January 18, 2022 Patient Education Webinar- Advocacy and Rare Disease Week 2022

Amethyst: Welcome everyone and thank you for joining us to learn more about Rare Disease Week 2022 and how you can become an advocate and share your story. This call is now being recorded. I'd like to thank you all for being on the call with us today and to our sponsors, Genentech, argenx, and Cabaletta Bio for making today's call possible. “Information is a key factor in treating and living with any condition. However, every patient's situation is unique. The IPPF reminds you that any information found on the Internet or during presentations should be discussed with your doctor or healthcare team to determine if it applies to your specific situation.” Now, before we begin, I would like to take a quick poll to see how many of you on the call, this will be your first year that you'll participate in Rare Disease Week? I'm going to go ahead and launch that poll and if you don't mind answering that real quick. And while you're doing that, let me introduce you to our guest speakers for today's webinar.

Amethyst: Katelyn Laws is the Rare Disease Legislative Advocates, RDLA, program coordinator for Every Life Foundation. Katelyn attended American University where she graduated with her master's in public policy. Before attending American University, Katelyn received her Bachelor's degree in Political Science at Appalachian State University in the mountains of North Carolina, where she grew up.

Amethyst: I'm going to go ahead and close that poll. So it looks like for many of you, this will be your first year participating in Rare Disease week. So we're really excited to have you advocate with us and thank you all for joining us today.

Amethyst: Our next speaker is Marc Yale. Marc is the IPPF’s Advocacy and Research Coordinator. In 2007, he was diagnosed with Cicatricial Pemphigoid. Like others, with rare diseases, he experienced delays in diagnosis, and difficulty finding a knowledgeable physician. Eventually, Marc lost vision in his left eye from the disease and this inspired him to help others with the disease. In 2008 he joined the IPPF as a Peer Health Coach and from 2016 to 2020 Marc served as the IPPF’s Executive Director. Marc continues to work with people to improve their quality of life and encourages them to become self advocates. Mindy Zimmerman is a PV patient who lives in South Florida. She is an active advocate for the International Pemphigus and Pemphigoid Foundation. Mindy is a Healing Hero, one of the leaders of the South Florida Support group and a patient advocate on behalf of the IPPF. Thank you all for

joining us. Before I begin, I would like to go over a few housekeeping slides… (Reviews Housekeeping Rules).

Amethyst: Now it is my pleasure to introduce Katelyn Laws, Marc Yale, and Mindy Zimmerman to tell us more about becoming an advocate and Rare Disease week. I will send control over to you here, Katelyn.

Katelyn: Great. Hi, everybody, my name is Katelyn. I am with the EveryLife Foundation for Rare Diseases. I am so excited that for so many of you, this will be your first Rare Disease Week. So I'm going to share with you a little bit more about what you can expect during Rare Disease Week and give you a little more details about what the week is going to look like. For starters, Rare Disease Week is virtual this year. We are located in D.C. and Covid is not so great here right now. So just for the safety of everyone, we're gonna continue virtual this year.

Katelyn: Registration for Rare Disease Week is now open. You can find the agenda, register, everything you need to do is at rareadvocates.org/rdw. Registration is going to be open until February 11th. So you have about three weeks left to register, so you can go ahead and go to that link now and get your registration in.

Katelyn: So here is the agenda for the week. We're gonna start on February 22nd where we will host our Rare Disease Caucus Briefing. That same afternoon, we'll host a documentary screening, and then the next day will be the first day of our legislative conference. Legislative conference is an opportunity to dive a little deeper into the different bills and federal legislation that you're going to be speaking to your members of Congress about later in the week. It's also a great opportunity to network with other advocates. So we will have the first day of that on Wednesday, February 23rd, and then that will carry on into a second day on Thursday, February 24th. Also that afternoon, we will be hosting a YAAR Meetup. YAAR is our Young Adults of RDLA representatives. These are our young adults ages 16 to 30, so you have to be within that age range 16 to 30 to attend the meetup but you do not have to be a current member of YAAR to attend. This is a really great opportunity for young adult advocates to network and meet each other, and learn about different opportunities. So I highly suggest anyone within that age range, attend. Also, just a note that the YAAR Meetup registration is separate from the Rare Disease Week Registration and you can find the link for that on the Rare Disease Week website. So then the next week on Monday, February 28th, we will have

