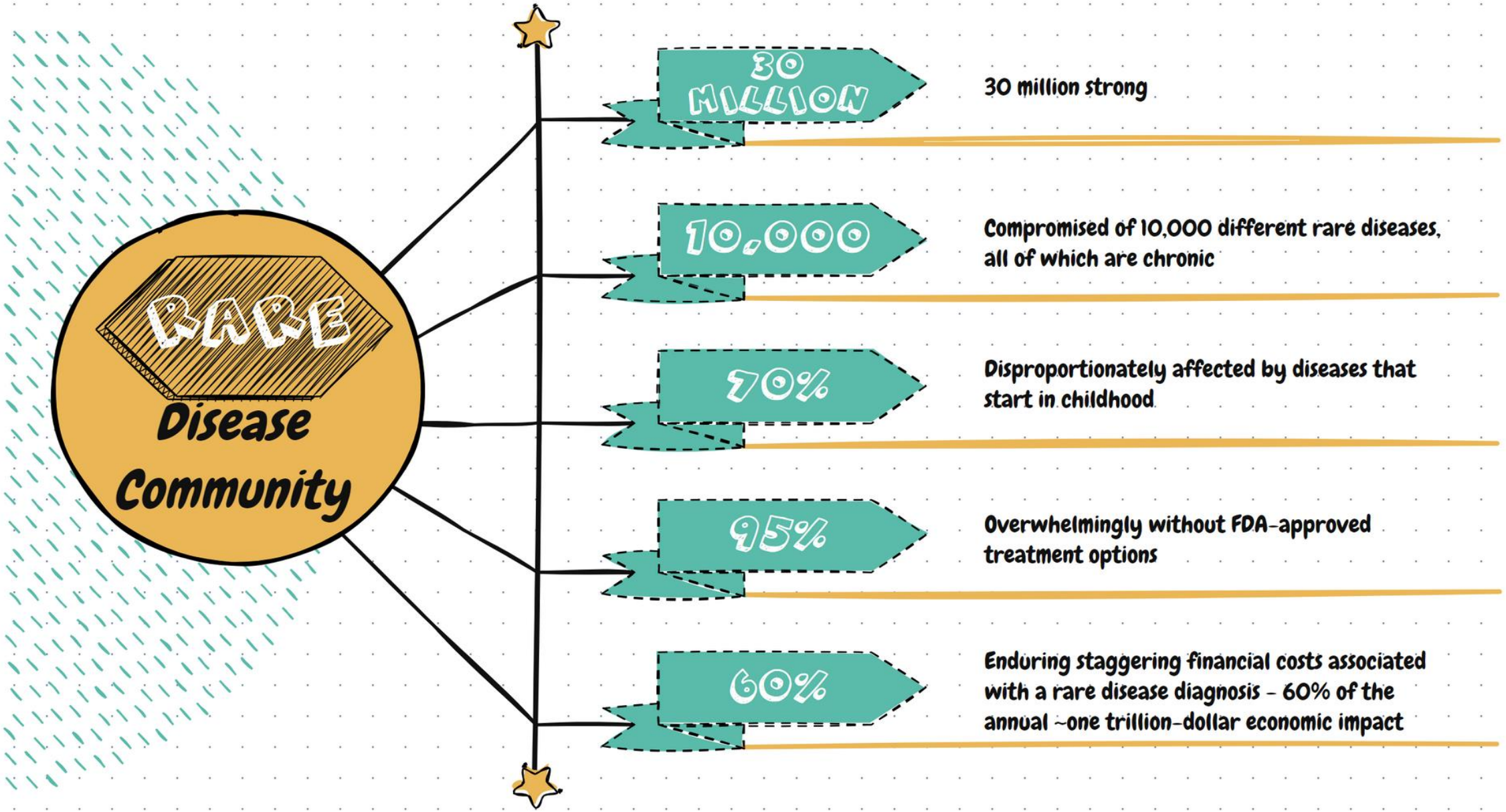
Stronger Together –
Rare Disease Federal and State Policy & Advocacy

Annie Kennedy
Chief of Policy, Advocacy & Patient Engagement
EveryLife Foundation for Rare Diseases


RARE
Disease
Community
30 MILLION
30 million strong
10,000
Compromised of 10,000 different rare diseases, all of which are chronic
70%
Disproportionately affected by diseases that start in childhood
95%
Overwhelmingly without FDA-approved treatment options
60%
Enduring staggering financial costs associated with a rare disease diagnosis – 60% of the annual ~one trillion-dollar economic impact

EVERYLIFE FOUNDATION FOR RARE DISEASES

*The EveryLife Foundation for Rare Diseases is a 501(c)(3) nonprofit, nonpartisan organization dedicated to **empowering the rare disease patient community** to advocate for impactful, science-driven legislation and policy that advances the equitable development of and access to lifesaving diagnoses, treatments and cures.*

