Transcription of DTRF Patient Meeting Webinar #2

Please remember that desmoid tumors are very complex and the below information is not intended as medical advice for any individual problem, or as a diagnosis, treatment plan, or recommendation for a particular course of action, and should not be used as a substitute for professional medical advice and services. Please do not delay in seeking professional medical advice regarding your individual circumstances.

Jeanne Whiting: Hello, everyone.

We'll just wait, just a few more seconds for people to log in.

Welcome to the second session of the Annual Desmoid Tumor Research Foundation Patient Meeting.

This meeting is for patients, caregivers and medical professionals and we appreciate you all attending. We actually have more than 275 people registered

to attend the meeting from 21 countries so it's a great worldwide platform that we now have with the virtual meetings. And it's always our favorite meeting as

DTRF because we're working all year and this is the time when we especially get to connect with patients.

And this meeting is especially for you. I'll introduce myself, I'm Jeanne Whiting, resident and co-founder of the Desmoid Tumor Research Foundation. Behind the scenes, we also have Marlene Portnoy who is executive director and co-founder. Marlene and I met

16 years ago we founded the Foundation. Her husband had a desmoid tumor and I was a desmoid tumor patient at the time. We also have behind the scenes Lynne

Hernandez who is running all of our technology and Dr Maneesh Kumar who was recently hired as our new research director at the Foundation.

We thank our sponsors for our patient meetings - SpringWorks Therapeutics and Ayala Pharmaceuticals, who are both in attendance as well.

And we thank the presenters who are giving up time on a precious Saturday to be here with us to reach the patients when we're most available.

Just a couple of things - we invite you all to keep in touch with the Foundation via Facebook.

We're on as the Desmoid Tumor Research Foundation and on instagram and Twitter Twitter, we are @dtrfoundation on instagram and Twitter.

And please sign up for your for our newsletters. We have a physicians newsletter and a patient and caregiver newsletter. So those are on our website at

dtrf.org. So let me explain again how the meeting will work, they are true two streams open. You'll see the button for chat.

That will be open, you can chat with one another and comment, as you like, but the chat will not be monitored. We will only be monitoring the Q&A stream.

Q&A if you have a question for our presenters so you can ask your question at any time in writing.

This is webinar format format, so we won't have any verbal comments from the audience, but we make it available for you to and to write your questions in writing, we asked you to keep it to the subject at hand.

The speaker that's presenting at the time, if possible, and then Dr Kumar is the one who's going to be monitoring the Q&A

and passing the questions out to the presenters. If your question isn't addressed during the limited time that we have

We will be submitting the unanswered questions to the presenters and they will answer in writing and we'll send you an email with the answers, so we hope we can get to all of your questions.

So for webinar number two, this is our agenda. We'll start out with a discussion about learning about the clinical trial experience. Dr Rashmi Chugh of the University of Michigan will first present live.

And then we'll have comments and experiences shared by a clinical trial participant, Jon Fields. Dr Chugh, turning over to you.

You need to unmute.

Rashmi Chugh: Yes, I'm here.

Thank you for that

kind introduction, Jeanne, and I wanted to just thank the DTRF team just for being such great advocates for your patients, as well as us physicians and physician investigators and

You know she mentioned we're here to talk about today the clinical trial experience. I'm a physician over at University of Michigan and I specialize in connective tissue

tumors including desmoid tumors as well as clinical trials. So kind of the first thing what I like to talk about is just

What is a clinical trial? So there's lots of different definitions that we have. This is one specifically that comes from the National Institutes of Health.

And a clinical trial is essentially a research study in which one or more humans or patients are assigned to some type of treatment or intervention.

You know that could include something like a sugar pill or a placebo and and we really are going to look at how those interventions play out in terms of the effects on your own health and outcomes.

So that's what a clinical trial is. Now clinical trials in desmoid tumors are notoriously hard to conduct. You know you all know that this is a pretty rare tumor so sometimes it's hard to get enough patients.

And every patient is a little bit unique whether your, you know, tumor's in the neck or the arm or it causes pain or blocks your bowel,

It's also sometimes difficult to find support for studies, you know studies are expensive and and in order to conduct them, we need the support of either pharmaceutical companies willing to allow

Their drug to be used and to give funding for that or sometimes we can look independently for grants or funding from national institutions or or philanthropy.

So clinical trials in desmoid tumors have been hard to conduct for years and for years we really had to rely on what we call retrospective studies or kind of looking back at how desmoid tumor patients have done in the past to try to understand you know how to best treat patients.

These types of studies can be helpful and can teach us something you know, but unfortunately they can be biased and then they can have all different types of outcomes that are different to interpret.

So just to kind of help you understand what this means, so you know we could you know, decide to look back at all our patients that were treated with anti inflammatories.

And if you look back at studies that have been published, you can see that you know anti inflammatories can help patients anywhere from 25 to 50 some percent of the time.