Rare Disease Day at the NIH. And again, this is a separate registration link and it can also be found on the Rare Disease Week website. Then on Tuesday and Wednesday, we will meet with our Representatives and Senators. So these meetings, they're going to look a little different depending on what state you're in. If you are in a state like Florida, New York, California, we have a lot of advocates joining us, so you will be in larger meetings, which means the speaking parts will be split up differently based on where you're comfortable with. If you're in a smaller meeting, everyone will have the opportunity to speak, if they would like to. How much you participate in these meetings is completely up to you but we will provide you with five different Asks that you can go in and ask your member of Congress to complete. We're still working on finalizing those, but one is always asking them to join the Rare Disease Congressional Caucus. Just for example, we provide you with a page on what the caucus is, what they do, who are current members, so you have all the information you need, and we provide you with a page for each of the Asks. It's kind of like a little cheat sheet for you and then you can leave it behind at the meeting for the member's office to refer back to, should they have any questions once you leave. We really prepare you for those meetings. I know it sounds intimidating and it can be overwhelming but we offer these training webinars. Again, these you register for separately, the link is on the Rare Disease Week website. As I stated before, registration will close on February 11th. On the 2nd we will have our first training webinar. This is for anyone to attend. You don't have to be a new attendee, it doesn't have to be your first time attending, this to open for everyone. We will just cover what you can expect in your meetings with your members of Congress, will give you a few tips and tricks, and make sure that you're prepared and feeling good going into these meetings. On February 9th, we will have a webinar, specifically for our team coordinators. As I stated before, we do have states that have a large number of participants and for those meetings, we assign a team coordinator. and This person just helps organize the meeting, helps keep everyone in contact with each other. They really just make sure that this is as easy as it can be for a large group. So, we'll meet with our coordinators and go over what is expected of them. You are only required to attend this if you're a team coordinator. So don't worry about that one if you don't want to sign up for it, you don't have to. It's on the registration page if you're interested, you can click Yes and if we need, you will contact you. Then, on February 10th, we're going to have a Share Your Story with Policy Makers webinar. This is new this year. It's going to somewhat look like our Fast Forward to Rare webinars, if that's something that you're familiar with. Basically what this webinar is, is just a chance to practice your rare disease pitch that you would give to your member of Congress. We'll split into breakout rooms and we have coaches in each room. Everyone will go around and practice giving their pitch and you'll receive tips from your coach and also from fellow advocates who are in the breakout room with you. So, this is a great opportunity to really practice what you're going to say when you go into your meetings. Then, on

February 11th, we will host a virtual office hour for our new attendees. This office hour is just for people who are brand new to Rare Disease Week. If you attended the training webinar and you still have some questions or if you couldn't attend, this is a great time to come and ask any questions that you have. It's very informal. It's just going to be Shannon and I sit in a zoom meeting, waiting for people to pop in and ask questions. So you can pop in and ask a question and leave, you don't have to stay the whole hour, but I recommend staying and listening to everyone's questions because someone may have a question that you haven't thought of. It's a really great opportunity just to get all your questions answered and any details ironed out before we go into the meetings. Then on February 14th, we will have our virtual office hour for everyone, again, it's going to be the same layout, just a chance to come ask questions. Then on February 15th we will have our last office hour only for team coordinators. If you opted to be a team coordinator and you received an email, but you still have questions, this is a great time to come to get those questions answered.

Katelyn: Now for our next slide. Last year was the first time that we were able to do the scavenger hunt for Rare Disease Week and we're going to do it again this year. For this scavenger hunt, advocates will have the chance to win up to $5000 for their favorite Rare Disease organization during Rare Disease Week. The way this works is you can earn points for attending events, virtual hill meetings, and other activities during Rare Disease Week. Whenever you attend a webinar you will get a code. You just go into the platform tab type in your code and get your points. Please note that all of the events I just talked about will be recorded. If you can't attend at that time, you can watch the recording but you will not receive points for the recording. The points are only for those who attend the events live, just to make things fair. The top 50 point earners will win between $1,000 and $6,000 for their organization of choice. The donation will be made by us in your name. This is a great opportunity, not only to come learn more about what is going on in the rare disease space on the Hill, to talk to your member of Congress, and advocate for yourself, but also a great opportunity to give back to the organization that you're a part of. So, I encourage everyone to register. We have a few states that, as of right now that we have low registration in. So if you're in Wyoming, South Dakota, Puerto Rico, North Dakota, Montana or Mississippi, then I encourage you to register and share this event with any of your friends in the rare disease community in those states. We want to make sure that we have as much representation as possible on the Hill, just to get the word across and get some changes made on these pieces of legislation. I hope that you all can join us, and I'll take any questions that you guys may have.

Amethyst: Great, thank you. That was a great overview and we all hope that you will join us after learning a little bit more. Katelyn, real quick, do you mind going back to this slide with the registration website just so everyone can jot it down? If you have not registered yet for Rare Disease Week, please go online and register with that link that Katelyn is going to share and then afterwards, if you will contact Marc and I'll share his information as well. Just so we can set up a personal IPPF training.

