
YOUR VOICE MATTERS: HOW (AND WHY) TO TAKE ACTION ON PUBLIC POLICY



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WHAT IS PUBLIC POLICY - AND WHY DOES IT MATTER?

Public policy is a set of actions the government decides to take when approaching a problem that affects society as a group, rather than on an individual level.*

Public policy through **LEGISLATION** and **REGULATION** can help address some of the challenges faced by patients and their families as well as with the development and accessibility of new treatments.



Photo credit: Wikipedia

HOW PUBLIC POLICY CAN MAKE A DIFFERENCE: 21ST CENTURY CURES ACT

The 21st Century Cures Act was signed into law in December 2016 with support from hundreds of patient and medical specialty groups as well as academic researchers, industry trade organizations and other stakeholders that provided input throughout the multi-year process to determine how to best improve the discovery, development and delivery of new treatments to patients. It included:

- \$4.8 billion in new funding for the National Institutes of Health (NIH) and \$500 million in new funding for the Food and Drug Administration (FDA)
- Formal establishment of the Precision Medicine and Cancer Moonshot initiatives
- Funding for the establishment of a national neurological disease surveillance system coordinated by the Centers for Disease Control and Prevention (CDC)
- Improved biomarker qualification

HOW PUBLIC POLICY CAN MAKE A DIFFERENCE: ORPHAN DRUG ACT (ODA) OF 1983

- **ODA provides incentives to biopharma companies to develop treatments for rare disease including the Orphan Drug Tax Credit (for 50% of qualified clinical testing expenses) and waiver of Prescription Drug User Fees.**
- **It works!**
 - In the decade before the ODA, only 10 medicines were developed by industry for rare diseases.
 - Since 1983, more than 3,500 potential treatments have been designated as orphan drugs and more than 500 orphan therapies have been approved by FDA.
- **However, the Orphan Drug Tax Credit was cut in half by the Tax Cuts and Job Acts which was signed into law in December 2017.**
 - The House bill cut the Orphan Drug Tax Credit completely and the Senate bill reduced it to 27.5%.
 - The National Organization for Rare Disorders (NORD) led efforts to defend the Orphan Drug Tax Credit which are credited with saving it from elimination.

LESSONS LEARNED

- **Ongoing Education of Congress is CRITICAL!**
 - **Members of Congress need to hear from residents of their community** about their unmet needs and how they are impacted by rare disease. Meeting patients, families and researchers makes it personal.
 - **Building relationships with Congressional staff is also important.** They are the ones researching issues, advising the Members, signing Members on to legislation and sometimes advising their peers in other offices.
- **Numbers Matter**
 - **We need to get more patients, caregivers, researchers and other allies engaged in public policy in each Congressional district.** The number of calls and emails on an issue can determine how a Member votes.
- **In addition to championing legislation that would benefit us, we need to actively oppose legislation that would be harmful to the rare disease community.**

“Don’t get intimidated.
Get started!”

-Ron Bartek, Co-Founder and President of the Friedreich’s Ataxia
Research Alliance (FARA)

ESTABLISH A RELATIONSHIP WITH YOUR ELECTED OFFICIALS AND THEIR STAFF

- Call, email AND meet with your U.S. Representative and Senators on a regular basis.
 - Let them know rare disease issues are important to you, and why.
 - Thank them for cosponsoring and/or voting for legislation you support.
 - Attend their town halls and other events.
- Developing a relationship with their staff is equally important.



There are rare disease organizations to help you with this effort!

GET INVOLVED: RARE DISEASE LEGISLATIVE ADVOCATES (RDLA)

- Educates patient advocates about how public policy impacts availability and access to treatments.
- Provides resources to patients, caregivers, physicians and others so they can be effective legislative advocates.
- Provides an online advocacy center and legislative clearinghouse for rare disease legislation at the state and federal level.
- Builds awareness on Capitol Hill and ensures Congress hears directly from patients and others in the rare community.



All RDLA resources and events are FREE for patients, caregivers and other advocates!

RARE DISEASE LEGISLATIVE ADVOCATES (RDLA)

Monthly Webinars

- Any individual or organization is welcome to contribute agenda items, from pending legislation of interest to the rare disease community to new resources such as reports and policy papers.
- Questions from attendees are welcomed.
- Webinars are available online afterwards as a resource.



Monthly Newsletters

- Update on policy news
- Action alerts
- Upcoming events including Congressional briefings and public workshops hosted by the Food and Drug Administration (FDA) and National Institutes of Health (NIH)

<https://everylifefoundation.org/rare-advocates/>

RARE DISEASE LEGISLATIVE ADVOCATES (RDLA)



Brings 900 advocates from across the country to Washington, DC to learn how to build effective relationships with Congress and partner with federal agencies



Empowers advocates to meet with their Members of Congress in their local offices (or virtually) during the summer district work period



Hosts quarterly briefings to educate Members of Congress and their staff on issues of importance to the rare disease community



Recognizes advocates, legislators and Congressional aides making a difference in DC and state capitols



Instills confidence in the next generation of rare disease advocates (ages 16 to 30) and facilitates peer support in the transition to adult advocacy

GET INVOLVED: NATIONAL ORGANIZATION FOR RARE DISORDERS

- Provides robust background on public policies to:
 - Advance basic and translational research
 - Strengthen the drug development process
 - Ensure access to affordable treatment
- Issues action alerts to encourage advocates to email or call Congress



<https://rarediseases.org/>

GET INVOLVED: NATIONAL ORGANIZATION FOR RARE DISORDERS

NORD hosts the **RareAction Network** which focuses on policy issues at the state level. It is free to join and enables advocates to:

- Connect with other patients, caregivers and stakeholders within their state
- Participate in regional and local events as well as virtual discussions to connect, learn and address policy issues
- Develop relationships with key decision-makers and opinion leaders
- Share their stories to help raise awareness of a specific rare disease and rare diseases in general
- Receive news, information and action alerts



<http://rareaction.org/get-involved/join-rare-action/>



| Subject | Grade |
|----------------------------------|------------|
| Medical Nutrition | F |
| Rx Out-of-Pocket Protections | F |
| Newborn Screening (NBS) | B |
| Individual Insurance Protections | F |
| Medicaid Eligibility | F |
| Medicaid 1115 Waivers | Incomplete |
| Home & Community-Based Services | B |
| Step Therapy | F |
| Rare Disease Advisory Council | Incomplete |

ENGAGING WITH CONGRESS: FIRST STEPS

- Look up your U.S. Representative and Senators based on home address at <https://www.govtrack.us/congress/members>.
 - Follow them on Twitter and/or Facebook
 - Visit their official websites and sign-up for their email newsletters

Each state elects two senators to the United States Senate for staggered 6-year terms. Senators represent the entire state. Pennsylvania's senators are:



Robert "Bob" Casey Jr.

Senior Senator for Pennsylvania
Since Jan 4, 2007 (next election in 2024)
Democrat

[@SenBobCasey](#) · [Official Website](#) [↗](#) · [202-224-6324](tel:202-224-6324)

[View Legislative Profile & Get Alerts »](#)



Patrick "Pat" Toomey

Junior Senator for Pennsylvania
Since Jan 5, 2011 (next election in 2022)
Republican

[@SenToomey](#) · [Official Website](#) [↗](#) · [202-224-4254](tel:202-224-4254)

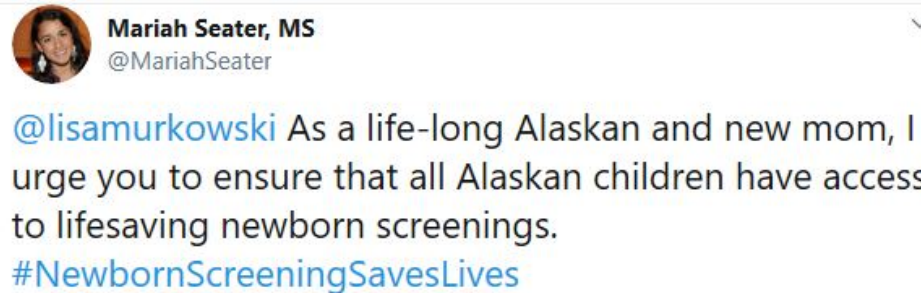
[View Legislative Profile & Get Alerts »](#)

- Sign up for Rare Disease Legislative Advocates (RDLA) emails for monthly newsletters as well as invitations to monthly webinars and other FREE events:
<https://everylifefoundation.org/newsletter-signup/>
- Sign up for National Organization for Rare Disorders (NORD) emails:
<https://www.tfaforms.com/4728157>

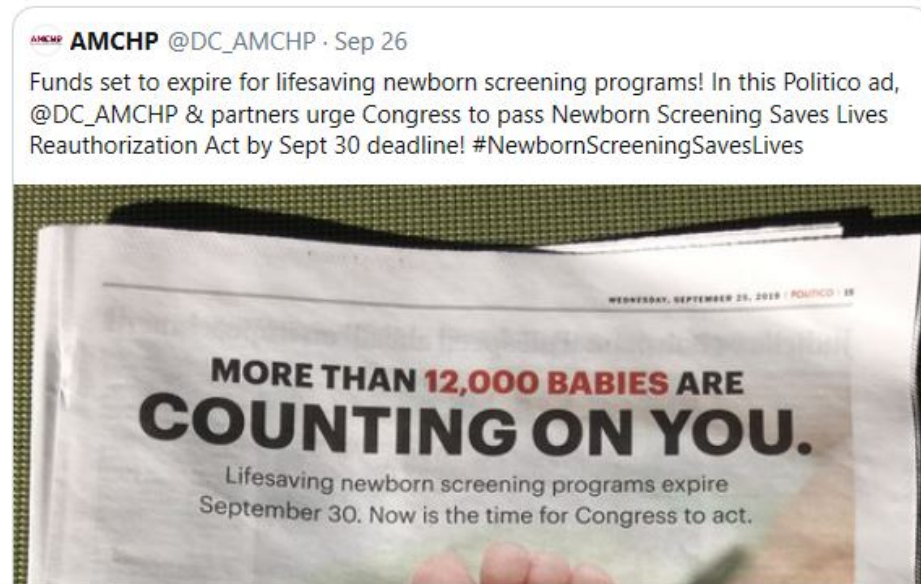
USING SOCIAL MEDIA FOR ADVOCACY

Use your Twitter account to ask your followers to take action.


Retweet action alerts from organizations that you support in order to expand the number of people who see it and participate.



Mariah Seater, MS @MariahSeater
@lisamurkowski As a life-long Alaskan and new mom, I urge you to ensure that all Alaskan children have access to lifesaving newborn screenings.
[#NewbornScreeningSavesLives](#)



AMCHP @DC_AMCHP · Sep 26
Funds set to expire for lifesaving newborn screening programs! In this Politico ad, @DC_AMCHP & partners urge Congress to pass Newborn Screening Saves Lives Reauthorization Act by Sept 30 deadline! [#NewbornScreeningSavesLives](#)



WEDNESDAY, SEPTEMBER 25, 2019 / POLITICO 19
MORE THAN 12,000 BABIES ARE COUNTING ON YOU.
Lifesaving newborn screening programs expire September 30. Now is the time for Congress to act.



Leah Trotter @metabolic_mama
Are you following @EveryLifeOrg? There are now 35 [#Cosponsors](#) for the CRITICAL [#NewbornScreening](#) saves lives reauthorization act! !! Thanks to all [#advocates](#) for working hard to show congress how important this legislation is to our community!
[#EpilepsyAwareness](#) [#RIPCameronBoyce](#)



EveryLife Foundation @EveryLifeOrg · Jul 6
Thanks to [#rareisease](#) advocacy efforts, there are NOW 35 total cosponsors for [#NewbornScreening](#) Saves Lives Reauthorization Act of 2019 (H.R. 2507)! Together we CAN [#changepolicy](#) & [#savelives](#)! THANKS @HerreraBeutler for cosponsoring this lifesaving legislation! [#rareadvocate](#)



USING SOCIAL MEDIA FOR ADVOCACY

You can also increase the number of people who participate in action alerts by sharing them on Facebook and/or LinkedIn.

Rare disease advocacy organizations rely on help from individual advocates who not only take action, but call on their friends and family to do so as well.

National Organization for Rare Disorders, Inc. (NORD)
November 15, 2016 · 🌐

TODAY! Please join us, EveryLife Foundation for Rare Diseases and Global Genes for a united #RareDisease Day of Action in support of the 21st Century Cures Act (#CuresNow), a piece of legislation that is a game-changer for patients with rare diseases. A delay in this legislation is a delay in life-saving treatments and we need your support to make sure Congress gets this message loud and clear! Please take a moment to call your elected officials using the tool below. You can also share this post and your own social media posts to help spread the word! <http://bit.ly/2gcn3cn>

TIME IS RUNNING OUT
WE NEED #CURESNOW

SALSA3.SALSALABS.COM
CLICK HERE TO CALL CONGRESS
The rare disease community NEEDS #CuresNow! We must make sure...

👍❤️ 101 9 Comments 148 Shares

USING SOCIAL MEDIA FOR ADVOCACY

Encourage your legislators to support or oppose specific legislation, and thank them when they do. Use their official social media handles so their offices receive a notification.



Thank legislators (or their staff) for meeting with you, especially if they agree to something you requested such as supporting legislation. Take a photo to include in your post, if possible.

USING INSTAGRAM

It can be more difficult to spur others to action on Instagram since hyperlinks cannot be included in posts but you can raise awareness of an issue as part of an effort to affect change.

Klippel-Feil Syndrome Freedom

“21st Century Cures is the innovation game-changer that patients, their loved ones, and the nation’s researchers and scientists so desperately need.”
-Rep. Fred Upton & Rep. Diana DeGette

Friday or Monday

Please call your Senators. (again)

Their vote makes or breaks the

21st Century Cures Act HR 6.

Thank you!

rosiegal8 • Following

rosiegal8 Make a few quick calls to your Senators today and Monday. Say “Please ask the Senator to support 21st-century-cures early next week, and vote ‘yes’ to #CuresNow!” This will help everyone who ever has a health issue - this includes you!
@senatorkirk @durbincampaign @repfredupton @globalgenes @energyandcommerce @adam_kinzinger #raredisease

351 likes
DECEMBER 2, 2016

Add a comment... Post

susangkomen • Follow

susangkomen “Step therapy, sometimes called “fail first”, requires a patient to first try a preferred drug (often generic, cheaper alternative) prior to receiving coverage for the drug the doctor prescribed. #KomenAdvocacy is working to end this harmful policy in your state! You can help by becoming an advocate. Tap the #linkinbio to sign up to receive alerts when we need your voice.

#advocacy #yourvoicematters #healthcare #womenshealth #menshealth #breastcancer #yourhealthmatters #advocate #advocacymatters #advocacyinaction #cancer #advocateforchange

351 likes
NOVEMBER 19

Add a comment... Post

Patients should never be forced to have a treatment fail them.

Stop step therapy.

FIRST STEPS TO USE SOCIAL MEDIA FOR ADVOCACY

- **Find and follow your U.S. Senators on Twitter:**
<https://twitter.com/cspan/lists/senators/members?lang=en>
- **Find and follow your U.S. Representative on Twitter:**
<https://twitter.com/cspan/lists/u-s-representatives/members?lang=en>
- **Check out the tips in this RDLA blogpost on using social media to engage policymakers:**
<http://rareadvocates.org/utilizing-facebook-twitter-engage-policymakers-key-part-advocacy/>
- **Follow the Cutaneous Lymphoma Foundation on Facebook and consider sharing action alerts with a personal note about why it is important to you:**
<https://www.facebook.com/clfoundation>



RESOURCES

Global Genes doesn't focus on public policy, but they provide free resources to patients, caregivers and advocacy organizations. They have FREE toolkits on a variety of relevant topics including:

- *Building Relationships with Your Representatives*
- *Bringing Rare Disease to Capitol Hill: Advocating for Your Cause*
- *Speak Easy: A Guide to Public Speaking* (which includes tips on media outreach and crafting an elevator speech, both of which can be helpful in advocacy)
- *Using Storytelling to Raise Awareness of Your Rare Disease* (which can help you craft what you want to say about your experience living with a rare disease as a patient or caregiver)

THANK YOU!



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