PR

THE PRIORITY REVIEW VOUCHER

"I'VE SEEN HOW RESEARCH CHANGES FUTURES."

PATIENTS RISING

FROM RED TAPE TO RESEARCH

THE PRV IS ONE OF THE STRONGEST TOOLS KEEPING RARE DISEASE THERAPIES MOVING

LET'S GIVE KIDS A CHANCE

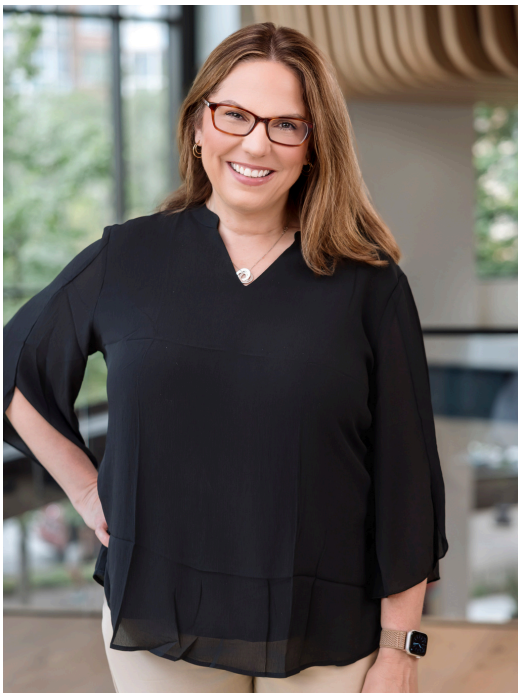
The Priority Review Voucher

Patients Rising supports the reauthorization of the Rare Pediatric Disease Priority Review Voucher program through the Give Kids a Chance Act, because incentives for innovation mean little if promising therapies never reach the children who need them. For many rare pediatric conditions, barriers begin long before treatment. Families face limited research investment, few opportunities for clinical trials and conditions that remain outside the development pipeline. Without meaningful incentives, these diseases are left in the shadows while children wait for progress that does not come.

The PRV program was designed to shift that reality. By awarding companies a voucher for a faster FDA review after approval of a rare pediatric therapy, it gives developers a reason to take on conditions the market alone would not support. Since its creation, more than 30 therapies for pediatric rare diseases have won FDA approval with the support of a voucher. More than 200,000 patients across 47 rare diseases have already benefited, and over 90 percent of those vouchers went to conditions that previously had no treatments. Without reauthorization, the momentum built over the last decade is at risk.

In this issue of PR Magazine, you will learn about families who illustrate why the PRV matters:

- Amanda and her son Raylen, who lives with Glycogen Storage Disease. His care depends on glucose monitors and feeding schedules, yet no therapy exists to treat the root cause.



Co-Founder + Chief Mission Officer
Terry Wilcox

The PRV program represents hope that research will deliver more than daily workarounds.

- Jennifer and her son Rocco, diagnosed with Prader-Willi syndrome before his first birthday. He is now in a clinical trial, and the PRV program ensures that if the therapy succeeds, it has a path from research to approval.

