WELCOME:
8:45 – 9:30 AM
• Ronald Reagan Building Atrium Hall and Livestream
  • Meet Darlene Shelton, a grandparent advocate. Darlene serves as Founder and President for Danny's Dose Alliance. Darlene will share her incredible story of how she took legislative action to ensure the protection of her grandson, after he was diagnosed with severe Hemophilia B. Julia will also provide an overview of the EveryLife Foundation’s mission to advance the development of treatment and diagnostic opportunities for rare disease patients through public policy.
  • Julia Jenkins, Executive Director, EveryLife Foundation for Rare Diseases
  • Darlene Shelton, Founder, Danny’s Dose

THE NEW CONGRESS: HEALTH POLICY FORECAST
9:30 – 10:00 AM
• Ronald Reagan Building Atrium Hall and Livestream
• Healthcare continues to be one of the most challenging issues for Americans. Hear from Congressional and Administration staffers about how the new Congress will impact healthcare policy in 2019, and how you can help shape these trends.
  • Moderator: Christopher Porter, Vice President of Government Affairs and Policy, Retrophin
  • Tim Pataki, Special Assistant to the President and Deputy Director, Office of Public Liaison
  • Wendell Primus, Senior Policy Advisor, Office of Speaker Nancy Pelosi
  • Grace (Stuntz) Graham, Health Policy Director, Senate Health, Education, Labor, and Pensions Committee, Majority Staff
2019 LEGISLATIVE ASKS
10:00 – 11:00 AM
- Ronald Reagan Building Atrium Hall and Livestream
- We encourage advocates to make one specific “ask” during their meetings with Members of Congress. This panel highlights some potential “asks” that are priorities for the rare disease patient community including: supporting newborn screening reauthorization, increased funding for the National Institutes of Health and the Food and Drug Administration, and the importance of joining the rare disease caucus.
  - **Moderator:** Taylor Kane, Founder and CEO, Remember the Girls and YARR Representative
  - Rebecca Abbot, Deputy Director of Federal Affairs for Public Policy, March of Dimes
  - Ellie Dehoney, Vice President of Policy and Advocacy, Research!America
  - Dennis Sills, Legislative Director, Representative G. K. Butterfield
  - Shayne Woods, Legislative Assistant, Representative Gus Bilirakis

BREAK
11:00 – 11:15 AM

BREAKOUT SESSIONS
11:15 – 12:00 PM

TRACK A: DEEP DIVE POLICY - NEWBORN SCREENING (NBS) REAUTHORIZATION
- Ronald Reagan Building Atrium Hall and Livestream
- Newborn Screening (NBS) helps identify babies who have certain serious medical conditions, so they may receive proper treatment. NBS programs in the U.S. have been successful, preventing thousands of premature illnesses and deaths. Learn about the current NBS federal legislation and how you can help to reauthorize this critical legislation to make sure every child has a chance at a healthy life.
  - **Moderator:** Jaimie Vickery, Vice President of Policy and Advocacy, Cure SMA
  - Debra Schaefer, Patient Advocate
  - Rebecca Abbot, Deputy Director of Federal Affairs for Public Policy, March of Dimes
  - Natasha Bonhomme, Chief Strategy Officer, Genetic Alliance
  - Sarah Gelbard, Newborn Screening Policy Fellow, EveryLife Foundation

TRACK B: INTRODUCING YOUR OWN LEGISLATION
- Ronald Reagan Building Atrium Ballroom A
- This session is designed for advocates who have an idea for legislation, but don’t know where to start. Learn from a former congressional staffer as well as seasoned advocates about how to identify the problem and work with Congress to create a law that provides a meaningful solution.
  - **Moderator:** Steve Silvestri, Director of Public Policy, EveryLife Foundation for Rare Diseases
  - Nancy Goodman, Founder and Executive Director, Kids v Cancer
  - Heather Ferguson, Founder and Executive Director, Lymphedema Advocacy Group
  - Ashley Valentine, CEO, Sick Cells

TRACK C: LOBBYING 101 - MOCK MEETING
- Ronald Reagan Building Atrium Ballroom B
- This is a must-attend session for those individuals who are new to advocacy. Hear from professional lobbyists about how legislation is passed and how to be most impactful in Congressional meetings. Seasoned advocates will present some dos and don’ts in a mock Congressional meeting skit. This is a great opportunity to get questions answered by experienced rare disease advocates.
  - **Jennifer Bernstein,** Executive Vice President, Horizon Government Affairs
  - **Ryan Fischer,** Sr. Vice President of Community Engagement, Parent Project Muscular Dystrophy
  - **Jennifer Van Houtan,** Co-founder, Noah’s Hope Foundation
  - **Skye Miu Steppe,** Rare Disease Advocate
  - **Sarah B. Tompkins,** Founder, EDS Northwest

Presented by:
#RareDC2019
GROUP PHOTO
12:00 – 12:30PM
• Ronald Reagan Building Atrium Stairs

NETWORKING LUNCH
12:30 – 1:15PM
• Ronald Reagan Building Atrium
• Advocates enjoy good food, meet fellow advocates, and open their icebreaker fortunes!

PREPARING FOR SUCCESSFUL MEETINGS
1:15 – 2:15 PM
• Ronald Reagan Building Atrium Hall and Livestream
• Mandatory for advocates participating in Lobby Day (family room will be closed at this time). Advocates receive their Lobby Day schedules and strategize with their teams to make the most of their meetings on Capitol Hill.
• Mike Gula, Co-founder, Prime Advocacy
• Shannon von Felden, Program Manager of Rare Disease Legislative Advocates, EveryLife Foundation for Rare Diseases

SPECIAL SESSION FOR THOSE NOT ATTENDING LOBBY DAY
BUILDING EFFECTIVE PARTNERSHIPS, A CASE STUDY
• Ronald Reagan Building Ballroom A
• Those not attending Lobby Day are invited to this deep dive case study on developing a meaningful partnership with patient advocacy groups, industry, and the Food and Drug Administration (FDA). Hear from the Amyotrophic Lateral Sclerosis (ALS) Association and representatives from ALS patient community about how they worked with the FDA and industry stakeholders to develop a community patient survey (ALS PREFER), as well as ALS draft guidance.
• James Valentine, J.D., M.H.S., Associate Attorney, Hyman, Phelps & McNamara, P.C.
• Neil Thakur, Ph.D., Executive Vice President, Mission and Strategy, The ALS Association
• Kristina Bowyer, Executive Director of Patient Advocacy and Engagement, Ionis Pharma
• Bonnie Charpentier/Amy Laverdiere, Cytokinetics

SNACK BREAK
2:15 – 2:45 PM

Presented by:

Genentech

#RareDC2019
BREAKOUT SESSIONS
2:45 – 3:30 PM

TRACK A: DEEP DIVE POLICY - NIH/FDA APPROPRIATIONS
- Ronald Reagan Building Atrium Hall and Livestream
- Investment in rare disease biomedical research conducted by the National Institutes of Health (NIH) ultimately leads to treatments for patients. Investment in the U.S. Food and Drug Administration (FDA), which serves as the gateway for rare disease products entering the U.S. market, is equally important to facilitating patient access to treatments. Learn how to support consistent and robust funding for both agencies, which will bring more life-saving treatments to the 30 million Americans living with one of more than 7,000 known rare diseases.
  - Christina Hartman, Senior Director of Public Policy and Advocacy, EveryLife Foundation for Rare Diseases
  - John Bartram, Partner, Squire Patton Boggs
  - Steve Grossman, Co-Founder and Deputy Executive Director, Alliance for a Stronger FDA
  - Patrick Carroll, Professional Staff, Senate Appropriations Subcommittee on Agriculture, Rural Development, Food and Drug Administration, and Related Agencies

TRACK B: ADVOCACY FOR YOUNG ADULTS: YOUNG ADULT REPRESENTATIVES OF RDLA (YARR)
- Ronald Reagan Building Atrium Ballroom A
- This session is for young adults ages 16 – 30 years old. In this session young advocates will learn how to make a difference on Capitol Hill and how to have an effective meeting with their Member of Congress.
  - Lauren Cox, YARR Representative
  - Anna Laurent, YARR Representative

TRACK C: LOBBYING 101 - PRACTICE YOUR PITCH
- Ronald Reagan Building Atrium Ballroom B
- This session is for new advocates who are looking for some extra practice before their Hill meetings. Advocates will learn how to tell their story, cohesively tie in their “ask”, refine their elevator pitch and fine-tune their legislative talking points.
  - Chris Smith, President and Chief Executive Officer, SmithSolve
  - Joel White, Founder and President, Horizon Government Affairs

BREAK
3:30 – 3:45 PM

The Family Room
- The Family Room at the Ronald Reagan Building Atrium Gallery, will be available for families all day except from 1:15 – 2:15 pm, when families are required to attend the “Preparing for Successful Meetings” session. We also encourage families to eat in the Atrium with their state teams.

