

## Access to Care: Insurance and Reimbursement Support Programs for New PKU Treatments

Webcast

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### **Introduction**

#### **Andrew Schorr:**

Hello and thank you for joining us for the second of our live webcasts on PKU. We had one as many of you know on May 8th, and we discussed the new landscape of treatments. Today we're going to discuss access to care, insurance, and reimbursement support programs for new PKU treatments, and we're going to have an array of guests who will help you understand how you get access to the care you or a loved one need and deserve.

Now remember that on June 19 we'll come back with the third in our series of programs, and that will be living with PKU, patient approaches to the management of PKU. So again then we will discuss more treatment issues, but this is really how do you get the medicine that is right for you.

Now we're in a new era, and as you heard in the last program about PKU, where there is an FDA approved medication Kuvan. Well, what's your insurance these days? Do you have a job? What are the co-pays? What's the coinsurance? What's the cap? All these come into play, and so you're going to hear from people who can give you answers, numbers to call, people to help you on your case, and really make you feel a valued part of the PKU community. Thanks again to the unrestricted grant from BioMarin to help make this series possible. We really appreciate them, and the whole PKU community coming together. I've been learning about it along the way, and I just really feel a tremendous amount of warmth and support as people seek information and try to help each other as we develop newer and better therapies to help people go on with hopefully a long and high quality of life.

First I'd like to introduce you to someone who's lived it, because she has a nine-year-old son, Devon, who has PKU, and that's AnneMarie DiGeorgio. AnneMarie lives out on long island, about 45 miles east of New York City in a place called Center Reach, and

then her husband is a New York City policeman and goes into the city, but she's out there with Devon, who is doing really well. But keeping his Phe levels just right has been a challenge, right AnneMarie?

**AnneMarie:**

Yes, it's been a challenge.

**Andrew Schorr:**

So there you are now. You are quite the mom, because not only with your only child, Devon, you're working on all of that, but also you're I know, President of the Inherited Metabolic Disorder Support Group of Suffolk County, New York, and you had a gathering just a week or so ago. Didn't you?

**AnneMarie:**

Yes we did at Piccolos Restaurant in Bellmore New York. We had a lot of fun there.

**Andrew Schorr:**

Yeah, that's great bringing people together. Will we are all together tonight. Tell us a little bit about Devon and about how it was difficult before Kuvan to get those Phe levels right and how that was a problem in his life and yours as well.

**AnneMarie:**

Well, doesn't be a variant is in between being a classical and a hyper-Phe, which means he's not as restricted as a classical PKU person would be, but he does need to be on diet and does need formula. So because he has those levels that jump around a lot, there are times where he can eat a lot of Phe, and his level would be fine, then you could go the next week and he'd eat the same amount of Phe, and his levels would jump up. So with the Kuvan, that's balanced out his levels.

**Andrew Schorr:**

Now, he ended up being in the clinical trial, and so you found out as they were still learning about Kuvan that it was helpful for him.

**AnneMarie:**

Right.

**Andrew Schorr:**

And so then the trial ends, and then the drug is approved, and so then obviously there's a cost to the drug. How if you worked that out? We are going to hear all the latest related to the BPPS program in a minute, and we are going to explain what that means, the support program for people who want access to Kuvan and may have insurance issues or if they need some help, but how did that work out for you? How did they help you, because I know that your insurance did have some limitations.

**AnneMarie:**

Well with our insurance being self-insured our prescription plan was through the PBA, which is the Police Benevolent Association, and what happened was they were going to cover the drug, but unfortunately we had caps, and the cost of Kuvan exceeded the cap. So the drug would have been out of reach in our case. So what happened was BioMarin stepped in. They put us in the BPPS program, and they've been covering the medicine for Devon so far.

I know a lot of insurance companies are covering it, but some insurance companies have caps where it's really an issue, and it's just unaffordable for the average person.

**BioMarin Patient and Physician Support Program (BPPS)**

**Andrew Schorr:**

Right we are going to learn all about coinsurance and co-pays and caps and under insurance and all these things can be helped, and you're going to hear about that in a minute. Well, AnneMarie, I want you to stand by and chime in as you can as it's appropriate. Let's meet the expert from BPPS, or the BioMarin Patient and Physician Support Program, and that's Tony Wicks. We are going to go across the country from way out on Long Island to way over to California through the miracle of our broadcast here to Tony Wicks. Tony. Thank you for joining us. Help us understand what your support program can do, a little bit of how it works, who should call to find out whether any of these what could be obstacles; co-pays, coinsurance, caps; can be dealt with, helpfully, so that someone can get the treatment they need.

**Mr. Wicks:**

Sure, so thank you for having me. BioMarin Patient and Physician Support is actually a service offered by BioMarin to really anyone who is attempting to gain access to Kuvan or associated with people attempting to gain access to Kuvan. That includes patients, family members, caregivers, as well as the physicians as well as dietitians providing care for those patients, and so anyone who reaches out to us. Our job is really to guide you through the process of what's involved in getting on Kuvan. There can be a number of steps, and in some cases be very straightforward and in some cases there's a great deal of red tape that we may have to go through with the insurance companies, but our job here is to guide you through that process, hold your hand, and try to take a lot of the guesswork and a lot of the hard work out of that process for you and really just try to make the process as smooth as possible.

