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Founded to tackle one of the most persistent blind spots in rare disease policy, the Rare Access Action Project (RAAP) focuses on what happens after FDA approval, when therapies meet the realities of coverage, reimbursement and real-world access. Led by Executive and Founder Michael Eging, RAAP brings together patient organisations, life sciences leaders and policy stakeholders to address the structural barriers embedded in Medicare, Medicaid and state-level systems that can delay or deny treatment to patients who have already waited years for a diagnosis. Shaped by lived experience and grounded in policy expertise, RAAP has become a critical voice in reframing access not as a cost problem, but as the final and decisive step in turning rare disease innovation into patient impact.

Could you start by introducing RAAP – its mission, positioning, and current policy priorities?

RAAP is a non-profit coalition bringing together patient organisations, life sciences stakeholders, and other partners to address access challenges patients face after FDA approval. While many organisations focus on pre-approval issues – which are significant – our work concentrates on what happens once a product is approved and patients need real-world access. We focus on state and federal payment and access issues, including Medicare, Medicaid, and other programs.

RAAP began in 2016 as an ad hoc coalition and became a non-profit in 2018. It started as a grassroots effort with people coming together around shared needs. My involvement is deeply personal – my father died of a rare disease while I was in college. Being able to apply my experience in life sciences to tackle the same questions my family faced – how to access care, what treatments exist, and what a diagnosis truly means – has been particularly meaningful.

That lived experience is reflected across the RAAP team. Many of our staff members have been personally affected by rare disease – through a child, grandchild, or other family member. We bring not only professional expertise, but a personal understanding of the challenges patients and families face.

You mentioned FDA approval is just the first step. What are the biggest disconnects between approval and patient access?

The largest disconnect is that much of the healthcare system is structurally designed to say “no.” Rare disease therapies require tremendous effort to move from discovery through Phases I, II, and III, often with Phase IV commitments. That takes enormous risk, investment and collaboration.

Yet once a therapy receives FDA approval, the response often shifts to: “We are so excited you are on the market, but…” Patients then face prior authorizations, step therapy, and lengthy formulary reviews. Some therapies can take 18 to 36 months, or longer, to gain access through Medicaid programs.

The dissonance is stark. We invest to demonstrate life-changing benefits, but the post-approval system prioritizes cost containment over access. Patients face the fundamental questions: Can I get diagnosed? If I am diagnosed, is there a treatment? If so, can I actually access it through insurance, Medicaid, or Medicare?

How does the fragmentation of the US payer system, across commercial insurance, Medicare, and Medicaid, affect patient access compared with more centralised European systems?

Many rare disease therapies approved in the US never launch in other markets. Patient populations are extremely small, and navigating the regulatory and access frameworks outside the US are uncertain. Even in European single-payer systems, innovation does not always translate into

access.

For patients in the US, navigating Medicaid, Medicare, and commercial insurance is complex. Each program has different requirements, and patients often need substantial support. RAAP connects patients with these resources while advocating to address policy barriers.

I saw this first-hand during the launch of a rare pediatric epilepsy therapy. FDA approval was achieved, and children could finally access the treatment. But as we navigated Medicaid, in one state the therapy was routed through an ophthalmology P&T committee. There were no rare disease specialists involved. The result was a burdensome prior authorisation process that discouraged clinicians and families.

These disconnects are common. Some advocacy organisations have strong voices, but many lack resources to sustain advocacy or even alert patients to barriers they will face.

Given the expertise gaps on review committees and the insurance and policy barriers you described, what policy priorities is RAAP focusing on today?

Our priorities focus on removing post-approval barriers. One example is model legislation requiring states to embed patients and rare disease specialists in decision-making. Beyond rare disease advisory councils, which organisations like NORD support, this legislation would require P&T committees, DUR boards, or PDABs to include a patient, a patient advocate, and a physician with relevant rare disease expertise **as a voting member** whenever a rare-indicated therapy is under review.

This builds on lessons from CURES 2.0, where patients were embedded throughout the therapeutic journey – contributing to disease histories, supporting clinical trials, aiding recruitment, and interpreting data for real-world impact. That experience should carry forward into access and coverage decisions.

By integrating patients and clinicians into these discussions, we connect scientific and regulatory understanding with lived experience. The result is better-informed coverage decisions and a smoother patient journey beyond FDA approval.