Amethyst: I'm also going to invite Mindy Zimmerman to come on screen with us to share a little bit about her experience with Rare Disease Week. Mindy, are you available?

Mindy: I am here. Can you hear me?

Amethyst: Awesome. Thank you, Mindy, for joining us as well. Mindy is an amazing advocate for our community. Mindy, I have some questions for you. Now, that we've heard a little bit from Katelyn about what Rare Disease Week is and how they can participate and the meetings that are required, do you mind sharing a little bit about your experience and why do you feel it's important for patients like those on the line to participate in Rare Disease Week?

Mindy: Absolutely. But first let me just let you know about 45 minutes ago my power went out in my neighborhood, so I'm doing the best I can. Sorry, you can't see me. I guess I didn't set up a camera on my phone. I have about 40% battery left on my phone so hopefully, I'll be able to stay for the whole meeting.

Amethyst: Well, thank you for joining us, despite that little hiccup.

Mindy: I feel it's really empowering. The first time I went it made me feel like I can make a difference and help to make change. Telling my story was something I was able to do the first time I was on Capitol Hill. Wow, it made me feel so empowered. I had the advantage, I did have a Congressman who did Cosign one of the Asks that I asked for and it made me feel like I made a difference because I was listened to. I'm learning that it's a process, everything doesn't happen that fast, and that it's important for all of us to tell our story because it's about our stories, it’s about my story, that made my legislative aide listen and pass that onto my Congressman and that's why change started to happen. So that's why I think everyone should get involved.

Amethyst: Great, thank you. Real quick, you talk about sharing your story, can you kind of explain to everybody since a lot of the people on the line are first timers, what does it mean to share your story? How does a meeting go? How much time do you have to share your story?

Mindy: Yes. Well, I'm in one of those states with lots of people, I live in Florida. So typically, at our senator meetings the person who is the team coordinator selects three to four people to share about their story. And your story basically is a little bit about your disease journey and how legislative action that we're that we're asking these these legislators to vote on would have changed your journey to make it maybe a quicker diagnosis, or maybe not have to have the step therapy that you had to go through for your rare disease or other things. Meetings are only 15 to 20 minutes. That was an eye opener for me. So in 90 seconds you have to say a lot, or a minute, or three minutes, or five minutes. What you want to be able to do is figure out a way that your story fits in with the legislative Asks. Like I said, whether it's step therapy, or finding a cure, or finding new funding for, I don't know what the Asks are this year so I am not exactly sure but basically finding out about the funding that is available and how that works with rare diseases and clinical trials. Depending on what the Asks are, how it fits into your journey of your rare disease.

Amethyst: That's really good advice because like you said, your story is very powerful. And really a lot of these Asks impact our disease community as a whole and hopefully, you can relate to some of these Asks personally. It really makes a difference when the aids or if you meet with a representative, hear your story and how this legislation truly impacts you or could have made a difference in your journey. Great, thank you. Like you said, the first time that you did it, obviously, you were probably a little bit nervous and I'm sure many people on the call are as well. Do you have any good advice for advocates on the line, how to kind of muster up that courage? Or any good advice on how to encourage them to share their story?

Mindy: Sure, absolutely. Actually, my first visit to Capitol Hill was virtual, which yours will be as well and that made it easier. I think for me. I've talked to advocates who have done it live and who've only done virtual, or done both. But for me, the travel right now is a little scary, so I wouldn't travel even if it was going on. But I'm not a good traveler. So for me, being virtual really helped me to decide to advocate. The first time, I was really anxious. I spent hours and hours researching the legislative Asks, despite the fact that I got all the information I needed from the RDLA because they really did a great job of presenting the information, the one pagers and more information if I needed it. Also, Marc with the IPPF was instrumental in giving me additional