Anti hormone treatment can help patients anywhere from 12% of the time to 60%. Chemotherapy 12 to 80%. How do we make sense of these numbers, when we look back in time?

So really kind of the information that we get from retrospective studies often you know here, you know, create a lot more questions.

There, there have been some good studies some some good information, you know, one example of which of a really important retrospective study that was

performed that gave us a lot of information about how to treat desmoid patients is some of the wait and see approaches retrospective studies, where

You know groups looked how patients were treated in the past. This particular study was performed in Europe and they looked at 142 patients with desmoid tumors

That were treated without surgery so for years, as you guys know, we we started off with removing the tumors and then learned, maybe that's not the best the best idea.

And we look back and saw, well you know of these patients that were treated with a non-surgical approach you know, over half of them were just monitored, some of them received medications.

After five years, half of them that were just observed didn't have growth of the tumor.

About 60% that were treated with medications didn't have growth of the tumor, and so this type of study gave us confidence to kind of not do surgery.

And that made us, you know kind of even spend more time looking at what medications can be helpful and and not focusing just on on surgeries.

So those are kind of retrospective studies. Now let's talk

What we've learned from clinical trials and kind of how that has been a useful part of learning about patient care in desmoid tumors.

So this is a study most many of you might have heard about this is an example of a clinical trial that I wanted to to give as we talk about

what's involved and, and this was a study that was performed it's called a phase 3 randomized study where 87 patients were randomly assigned either to receive this pill

That is kind of a protein blocker and again I'm going to try to simplify this as best as possible to kind of make the point that

What we learned from this study is when we gave 49 patients this this study medication and 35 patients placebo,

We were able to really objectively say that the tumor shrank about a third of the time when we receive in patients that received that protein blocker and there is no growth in two years, in about 80%.

And when you compare that you saw that these patients that were treated with this did better than if they received a sugar pill, but we also learned that objectively about 20% of the time,

The tumor shrank even on a sugar pill. So so this so studies like this provide invaluable information to us as physicians to treat you to know you know, to let you know what to expect and to also you know, give us even more confidence and sometimes you know no treatment is okay.

Another study that was very important in kind of

You know, teaching us, as is one where a similar similarly was looking at two different types of treatment, this is called the DESMOPAZ study was conducted in Europe and that looked at at 72 patients that had progressive desmoid tumor.

48 received this protein blocker medication called pazopanib and 20 received kind of a more standard IV chemo therapy that we use for years.

And that, even though this study wasn't designed as a direct comparison, it gave us a better idea of how often we saw tumor shrinkage with one drug versus another, and this is a lot more information than we can gather from retrospective studies.

So now, you know what's involved in these studies? Well well, first of all

We are so lucky that now there's more clinical trials to choose from than ever. When I first started doing this, it was pretty rare to have a clinical trial in desmoid tumors.

Now all of the clinical trials has to be listed on a website called www.clinicaltrials.gov.

And there's 13 studies kind of internationally that are recruiting and there's 49 that are listed as as even as in some stage of development.

Studies can be very specific just include desmoid tumor patients and sometimes they're they're more just inclusive to all different types of tumors and cancers and desmoids happen to be part of them.

Studies can study different things I've been I'm a medical oncologist so I've been talking about treatments or interventions or medications that we can use but there's also studies of prevention or natural history or imaging and scans.

So what's involved in these studies? So one of my tasks was to kind of give you guys a little bit of from a physician perspective what's involved in a study.

And I know this is a super busy slide but I will tell you it's a pretty complex process.

So the first part of of a study is really just finding either an idea or finding support, having you know, a sponsor a drug company interested in in conducting a study or some type of funding in a grant to actually support a study.

Once that gets through that process there's kind of this really large document called a study protocol that's generated.

That can be anywhere from 50 pages to 300 pages that outlines the details of what the study would do - you know what the treatment entails what the follow up, all of the procedures.

And then every institution is a little bit different. In our institution, you know we have to get you know first we have to make sure that the supporter or sponsor you know approves the

institution to participate, and then the research team has to prove that it's a good fit for our team to do this study here, and once we get through that process

Then there's a series of study reviews. There's a official protocol review committee, there's a pharmacy committee, there's an imaging committee and then of course we get lawyers involved and there's a contracting and finance so there's a number of reviews that have to take place.

Eventually, a document called an informed consent document so that we can tell our patients what's involved

You know, is presented, and we have to get ethics approval to make sure that the study conduct and everything meets the requirements for safety and monitoring that our institution puts forth.

And then we have to kind of get through, well the logistics, how do we conduct the study and then eventually we start this study.

So how long does this all take? Well you know even just this first part, finding support can take you know months to really it can take years to kind of get a good study.

Then we it can take a few months to get through the initial approvals

and other three four months to get through some of the study reviews and another few months to get through the ethics approval and really

You know past the part of finding support, it can really take 6 to 12 months to get a study up and going.

And this is all while you know we know we have patients that we're treating and that you know we're waiting to kind of get these studies engaged, so we can give an opportunity to participate.