Presented by:

EVERSANA™
BREAKOUT SESSIONS
3:45 – 4:30 PM

TRACK A: MAKING THE CASE FOR A RARE DISEASE BURDEN STUDY
- Ronald Reagan Building Atrium Hall and Livestream
- Scientific evidence is critical to addressing public health issues, because ill-informed policy-making can result in failed strategies and programs. One way to gather evidence about rare diseases is to conduct a burden study. Burden studies determine the impact of a health problem as measured by financial cost, mortality, morbidity, or other indicators. In this session, advocates will learn how a burden study could help the rare disease community by predicting future needs and setting priorities to address these needs.
  - Moderator: George J. Wan, PhD, Vice President of Health Economics Outcomes Research, Mallinckrodt Pharmaceuticals
  - Jennifer Pollack, Associate Director of Federal Affairs, Alzheimer’s Association
  - Charlie Arnowitz, Senior Legislative Assistant, Congressman Andre Carson
  - Lizzy Fox, Legislative Assistant, Congressman Eric Swalwell
  - Deanna Portero, Executive Director, Fibrous Dysplasia Foundation

TRACK B: ADVOCACY FOR YOUNG ADULTS - PRACTICE MEETINGS WITH CONGRESSIONAL STAFF
- Ronald Reagan Building Atrium Ballroom A
- This session is for young adults (ages 16-30) to practice telling their stories and asks to Congressional staffers. Attendees will learn tips and tricks for successful meetings on the Hill from seasoned advocates.
  - Moderator: Shira Strongin, Founder, Sick Chicks and YARR Representative
  - Michelle Greenhalgh, Health Policy Director, Office of Rep. Diana DeGette
  - Taylor Hittle, Legislative Director, Office of Rep. Markwayne Mullin
  - Kimberly Miller-Tolbert, Legislative Assistant, Office of Senator Cory Booker
  - James “J.P” Paluskiewicz, Republican Chief Counsel for the House Committee on Energy and Commerce, Health Subcommittee

TRACK C: ACCESS TO TREATMENT OF RARE DISEASES – A SPOTLIGHT ON RARE CANCER
- Ronald Reagan Building Atrium Ballroom B
- There are many challenges of rare diseases such as late or incorrect diagnosis, lack of access to appropriate therapies, limited number of clinical trials, and lack of interest in developing new therapies, to name a few. In this session, healthcare advocates will examine the challenges of access and value as it pertains to cancer care. Participants will gain an understanding of how to make better decisions about the management of rare disease.
  - Moderator: Danielle Leach, Senior Director of Government Relations and Advocacy, St. Baldrick’s Foundation
  - Elizabeth Fox, Children’s Hospital of Philadelphia
  - Rebecca Kirch, EVP of Healthcare Quality and Value for National Patient Advocate Foundation

CLOSING REMARKS
4:30 – 4:45 PM
WELCOME & OVERVIEW
8:45 – 9:30 AM

Julia Jenkins, Executive Director, EveryLife Foundation for Rare Diseases

Julia has worked for the EveryLife Foundation for nearly 10 years. She was appointed Executive Director and Board Secretary in 2013 after serving as the Director of Public & Government Relations since the Foundation was founded in 2009. Prior to working at EveryLife, Julia worked as a Political Communications Consultant with the Lew Edwards Group, creating strategic plans to organize communities to support local funding measures. Julia was Legislative Director and registered California State lobbyist for Public Employees Union Local #1. She coordinated all political action at local, state, and federal levels, including efforts to ensure every Californian would have access to affordable quality healthcare. She led efforts for the San Francisco Democratic Party, helping to defeat the anti-labor propositions in the 2005 Special Election and worked for House Minority Leader Nancy Pelosi. Prior to working in politics, Julia was a fundraiser for the Muscular Dystrophy Association. Julia did her Masters work in Political Science at San Francisco State University and her undergraduate work in Communications at St. Mary’s College of California.

Darlene Shelton Founder, Danny’s Dose

After her first grandson was unexpectedly diagnosed with severe hemophilia B, Darlene discovered protocols prohibiting paramedics & emergency room physicians from administering specialty medications carried by rare disease patients. Realizing the danger, she and her family knew they had been given this trial to save others so they stepped into action creating the Danny’s Dose Alliance in 2015 and embarked on a journey to spur change. Starting in Missouri working with the state EMS Association, Darlene realized the problem pertains to all patients with special medical needs for specific treatment or medications. In just a few short years, Darlene has become a strong advocate in the rare community and is proud to be working with and creating collaborations with families, organizations, state and national EMS associations, HRSA and the American Academy of Pediatrics on not only the need for legislation and protocol changes but in creating a national paramedic educations campaign and actively educating families in emergency preparedness.

THE NEW CONGRESS: HEALTH POLICY FORECAST
9:30 – 10:00 AM

Moderator Christopher Porter @RetrophinRare Vice President of Government Affairs and Policy, Retrophin

Chris has over 20 years of Government and Public Affairs experience, with deep healthcare policy know-how and advocacy successes spanning Congress, the Executive Branch, State governments, and the globe. Porter served in Congress for 10 years in senior health policy roles and played key behind-the-scenes roles on major initiatives at the House Energy and Commerce Committee in Medicare, Medicaid, FDA, and public health. Prior to working for Retrophin, Porter spent 10 years at Novo Nordisk, helping build and ultimately lead the US government affairs and global public affairs teams in Washington and Copenhagen respectively. Porter also served as CEO of internet media companies YourCongress, Inc and Blutersky Media LLC. Currently, Chris serves on the Children’s National Medical Center’s Corporate Advisory Board, as well as the Board of Directors for the Dance Institute of Washington, the Public Affairs Council, and the bipartisan Keystone Policy Center.

Tim Pataki Special Assistant to the President and Deputy Director, Office of Public Liaison

Tim previously served during the Trump Administration as Special Assistant to the President for Legislative Affairs. Tim is a Maryland native and proud graduate of The Ohio State University, whose Capitol Hill experience included positions with House Majority Leader Kevin McCarthy of California, for whom he served as special assistant to the chief deputy whip; and as a floor assistant for former Majority Leader Eric Cantor of Virginia. More recently, Tim worked for the House Energy and Commerce Committee.
THE NEW CONGRESS:
HEALTH POLICY FORECAST
9:30 – 10:00 AM

Wendell Primus, Ph.D.
@SpeakerPelosi
Senior Policy Advisor, Office of Speaker Nancy Pelosi

Wendell is the Senior Policy Advisor on Budget and Health issues to Speaker Nancy Pelosi. In this capacity, he was the lead staffer in developing the Affordable Care Act. He also played a major role in the Sustainable Growth Rate legislation in 2015 and various budget agreements. Prior to this appointment in March, 2005, Dr. Primus was the Minority Staff Director at the Joint Economic Committee. He has also held positions at the Center on Budget and Policy Priorities, served in the Clinton Administration at the Department of Health and Human Services and also served as Chief Economist for the House Ways and Means Committee and Staff Director for the Committee’s Subcommittee on Human Resources. Dr. Primus received his Ph.D. in economics from Iowa State University.

Grace (Stuntz) Graham
Health Policy Director,
Senate Health, Education, Labor, and Pensions Committee, Majority Staff

Grace has worked on the Senate Health, Education, Labor, and Pensions (HELP) Committee since 2011. She started working for Senator Enzi and continued working for Senator Lamar Alexander on HELP starting in 2013, focusing on policy related to the Food and Drug Administration. She has worked on legislation such as the FDA Safety and Innovation Act and the Drug Quality and Security Act. She received her degree in biomedical engineering and Master of Public Policy from the University of Virginia.

2019 LEGISLATIVE ASKS
10:00 – 11:00 AM

Moderator
Taylor Kane
@TaylorKane23
Founder and CEO,
Remember the Girls

YARR Representative Taylor is the founder and CEO of the non-profit organization, Remember the Girls, an international support and advocacy group which unites, educates and empowers female carriers of rare genetic disorders—a group which is underrepresented and often overlooked by the medical profession. Taylor learned that she was a carrier of the rare genetic disease Adrenoleukodystrophy (ALD) after her father died from the disease when she was five years old and has been a fierce advocate ever since, having helped raise more than $250,000 for ALD research. In 2013, she successfully lobbied the New Jersey legislature and Governor to enact a law requiring the screening of newborns for ALD, as the disease can only be cured if treated before symptoms develop. Shortly thereafter, she founded a campaign called YAC (Young ALD Carriers) to support young females who carry the gene for ALD and to assist them in effectuating positive change through advocacy, social media and the legislative process. In addition, she currently serves as a leader of the Young Adult Representatives of the EveryLife Foundation for Rare Diseases, educating young adults with rare diseases to advocate for more affordable, safe, and effective treatments.

Rebecca Abbot
Deputy Director of Federal Affairs for Public Policy, March of Dimes

Rebecca serves as the March of Dimes’ primary advocate on federal policy issues related to public health, including immunizations, tobacco use, newborn screening, and substance use disorders. Prior to joining the March of Dimes in 2015, Rebecca worked on Capitol Hill for six years for a senior member of the Ways and Means Committee. She also spent three years at a DC-based government affairs consulting firm where she represented a diverse portfolio of health sector clients. Rebecca holds a Bachelor of Arts degree in political science from the University of Oklahoma. An Oklahoma native, Rebecca, her husband and her daughter live in northern Virginia.
2019 LEGISLATIVE ASKS
10:00 – 11:00 AM

Ellie Dehoney
@ResearchAmerica
Vice President of Policy and Advocacy, Research!America

Ellie has been vice president of policy and advocacy at Research!America since March of 2011. Previously, she served as legislative director for Sen. Sherrod Brown (D-OH), and as health legislative assistant and legislative director for Brown in the House of Representatives. Ellie’s background also includes serving as a health legislative assistant for Senate Minority Leader Tom Daschle (D-SD), as a special assistant in the Office of the Assistant Secretary for Planning and Evaluation (ASPE) within the Department of Health and Human Services, and in nonprofit and private sector roles focused on health care financing and delivery. Ellie received a BA in Economics and English from the College of William & Mary and an MSPH in Public Health from the UNC Chapel Hill.