**Andrew Schorr:**

How does it start if someone does call a toll-free number? So let's say somebody, let's say we have a family listening tonight, and their child was just diagnosed with PKU, their little baby, and this illness that we've talked about that maybe they've never heard of before, and they say, 'Oh my. How do I proceed?' Or as they're getting older, and now a prescription medicine comes into play. What number do they call, and what happens next?

**Mr. Wicks:**

Will they are definitely welcome to call us at 877-MYKUVAN, (1-877-695-8826), and we can provide them an overview of the services that we provide and the steps that are involved for going on therapy, but the most important thing is definitely sitting down with their physician and/or their dietitian and discussing their interest in going on Kuvan and initiating the process because the process is really kicked off by receiving a prescription from your provider. At that point, once we receive the prescription, we'll actually reach out and get the process going, which involves the families signing an authorization for us to provide services to them, and then we really start going. We look at the family's insurance situation, what their benefits are, and then go through the whole process of securing coverage.

**Andrew Schorr:**

Now I should say that your group is pretty expert at dealing with this with probably the insurance companies throughout the country, so I know you've encountered many situations. So when they say, 'Oh I have Empire Insurance.' or, 'I have United,' and then they tell you a little bit about their plan, you can find out pretty quickly, what is stated in their policy of what might be available to them, and then sometimes you have to go to bat and appeal things further, right?

**Mr. Wicks:**

Right. So the first thing that we're going to do is we are going to make sure we get a copy of their insurance card, which gives us their policy number, their group member. One of the things to make sure that people are really cognizant of is just because you have United doesn't that mean that the plans are all structured the same. They have thousands of different plan structures, and so one person may have fantastic coverage and another person may have very limited coverage. Like what was mentioned earlier, some people might encounter things such as drug caps. The thing that we're going to do is we're going to call the insurance, and we are going to confirm all the benefit information that we can get for that patient regarding Kuvan. So we want to get an idea of what their financial responsibility might be, such as whether there's a deductible, whether there is going to be a co-pay or some kind of coinsurance, if there are any types of caps or lifetime maximums. We're going to try to get an overall picture of what their benefit situation looks like, so that we know all that information as we go forward with the process.

In some cases, an insurance may require a prior authorization for Kuvan. We will actually go through that process on behalf of the patient, and in some cases they may deny it, and if that happens we'll actually appeal that decision as well.

**Andrew Schorr:**

All right, so I want to underscore a couple of points for people. So first they call 1-877-MYKUVAN, (1-877-695-8826), and that connects them with someone at your organization, the support program; you learn about their situation, what insurance they

have or don't have, and you get into the very specifics of it. Also you can be in communication with their provider right? So let's say their provider did write a prescription, but you need some more information. The insurance company is saying, what about this and what about that? You all help orchestrate that process. It's not, I mean, when the family faces a diagnosis like this or are living with it for many years, it really can be scary, but it can be really fatiguing as well as of course the condition, so you're there to help that.

**Mr. Wicks:**

Right. You can think of us as the hub for the process, so there is going to be a number of different parties involved. We are going to be dealing with the provider. We're going to be dealing with the insurance company, a specialty pharmacy, and in some cases, if there's a significant financial responsibility, will be interacting with NORD as well, and so our job is, there will be a case manager that is assigned to that patient, whose job is to keep them, really just to keep them on top of everything that's going on in an effort to get them on Kuvan. So that case manager will interact with them periodically to say, here's the latest update, we've got these benefits back from your insurance company, or your case has been transferred to this pharmacy, and you can be expecting a call from them to schedule shipment for the drug, or if you need financial assistance. We refer your case over to NORD, and someone should be contacting you about evaluating you for assistance there.

Our job is really to be the single point of contact for that family and to really manage all the different activities that need to happen in order to get them on therapy.

**Andrew Schorr:**

And typically there's one person, they may deal with, right? There may be Sally in your office, and she is in a sense their case manager, and that's who they talk to.

**Mr. Wicks:**

That is correct.

**Andrew Schorr:**

Yes, well, that sounds like tremendous support. I just want to clarify one thing you mentioned too. You used the phrase "specialty pharmacy." A lot of people may not know what that is, but you work out with the insurance company as things get approved for the medicine to be mailed to the family, right? There are different specialty pharmacies because the drug needs special handling that you usually won't get at the corner pharmacy, and you'll work out where does that come from, and AnneMarie, I think you told me that for Devon, the medicine actually comes from a location in Montana.

**AnneMarie:**

Right, a specialty pharmacy in Montana.

## **National Organization for Rare Disorders (NORD)**

### **Andrew Schorr:**

Right, and that's the one that works for the program you are in. So Tony's organization coordinates all that. Tony also mentioned NORD. Let's explain what that is, and meet one of the guiding lights of NORD. That Maria Hardin. Her organization is, she is vice President of Patient Services for the National Organization for Rare Disorders or NORD, and they have a whole variety of programs to help people when their insurance falls short, they are underinsured, and there are support programs there too, and also those extend actually in many cases to formula and medical foods, so the whole PKU picture, but I don't want to steal Maria's thunder. She knows it a lot better than I do. Marie welcome. Thank you for joining us from Connecticut. Help us understand where NORD comes in when it comes to the PKU patient.