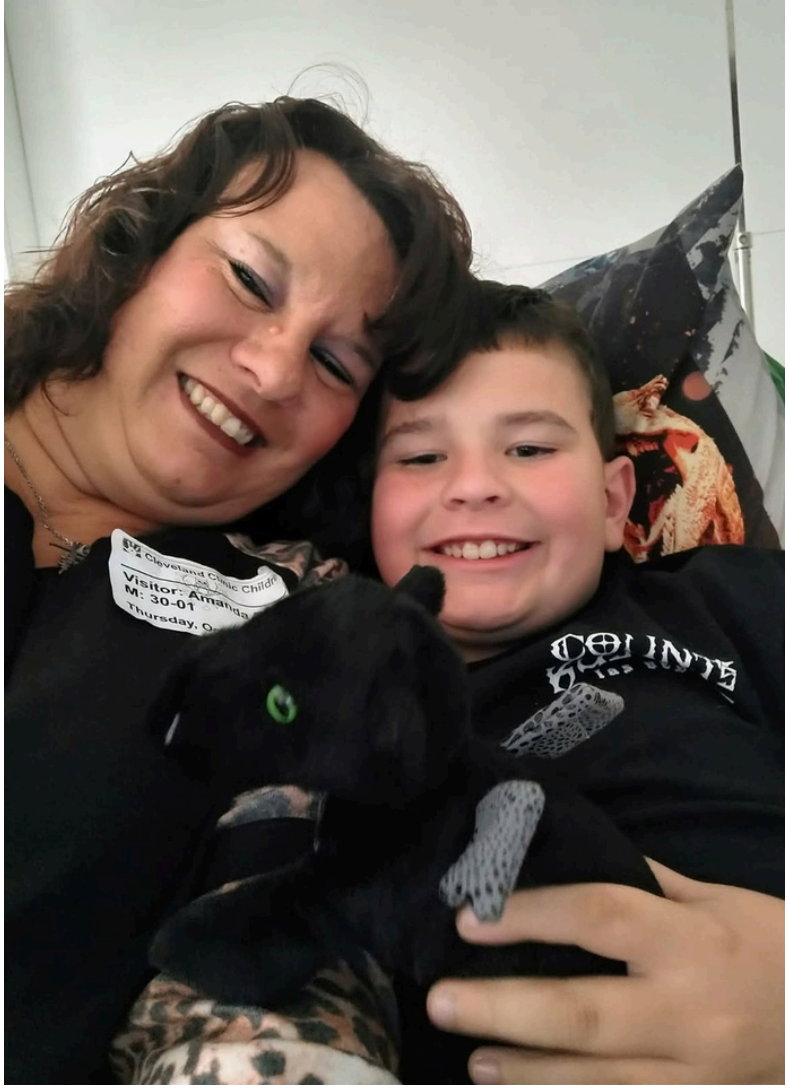
- Katie and her son Cade, also living with Prader-Willi syndrome. Their story highlights how fragile coverage can be, even with private insurance and Medicaid. The PRV program drives the research that could provide a treatment no insurance system can currently deliver.

Reauthorization is also about stability. Rare disease research takes years, sometimes decades, and companies will not commit to that kind of investment if programs that make it feasible are at risk of disappearing every few years. The Give Kids a Chance Act provides the consistency needed to keep research moving, so families are not left wondering if promising science will lose support halfway through development.

The program's reach extends beyond the lab. Every therapy that advances under the PRV creates knowledge, builds infrastructure and strengthens the overall pipeline for rare conditions. Even when treatments target small populations, the lessons learned in discovery, trial design and approval benefit the broader medical community. This ripple effect is one of the most overlooked strengths of the PRV, yet it is central to why it must continue.

Looking ahead, the challenge is not whether the science exists, but whether the incentives remain strong enough to carry it forward. Families like those featured in this issue know what it means to live in the gap between possibility and reality. The PRV is one of the few tools that narrows that gap. Reauthorizing it through the Give Kids a Chance Act means keeping open the only door many children have to a different future.

