PR
PATIENTS RISING
PRADER-WILLI AWARENESS

THE SCIENCE BEHIND PWS IS COMPLEX. ACCESSING CARE CAN BE EVEN HARDER.

THE ADULTHOOD THAT COMES WITH DENIAL LETTERS

FOR ADULTS LIVING WITH PRADER-WILLI SYNDROME, TURNING 18 CAN TRIGGER A NEW FIGHT OVER GROWTH HORMONE THERAPY

FROM THE FOUNDER

Why Families Living with Prader-Willi Syndrome Deserve Better

When most people hear the phrase "rare disease," they think of statistics. A small patient population. A condition they may never personally encounter. What they often do not see is the reality inside the homes of families living with Prader-Willi syndrome, where caregiving becomes a 24-hour responsibility shaped by medical complexity, constant supervision and systems that are not built to respond quickly or compassionately.

For many families, daily life revolves around routines that are necessary for safety and survival. Food security inside the home often requires locks, alarms and constant vigilance. Medical care can involve endocrinologists, behavioral specialists, nutrition support, therapists and ongoing coordination between schools, providers and insurers. Parents are not simply caregivers. They often become care coordinators, case managers, advocates and crisis navigators all at once.

And yet, despite the intensity of what these families manage, they are too often met with barriers at every turn.

Prior authorizations delay treatment. Insurance formularies create uncertainty around access to medications and therapies. Provider shortages leave families traveling long distances for specialty care. Caregivers are left fighting systems that frequently underestimate the seriousness of a condition because its challenges are not always visible from the outside.

At Patients Rising, we understand that

healthcare policy is not abstract for these families. It shapes whether a child can access care on time, whether a parent can keep working while managing appointments and whether support systems remain financially sustainable long term. That is why we believe advocacy matters.

We have seen firsthand what happens when caregivers and parents are given the tools and platform to tell their stories. Policymakers stop seeing a diagnosis on paper and start understanding the human impact behind delayed access, restrictive coverage and fragmented care. Real stories change the temperature of a conversation in ways statistics alone often cannot.

Families living with Prader-Willi syndrome should not have to become policy experts just to protect their children. Yet many have been forced into that role because the stakes are too high not to speak up.

Patients Rising remains committed to supporting rare disease advocates by helping elevate their experiences, connect them with educational resources and ensure their voices are heard in healthcare conversations that directly affect their lives. Caregivers and parents in the PWS community carry extraordinary responsibility. They deserve systems that recognize that burden instead of adding to it.

Too often, families affected by rare diseases are expected to navigate some of the most complicated parts of the healthcare system with the fewest resources and the least margin for error. Parents of children with PWS are making high-stakes decisions every single day while balancing emotional strain, financial pressure and long-term uncertainty about the future. Advocacy does not remove those challenges overnight, but it creates visibility, accountability and momentum, which are all necessary if meaningful change is going to happen.

Yours in Advocacy,

Terry Wilcox

Terry Wilcox
Co-Founder and Chief Mission Officer
Patients Rising

FOR ADULTS WITH PWS, ADULTHOOD CAN TRIGGER A FIGHT FOR CONTINUED CARE

By: Jennifer Garzia

For families living with Prader-Willi syndrome, adulthood can become the beginning of a new healthcare crisis.

My son Rocco is 21 years old, and he has Prader-Willi syndrome, a rare genetic disorder that affects metabolism, muscle tone, development and hunger regulation. Families raising children with PWS learn quickly that maintaining health requires far more than routine medical care. Therapies that support muscle development, body composition and overall physical stability often become part of everyday life from early childhood forward, which is why growth hormone therapy has remained such an important part of his care.

For many in the PWS community, Genotropin became a remedy that has helped improve both health outcomes and life expectancy. Decades ago, families were often told individuals with PWS might only live into their twenties, but advances in medicine, earlier intervention and more consistent treatment approaches have helped many individuals with PWS live longer, healthier lives into adulthood.

Healthcare policy and insurance structures, however, still treat adulthood as though those medical realities never evolved.

My son, Rocco, and I met with congressional members in 2025 to discuss our experiences with gaps in his care with Prader-Willi Syndome.

> Because of science, Rocco is afforded the opportunity to age, but I want the quality to be there with the medications that got him there, and TrumpRx offers that backup plan to ensure that therapies aren't interrupted.

