Early Navigation with PKU

Q1: What was the PKU diagnosis like for you and your family?

Kimberly:

Yes, so at the time our third born was born, I had a two-year-old and a one-year-old, so the life was a lot and it was crazy. And then after our son Eli was five days old, we got the call that his levels were elevated. And it was just a lot to think. You know, nobody wants to hear that their brand new baby has anything potentially not okay with them. And it was a lot to process and hear once they did officially diagnose him with PKU. And then fast forward a couple of years, I was pregnant again with our fourth born. And going into it, we were a little bit more aware, obviously, of the component that PKU was a possibility. And so going into the hospital before she was born, we were able to expedite the process of getting her blood samples and getting tested. And so, after I think she was two, maybe three days old, we found out, and there was still an array of emotions. We, again, didn't desire for a child of ours to have a genetic condition to live with for the rest of their lives. But there was also comfort in knowing that she has a sibling that she shared this reality with. So yeah, still an array of emotions, but different in the sense that we knew some of the practicalities and we were grateful that they had one another to walk this road with as we sought to support them.

Q2: How did you learn to manage your children's PKU day-to-day?

Kimberly:

So, it was it was a one day at a time kind of adventure. It was moment by moment, a lot in the early days. Everything was new. Everything was fresh. As I mentioned, our clinic was a huge resource for us. Just really holding our hand in the process of showing us what to do, how to do it, you know, measuring [medical] formula. I remember feeling like I was back in chemistry class as I measured on a scale the [medical] formulas, and made the bottles and, so, yeah, everything felt new and daunting. Very much, after a couple months, we were able to meet some families who had PKU in their family as well with children through the network that we have in our state. And that was a huge help, just to kind of learn from people who have gone before us and see children a little bit further down the road and even further than that, and see how they were thriving and living very typical lives in many ways, which was just, it was it was helpful. It gave us endurance to keep going with the nitty gritty of day to day, managing the diet. And it's nice because you begin, you know, with all infants as just, you know, [baby] formula fed or breast milk. And so it's simplified. We weren't, you know, counting and weighing food. So we were able to do baby steps even though it was a lot, taking their blood at home felt daunting but soon became routine, as things do when you do them, even if they're, not what you anticipated at the beginning.

Q3: How did you first hear about JAVYGTOR (sapropterin dihydrochloride)?

Kimberly:

So we work with UIC clinic and our dietitian, a nurse practitioner had mentioned it, probably a number of appointments before we had, or, you know, we kind of had ongoing conversations, but, I think at one point we just said, this is something we said to Eli first, kinds of “is this something you would be interested in trying?” And we weren't sure if he would be responsive. And he said “yeah”. And so we went from there. But it came from our clinic’s office at first.

Q4: How was the New Patient Free Trial? Kimberly: So it was helpful to just know we could try it for 30 days, it was covered financially at that point, and decide by then, you know, how responsive Eli was to it or if it was doing what we wanted it to. And I think, at that point, our clinic did the communications with Cycle Vita to get that all set for us, which was great. And then the shipment of JAVYGTOR™ (sapropterin dihydrochloride) came straight to our house and, had some phone calls talking through the process, and it was very simple in every way. So we were grateful for that option. Q5: What role did your Cycle VitaTM Clinical Education Manager play at this time? Kimberly: Yes, they were very helpful. My husband did a lot of the communications just because it was, again, another way he could help. So he was doing the phone communication, but I did get on a phone call with them at that time, just to learn. Yeah. Just really to have an opportunity to ask any questions and to hear their input and advice and tips and it was very helpful. They've always been very responsive and available at anything we've would want or need. And yeah, just, very helpful. So we're grateful for that support.

Important Safety Information JAVYGTOR Tablets for Oral Use and JAVYGTOR Powder for Oral Solution

What Important Information Should I Know About JAVYGTOR Tablets for Oral Use and Powder for Oral Solution?