Does patient-led development translate into patient involvement in access decisions, and how are payers responding?

At RAAP, patients are genuinely at the table. We have a Patient Engagement Caucus that is patient-led, and our state and federal committees are co-chaired by life sciences and patient advocates. Many issues originate from the patient caucus and are elevated through that structure.

We support patients through education – webinars, white papers, and outreach. We also bring them to policymaker meetings, for example, through the National Conference of State Legislators or Women in Government, so they can discuss gaps and potential solutions.

Topics include legislation, prescription drug affordability boards, and issues such as co-pay accumulators, where pharmacy benefit managers collect funds without crediting patients' annual out-of-pocket costs. Our approach is patient-centred, from staff structure to advocacy at state and federal levels.

Why has the exclusion of rare disease therapies from the Inflation Reduction Act become controversial, and what does it reveal about how cost and value are being measured?

We have been involved with the tools that became the IRA since the Biden administration introduced them. Initially part of the Build Back Better toolkit, early analysis looked at broad system-level impacts and predicted some product loss and creation of some access challenges.

Then we asked a different question: what happens when these tools are applied to rare diseases, especially therapies serving fewer than 100 patients? Whether it was increased Medicare Part D rebates, Part B rebates, or drug price negotiation, we modelled the impact on very small populations.

We found reductions in investment, decreased access post-FDA approval, and fewer products progressing through the pipeline. A therapy that is never developed is, by definition, an access issue. We also examined whether price controls could create a European-like market dynamic, where 30 to 50 percent of products never launch.

This helped educate policymakers that investment, development, and access dynamics for rare and ultra-rare diseases differ from chronic conditions. Congress recognised this, and carve-outs for rare disease began with the IRA and were clarified further in the Big Beautiful Bill.

Much of my work focuses on ultra-rare conditions. I have been involved in launches for therapies serving fewer than 300 patients and worked with investors balancing innovation with small

populations. These policy tools can disrupt planning and discourage investment. If a therapy does not make it through the pipeline, there is no opportunity to address access with Medicaid.

The Orphan Drug Act (ODA) is foundational for rare disease. How does it function in today's environment?

Senator Orrin Hatch, the lead sponsor of the ODA, was my mentor after college. The ODA was deeply personal – my father died of a rare disease without a treatment.

Today, 40-plus years later, the landscape is different. There are therapies even for my father's cancer. A family friend lived nearly 18 months beyond diagnosis, compared with my father's six months. That is real progress.

The ODA provided the foundation, but accelerated approval pathways, surrogate endpoints, streamlined clinical programs, and pediatric rare disease vouchers have transformed the development landscape. They allow developers to ask if pursuing a therapy for 200 patients can both impact lives and generate a return on investment.

The mix of incentives matters – CURES, CURES 2.0, and subsequent efforts address erosion of the ODA from the IRA. Additional steps can be taken, and the administration is exploring them.

There are proposals to restrict Medicaid reimbursement for accelerated approval therapies, arguing that evidence is insufficient. In rare disease, particularly ultra-rare conditions, traditional trials may be impossible. These tools exist to deliver life-changing treatments.

I was involved in launching a gene therapy for spinal muscular atrophy. Historically, many children died before age two. Today, some are attending kindergarten. This demonstrates that early patient engagement, combined with thoughtful policy and access planning, doesn't just accelerate approval – it ensures therapies actually reach the patients who need them. Going forward, additional tools, including AI-enabled modelling and smarter priority review mechanisms, can further accelerate discovery and improve both outcomes and access.

How far have outcomes-based and risk-sharing payment models progressed in rare disease, and what would it take to scale them meaningfully in the US?

We operate in a 21st-century system built largely in the 1990s and early 2000s, with remnants from the 1970s and 1980s. That system struggles to absorb personalised therapies.

For rare disease, we need models that stabilise the marketplace and ensure patients can access therapies at FDA approval. Value-based pricing and payment over time, particularly for cell and gene therapies, are increasingly important. Early pilots with NEWDIGS in New England showed real potential.

I am especially interested in Medicaid reinsurance and secondary reinsurance markets to stabilise costs. By integrating AI and analytics, we can improve coverage decisions, manage costs, and understand patient populations. Too often, reinsurance is treated as an afterthought. Integrated properly, it can be transformative.

