

EPICrd – Bridging Innovation and Medicaid Access for Rare Genetic Conditions

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Sponsored by BioMarin & Ultragenyx

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What We'll Cover Today

- Examine how recent advancements in rare disease technologies are outpacing Medicaid coverage.
- Highlight the urgent need for federal legislation to ensure equitable access to innovations.
- Discuss how you can get involved.



EPICRD

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Ensuring Parity through Individualized Care for Rare Disorders

EPICrD

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EPICrD

Three Individual Pillars of Medicaid Access Policy

EPICrD Act

Rare disease therapies must not be hindered; rather, medical necessity determinations by a physician should negate need for prior authorization and step therapy.



Access to specialized care across state lines must be easier.

More to Come...

Patients with rare genetic disorders need more than access to medicines.

How We Got Here



Together, we:

- Collaborated over three-plus years.
- Considered the rare community's challenges to Medicaid access and identified possible solutions.
- Worked with BioMarin to develop federal legislative language and an outreach strategy.
- Successfully secured an original bill sponsor, Rep. Pfluger (R-TX 11).

Now, we ask you to join us in supporting EPICrd

EPICrd

Developed by Advocacy. Supported by BioMarin.

Two Bills are Positioned to Be Introduced



EPICrD

EPICrD Act

Medical necessity, prior authorization, and step therapy in Medicaid.



Medicaid access to out-of-state providers.

More to Come



Medical Necessity, Prior Authorization, and Step Therapy

EPICrD Act

Timely Access to Rare Disease Therapies in Medicaid

- **Streamline prior authorization for rare disorder drugs.**
 - Prescriber attestation to medical necessity.
 - Protection from step therapy and other coverage restrictions.
- **Include patients, caregivers, expert clinicians in decision-making about formulary replacement and coverage criteria.**
 - Engagement in Medicaid drug utilization review (DUR) boards and pharmacy & therapeutics (P&T) committees.
 - Review of consensus-based standards of care or treatment guidelines; peer-reviewed literature on drugs class; and real-world data.



Timely Access to Rare Disease Therapies in Medicaid

- Prevent use of section 115 Medicaid demonstration waivers to establish restrictive drug coverage
- Define appropriate rare disease therapy categories and classes to reflect patient need, latest science, and current standard of care
 - NIH Office of Rare Diseases to work with stakeholders to develop and maintain lists of categories and classes to set minimum drug coverage requirements



Medicaid Access to Out-of-State Providers





Medicaid Access to Out-of-State Providers

- Set up five-year demonstration project to improve access to out-of-state specialists at center of excellence, in person and via telehealth.
 - Streamlined out-of-state provider screening and enrollment and medical licensure reciprocity for telehealth.
 - Payment for telehealth (including both providers in peer-to-peer consult) and drug acquisition and administration at no less than that of provider's state's rate.
 - Temporary Medicaid quarterly furnishing fee to the states to offset excess payments to out-of-state providers.

***Builds on
Accelerating Kids
Access to Care
Act***



Medicaid Access to Out-of- State Providers

- Ensure out-of-state access to physicians-administered drugs, inpatient and outpatient for complex or disabling rare conditions.
- Leverage telehealth services from full-range of clinical experts in multidisciplinary teams.
 - Diagnosis, treatment, care management.
 - Peer-to-peer consults.
 - Patient education, including REMS requirements.
 - Monitoring after gene therapy.

**For more information,
EPICrD.org**

EPICrD



GET INVOLVED

Kari Lato

Sr. Director, Policy & Advocacy

Rx4good

Effective Advocacy When Legislation is Introduced



We will draft a group support letter and invite your organization to sign on.



Once legislation is introduced, we will need your help to get co-sponsors.



When the bill is referred to committee for a hearing, testify in person or submit written comments in favor of the legislation.



Meet with decision-makers to share your story and support of EPICrd.



Use social media to educate and bring awareness to the issue – make sure to tag legislators and include relevant hashtags.

Communicating with Legislators After Bill Introduction



- Introduce yourself and the Rare & Ready Coalition through written communications.



- Find template letters at www.rareandready.org/take-action/ or reach out to us and we can help.



- Follow-up with a phone call or personal visit.
- Follow and tag your legislator on social media.

Providing Testimony on Legislation



- ✓ Rare & Ready will make you aware of the committee's rules for testimony.
- ✓ Keep your testimony simple, short, and focused. Avoid medical jargon and acronyms.