COMMUNITY CONGRESS
POWERED BY THE EVERYLIFE FOUNDATION

RDLA
Rare Disease Legislative Advocates
POWERED BY THE EVERYLIFE FOUNDATION

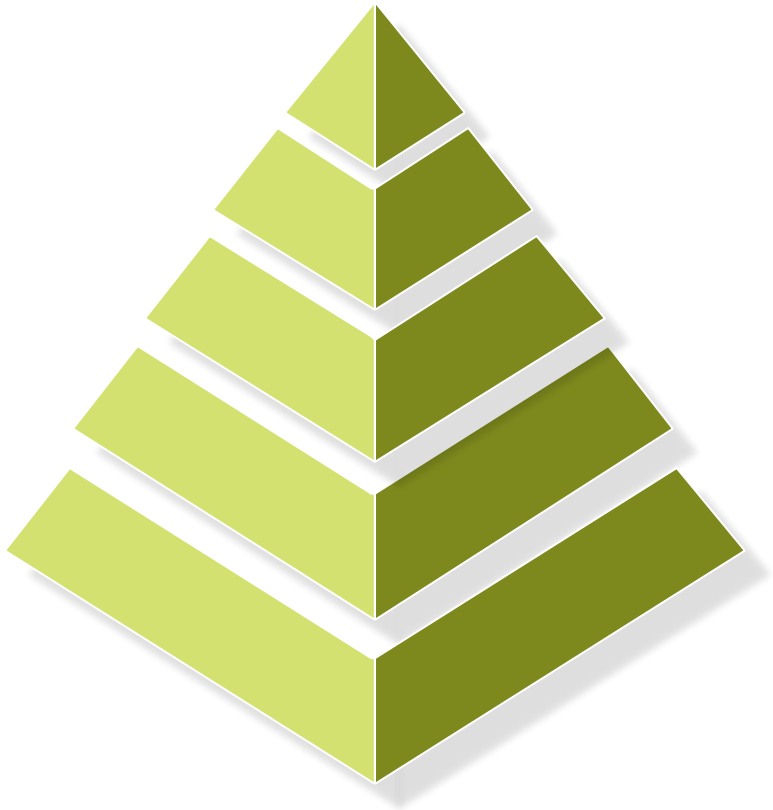
RARE DISEASE WEEK ON CAPITOL HILL

MONTHLY WEBINARS

RARE ACROSS AMERICA

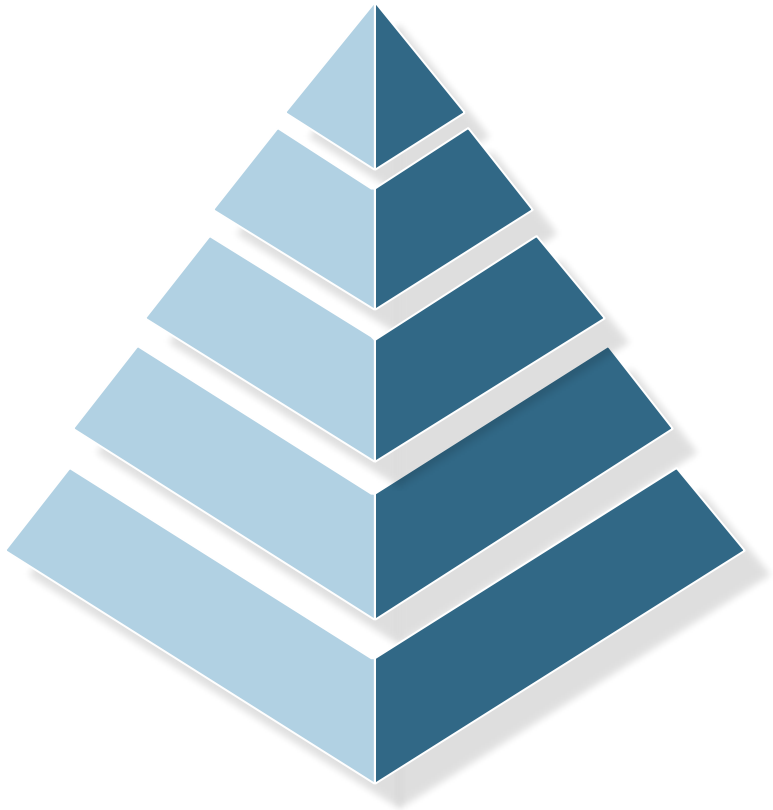
EveryLife Policy Priority Areas

Everything we lead, support, monitor, and engage in maps to one or more of these 3 policy areas



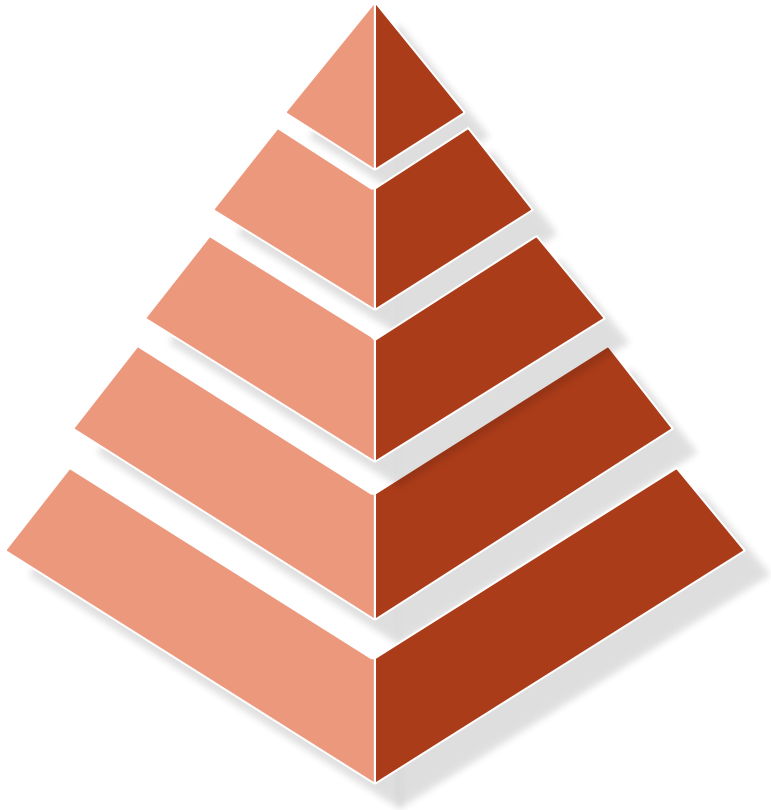
Elimination of Dx Odyssey

Reduction & elimination of the diagnostic odyssey.



Therapy Development

Development of expedient pathways for discovery and approval for effective therapies for rare diseases



Access & Value

Access to approved therapies

Rare Disease Legislative Advocates (RDLA) Programs

RARE DISEASE WEEK ON CAPITOL HILL

MONTHLY WEBINARS

Rare Advocacy Learning
EMPOWERING EVERY VOICE

RARE ACROSS AMERICA

RARE DISEASE CONGRESSIONAL CAUCUS

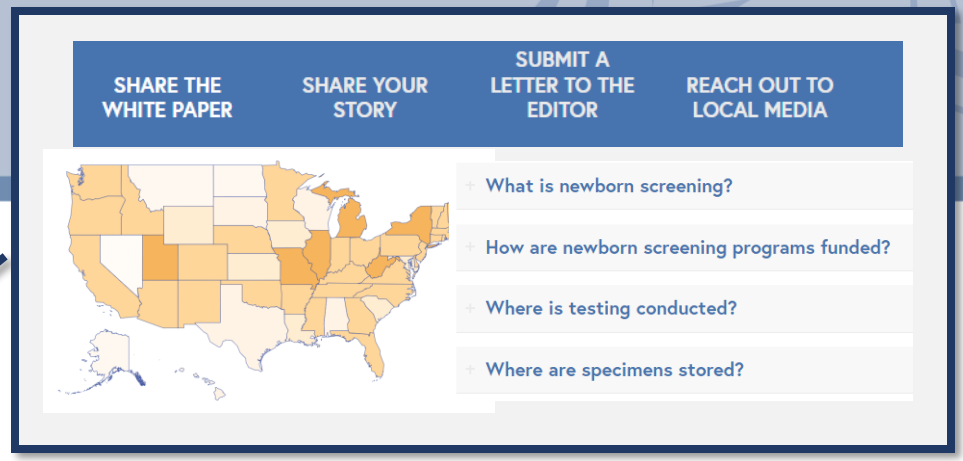
RDLA
Rare Disease Legislative Advocates
Youth & Teen Advocacy Day
POWERED BY THE EVERYLIFE FOUNDATION