PRVS TURN DAILY SURVIVAL INTO FUTURE PROGRESS

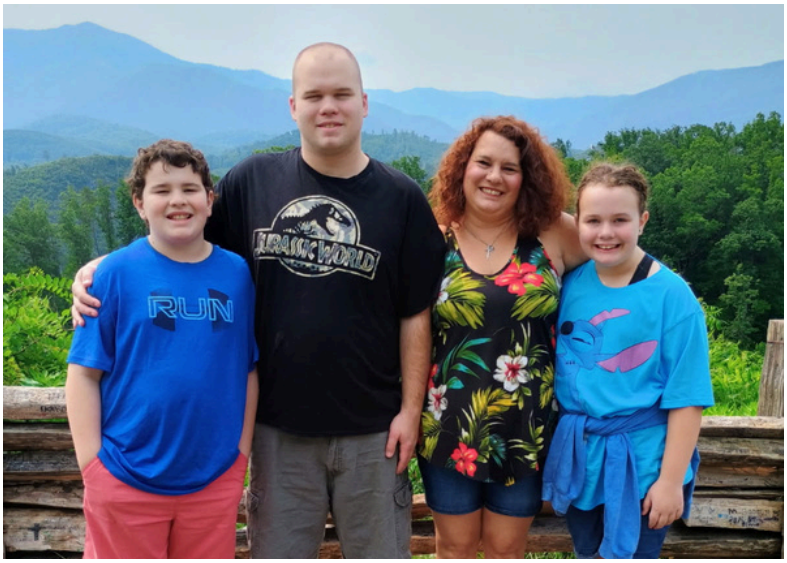


Morning starts with numbers. Amanda checks her son Raylen's blood sugar before he's fully awake, making sure his body has enough energy to carry him into the day. His schedule is strict, his feedings timed to the minute, his medical supplies always within reach. Ketone strips, glucose monitors and prescription supplements line the counter like permanent fixtures in the kitchen. This is life with Glycogen Storage Disease, a rare genetic condition that disrupts how the body stores and releases sugar.

Managing the condition is constant, but Amanda has learned to keep pace. What she cannot control is the system wrapped around it. Raylen's coverage comes through his father's employer, a national grocery chain that provides the insurance, owns the pharmacy and runs the pharmacy benefit manager. With so much power in one place, access can collapse over something as simple as a prescription.

When Raylen's doctor ordered liquid Vitamin D and iron, the pharmacy refused to fill it. Because he is a child, pills were not an option, yet Amanda was told to buy the liquid off the shelf. Once she did, the purchase coded as groceries, not medicine, and her Health Savings Account card was declined. The prescription sat in her hand, but the system had no way to recognize it.

"Families like mine are already exhausted from navigating a system that wasn't built with us in mind," Amanda said. "We fight to get insurance approvals. We fight to access specialists. We fight to afford the medications that keep our children alive. And far too often, we're fighting alone."