•Sapropterin Dihydrochloride can cause serious side effects including:

Severe allergic reactions, including anaphylaxis – JAVYGTOR is not recommended in patients with a history of anaphylaxis to Sapropterin Dihydrochloride. Stop taking JAVYGTOR and get medical help right away if you develop any of these symptoms of severe allergic reaction:

•Wheezing or trouble breathing

•Coughing

•Flushing

•Nausea

• Feeling lightheaded or you faint

• Rash

Inflammation of the lining of the stomach (gastritis) or esophagus (esophagitis) - Gastritis or esophagitis can happen with Sapropterin Dihydrochloride and may be severe. Patients should be monitored by their doctor for signs and symptoms of upper gastrointestinal mucosal inflammation. Call your doctor right away if you have any of these signs or symptoms:

• Severe upper stomach area (abdominal) discomfort or pain, nausea and vomiting

• Blood in your vomit or stool

• Black, tarry stool

• Difficulty swallowing

• Loss of appetite

• Pain in the throat

Phenylalanine (Phe) levels that are too low - Some PKU patients have experienced low blood Phe during treatment. Some children under the age of 7 years who take high doses of Sapropterin Dihydrochloride each day may experience low Phe levels.

Too much or constant activity (hyperactivity) can happen with Sapropterin Dihydrochloride - Tell your doctor if you have any signs of hyperactivity, including:

• Fidgeting or moving around too much

• Talking too much

• It is not possible to know if Sapropterin Dihydrochloride will work for you until you start treatment. Your doctor will check your blood Phe levels when you start taking JAVYGTOR to see if the medicine is working

• All patients with PKU who are being treated with JAVYGTOR should also be treated with a Phe restricted diet, including dietary protein and Phe restriction

• Your doctor should continue to monitor your blood Phe levels often during your treatment with JAVYGTOR to make sure that your blood Phe levels are not too high or too low. Frequent blood monitoring is recommended in the pediatric population

• Avoid any dietary changes which may affect your blood Phe level. Follow your doctor's instructions carefully and do not make any changes to your dietary Phe intake without first talking with your doctor. Even if you take JAVYGTOR and your Phe blood levels are not well controlled, you can develop severe neurologic problems

• Tell your doctor as soon as possible if you are sick or have fever as this may increase your blood Phe level

• Take JAVYGTOR exactly as your doctor tells you to

• Do not take 2 doses in a day. If you take too much JAVYGTOR, call your doctor for advice or seek medical attention

Who Should Not Use JAVYGTOR Tablets for Oral Use and Powder for Oral Solution?

• JAVYGTOR Tablets for Oral Use and Powder for Oral Solution are not recommended in patients with a history of anaphylaxis to Sapropterin Dihydrochloride or any of the ingredients in Sapropterin Dihydrochloride

What Should I Tell My Doctor Before Taking JAVYGTOR Tablets for Oral Use and Powder for Oral Solution?

Before taking JAVYGTOR Tablets for Oral Use or Powder for Oral Solution, tell your doctor if you:

• Are allergic to Sapropterin Dihydrochloride or any of the ingredients in Sapropterin Dihydrochloride

• Have poor nutrition or have loss of appetite

• Are pregnant or plan to become pregnant

• Are breastfeeding or plan to breastfeed

Provide your doctor with a complete list of medications you take, including prescription and over-the-counter medications, vitamins, and dietary or herbal supplements. JAVYGTOR can interact with other medications that you are taking. Especially tell your doctor if you take:

• A medication that contains levodopa

• An antifolate medication, such as methotrexate, phenobarbital, trimethoprim, valproic acid

• Sildenafil (Revatio, Viagra), tadalafil (Adcirca, Cialis), vardenafil (Staxyn, Levitra)

Tell your doctor if you are unsure if your medication is one of the above listed. Always share your complete list of medications with your doctor and pharmacist.

What Are the Most Common Side Effects of JAVYGTOR Tablets for Oral Use and Powder for Oral Solution?

• Headache

• Runny nose and nasal congestion

• Sore throat

• Diarrhea

• Vomiting

• Cough

These are not all of the possible side effects of JAVYGTOR. Call your doctor for medical advice about side effects.

To report SUSPECTED Side Effects, contact Dr. Reddy's Laboratories, Inc. at 1-888-375-3784 or by email: medinfo@drreddys.com, or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Living with PKU and Looking to the Future

Q1: What are some things you've learned that make living with PKU easier?

Eli:
Okay, a few things that I've learned. I would say, getting like a routine, like a solid kind of just get a schedule for your week. So for me, it's like, when to drink my [medical] formula. I'll usually do one in the morning, one around midday, and one cup at nighttime. And then I kind of try and do the same thing with, like, balancing the amount of Phe I can have in a day or protein I can have in a day, meal to meal. So I kind of spread it out evenly. That's helped a lot. And then also just kind of, I don't know, I think when I have other things going on in my life, I don't think about it as much. So doing things like sports or music or other hobbies or just hanging out with people helps me kind of to take my mind off it. So I'm not as worried about it.

Q2: What would you say to others living with PKU who feel frustrated about their diet?

Ruby:
I would just, like, remind them that it's not all that bad. Like. I mean, yes, if you explained it to someone else, they'd be like, that sucks. And but I feel like there's some really good, positive things about it. But just try to remember them to stay positive.

Eli:
I would first of all, just tell them I've been there and that it can be frustrating and, but I think I would, yeah, encourage them. Like Ruby said, just stay positive because there's a lot of good things that come from a challenge like this. I think it produces, like, character. And, I mean, not many people could be on a diet this strict their whole lives. And I think people with PKU are forced into this position and it can really build character. And I would probably just, yeah, encourage them to stay strong because it will benefit them in the end.

Q3: How have you helped Eli and Ruby feel confident making food choices as they have grown more independent?

Kinberly:
So I think we've done a variety of things. I think, I mean, I remember a time where they would look at the recipes themselves online and kind of pick and choose maybe what sounded good or something newer to try. And then I or my mom would try it for them, and then they would try it, a little bit of kitchen food involvement. I mean, they both like to make waffles and pancakes themselves. They mostly prepare or kind of heat up prepared things. So they're not necessarily making everything from scratch on their own, but they're very capable and executing things in the kitchen. And I think just time and practice, learning what they liked, what was familiar, and then just giving them opportunity to ready those things for themselves.

Q4: Do you feel like you have a good support system that will help you through that transition?

Eli:
I'm definitely like, very excited. Definitely a little nervous, just for the, yeah. I haven't had to live with this diet on my own yet. But I do just feel like I do have such a good support system with my family. Specifically my parents and my grandma, always cooking foods for us and consistently just helping us kind of grow and asking those questions about our diet. I think that through high school, I've become more and more kind of independent, managing the diet on my own. So I feel like I'm fully capable to do it on my own in college, and I'm excited for the prospect of getting to do this and, kind of more responsibility on my shoulders, but I think it'll be good for me.

Q5: What does independence mean to you and when do you feel it the most?

Ruby:

I feel like when I'm at school, obviously now, because I used to be homeschooled. So, like, my mom would always be there when I like, needed her to check my Phe level or like, help me with whatever food I wanted to get. And just

like, being at school has helped. Like, I have teachers that help me know when I need to drink my [medical] formula and like, just a lot of good people to help me. But I also feel like, I feel like most when I'm at school and I have got like my routine down when I should be drinking and what I should eat and what I should not eat.

Important Safety Information JAVYGTOR Tablets for Oral Use and JAVYGTOR Powder for Oral Solution
What Important Information Should I Know About JAVYGTOR Tablets for Oral Use and Powder for Oral Solution?

•Sapropterin Dihydrochloride can cause serious side effects including:

Severe allergic reactions, including anaphylaxis – JAVYGTOR is not recommended in patients with a history of anaphylaxis to Sapropterin Dihydrochloride. Stop taking JAVYGTOR and get medical help right away if you develop any of these symptoms of severe allergic reaction:

•Wheezing or trouble breathing

•Coughing

•Flushing

•Nausea

• Feeling lightheaded or you faint

• Rash

Inflammation of the lining of the stomach (gastritis) or esophagus (esophagitis) - Gastritis or esophagitis can happen with Sapropterin Dihydrochloride and may be severe. Patients should be monitored by their doctor for signs and symptoms of upper gastrointestinal mucosal inflammation. Call your doctor right away if you have any of these signs or symptoms:

• Severe upper stomach area (abdominal) discomfort or pain, nausea and vomiting

• Blood in your vomit or stool

• Black, tarry stool

• Difficulty swallowing

• Loss of appetite

• Pain in the throat

Phenylalanine (Phe) levels that are too low - Some PKU patients have experienced low blood Phe during treatment. Some children under the age of 7 years who take high doses of Sapropterin Dihydrochloride each day may experience low Phe levels.