Dennis Sills
Legislative Director, Representative G. K. Butterfield

Dennis serves as Legislative Director to U.S. Representative G. K. Butterfield (NC-01) and has worked for the Congressman since January 2010. Rep. Butterfield serves on the powerful House Energy and Commerce Committee and was named the Vice Chair of the Health Subcommittee and Dennis assists the Congressman with those assignments. Rep. Butterfield is also the co-chair of the Rare Disease Caucus and the Childhood Cancer Caucus, and Dennis assists with those responsibilities. Dennis also assists the Congressman with his assignment to the Energy Subcommittee and works on other issues including environment, natural resources, education, labor, transportation and infrastructure, budget, appropriations, and tax policy. Dennis earned a bachelor’s degree in Public Policy from Duke University and an MBA from the University of Maryland, College Park.

Shayne Woods
Legislative Assistant, Representative Gus Bilirakis

Shayne currently serves as a Legislative Assistant for the Hon. Gus M. Bilirakis of Florida. In this capacity, he plans and develops legislative initiatives while tracking legislation through the legislative process related to Public Health, Energy, Foreign Affairs, Trade, Agriculture, Education & the Workforce, Natural Resources, and Oversight & Government Reform. Additionally, Mr. Woods prepares public statements, drafts correspondence, and meets with constituents and advocacy groups in regard to the aforementioned issues. Mr. Woods also founded and ran a healthcare consulting practice that advised key industry stakeholders on the Affordable Care Act while providing business development assistance. Mr. Woods holds a B.A. from Washington University in St. Louis.

BREAKOUT SESSIONS
11:00 PM – 12:15 PM

TRACK A: DEEP DIVE POLICY - NEWBORN SCREENING (NBS) REAUTHORIZATION

Moderator
Jamie Vickery
Vice President of Policy and Advocacy, Cure SMA

Jamie oversees all Cure SMA’s advocacy, political and regulatory efforts at the federal and state level. She has over 20 years of advocacy, policy, and political experience, including five years on Capitol Hill, serving as a Senior Legislative Aide to former Representatives Steve Horn (R-CA) and Judy Biggert (R-IL). Ms. Vickery also served as a lobbyist for the March of Dimes Foundation, American Cancer Society, and 340B Health. A native of Southern California, she holds a BA of Political Science from Azusa Pacific University and a Masters of Public Policy from Georgetown University.
TRACK A: DEEP DIVE POLICY - NEWBORN SCREENING (NBS) REAUTHORIZATION

Debra Schaefer
Patient Advocate

Debra worked as a US Army civilian for 40 years before retiring five years ago from HQDA to help care for her second granddaughter. Debra and her husband, Johnny, are from Woodbridge, Virginia, and now reside in Spotsylvania, Virginia with their dog and three horses. They have two daughters (Jen and Jessica), two grandsons (James and Adam, sons of Jen), and two granddaughters (Madison and Bailey, daughters of Jessica). One of the founding members of the Virginia Chapter of Cure SMA, Debra is active in family support, fundraising, and Newborn Screening Advocacy. She travels with Jessica and Bailey every four months to NYC for Bailey's lifesaving treatment for SMA.

Sarah Gelbard
@EveryLifeOrg
Newborn Screening Policy Fellow, EveryLife Foundation for Rare Diseases

Sarah joined the EveryLife Foundation for Rare Diseases as the Newborn Screening Fellow in 2018. Prior, she worked as a research coordinator in the Friedreich's Ataxia Program at the Children's Hospital of Philadelphia to advance natural history and clinical trial research. Sarah received a BA in English Literature and International Relations from the University of Rochester in New York, and an MPH from the Emory University Rollins School of Public Health in Georgia. She worked in reproductive health in Atlanta and Cambodia, and conducted research on water, sanitation, hygiene, and reproductive health access barriers for disabled women in rural areas of Cambodia. Her work has been published by the Annals of Clinical and Translational Neurology, Arts Connect International, and the Emory Global Health Institute.

Natasha Bonhomme
Chief Strategy Officer, Genetic Alliance

Natasha brings 15 years of organizational management and maternal and child health experience to her work at Genetic Alliance. She launched and oversees the nation’s center on newborn screening education, Baby's First Test. As director of Baby's First Test, Natasha has testified before the US Senate on the importance of public education and inclusion of family experiences throughout the newborn screening system. As part of her role to oversee maternal and child health initiatives for the organization, she launched Expecting Health, an initiative to bring a range of consumer and professional stakeholders together to address the need for clear, science-based information for families and individuals through tangible, actionable messaging. Natasha serves on a range of committees including: as a Co-Chair of the Genetics and Bioethics Committee, American Public Health Association and the Secretary’s Advisory Committee on Heritable Disorders in Newborns and Children.

TRACK B: INTRODUCING YOUR OWN LEGISLATION

Moderator
Steve Silvestri
@EveryLifeOrg
Director of Public Policy, EveryLife Foundation for Rare Diseases

Steve is Director of Public Policy at the EveryLife Foundation for Rare Diseases. Prior to joining Everylife, Steve served as Senior Legislative Assistant to Congressman Rodney Frelinghuysen (NJ), former Chairman of the House Appropriations Committee. In this role he was responsible for driving the Congressman’s health policy agenda, including the enactment of robust funding increases for the National Institutes of Health, All of Us initiative, regenerative medicine research, and related programs, as well as new funding authorized by the 21st Century Cures Act to accelerate drug development at the FDA. Steve received his B.A. in Government and Politics, with a supporting sequence in Economics, from the University of Maryland.
Nancy Goodman
@KidsvCancer
Founder and Executive Director, Kids v Cancer

Nancy is Founder and Executive Director of Kids v Cancer and was lead advocate and author of two Federal laws to incentivize and require pharmaceutical companies to develop drugs for children. The Creating Hope Act pediatric priority review voucher program, passed into law in 2012 as 12 U.S.C. 360ff, established a market-based incentive, a voucher, for companies to develop drugs expressly for children with pediatric rare diseases, including pediatric cancers. Over $1.2 billion in vouchers have been traded since the establishment of the program. The RACE for Children Act, which amends the Pediatric Research Equity Act (PREA), 21 U.S.C. 355c, was passed into law in 2017, authorizes the FDA to require companies developing cancer targeted therapies to undertake pediatric studies when the molecular targets of the drugs are substantially relevant to pediatric cancers.

Heather Ferguson
Founder and Executive Director, Lymphedema Advocacy Group

Heather is the Founder and Executive Director of the Lymphedema Advocacy Group (LAG), a nationwide, all-volunteer, grassroots organization comprised of patients, caregivers, clinicians and industry partners. Heather’s son Dylan was born with primary lymphedema in 2006. In 2009, she worked with her State Representative to introduce and pass the North Carolina Lymphedema Diagnosis and Treatment Act. Later that year she met with her Congressman, and in 2010 he introduced the Lymphedema Treatment Act. Through LAG’s advocacy, lymphedema has gone from a little-known condition on Capitol Hill to being the most supported healthcare bill of the 115th Congress, finishing with 451 House and Senate cosponsors. Heather hopes to achieve passage of the Lymphedema Treatment Act during this Congress, so that her son and all lymphedema patients will have insurance coverage for their vital compression therapy supplies.

Ashley Valentine
@sickcells
CEO, Sick Cells

Ashley is the co-founder of Sick Cells and a recognized leader in the sickle cell community at a local and national level. She brings a wealth of experience to the field of sickle cell disease (SCD) both through her professional and personal experience. Ashley has worked as a policy research analyst and monitored programs for federal clients like the Centers for Medicaid and Medicare Services and the Centers for Disease Control and Prevention. She has also worked as a clinical trial coordinator in DC where she learned the complexities of the medical system and running clinical trials at the hospital level. In her personal life, Ashley has an older brother that lives with sickle cell disease. Growing up in a household where the hospitals were like a second school, Ashley understood the importance of advocacy and saw first-hand how important it was to share their personal story. Some milestones for Ashley were testifying at the FDA to help get a sickle cell disease medication approved and working within the sickle cell community to help pass federal legislation.

Jennifer Bernstein
Executive Vice President, Horizon Government Affairs

Jennifer joined Horizon Government Affairs in December 2008, where she currently serves as Executive Vice President, focusing on legislative and regulatory developments within the pharmaceutical and biotechnology sectors. Jennifer is also a former Capitol Hill staffer, as well as a former staffer for the Pennsylvania House of Representatives. She has worked closely with RDLA since its inception and is personally and professionally committed to advancing the needs of the rare disease community.
Legislative Conference Guest Speakers

**TRACK C: LOBBYING 101 - MOCK MEETING**

Ryan Fischer  
@ParentProjectMD  
Senior Vice President of Community Engagement, Parent Project Muscular Dystrophy

Ryan serves as the Senior Vice President of Community Engagement for Parent Project Muscular Dystrophy and has been with the organization for 14 years. Parent Project Muscular Dystrophy is the leading organization in the fight to end Duchenne. Ryan oversees strategic community engagement and grassroots advocacy, guiding the patient community on best practices for interacting with members of Congress and federal agencies. Ryan also leads PPMD’s work in patient preference research, with the aim of advancing the science of patient input. In 2012, Ryan was a member of the core team (in partnership with Johns Hopkins School of Public Health) who developed and produced the first patient preference study on caregiver benefit risk preferences and priorities for emerging treatments in Duchenne. Since then, Ryan has led the development of five additional preferences studies in Duchenne.