information and how maybe it relates to my specific disease of Pemphigus Vulgaris and how I can relate that to my legislator's aids. All that information that I got, I decided to spend hours more because I was a nervous wreck and I tend to overthink things, but you don't need to. You don't need to do that. I attended the Rare Disease Week RDLA webinars, they were extremely helpful. The staff, including Katelyn, who is on this call are easy, accessible, and willing to help. If you have any questions. There's no reason to be nervous, apprehensive or anything like that. The same thing with Marc Yale, he is extremely knowledgeable. He has been advocating, he'll tell you how long, I don't know how many years, but for a while. And he understands the system and he can help you with those questions that you have. I believe, for those of you who are doing this for the first time, he will plan special webinars just for you, so you can get those questions answered. A few tips that I wrote down when I was preparing for this call today. You have to be comfortable. Believe me, being on the phone and you're not seeing me right now, is not comfortable for me. So, I have a glass of water because I have to hydrate, I get cotton mouth that's what happens to me. So hydrate. You hear about hydrating all the time. I live in South Florida, we have to hydrate all the time. Maybe you can tell by listening to my voice that I'm smiling because that's important. When you smile it is seen and heard, it really is. So, practice, practice, practice, practice, don't over practice but relax and be comfortable. And I think one of the most important things is, when asked a question by the aids, who are usually the people who attend. On one of the advocacy events that I did other than Rare Disease Week. So one one of those that I, the Representative, actually showed up. Typically, they're doing what I'm doing. They call from a phone and you don't see them. Don't look at that as a negative. I know some people who've gone to actual Capitol Hill for Rare Disease week on Capitol Hill in person say that the experience is very different and virtual, but the virtual experience, even if they're just on the phone, try to keep them engaged by asking questions as you're talking and don't be disappointed that they're just there and you don't see them. Make the same empowering speech that you would make if you were face-to-face with someone because that'll make a difference. You can hear the emotion in my voice, even though you don't see me, remember to smile, relax, and don't do what I did, I took a little too long, be brief. Thank you.

Amethyst: Those are great tips though. And I think you're right, Mindy. It's just as important. It's definitely different but these bills and these laws, things are getting passed every day. So even though we're not there on the Hill, we have just as much impact virtually. It's important to represent our community. So thank you, those are amazing tips. I have one other question for you. You were talking about how things don't necessarily happen all the time or as quickly as it did for your first experience where your representative signed onto the bill, co-sponsored the bill. What would you say about your interactions after Rare Disease Week? How important is it to stay

connected with the aides or your representatives afterwards via email? Or are you having many relationships with them afterwards?

Mindy: Absolutely important. It's about the relationship. As time goes on, I expect that some of the aids will continue to be the same aids, and they'll get to know me more and they'll recognize my name. I've communicated by email after the meetings, and then try to follow up every few months. Not too often, unless something's about to be voted on, then maybe a little more often. But if my name is one of those names that comes across the desk more often, my story will be remembered. That's how it works. It's all about the relationship. Don't worry about getting the email information, the RDLA is awesome. This information was given to me before my meetings, and if you don't have an email for them, you can always ask at the meeting for that email. Thank you.

Amethyst: Great. Thank you so much Mindy for sharing about your experience. Just to let you know, Rochelle wrote in and said, “Thank you, Mindy. You have given us excellent advice and great tips.” We appreciate you being on the call with us, and I'm going to invite Marc now on the line to share a little bit about the IPPF’s advocacy efforts and what the IPPF hopes to do this year at Rare Disease Weeks.

Marc: Thanks Amethyst. And thank you, Mindy and Katelyn for presenting today. Thank all of you for joining. I see we've got some return advocates and there are lots of new advocates attending today's webinars. So thank you all so much for joining. My name is Marc Yale, I know many of you and I am the IPF Advocacy and Research coordinator for the IPPF. Both Mindy and Katelyn talked about how intimidating and how scary this process can be. And I have to tell you, my very first time to Capitol Hill back in, I think it was 2013, I was really scared and I just didn't know what to expect. It turns out it was one of the best and greatest experiences that I've had in my life because it really helped me understand, not only the process, but really helped me share my story about my disease. As Mindy said, it really empowered me to be able to create disease awareness about this disease and speak up for others with the disease. Like many of you on the phone, after being diagnosed I really wondered, why don't more doctors know about this? Why don't more people know about this disease? And advocating on Capitol Hill is one way, one of the many ways that we can create more disease awareness, shorten diagnosis times, as Mindy mentioned and just help ourselves and others that have this disease. Next slide, please.

Marc: I want to talk a little bit about why, why do we advocate? I mentioned a little bit already, why do we do this? Well, we want to speak up for everyone that's like you. You

have the opportunity to represent not only yourself, but others with the disease and not everybody can do this. So by meeting with your representatives, virtually this year, you'll have the opportunity to speak up and speak out about your disease and create that awareness. Really, advocating encourages and creates that positive change for legislation that benefits all of us. I think we all know that the environment in Washington right now is very challenging. The thing that I really learned, starting to advocate, is that change only happens if you speak up. The people that are speaking up and sharing their stories and sharing their voices, that's how things happen and that's how things change in Washington. So it's really important that we do that. We talk to you about creating disease awareness. Super important right now. Our diseases are rare diseases, pemphigus and pemphigoid are rare diseases and there are not great therapies or treatments for these diseases. By creating more disease awareness, it encourages drug developers, encourages funding, and really will lead to new treatments. It encourages new research. All of these things are very important pieces in developing new treatments and therapies. Then of course, helping future patients that have the disease. When I started to get involved with the IPPF I had a pretty severe disease activity. I had Mucous Membrane Pemphigoid and it covered my entire body. I decided to get involved with the IPPF because I didn't want people to experience what I experienced. I was hoping to make a difference in other people's lives. It was very cathartic for me, it was part of the healing process for me to be able to help others with the disease. So I encourage you to and obviously advocate because it can be part of that healing process. Just remember that you lead the way in voicing the needs of our community. As I said, not everybody can do this. You have the opportunity to share your voice and be a leader in our community. Through the IPPF's efforts, we do work with RDLA and EveryLife Foundation and the National Organization for Rare Disorders, we work with a lot of groups, like minded advocacy groups, that help us understand federal and state legislation that impact our community. And that really helps us decide what the IPPF priorities are going to be as we move forward. So, just keep in mind that together, we can do this. We're a team, we're a community, and together we can do this. Next slide.