Growth hormone therapy received FDA approval for pediatric use in Prader-Willi syndrome years ago, which has allowed families to secure insurance coverage while children were young. Once patients enter adulthood, coverage becomes significantly more unstable. The medication itself does not suddenly lose relevance at 19 years old, yet families often find themselves facing coverage denials simply because their child aged out of the framework insurers are most comfortable recognizing.

That instability carries real consequences for families managing lifelong conditions.

This issue deserves far more attention because medicine has already succeeded in helping many individuals with PWS live longer lives. Public policy and insurance coverage standards now need to acknowledge the reality that these patients are aging into adulthood and still require access to the therapies that helped sustain them.

That is why programs like TrumpRx matter to families like mine.

Genotropin recently became available through TrumpRx at a substantially discounted rate, creating an option many families have never previously had access to during periods of insurance instability.

For some households, that reduced pricing can mean the difference between maintaining continuity of care and abruptly stopping treatment because coverage disappeared.

This conversation should not be reduced to politics or slogans. Families caring for loved ones with rare diseases understand healthcare in practical terms.

We understand what happens when therapies work. We understand what happens when access disappears. We also understand the growing tension between advances in medicine and insurance structures that still operate inside outdated assumptions about survival, adulthood and long-term care.

Because of science, Rocco is afforded the opportunity to age, but I want the quality to be there with the medications that got him there, and TrumpRx offers that backup plan to ensure that therapies aren't interrupted.

That is ultimately what this conversation is about for families like mine. Longer life expectancy only carries meaning if patients are able to maintain access to the therapies that helped make that future possible in the first place.

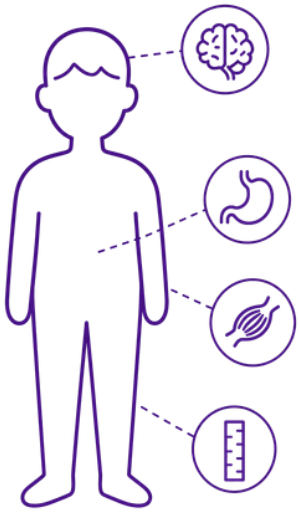
PRADER-WILLI SYNDROME

breaking down the symptoms, science and challenges

ABOUT PWS

Prader-Willi Syndrome is a complex genetic condition that affects many parts of the body, including muscle tone, growth, appetite, behavior, learning ability and more.

It's caused by the absence of function of genes on **chromosome 15.**



BY THE NUMBERS

1 in 15,000
the number of births affected by PWS

PWS affects males and females, as well as all races and ethnicities with **equal frequency.**

COMMON TERMS

HYPERPHAGIA

An intense, chronic feeling of hunger that is a hallmark of PWS and typically begins in early childhood

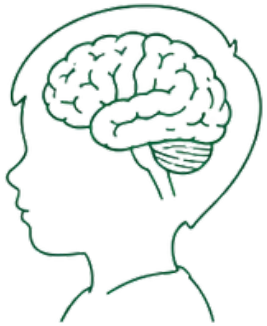
GROWTH HORMONE THERAPY

A common treatment that can improve growth, body composition, muscle tone and overall development.

THE BRAIN SIGNAL

In PWS, the brain has difficulty receiving the stomach's "I'm full" message.

This can lead to an ongoing feeling of hunger, even after eating, making food a constant focus and challenge.



HOW PWS SYMPTOMS EVOLVE
through different developmental phases

INFANCY
0-2 YEARS

Early signs of PWS often begin in infancy and can be subtle at first. Babies may experience low muscle tone, feeding difficulties and delayed developmental milestones, which can make everyday activities like feeding and movement more challenging.

EARLY CHILDHOOD
0-2 YEARS

As children grow, appetite and interest in food may begin to increase alongside developmental and behavioral challenges. Structured routines, therapies and early support services can play an important role during this phase.