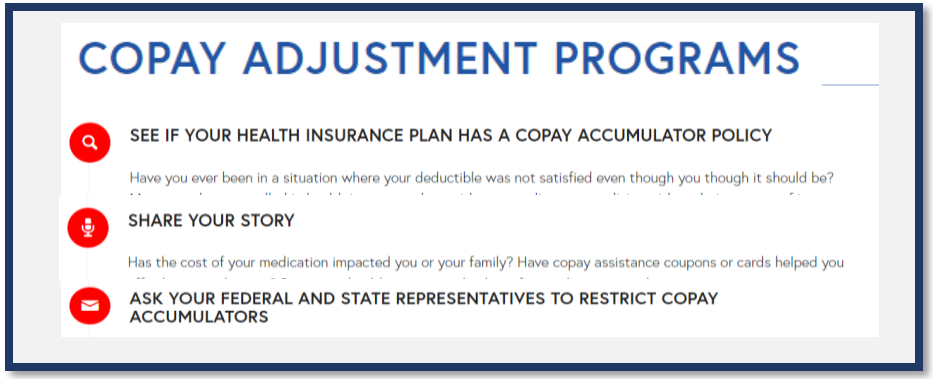
RAREVOICE Awards

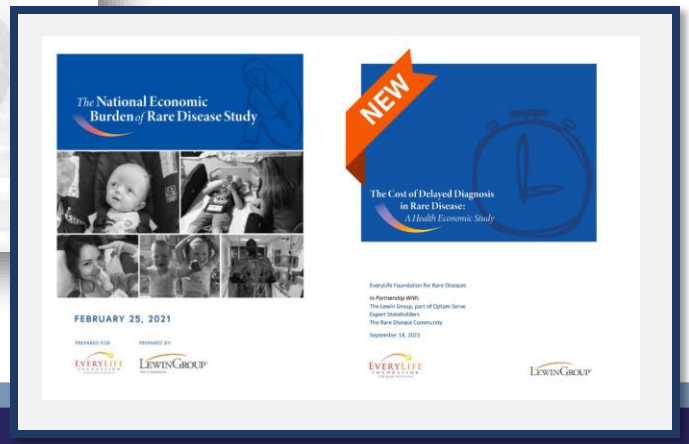
Rare Giving
LIFTING EVERY VOICE

EveryLife Policy Resources

www.everylifefoundation.org







2025 FEDERAL LEGISLATIVE POLICY PRIORITIES

EVERYLIFE FOUNDATION FOR RARE DISEASES | COMMUNITY CONGRESS

Lead

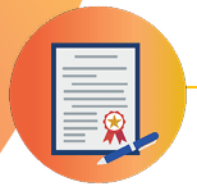
- **Newborn Screening: Federal Reauthorization & Modernization Efforts**
- **Protect & Strengthen Accelerated Approval Pathway**
- **Orphan Drug Incentives**
  - → Rare Pediatric Disease PRV Reauthorization (Give Kids a Chance Act H.R 1262/ S. 932) ▸▸
  - → Protect & Restore Orphan Drug Tax Credit (Cameron's Law H.R 1414) ▸
- **Appropriations**
  - → FDA Rare Disease Innovation Hub
  - → Federal Biomedical Research and Public Health Programs

Engage

- BENEFIT Act ○
- Scientific EXPERT Act (H.R 1532/ S. 822) ▸▸
- **Medicaid Coverage**
- **Genetic Testing Coverage**

Support

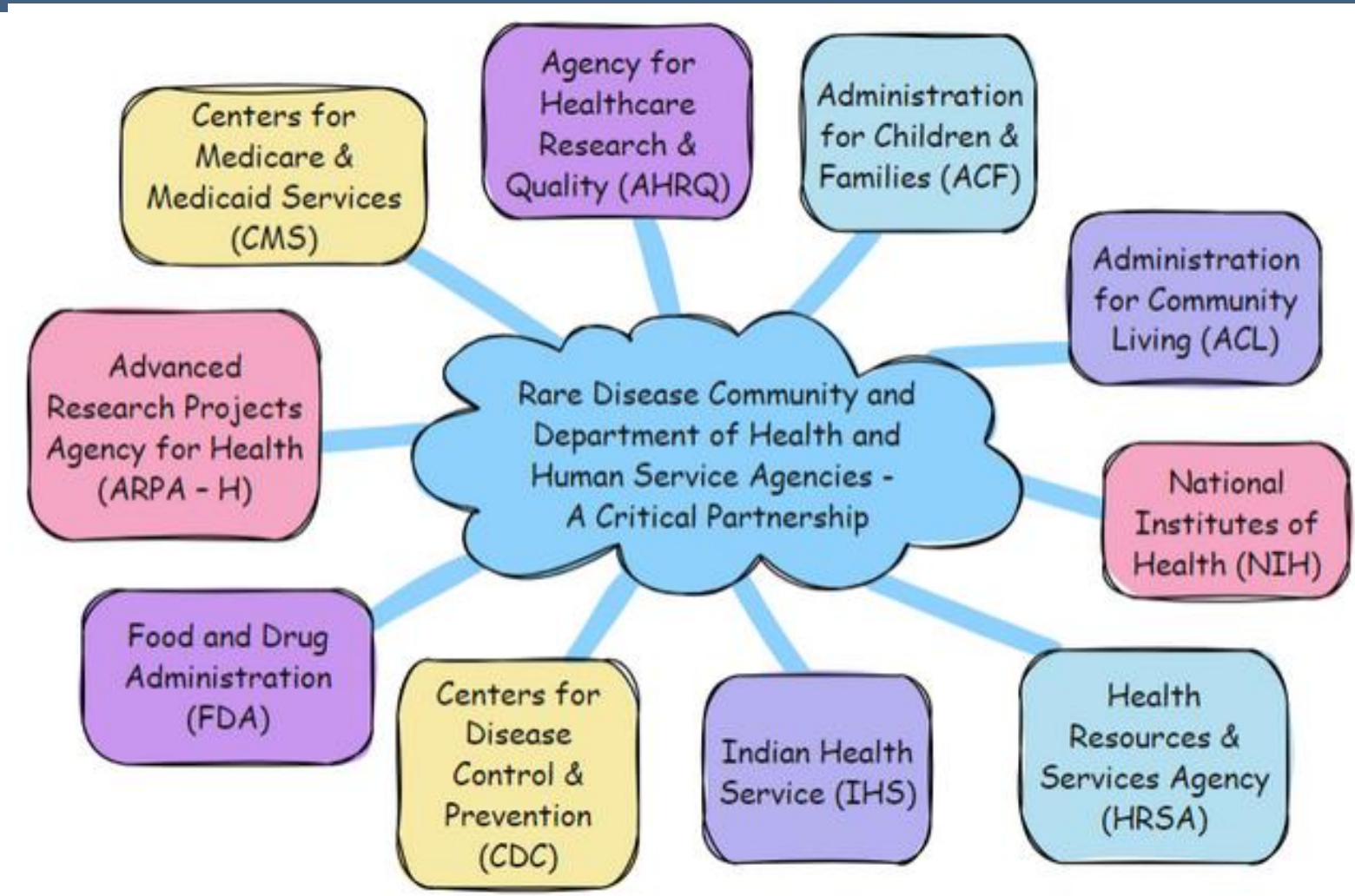
- HELP Copays (S. 864) ▸
- Accelerating Kids Access to Care Act (H.R 1509/ S. 752) ▸▸
- PROTECT Rare Act ○
- PASTEUR Act ○
- MVP Act ○
- Joe Fiandra Access to Home Infusion Act ○
- RARE Act ○
- Safe Step Act ○
- MINI Act ○
- SOAR Act ○
- ENABLE Act (H.R 1436/ S.627) ▸