Marc: I just want to go over briefly, when we talk about advocacy for the IPPF, one of the things that IPPF is looking to prioritize and this is based on things that we've heard from our community. This is based on the feedback that we have received from patients like you on the line. So, the things that we have prioritized are the patient perspective in the drug development and approval process. It's very important that we are sharing our voice and providing what's important to patients, is extremely important. So we need to be telling the FDA that about these things, we need to be telling drug developers about what's important to us as patients, so that when they are developing drugs or drugs are being approved they keep all of our interests in mind. Extremely important. This year,

we plan to have what they call a Patient Focused Drug Development Meeting, where we can help inform the FDA about what's important to us. Simple things like quality of life issues, like just being able to brush your teeth or take a shower or even eat. These are all important aspects of our disease and that's what's really important to us. So we need to make sure we're informing both the FDA and drug developers about these things. Next, we really focus on things like access to different therapies. So there's a lot of drugs out there right now that are not approved by the FDA even though they're being used by all of our doctors as part of what we call the standard of care, but they're not approved by the FDA. And so sometimes our insurance doesn't give us access to these drugs. So we really want to make sure that we focus on getting access to those drugs for all of our patients in the community. Then there's things like restrictive formularies or things that aren't on what they call a drug compendia. There are certain drugs you are not allowed to have based on your insurance. There's things like step therapy. I know Mindy mentioned that, where insurance companies ask you to try certain drugs before you get the lifesaving drugs that you really need. So there's that type of priority for us. Then there's drug pricing. We all know that the price of drugs is just outrageous so we really want to make sure we're focused on the pricing of drugs to make sure they're accessible and affordable for patients. Make sure that there's transparency in the process so that we know what we're being charged for and why we're being charged for it. Then try to eliminate any price spikes, so if there's drugs that used to be a certain price and all of a sudden the insurance companies are charging us more. Then there's also a thing called tiering, where depending on the drug, the insurance company will charge a certain price depending on what tier it's in. So that's certainly an issue for us. Pre-authorization is another huge challenge. Many of us go to the doctor and it takes them forever just to get the authorization from our insurance company to give us the drugs they're prescribing, and so that pre-authorization issue causes delays in treatment, which of course makes our diseases worse while we're waiting just to get the drugs that are doctors want to give us. Then co-pay assistance and co-pay accumulators. These are all insurance issues where insurance companies are making it more difficult for patients to get the therapies that they need and charging exorbitant amounts of money just for patients to get them. Next slide.

Marc: I want to just quickly talk about our Asks for this year. Katelyn mentioned that the RDLA will have a set of Asks that they'll put out. When you're in your meetings, you'll be in a group with maybe a large group or small group as Mindy mentioned it just depends on what size your group is. There will be Asks that you'll be putting in front of your legislative aides and or your Congress members. Some of those include, as Katelyn mentioned, joining the Rare Disease Caucus, or particular Asks. You'll be in these meetings with other advocates from other advocacy groups so some of them might have their own individual Asks, along with the RDLA Asks. The IPPF this year