CHILDHOOD
6-12 YEARS

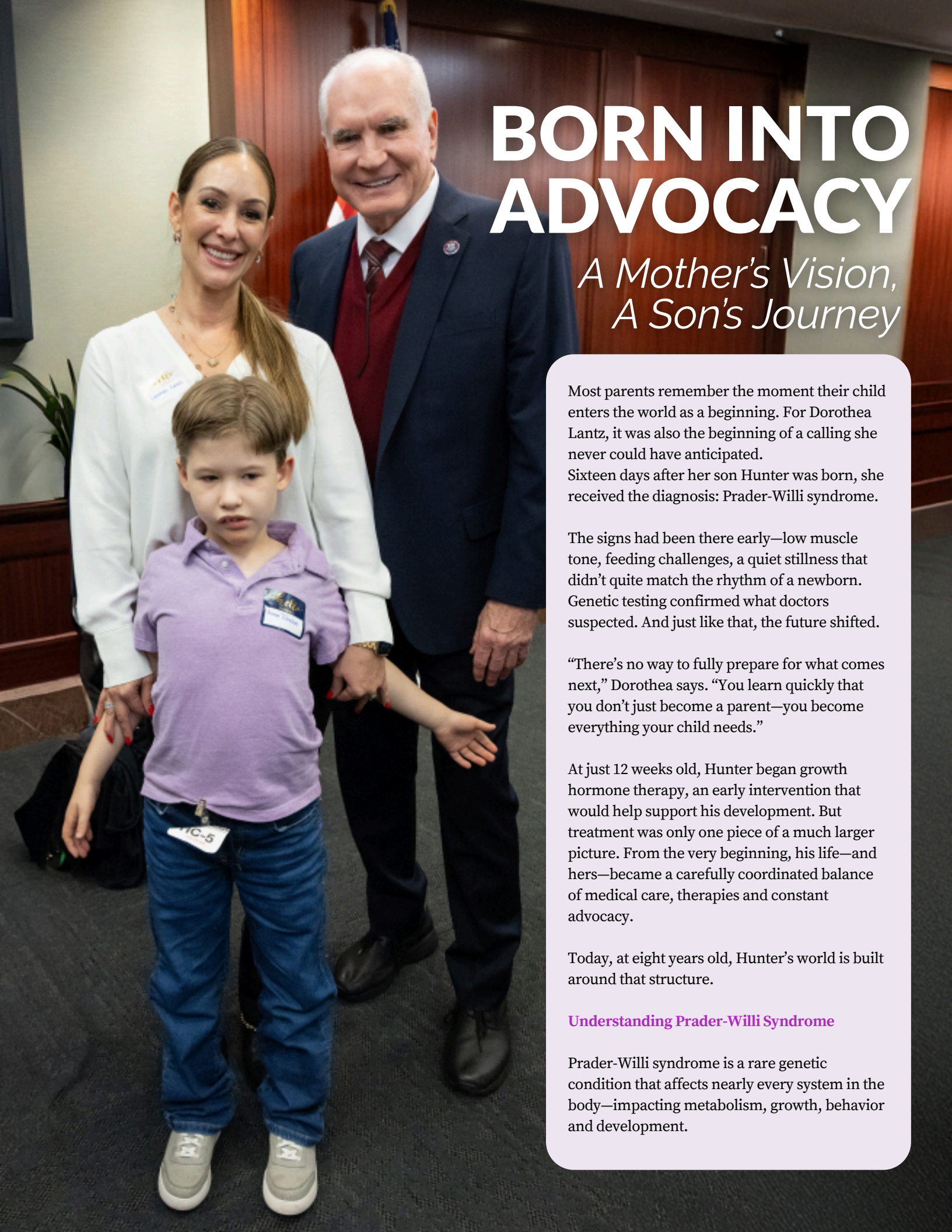
During childhood, food-seeking behaviors and hyperphagia often become more pronounced. Consistent supervision, predictable structure and coordinated support systems are important for safety, emotional well-being and learning development.

ADOLESCENCE
12-18 YEARS

Adolescence can bring increasing emotional, social and physical complexities. Hormonal changes, mental health concerns and the continued need for food security measures often require ongoing support and individualized care strategies.

ADULTHOOD
18+ YEARS

PWS is a lifelong condition that continues into adulthood. Many individuals benefit from structured support systems, coordinated healthcare and environments that promote independence, safety and long-term quality of life.



BORN INTO ADVOCACY

A Mother's Vision, A Son's Journey

Most parents remember the moment their child enters the world as a beginning. For Dorothea Lantz, it was also the beginning of a calling she never could have anticipated.
Sixteen days after her son Hunter was born, she received the diagnosis: Prader-Willi syndrome.