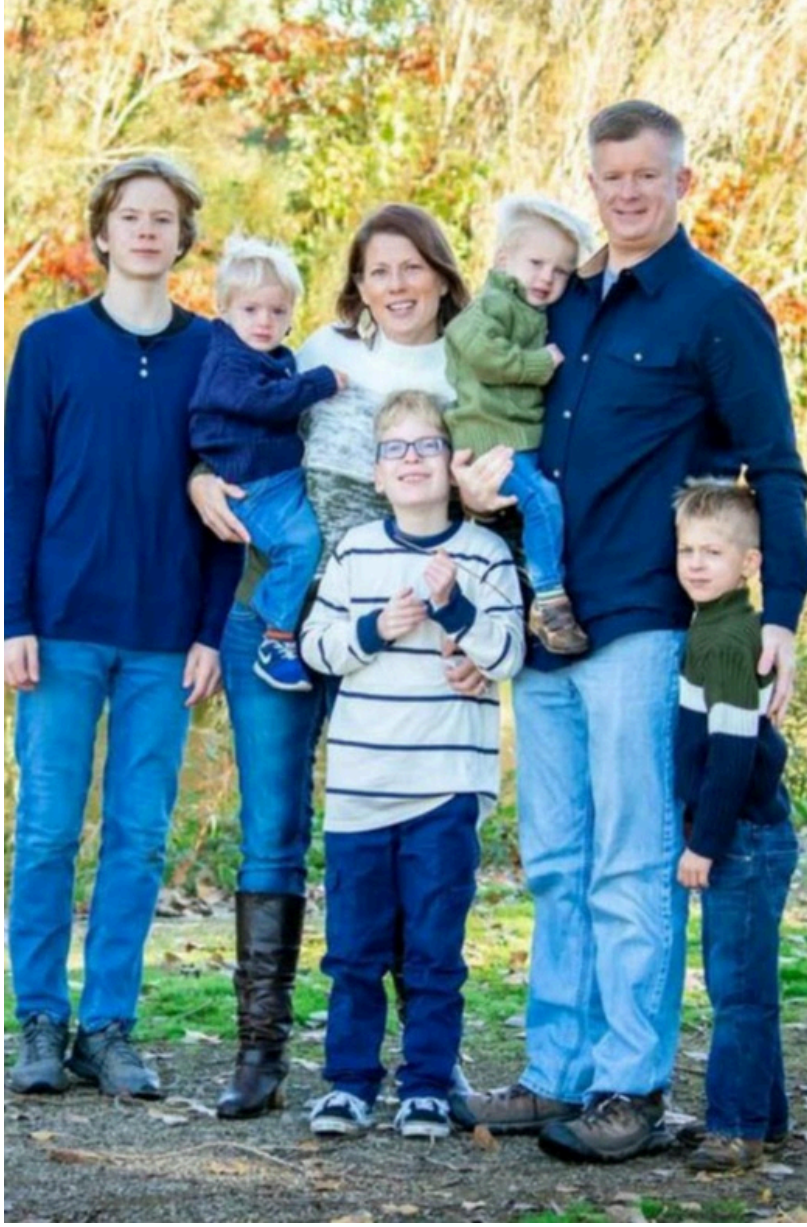


That kind of breakdown is common in rare disease care. Families spend hours navigating coverage rules and financial traps, but none of it moves the science forward. Glycogen Storage Disease has no approved therapy that treats the root cause. Families manage symptoms, but the disease itself remains unchanged.

That is why the Rare Pediatric Disease Priority Review Voucher program matters. The PRV creates an incentive for drug developers to take on conditions the market would otherwise leave behind. It changes the calculation from impossible to possible. Since the PRV program began, more than 30 therapies for children with rare diseases have won FDA approval with the support of a voucher. These are therapies that once had little commercial hope but now reach families who were told for years that nothing could be done.

Amanda has seen the toll the current system takes. "I've seen the bills. I've seen the denials. I've stayed up all night researching alternatives, talking to other parents, calling financial assistance programs to do whatever it takes to keep my child safe and my family afloat," she said. "The PRV keeps research alive when nothing else does."

YOU CAN'T APPEAL YOUR WAY TO A CURE, BUT PRVS CAN GET YOU CLOSER



Katie Moureau's daughter, Cade, lives with Prader-Willi syndrome, a rare genetic disorder that requires continuous and highly specialized care. Cade sees multiple providers, needs carefully tailored medications and depends on steady insurance coverage. Medicaid was meant to bridge the gaps, yet it often added another layer of barriers.

"The hardest part isn't the paperwork," Katie said. "It's finding doctors who understand her condition and are actually in-network."

The family's private insurance helped shield them from the worst delays, but Medicaid was supposed to expand Cade's access, not complicate it. Even after approval, Katie found herself locked in a cycle of advocacy, appeals and negotiations for services already deemed medically necessary.

"You expect that once you're approved, care will be straightforward," she said. "But it isn't. You're always in a position to prove your child deserves the care the system has already agreed to cover."

