CLEARING HURDLES
TO ACCESS RARE DISEASE THERAPIES

RARE READY

A GENETIC CONDITION COALITION

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What We'll Cover Today

- Medicaid Access to Treatments for Rare Conditions
- How to Get Involved

RARE READY

A GENETIC CONDITION COALITION

MEDICAID ACCESS TO TREATMENTS FOR RARE CONDITIONS

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BioMarin

Rare Disease & Medicaid Landscape

- 48% of children with special healthcare needs and 45% of nonelderly adults with disabilities are covered by Medicaid.[1]
- Nearly two-thirds of Medicaid/CHIP-only children with special healthcare needs live in a household with income at or below 138% of the federal poverty level.[2]
- Medicaid enrollment has increased throughout the pandemic, which could influence trends in Medicaid's coverage of prescription medicines.[3]
- End of the Public Health Emergency (PHE) will have significant implications for Medicaid enrollment and spending. Millions of people could face administrative barriers during the process despite remaining eligible.[4]

1-2: Children with Special Health Care Needs: Coverage, Affordability, and HCBS Access. Kaiser Family Foundation. 2021. www.kff.org/medicaid/issue-brief/children-with-special-health-care-needs-coverage-affordability-and-hcbs-access/. Accessed March 12, 2022.
3: Medicaid Outpatient Prescription Drug Trends During the COVID-19 Pandemic. Kaiser Family Foundation. 2021. www.kff.org/medicaid/issue-brief/medicaid-outpatient-prescription-drug-trends-during-the-covid-19-pandemic/. Accessed March 12, 2022.
4: The Impact of the COVID-19 Recession on Medicaid Coverage and Spending. Kaiser Family Foundation. 2022. www.kff.org/medicaid/issue-brief/the-impact-of-the-covid-19-recession-on-medicaid-coverage-and-spending/. Accessed March 12, 2022.

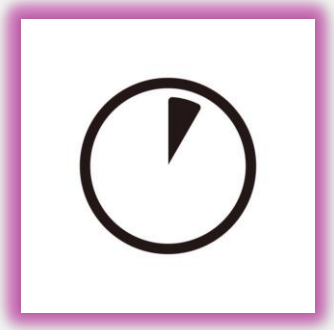
Drug Spending & Budget Impact

Drug Spending

- Spending on prescription drugs increased between 1980 and 2006 (Congressional Budget Office).
- After 2006, the availability of generics and market competition drove down drug prices as an overall percentage of health care expenses, ***even while other health care costs continued to rise.***
- Drug spending has remained **relatively flat since 2015.**

Budget Impact

- Rare disease/orphan therapies have increased in cost over time.
- Overall budget impact of rare disease treatments remains relatively small.

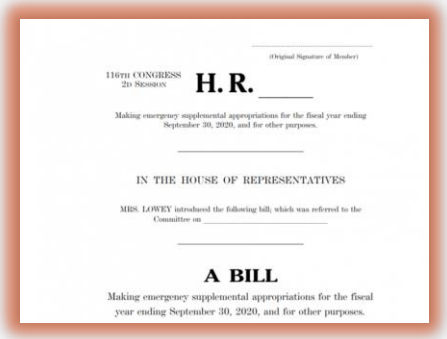


1. Prescription Drugs: Spending, Use, and Prices. Congressional Budget Office, January 2022

Rare Disease Therapies

Rare Disease Drugs

- Since the enactment of the Orphan Drug Act in 1983, the Food and Drug Administration (FDA) has approved 564 orphan products treating 838 rare diseases.[1]
- Only a few hundred of the 7,000 known rare diseases currently have an FDA-approved treatment.
- Approximately 90% of rare diseases still have no treatment available.



(Original Signature of Member)

116TH CONGRESS
2D SESSION

H. R. ____

Making emergency supplemental appropriations for the fiscal year ending September 30, 2020, and for other purposes.