Now that wasn't the hard part, now we have to kind of after the study is open, you got to find the right patient.

That patient has to meet certain criteria that we call the eligibility criteria, to ensure that we have kind of a population that we can learn from.

A lot of those criteria can be just that they have a desmoid tumor or that the tumor's growing or a certain size or causing symptoms.

Then we have to find make sure patients interested in participating, we give an informed consent document, so that they can understand what's involved in the study.

Once we find the right patient, there's kind of this screening period where we see if a patient needs all of those qualifications for certain and does some certain tests.

Eventually, we get to start treatment and do follow up but kind of behind the scenes while all this is going on, you know there's a lot of work that's being done by the study investigator, and the research nurse and coordinating all of this.

There's a data manager behind the scenes that are communicating with the sponsor or supporter of the studies, as well as the ethics board trying to communicate what side effects are seeing what are the outcomes we're seeing.

There's an investigational drug pharmacy that's preparing the treatments and then there's a research unit office that's coordinating all of these required procedures. It's a pretty complex process clinical trials in general and, and in desmoid tumors is definitely you know, a unique process.

All in all, told, studies are time and resource-intensive so a lot of you know, money goes into it. Average cost per center

is about 10 to $30,000 per patient we treat on the study. Usually we can get support from the sponsor for that, sometimes we kind of couple it with some philanthropy support or kind of our own resources in order to bring these important studies to patients.

So kind of my last piece, I wanted to talk about which is you know, one of the most important pieces for you as patients.

You know why, would you participate, this seems pretty involved or why would you not participate? So there's always kind of pros and cons and you know bottom line is it's a very individual decision, what makes sense for you.

You know, one reason to participate is is really, this is a chance to get access to a new treatment, sometimes that's, the only way we can get access to a new treatment if it's not approved, you know, or if insurance doesn't cover it.

The downside is, you may not always know what treatment you're getting you know, sometimes there are there are these placebo studies that are not active treatment.

A lot of times we can't tell you what the effects might be we can't tell you how often it might work. We can't tell you what all the side effects are so there's a lot of unknowns in there.

Another reason to participate is that you do follow a very regimented treatment, a very regimented protocol.

You know everyone gets kind of a standard dosing, standard evaluations, you get a little extra attention by a research nurse and coordinator so sometimes.

That regimented treatment can be a really nice advantage, especially in a disease, where where maybe there aren't as regimented things to follow.

The downside is that you get a very regimented treatment, so you also you, you know participating in this study is a big time commitment.

There are times, where you have to be you know kind of at your center getting evaluations, getting seen

When it's not convenient to you, you know when you want to go to the football game, when you have to work and but the rules are that you have to be there at a certain time.

You know that comes at an expense, a travel expense, sometimes. Sometimes those expenses are covered, sometimes as an expense to not being able to work at a certain time, so those are all things that you have to kind of consider as an individual.

Another amazing thing about participating in this study is you really do make an incredible contribution to a greater understanding of the disease and whether a drug can work or not, and whether it works in you

You know you're still contributing and whether you have side effects or not, those are all incredibly important things.

But kind of the downside is that you know we've got to follow rules, you know there's a lot of rules in the protocol that we have to follow about you know when we can start and stop a study drug.

A lot of rules about you know dosing and things and those rules don't always make sense for the individual.

You know, one example of this is, you know some studies that have placebo, say, well, we can switch you over to the real drug or let you know what you're taking if your tumor grows a certain amount.

And sometimes that tumor doesn't grow that exact amount, but you still think it's it's not helping a patient, and we have to wait and see to we kind of follow that rule in terms of

When we can take you off study, if your tumor has grown by 19%, we can't. If it's 20%, we can, so you know it sometimes it doesn't seem right or fair to have to follow these rules, exactly, and it can be frustrating.

But overall clinical trials are an incredible way to learn more and contribute to this disease and I've talked a lot about kind of my experiences and, from my perspective.

Now I'm going to hand it over to Jon Fields, who is a who were patient, he's also a clinical trial participant cat lover and much, much more, and I appreciate the time he's going to spend talking about it from his perspective.

Jon Fields: Just had to throw in cat lover.

Rashmi Chugh: I had to.

Jon Fields: Alright, so um.

Thank you for that Dr Chugh, appreciate it. I kind of want to just go over my what my experience has been from start to finish, and I want to preface it by saying that I know that

everyone's is very different and I've had a I've had a good experience, I know that that's not necessarily the case for everybody, so, basically, I had a

I had a surgery back in beginning of 2019 and then a recurrence - so I have a right gluteal desmoid and and then recurrence

middle of the year so about four months after surgery they removed all of it came back you know same size or bigger

And I was looking into my options. This is before I really found a specialist I was at my local hospital here in San Diego Sharp and there's no doesn't specialist there anything so I didn't really know what I was doing

and found the desmoid Facebook group, first of all, which was a huge saver for me in terms of

You know what direction, I wanted to take things and when I went and saw my doctor here they're recommending you know certain chemotherapy drugs and that sort of thing and

I knew that I wanted to check out all my options first, so I think it's kind of hard to remember exactly how how it all started, but in the Facebook group I was hearing about a couple clinical trials.