Monitor

- **Clinical Trial Diversity Policies**
- **Drug Pricing Policies**
- **Telehealth & Data Security Policies**
- **Prior Authorization Reform**
- **Laboratory Developed Test Regulation**
- **PBM Reform**
- **QALY Bans**
- **NIH Reform**
- **Alternative Funding Programs**

Status: Not yet Introduced ○ | Introduced in One Chamber ▸ | Introduced in Both Chambers ▸▸ | Advanced Out of Committee ➡ | Passed the House ✦ | Passed the Senate ✶ | Enacted ☑


Rare Disease Community and Department of Health and Human Service Agencies - A Critical Partnership
Agency for Healthcare Research & Quality (AHRQ)
Administration for Children & Families (ACF)
Centers for Medicare & Medicaid Services (CMS)
Administration for Community Living (ACL)
Advanced Research Projects Agency for Health (ARPA - H)
National Institutes of Health (NIH)
Food and Drug Administration (FDA)
Centers for Disease Control & Prevention (CDC)
Indian Health Service (IHS)
Health Resources & Services Agency (HRSA)

Reauthorize the Rare Pediatric Disease Priority Review Voucher Program

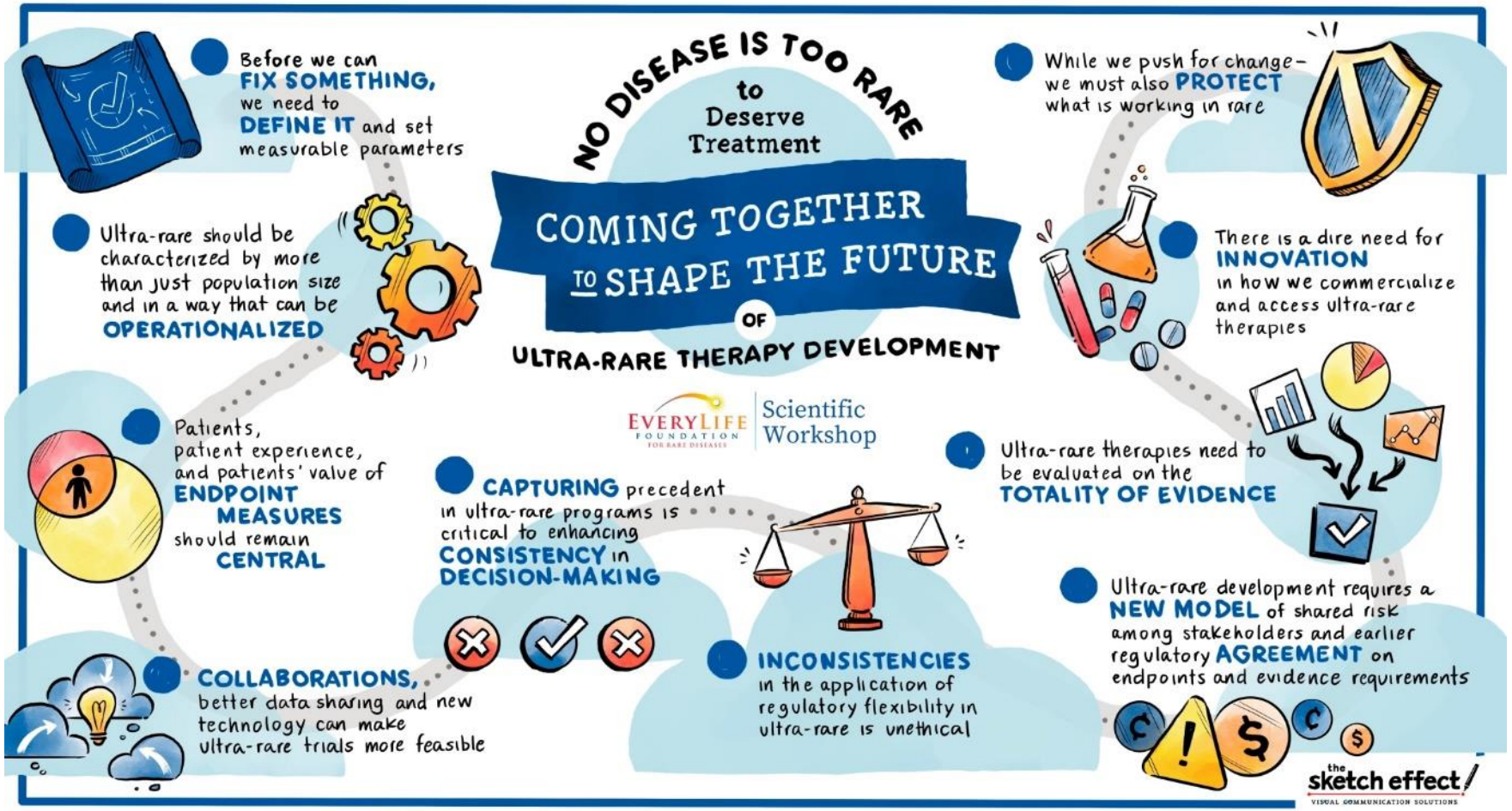
- Led to new treatments for 40 rare diseases
- No cost to the tax-payer
- Program expired on December 20, 2024, despite reauthorization from the House.

Support Successful Implementation of the Rare Disease Innovation Hub at the FDA

- Improve consistency and predictability of therapy approaches
- Building FDA regulatory science expertise in rare disease
- Advocating for resources for the RDIH

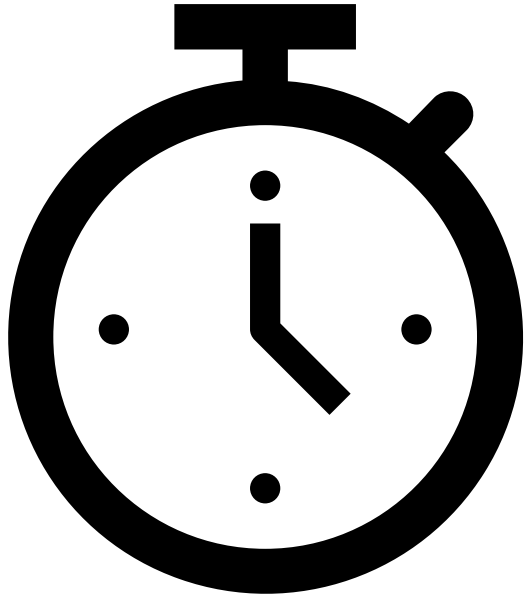
Developing Treatments for Ultra-Rare Diseases

- **Address concerns surrounding limited natural history data and costly clinical evaluations**
- **Enable pipelines for small populations to thrive**



Support policies that facilitate timely and affordable access to approved therapies

- Eliminated time and cost as patient access hurdles
- Ensure access to appropriate FDA-approved therapies without burdensome and medically inappropriate utilization management requirements
- Enable innovative payment models that incorporated meaningful community input



NATIONAL RARE DISEASE COMMUNITY PETITION

EVERYLIFE FOUNDATION FOR RARE DISEASES

More than 11,000 rare disease advocates from all over the country signed a petition **urging Members of Congress to encourage the President and the Secretary of Health and Human Services to support strong federal agency leadership, sustained biomedical research funding, and public health agency resources.**

The petition was delivered to every member of Congress on February 28 in honor of Rare Disease Day.