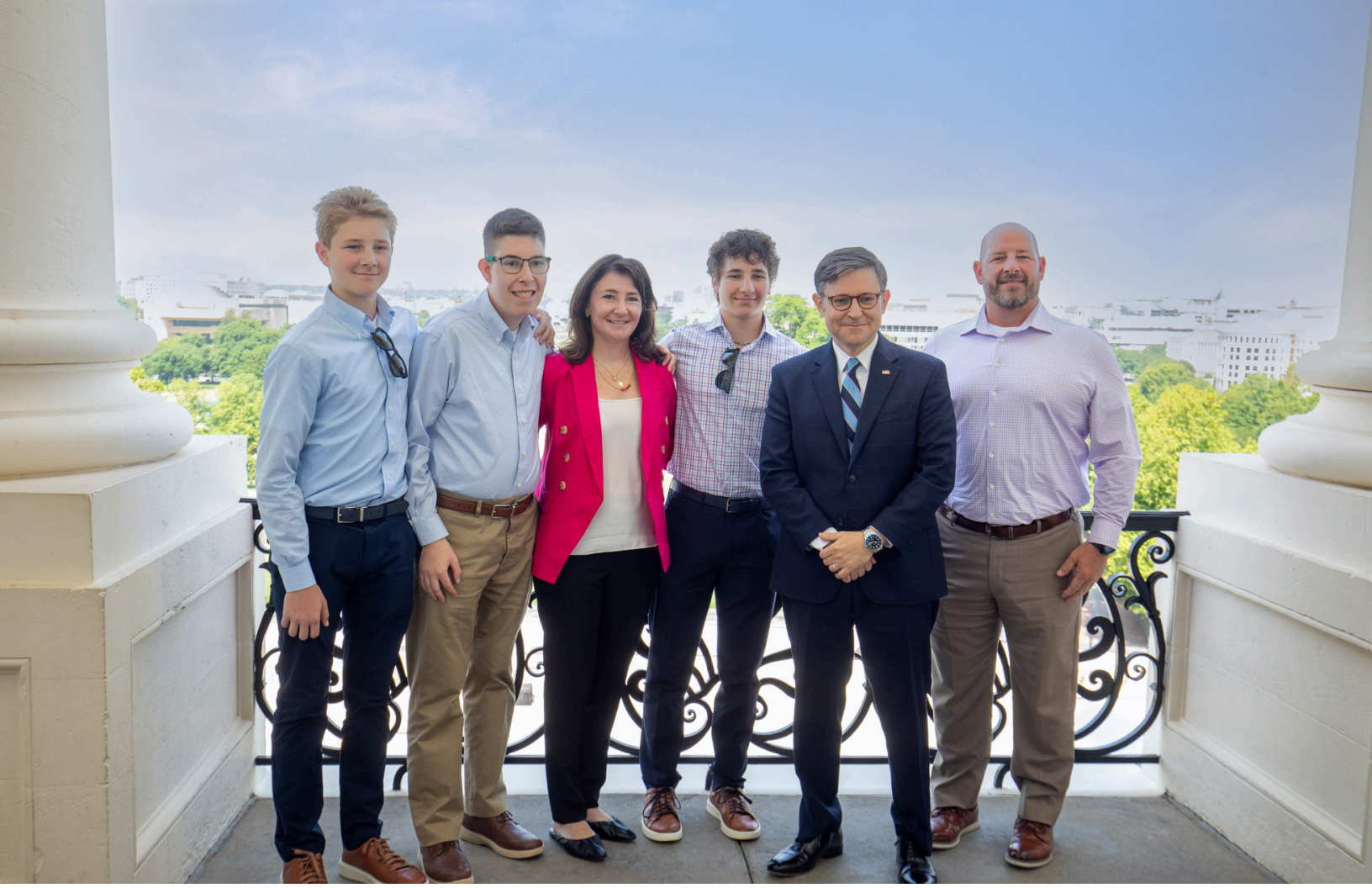
Prescription access is another obstacle. Pharmacies that accept the family's private plan often do not take Medicaid, leaving Katie with the exhausting task of finding one that works with both. And now with a new pharmacy benefit manager controlling access, authorizations are delayed, formularies shift without warning and there is no process to accommodate children with rare conditions.

For Cade, stability is everything. When medications lapse or therapies stall, the setbacks are immediate. Katie has learned to fight for every piece of her daughter's care, but she also knows there are limits to what any parent can fix. No amount of paperwork or appeals can make up for the absence of real treatments that target the underlying disease.

That is where the Rare Pediatric Disease Priority Review Voucher program comes in.

The PRV gives companies a reason to take on conditions like Prader-Willi syndrome that have been historically overlooked. It pushes development forward by making investment in rare pediatric therapies viable, ensuring that promising science does not stall in the pipeline.

The evidence is clear. More than 200,000 patients across 47 rare diseases have already benefited from therapies supported by PRVs, and over 90 percent of those vouchers went to conditions with no prior FDA-approved treatments. For families like Katie's, that progress represents more than policy. It represents a path toward therapies that could reduce dependence on fragmented systems and allow children like Cade to thrive.



THE VOUCHER THAT TURNS RARE DISEASE RESEARCH INTO REALITY

When Jennifer Garzia's son Rocco was born, the differences showed almost immediately. His muscles lacked tone, his movements were minimal and feeding did not come naturally. He was quiet in a way that felt clinical rather than calm, and those early signs led doctors to act quickly.

Within 30 days a geneticist delivered the diagnosis: Prader-Willi syndrome, a rare disorder caused by the loss of expression of certain genes on chromosome 15. For Jennifer, the diagnosis was both a relief and a reckoning. It gave a name to the symptoms yet it opened the door to a maze of specialists, therapies and systems that were never designed with children like Rocco in mind.

Prader-Willi syndrome is lifelong and demanding. It affects the hypothalamus, which regulates hunger, hormones and temperature. Children often miss milestones, face cognitive delays and develop an unrelenting drive to eat that requires constant supervision. Families must structure daily life around safety, from locked refrigerators to carefully planned environments.