So I said, you know what I'm going to start doing some research on that so in combination with what I was hearing some people say in the Facebook group and then doing my own just simple literally googling

Clinical trials for desmoid tumors I found I think Dr Chugh referenced it clinicaltrials.gov and that's where I found

a trial that kind of piqued my interest and I thought I might be a good candidate for so the first thing that I did was I checked

To see if there was any locations in San Diego where I live, and there were not so I was kind of seeing what was closest to me, so I could see if I might be able to

Perhaps commute in the event, I was

A candidate and I found that LA had a couple locations, so I pretty much started out by finding figuring out on the clinicaltrials.gov it shows you where you know where the locations are and I reached out to the trial coordinators at a couple different places and I

ended up driving up to LA, meeting at a couple different

one was at UCLA and then some other center I forget what it was

and finding what was the best - you know, and at that time I didn't know if I was going to qualify - but I was looking for what was the most comfortable and best fit for me because I really didn't know what to do, I was in a lot of pain and all that.

So fast forward a little bit I figured out where I wanted to be, which doctor kind of made me feel most comfortable and

Really listened and I was, I was fortunate enough to have something somewhat close by, I think a lot of people that are trying to get into clinical trials, you know, when you look at

locations and if you don't have a location anywhere near you, then in some instances, I'm sure people get driven away from it, or there are cases where once you're in the trial, travel can be paid for.

And kind of jumping around here I've been lucky enough to have the trial that I'm in has a third party that

You know, books my hotel when I'm up there, and reimburses for gas and food and that sort of thing I understand that that's not

Across all trials, but so that was very helpful and i've talked to a couple people who were even even having to take flights to different states, and that was getting covered so that's always

When you're meeting with the doctor upfront to see if you're a candidate, I think that's a definitely a reasonable

question to be asking the doctor and the trial coordinator to try to figure out, okay, what are the logistics here sure I made it to

here for the meeting to see if i'm a candidate, but, at the very beginning of the trial that I'm in, it was weekly trips there for a month or a month and a half.

So that can be very difficult, if you don't either live, where the trial is or can just drive a couple hours, like, I was able to, so I think that's a very

important point is just finding that a the trial that's the location that's going to make you feel the most comfortable and has what you need and then figure out the logistics of getting there.

Aside from that, it was my first appointment, when I went up there, before I went up, I had to I sent all my previous MRIs,

biopsy reports, surgical reports all that kind of thing, so that they kind of save my time by looking over at first and then telling me, yes, you should come see us it looks like this could work. So upon going there kind of evaluated everything and turned out, I was a candidate

and basically from there, there was some I'm trying to remember, there was some waiting period, once you actually signed up for the trial.

There was a baseline MRI done so that you had something to go off of, and you know I should go back and say that the the criteria is fairly strict for getting into it, I think the one i'm in was like that showed 20%

increase over a 12 month period, or something. I think that was pretty easy for me, because it was just a recurrence from surgery

So, and on the website, the clinicaltrials.gov it typically lists, like all of the criteria so that's all stuff kind of preliminary stuff that you can look at before you dive too far in.

But, as for the trial experience in general, once I actually started the taking the medication, one of the one of the biggest risk I'd say, is the fact that you have

50% in a double blind trial in Phase three double blind, we have at least that I'm in, you have 50% chance of getting a placebo.

So that was a big concern for me, I said, should I just go with the SURE thing that you know i'm going to be on a drug, by taking one of the chemos that my doctor down here was discussing with me or do I want to roll the dice a little bit and see if I get the drug or the placebo.

Something that I'd say to somebody was considering a trial would be - if it is a double blind trial,

The

One of the previous presenters one of the previous doctors was mentioning the the method that they have of measuring the tumors and

It can be very different from what you're just looking at on an MRI or what you know what your MRI report says. I've seen i've seen, unfortunately, a lot of people that are

get very frustrated because the measurements that the studies going off of is very different than

You can think wow I had 20 they tell you if you get 20% growth, then you can be unblinded if you're getting the placebo, and given the drug and so I have had, I have

communicated with people who've had a really difficult time, because what they're seeing is 20% growth, but the way the studies measuring it is in a completely different method and a lot more strict.

So that's something to keep in mind it's it's my my experience was that people that are in this trial have not had as easy of a time switching over

From having growth on an MRI as they might have thought, to begin with. I was kind of told fairly nonchalantly by my trial coordinator that, "Oh, you have something you have some increase in size, you can be unblinded and move over."