has developed three specific Asks based on those policy priorities that I just mentioned in the previous slide that really affect our community directly. I want to just take a few minutes and go over those Asks with you. We call them Asks because what we're asking to do is, we're asking our congressional members to co-sponsor these pieces of legislation. What happens is, Congressional members introduce legislation and then they get other members of Congress to co-sponsor this legislation. Then if they get enough sponsorship or co-sponsorship for this legislation, then it goes to the floor for a vote. Then once it's voted on, then of course, the legislation can be passed and then become a law. So the co-sponsorship process is an extremely important part of the process to get these particular pieces of legislation and this policy to become a law. Really important. The first legislative Ask that we have this year is around what I had mentioned, patient perspective in the drug development and approval process. The first piece is called the BENEFIT Act, because it will benefit all of us. That is H.R. 4472 and S. 373. You might be saying, what are these H.R. and S. mean? H.R. means a House Resolution and S. means a Senate Bill. Those are the numbers for the pieces of legislation. I'm going to provide you with all this information when we get into our IPPF training webinar and we're going to talk a lot more in depth about this legislation during those webinars. I'll let you know when those webinars are going to be. The first step of course is registering for Rare Disease Week. Then reach out to me to let me know that you registered and then we'll get you set up for these training webinars so you know what we're talking about. What does the BENEFIT Act do? It ensures the patient's experience, how your daily life is affected by this disease, the patient focused drug development data that's collected, including information from clinical trials and product sponsors or any third parties or even patient advocacy organizations like the IPPF or even academic institutions, all of this data and information is considered as part of the Benefit Risk Assessment by the FDA. So the FDA has to decide, is this particular therapy, are we going to approve it, or are we not going to approve it? Right now, they're not really taking the patient experience into account when they're deciding this information. They're looking at statistics and data and numbers, and they're not really taking into account the patient perspective. So this piece of legislation will require them to do that. It's extremely important that they understand how this disease affects you. Can you brush your teeth? Can you take a shower? Can you eat normal food? When you wake up in the morning, is there blood all over your sheets? Or do you have difficulty wearing clothes? All of these things that affect all of us, the FDA needs to take those things into account and the BENEFIT Act will help us do that.

Marc: The second piece of legislation is around restrictive formularies and drug compendia, one of the topics priorities that I mentioned earlier. This piece of legislation is called the Access to Rare Indications Act, H.R. 6160. Currently, it's only introduced in the House of Representatives, but the IPPF is seeking sponsorship in the Senate right

now but it has only been introduced in the house. So if you're having meetings with your senators let them know, this has only been introduced in the House but we're looking for Senate sponsorship. What this piece of legislation does is it defines what is considered to be medically acceptable to include off-label therapies. This is really important to the IPPF community because almost all of the therapies that are used for our diseases are considered off-label. So what that means is, that they're currently not FDA approved. What happens is, you go to your doctor, the doctor says I want to give you this medication, and then they get it pre-authorized from the insurance company but then when it comes time to get it approved through the insurance company, they deny you. They say you can't use this medication because it's not FDA approved and it's not on the drug compendium. In other words, it's not listed as a drug that's being used specifically for your disease because it's off-label, it's been used for something else. An example might be Cellcept or Mycophenolate, this is a kidney rejection medication. So it's used for kidney transplant patients. That means that it's not FDA approved for pemphigus or pemphigoid. Yet, it's being used by all of our doctors for our diseases. So the insurance company has the ability and the power to say, you can't have that medication because it's not for your disease. So what this legislation will do, it will change that process with the insurance company so that if there is enough evidence, whether it be through medical publications, or expert opinions, showing that this medication is being used for the standard of care, that they have to approve it. So, this is huge for our community. A huge opportunity for our community to get the access to therapies that they need.

Marc: Then, the last Ask, we always have three, our last Ask is around Step Therapy. Many of you already know what this is and, again, this is what we call the fail first approach by the insurance company. Your doctor says, we want to give you this therapy and then the insurance company says, no, sorry, you have to try this other medication first and then fail that medication before we'll allow you to try the medication that your doctor originally prescribed and knows works better. This is called Step Therapy. So, the Safe Step Act, which is H.R. 2163, and S.464 requires exemptions to Step Therapy protocols so that insurance plans have to accept them. Then if they do deny you that, there's a clear appeal process allowing patients to get the drugs that they need based on that exemption process. So, those are the three pieces of legislation that we're gonna be focusing on this year, and again, we're going to talk more specifically about them in the IPPF training webinars. But I just wanted to quickly introduce those to you so everybody knew what those bills were. Next slide.

Marc: Katelyn had mentioned, here's the information to register for Rare Disease Week. Please make sure you jot it down. A couple of things to remember as you're

registering. Number one, make sure you give RDLA permission to share your contact information with the IPPF. So, if they reach out to you and say, is it okay if somebody from the IPPF contacts you? Please let them know, they can do that. Then, when you are registering, you have the ability to put down who you are and what and how you're affiliated with the IPPF. Make sure you include that in your registration when you're signing up. Then after you register, attend one of the IPPF training sessions that I mentioned. We have two sessions setup. One is Tuesday, February 1st at 4:00pm PST, or 7:00pm EST. We have a second session on Thursday, February 3rd at 9:00am PST or 12:00 pm EST. You're free to attend one or both of those sessions, and then as Mindy mentioned, if you need individual help just reach out to me and I'll set something up with you individually. What's important is, I want you to feel comfortable sharing your story and how your experience relates to one of those three pieces of legislation that we just talked about. That's what's really important. How did not having access affect you? Did you have to wait, did your disease get worse? Was there a delay in treatment or delay in diagnosis? Or did you have to fight with your insurance company just to get the access that you needed to the drugs that you needed? I'm available and willing to meet with you individually, anytime you just reach out to me.