Too much or constant activity (hyperactivity) can happen with Sapropterin Dihydrochloride - Tell your doctor if you have any signs of hyperactivity, including:

• Fidgeting or moving around too much

• Talking too much

• It is not possible to know if Sapropterin Dihydrochloride will work for you until you start treatment. Your doctor will check your blood Phe levels when you start taking JAVYGTOR to see if the medicine is working

• All patients with PKU who are being treated with JAVYGTOR should also be treated with a Phe restricted diet, including dietary protein and Phe restriction

• Your doctor should continue to monitor your blood Phe levels often during your treatment with JAVYGTOR to make sure that your blood Phe levels are not too high or too low. Frequent blood monitoring is recommended in the pediatric population

• Avoid any dietary changes which may affect your blood Phe level. Follow your doctor's instructions carefully and do not make any changes to your dietary Phe intake without first talking with your doctor. Even if you take JAVYGTOR and your Phe blood levels are not well controlled, you can develop severe neurologic problems

• Tell your doctor as soon as possible if you are sick or have fever as this may increase your blood Phe level

• Take JAVYGTOR exactly as your doctor tells you to

• Do not take 2 doses in a day. If you take too much JAVYGTOR, call your doctor for advice or seek medical attention

Who Should Not Use JAVYGTOR Tablets for Oral Use and Powder for Oral Solution?

• JAVYGTOR Tablets for Oral Use and Powder for Oral Solution are not recommended in patients with a history of anaphylaxis to Sapropterin Dihydrochloride or any of the ingredients in Sapropterin Dihydrochloride

What Should I Tell My Doctor Before Taking JAVYGTOR Tablets for Oral Use and Powder for Oral Solution?

Before taking JAVYGTOR Tablets for Oral Use or Powder for Oral Solution, tell your doctor if you:

• Are allergic to Sapropterin Dihydrochloride or any of the ingredients in Sapropterin Dihydrochloride

• Have poor nutrition or have loss of appetite

• Are pregnant or plan to become pregnant

• Are breastfeeding or plan to breastfeed

Provide your doctor with a complete list of medications you take, including prescription and over-the-counter medications, vitamins, and dietary or herbal supplements. JAVYGTOR can interact with other medications that you are taking. Especially tell your doctor if you take:

• A medication that contains levodopa

• An antifolate medication, such as methotrexate, phenobarbital, trimethoprim, valproic acid

• Sildenafil (Revatio, Viagra), tadalafil (Adcirca, Cialis), vardenafil (Staxyn, Levitra)

Tell your doctor if you are unsure if your medication is one of the above listed. Always share your complete list of medications with your doctor and pharmacist.

What Are the Most Common Side Effects of JAVYGTOR Tablets for Oral Use and Powder for Oral Solution?

• Headache

• Runny nose and nasal congestion

• Sore throat

• Diarrhea

• Vomiting

• Cough

These are not all of the possible side effects of JAVYGTOR. Call your doctor for medical advice about side effects.

To report SUSPECTED Side Effects, contact Dr. Reddy's Laboratories, Inc. at 1-888-375-3784 or by email: medinfo@drreddys.com, or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Advice for Families living with PKU

Q1: What are your hopes for other families living with PKU?

Kimberly:
Yeah, I hope that, they can recognize that as hard as PKU is, there's a lot to be grateful for with it. The reality is that our children can live, you know, more or less typical lives and to grow and to thrive. So, yeah, just kind of the gratefulness in the midst of the hard that it brings, and that they would have support rallied around them and to see families beyond them and know that there are good things ahead even as you're learning to navigate a hard thing.

Q2: What message would you share with parents who are just starting their PKU journey?

Kimberly:
I think I would just share that it's going to be okay. I think that was nice to hear at the beginning when everything was so new and unfamiliar. But just to be reminded that it's going to be okay.