Jennifer Van Houtan  
Co-Founder, Noah's Hope Foundation

Jennifer has been a focused and results driven marketing leader with extensive experience in corporate and field marketing during her career with a Fortune 500 company for over 20 years. In 2009, two of Jennifer’s four children were diagnosed with CLN2 Batten disease, a terminal neurodegenerative disease. Understanding the daily needs of a rare disease patient, Jennifer and her husband, Tracy, co-founded “Noah’s Hope” and learned to be a rare disease advocate, shared her family’s story and raised critical research funds. In April 2017, the FDA approved the first Batten treatment, with support of Noah’s Hope Foundation. Jennifer leverages her hands-on practical patient advocacy experience with her previous career in marketing. Jennifer graduated from Loyola University Chicago with a B.S. in Psychology.

Skye Miu Steppe  
Rare Disease Advocate

Skye is a 24-year-old rare disease advocate. At 12 years old Sky was diagnosed with the rare bone disorder, Fibrous Dysplasia (FD). She had three facial reconstructive surgeries, two bone grafts, and one cadaver bone to build a new eye socket. Sky spent the last 4 years raising awareness and advocating for FD and all rare diseases. She has lobbied on Capitol Hill for three years, been nominated for the RareVoice and Patient of Hope Award, and given the honor of speaking at multiple conferences. Sky believes that if rare patients band together, they will change the world for future generations.

Sarah B. Tompkins  
Founder, EDS Northwest

Sarah is a patient advocate for Ehlers-Danlos Syndrome (EDS), Dysautonimia (POTS), and more (GP, MALS). She has received many stabilizations, surgeries and procedures to make mobility and traveling for advocacy possible. Sarah began patient advocacy in 2015, sharing her rare disease story in support of rare disease legislation such as the 21st Century Cures, OPEN ACT, and RARE ACT. While serving as In-District Lobby Days Leader for Washington State, Sarah witnessed the power of utilizing personal stories to affect policy change. Sarah is excited to begin the process of creating a nonprofit for Ehlers-Danlos Syndrome in the Pacific Northwest called EDS Northwest. Sarah believes that following up and continuing relationships with legislative offices is just as important as the initial ask.

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“We may encounter many defeats, but we must not be defeated.”  
- Maya Angelou
### PREPARING FOR SUCCESSFUL MEETINGS

**1:15 – 2:15 PM**

**Mike Gula**
@prime_advocacy
Co-founder, Prime Advocacy

Mike is the co-founder of Prime Advocacy, the leading Washington, D.C. Advocacy Day firm. Since 2009, Gula Graham has raised over $250,000,000 for House and Senate members of Congress. Since 2010, Prime Advocacy has scheduled over 26,000 meetings for 30,000 attendees. When Congress was in session, Prime Advocacy secured a meeting with the actual member of Congress 39% of the time in 2018 and confirmed meetings with 96% of the Congressional offices where meetings were requested. A native of Austin, Texas, Mike received his Bachelor of Arts degree from the University of Richmond, where he also served as captain of the baseball team.

**Shannon von Felden**
@RareAdvocates
Program Manager of Rare Disease Legislative Advocates, EveryLife Foundation for Rare Diseases

Shannon manages the Rare Disease Legislative Advocates program at the EveryLife Foundation for Rare Diseases where she works with advocates from across the country to engage at the local, state, and federal level. She began her career on Capitol Hill as a Legislative Assistant for Congresswoman Shelley Berkley (NV) working on health care and veterans affairs issues. Shannon has worked with national nonprofit organizations to further their policy and advocacy goals including the Juvenile Diabetes Research Foundation and National Osteoporosis Foundation. She received her Master of Public Policy from American University.

### BREAKOUT SESSION: BUILDING EFFECTIVE PARTNERSHIPS, A CASE STUDY

**James Valentine, J.D., M.H.S.**
@fdalawblog
Associate Attorney, Hyman, Phelps & McNamara, P.C.

James is an associate attorney at Hyman, Phelps & McNamara, P.C. where he assists medical product industry and patient advocacy organization clients in a wide range of regulatory matters, including new drug and biologic development and approval issues. Mr. Valentine has been central to the transition of the FDA Patient-Focused Drug Development, or PFDD, program to externally-led meetings, having helped with the planning of fifteen such meetings and having moderated 14 of those, and is also working to implement novel methodologies for capturing patient experiences and preferences. Before joining the firm in 2014, Mr. Valentine worked at FDA where he facilitated patient input in benefit-risk decision-making, including helping facilitate the FDA Patient Representative Program and with the launch of the PFDD program.

**Neil Thakur, Ph.D**
@alsassociation
Executive Vice President, Mission and Strategy, The ALS Association

Neil is Executive Vice President of Mission Strategy at the ALS Association where he leads the advocacy, care services, and research programs for the Association. Prior to joining the Association, Neil served in the National Institutes of Health (NIH) Office of the Director, where he helped make NIH research more open and less burdensome. He managed the world’s largest policy to make biomedical research papers publicly accessible and co-chaired the White House task force that lead to the requirement that all federal science agencies adopt similar policies. He also spent a year on detail to the US Senate Special Committee on Aging, working on quality of long-term health care, particularly Alzheimer’s care and pharmaceuticals. He holds a Ph.D. in Health Policy from Yale University School of Public Health.
BREAKOUT SESSION: BUILDING EFFECTIVE PARTNERSHIPS, A CASE STUDY

Kristina Bowyer  
@ionispharma  
Executive Director of Patient Advocacy and Engagement, Ionis Pharma

Kristina has worked at Ionis for over 26 years and has spent the last seven years successfully building Ionis’ internal patient engagement program to ensure that the patient perspective is incorporated into every aspect of drug development from research and target identification through market approval. Kristina’s expertise is in rare and neurological diseases where she has developed new strategies to capture burden of disease from both the patient and the caregiver perspectives through innovative partnerships across multiple programs and organizations, such as spinal muscular atrophy, amyloidosis, myotonic dystrophy and ALS. Ionis currently has an extensive rare disease pipeline of RNA targeted therapeutics and has established strategic alliances with global pharmaceutical companies with the expertise to successfully launch rare disease treatments and ensure broad patient access.

BREAKOUT SESSIONS  
2:45 – 3:30PM

TRACK A: DEEP DIVE POLICY - NIH/FDA APPROPRIATIONS

John Bartrum, Partner, Squire Patton Boggs

John is a partner at the law firm Squire Patton Boggs. He previously served as senior professional staffer to the US House Appropriations Committee since 2009 and was also a member of the federal Senior Executive Service. Before joining the House Appropriations Committee, he was an associate director of the NIH. John previously served as part of the National Security Division of the Office of Management and Budget (OMB) in the Executive Office of the President. In addition to this substantial healthcare experience, John has more than 30 years of military experience in both active duty and as a reserve officer.

Steve Grossman, Co-Founder and Deputy Executive Director, Alliance for a Stronger FDA

Steven is President of HPS Group, LLC, a DC-based policy and regulatory consulting firm that he started in 2001. He was a co-founder of the Alliance for a Stronger FDA and continues to serve as the organization’s Deputy Executive Director. The Alliance is a multi-stakeholder coalition that advocates for increased resources for the FDA. Active since 2008, the Alliance has contributed to a doubling of FDA’s appropriation. Steven serves on the Board of Directors of the National Organization for Rare Disorders (NORD) and sits on the board’s executive committee. This reflects a commitment from his years on Capitol Hill, where he was one of the chief Senate negotiators for the Orphan Drug Act of 1983. After leaving the Senate, he served as a Deputy Assistant Secretary for Health at HHS.

Patrick Carroll, Professional Staff, Senate Appropriations Subcommittee on Agriculture, Rural Development, Food and Drug Administration, and Related Agencies

Patrick joined the Senate Appropriations Subcommittee on Agriculture, Rural Development, Food and Drug Administration, and Related Agencies in 2015. He is responsible for funding and policy issues related to the Food & Drug Administration, and has served 13 years in the House of Representatives as Legislative Director for former Congressman Kevin Yoder (KS) and Legislative Assistant for former Congressman Ray LaHood (IL). In addition, Patrick worked for over eight years in the private sector, including for three years as Government Relations Director for the Biotechnology Industry Organization, where he was the primary advocate for the biotechnology industry for issues that fall within the scope of the federal Appropriations process. Patrick graduated from Syracuse University in 1993.
**TRACK A: DEEP DIVE POLICY - NIH/FDA APPROPRIATIONS**

Christina Hartman, MPH  
@EveryLifeOrg  
Senior Director of Public Policy and Advocacy, EveryLife Foundation for Rare Diseases

Christina is a DC-based policy and advocacy expert with a background in building alliances. Christina is experienced in elevating the voices of patients, parents, scientists and clinicians to have a positive impact on health and nutrition policy. She worked with staff and member leadership at the American College of Cardiology to develop an agenda for improving cardiovascular health outcomes. At the Pew Charitable Trusts, she worked with a broad range of partners to advance legislative goals that incentivize the development of new antibiotic drugs. Prior to that, she served as an analyst at the Centers for Disease Control and Prevention in Atlanta and in the Office of the Secretary for the U.S. Department of Health and Human Services (HHS) in Washington, D.C. Christina served as Project Officer for a Cooperative Agreement between HHS and the World Health Organization. She has also worked in the food and beverage industry, including at the Beer Institute, where she pursued outcomes favorable to industry on a wide range of domestic and international public health and policy issues. Christina was attracted to the opportunity to build and lead an advocacy team at the EveryLife Foundation in Washington, DC based on her own experience with the diagnostic odyssey – her recent journey with her youngest daughter, Charlotte.