- ✓ Clearly present your position: "I support" followed by bill number.
- ✓ Say why you have taken your position. Share your personal story.
- ✓ Ask for specific action: "I urge you to vote in favor of EPICrd (bill number)."



- ✓ Thank the committee for the opportunity to speak and offer to answer questions.
- ✓ Type your testimony and make copies for legislators, include your contact information.

Other Ways You Can Help

Join the Coalition

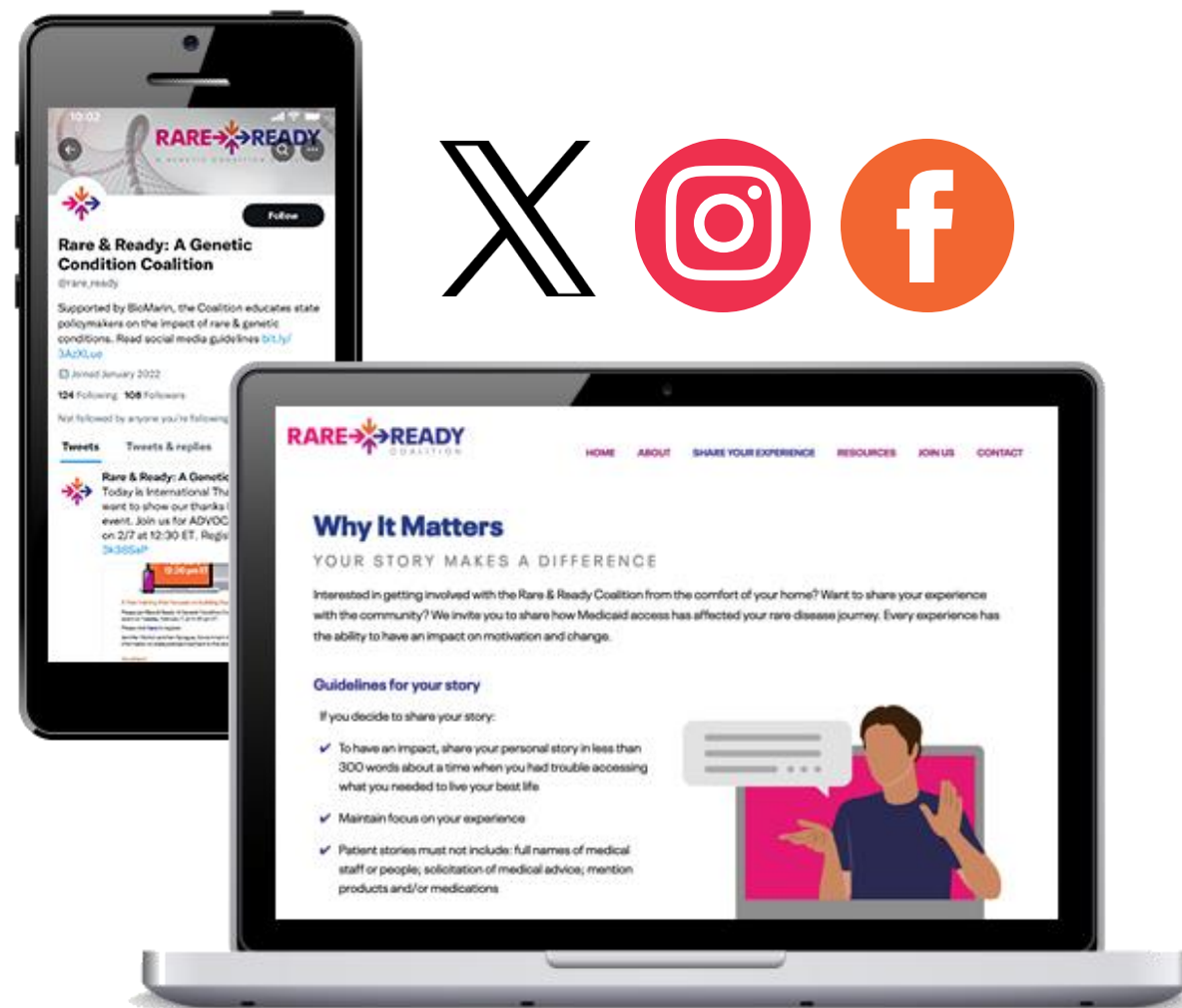
- Contact kari.lato@rx4good.com to join
- Ask others to join

Share Your Experience

- Share your story on www.rareandready.org
- Meet with decision-makers

Use Social Media

- Follow [@rare_ready](https://twitter.com/rare_ready)
- Repost on your social media channels



THANK YOU