SCAN TO SIGN PETITION

2025 STATE POLICY PRIORITIES

EveryLife Foundation for Rare Diseases | Community Congress

Lead

Represents policies that the EveryLife Foundation will initiate and drive. Activity may include crafting strategy, conducting direct lobbying activities, activating advocates to support, and more.

- Newborn Screening RUSP Alignment

Engage

Represents policies that the EveryLife Foundation will drive in collaboration with other entities, coalitions, or organizations. Activity may include representing rare disease perspectives, crafting strategy, conducting direct lobbying activities, activating advocates to support, and more.

- Payer Decision-Making: Transparency, Accessibility, and Rare Disease Expert & Patient Representation

Support

Represents policies led by existing entities, coalitions, or organizations that the EveryLife Foundation will proactively act on. Activity may include joining sign-on letters, submitting testimony, engaging state legislatures, participating in joint meetings, or sharing opportunities to provide input with advocates.

- Access to Genetic Testing Services
- Copay Adjustment & Maximizer Programs
- Genetic Data Non-Discrimination
- Interstate Medical Licensure Compact
- Interstate Telehealth
- Prior Authorization Requirements

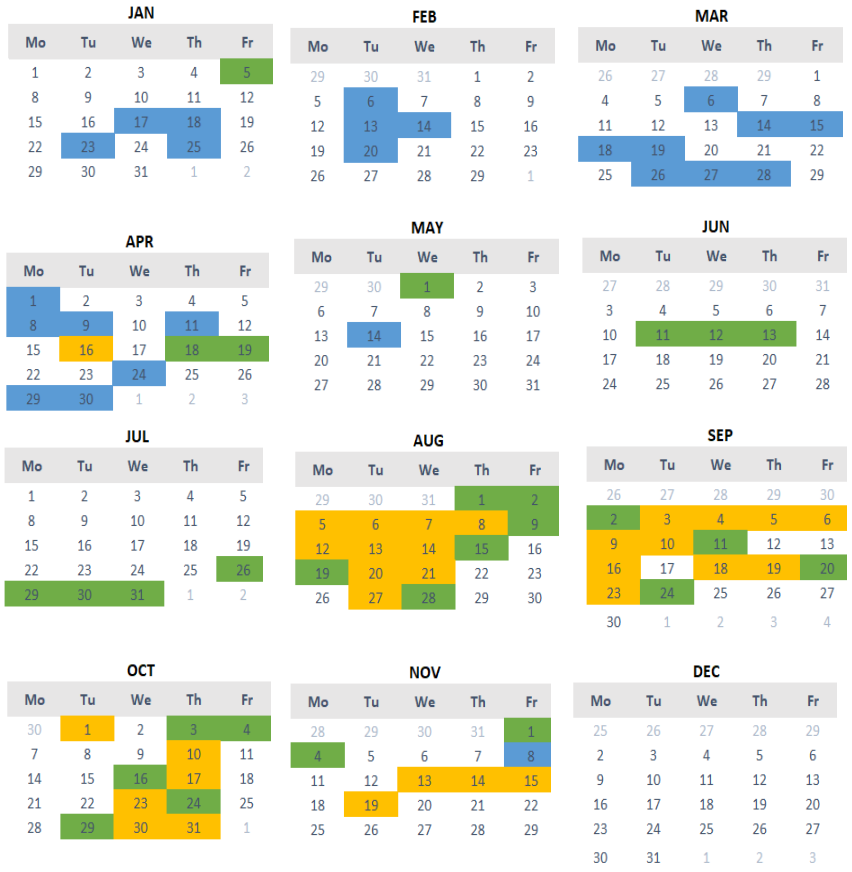
Monitor

Represents policies that are relevant to the rare disease community. The EveryLife Foundation will actively consider proposals from Community Congress members or changes in the policy landscape that necessitate further consideration or action.

- Access to Genetic Testing Services
- Access to Therapies Approved via Accelerated Approval
- Alternative Funding Programs
- Biomarker Testing
- Cost-sharing
- Intrastate Telehealth
- Medical Debt
- Medical Foods Equity
- Personal Data Privacy
- Prescription Drug Affordability Boards
- Rare Disease Advisory Councils
- Step Therapy
- Value Based Payments & Assessments

2024 - State Policy Activity

- Meeting w/ Legislator
- Submit Testimony/Letter
- Share Policy Resource w/Legislator