Important Safety Information

JAVYGTOR Tablets for Oral Use and JAVYGTOR Powder for Oral Solution
What Important Information Should I Know About JAVYGTOR Tablets for Oral Use and Powder for Oral Solution?
• Sapropterin Dihydrochloride can cause serious side effects including:
Severe allergic reactions, including anaphylaxis – JAVYGTOR is not recommended in patients with a history of anaphylaxis to Sapropterin Dihydrochloride. Stop taking JAVYGTOR and get medical help right away if you develop any of these symptoms of severe allergic reaction:
• Wheezing or trouble breathing
• Coughing
• Flushing
• Nausea
• Feeling lightheaded or you faint
• Rash
Inflammation of the lining of the stomach (gastritis) or esophagus (esophagitis) - Gastritis or esophagitis can happen with Sapropterin Dihydrochloride and may be severe. Patients should be monitored by their doctor for signs and symptoms of upper gastrointestinal mucosal inflammation. Call your doctor right away if you have any of these signs or symptoms:
• Severe upper stomach area (abdominal) discomfort or pain, nausea and vomiting
• Blood in your vomit or stool
• Black, tarry stool
• Difficulty swallowing
• Loss of appetite
• Pain in the throat
Phenylalanine (Phe) levels that are too low - Some PKU patients have experienced low blood Phe during treatment. Some children under the age of 7 years who take high doses of Sapropterin Dihydrochloride each day may experience low Phe levels.
Too much or constant activity (hyperactivity) can happen with Sapropterin Dihydrochloride - Tell your doctor if you have any signs of hyperactivity, including:
• Fidgeting or moving around too much
• Talking too much
• It is not possible to know if Sapropterin Dihydrochloride will work for you until you start treatment. Your doctor will check your blood Phe levels when you start taking JAVYGTOR to see if the medicine is working

• All patients with PKU who are being treated with JAVYGTOR should also be treated with a Phe restricted diet, including dietary protein and Phe restriction
• Your doctor should continue to monitor your blood Phe levels often during your treatment with JAVYGTOR to make sure that your blood Phe levels are not too high or too low. Frequent blood monitoring is recommended in the pediatric population
• Avoid any dietary changes which may affect your blood Phe level. Follow your doctor's instructions carefully and do not make any changes to your dietary Phe intake without first talking with your doctor. Even if you take JAVYGTOR and your Phe blood levels are not well controlled, you can develop severe neurologic problems
• Tell your doctor as soon as possible if you are sick or have fever as this may increase your blood Phe level
• Take JAVYGTOR exactly as your doctor tells you to
• Do not take 2 doses in a day. If you take too much JAVYGTOR, call your doctor for advice or seek medical attention

Who Should Not Use JAVYGTOR Tablets for Oral Use and Powder for Oral Solution?

• JAVYGTOR Tablets for Oral Use and Powder for Oral Solution are not recommended in patients with a history of anaphylaxis to Sapropterin Dihydrochloride or any of the ingredients in Sapropterin Dihydrochloride

What Should I Tell My Doctor Before Taking JAVYGTOR Tablets for Oral Use and Powder for Oral Solution?

Before taking JAVYGTOR Tablets for Oral Use or Powder for Oral Solution, tell your doctor if you:

• Are allergic to Sapropterin Dihydrochloride or any of the ingredients in Sapropterin Dihydrochloride
• Have poor nutrition or have loss of appetite
• Are pregnant or plan to become pregnant
• Are breastfeeding or plan to breastfeed

Provide your doctor with a complete list of medications you take, including prescription and over-the-counter medications, vitamins, and dietary or herbal supplements. JAVYGTOR can interact with other medications that you are taking. Especially tell your doctor if you take:

• A medication that contains levodopa
• An antifolate medication, such as methotrexate, phenobarbital, trimethoprim, valproic acid
• Sildenafil (Revatio, Viagra), tadalafil (Adcirca, Cialis), vardenafil (Staxyn, Levitra)

Tell your doctor if you are unsure if your medication is one of the above listed. Always share your complete list of medications with your doctor and pharmacist.

What Are the Most Common Side Effects of JAVYGTOR Tablets for Oral Use and Powder for Oral Solution?

• Headache
• Runny nose and nasal congestion
• Sore throat
• Diarrhea
• Vomiting
• Cough

These are not all of the possible side effects of JAVYGTOR. Call your doctor for medical advice about side effects. To report SUSPECTED Side Effects, contact Dr. Reddy's Laboratories, Inc. at 1-888-375-3784 or by email: medinfo@drreddys.com, or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.