So lucky, for me, I do believe I'm on, I do believe I'm on the drug due to side effects that I've had and some benefits that I've had from it, but

That will be once we get unblinded which is make might be any month now, I was supposed to be like about a two years worth of

Double blind so then we'll know for sure, at that point. I'm looking at my notes here at a couple other things.

Jeanne Whiting: Thank you we're at the end of our time here but we'd like to have.

Jon Fields: I rambled, I'm sorry.

Jeanne Whiting: No, no, a great presentation we wanted to add five minutes for questions. Maneesh

Was monitoring the Q&A. Do you want to pass those over?

Maneesh Kumar (DTRF): Sure, Jon I think the first question is for you. It sounds like at the beginning, you did a lot of research on your own through Facebook and Google. Did you ever talk to your physician about clinical trials or did a physician bring up clinical trials to you?

Jon Fields: So my physician here in San Diego

I was seeing somebody who wasn't it wasn't even a sarcoma doctor, it was they just it was a very general oncologist and he they had

There was nobody at my hospital here that I that I was working with that had any idea about any of it, so it was all on me and honestly, had I even found a Facebook group prior to having surgery I probably would have never had surgery.

So yeah that's I it was it was the Facebook group and then my own research. "Desmoid tumor clinical trials" and then I found clinicaltrials.gov.

Maneesh Kumar (DTRF): Great, Rashmi, do want to comment on that at all as well? I mean I think it's really important for patients to do their research and do their work, but from a physician's point of view?

Rashmi Chugh: I agree, and you know, one of the points I like to make is you know there's a ton of amazing oncologists out in the community and in academic centers even

But you know, this is a pretty rare tumor and to really get you know people that are educated about it, you do need to kind of find a center

Where they specialize in in it, and even if they don't have a study open, they're more likely to kind of refer you there, educate you about what are the options so

So that's why we're lucky to have DTRF because that's something that comes up when you Google, and so you can get kind of you know, refer to a place that's you know comfortable with with educating you on these things. Yeah.

Maneesh Kumar (DTRF): And Jon maybe I'll come back to you again for the next question. It sounds like the beginning of the trial was pretty time intensive with a lot of trips and visits. At any point did you consider stopping the trial?

Jon Fields: No, because I like I had said I was I knew pretty quickly and I shouldn't say I know I know like you know

Mostly I haven't been on blinded I knew pretty quickly, I was having benefits

So there was just no way I was going to stop, but I could totally see how if somebody's not feeling like they're getting anything out of it that they

might consider that. I just knew my other options, I just didn't like my other options, so I thought I'd tough it out, even if I was on the placebo.

But it is time-intensive. I work for myself, fortunately, so I don't I didn't have to do the whole like asking for time off, which can be very, very difficult and stressful for people

So, and then I just had like a two hour drive, so it wasn't too bad so those two things definitely worked in my favor.

Maneesh Kumar (DTRF): And then, Dr Chugh do you want to comment on a little bit about the patients' rights in a clinical trial? If they start a clinical trial, can they stop or do they have to finish it once they start?

Rashmi Chugh: Absolutely, and I think that you know utmost importance is the patient, so you know sure we're trying to get information and learn about a drug, but the priority is always with you.

So at anytime you can withdraw. At any you know we're always going to prioritize you if we think that the study, is no longer in your best interest, you know you can take you off the study and do something different.

Jeanne Whiting: But Dr Chugh if you do withdraw, we've had some patients have the issue of they've not been told whether they were or they were not on the drug. How does that work?

Rashmi Chugh: That is a very frustrating thing and that's happened to you know you know there's certain reasons why the sponsors won't kind of allow that to happen.

Because they they still need to try to you know you know get their drug approved and kind of complete this study, based on the obligations they they put forth in their protocol.

So you know you always have the right to withdraw, but there are certain things that you know you might not be able to get the data from the sponsor, you know as it pertains to you.

Jeanne Whiting: Which could impact their ability to join another trial or have another treatment, correct? Can you explain that?

Rashmi Chugh: Yeah so so some studies, you know so, so there are there are some examples of studies, where they will say you are excluded from treatment if you've already had this class of drugs

And if you participate in a study and you weren't ever told what class of you know whether you receive the drug or not

You might not be eligible for this study you know. And it's a real something real that happens, I have a patient that I'm pretty sure was on placebo

And we had to withdraw from treatment because we didn't think it was helping him and we couldn't wait for it to grow 20%.

And so now when we're looking at other studies, you know, unfortunately we're having trouble

You know enrolling on a study that excludes prior treatment because we can't say for sure he was receiving placebo or not. We're hoping we're trying to advocate so that we can get that knowledge, but you know there, there are some regulations things that we're limited on, unfortunately.

Jeanne Whiting: OK, Maneesh, I was wrong on time, we have actually five more minutes if you want to convey some more questions.

Maneesh Kumar (DTRF): Sure, so I think Ravin's been doing a good job of answering questions via text but maybe I'll post those to Dr Chugh as well.