420
Bills Tracked

48
Meetings with Legislators

28
Testimonies or Letters Submitted

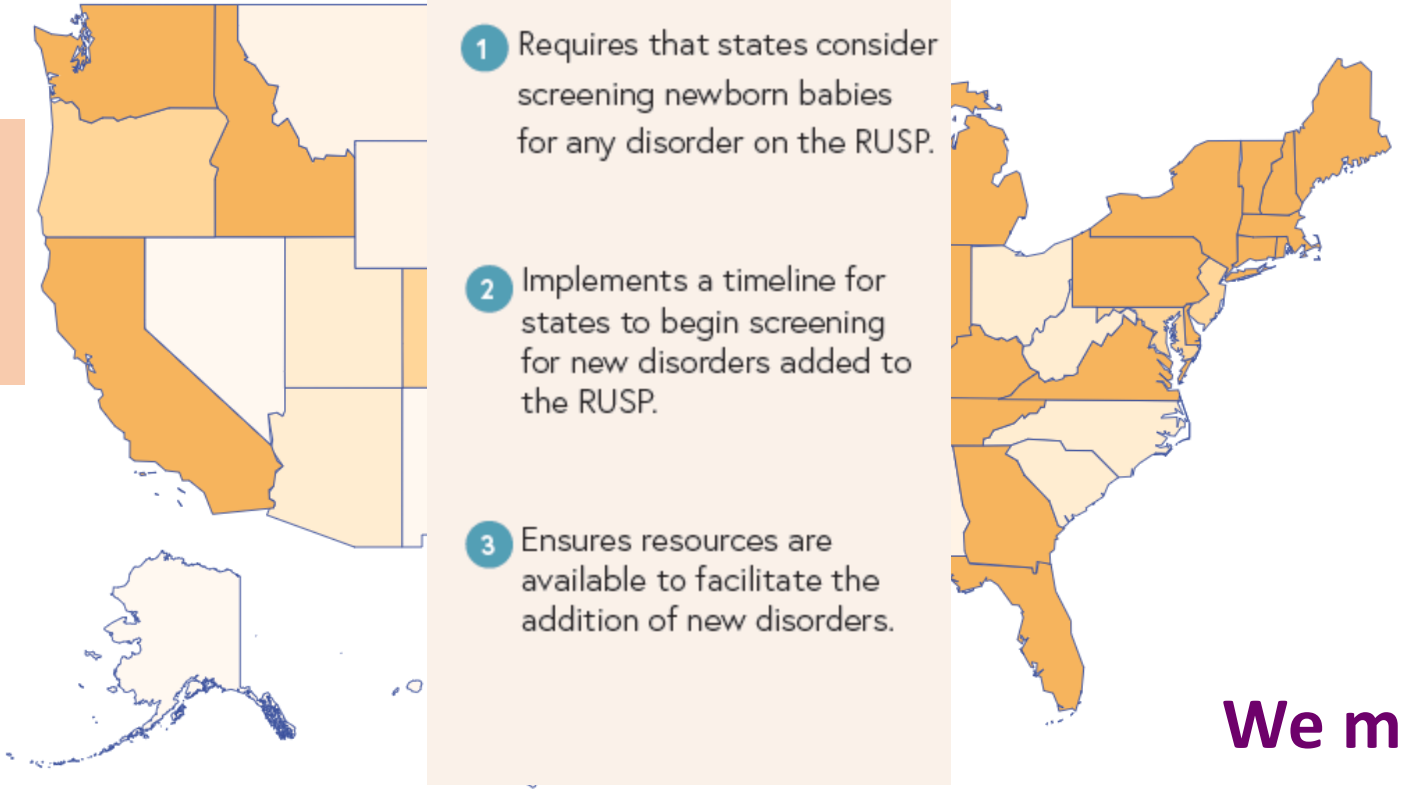
183
Legislators Contacted

An Example of Advocacy Activation in Action

State Newborn Screening Legislation

Programs vary widely b… …isparate health outcomes.

Some states screen for fewer than **30 diseases.**



What is RUSP Alignment Legislation?

1. Requires that states consider screening newborn babies for any disorder on the RUSP.
2. Implements a timeline for states to begin screening for new disorders added to the RUSP.
3. Ensures resources are available to facilitate the addition of new disorders.

Other states screen for more than **60 diseases.**

We must do better.

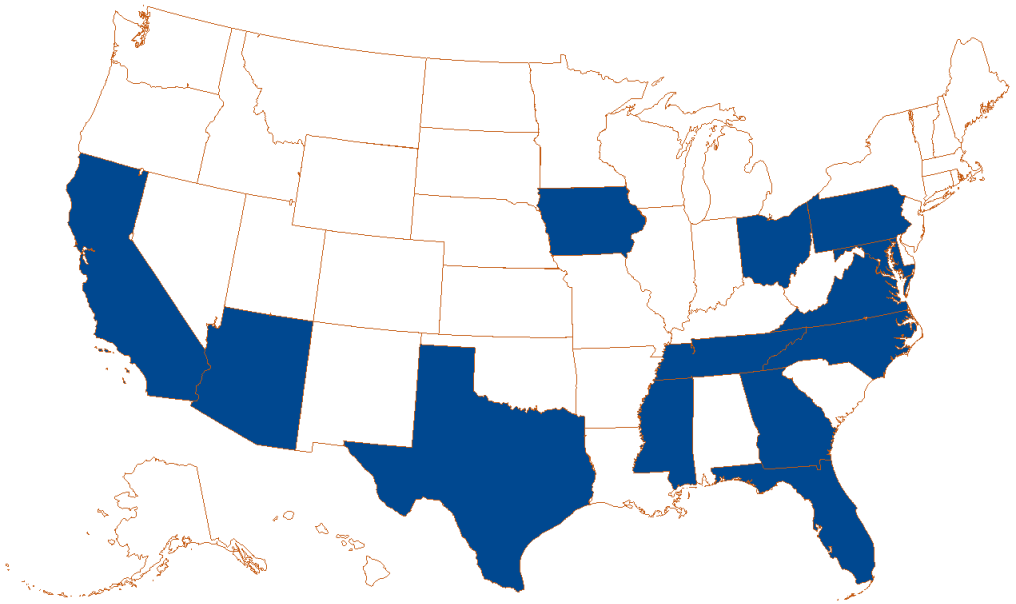
2025 State Policy Accomplishments

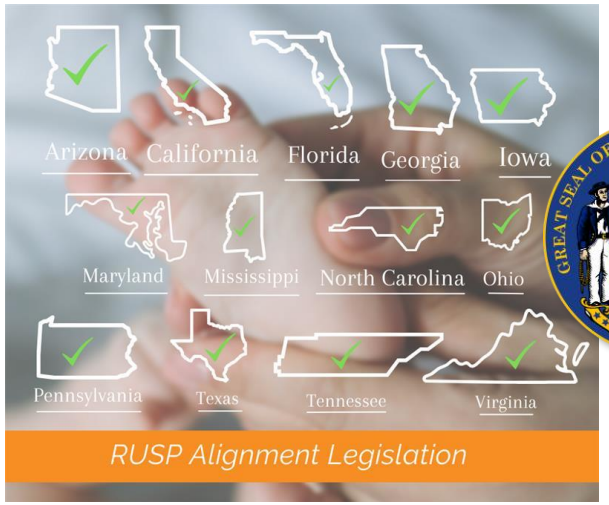
Virginia HB 1782- signed by the governor

- > 95,000 babies born in VA Annually
- > 300 annual diagnoses through newborn screening
- > 40 patient organizations supported

52% of babies are born in RUSP aligned states

- Virginia is the 13th state to become RUSP aligned
- RUSP alignment = timeline, funding, and screening language





Newborn Screening Across The States

The Recommended Uniform Screening Panel (RUSP)

- **A list of disorders** recommended for states to screen
- 37 recommended conditions

The Advisory Committee on Heritable Disorders

- **Provides RUSP recommendations** to the Secretary of Health and Human Services.
- Doctors, scientists, parents, ethicists, and researchers

Evidence Development By Patient Groups

RUSP Nomination to Secretary's Advisory Committee on Heritable Disorders in Newborns & Children

If package not accepted, continue to build evidence

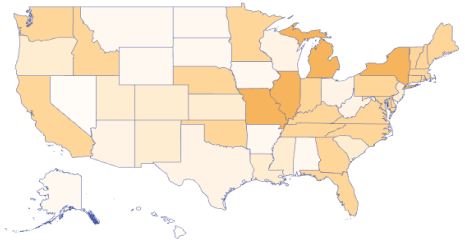
Nomination package review by Evidence Review Committee

Review by Advisory Committee

If not approved, continue to build evidence

Approved!

