Recently, CanPKU spent time with Nicole McWhorter, a metabolic dietician who has worked in PKU clinics and worked on multiple clinical trials for PKU treatments. Nicole is currently consulting on a clinical trial called SynPheny with the company Synlogic. SynPheny is a trial to evaluate a potential new treatment option for those living with PKU. Let’s learn more about it!

**Q. Can you tell me more about yourself, what your job is?**

I am a metabolic dietician and have been working with the PKU community for about 11 years, first at a clinic working closely with many patients with PKU and their families. Helping with the many difficulties of living with PKU got me interested in helping companies to bring forward new treatment options.

**Q. Tell me about this ongoing clinical trial?**

First, remember a clinical trial is designed to study potential new drugs. Clinical trials are the only way to bring potential new therapies forward to help patients in need. In this case, the study drug is called investigational because is not approved by Health Canada or the U.S. FDA yet.

The clinical trial I am helping with is called SynPheny. The SynPheny study is researching a study drug that was created by designing a specific probiotic using special engineering that allows it to break down phenylalanine (Phe) in the diet. The hope is that by doing so, it can help manage and prevent symptoms of Phe levels associated with PKU.

**Q. Who would qualify?**

To be in the study:

* You must be 18 years old or older and have PKU.
* At this time, your Phe levels >10 mg/dl or 600 micromol/L.
* For current treatment, the study is accepting people with PKU who are:
	+ Untreated (not taking a specific medication for PKU);
	+ You could be taking sapropterin (brand name is Kuvan)
* The study is not accepting people who have taken Palynziq within the last month before their first study visit (the screening visit).

This study is only for adults. Future studies may include teenagers (people age 12 and older) and pediatric patients.

**Q. Where is the study being held?**While there are no sites in Canada, the good news is that Canadians can participate through US-based sites. Travel expenses would be covered by the SynPheny team.

Visit [www.pkuresearchstudy.com](http://www.pkuresearchstudy.com) to learn more.

**Q. What is the treatment like?**

The study drug is a powder that you mix with water: 4 oz/100 ml (~half of a small Starbucks cup) and drink. You would take this drink three times a day, after meals.

**Q. Sometimes in clinical trials, some people get the treatment drug being studied, and some people get a placebo. Is this how it will work with this study?**

In SynPheny, there is no placebo arm. Every patient participating in Synpheny will receive the study drug.

**Q. Does the drug work? What are side effects?**

As a reminder this is an investigational drug. That means it has not been studied in enough people with PKU to confirm how well it works or all the side effects to make it approved by HealthCanada or the FDA. What I can tell you is that promising data from two earlier studies in a small number of people has led Synlogic to support its advancement to this stage (Phase 2), and that we’re very excited about its potential to help.

The most common side effects were GI-related, such as nausea and gas.

**Q. What is the time commitment?**

In general, there are three parts to the study:

* 1st - Diet run-in. During this time, a dietitian will work with you to on a food plan that will work for you during the study
* 2nd - Treatment period: You will take the study drug three times a day, for 15 days. During this time, you will need to visit a study location for regular check ins, or you have the option to stay overnight for the two-weeks at a study center in Salt Lake City, Utah.
* 3rd - Follow up: For about two weeks, after stopping the study drug, you’ll continue to provide information to the study site. You will then need to visit a study site for a final visit.

From beginning to end study participation could be 2-3 months.

**Q. What do I have to do with my diet during the study?**

For the study, you follow the diet that you are eating right now. The most important part is consistency during the study itself. A dietitian will assess how much protein you are eating right now and help with ideas and tools to make that consistency as easy as possible.

**Q. How can people who are interested learn more?**

Visit [www.pkuresearchstudy.com](http://www.pkuresearchstudy.com), enter in your contact information and a

 SynPheny team member will be in touch to discuss the clinical trial with you.