



How Can You Be a Powerful PKU Patient & Advocate for Yourself or a Loved One?

New Thoughts on PKU Therapy

- Keeping my Phe near the target range is okay... **Lower is better!**
- Diet is enough to achieve the best possible Phe levels... **For many, medication can improve Phe levels.**
- Once on medication, I can stop my Phe-restricted diet... **Use medication and diet together to achieve optimal results.**
- Long-term safety data on new medication does not exist... **The active ingredient BH4 has been used up to 10 years in Europe and Japan.**

Key Program Takeaways from the August 13 Patient Power Webcast

Gerard Vockley, M.D., Ph.D. – Chief of Medical Genetics, Children’s Hospital of Pittsburgh of UPMC

- For families and people with PKU, the most important thing to recognize is that you are your own best advocate. You can't wait around for the medical system to take care of you. You have to take charge of your PKU.
- There are three ways that patients and families with PKU can empower themselves. The first is being proactive about therapy, and recognizing it is diet for life.
- The second way to empowerment is getting involved with the PKU community and raising awareness.
- The third way to empowerment is to push the envelope of therapy. Be aware of the trials that are going on in PKU research and get involved. This helps everyone.

Lauren Leviton, M.A., L.S.W. – Education Coordinator, Division of Genetics, Birth Defects & Metabolism, Children’s Memorial Hospital

- If you are vowing to more actively manage your PKU, remember: change is not an event; it's a process over time).
- The Internet is a great resource for connecting with other people. There are groups and websites for adults to network with one another; chat and share experiences. Useful websites to gain additional information about PKU and connect with other individuals living with this disorder include Patient Power (www.PatientPower.info), www.PKU.com, and the National PKU Alliance (www.npkua.org).

Tim Kelly – Adult with PKU

- You can't feel high blood pressure, but there are consequences if you don't manage it. It's the same with PKU and cognition, and it's in your best interest to manage it.
- Managing your PKU is a choice that has to be made every day.

Shideh Mofidi, M.S., R.D., C.S.P – Metabolic Dietitian and Clinical Coordinator in the Inherited Metabolic Disease Center, Maria Fareri Children’s Hospital

- PKU does not define you. You are an individual with other capabilities, other powers and talents. PKU is the second part, not the central part.
- Families who understand what's happening and deal with it do a lot better than families that are in denial.
- Education is the key to managing PKU and it's important to start early. Parents should educate themselves and their children. The children will transition into model patients as adolescents and adults.

Notes:
