



## Legislative Ask #2

### PRMRP

*Peer Reviewed Medical Research Program*

## Background

Prader-Willi syndrome (PWS) is a rare genetic disease that affects approximately 1 in 15,000 live births and is the most common genetic cause of life-threatening childhood obesity.

The hallmark symptom of PWS is hyperphagia, the uncontrolled desire to eat. Those living with hyperphagia feel constant hunger regardless of what, how much, or when they last ate. Essentially, they feel as if they are always starving. Without proper supervision, a person with PWS could literally eat themselves to death. To make the condition even more complicated, many individuals with PWS maintain a much lower-than-normal metabolic rate; some as low as 50% below their peers.

In addition to hyperphagia, PWS brings many other characteristics, which makes it difficult to cope with this condition. These characteristics include severe anxiety, compulsive/aggressive behaviors, hypotonia, scoliosis, speech apraxia/dyspraxia, sleep disturbances, seizures, high pain tolerance, struggles with temperature regulation, and low to moderate intellectual disability.

## Solution

PWS is considered a rare disease, and because of this, our community is faced with an uphill battle in funding research that would provide therapies for our loved ones. The pandemic we recently faced severely impacted the progress of new therapies in the pipeline. We are desperate to fund research that will aid the unmet needs of our loved ones with PWS.

Adding Prader-Willi syndrome as a recognized topic area for DOD research under the CDMRP's (Congressionally Designated Medical Research Program) Peer Reviewed Medical Research Program (PRMRP), scientists, researchers, and patient advocacy groups who work with our community would have an opportunity to fast-track multiple research projects that may lead to therapies for our loved ones with PWS. Time is of the essence for our families. Being a caregiver for a rare disease such as Prader-Willi syndrome is a monumental task. Many individuals with PWS have no alternative but to live in a group home. For those families who can keep their loved ones home, they often cannot afford to work outside of the home because their loved ones with PWS can't be left unattended. Couple that with constant specialist appointments and the intense therapies that are essential for people with PWS, our families run the risk of constant caregiver burnout, depression, anxiety and so much more.

We ask that you help open the door for research funding through the DOD's Peer Reviewed Medical Research Program so that our community can work together and give our loved ones with PWS an opportunity to live fuller, longer, and more productive lives.