### **Ms. Hardin:**

Thanks Andrew. I would just like to share, I know that some of the listeners might not even know what NORD is, so I'm just going to give just a little brief background to let you know that we really represent about 148 health organizations, and they're all dedicated to the prevention, treatment, and cure of rare orphan diseases. Now, we were created in 1983 as the first organization dedicated to rare diseases. Up to that point, no one really took ownership of a rare disease, PKU being a rare disease, and it was the result of an extraordinary vision of patients and families, who worked to get the historic Orphan Drug Act passed by Congress.

Now, we're very unique. I don't think there's another organization like us. We help people with educational and advocacy issues, research, and I oversee the patient services programs that really take care of more than 25 million Americans and their families that are affected with rare disorders.

Now since 1987, we have administered patient assistance programs. You might know these as free drug programs. Originally that's what everything started out to be as far as access to drugs for the uninsured, and then we began to notice that there were issues with people who were insured. They still couldn't access their needed medications because of the escalating co-pays that were arising. These programs that we have are carefully tailored to the patients and the families we serve, and they are developed through close partnerships with like-minded, quality and caring pharmaceutical and biotechnology companies. We work with about 40 different companies at this point.

Now, people sometimes ask where does NORD's funding come from? Well, it comes from corporate donations, grants from concerned companies, such as BioMarin, foundation grants, public contributions, and membership dues. In addition to over 40 patient assistance programs, we are very excited, because we provide to programs for patients who require PKU treatment. Financial help is provided on a case-by-case basis to patients accepted into one or both of these NORD programs.

The first program, the one that Tony and Andrew have been talking about, is the PKU Premium and Co-pay Fund, and NORD is able to work directly with the family's insurance company and the specialty pharmacy that we were talking about earlier to arrange for payment of health insurance premiums, deductibles, co-payments to pay for medication. The program can also provide direct reimbursement for lab tests associated with starting or staying on medication to treat PKU.

We're very pleased to announce the launch of a medical foods assistance program for PKU patients that may provide direct reimbursement for prescription medical foods and formula. Now the impetus for developing such a program was due to the fact that because these foods are manufactured in small volumes for small populations, they are expensive. On average, medical food costs about three to five times more than regular foods, and they're also frequently not covered by most insurance plans, resulting in a huge expense not only for the families but for the medical community that's trying to provide care for the patients.

PKU families are dealing with the fact that no federal mandate nor standard exists to address the metabolic diseases like PKU. Many states have introduced legislation addressing this, but right now there's a patchwork of approaches with restrictions that support low caps and age limitations, and 13 states have no mandates whatsoever. Families who find themselves having to relocate to from one state to another face disastrous consequences.

So I feel that the program serves as a new safety net that provides financial support for PKU patients and families without benefit coverage in order to obtain the preferred medical food, and at this point in time assistance in the form of reimbursement, coupons, or manufacturer discounts allow for families enrolled in the program. Unfortunately right now it's only limited to amino acid modified products that contain a synthetic source of protein and don't contain the phenylalanine.

**Andrew Schorr:**

I have trouble pronouncing it too. I know we all have trouble. Maria, I was just going to jump in here for a minute and that is so well that's great. So for a family that probably has been relying on formula or medical food, they should be talking to the National Organization for Rare Disorders, NORD, your organization. Now do you have a phone number for them to call you directly related to that?

**Ms. Hardin:**

Yes, if they're calling about the medical foods program, they need to dial 1-866-924-0100, but you can also visit NORD's website, and I'll give that to you. It's [www.rarediseases.org](http://www.rarediseases.org), and when you go on, you will see, on the front page you will see an article about the new program, and it will link you, and you can download the application.

**Andrew Schorr:**

All right. I want to get to a lot of questions. Not just to go back, Tony, I want to go back to you. In our last program, the medical experts were discussing that they're really trying Kuvan on a wide variety of patients now and trying to understand who it's most effective for and what a difference it makes. So it may be that many people start by calling 1877-MY-KUVAN, and then often there's a handoff or coordination with NORD, right, as that comes into play.

**Mr. Wicks:**

That's correct.

**Working with Your Healthcare Provider**

**Andrew Schorr:**

Okay, all right. We're going to get into more of the details. I want to bring in the provider's point of view, and we're going to hear from Belkys Prado, and she's a nutritionist down in Florida, and she couldn't be with us live, but she is on tape, and let's hear from her, and then pretty quickly were going to get to your questions. I want you to be thinking about them. Remember, you can call us or send us an e-mail at [PKU@patientpower.info](mailto:PKU@patientpower.info). If you're connected on the phone with us, you just press \*1. .

Okay, let's hear from Belkys Prado:

Joining us now on tape is Belkys Prado. For 11 years Belkys has been a metabolic nutritionist at St. Joseph's Children's Hospital in Tampa. She's a specialist in helping both children and adults with PKU and has her own perspective as a healthcare provider. Belkys, thanks for joining us.

**Ms. Prado:**

Thank you Andrew.

**Andrew Schorr:**

Belkys, so let's talk about it from your perspective. So first of all, now that we have Kuvan as they've approved medication people are asking lots of questions. What guidance would you give people on how to have a productive discussion with someone like you, a metabolic nutritionist to say, should we try this drug, would it be helpful, and how would we get it paid for?

**Ms. Prado:**

Well, what we've done with our patients is we initially had a patient group meeting and a family group meeting where we invited all patients in our clinic and also in other clinics in the state of Florida and gave them information regarding the expanded access program that we were involved in. So that was the first step that we took to allow patients to know about Kuvan. Patients since then have contacted me, and they've asked how can my child or how can myself, how can we start Kuvan? At that point, I

talk to them individually. I also send them information regarding Kuvan and then guide them to the website that BioMarin has regarding Kuvan so that they can get more information there, and then the dialogue starts that way.

**Andrew Schorr:**

Now, I'm sure that there have been responses from insurance carriers saying, 'Wait a minute. This is really new, and we don't know if it applies in your case, and you've been managing with diet, and that's good enough.' I mean, there's always that tension and that debate. Do you have any guidance for people, or is there something they should ask of their nutritionist to document their case, write a letter, what would you suggest?

**Ms. Prado:**

Well we work very closely with BPPS, which is the BioMarin Physician and Patient Support, and we send all of the documentation to them. They are the liaisons between the patient and the insurance company, so really all of the work is being done by BPPS to get authorization for the medication. So I actually tell my patients to not call their insurance and let BPPS do what they do best.

**Andrew Schorr:**

Now one of the questions, we're in this new period of Kuvan is, as it's tried for a wide array of people with PKU, is will it be effective in your case and giving it time to assess that. So then the question is, you will want to make sure you have reimbursement in place over that time, and then ongoing if the drug is effective. Any guidance you could give people so that somebody doesn't try to pull the plug on it too fast?

**Ms. Prado:**

Right. I tell my patients that were going to try it for at least a month. Sometimes it takes longer to determine if the patient is a responder or to reach their maximum response, so when I write the prescriptions, or my physician writes the prescriptions, we write it for a three month supply. That way when BPPS requests the prior authorization we have enough medication to cover them at least through the trial period.

Now if co-pays or deductibles are an issue. There are programs that are set up through BioMarin and NORD to help patients that are at financial need with the medication. Now if the insurance denies coverage of the medication, which has happened to two of my patients, we've had to provide additional documentation, letters of medical necessity, with the help of BPPS to get the medication approved and covered, and that has been successful thus far.

**Andrew Schorr:**

So is it just that it's so new, and that after all were talking about, of all the things insurance companies do, a rare condition, and that you really just have to be the squeaky wheel?

**Ms. Prado:**

I think that sums it up pretty well. I think it's a matter of insurance companies not knowing what PKU is and what the effects of having high levels are as well as it being a new drug.

**Andrew Schorr:**

Now on your site, on the clinical side, what are you looking for as we talk about these trial periods? What are your sort of measurements to say it is working, we should stick with it, reimbursement should stay in place. How do you assess that?

**Ms. Prado:**

That is different from patient to patient, but typically the first thing we're looking for is a drop in phenylalanine levels. A lot of patients are either off diet or have had poor control for a long time. So my initial assessment of whether the drug is working or not it is the drop in Phe levels, and we look for a certain amount obviously, you know a 20 to 30% drop. Each clinic is going to have a different guideline, but in the EAP that's what we were looking for was a 30% drop in Phe levels. Now they may have other improvements in their daily lives and their thinking in their memory etc., and obviously those would also play a part in whether someone is determined to be a responder or not.

**Andrew Schorr:**

Belkys, you deal with both little infants and children and adults. Do you see related to reimbursement any different scenarios there or any more difficulty for adults if they had been living with a restricted diet for a long time, that encouraging reimbursement or getting reimbursement may be more of a battle because they've been sort of getting along for awhile before that?

**Ms. Prado:**

I haven't noticed a difference in my patient population.

**Andrew Schorr:**

That's good to know, and then my last question for you is so how would you guide people then in how to work with their healthcare team? We know how it works there, but just people will be hearing this all over; just sort of action points for people so that if their healthcare team didn't have a meeting, or maybe they don't see as much PKU, how they can get the discussion going and enlist their assistance in having access to medication that could be helpful?

**Ms. Prado:**

I think it would be helpful for families and adult patients to learn about the medication, know what's expected of them, and then contact their clinic to see if they are willing to start the patient on the medication, and then just follow their instructions, because that's going to make the whole process smoother, and it's going to make it quicker hopefully in determining whether they are a responder or not.

**Andrew Schorr:**

Belkys Prado, a metabolic nutritionist at St. Joseph's Children's Hospital of Tampa, Florida. Thank you so much for joining us and your information and your devotion to both children and adults with PKU.

**Ms. Prado:**

Thank you.

**Andrew Schorr:**

We are back live on our live webcast, and we're coming up on a break, but before we do I just want to tell you again, how you can ask questions, because this is such an important program; access to care, insurance and reimbursement support programs for a new PKU treatments. Just give us a call, 877-711-5611 or send us an e-mail, [PKU@patientpower.info](mailto:PKU@patientpower.info). If you're already on the phone just press \*1, and you'll be connected with one of our producers. We are visiting with some of the leading experts. Tony Wicks who helps run the BioMarin Patient and Physician Support Program you've heard about, BPPS, and also Maria Hardin, Vice President of Patient Services at the National Organization for Rare Disorders or NORD, and then AnneMarie DiGeorgio who is the mom of Devon who has PKU and also runs a support group out on Long Island. Much more coming up and your questions as we continue Patient Power on our live webcast on PKU right after this.

And we are back live, so we're going to spend the rest of the time just taking your questions. We've gotten many already. Bottom line, you are not alone. There's a wonderful support program for you called the BPPS for short, BioMarin Patient and Physician Support Program, and Tony Wicks has a whole group there where they support you and deal with the insurance companies, find out the ins and outs if you have insurance, or if you just have a little, if you have co-pay issues, coinsurance issues, financial cap issues in an individual year, or whatever it is, and then also there's another layer support you heard about from Maria Hardin at the NORD or National Organization for Rare Disorders, and then we will give all those phone numbers again, and websites, and there you go.

**Listener Questions**

**Andrew Schorr:**

Okay, let's talk about questions. This one came in from Kelly. I think this is going to be for you Tony. 'We are parents of two children with PKU. One is 15. One is 12. They are both on diet and doing very well. But of course they'd love to be able to eat regular foods. My question is about coverage of the drug. We move frequently with my husband's company, and our insurance changes with each move. We are concerned about one company agreeing to the drug, and then if we move the next company turning us down. I realize there's an assistance program to help families when the insurance companies refuse this treatment, but there's a salary cap for this assistance.

With two children and no assistance, this treatment is not available to our children and then they'd be devastated not able to receive the drug. What options are available for children with these circumstances?' So again, people move, change jobs, how do you help through that Tony?

**Mr. Wicks:**

Okay, so there are a number of steps that are going to take place. First of all, it is very important that if you currently are on Kuvan, and you are preparing to move, definitely contact BPPS and let us know because we will assist you through that process of changing insurances. In particular, the thing that we're going to want to do right away is contact your new insurance to figure out what the benefits are going to look like, whether or not they're going to be different from the current coverage, and whether or not it's going to be different from the current coverage that you have. One of the key benefits that you do have as you're transitioning from one insurance to another, if you're already on therapy and you are seeing a clinical response is; the strongest argument that you have for an insurance company covering a drug is the fact that the patient is actually responding to therapy. So it just makes it that much easier sell when we go to an insurance company saying that this patient is actually a good candidate for this therapy, it's working for them, and were able to show an argument of medical necessity.

So, that is the first step that were going to do is we're going to try to figure out what the benefits for that patient will look like on the new insurance, and we're going to make sure we have a very strong clinical argument. If they deny it then what were going to do is we're going to appeal that decision. There may be some involvement necessary from the patient as well as from the physician and dietitian to assist with that, and we will continue to appeal that decision as high as possible. In some cases, you can actually appeal the decisions to outside parties such as state insurance boards or even to employers to try to get coverage.

Typically, in the event that if we run into a situation where we can't get that patient covered, that patient will be rendered uninsured, and at that point they will qualify. If they meet the financial criteria, they will then qualify for free drug via the BioMarin Patient Assistance Program.

**Andrew Schorr:**

Okay. Here's a question that's for you Maria from Sandra and Melbourne, Florida. She says, 'I've been turned down for Social Security disability and Medicaid. My car is always breaking down, and it's very hard to work without my supplement. What should I do?'

**Ms. Hardin:**

One thing I will say is don't give up when you are applying for Social Security disability. It's pretty automatic that you're going to get denied the first go round, and we tell people they need to go back and re-appeal, and as they go up the ladder and they re-

appeal, the timeframe get shorter, and your chances of getting approved are much better. We deal with this throughout the rare disease community. Most people are chronically ill that have rare diseases, but I certainly would tell her that if she can show that she doesn't qualify for Medicaid or disability that she definitely needs to get in touch with BPPS. We are in constant communication. If they are able to find insurance for her and she cannot afford the premium, she certainly could become a candidate for the NORD program.

**Andrew Schorr:**

Okay, thank you for that. Now here's another ripple in all this. Jim writes in, and he said, 'How to you deal with self-insured companies for Kuvan coverage, especially with diet and formula being the most prescribed regimen up until this new era of Kuvan. When the insurance administrators see the cost of Kuvan, they will tell the employee that the diet is the best method and will not cover Kuvan.' Tony, you may have encountered that. Help us understand what these self-insured program.

**Mr. Wicks:**

Well, typically, the self insured fund means that it's actually funded by, it's typically funded by an employer for example, and in those kinds of situations even whether it's a self-insured plan, or even if it's a larger plan, you're going to see these issues play out regardless and especially when it comes to diet and formula versus prescription drug. A really important thing to remember is that those two things are covered under very different benefits, and really it's not up to the insurance company to decide the appropriate treatment regimen. It's really a decision that's made by the provider as well as the patient. So in those situations where an insurance company is saying we are not going to cover it because we think that food and formula is an appropriate regimen for this patient, again, we're going to have to appeal that decision and we're going to probably have to use an argument of why it is medically necessary for this patient be on Kuvan, and that again requires some assistance from your physician as well as dietitian and from you as far as building that argument with the insurance company.

**Andrew Schorr:**

Now PKU is a rare condition, and the first time you go at the insurance company, they may say, whoever you get may say, 'I don't know anything about it,' or maybe they know about the medical food or formula, but as we just heard in the last question. They don't know anything really about the medication or what a difference it can make. So that's when appeals come in. So tell us about how you help navigate the appeals process, and what success you've had with that.

**Mr. Wicks:**

The way that the appeals process works with Kuvan is actually a little bit unique. The first thing that we actually want to do is make sure that someone is a good candidate for Kuvan. What we do is once we receive at a denial from the insurance that patient will then go on a 30-day trial of Kuvan, and the reason for that is because we want to give the patient and the provider an opportunity to make sure that that person is a

good candidate for therapy. So they go on the drug for 30 days. At the end of that 30-day period, they actually measure Phe levels to see if there is a change in Phe levels and really to just gauge whether or not this patient is a good candidate. At that point, they sent that information in to us, and if they want to go forward with the process of appealing, we will then commence that process.

So, what really helps us in that situation is now not only are we drafting an argument about why this patient should be on therapy, but now we've got clinical evidence that this treatment works for this patient. So we then proceed with drafting a letter coming up with our argument for why the patient should be approved for therapy. In some cases we're going to provide some additional educational information about Kuvan and various treatments for PKU just so we can educate the insurance company a little more, and what you'll see is that typically as you go through the appeal process which each level of appeal it's going to be a slightly more clinical more technical person who's going to be reviewing that case, so it can either be a straightforward process, or it can be a pretty long drawn out process, but the key to going through the appeals process is really persistence, staying on top of them, and making sure that you're coming up with a thorough argument, which is what we're able to really do when we engage the physician, as well as the patient in that process.

**Andrew Schorr:**

AnneMarie, you're a New Yorker. New Yorkers can be pretty feisty, and you run a support group there. What is say to people as they navigate all this? One is I want to get the right treatment for myself or loved one; oh my god, I want to get this financial support of its available, or certainly get what is due from any insurance that I have or my employer does if I have a job. So what advice would you give people?

**AnneMarie:**

Well, a lot of people with self-insured programs are usually somehow involved with the union, and a big help I know with us was getting in touch with the union rep because they often know somebody at the insurance company that might be higher up that can help you. I'm talking about as far as medical food and formula, they often know somebody that could get you in touch with the right people, because I have to say PKU awareness is a major thing. Nobody knows what it is, like you said. You call, you get some kind of representative from the insurance company, and they have no idea what PKU is. They tell you it's not covered, all kinds of things.

**Andrew Schorr:**

They give you the quick answer.

**AnneMarie:**

My advice is to get in touch with somebody from a union or go to a medical management program within the insurance company.

**Andrew Schorr:**

Right, of course, what's neat is Tony is that you've got people there at BPPS that go to bat for you, and so this can otherwise be very stressful. I think it's a wonderful service. I want to give that number again, 1-877-MYKUVAN, (1-877-695-8226).

Here's a question we got in. It's probably best for you Maria. It's from Laura Duncan who's actually a nutritionist. She has all sorts of letters after her name that she sent in, and she writes in, 'There are some insurance companies that have \$1 million cap on reimbursements. We are concerned that some of our patients with PKU may reach this cap when using Kuvan over an extended period of time, especially if they have some other experience which results in unintended medical treatment. What recommendations do you offer?'

**Ms. Hardin:**

Okay, well first of all, in the calls that we have been taking in talking with the patients, there is a misconception out there that the cap on the family plan is based on all of the family. It's each individual. In other words, the child with PKU or the family member with PKU would have to reach that million dollar mark. It wouldn't be an accumulation of other coverage for other members of the family. So we've been explaining that to people because it is something. This million dollar mark, I can remember 15 years ago, if you had a cap of \$1 million, you would never reach it in your lifetime, but with all of the new drugs that are coming out now, we are finding that \$1 million is nothing. I would really like for that to get out into the PKU community, and I think Tony would agree with me that this is something that is on people's minds, and when they start going on therapy this is one of the big issues that we hear when we're talking with the parents.

**Andrew Schorr:**

Okay, thank you for that. Here's another question we got in from Mary in Sullivan, Indiana, and she writes in, 'I have a five-year-old son with PKU and I'm wondering about insurance coverage for food. Our insurance currently covers formula that 80%, but we have not explored the option of food coverage yet. Our insurance plan is an ERISA. Are there different steps to follow with these, and what are those steps to finding out if insurance will cover any of the food costs?' Who wants to start with that one?

**Ms. Hardin:**

I think we need to let our listeners know what ERISA stands for. It's the Employee Retirement Income Security Act, and there's a website that people who are involved in ERISA can go to, and if you'll just wait a minute I'll pull it up here. It's [www.DOL.gov/EBSA](http://www.DOL.gov/EBSA), (DOL stands for Department of Labor) and when you go on there, there's an article that says "Ten Top Ways to Make Your Health Benefits Work for You" if you are under ERISA, and under item number four. I was really intrigued about this, and they tell you that your plan summary that you get provides a wealth of information that you should read. I know when I get these I never read them, I just tuck them in

my file cabinet, and at the end of it, but it's a wealth of information. When you are involved with such a serious situation like this wondering if you're going to have coverage and how much are going to have, and what it's going to cost you a pocket. Tony, I don't know if you want to add any more.