IN THE HOUSE OF REPRESENTATIVES

MRS. LOWEY introduced the following bill, which was referred to the Committee on _____

A BILL

Making emergency supplemental appropriations for the fiscal year ending September 30, 2020, and for other purposes.

Hurdles to Treatments

- 61% of patients have been denied or faced lengthy delays in accessing rare disease therapies that required pre-approval.[2]
- Denied referrals by insurers are an impediment to access to rare disease treatment(s).

1: BARRIERS TO RARE DISEASE DIAGNOSIS, CARE AND TREATMENT IN THE US: A 30-Year Comparative Analysis. NORD. 2020. https://rarediseases.org/wp-content/uploads/2020/11/NRD-2088-Barriers-30-Yr-Survey-Report_FNL-2.pdf. Accessed March 12, 2022.
2: BARRIERS TO RARE DISEASE DIAGNOSIS, CARE AND TREATMENT IN THE US: A 30-Year Comparative Analysis. NORD. 2020. https://rarediseases.org/wp-content/uploads/2020/11/NRD-2088-Barriers-30-Yr-Survey-Report_FNL-2.pdf. Accessed March 12, 2022.

Challenges in Accessing Rare Disease Therapies

ICER and Other Cost-Effectiveness Analysis

- State Medicaid agencies relying on the Institute for Clinical & Economic Review (ICER) analysis and reports to evaluate treatments.
- Disproportionate impact on rare disease patients/therapies because of the use of quality-adjusted life-year (QALY).
- Medicaid agencies (CA, MA) working directly with ICER or leveraging reports when developing coverage criteria.

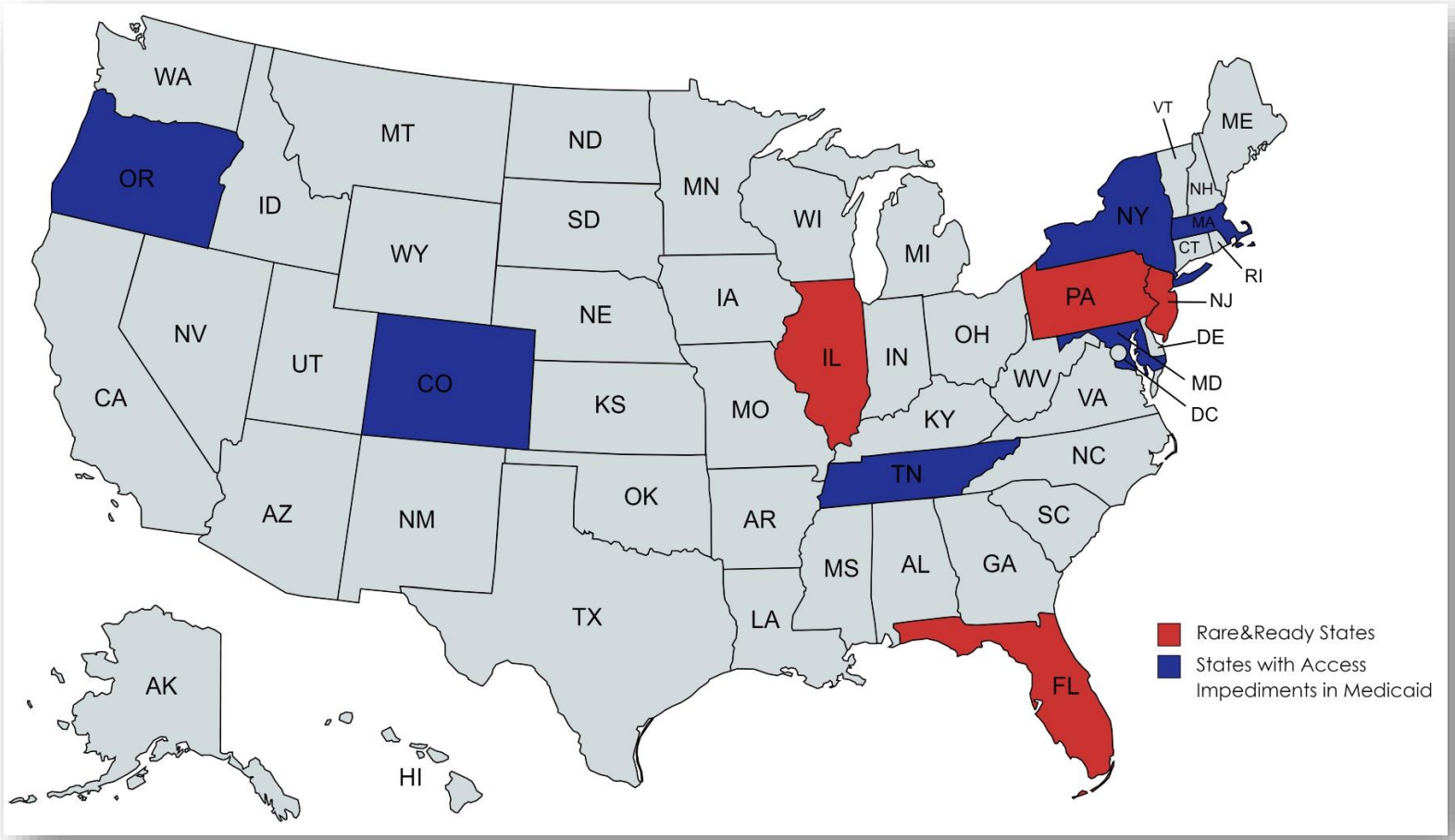
State Medicaid Agencies Lack of Coverage