There was a question about trials specific to your desmoid tumor location. Now as far as I know, there's not like a trial that specific for desmoid tumors in the breast, for example.

And I think they're pretty much open to desmoid tumors in any locations, but does the location of the decimal would affect your ability to participate in a trial.

Rashmi Chugh: No, no, it doesn't usually there's a lot of unique features about desmoid tumors in different locations that we've learned about mostly from retrospective studies, but most prospective studies

don't exclude by location, you know they they might exclude based on side effects, people are having from there from their desmoid tumor, for instance

If you have a tumor within the abdomen that makes you not able to tolerate pills well,

You know they they part of the criteria might be you should be able to tolerate pills, because the medication is a pill. So but it wouldn't be specific to the location, it's more criteria that would make sense, based on you know kind of the way the drug would work.

Maneesh Kumar (DTRF): And I think this is a question, specific to Jon but I'll turn it to him first and and maybe broaden it to Dr Chugh. Jon, did you have to have frequent MRI with gadolinium to monitor treatment in the trial?

Jon Fields: So of course I'm allergic to gadolinium. Classic. So the first time I ever had I had with gadolinium and I got a few hives, and so they told me to not take

Well, I have to pre-medicate like prednisone and Benadryl before it. My trial doctor said that he doesn't want me to have to pre-medicate so I've been doing all of my MRIs without gadolinium

and

Most people are, yes, every three, the answer's every three months we get an MRI and if you're not allergic to gadolinium, you should be getting gadolinium is what I understand.

Maneesh Kumar (DTRF): Dr Chugh, maybe broaden it a little bit just to the idea of imaging and clinical trials. Now you mentioned there's a lot of rules, but it sounds like there may be a little bit of flexibility of these with gadolinium and MRIs.

Um yeah you know there there certainly is some flexibility.

You know if you have it, as long as you can get consistent imaging sometimes it's CTs sometimes it's MRI.

Gadolinium, for those of you who aren't familiar, is kind of a contrast we use with MRI that helps us see the desmoid tumors a little bit more

Clearly, but it isn't required always and sometimes we can get a good view, without it, so there is some flexibility, but we still do need to get those you know images at certain time points.

You might ask, "well gosh every three month MRIs? I was getting them done every six months before you know. Is insurance going to approve it?"

Usually, we can get the sponsor to pay for studies that we don't think is standard for insurance to cover. So for our study you know the sponsor covers every other month scan every other scan

Because it's pretty customary to get scanned zone every six months for most patients or you know, but that may vary a little bit by center and by study.

Maneesh Kumar (DTRF): Great and then one last question, I think. So regarding these rules about the tumor growing enough

So you can

Transfer over to the other arm.

Growth of the tumor is not always what maybe the patient cares about maybe they're more concerned about pain or range of motion.

So there are opportunities to design trials that may be focused more on that versus growth? And maybe that's a forward-thinking question, maybe that's not an appropriate one for you, Rashmi, but if you had any thoughts on.

Rashmi Chugh: Oh that's a fantastic and a very insightful question. You know I think the you know what what matters to us when we take care of patients is kind of

What effect that desmoid tumor has on their life? Not necessarily if it's a bigger is it smaller, you know. Is it causing pain? Is it blocking your bowels? Is it about to block your bowels - like things like that.

But the problem is, those are harder to objectively measure than something like size.

And so, because of that a lot of you know, drugs can get approved by these objective measures by how often it shrinks the tumor, how long it delays progression of the tumor, and it's a much harder thing to kind of you know nuance these you know issues about symptoms and pain.

You know that's something we're definitely working on there's some you know patient related outcomes measures that

You know, those of you are participating on studies know there's extensive kind of questionnaires and things like that that you have to fill out, and so, hopefully, in the future we'll be able to do a better job at actually

You know, showing and caring about what matters to patients the most.

Maneesh Kumar (DTRF): Great

Just to summarize very quickly, I think it was a fantastic session and I think what I learned is that clinical trials can be complicated, it can be very challenging but there's a lot of benefit to it

As well. From Jon's experience, personal benefit from it, from Rashmi's talk, benefit just understanding more about the disease and how drugs can can help them.

But then, also the need to really ask a lot of questions and understand the clinical trial before you participate in it, but then also while you're participating and keep asking questions and if it's not for you, you always have the right to stop.

Jeanne Whiting: Okay, thank you very much, really an incredible discussion and again, you can post questions in the chat, but we will be moving on to a discussion about compassionate use.

Also, known as expanded use, we have to know that these are these are just different terms for the same thing "compassionate use" or "expanded access."

And we're going to hear a presentation by Dr Ravin Ratan from MD Anderson Cancer Center with comments from Amanda Hoffman, who is a beneficiary of expanded access. Dr Ratan.

Ravin Ratan: Thanks, very much for the introduction, Jeanne, and thank you to DTRF for hosting this wonderful event and for for having us here. This is really a privilege and I'm really excited to come talk to you folks today and answer your questions kind of keep my comments brief and

Trying to introduce the topic, but I think the discussion is the fun part and I really want to hear what

Amanda has to say as well.