**Andrew Schorr:**

Tony, anything you want to add?

**Mr. Wicks:**

I think you pretty much covered it. I mean, I think the other thing that you can definitely do, and I'm sure there probably is a number on that website, but any situation like this, in which you're trying to get an idea of what your coverage looks like for food and formula, the first thing I would do is actually call the number that's normally provided where you can get information about your benefits, simply because they can provide you a little more detail typically regarding what your benefits are going to look like for food and formula on that particular plan.

**Andrew Schorr:**

Okay, I believe we have a question on the phone. I believe we have Shawna calling in from California. Shawna, are you there?

**Caller:**

Yes, I am.

**Andrew Schorr:**

Hi Shawna. So is it you or a loved one who has PKU?

**Caller:**

I have two boys, 10 and 11, with it.

**Andrew Schorr:**

Wow, and we just had, you heard there was a question from somebody with two children, 15 and 12, okay, well what's your question?

**Caller:**

My question is, with the Kuvan if they go on it for a period of time, and then they have to get off of it for some reason, is that going to affect them medically if they happen to be on it for a certain amount of time, and then suddenly having to stop?

**Andrew Schorr:**

Well, that's a medical question maybe we're not great at answering today, because we had it in our last program, you know all the Ph.D.'s and super MDs, and we're going to do that again, but let's turn that around a little bit if we could. If for any reason from Tony's point of view, because he deals with supporting people, if somebody is on the drug and then for whatever reason between them and their provider they stop, and

actually Corinna who we had in the last show, her son did stop for a little while, does not create any problems? You work so hard to get it going and get it paid for Tony, and if for whatever reason there's sort of a hiatus, is it a problem starting again, or how does that work?

**Mr. Wicks:**

Generally the only risky one is, depending on the amount of time that the patient goes off therapy what could happen is, I don't know, their insurance might change or if they're coming up towards a time where they might be due for a recertification since most of the insurances will cover it for a certain period of time and then set a date at which they reevaluate that coverage. If the patient is off therapy during that period it could just very well be that that patient's benefits have changed. Typically, if somebody is going to go off therapy for a while, just notified BPPS, and we'll actually set up a task to follow up at a certain period of time to make sure that none of that information has changed, and if it has then will evaluate what kind of impact it may have.

**Andrew Schorr:**

Okay Shawna, I hope that helps. We'll get to the clinical issues maybe again in our next program, and hopefully we'll do others, and also listen again to the earlier program we did just earlier in the month with the two medical experts too. All the best to you.

Here's a question, we just got in. We just had Shawna, and now we have Shayna, and Shayna writes in, and I don't know AnneMarie whether you can weigh in on this one, but she's talking about her kid in school. Does the school have to pay for special lunches that are offered to students with PKU by the special companies, or is it the responsibility of the parents? She said she had heard that the school should pay since it falls under the Americans with Disabilities Act, but she's also heard that it's the parent's responsibility. Which is true? AnneMarie, you know?

**AnneMarie:**

I think it varies, but there is some kind of federal law. I never had to deal with it with Devon, but I know other parents that have, and I do believe the school is supposed to provide the lunch. I know Cambrooke's Green Light program, and they have a lunch program also that deals directly with the school, so she might want to call Cambrooke Foods on that, and they might be able to help.

**Andrew Schorr:**

Maria, do you have any information on that?

**Ms. Hardin:**

No, but it's a good topic to look into since so many of the patients of school age.

**Andrew Schorr:**

You know what, we're going to cover that. This is a wonderful opportunity for me because we have another program that's going to come up on June 19th. Take a look

on our website in the special edition section of [www.patientpower.info](http://www.patientpower.info), and you'll see June 19th Living with PKU, Patient Approaches to the Management of PKU. So we're going to give family to family information, and some other experts, and so will deal with some of those school issues as well too, so lots there.

Okay, let's continue our discussion. We've got another question for you. This is from Erica in Fall River, Massachusetts. I imagine, probably for you Maria. 'I've been off diet for 32 years, and I started Kuvan about a month ago. Kuvan is expensive for me, and I understand why. My insurance makes you pay a co-pay of \$50, which I can't afford. Will Kuvan come down in price?' You may not know that one, 'But in Massachusetts medical food and formula is no charge to you. Insurance covers it all for PKU. Any chance this will be the case for Kuvan? I don't want to have to stop taking Kuvan, because I can't afford it, and what if you change medical insurance companies or lose health coverage or get coverage by the state?'

We'll get to that with Tony in a minute. But what about the question of the co-pay? Let's start with you Maria on that. Help us understand that.

**Ms. Hardin:**

My first question would be, has she applied to the BPPS program, and she would be referred to NORD, and we would certainly look at her case and possibly relieve her of having to pay that \$50 a month, which obviously is hard for her to do. So that would be my first suggestion.

**Andrew Schorr:**

Now, it's Massachusetts, especially a good place to live related to some of these benefits that the state provides?

**Ms. Hardin:**

Some people say so. You know, if it's one of those states that covers food and formula at no charge, that's a blessing, because there are, as I say, there are 13 states that don't have any kind of assistance whatsoever.