The signs had been there early—low muscle tone, feeding challenges, a quiet stillness that didn't quite match the rhythm of a newborn. Genetic testing confirmed what doctors suspected. And just like that, the future shifted.

"There's no way to fully prepare for what comes next," Dorothea says. "You learn quickly that you don't just become a parent—you become everything your child needs."

At just 12 weeks old, Hunter began growth hormone therapy, an early intervention that would help support his development. But treatment was only one piece of a much larger picture. From the very beginning, his life—and hers—became a carefully coordinated balance of medical care, therapies and constant advocacy.

Today, at eight years old, Hunter's world is built around that structure.

Understanding Prader-Willi Syndrome

Prader-Willi syndrome is a rare genetic condition that affects nearly every system in the body—impacting metabolism, growth, behavior and development.

“Prader-Willi syndrome is more than a medical term—it’s a whole constellation of challenges that touch every part of an individual’s life and an entire family’s life,” Dorothea explains. “It means a hunger signal that never switches off—not an appetite you can satisfy, but a biological drive as relentless as breathing.”

For families, this isn’t simply a matter of appetite. In practical terms, that means food safety becomes part of daily life—locked kitchens, constant supervision and carefully structured routines. But hyperphagia is only one part of the story.

“PWS impacts everything,” Dorothea continues. “It’s low muscle tone and delayed motor skills—the effort it takes Hunter to put on a shirt can feel like a full workout. It’s sleep that is light and fragmented, so a full night’s rest—anything that resembles what you and I know—is rare, often nonexistent. And that makes learning, regulation and just getting through the day that much harder.”

Navigating a Dual Diagnosis: PWS and Autism

Hunter is also living with autism spectrum disorder (ASD), adding another layer of complexity to his daily life.

Hunter is non-verbal, and while he uses an augmentative and alternative communication (AAC) device, his ability to fully access it remains limited.

“Hunter communicates in ways that are uniquely his,” she says. “You learn to listen differently—to understand what isn’t being said out loud, and to celebrate the moments when connection breaks through.”

For Hunter, structure is everything. Predictability. Routine. Consistency. These are not preferences—they are essential for his ability to function, learn and feel secure in the world around him.

A Life Built on Support and Possibility

Hunter attends the Miami Learning Experience School in Miami, Florida—a school designed to meet children where they are and support their individual needs.

His days are full. Purposeful. Structured. A full curriculum structured around learning and therapy: Physical therapy. Occupational therapy. Speech therapy. Applied Behavior Analysis (ABA).

Each discipline works together to support his development. Progress doesn’t always come in big, visible milestones.

Sometimes it looks like a new form of communication. A smoother transition. A moment of calm where there once was frustration.
For Hunter and his family, those moments are everything.

The Hidden Realities of Caregiving

For Hunter, part of this journey means navigating a world that isn't built for children like him. It also means making difficult choices—every single day.

"It means saying 'no' a lot," she says. "No to family gatherings, to birthday parties and really any type of social outing—dozens of times a day or week—until people you've known your entire life just stop asking. And Hunter, like so many of our loved ones, doesn't understand why."

The isolation can be deafening.

"In our family, we say Hunter's challenges aren't just hunger, anxiety or sleep disruption—it's his 'human hard drive,'" Dorothea says. "And yet, inside every person with PWS is the same curiosity, love and humor and heart you'd see in any child. They're just navigating a body and mind that can give off the wrong commands at the wrong times."

Building a Better Future from the Start

That same philosophy guides her work as Chair of the Community Advisory Board for the Florida Sunshine Genetics Program.

This program represents one of the most ambitious rare disease and precision medicine initiatives in the country—an effort designed to transform how rare genetic conditions are identified and treated from the very beginning of life. Spearheaded by the efforts of Representative Adam Anderson and led through the Florida Institute for Pediatric Rare Diseases at Florida State University, the Sunshine Genetics Program is a voluntary newborn screening initiative that uses whole genome sequencing to help identify rare, actionable genetic conditions earlier than traditional

screening methods often allow.

"Programs like this have the potential to change entire trajectories—not just for children, but for families." For many families living with rare disease, diagnosis can take years—a journey often referred to as the "diagnostic odyssey." By the time answers are found, critical developmental windows may already have been lost.

"The goal is simple but life-changing," Dorothea explains. "To shorten or even prevent the diagnostic odyssey so families can access interventions, therapies and care before irreversible harm occurs."

The program has the potential to screen newborns for hundreds of treatable or medically actionable conditions—far beyond what traditional newborn screening currently detects. Florida lawmakers and medical leaders hope the initiative will position the state as a national leader in genomic medicine, pediatric rare disease research and early intervention.

But for Dorothea, the importance of the program extends beyond science and technology.

"As a parent, I know what it feels like to sit in uncertainty while your child struggles and no one has answers," she says. "Programs like this aren't just about sequencing genes—they're about giving families clarity, direction and hope earlier in their journey."

For families living with conditions like Prader-Willi syndrome, where early diagnosis and intervention can dramatically improve outcomes, that shift matters deeply. Because for families living with rare disease, policy is never abstract.

Ensuring Every Voice Is Heard

Advocacy, for Dorothea, is ultimately about people—and about making sure those most impacted are not just represented, but truly heard.

That work includes immersive storytelling training designed to help advocates translate lived experience into meaningful policy conversations.

"There is nothing more powerful than hearing directly from someone living with PWS," Dorothea says. "And it changes how people listen."

To further strengthen their advocacy, PWSA | USA partnered with Patients Rising to develop a first-of-its-kind Advocacy Master Class—an in-depth training program designed to teach families how healthcare policy works and how to effectively engage in shaping it. There's even a PWS-specific Master Class, tailored to the unique challenges of the condition and the real-world barriers families face.

Hunter's story is still being written. It unfolds in school days, in therapy sessions—in quiet moments of connection. It unfolds in the work his mother does to make the world more accessible—not just for him, but for thousands of others. And it unfolds in the belief that while Prader-Willi syndrome and autism shape Hunter's path, they do not define his destination.

"This is what keeps our families fighting," Dorothea says. "For better treatments. For better systems. For a future that matches the potential we see in our children every single day."

RARE DISEASE DOESN'T END AT 18

When Michelle Torbert gave birth to her daughter, Leslie, 28 years ago, she had no way of knowing how profoundly Prader-Willi syndrome would shape the course of their lives. At just three weeks old, Leslie was diagnosed with the rare genetic disorder, a condition that affects muscle tone, development and the hypothalamus, the part of the brain responsible for regulating hunger.

"The doctors explained the diagnosis, but I don't think anyone can fully prepare you for what daily life actually looks like," Michelle said. "You learn as you go, because you have to."

In Leslie's earliest years, life revolved around therapies, medical appointments and developmental support. Many infants with Prader-Willi syndrome require feeding tubes because of low muscle tone and difficulty feeding, but through persistence and intervention, Leslie learned to take a bottle. Physical therapy, occupational therapy, speech therapy and specialized programs soon became part of the family's daily routine.

One of the most misunderstood aspects of Prader-Willi syndrome is also one of the most dangerous: individuals with the condition never feel full.

"The hunger isn't behavioral in the way people assume," Michelle explained. "It's neurological. Leslie's brain does not send the signal that she's full, so safety has to become part of everyday life."

Inside their home, the refrigerator, freezer and pantry remain locked, not as punishment, but as protection.

Outside the demands of caregiving and advocacy, Michelle also built something deeply connected to the way she approaches health and stability inside her own home. Through Torbert Farms, she has spent years educating families about fresh food, nutrition and the impact highly processed foods can have on the body and brain. For individuals with Prader-Willi syndrome, food is never a casual subject, and Michelle says she noticed behavioral changes tied to certain processed foods, artificial ingredients and blood sugar fluctuations long before many people around her understood the connection. Fresh foods, structure and consistency became part of Leslie's care, not as a wellness trend, but as another layer of protection in managing a complex neurological condition.

Years of medical care, family intervention and behavioral support helped stabilize many of those issues, but Leslie still requires around-the-clock supervision. Now, as Leslie approaches her late twenties, Michelle finds herself confronting one of the hardest realities: planning for a future where she may no longer be able to provide care herself.

Through the Prader-Willi syndrome community, Michelle found support and eventually discovered a sense of purpose in advocacy. She realized many families lacked the financial flexibility, resources and support systems her own family had been fortunate enough to access.

"That's why I keep showing up," she said. "Not every family has the same support. Not every family knows where to turn. Advocacy is about making things better for the families coming behind us, too."

Torbert Farms

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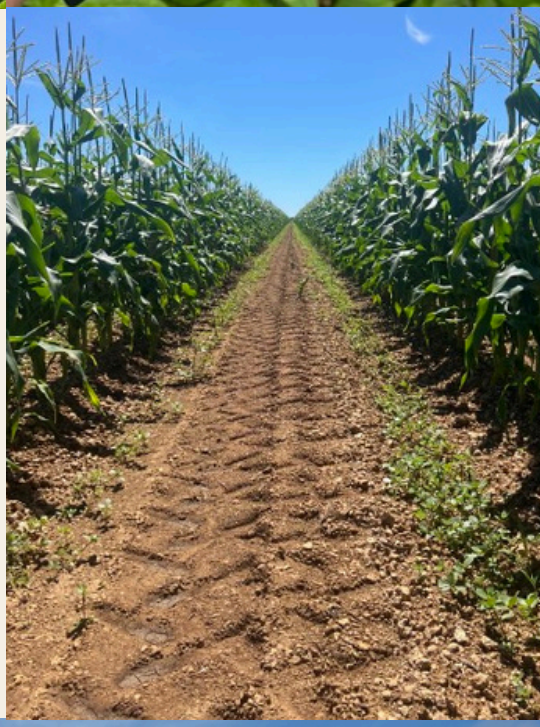
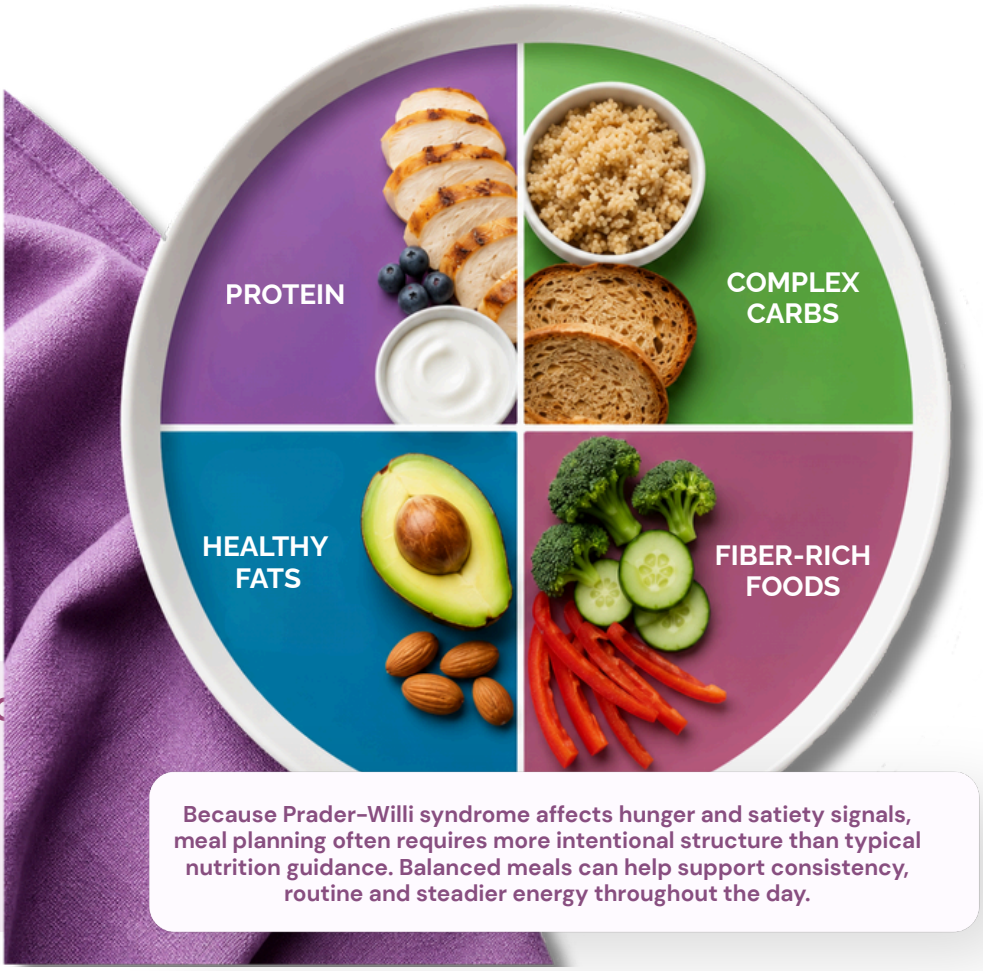


BUILDING STRUCTURE AROUND NUTRITION

How balanced meals support better health for those living with Prader-Willi Syndrome



Because Prader-Willi syndrome affects hunger and satiety signals, meal planning often requires more intentional structure than typical nutrition guidance. Balanced meals can help support consistency, routine and steadier energy throughout the day.