So Just quickly disclosures I do, in the course of my work ,interact with some drug companies, including some that do things in the desmoid space. I don't think it's going to impact what I'm talking about here.

And I want to say very clearly they're not discussing any specific drug here. I'm trying discuss the process generally

about getting compassionate or expanded access to to medicines. Okay, this is not a history lesson, but I think that the

The context is helpful right, so why do we do it this way? Why is the research process that Dr Chugh sort of discussed with you folks previously so labor-intensive and the answer is that there have been

Historical incidents that have caused harm to patients right so 1960s, when thalidomide is widely used to treat nausea and pregnant women - that is sort of what women are told to use it for,

We find out it's sort of through the 50s it's used, in the 60s, we find out that it causes devastating birth defects and so, around this time Congress

Charges the FDA which existed, back then, but had a very different job - didn't approve drugs, the way that it does now to ensure that drugs that are marketed are both safe and effective.

And the process that's put into place has some very positive benefits in terms of making sure that we under have a better understanding, I should say of the drugs that we use to treat treat patients

But it delays the availability of drugs, you can't just create a therapy and give it to patients widely.

And the 1980s, a new challenge arises, which is the HIV epidemic and so you know if you many of us who were younger than will still remember

That patients in the 80s, who had HIV, it was a death sentence it's not that way anymore it's still very challenging disease

But as therapies which, some of which ended up being quite effective became available there was an outcry to make these therapies available

to patients and not wait seven to 10 years to get them approved by the FDA, and so the FDA at that point really flushes out this compassionate use procedures pathway to facilitate drugs for HIV patients. That then is used for other serious diseases like cancer,

metabolic diseases, ALS, neurological diseases. And the current regulations around expanded use also called compassionate access are put into place in 2009.

Okay, and something you may have folks may have heard about just in the last few years 2014 to 2018 is so-called Right to Try laws which are related but separate from this FDA pathway, which are past the state level and something like 40 or 41 states and then federally in 2018.

And I'll tell you that these basically limit the liability of people like insurance, the physician, the company is providing drug

and try to ease some of the regulatory burden, but as I sort of talked about earlier some of that regulatory

Burden exists to protect patients, and so I would tell you that the implementation of these Right to Try laws has been pretty limited.

So that's a history lesson. I think it's helpful context to sort of talk about why it's not necessarily so straightforward to get compassionate access to drugs.

But let's we'll talk a little bit about the actual pathway okay.

So expanded access is a Food and Drug Administration (FDA) pathway that allows patients access to experimental medicines okay.

It is a resource and time intensive process which is similar to the the clinical trial process that Dr Chugh just discussed

With some modifications. And it absolutely requires going through physicians, this is not a situation where a patient or a loved one

can take it upon themselves to approach a drug companies saying, I would like, for you to give me the drug. You have to go through a treating physician.

Okay, and these are the criteria right off the FDA website. It's for patients with immediately life-threatening or serious disease or condition to get access to the medical product. The patient should not have any comparable or satisfactory alternative.

The patient shouldn't be eligible for the clinical trial with the agent and that's really important right. So as as Jon and Dr Chugh just discussed, you know if you're on if you're running a study which is required potentially by the FDA to be 50% placebo and 50% drug

You know, we don't want to shunk patients to just getting compassionate access to that drug, because then that trial will never get done,

The drug will not be widely available to other patients. And so you don't want to cannibalize the clinical trial by making it widely available prior to that being available.

To say nothing of the fact that we don't know if that's safe and so that next criterion is that the potential benefit of the drug has to outweigh potential risks and that can be very squishy.

For some drugs it's that we have some early phase, maybe phase one or phase two clinical trial data that shows some signs of effectiveness

and gives us some sense of how safe the drug is. In other cases and very unusual situations and may be that there's just a scientific rationale for why trying, this makes sense and nothing is about nothing else is available to that patient.

that's less common the desmoid space, but I'm sure you can imagine, this being the case in certain neurological diseases.

And certainly was true for some agents during the COVID 19 pandemic early on, before we had a whole lot of data.

So there's three op sort of broad options that the FDA allows for and the most common one, that we do

In the demoid space and this changes, based on sort of where different drugs are in their approval processes. It's what's called a single patient

IND and that's a fancy way of saying that we basically open a single patient clinical trial, the FDA has to approve it, they review that they review the application, the company providing the drug reviews the application, we generate some of the paperwork and we'll talk about that.

As, as you can imagine that quickly becomes limiting if you're doing these for 20 or 30 or 40 patients in a practice.

And so there are pathways to do this on a larger scale so intermediate size patient population protocols will allow the FDA will allow a company or a center to treat

You know 10s maybe even hundreds of patients without having to go back each time to get separate approvals.