Amethyst: Awesome. Thanks, Marc. I think that was your last slide. I'll leave that slide up actually, so everyone can jot down the registration link if you haven't yet. Go ahead and register today. Like Marc said, please contact him afterwards and let them know that you've registered that we can get you signed up for one of the IPPF training sessions. With that, I think we have a few additional questions, so if Katelyn and Mindy, you're still on, we'd love to ask you guys a few more questions about Rare Disease Week. Katelyn I know you had discussed some of the training, and Marc also discussed the specific IPPF training. Are there any required trainings that a new advocate or just a returning advocate must attend?

Katelyn: Yeah so none of the webinars or trainings are required, they're completely optional. Same for the office hours, it's just if you feel like you want to come and you feel like you need it, you're welcome. If not, that's okay too.

Amethyst: Like you said, I know not all of them are not required, but do you recommend that they go to every training or event, if possible?

Katelyn: I do. I recommend, if you haven't attended before, definitely coming to the general training webinar and the new advocate office hours. If you have attended before, I still recommend coming because we're going to go over the legislative Asks

and so we may cover some new material that you haven't heard before. Then the Share Your Story webinar is totally new this year, so I recommend that for everyone even if you've attended Fast Forward for Rare. It's going to be a little bit different than that. So I'd definitely recommend it.

Marc: One thing I would mention is that RDLA has made this really easy for us and we really appreciate it. It's super flexible, as Katelyn mentioned, everything is recorded, so if you're busy working or you have another commitment, it's super easy to be able to go back and pop in and out of these things. Then, as far as the meetings are concerned, they're pretty flexible too. If you get a schedule and they're gonna send these schedules out in advance, and you're unable to attend because you have a prior commitment, you just reach out to RDLA or reach out to the leader of your group and you just let them know that you have a prior commitment. These meetings are super flexible and it's really about just showing up and showing support and lending your voice to the Asks and the legislation.

Mindy: As a patient advocate, I recommend all these workshops and because they are recorded, if it seems overwhelming, then watch it at your own time. Watch it twice if that helps you and it helped me. So I watched some of those a couple times and I learned more information. Thank you.

Amethyst: Did you have something to add Katelyn?

Katelyn: I was just going to add, to kind of piggy back off of what Marc said, if you're unable to attend, definitely email me and let me know. If you're the only person in the meeting, sometimes that happens, if you're unable to attend and you're the only person, then we can try to get it rescheduled. If it's a larger meeting and you're not the only person, then we will remove you from the meeting but unfortunately we won't be able to reschedule it. So I just wanted to add that in.

Amethyst: Great, thank you. And Marc had mentioned, Katelyn that the attendees will get a schedule. How early do they get a schedule or when does a team leader reach out to them so they can come up with a game plan for their meeting?

Katelyn: Yeah, so this should all happen about two weeks out. We are starting to send over our list of registrants to the third party scheduling group that we use. We're getting things set in gear and then they should all be ready to come out to you guys about two weeks before meetings. They have their own platform that they use, but it's fantastic. It will show you your meetings. There'll also be pages about each one of your members of Congress that you can click on and learn more about them. It will give you the emails for the people in your group. So, if you have a group of maybe 3 or 4 people and you don't have a team coordinator, you'll still have everyone's mail. And we still encourage you all to communicate with each other but if you have a team coordinator, they'll reach out to you. They get their schedules a little before everyone else, just to make sure they're able to attend before they reach out to everyone else. They may reach out to you a little bit before you get your schedule just to go ahead and get the ball rolling. So probably 2 or 3 weeks out, things will start to come to you and you can start planning.

Marc: To expand on what Katelyn is saying it just really depends. The number of people in your group will depend on what state you're from. One of the exciting things that I always enjoy about rare disease week is I get to meet other advocates from other disease communities and I get to learn about other rare diseases. That's really kind of one of the cool things about doing Rare Disease Week. You really feel like you're not alone in the process and you get to meet all these other people. Katelyn mentioned that you'll their emails, people in your groups emails. I recommend reaching out to them and introducing yourself and getting to know those people. It's an opportunity to just learn more and feel more comfortable. Then if you have a group leader, as Katelyn mentioned, they'll reach out to you in advance. There will be plenty of time to get together and talk about how you're going to do your meeting, who's going to say what, that sort of thing.