PROTEIN

- Chicken, turkey, fish
- Eggs, Greek yogurt
- Beans, lentils, edamame
- Nuts and seeds

COMPLEX CARBS

- Brown rice
- Oats
- Quinoa
- Sweet potatoes
- Whole grain bread

HEALTHY FATS

- Avocados
- Nuts and seeds
- Olive oil
- Fatty fish (salmon, mackerel, sardines)

FIBER-RICH FOODS

- Vegetables
- Fruits
- Beans and lentils
- Whole grains

Why Structure is Important

For individuals living with Prader-Willi syndrome, meal structure can play an important role in daily health and routine. Predictable meals, balanced nutrition and consistency throughout the day may help support steadier energy, fullness and a more manageable eating environment.

Why Balanced Plates Are Best

Balanced meals often work best when protein, fiber-rich foods, complex carbohydrates and healthy fats are included together. Pairing nutrients instead of relying on carb-only snacks may help meals feel more satisfying and support steadier energy throughout the day.

Small Changes Make a Difference

Nutrition routines do not need to be perfect to be meaningful. Small, consistent habits over time, including meal planning, balanced food choices and supportive routines, can help create a more sustainable approach to nutrition in Prader-Willi syndrome. These habits are building long term results!

WHEN SERVICES CONNECT TO REAL LIFE, FAMILIES CAN THRIVE

When Jennifer Garzia's son was born, the differences were noticeable almost immediately. His muscles lacked tone, his movements were minimal and feeding didn't come naturally. He was quiet in a way that felt clinical rather than calm.

These early signs brought him into the NICU, where he was evaluated by a geneticist and underwent targeted testing. Within 30 days, he was diagnosed with Prader-Willi syndrome, a rare genetic disorder caused by the lack of expression of certain genes on chromosome 15. The diagnosis offered clarity, but it also marked the beginning of a long and complicated relationship with the systems responsible for his care.

Prader-Willi syndrome affects the hypothalamus, disrupting the body's ability to regulate hunger, hormones and temperature. Children with PWS often experience delayed milestones, cognitive challenges and behavioral rigidity, and many develop an intense drive to eat that requires constant supervision.

The condition is lifelong and demands coordinated care across medical, nutritional and educational settings.

In the early years, access to care came more easily. The Garzia family lived in Pennsylvania, where Rocco qualified for Medicaid based on his disability. That coverage allowed for timely therapies, regular evaluations and support that aligned with his diagnosis rather than his family's income. For a condition as complex and multifaceted as PWS, that early access helped stabilize what could have easily spiraled into crisis.

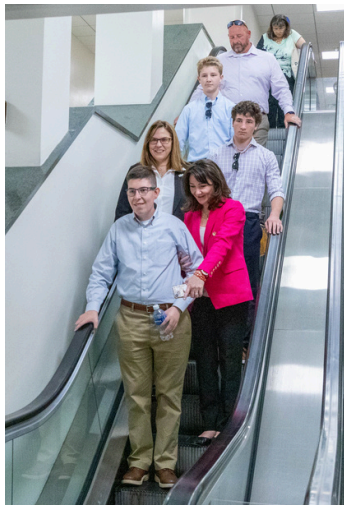
As Rocco grew, new challenges emerged. His behaviors became harder to manage, especially within school settings that were not equipped to respond appropriately. He was enrolled in a special needs program, but the school's approach emphasized compliance over understanding. Restraints were used routinely, and seclusion replaced support. In one incident, Rocco was held down to the point of respiratory distress, so Jennifer pulled him out of the

classroom. She could no longer trust the environment designed to educate him.

The family moved to Florida hoping for a fresh start. Instead, they encountered a system governed by different rules.