- State Medicaid agencies may use waivers (§1115 waivers) and other avenues to limit access to innovative therapies.
- Operating a commercial-style closed formulary or not covering accelerated approval drugs have become more commonplace.
- While some proposals (waivers) have not been approved by the Centers for Medicare and Medicaid Services (CMS), more continue to "pop up."

Price Controls

- Price setting through prescription drug affordability boards (PDAB's) or reference pricing legislation is a common tactic used by state Medicaid agencies.
- Laws/proposed legislation allow states to limit reimbursement levels for certain innovative therapies.
- These laws are an existential threat to the incredible and innovative R&D pipeline for rare disease therapies.

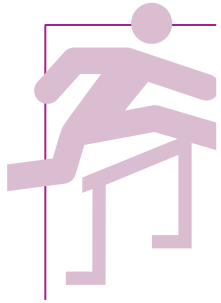
State-Specific Medicaid Challenges



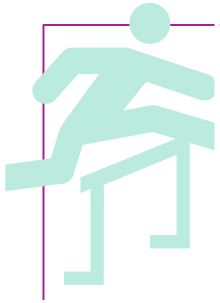
- **Many states legislating and/or regulating changes to their Medicaid programs.**
- **States utilizing the following strategies:**
  - *Waivers to seek permission to operate closed formularies/limit access to accelerated approval drugs (MA, OR, TN).*
  - *Pass legislation aimed at controlling the cost of prescription drugs (CO, MD, NY).*
- **Rare & Ready focusing on four states to mitigate access challenges:**
  - *Florida – Medicaid Managed Care plans are slow to promote coverage to newly approved therapies.*
  - *Illinois – Medicaid Fee for Service and Managed Care Organizations do not always maintain consistent coverage criteria.*
  - *New Jersey and Pennsylvania – continued scrutiny of rare disease therapies.*

Hurdles Impede Access to Treatments

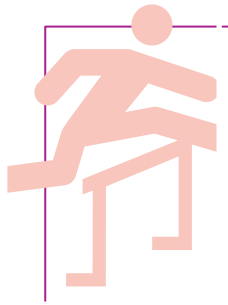
Several “operational hurdles” pose a threat to rare disease therapy access



