Rare Disease
Company Coalition

Advancing Patient Access to Rare Disease Therapies

Contents

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Even the most innovative and effective rare disease treatments are only as valuable as they are accessible. For rare disease patients who are fortunate to have an FDA-approved treatment for their condition, we must advance policies that facilitate timely access so patients can benefit from treatments as soon as they are available.

1 Accelerate Review of Newly Available Therapies

Patients living with rare diseases in the United States can experience a a range of financial barriers to diagnosis, care, and treatment depending on their type of health insurance. In Medicaid, for example, although states are required to cover drugs as long as the manufacturer enters into a Medicaid Drug Rebate Program Agreement, Medicaid agencies have imposed access barriers more restrictive than the FDA-labeled indication, particularly for rare disease therapies due to concerns around cost and budget impact.

Federal law requires that FDA-approved drugs subject to a rebate agreement be covered by each state Medicaid program upon availability. Any delays in such coverage due to lengthy pharmacy and therapeutics (P&T) Drug Utilization Review Board (DURB), an entity that authorizes and reviews the prescribing, distribution and use of medication proceedings contradict such a requirement and can hinder access to newly available therapies for patients in need. Each state P&T Committee or DURB follows its own new-drug review law and policy, which in many states permits or even mandates extended review timelines. Such delays can result in unreasonable access restrictions on new drugs. To put it in perspective, more than half of all rare disease patients re children.[1] Many of their conditions are progressive and life-threatening - they cannot afford Medicaid's extended delays before they can access a needed treatment.

"Policies that incentivize accelerated coverage reviews and streamline approval processes of new-to-market rare disease therapies are critical to facilitating timely access for both patients who are in urgent need of groundbreaking medicines and those transitioning from clinical trials."[2]

~ Scott Braunstein, M.D., Chairman & Chief Executive Officer, Marinus Pharmaceuticals

Proposed Policy Solutions:

- **Congress should work with the Centers for Medicare & Medicaid Services (CMS) to ensure timely patient access to treatment and to achieve shorter, streamlined drug coverage approval processes.** This includes enforcing existing Medicaid outpatient drug coverage obligations for state fee-for-service and contracted managed care organizations (MCOs) to ensure that when a drug is prescribed for its medically accepted FDA-approved indication, and a manufacturer has a signed Medicaid drug rebate agreement, the patient is promptly approved for treatment. CMS should instruct state Medicaid programs (including its MCOs) to reduce burdensome and unnecessary prior authorization processes to facilitate timely access to medicines.
- **Congress should establish rapid review incentives for new rare disease therapies.** While states and MCOs must approve prescription requests when a drug is prescribed for its medically accepted indication, Congress should consider establishing incentives, such as bonus payments, for states to conduct expedient coverage reviews of newly available rare disease therapies through P&T and DURB reviews. For example, bonus payments could be offered if coverage reviews are considered by the next scheduled DURB meeting after availability and in no case later than 90 days.
- **States should consider establishing a fee-for-service coverage policy aligned to a drug's medically accepted indication** and share the service on its website to support provider and patient awareness, and serve as a model for a state's MCOs. In developing a coverage policy, states should also consult with a rare disease physician expert who prescribes the treatment.

2 Facilitate Medicaid Access Across Borders

Rules for credentialing providers are complex, and disincentivize coverage for out-of-state providers. This results in a patchwork system that discourages patient access to care. Currently, each state develops and sets its own credentialing criteria. Therefore, a Medicaid provider is not obligated to treat an out-of-state Medicaid patient except in emergency situations. Further, an out-of-state provider can generally only bill and accept payment from an out-of-state Medicaid program if it is credentialed by that program -a time-consuming and expensive process.

"The needs of people living with rare diseases are universal and patients continue to experience significant burden in managing their daily lives. We are committed to innovating to meet the needs of the rare disease communities we serve, and a key part of this is prioritizing patient access so they can reach the care they so desperately need [...]."[3]

~ Tim Van Hauwermeiren, Chief Executive Officer, argenx

Proposed Policy Solutions:

- **CMS should build upon its recent *"Guidance on Coordinating Care Provided by Out-of-State Providers for Children with Medically Complex Conditions"*[4]** to best ensure out-of-state care for children with rare diseases.
- **CMS should create a nationwide, standardized enrollment process for out-of-state providers treating rare disease patients.** A single, uniform process for credentialing would allow providers to meet the needs of the rare disease community and treat out-of-state patients.
- **Policymakers should mandate coverage for interstate telehealth.** Both in-person and telehealth visits for patients with a rare disease should be covered when clinically appropriate. When out-of-state medical assistance is required due to the nature of a patient's symptoms or diagnosis, a plan should immediately recognize the out-of-state provider as a participating provider.
- **Congress should improve Medicaid patient access to out-of-state providers** by eliminating the burdensome provider screening and enrollment processes necessary for Medicaid payment. Instead, CMS should establish a national web-based Medicaid provider enrollment portal with a uniform, streamlined submission process.
- **States should support a national credentialing system that ensures physicians with the proper training and expertise can care for patients with rare diseases.** This would more effectively distribute health care costs across states and more effectively address the health care 'deserts' that plague rare disease therapy access.
- **States should pursue state plan amendments (SPAs) and mechanisms that enable states to make modifications based on patient need that could expand access to rare disease therapies for patients in states with access issues.** These mechanisms can provide a model for activity at the federal and national level and may allow for quicker access to therapies under state Medicaid plans that could provide a model for payment policies at the national level.

3 Ensure Protections for Rare Conditions in Employee Health Plans

Many self-insured employee health plans are not subject to the Affordable Care Act Section 1557, which prohibits discrimination in health care plans and activities. In fact, the U.S. Department of Health and Human Services (HHS) and its Office for Civil Rights (OCR), as well as the Equal Employment Opportunity Commission (EEOC) and other agencies, explain that self-insured plans are subject to anti-discrimination regulations (Age Act, ADA, Civil Rights Act, etc.) However, there is evidence[5] that such plans are restricting coverage for rare disease patients through discriminatory policy designs based upon protected characteristics such as age and disability.

"It is critical that we work together to increase the number of safe, effective, and affordable treatments that are available for people with rare diseases."[6]

~ Senator Amy Klobuchar, Co-Chair, Congressional Rare Disease Caucus (D-MN)

Proposed Policy Solutions:

- **Regulators should hold self-insured plans governed by the Employee Retirement Income Security Act of 1974 (ERISA) accountable to the Transparency in Coverage rule[7] regarding rapid enforcement to ensure plan assets are being applied in the best interest of rare disease patients.** Insurance carriers and employers (for self-insured plans) should be required to publicly post the U.S. dollar costs of in- and out-of-network health care services to participants, beneficiaries, and enrollees through machine-readable files.
- **Congress should present rare disease patients who elect to leave an ERISA plan due to poor benefit design with annual open enrollment periods, special enrollment periods, and renewal periods** on the exchanges.

4 Enable Rare Disease Patient Access to Accelerated Approval Therapies

Rare disease drug development poses unique challenges due to the small number of patients available to participate in clinical trials, variability in disease presentation, and varied progression of some diseases. The accelerated approval pathway (AAP) is a critical tool that can address these unique circumstances and provide rare disease patients with earlier access to treatment based initial evidence of safety and effectiveness, while confirmatory studies required to verify clinical benefit are ongoing.[8]

In 2022, Congress passed legislation that empowers the FDA to ensure the timely completion of confirmatory trials,[9] which are required for sponsors seeking accelerated approval, and implements streamlined withdrawal procedures. However, recent federal and state proposals[10] have shown a willingness by public and private payors to modify coverage protocols based on arbitrary conditions of a drug's effectiveness in certain populations, which would limit patient access to life-saving treatments. A recent study[11] found that these types of changes to accelerated approval would put at risk of withdrawal therapies that have the potential to address the needs of up to 3.6 million patients.

As Medicaid plays a critical role in supporting the rare disease community, particularly children with rare genetic conditions, these proposals would have a devastating impact on patients benefiting from innovation and create a greater health equity divide, all while resulting in limited budget savings. Research shows that targeting accelerated approval drugs will not save state Medicaid programs money, as the therapies represent less than one percent of program spending.[9]

Any future policy reforms to the accelerated approval pathway should protect patient access, build upon Congress' recent steps to boost FDA oversight of accelerated approval products, leverage and enhance its existing authorities, and protect the integrity of this critical pathway.

"The accelerated approval pathway is working as intended: delivering better health to those in dire need. We must value and protect it for the benefit of patients where the opportunities of accelerated approval have yet to be realized."[12]

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

Proposed Policy Solutions:

- **Congress should protect FDA's regulatory authority and reject proposals that arbitrarily limit patient access to safe and effective therapies.** The FDA is the sole authority to approve drugs for human use and evaluate their safety and effectiveness. Congress should reinforce FDA's regulatory authority and ensure that CMS, state Medicaid, and commercial payors do not undermine FDA's determination of a product's safety and efficacy by re-adjudicating FDA's decision. Congress should also prohibit any federally funded health care program from using payment as leverage to modify or counter FDA's evaluation of the safety and effectiveness of a rare disease treatment.
- **Payors should defer to FDA's expertise and authority** on the intended patient population that would benefit from a rare disease treatment and should not separately limit the covered patient population.