Early therapies gave Rocco stability, but as the family moved between states, Jennifer learned how fragile that stability could be. In one place disability status guaranteed coverage, in another it did not. At times bureaucracy determined eligibility more than his medical needs. She learned to keep meticulous records, chase down caseworkers and appeal denials. Her advocacy became survival.

Today Rocco is part of a clinical trial for Prader-Willi syndrome. For Jennifer, that trial represents both hope and uncertainty. Every appointment, every test, every progress note carries the possibility of change. But even if the trial succeeds, there is no guarantee the therapy will make it to market. Rare disease drug development is costly, risky and often abandoned before reaching approval.

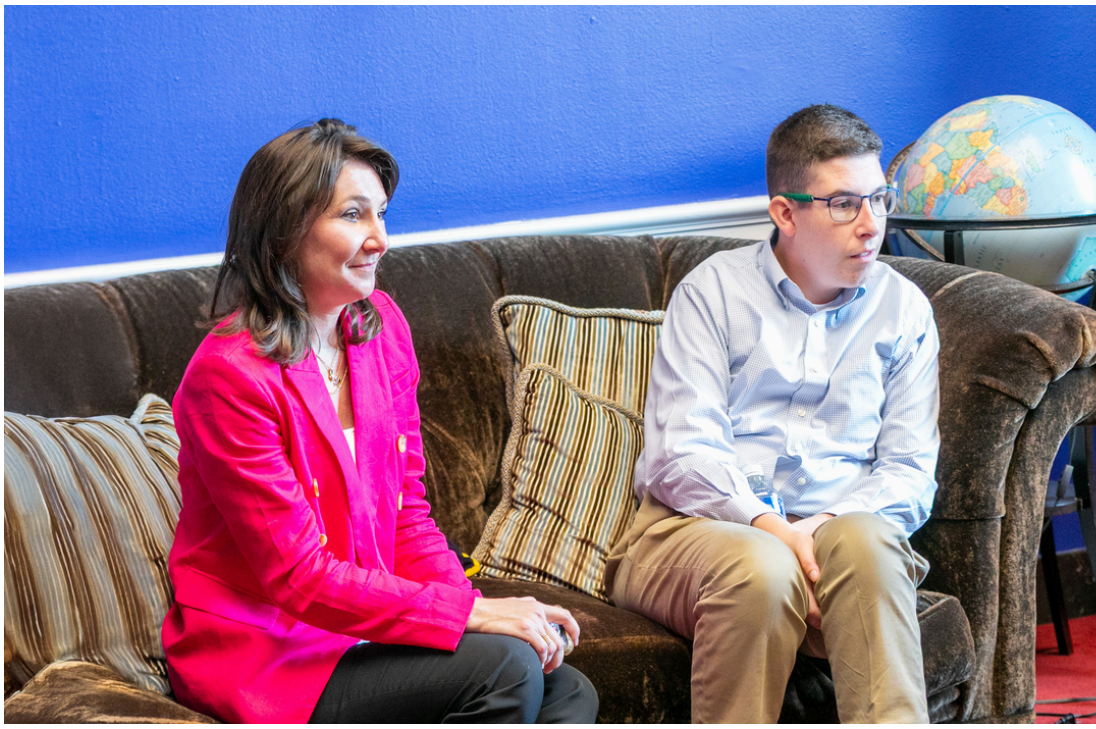
That is where the Rare Pediatric Disease Priority Review Voucher program comes in. The PRV provides a powerful incentive for companies to continue through the hardest part of the journey: moving from a promising clinical trial to an FDA-approved treatment. By offering a valuable voucher for