**TRACK B: ADVOCACY FOR YOUNG ADULTS: YOUNG ADULT REPRESENTATIVES OF RDLA (YARR)**

Lauren Cox  
YARR Representative

Lauren is a twenty-six-year-old mother of two, and a proud wife. She was born with Fibrous Dysplasia, a rare bone disease that has no cure. She has undergone ten facial reconstructive surgeries. At nineteen years old, she began to engage in patient advocacy by working with the Fibrous Dysplasia Foundation (FDF) and The Magic Foundation (MAGIC). She designed the official awareness ribbon for Fibrous Dysplasia and McCune-Albright Syndrome (FD/MAS) in September 2012. After becoming the largest distributor for FD/MAS merchandise worldwide, she joined forces with FD Warriors Inc. to advocate on local and federal level for rare diseases. Lauren has been nominated for the Global Genes Champion of Hope Award for five consecutive years, and also been nominated for the Rare Voice Award. Recently, she has worked towards launching the first “FD/MAS Global Awareness Week”, became a member of YARR (Young Adult Representatives of RDLA), and designed the YARR logo.

Anna Laurent  
YARR Representative

Anna is a Community Prevention Specialist at Lotus Sexual Violence Resource & Children’s Advocacy Center. Born and raised on a family farm in Kentucky, she has been advocating for rare diseases for many years. Anna co-founded a Facebook support group for teens and young adults with ALGS and became the first Junior Board Member of the Alagille Syndrome Alliance in 2015. She then served as a Board Member and was the keynote speaker at the 7th International Symposium on Alagille Syndrome. Anna was a speaker at the 8th Annual SBP Rare Disease Day Symposium, the first scientific meeting on Alagille Syndrome. Anna is a member of the Young Adult Representatives of RDLA and plans to continue advocating for years to come.

“Hardships often prepare ordinary people for an extraordinary destiny.”  
- C.S. Lewis
**Track C: Lobbying 101 - Practice Your Pitch**

Chris Smith  
@SmithSolve  
President and Chief Executive Officer, SmithSolve  

Chris has over 25 years of experience in healthcare communications, including leadership positions with global pharmaceutical, biotechnology, and public relations firms. As an expert in rare disease communication, he knows how to address the scientific, medical, clinical, social, and economic aspects of orphan drug development. His work spans more than a dozen biopharma companies, including patient advocacy support, patient panels and meet-ups, media relations, investor events, patient days, clinical trial recruitment, corporate branding and website development, pipeline communications, data announcements, and more. Chris has worked across diverse therapeutic categories and has represented many leading healthcare companies, including Alexion, AstraZeneca, Johnson & Johnson, Pfizer, and Sanofi. He is a founding member of the Rare Collective®, a group of trusted independent advisors in orphan drug development.

**Joel White**  
Founder and President, Horizon Government Affairs  

Joel is the President of Horizon Government Affairs (HGA), a full-service government affairs consultancy specializing in actionable strategic and tactical advice for navigating the Congressional and regulatory processes. HGA assists clients in their quest to improve health and lower health related costs. Joel is also the Executive Director of the Health IT Now Coalition and is the President of the Council for Affordable Health Coverage, two HGA-managed coalitions. Joel spent twelve years on Capitol Hill as professional staff for the Ways and Means Committee and two Members of Congress. He helped enact nine laws, including the 2002 Trade Act, which created healthcare tax credits, the 2003 law that established the Medicare prescription drug benefit and Health Savings Accounts, the 2005 Deficit Reduction Act, and the 2006 Tax Reform and Health Care Act, which reformed Medicare payment options.

**Breakout Sessions**

**Track A: Making the Case for a Rare Disease Burden Study**

**Moderator**  
George J. Wan, PhD  
@MNK  
Vice President of Health Economics Outcomes Research, Mallinckrodt Pharmaceuticals  

George Wan, Ph.D., is Vice-President, Health Economics and Outcomes Research at Mallinckrodt Pharmaceuticals where he is responsible for leading the development and execution of the value evidence generation and dissemination strategy for the company. He has over 20 years of experience in this area. Prior to this, he lived in Belgium as Global Market Access Leader with Janssen, a division of Johnson & Johnson (J&J). Previously, he held roles of increasing responsibility at other J&J affiliates, Wyeth and Merck. He has co-authored over 60 manuscripts and given over 100 presentations at conferences. George holds a B.A. in Economics/Political Science from the University of Richmond, a Master’s in Public Health from Virginia Commonwealth University and a Ph.D. in Health Services Research/Epidemiology from Saint Louis University.

**Charlie Arnowitz**  
@RepAndreCarson  
Senior Legislative Assistant, Congressman Andre Carson  

Charlie currently serves as Senior Legislative Assistant for Congressman André Carson (D-IN), and is the Congressman’s primary advisor on a wide range of domestic policy issues including healthcare, education, immigration, and human services. He previously worked as a Legislative Aide to Senator Mark Warner (D-VA) and as a Legislative Assistant at the Religious Action Center of Reform Judaism, a nonprofit advocacy group focused on the domestic safety net. Arnowitz graduated magna cum laude from Middlebury College, where he studied political science and Chinese and was president of the student government. He is a native of Illinois.
**TRACK A: MAKING THE CASE FOR A RARE DISEASE BURDEN STUDY**

Jennifer Pollack  
@alzassociation  
Associate Director of Federal Affairs, Alzheimer’s Association

Jennifer is an Associate Director, Federal Affairs for the Alzheimer’s Impact Movement (AIM) and the Alzheimer’s Association. She is primarily responsible for the care and support portfolio and works closely with Congress to address the urgent needs of individuals living with Alzheimer’s disease and related dementias and their families. Previously, Jennifer worked at the Medical Group Management Association (MGMA) and as a Legislative Assistant for former Congressman Ed Pastor. Prior to that, she served as an associate attorney at a law firm where she researched and advised on regulations relating to the Affordable Care Act. Jennifer earned her law degree from The Catholic University of America, Columbus School of Law and her bachelor’s degree in psychology from The George Washington University.

Deanna Portero, Executive Director of the Fibrous Dysplasia Foundation.

Since joining the FDF in 2015 as its first full-time staff member, Deanna has led the organization through a period of unprecedented growth and achievements, including the launch of a highly successful patient registry, and awarding approximately half a million dollars in research grant funding. Prior to leading FDF, Deanna held positions at the National Organization for Rare Disorders where she contributed to a diverse set of programs, including digital supporter engagement, registry development, research grant administration, and other project management. Deanna is a graduate of Dartmouth College. She currently serves as co-chair of the Public Policy Working Group of the Everylife Foundation’s Community Congress.

**TRACK B: ADVOCACY FOR YOUNG ADULTS - PRACTICE MEETINGS WITH CONGRESSIONAL STAFF**

Moderator  
Shira Strongin  
@ShiraStrongin  
Founder, Sick Chicks  
YARR Representative

At 19, Shira is an accomplished, award-winning activist and writer who has multiple rare diseases, but does not let them stop her from pursuing her passions. She is the founder of Sick Chicks, an international community that works to empower and unite women with disabilities and illnesses. Her writing has been featured in publications such as Forbes, and she has a passion for speaking on topics such as disability rights, women’s rights, and engaging the young adult community in advocacy. Shira is also a co-founder of YARR (Young Adult Rare Disease Representatives).

Lizzy Fox, Legislative Assistant, Congressman Eric Swalwell (D-CA)

Lizzy is Legislative Assistant to Congressman Eric Swalwell (D-CA) where she handles health policy. Congressman Swalwell was the lead sponsor of the Advancing Access to Precision Medicine Act (H.R. 5062) in the 115th Congress.

**Most of the important things in the world have been accomplished by people who kept on trying when there seemed to be no hope at all.”**  
- Dale Carnegie
**Legislative Conference Guest Speakers**

**TRACK B: ADVOCACY FOR YOUNG ADULTS - PRACTICE MEETINGS WITH CONGRESSIONAL STAFF**

Kimberly Miller-Tolbert  
@CoryBooker  
Legislative Assistant, Office of Senator Cory Booker

Kim currently serves as a Legislative Assistant for Senator Cory Booker (D-NJ). In this capacity, she assists Senator Booker in his work to increase access to quality, affordable health care and improve the health and well-being of Americans. She has had the privilege of working on legislation that would improve the lives of people living with a rare disease, including the Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act. Before joining Senator Booker’s office, Kim worked for Senator Harry Reid (D-NV), and she is a graduate of the University of Michigan (BA) and Yale University (MPH).