Significant delays to meaningful coverage in Medicaid upon FDA-approval



Medicaid coverage criteria being more restrictive than the FDA-approved label



Discrepancies between managed care coverage criteria and fee-for-service

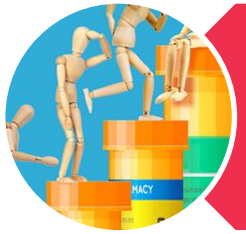
"Time to Treat" Legislative Concept & Principles

Mandated time frame for completion of clinical review and development/publishing of coverage criteria – *require Medicaid programs to complete/publish* ***within 60 days of FDA approval*** *(caveat for manual review included)*

Rationale → Prevents lengthy delays to development of coverage policies

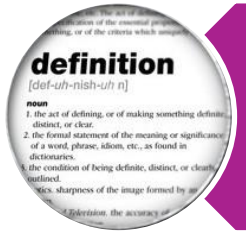
Coverage policy developed cannot be more restrictive than the indications and usage section of FDA-approved label

Rationale → Prevents Medicaid/managed care organizations from developing coverage policy based on trial inclusion/exclusion criteria



Easy exception process for step therapy if deemed medically necessary by provider – *creates a straightforward exceptions process - mandates response to request* ***within 72 hours for request, 24 hours for emergency request***

Rationale → Helps mitigate some of the onerous utilization management strategies implemented by plans and Medicaid



Defines "qualifying rare disease therapy" as rare pediatric therapy, orphan therapy, breakthrough designation or RMAT (Regenerative Medicine Advanced Therapy) designation

Rationale → Limits scope of legislation to increase likelihood of passage

Other Issue Areas to Prioritize for Advocacy

Maintaining access to accelerated approval therapies in Medicaid

- States are scrutinizing therapies that are approved under FDA accelerated approval pathway.
- Attempting to limit coverage through waivers (MA, OR, TN).
- Educate policymakers and/or mitigate attempts to block access to these therapies.

Promoting access to gene and cell therapies through outcomes-based arrangements/value-based arrangements

- State Medicaid programs concerned about cost of therapy and durability.
- Outcomes-based arrangements would allow manufacturers to “share in risk.”

Maintain expanded access to telehealth

- Access to health care through telehealth increased during the height of the pandemic.
- Some states do not allow patients with rare conditions to use telehealth with experts who practice medicine in other states.
- States should adopt policies expediting licensure for out-of-state healthcare providers treating rare disorders with telehealth.

QUESTIONS

HOW TO GET INVOLVED

Kari Lato
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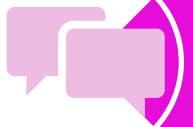
Rx4good

Why Advocacy Matters

Gives a voice to those most impacted by policy decisions.

Serves as a powerful tool for raising disease awareness and offering valuable insight into the patient and caregiver experience.



Messages delivered through storytelling are **22 times more memorable** than facts.[1]

Increases awareness to initiate changes that create better outcomes.

1: A Good Presentation Is About Data And Story. Forbes. 2015. https://bit.ly/3IjJQL0. Accessed on 3/12/22.

Advocating for Change

Simple ways you can advocate for policy change:

- ✓ Build awareness, change public perception and move people to action.
- ✓ Educate and engage policymakers and other stakeholders.
- ✓ Build a strong and diverse coalition to amplify your voice and impact.