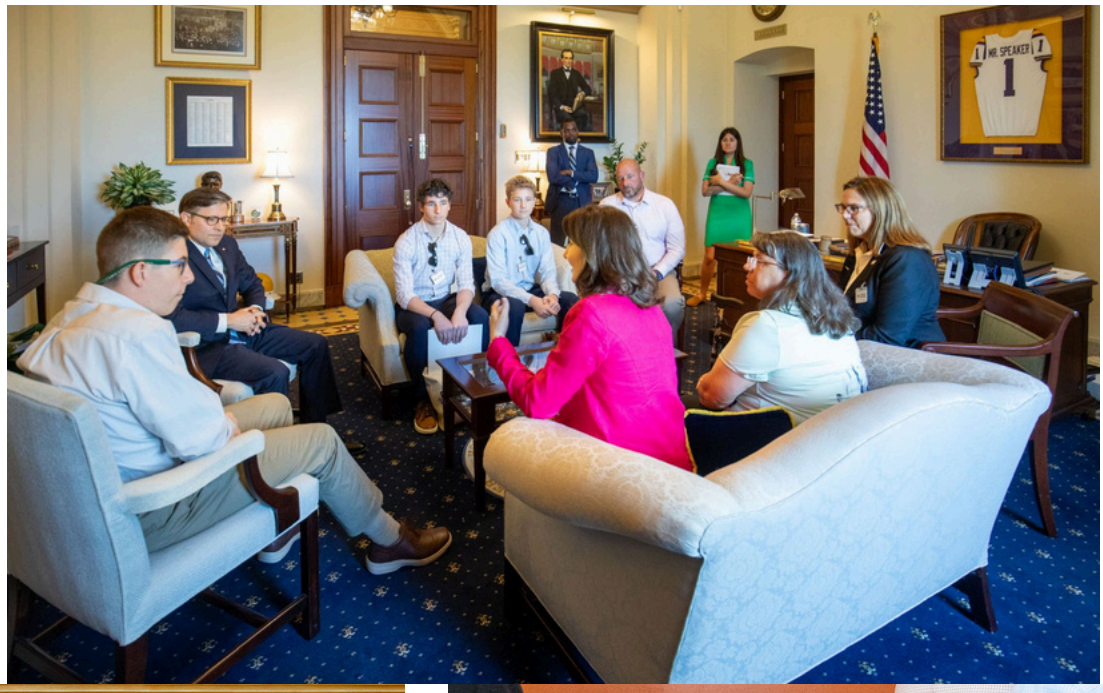
a faster review of another drug once the rare therapy is approved, the program makes the investment viable. It helps keep rare disease therapies from stalling out in the final stretch.

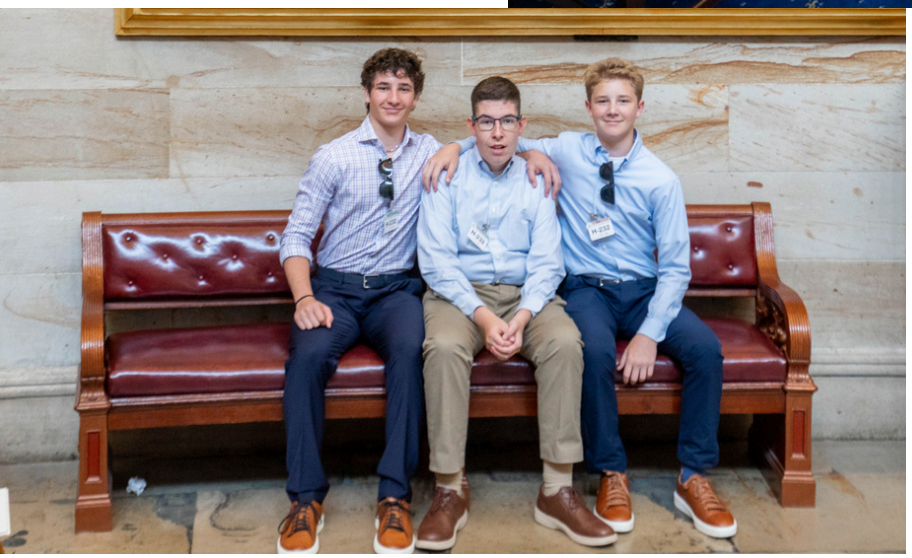
The impact is not abstract. More than 200,000 patients across 47 rare diseases have already benefited from therapies supported by PRVs, and over 90 percent of those vouchers were awarded for diseases that had no approved treatments before. Without this program, many of those therapies would still be on the sidelines.