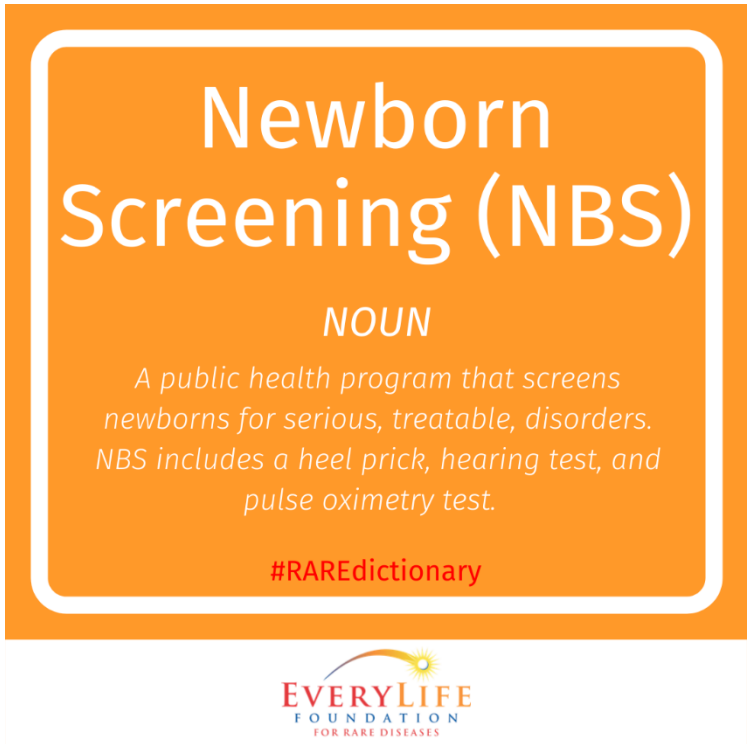
Await HHS Secretary Approval



Restore & Modernize our Nation's Newborn Screening Ecosystem

- Restore & Update Mechanism for Evidence Review of new Conditions to RUSP

- New policies are needed that address:
  - The role of genetic testing
  - increased investment in public health infrastructure, education, and data systems to support a more effective and equitable newborn screening program



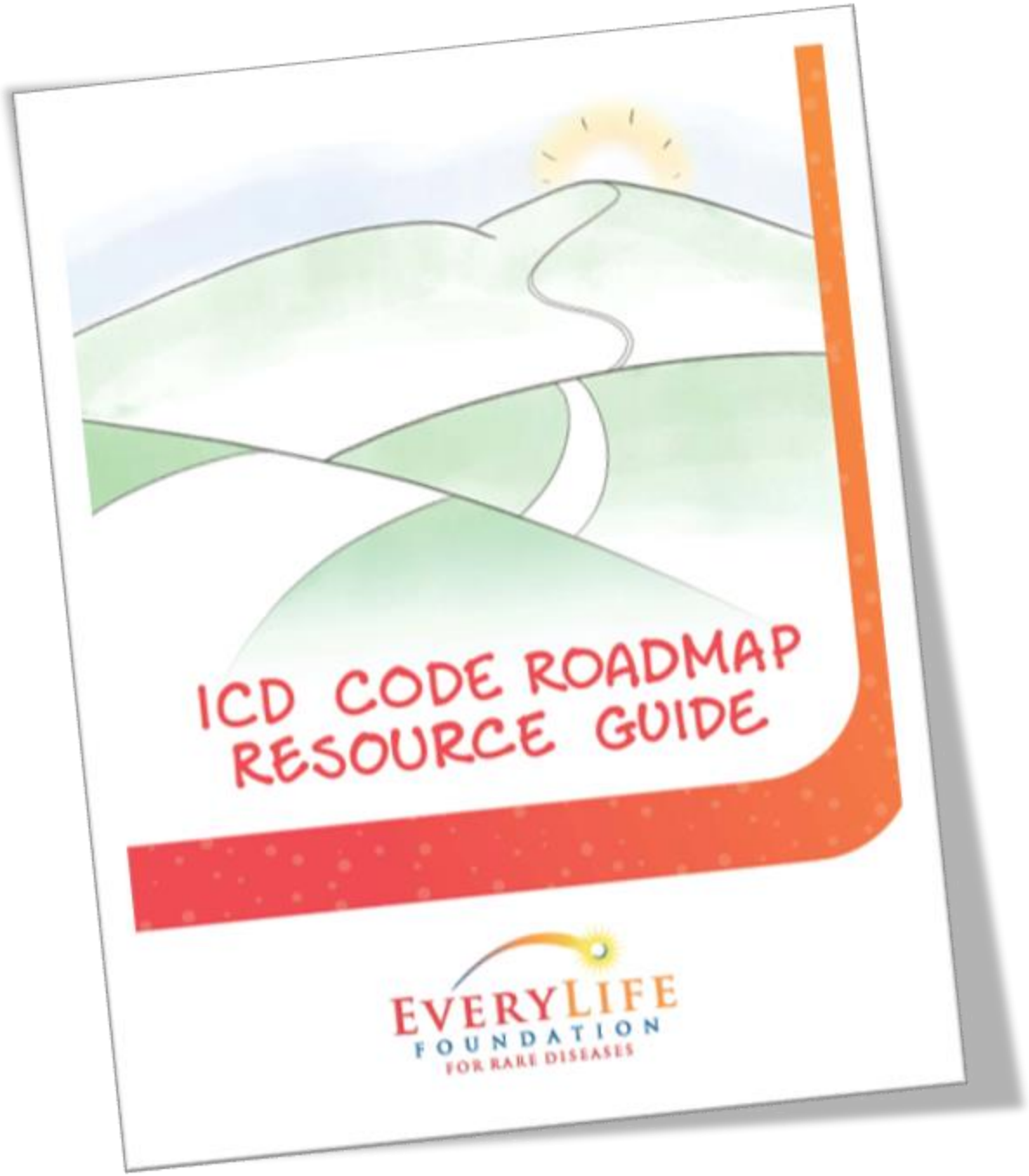


TABLE OF CONTENTS

For all who seek to better understand how diagnosis codes are assigned, updated and revised in the United States' health information system, this guide is for you.

It starts with basic information about the coding system and then describes the process by which patient advocacy organizations and others can propose changes to existing codes or introduce new codes, including the necessary components of a proposal.

Case examples and links to additional resources provide the information you will need to determine whether and how to get involved in the continuous process of refining the diagnostic coding system used in the United States.