Rare & Ready: A Genetic Condition Coalition

Batten Disease Support and Research Association

Bleeding Disorders Alliance Illinois

the chandler project — Advocacy • Awareness • Research • Support

The Children's Medical Research Foundation, Inc. — A Cure for Kirby

HCMA Hypertrophic Cardiomyopathy Association

Little Hercules Foundation

The MAGIC Foundation

MPS SuperHero Foundation

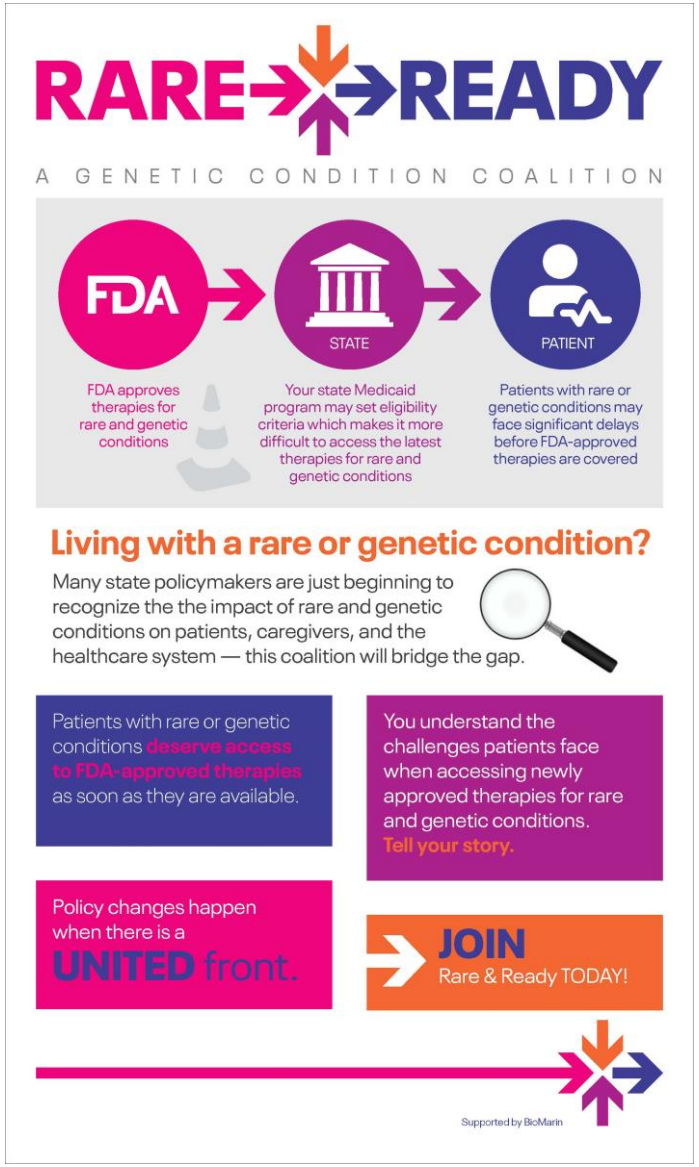
National MPS Society — Support for Families. Research for a Cure.

nnpdf — National Niemann-Pick Disease Foundation, Inc.