Michelle Greenhalgh  
@RepDianaDeGette  
Health Policy Director, Office of Rep. Diana DeGette

Michelle serves as Health Policy Director for Rep. Diana DeGette (D-CO), Chair of the House Energy & Commerce Oversight and Investigations Subcommittee. In this capacity she advises the Congresswoman on all health care issues, including the management and leadership of the House Pro-Choice Caucus. She previously served as a legislative assistant for Senator Jeanne Shaheen (D-NH) and Rep. Joe Courtney (D-CT). Prior to Capitol Hill, Michelle was a lobbyist for the American Academy of Family Physicians, the nation’s largest association representing family physicians. She also worked as an academic medical researcher on clinical trial ethics at the Johns Hopkins Berman Institute of Bioethics after completing her graduate degree. Michelle received her master’s from the Johns Hopkins University and holds a bachelor’s from the University of New Hampshire.

Taylor Hittle  
@RepMullin  
Legislative Director, Office of Rep. Markwayne Mullin

Taylor Hittle currently serves as the Legislative Director for Congressman Markwayne Mullin (R-OK). Taylor has been on the leading edge of healthcare issues for a number of years and has been a champion for the rare and undiagnosed disease community throughout her time on Capitol Hill. Taylor has spent the past several years on Capitol Hill, previously serving as Health Policy Adviser and Senior Legislative Assistant to Congresswoman Mimi Walters (R-CA), Staff Assistant for the House Energy and Commerce Health Subcommittee, and Legislative Assistant and for Congressman John Carter (R-TX). A graduate of the University of Texas at Austin, Taylor lives in Washington, D.C. with her husband and adorable dogs, Shiner and Noel.

James “J.P.” Paluskiewicz  
Republican Chief Counsel for the House Committee on Energy and Commerce, Health Subcommittee

J.P. oversees all policy under the Subcommittee’s jurisdiction, helps coordinate Committee activity, and leads bipartisan, bicameral negotiations. In addition, he is the sole policy staffer overseeing Medicare Parts B and D and health information technology. Before formally joining the Committee staff, J.P. served for almost seven years as Deputy Chief of Staff to the Honorable Michael C. Burgess, M.D. (R-TX). During that time, he oversaw the Congressman’s legislative agenda and acted as his chief advisor in all public policy. J.P. also served the Honorable Charlie Norwood, D.D.S. (R-GA), a senior Energy and Commerce Committee member and one of the most engaged Members of Congress in health care policy, as his Legislative Director and Health Policy Advisor for almost five years before his death in 2007. J.P. is a recipient of the Bipartisan Congressional Staff Award from the Alliance for Health Policy, the Special Recognition Award from the American Psychiatric Society, the Gift of Life Legislator of the Year Award from the American Society of Transplantation, the Congressional Staff Leadership Award from America’s Essential Hospitals, the Charles G. Norwood Memorial Advocacy Award from the Coalition for Pulmonary Fibrosis, and the Congressional Staffer of the Year award from the National Multiple Sclerosis Society. In addition, J.P. was identified by Politico as an “Emerging Healthcare Leader” in 2012.
For your generous support of Rare Disease Week on Capitol Hill

Thank you to

VERTEX

#RareDC2019

22
TUESDAY, FEBRUARY 26
7:00 – 8:30 AM

- Washington Court Hotel
  525 New Jersey Avenue NW
  Washington, DC 20001

Join the EveryLife team and rare disease advocates for the Lobby Day Breakfast.

FEATURING ADDRESS BY
- James “JD” Derderian,
  Founding Partner,
  Stanton Park Group,
  Pediatric Cancer Parent Advocate

Mr. Derderian is an experienced advocate and former House Committee on Energy and Commerce Staff Director with expertise in healthcare, technology, and telecommunications. He also leads Imagine An Answer to Kids’ Brain Cancer, a charity group that aims to be a catalyst for curing Pediatric Low Grade Astrocytoma-JPA. Imagine An Answer exists because J.D.’s son, Clay, was diagnosed with brain cancer at age three.

- Note: At the breakfast, attendees will receive any updates or last-minute changes to their Lobby Day schedules. Staff and Prime Advocacy will also be available for questions.

Hospitality Room
- Join us between meetings from 10:00 AM - 5:00 PM in our Hospitality Room - Cannon House Office Building, Room 121
Lobby Day
Issue Information

TALKING POINTS
✓ This bill will reauthorize critical existing programs that provide assistance to states to improve and expand their newborn screening programs, support parent and provider education, and ensure laboratory quality and surveillance for newborn screening.
✓ Diagnosis through newborn screening enables early intervention that can reduce the progression of irreversible harm and significantly reduce long term treatment costs.
✓ Newborn Screening SAVES LIVES.

KEY BILL PROVISIONS
✓ Reauthorizes the Health Resources and Services Administration (HRSA) grants to states to expand and improve their screening programs, educate parents and health care providers, and improve follow-up care for infants with a detected condition.
✓ Reauthorizes the Secretary’s Advisory Committee on Heritable Disorders in Newborns and Children, which provides states with a Recommended Uniform Screening Panel (RUSP) to help ensure every infant is screened for conditions which have a known treatment.

NEWBORN SCREENING FACTS
✓ Newborn screening reaches each of the 4 million babies born in the U.S. every year.
✓ Approximately 1 in 300 newborns has a condition that can be detected through screening.
✓ Newborn Screening is the practice of testing every newborn for certain genetic, metabolic, hormonal, and functional conditions that are not otherwise apparent at birth.

Legislative Ask #1
Co-Sponsor Reauthorization of the Newborn Screening Saves Lives Act
Support Robust Funding for the National Institutes of Health and the Food and Drug Administration

**TALKING POINTS**

- We request an increase in the annual discretionary funding for the National Institutes of Health (NIH) in the Fiscal Year 2020 Labor, Health and Human Services, Education Appropriations Act; and an increase in funding for the Food and Drug Administration (FDA) in the Fiscal Year 2020 Agriculture Appropriations Act. While significant increases in funding have been made by Congress in recent years, the historical pace of healthcare inflation often outpaces annual funding increases.

**NATIONAL INSTITUTES OF HEALTH (NIH)**

- Funding for the NIH supports research that strengthens the biomedical infrastructure across America while driving forward science that can lead to new therapies and cures.
- NIH is the world’s leading funder of basic biomedical research, supporting breakthroughs in how we understand and treat deadly and debilitating diseases.
- More than 80% of the NIH’s funding is spent outside its own laboratories through competitive grants to researchers at more than 2,500 universities, medical schools, and other research institutions, with a presence in all 50 states.
- Every dollar invested in research by the NIH generates an estimated $2.20 in local economic growth.

**FOOD AND DRUG ADMINISTRATION (FDA)**

- The FDA plays a critical role in bringing cures into the hands of patients through the review and approval of innovative medicines and devices.
- Increased funding for the FDA supports the infrastructure and staff that move treatments through the review pipeline.
- Expanded resources allow FDA to conduct this often-lengthy process more quickly, ultimately lowering costs, increasing safety and bringing treatments to patients sooner.
- This funding supports programs specific to rare diseases and orphan drugs that have yielded vital new treatments.
- With its current budget, the FDA must effectively regulate products that account for 20 cents of every dollar spent by U.S. consumers.
TALKING POINTS

✓ The Rare Disease Caucus is a bipartisan, bicameral caucus that works to raise awareness of rare diseases.
✓ Rare diseases affect more than 30 million Americans and their families.
✓ One in ten Americans has a rare disease.
✓ There are more than 7,000 known rare diseases but unfortunately, the vast majority (over 90%) do not yet have a treatment approved by the FDA.
✓ Rare or orphan diseases are defined as diseases affecting fewer than 200,000 people in the U.S.
✓ More than 80% of rare diseases are considered ultra-rare, affecting fewer than 6,000 people. Some diseases affect fewer than 100.
✓ Rare diseases include rare cancers, tropical or neglected diseases, genetic diseases and many pediatric diseases including cancers. Many of these diseases are life-threatening and have no treatment options.
HOUSE