Guide To Patient Involvement In Rare Disease Therapy Development

A PUBLICATION OF THE RARE DISEASE PFDD COMPENDIUM WORKSHOP SERIES

HOSTED BY: EveryLife Foundation for Rare Diseases · Bio Biotechnology Innovation Organization · National Health Council · PhRMA Research · Progress · Hope

APPENDIX:

THE RARE DISEASE PFDD GUIDANCE COMPENDIUM WORKSHOP SERIES

HOSTED BY: EveryLife Foundation for Rare Diseases · Bio Biotechnology Innovation Organization · National Health Council · PhRMA Research · Progress · Hope

OVERVIEW OF THE PFDD COMPENDIUM INITIATIVE

THE RARE DISEASE PFDD GUIDANCE COMPENDIUM WORKSHOP SERIES

WORKSHOP 1 // June 23, 2021

RESEARCH AND EARLY DEVELOPMENT

"There was general agreement that engagement at this early stage has tremendous benefits to all stakeholders."

THE RARE DISEASE PFDD GUIDANCE COMPENDIUM WORKSHOP SERIES

WORKSHOP 2 // July 29, 2021

CLINICAL DEVELOPMENT

"Although FDA guidance speaks to these topics, the content is generally high level and not always specific to rare disease drug development."

THE RARE DISEASE PFDD GUIDANCE COMPENDIUM WORKSHOP SERIES

WORKSHOP 3 // September 17, 2021

HEALTH AUTHORITY REVIEW & MARKETING AUTHORIZATION

"PROs are the form of patient experience data most often cited by FDA reviewers, per a recent report."

THE RARE DISEASE PFDD GUIDANCE COMPENDIUM WORKSHOP SERIES

WORKSHOP 4 // October 7, 2021

POST-MARKETING

SUMMARY OF WORKSHOP 4: OPPORTUNITIES FOR PATIENT INPUT IN THE POST-MARKET SETTING

"Once a new therapy is approved by regulators, a new stage of concerted effort is required to ensure it reaches intended patients."

TABLE OF CONTENTS

8 FOCUS AREAS

BY THE NUMBERS

23 Leadership and Steering Committee Members

88 TOTAL — Subject Matter Experts

- 8 Academia
- 40 Industry
- 28 Patient Advocacy Organizations
- 5 Payer Organizations
- 7 Other

20 FDA Guidances

12 Hours of Workshops

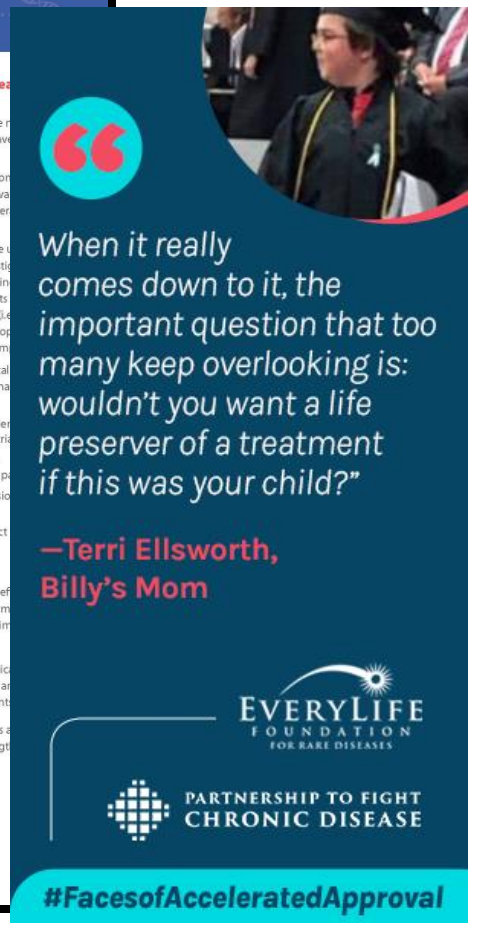
8 Cross-Cutting Topics and Sets of Action Steps

4 Workshop Summaries

112 Resources Linked in This Guide

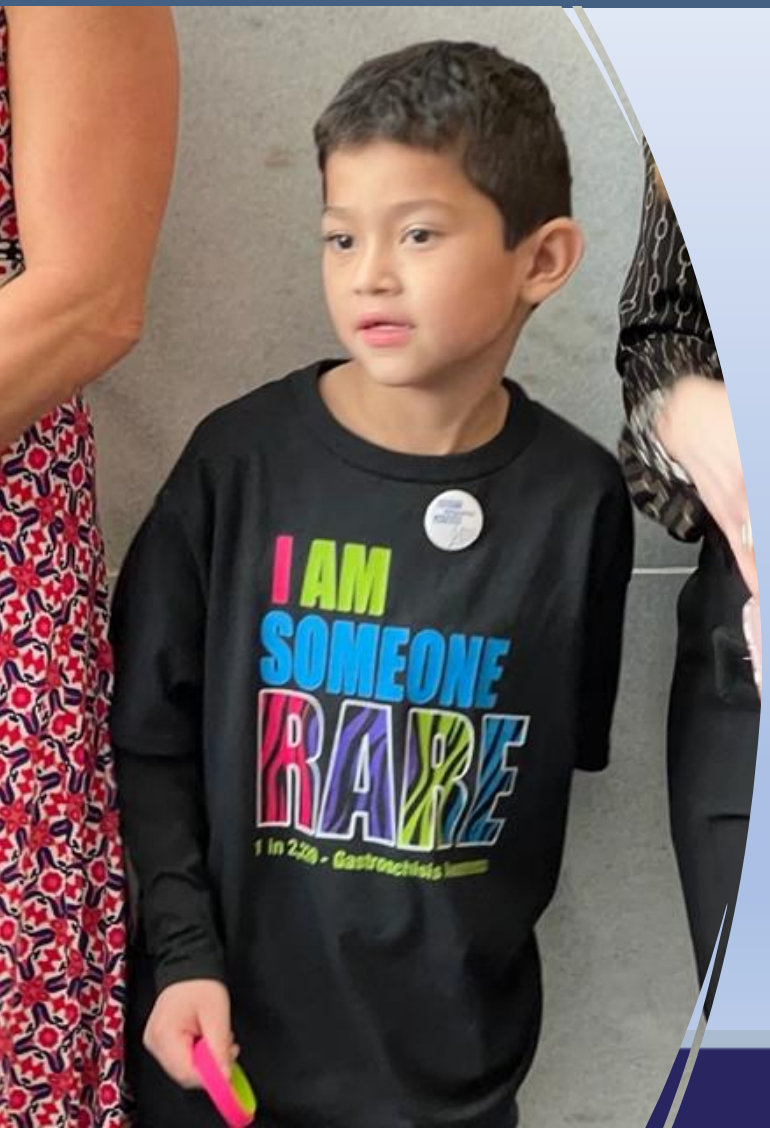
Accelerated Approval Resources

https://everylifefoundation.org/accelerated-approval/



EVERYLIFE FOUNDATION FOR RARE DISEASES

Thank you!



“Surround yourself with relentless humans.
People who plan in years,
but live in moments.
Who work like savages,
but create like artists.
People who know this is finite,
and choose to play infinite games.
Find people going up mountains.
Climb together.”

~ Original Source Unknown, Loved by Annie