5 Streamline Value-Based Payment Methodologies for Rare Disease Therapies

As the federal and state governments look for ways to rein in health care spending, some are turning to value assessments as a way to determine how much to pay for medicines and other treatments. Yet assessments often use a "one-size-fits-all" approach when considering the value of treatments that can undermine access for people living with rare diseases.

Measures such as Quality-adjusted Life Years (QALYs), often used as inputs in value assessment, do not reflect those patient-centered perspectives and devalue the lives of people who are older, living with disabilities or are from disadvantaged communities. QALYs may undervalue orphan drugs that are particularly beneficial for racial and ethnic minority patients or other vulnerable patient populations, and inadvertently perpetuate existing health disparities.

State Prescription Drug Affordability Boards (PDABs) and similar groups may use value assessments to set upper limits on drug prices, leverage and improve state purchasing power. Depending on the methodologies and inputs they use, their assessments could be based on false standards and inequitable criteria and limit patients' access to needed treatments.

Value assessments for rare disease treatments must be patient-centered, and account for a treatment's impact on the holistic patient experience. To do this, value assessments should reflect population-level information, but should look at individual patient viewpoints and disease journeys. Additionally, value assessments must consider both the direct and indirect benefits of a treatment, particularly those most important to the patient.

"Access to the best medical treatments is crucial for patients living with rare and ultra-rare diseases, and that is why we must prioritize the work to close the innovation gap for rare disease therapies."[13]

~ Congresswoman Doris Matsui, Co-Chair, Congressional Rare Disease Caucus (D-CA)

Proposed Policy Solutions:

- **Policymakers and state bodies should eliminate the use of one-size-fits-all value assessments and ensure that value assessments for rare disease therapies are patient-centered.** Value assessments for rare disease treatments should reflect the available evidence for rare disease treatments including both the direct and indirect benefits of a treatment, particularly those most important to the patient. They can do so by accounting for measures the progression of disease, symptom control, a treatment's impact on productivity, ability of a patient to participate in activities of daily living, impact on caregiver burden, improvement over alternative treatments, impact on public health, and the value of hope.

"[...] when new therapies for rare diseases are finally approved under the current structure, patients often face unnecessary delays and barriers to access, resulting in avoidable health deterioration. Tragically, many of these patients cannot afford to wait decades for help."[14]

~ Congressman Gus Bilirakis, Co-Chair, Congressional Rare Disease Caucus (R-FL)

"Access to rare disease therapies should be declared a public health priority. It is critical that we not only bring important new therapies to market, but also collaborate with government and advocacy organizations to ensure policies increase, not decrease, patients' ability to access therapies and specialists who really understand rare diseases."[15]

~ Giacomo Chiesi, Head of Chiesi Global Rare Diseases

6 Streamline Medical Necessity Determinations in Medicaid Prior Authorization for Rare Disease Therapies

Increasingly, Medicaid plans are substituting their own judgment about the efficacy of a rare disease drug for that of the patient's prescriber. Some state Medicaid plans, under the guise of prior authorization, are exploiting loopholes in federal law to implement restrictive coverage criteria that are constructed to deny access to some covered outpatient drugs without consideration of the physician's determination of their medical necessity. Because "medical necessity" is not defined in statute or regulation, states have had wide latitude in establishing discriminatory coverage policies. For transformative therapies that are satisfying an unmet need or improving the standard of care for rare diseases, there should be a clear, streamlined pathway for the prescriber to establish medical necessity, and thus ensure payment in Medicaid.

Medical necessity should be determined by the prescriber, not state bureaucrats. Clarifying federal legislation is necessary to prevent Medicaid drug coverage policies from harming patients, particularly children, with serious and life-threatening rare diseases. Although states must be good stewards of their Medicaid budgets, prior authorization criteria that fails to ensure patient access to "medically necessary" drugs conflict with not only the congressional intent of the Medicaid Drug Rebate Program, but also established policy of CMS. Delays or denials of "medically necessary" drugs under the guise of prior authorization have resulted and will continue to result in poor outcomes for Medicaid beneficiaries, particularly children with rare, genetic disorders that are typically characterized by early onset, rapid progression, and mortality.

"Medical and scientific innovation are only worthwhile if the people who can benefit from life-changing therapies have access to them. The continued challenge with rare disease is the unknown – there is no one-size-fits-all approach. We must remain committed to proactive listening and partnership with the entire health care ecosystem, including different Payor mix, to overcome these challenges and design solutions like value-based agreements that ultimately support access for rare disease communities."[16]

~ Yvonne Greenstreet, MBChB, Chief Executive Officer, Alnylam Pharmaceuticals

Proposed Policy Solutions:

- **Congress should streamline prior authorization for covered outpatient drugs**, and clarify that a simple attestation by a prescriber supporting the on-label use of certain rare disease therapies sold under an outcomes-based agreement determines "medical necessity" for the purpose of the Medicaid program.