- Mark Amodei NV-2
- Andy Barr KY-6
- Ami Bera CA-7
- Eddie Bernice Johnson TX-30
- Donald Beyer Jr. VA-8
- Gus Bilirakis* FL-12
- Lisa Blunt Rochester DE
- Suzanne Bonamici OR-1
- Mo Brooks AL-5
- Julia Brownley CA-26
- Vern Buchanan FL-16
- Michael Burgess TX-26
- Cheri Bustos IL-17
- G.K. Butterfield* NC-1
- Salud Carbajal CA-24
- Andre Carson IN-7
- Earl Carter GA-1
- John Carter TX-31
- Judy Chu CA-27
- David Cicilline RI-1
- Lacy Clay MO-1
- Gerald Connolly VA-11
- Susan Davis CA-53
- Rodney Davis IL-13
- Peter DeFazio OR-4
- Diana DeGette CO-1
- Suzan DelBene WA-1
- Ted Deutch FL-21
- John Duncan, Jr. TN-2
- Mike Doyle PA-14
- Eliot Engel NY-16
- Anna Eshoo CA-18
- Brian Fitzpatrick PA-8
- Jeff Fortenberry NE-1
- Ruben Gallego AZ-7
- John Garamendi CA-3
- Josh Gottheimer NJ-5
- Garret Graves LA-6
- Danny Heck WA-10
- Jaime Herrera-Beutler WA-3
- Jim Himes CT-4
- Jared Huffman CA-2
- Hank Johnson GA-4
- David P. Joyce OH-14
- Marcy Kaptur OH-9
- Joseph Kennedy, III MA-4
- Ro Khanna CA-17
- Peter King NY-2
- Raja Krishnamoorthi IL-8
- Darin LaHood IL-18
- Jim Langevin RI-2
- Dan Lipinski IL-3
- Dave Loebsack IA-2
- Zoe Lofgren CA-19
- Alan Lowenthal CA-47
- Blaine Luetkemeyer MO-3
- Carolyn Maloney NY-12
- Sean Patrick Maloney NY-18
- Kenny Marchant TX-24
- Tom Marino PA-10
- Brian Mast FL-18
- Doris Matsui CA-6
- Michael McCaul TX-10
- Tom McClintock CA-4
- Jim McGovern MA-2
- David McKinley WV-1
- Markwayne Mullin OK-2
- Seth Moulton MA-6
- Richard Neal MA-1
- Donald Norcross NJ-1
- Eleanor Holmes Norton DC
- Steven Palazzo MS-4
- Frank Pallone NJ-6
- Jimmy Panetta CA-20
- Bill Pascrell NJ-9
- Donald Payne, Jr. NJ-10
- Scott Peters CA-52
- Collin Peterson MN-7
- Chellie Pingree ME-1
- Bill Posey FL-8
- David Price NC-04
- Mike Quigley IL-5
- Jamie Raskin MD-8
- Kathleen Rice NY-4
- David Rouzer NC-7
- Jan Schakowsky IL-9
- Brad Schneider IL-10
- David Scott GA-13
- Albio Sires NJ-8
- Adam Smith WA-9
- Chris Smith NJ-4
- Lloyd Smucker PA-16
- Chris Stewart UT-2
- Steve Stivers OH-15
- Eric Swalwell CA-15
- Glenn Thompson PA-5
- Paul Tonko NY-20
- Fred Upton MI-6
- Juan Vargas CA-51
- Debbie Wasserman-Schultz FL-23
- Bonnie Watson Coleman NJ-12
- Joe Wilson SC-2
- Robert Wittman VA-1
- John Yarmuth KY-3
- Lee Zeldin NY-1

SENATE

- John Boozman AR
- Christopher Coons DE
- Tom Cotton AR
- John Hoeven ND
- James Inhofe OK
- Angus King ME
- Amy Klobuchar* MN
- Roger Wicker* MS
- Jeff Merkley OR
- James Risch ID
- Chris Van Hollen MD
Congressional Meeting Tips

✓ Start each meeting by thanking the Member/staffer for meeting with you.

✓ Share your personal story and explain why a specific issue is important to you. Explain the problem and how your "ask" can improve or solve it.

✓ Make a specific "ask". Give Congress the solution.

✓ You don’t have to be an expert on legislation. If you are asked a question that you are not sure how to answer, write it down and be sure to follow up.

✓ Respect the time of the Member, staffer and fellow advocates by limiting your story to no more than a minute or two. Typical meetings will last 15 minutes in total.

✓ Leave behind a one-pager with a summary of each issue as well as your contact information.

✓ It’s ok to ask for a photo with a Member of Congress. Make it a group shot and do it quickly.

✓ Report back to RDLA staff on how the meeting went.

✓ Follow up with a thank you note/ email reinforcing your asks.

Share Your Rare Experience

#RareDC2019   @RareAdvocates
Thank You to Our Rare Giving Sponsors

More than 650 patient advocates applied for travel stipends to attend Rare Disease Week on Capitol Hill. Thanks to our Rare Giving sponsors we were able to grant 105 stipends:

If you are a stipend recipient, please see the Foundation table set up near registration during the Legislative Conference to pick up your check. Please remember to bring your photo ID.
First and foremost, what is a hashtag?

- On Twitter and Facebook, the pound sign (#) turns any word or group of words that directly follow it into a searchable link. This allows you to organize content and track discussion topics based on those keywords. For instance, if you want to post about Rare Disease Week on Capitol Hill, you would include #RareDC2019 to join the conversation. You could then click the hashtag to see other posts on Rare Disease Week on Capitol Hill.

How do I ‘mention’ someone on twitter?

- Many Congressional offices have Twitter accounts to keep in touch with constituents. If you know your legislator’s Twitter handle, you can mention him or her in your post about #RareDC2019.
- If you don’t know your legislator’s Twitter handle, check his or her official website.

Before your meeting:

- Create a post tagging the Member’s office and the issue you will be talking about, for example:
- “We are excited to meet with @amyklobuchar for #RareDC2019 to talk about ways to bring more treatments to #Raredisease patients.”
- This is a good way to introduce yourself and your issue to the staff. This will add a face to the upcoming meeting and will help them remember you.

During the meeting:

- Ask to take a photo, preferably towards the end of the meeting.
- Write down any notes that might make for good tweets or quotes on your Facebook page.

After the meeting:

- Post your picture with a thank you note on Twitter and Facebook re-emphasizing the ask or any key points you discussed during the meeting, for example:
- “Thank you @amyklobuchar for joining the Rare Disease Congressional Caucus and supporting #Raredisease legislation! #RareDC2019”
Tuesday Young Adult Meetup

TUESDAY, FEBRUARY 26
6:00 PM

- Rare Hub
  1012 14th Street NW, Suite 500, Washington, DC 20005

- Join fellow young adults at the Rare Hub to enjoy light refreshments and discuss the day’s events with members of the Young Adult Representatives of the Rare Disease Legislative Advocates.

- The Young Adult Representatives of Rare Disease Legislative Advocates (YARR) are a highly motivated group of 16-30 year olds from the rare disease community. The main purpose of YARR is to instill confidence in the next generation of rare disease advocates and ensure young adults have a growing impact on public policy. YARR wants their peers to be supported during their transition from childhood to adulthood in regards to their advocacy journey. If you are interested in learning more or becoming a member of YARR, please visit: https://everylifefoundation.org/young-adultrepresentatives/

“There is no such thing as failure, there’s just giving up too soon.”
— Jonas Salk, developer of one of the first successful polio vaccines
Join Research!America and partners for a panel discussion and reception focused on progress on advancements in rare disease and the current development landscape. The program will provide expert discussion about the challenges and opportunities in rare disease research and the needs of the patient community. Approximately 50% of those affected by rare diseases are children, highlighting the need for robust research, as well as collaboration among patient and stakeholder communities to develop cures for these challenging diseases.

PROGRAM: 5:00 P.M. - 6:00 P.M.

Panel discussion on the challenges and opportunities in rare disease research:
- Moderator: Mary Woolley, President and CEO, Research!America
- Srini Ramanathan, PhD, Vice President of Developmental Sciences, Horizon Pharma
- Kurt Newman, MD, President and CEO of Children’s National Health System
- Shane DiGiovanna, Rare Disease Patient Advocate

RAREis Playlist presentation: My Georgia Home
- Song performed by Ralph Covert, based on a story written by ten-year-old Ja’cori about living with chronic granulomatous disease

NETWORKING RECEPTION TO FOLLOW: 6:00 P.M. - 7:30 P.M.

For questions, contact Sara Chang at schang@researchamerica.org
WEDNESDAY, FEBRUARY 27

• Location and time TBD

• Rare Disease Legislative Advocates with honorary co-hosts Representatives G.K. Butterfield (D-NC) and Gus Bilirakis (R-FL), Senators Roger Wicker (R-MS) and Amy Klobuchar (D-MN), the Co-Chairs of the Rare Disease Congressional Caucus invite you to a Rare Disease 101 Briefing.

Moderator

• Mark Dant, Chairman, EveryLife Foundation for Rare Diseases

Welcome

• Representative G.K. Butterfield
• Representative Gus Bilirakis

Rare Diseases

• Marshall Summar, MD, Director of RDI at Children’s National, and Chairman, National Organization for Rare Disorders

How NIH and FDA are Fighting Rare Diseases

• Francis Collins, MD, PhD, Director, National Institutes of Health
• Janet Maynard, MD, Acting Director, Office of Orphan Products Development, Food and Drug Administration

Tools and Techniques for Diagnosing Rare Diseases

• Pilar Magoulas, MS, CGC, Baylor College of Medicine

Challenges for Caregivers

• Angie Rowe, Interim Executive Director, Global Genes
Wednesday
Rare Artist
Reception

WEDNESDAY, FEBRUARY 27
5:00 PM

- Rayburn House Office Building, Foyer, 1st Floor
  45 Independence Ave. SW, Washington, DC 20515

- You’re invited to join us for the 2019 Rare Artist Reception. Enjoy works of art by the Rare Disease community and complimentary food and drink.

- The Rare Artist Reception is free and open to the public, learn more at rareartist.org

#RareArtist #RareArtist
Thursday
Rare Disease Day at the National Institutes of Health

THURSDAY, FEBRUARY 28
8:30 AM – 4:00 PM

- National Institutes of Health
  Main Auditorium, Natcher Conference Center, Building 45
  9000 Rockville Pike, Bethesda, MD 20892

- Join the EveryLife team and other rare disease advocates at Rare Disease Day at the National Institutes of Health. This year’s event will feature interactive panel discussions on collective research models for rare diseases, patient registries, and rare cancer research initiatives.