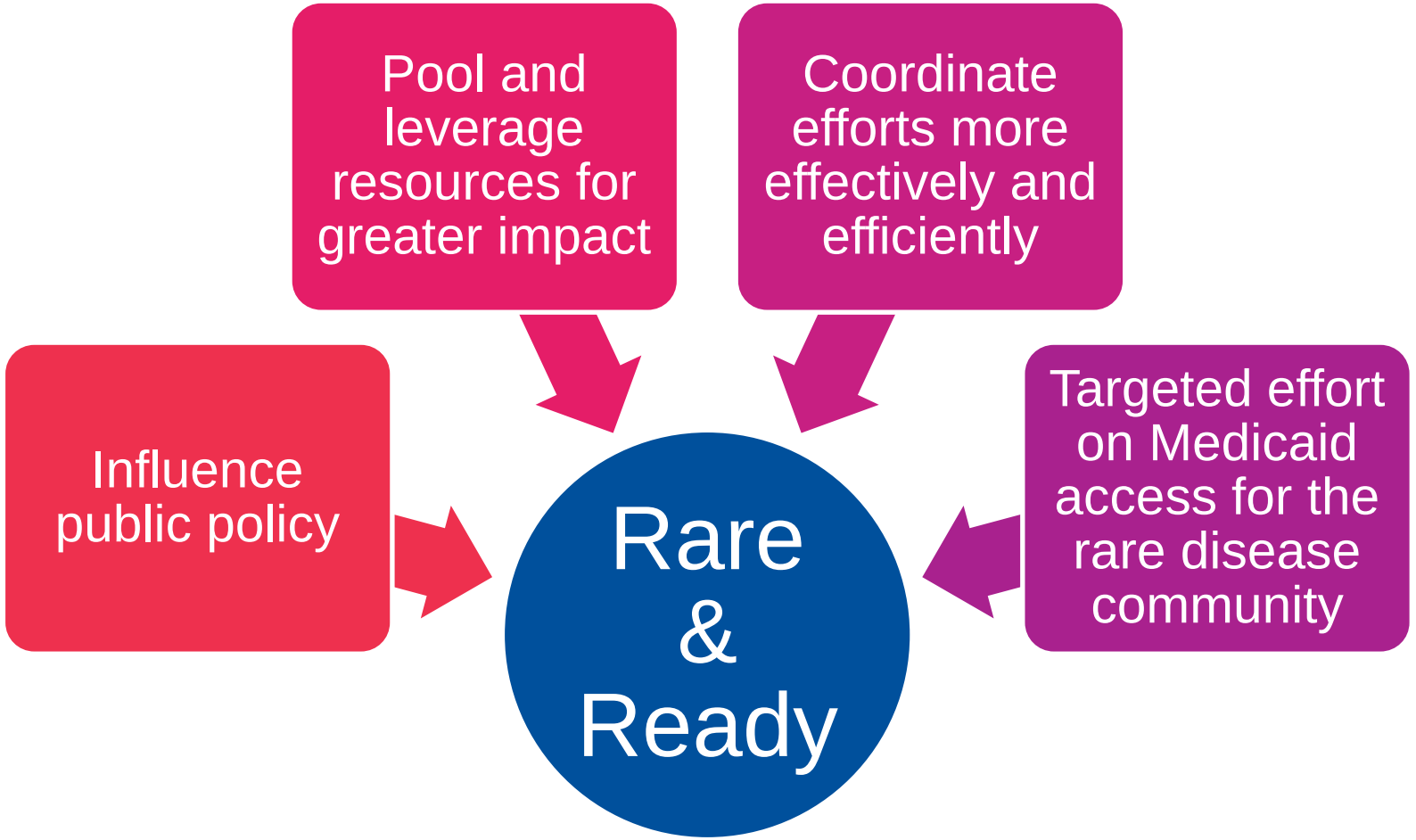
National PKU Alliance

noah's hope — Every Childhood Disease Deserves a Cure

SCRED — Sickle Cell Reproductive Health Education Directive



Power of a Coalition



Effective Ways to Advocate

- Join the Rare & Ready Coalition!
- Encourage others to join the coalition. The more voices – the bigger the impact.
- Meet with decision-makers to share your rare disease experience. Explain the impact of their policy decisions on your community.
- Write an op-ed or letter to the editor.
- Use social media to educate and bring awareness to the issue – make sure to tag and include relevant hashtags.
- Submit comments or feedback on policies and regulations.

Power of Social Media

#Hashtag your way into the conversation
- Keep up with legislative movements
- Follow @rare_ready – share tweets

Use Facebook Groups to spread the word
- Share messages from the Rare & Ready Coalition
- Share responses from elected officials

Let pictures/videos tell the story
- Instagram can paint a picture that emails can't
- Use videos to tell your rare disease experience

How You Can Help

Join the Coalition

- Contact kari.lato@rx4good.com to join
- Ask others to join

Share Your Experience

- Tell your story
- Meet with decision-makers

Use Social Media

- Follow @rare_ready
- Repost on your social media channels

Policy Changes Don’t Happen Quickly

THANK YOU

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