In Florida, Medicaid was income-based, and disability status no longer guaranteed access. Jennifer submitted documentation, made phone calls, asked questions and received little in return. The same diagnosis that had once qualified her son for coverage now triggered a bureaucratic impasse.

“When I explained what happened in the school, that’s when they finally called it a crisis. That’s the keyword. And only then did we get the Medicaid waiver.”



That one word made all the difference. It unlocked the services Rocco had needed.

Jennifer is unequivocally grateful for the waiver, but the process left its mark. “Even with good caseworkers, the process is exhausting. Your nerves are shot. You’re scared. You’re trying to figure out how to pay for something while waiting for the system to catch up.”

And she doesn’t take the support they’ve received for granted. Jennifer’s aware of the families still stuck in limbo.

“I believe Rocco’s journey is meant to shed light on what needs to be reworked, and he’s an example of how a program can support someone with complex needs.”

Jennifer stays hopeful, but continues to ask the bigger question: What can we do for the most vulnerable before they fall through the cracks?

CARE THAT EXISTS ON PAPER. NOT IN PRACTICE.

Prader-Willi Syndrome affects approximately 1 in 15,000 births, but for the families living with it, the condition is anything but small. PWS is a complex rare genetic disorder that impacts hunger regulation, metabolism, behavior and developmental functioning, often requiring lifelong support and highly coordinated care. For many families, daily life revolves around structured routines, specialized medical oversight and constant vigilance to protect the health and wellbeing of their loved ones.
In honor of PWS Awareness Month, families, advocates and rare disease leaders gathered in Washington, D.C. this week for the PWSA | USA Biennial D.C. Fly-In, an event focused on ensuring policymakers hear directly from the people most affected by rare disease policy and healthcare access challenges.

Among those advocates was Katie Moureau, the mother of a son living with Prader-Willi Syndrome. “Being in Washington, D.C. this month was important because we are at a pivotal moment where the Rare Disease Innovation Hub and advances in genomic sequencing have the potential to fundamentally change how rare disease patients are identified, supported and treated,” Katie said. “Families need policymakers to understand that diagnosis alone is not enough. We need coordinated systems that connect innovation to real-world access, care and educational supports. Rare disease patients and caregivers deserve a seat at the table while these national frameworks are being built, not after decisions are already made.”

Katie’s son, Cade, requires continuous and highly specialized care. He sees multiple providers, depends on tailored medications and relies on consistent insurance coverage to maintain access to treatment and support services. Medicaid was supposed to help fill the gaps left behind by traditional coverage. Instead, Katie says it introduced an entirely new layer of barriers.

“The hardest part isn’t the paperwork,” she said. “It’s finding doctors who understand his condition and are actually in-network.”

Their family has strong primary insurance, which helped protect them from some of the more severe delays families often face. Even so, Katie found herself constantly advocating, appealing and negotiating for services that had already been 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 has become another source of frustration. Pharmacies that accept the family’s primary insurance often do not accept Medicaid, leaving Katie to navigate a fragmented system that demands time and persistence many families simply do not have. More recently, the introduction of a new pharmacy benefit manager added another layer of instability, with changing formularies, delayed authorizations and few accommodations for children with rare, high-need conditions.

Medicaid was never intended to become an obstacle course for families already carrying enormous responsibility. Yet for many caregivers navigating rare disease, access to care is shaped by disconnected systems, administrative burdens and policies that fail to account for the realities of complex conditions.
Cade’s diagnosis may be rare, but the challenges surrounding his care are not. Katie’s story reflects a broader reality facing families across the rare disease community, where even strong insurance coverage can fall short when coordination breaks down and support systems become difficult to navigate. For advocates gathered in Washington this week, that reality is exactly why these conversations matter.

Advocacy Training with the Prader-Willi Syndrome Association | USA

Learn Advocacy. Understand Policy. Engage Effectively.

This LEAD cohort is developed in partnership with the **Prader-Willi Syndrome Association | USA (PWSA | USA)** and brings together families, caregivers, and individuals in the Prader-Willi syndrome community who want to better understand healthcare policy and engage constructively in shaping it.

Participants learn how healthcare decisions are made and how patient and caregiver voices can contribute meaningfully to policy conversations that affect access to care and support services.

DOROTHEA LANTZ
LEAD Cohort Leader

Sign up for the PWS LEAD cohort right here!

PATIENTS RISING

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

www.patientsrising.org