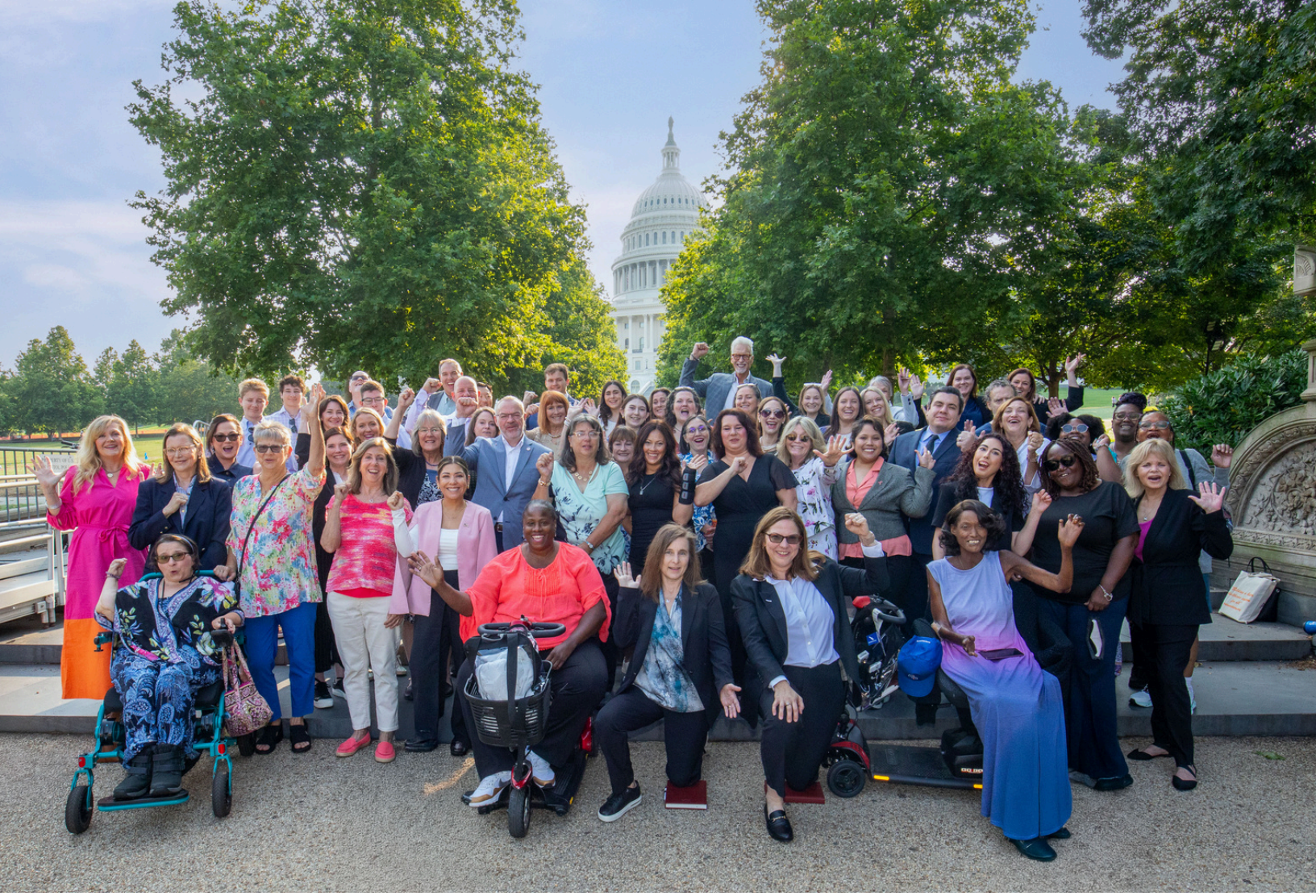
For Jennifer, the stakes are deeply personal. If Rocco's trial proves successful, the PRV could mean the difference between a therapy that sits in limbo and one that reaches the children who need it. It could mean that years of research translate into real change for families like hers.

"As Rocco's mom and one of the 1 in 15,000 families living with his rare disease, I have seen how research changes futures. Priority Review Vouchers keep companies investing in rare conditions and speeding potential treatments. Reauthorizing PRVs preserves that hope and momentum while delivering impact for the public good without any direct cost to taxpayers."









PATIENTS RISING

Empowering every patient in America to advocate for reforms placing them, alongside their doctors, in control of their healthcare choices.

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