Amethyst: Okay thank you. I know we have a few minutes left, and I don't want to put either of you on the spot but I'm going to. Marc or Mindy after hearing the IPPF's legislative priorities would one of you give an example of how you would tie one of those pieces of legislation into your personal story with pemphigus and pemphigoid?

Mindy: I could share it really quickly. As far as the legislation for the BENEFIT Act, I am sorry it was the legislation that is currently only in the house. I just got a call yesterday from the rheumatologist who is going to give me my first infusion. And last April, I did the blood tests to make sure I could get this transfusion. So this is how long it took and I'll find out the details of why it took so long and I'm guessing it's an insurance issue.

And even though it is an FDA approved treatment, it's very expensive. Whether this involves Step Therapy, because I have been on other protocols or this really involves getting the right treatment sooner to patients. And that's why we need this legislation for people in my situation or for people who have a disease worse in a rare disease state than I am in, that need treatments so they don't get hospitalized. It doesn't happen fast enough.

Marc: Yeah, that's great Mindy. That's a great example of explaining how something like this really affects you. In a meeting with my legislators, I'm going to say who, where I live then I am going briefly so I was diagnosed with a rare, autoimmune blistering skin disease and it affects my entire life. Then, if I'm talking about something like, for example, Access to the Rare Indications Act, I would say, unfortunately, most of the drugs as I mentioned, are off-label for our disease. We do have Rituxian which is actually the only FDA approved therapy for pemphigus but there's other off-label drugs that are being used for people like me. These off-label drugs continue to be denied by insurance companies because they're not on that drug compendia. So, it's really nearly impossible to get the drugs that I need and even if I go through the entire appeal process, in the end I still get denied anyway because the drug is not on the compendia. I would say that it's disheartening, because the prescribed use of the drugs for what is considered that standard of care for my disease are not being allowed by the insurance company, even though there's lots of evidence showing that these treatments are acceptable and being used. These are life-changing therapies and people like me are experiencing delays and while the delay is happening, my symptoms get worse. It causes a lot of unbearable physical and mental pain, and it's a huge burden and really makes my life miserable. So that's why I'm asking you to co-sponsor the Access to Rare Indications Act because it will allow patients like me to get access to the off-label therapies that they need. That's when I would say. So at the end, don't forget the Ask. That's important, you want to make sure that you put that Ask in there.

Amethyst: Great, thank you. Well, it's great to hear both of your short stories and how really easy it is to tie in these pieces of legislation with the many trials, tribulations, hard access that people are having in our community and how they can share their story and make a difference. That was a really quick hour so thank you all for joining us. Before we go, I do have a few quick announcements to make. Like Marc said, please register for Rare Disease Week. If you haven't yet, go online and register with that link that both Katelyn and Marc shared. Registration closes February 11th, so, please make sure that you register before then. Then contact Marc to let them know that you've registered and then when RDLA reaches out to you, let them know that you're okay

with them sharing your information with us. I'd also like to give a huge thank you for everyone joining us on the call and to our sponsors, Genentech, argenx and Cabaletta Bio for making today's call possible. Our next patient education webinar will be on January 31st with Dr. Aimee Payne, Dr. Neil Korman, and Dr. Nasser Said-Al-Naief. Our experts will answer your dermatologic and dental questions. We hope that you will join us. The IPPF makes these resources available to patients, caregivers, medical professionals and others through the generous support of people like you. If you would like to support this work, please text IPPF to 24365 and follow the prompts to make a donation. Again that's text IPPF to 24365. Thank you for your support! The IPPF has a number of upcoming virtual support groups across the country. If you are interested in attending a meeting, please check the IPPF's Event Page to register for a meeting. Also, we are always looking to expand our support network. If you are interested in starting a support group in your region please contact me at amethyst@pemphigus.org. It's easier than it sounds to start a support group and you can help connect others in your area with other patients. If you are interested in continuing to help support the IPPF you can become a healing hero. Healing Heroes fund the future of the IPPF community by making sustaining, monthly gifts to support our mission of improving the quality of life for all those affected by pemphigus and pemphigoid. No amount is too small, even a $5 or $10 monthly donation goes a long way and continues to allow us to provide for the greater good of our community. If you have not registered for the IPPF's natural history study we encourage you to do so. The IPPF Natural History study is a patient registry sponsored by the National Organization for Rare Disorders (NORD) and the US Food and Drug Administration (FDA). You can register today at www.pemphigus.iamrare.org. This online data system collects, stores, and retrieves patient data for analysis in research studies. The more data we can collect, the better the information we can give to researchers, the sooner they can find better treatments, earlier diagnosis, and one day – A CURE! This call recording will be sent out with the survey following this call. Thank you all for joining us and we hope to see you advocating with us this year at Rare Disease Week.