Arizona offers an example. Since the 1990s, the state has led in risk pooling and innovative financing. Rare disease is like a genetic lightning strike, but it is insurable – much like catastrophic events where insurance models already exist. We need systems that provide the right coverage at the right time, ensuring patients receive transformative therapies like gene therapies without unnecessary delays.

Delays can be devastating. Imagine launching a gene therapy and Medicaid takes 24–36 months to decide on coverage. Infants may age out of the label before treatment. Rare disease patients cannot wait. With 10,000 identified rare diseases and fewer than five percent having therapies, sustainable access models are critical to ensure research, development, and FDA approvals translate into real patient impact.

To what extent do state-by-state differences in Medicaid coverage create access barriers, and is greater national alignment realistic?

Medicaid is a federal–state partnership, not a single national program. If you have seen one Medicaid program, you have seen one Medicaid program. States operate under different waivers, payment models, coverage criteria and administrative rules, which creates significant variation in how care is accessed and reimbursed.

While there are some common elements – such as shared vendors for P&T committees – policymaking, implementation and enforcement are largely state driven. That flexibility can be useful, but it creates real challenges for rare disease patients.

Those challenges are most acute when care is not available in-state. Many rare disease patients, particularly pediatric patients who are more likely to rely on Medicaid, must travel across state lines to access centers of excellence. When they do, reimbursement rates, coverage criteria, prior authorization requirements and drug acquisition costs often differ. That creates an administrative burden for providers and significant exhaustion for patients and families.

The fragmentation becomes even more problematic when states introduce policies such as prescription drug affordability boards or plans launch co-pay accumulator programs. Further, if a prescription drug affordability board introduces an upper payment limit that does not cover the cost of acquiring a rare disease therapy, the financial risk shifts to providers or patients. Pharmacies may not stock the therapy, providers may be unable to administer it, and patients are left scrambling for alternative coverage, charity programs or foundation support.

From the patient perspective, this results in delayed treatment, non-medical switching and, in some cases, families relocating to states with more favorable coverage. There are ongoing discussions about aligning eligibility and access criteria across states, but today the system remains a patchwork. That fragmentation is a material barrier to access and a major source of burden for the rare disease community.

What other topics need to be further emphasized?

One area I would emphasize is the growing uncertainty patients face as a result of recent policy initiatives. Between the IRA, proposed fixes in Congress, and state-level price control mechanisms such as prescription drug affordability boards and most-favoured-nation pricing, we are seeing real instability in coverage. A patient who has access today may not have access tomorrow.

We are already seeing plans state explicitly state that they will not cover MFP, UPL or MFN-priced products. When those policies are imposed at the state level, patients are left scrambling, often with no viable alternatives. That uncertainty is not theoretical; it is happening now.

The issue is not whether costs matter – they do – but whether short-term, politically driven decisions actually reduce patient costs or simply shift them elsewhere. Too often, these policies create cost-shifting rather than savings, leaving patients worse off, particularly when a specific therapy is their only remaining option.

We need to step back and re-orient the discussion around long-term solutions that provide certainty of coverage. If patients are placed at the center of these conversations, it is possible to

lower out-of-pocket costs while maintaining a viable marketplace for plans and manufacturers and managing risk in rare disease coverage.

There are constructive paths forward: delinking patient cost-sharing from list prices, moving away from percentage-based PBM mark-ups toward capped fees, and designing payment models that reduce friction across the system. These are solvable problems, but they require collaboration rather than political point-scoring.

One lesson I learned working with Senator Hatch is that progress requires engaging even where there is disagreement. If policymakers are willing to bring in real-world data, the patient voice and a willingness to compromise, we can arrive at solutions that genuinely benefit patients. That is ultimately the benchmark that should matter.

What is your final message?

Many in your audience are pharmaceutical executives, and they already know rare disease patients well through development programs, clinical trials and FDA engagement. These patients are informed, articulate and deeply invested in their therapies.

Once a drug is approved, I would strongly encourage companies to support and empower those patients to stay involved. Advocacy does not stop at approval. If you have invested years of scientific effort, human capital and patient participation to bring a therapy to market, it makes no sense for access to become the next battleground without the patient voice present.

These patients are ready to engage. They understand the therapy, its impact and the stakes. Linking them with organisations such as RAAP and others working on access and policy ensures that the people most affected are part of the conversation. That is how we make sure the innovation your teams deliver actually reaches the patients it was designed to help.

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