Footnotes

[1] (2023, April 28). *State Medicaid and CHIP Applications, Eligibility Determinations, and Enrollment Data*. Data.Medicaid.gov. Retrieved May 3, 2023, from https://data.medicaid.gov/dataset/6165f45b-ca93-5bb5-9d06-db29c692a360.

[2] Braunstein, S. (M.D.). (May 3, 2023). Marinus Pharmaceuticals.

[3] Van Hauwermeiren, T. (May 10, 2023). argenx.

[4] Tsai, D. (2021, October 20). Guidance on Coordinating Care Provided by Out-of-State Providers for Children with Medically Complex Conditions. Medicaid.gov. Retrieved May 3, 2023, from https://www.medicaid.gov/federal-policy-guidance/downloads/cib102021.pdf.

[5] National Council on Disability (NCD) (2019, November 6). *Quality-Adjusted Life Years and the Devaluation of Life with Disability: Part of the Bioethics and Disability Series*. Ncd.gov. Retrieved May 3, 2023, from https://ncd.gov/sites/default/files/NCD_Quality_Adjusted_Life_Report_508.pdf.

[6] (2015, December 17). *Klobuchar, Hatch Become Senate Co-Chairs of Rare Disease Congressional Caucus*. U.S. Senator Amy Kobuchar. Retrieved May 3, 2023, from https://www.klobuchar.senate.gov/public/index.cfm/2015/12/klobuchar-hatch-become-senate-co-chairs-of-rare-disease-congressional-caucus.

[7] (2010, May 13). *Interim Final Rules for Group Health Plans and Health Insurance Issuers Relating to Dependent Coverage of Children to Age 26 Under the Patient Protection and Affordable Care Act*. Federal Register. Retrieved May 3, 2023, from https://www.ecfr.gov/current/title-45/subtitle-A/subchapter-B/part-147/section-147.210.

[8] (n.d.). *What is Accelerated Approval?* Everylifefoundation.org. Retrieved May 10, 2023, from https://everylifefoundation.org/accelerated-approval/#:~:text=What%20is%20Accelerated%20Approval%3F,serious%20conditions%20with%20unmet%20needs.

[9] Text - H.R.2617 - 117th Congress (2021-2022): Consolidated Appropriations Act, 2023. (2022, December 29). https://www.congress.gov/bill/117th-congress/house-bill/2617/text.

[10] Rare Disease Company Coalition (2023, March 22). *RDCC Urges Congress to Reject Proposals that Undermine the Intent of Accelerated Approval for Rare Disease Patients*. Rarecoalition.com. https://www.rarecoalition.com/2023/03/22/rdcc-urges-congress-to-reject-proposals-that-undermine-the-intent-of-accelerated-approval-for-rare-disease-patients/.

[11] (n.d.). *Calculating the Value and Impact of Accelerated Approvals: Final Findings*. Vital Transformation. Retrieved May 3, 2023, from https://vitaltransformation.com/2022/11/calculating-the-value-and-impact-of-accelerated-approvals-final-findings/.

[12] Kennedy, A. (n.d.). *What is Accelerated Approval?* Everylifefoundation.org. Retrieved May 10, 2023, from https://everylifefoundation.org/accelerated-approval/#:~:text=What%20is%20Accelerated%20Approval%3F,serious%20conditions%20with%20unmet%20needs.

[13] (2023, February 27). *MATSUI, WENSTRUP, WICKER, KLOBUCHAR, REINTRODUCE BENEFIT ACT.* Congresswoman Matsui. Retrieved May 3, 2023, from https://matsui.house.gov/media/press-releases/matsui-wenstrup-wicker-klobuchar-reintroduce-benefit-act.

[14] (2021, April 15). *Bipartisan measure can expedite development of therapies for rare diseases*. The Hill. Retrieved May 3, 2023, from https://thehill.com/blogs/congress-blog/healthcare/548358-bipartisan-measure-can-expedite-development-of-therapies-for.

[15] Chiesi, G. (May 2, 2023). Chiesi Global Rare Diseases.

[16] Greenstreet, Y. (MBChB). (May 3, 2023). Alnylam Pharmaceuticals.

Rare Disease Company Coalition

We are dedicated to being a reliable and trusted resource for our leaders in Congress and the Administration, and a productive partner to rare disease-minded industry, academic and patient groups, by educating on the issues and opportunities that affect rare disease companies and advancing our shared mission to improve the lives of people living with rare diseases.

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