**Andrew Schorr:**

Right, it's all over the map. Tony we talked about, where you may be changing insurance companies or you lose your health coverage or you lose your job, or you're in a different state now, but again, it sounds like you are there to help them navigate that.

**Mr. Wicks:**

That's correct, and also, you know, another question came up about moving over to state programs. We have definitely received approvals from Medicaid in numerous states, and whether it's a state program, versus a commercial plan, we are going to go through the exact same kind of process to try to make sure that a person is able to

continue on therapy, and again if we run into a situation where there's no longer coverage, they will be evaluated for patients assistance.

**Andrew Schorr:**

If someone calls there and then the NORD program comes into play, then you've helped make that connection.

On your end, Maria, is there someone like at the BPPS who helps them and sort of takes their case, if you will? How does that work?

**Ms. Hardin:**

Yes. I believe some of the patients had spoken to Evelyn, and then there is Jane Kane, who is another contact there, and I am certainly available to talk, if they can't reach either of them. As I say, we run 40 programs, and we have a pretty seasoned, well-trained staff. It's just that sometimes you might not get a live voice at that moment, but we do have a policy that we return the call as soon as possible.

**Andrew Schorr:**

Okay, that's so neat, and of course we mentioned about BPPS as a starting point if Kuvan is part of what therapy is right for you or a loved one, or there is that trial going on as well.

So Tony. When can they call, and when do you call them? So how does that work, and how quick, do you get a live person right away? If I were to call now, if someone in my family were diagnosed with PKU, the doctor writes out a prescription and says we want to try Kuvan, then how quickly things move along?

**Mr. Wicks:**

So you can call us at any time. There are live people on the phones from 6 a.m. to 4 p.m. Pacific Time, and once you call in you will be triaged to your appropriate case manager, who can assist you. As far as initiating the entire process, once the prescription is received, we will actually be reaching out to you. So we will enter all the information into our system, and your case manager will call you and introduce themselves and will give you an overview of what the process entails. For some people, like I said, it can be pretty quick, and in other cases there is a lot of red tape that we may have to go through, but in all those situations there is a case manager that you'll be working with who will guide you through that process.

In regards to Erica's question as far as if, for example, you have a situation where you think you're co-pay is a little bit too high, again, you can reach out to 877-MYKUVAN, and we will refer them over to NORD for assistance.

**Andrew Schorr:**

Maria, you've been at this a long time. Tony has too, but I know you've been at this many, many years. It sounds like at least in the case of PKU now it's coming together,

that there is a really pretty robust support programs that have shaped up for people. How would you characterize it?

**Ms. Hardin:**

All I definitely would say that it is robust. Again, this is a new kink in the out-of-pocket costs to patients having to pay high co-pays tiered, drugs that are on specialty tiers and the like, it's very disconcerting, so I would say that we are, the wonderful thing is that we are dealing with a company that runs the whole spectrum of the PKU needs, and we're very impressed with that. That's been a wonderful thing to see, and we use this as a model in many of our other programs when companies come to us and they want to establish something for a particular disease state.

**Andrew Schorr:**

Well, I'm really, on the outside I'm a cancer survivor, but observing this, I think there's tremendous support. Tony Wicks, I want to compliment you and your organization and mention again, you've said the phone number many times, but I think people would be foolish not call. I think it's worth the call. 1-877-MYKUVAN (1-877-695-8826), and Maria Hardin with NORD, thank you for all you do, and then also these programs that you're announcing as well for medical food and formula, that just really rounds everything out, so thank you so much.

Now I want to give the last word to somebody who lives with this every day as she looks at Devon, who's a pretty active kid now, isn't he AnneMarie?

**AnneMarie:**

Yes, Devon is active.

**Andrew Schorr:**

Okay, yeah, she said that like an understatement, okay. Now he's going to be a teenager, wait for that, but the blessing is that you are able to keep his Phe levels under control. So I guess nobody should feel alone. You have your support group in Suffolk County, but nationwide, people should feel like we're all in it together and that there is support.

**AnneMarie:**

There is support out there, and I have to say that BPPS, they've been wonderful with us as far as the Kuvan goes, and they were very helpful, and they kept all their promises. They got Devon covered, and that was the most important thing. Another important thing that I wanted to say was being on the Kuvan, he doesn't need as much formula, which is also a good thing that the insurance can look at too because he doesn't need as much formula is needed before being on the Kuvan.

**Andrew Schorr:**

Well AnneMarie, you've given me the opening to talk about our next show that is going to be on all these issues related to living with PKU, and there will be a lot of information

sharing going on there. I want to wish you all the best with Devon, and may you dance at his wedding and have grandchildren, and he's going to be keeping those Phe levels controlled for a lifetime. All the best to you, and thank you for joining us. Tony Wicks, thank you with BPPS, and Maria Hardin, thank you with NORD.

I want to mention that our next program, the third in our series, is June 19th at 8 p.m. Eastern, 5 p.m. Pacific, Living with PKU, Patient Approaches to the Management of PKU, and the information is all on our website, the special edition section of [www.patientpower.info](http://www.patientpower.info). Thank you so much. Send me an e-mail just at [PKU@patientpower.info](mailto:PKU@patientpower.info), let us know how we did, and remember knowledge can be the best medicine of all. Broadcasting live from Seattle, I'm Andrew Schorr. Thanks for joining us.

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