- New this year will be a presentation of the first ever Zebbie award for the NCATS Rare Diseases are Not Rare! Challenge. Other highlights include posters and exhibits by rare disease groups and researchers as well as artwork, videos, and tours of the NIH Clinical Center.

Admission
- Admission is free, and the event is open to the public.
  Pre-registration available at https://ncats.nih.gov/rdd

Visitor Information
- For Location and Visitor Information, please visit:
  https://events-support.com/events/Rare_Disease_Day/page/1950
Rare Disease Legislative Advocates is a program of the EveryLife Foundation designed to support the advocacy of all rare disease patients and organizations. By growing the patient advocacy community and working collaboratively, we can amplify our voices to ensure rare disease patients are heard at the local, state, and federal government levels.

RDLA provides many resources to the rare disease community to support grassroots advocacy across the country.

**ADVOCACY EVENTS**

- Rare Disease Week on Capitol Hill
- Legislative Conference
- Rare Disease Congressional Caucus Briefings
- In-District Lobby Days
- RareVoice Awards
- Monthly Conference Calls and Webinars

**RESOURCES**

- Monthly Newsletter and Webinars
- Action Alert creation and support
- Congressional Scorecards at RareAdvocates.org
- Online Advocacy Tools at RareAdvocates.org
- Rare Hub in Washington, DC
- Consulting and Support on Policy and Strategies

**ONLINE ADVOCACY TOOLS AT RAREADVOCATES.ORG**

The RDLA website and staff are available to support your advocacy efforts. We can help patients and organizations create advocacy campaigns for policies and legislation including creating action alerts.

An action alert is a specific action to take on a current policy issue. A well-crafted action alert is a powerful way to engage Congress and make your message heard by policymakers.

You can create an action alert to activate advocates to:

- Call their Members of Congress
- Email their Members of Congress
- Sign on to community sign on letters

Contact the RDLA staff at (202) 697-7273 for assistance.
A community work space created to improve cross-disease collaboration and legislative advocacy.

Join us in our newly remodeled office in the heart of Washington, DC. Our affordable shared office space fosters alliances and innovation among rare disease organizations to amplify the community’s voice on Capitol Hill.

For more information, contact:
Lauren Grinnals, Rare Hub Manager
LGrinnals@everylifefoundation.org

1012 14TH STREET NW, SUITE 500, WASHINGTON, D.C. 20005
(202) 697- RARE       RAREHUBDC.ORG

NEWBORN SCREENING BOOTCAMP

Empowering Advocates to Advance Life-Saving Screening

April 6, 2019 ● Chicago, Illinois

Join us the Saturday before the Association of Public Health Laboratories' Newborn Screening and Genetic Testing Symposium for a full-day advocacy bootcamp!

Hear from experts on how to advance newborn screening for your disease. Learn how to build coalitions, develop and validate your diagnostic assay, create a state pilot program and nominate your condition for the federal Recommended Uniform Screening Panel (RUSP). Travel stipends are available. Learn more at www.RareScreening.org.

Thank you to our sponsors:
Top 4 reasons why you should attend Rare on the Road:

**Learn**
how to tell your rare disease story

**Recognize**
how you can impact public policy and help save lives

**Connect**
with other patient advocates

**Discover**
how to get engaged on social media. *Featuring Mayo Clinic social media experts!*

We hope to see you on tour!
A limited number of $150 travel stipends are available.

For more information, please contact:
Lisa Schill - Lschill@everylifefoundation.org
Meredith Cagle - Meredithc@globalgenes.org

#RAREontheRoad • RAREtour.org
**Congressional Terms**

**Bill Sponsor** – A Representative or Senator who introduces a bill.

**Bill Cosponsor** – A Representative or Senator who formally signs on to support a bill. Only the first-named Member is the sponsor, all others are cosponsors, even those whose names appeared on the measure at the time it was submitted.

**Bicameral bill** – A bill that has been introduced in both the House and Senate.

**Bipartisan bill** – A bill that has at least one cosponsor from both parties.

**Congressional Budget Office (CBO)** – Agency within the legislative branch that produces independent analyses of budgetary and economic issues to support the Congressional process. Often calculates the cost or savings from enacting a specific bill. This is referred to as a “score”.

**Committee** – A panel with members from the House or Senate tasked with conducting hearings, examining and developing legislation, and conducting oversight.

- The Senate and House have separate versions of each committee, but occasionally a joint committee is made of members from both chambers. The Energy and Commerce Committee, Ways and Means Committee, and Appropriations Committee in the House and Health, Education, Labor and Pensions Committee (HELP), Finance Committee, and Appropriations Committee in the Senate have most of the jurisdiction over healthcare issues.

**Subcommittee** – A subpanel of a committee with a more specific jurisdiction. For example, the House Energy and Commerce Committee has a Health Subcommittee.

**Chair** – The member of the majority party on a committee or subcommittee who has formal responsibility over the panel’s agenda and resources, presides at its meetings, and can, in some circumstances, act on the committee’s behalf.

**Ranking Member** – The most senior (though not necessarily the longest-serving) member of the minority party on a committee or subcommittee. The ranking member typically oversees minority committee staff and may coordinate involvement of the minority party members in committee activities.

**Passed** – When a bill is approved in one chamber by a majority vote (most legislation requires a 60-vote majority in the Senate).

**Enacted** – When a bill is passed by both chambers and signed into law by the President.

**Hearing** – A formal meeting of a congressional committee (or subcommittee) to gather information from witnesses for use in its activities.

**Markup** – Meeting by a committee or subcommittee during which committee members offer, debate, and vote on amendments to a bill or other measure.
Government Agencies

**DEPARTMENT OF HEALTH AND HUMAN SERVICES (HHS)**
Secretary Alex Azar II, JD
A cabinet-level department of the U.S. federal government with the goal of protecting the health of all Americans and providing essential human services. This Department includes the below agencies, among others.

**NATIONAL INSTITUTES OF HEALTH (NIH)**
Director Francis Collins, MD, PhD
The nation’s medical research agency, tasked with making discoveries that improve health and save lives.

**FOOD AND DRUG ADMINISTRATION (FDA)**
Commissioner Scott Gottlieb, MD
Responsible for protecting the public health by ensuring the safety, efficacy, and security of human and veterinary drugs, biological products, and medical devices; and ensuring the safety of our nation’s food supply, cosmetics, and products that emit radiation.

**HEALTH RESOURCES AND SERVICES ADMINISTRATION (HRSA)**
Administrator George Sigounas, MS, PhD
The primary federal agency for improving access to health care services for people who are uninsured, isolated or medically vulnerable. This agency administers a number of newborn screening programs.

**CENTERS FOR DISEASE CONTROL AND PREVENTION (CDC)**
Director Robert Redfield, MD
Tasked with protecting the nation from health, safety and security threats, both foreign and in the U.S. Monitors reported disease and maintains information databases on prevalence, region, etc.

**CENTERS FOR MEDICARE AND MEDICAID SERVICES (CMS)**
Administrator Seema Verma, MPH
Administers healthcare/ reimbursement programs including Medicare, Medicaid, and the Children’s Health Insurance Program (CHIP).
WHERE OTHERS SEE COMPLEXITY, WE SEE HOPE FOR PATIENTS AND FAMILIES

At Mallinckrodt, our focus is to improve the lives of patients worldwide. Making a difference is what drives us every day as we work to develop innovative therapies and cutting-edge technologies for patients with severe and critical conditions.

We see challenges as opportunities to change lives. It is our passion. It is Mallinckrodt.

Learn more at Mallinckrodt.com.

Mallinckrodt is proud to be a sponsor of Rare Disease Week on Capitol Hill 2019.

GIVING PATIENTS A CHANCE AND PROVIDING HOPE IS WHAT DRIVES US.

Retrophin is proud to support the EveryLife Foundation’s Rare Disease Week on Capitol Hill 2019.

Retrophin.com @RetrophinRare #RareDC2019
Better Health, Brighter Future

There is more that we can do to help improve people’s lives. Driven by passion to realize this goal, Takeda has been providing society with innovative medicines since our foundation in 1781.

Today, we tackle diverse healthcare issues around the world, from prevention to life-long support and our ambition remains the same: to find new solutions that make a positive difference, and deliver better medicines that help as many people as we can, as soon as we can.

With our breadth of expertise and our collective wisdom and experience, Takeda will always be committed to improving the future of healthcare.

Takeda Pharmaceutical Company Limited
www.takeda.com

Our mission is clear. We are pioneers in neuroscience.


Founded in 1978, Biogen is one of the world’s oldest independent biotechnology companies.

www.biogen.com
Share
Your Rare Story

My name is


My rare disease legislative ask is


My or my loved one's biggest challenge living with a rare disease is


This legislative ask would help me or my loved one with a rare disease by


Remember

Practice your rare story and legislative ask to a friend or record it using your phone. Make necessary adjustments to make sure your story and ask grabs the attention of the legislative office you are visiting. Keep stories short - no more than a minute.
“Patients have been the driving force of drug development policy for the last 35 years and will continue to be critical in driving new policy. We need all patients to share their rare stories!”
- Julia Jenkins, EveryLife Foundation Executive Director