And then finally there's expanded access for widespread use, and this is typically for drugs that are that have a lot more information regarding their safety and efficacy, but are

are not yet FDA approved that this is a way that the company can make the drug widely available when there's there's a real need for it.

So on the patient side, I think that there's a couple things need to be aware of. So access to a experimental therapy is not available on demand.

And in fact there really isn't an obligation for the pharmaceutical company or the FDA or the center to make it available at all, and so you can't sort of just go in and say I want access to this drug can because very often that may not be possible.

It's important to recognize that as an experimental agent we don't have a full handle on the side effects and how likely it is to help you.

And when you go to a physician, especially if it's someone that you haven't seen before, and request compassionate access to a drug,

You know my obligation in that situation is to have a really thorough conversation with you about the other treatment options that are available

And to offer you the treatments that are not experimental as a first step, and indeed that's a condition that's often set that is set by the FDA and the sponsors that are providing the drugs.

On the company side,

You know the company's looking after the developmental program they want to get a drug, study it fully, get it approved by the FDA, and bring it to the market and there's a theoretical risk if you have a patient

Who is not as carefully selected as a clinical trial patient going on a medicine, having a bad experience, which could then potentially although not terribly likely to

Have have consequences in terms of that drug getting approved or certainly just getting a bad rap in the public.

And, as I mentioned earlier, easy access to experimental medicine may prevent a patient from enrolling clinical trials.

That being said, the opposite consideration is also true right? If there's a really promising therapy, making sure that there's a compassion there's a

Robust compassionate access pathway available for patients who don't qualify for your clinical trial

can be helpful in ensuring that physicians don't feel pressured to sort of put patients who really shouldn't be on a clinical trial, and there's different reasons for that that we can talk about,

On a trial, so there are good reasons as well, they actually help some aspects of your study, although enrollment can be impacted.

Smaller companies may just not have enough drug kicking around I mean it costs money to make drug, they are trying to get their trials done, that has to be their priority, so they may choose not to make a drug available, simply because they don't have enough available.

There's geographic considerations here and then most of this talk is sort of targeted at a US audience, but I recognize we have people coming in from many different countries.

And these expanded access programs are regulated different in different countries. Companies may especially smaller ones may not be able to operate in very many providing compassionate access to drugs.

And that being said, you know, having patients on treatment good experiences is great PR. It's a way to get buzz out about your drug.

And I think that certainly people who work in pharmaceutical companies also have an altruistic desire to help the community. A lot of people are in it to help the patients, and so I think that there's lots of reasons why they want to do this and some why they might not.

I will tell you, as a doctor who takes care of a lot of patients with desmoids and does a lot of compassionate access when it's appropriate,

It's a labor intensive process, so my job, and someone is going on compassionate access for a drug

is to file with the FDA, to file with the company that controls the drug, and to also file internally

With mechanisms that we have to make sure that what we are offering a patient is safe and those are called internal review boards.

Which are the same committees that oversee clinical trials, to make sure that patients are being cared for properly.

So that's similar to the process that Dr Chugh discussed earlier. I will tell you it is a little bit more concise we don't have to write an 80 page protocol on every patient.

But we do have to write a protocol, we do have to come up with an informed consent document that we go through with the patient.

And then there are reporting requirements. If patients have side effects, we have to notify the company potentially the FDA and potentially the internal review boards, so this does create an administrative burden.

And different from clinical trials, this is not paid for, so when we're running a clinical trial for a company or with a company,

A lot of those costs are covered by the company that sponsoring the clinical trial. That is typically not the case with compassionate access.

Though we're using the same people and sort of similar man hours, and so you know, having having 30 patients on compassionate access protocols can actually

You know gum up the works and potentially slow activation of our clinical trials that might get a drug approved.

So you know key messages that I'm trying to convey or that expanded access or compassionate use whatever you want to call it,

it's considered extraordinary, it's not a standard of care, it's not an obligation for a company to provide it, or for a physician to provide it. It's generally meant for patients who have exhausted other options.

The best way to get access to experimental treatment if you're eligible is to get on the clinical trial, although we fully recognize that there are

Significant reasons why you might not want to do that, and I would mention that if if some of those reasons are medical - things like you can't take pills, to use Dr Chugh's example,

Or you know your tumor it would be unsafe if it progressed by the amount necessary in order to cross over to the different treatment arm and trials, Jon alluded to,

Those might be reasons that you are not appropriate for clinical trial and may qualify for compassionate access if you've exhausted other treatments.

So in the right setting, it really can provide access to important treatments to patients in need. There are success stories out there and I'm

happy to talk or have Amanda talk about hers. And I'll stop there, and hand it over and be looking forward to answering your questions in a few minutes.

Jeanne Whiting: Amanda,

The time is yours.

Jeanne Whiting: Be sure to unmute unmute.

Amanda Hoffman: That's what happens when I wait too long, just sit there on mute. Hi everybody, thank you for having me.

That was such an educational presentation for me and I'm one of the people who's benefiting from this that was great to know the context and the history and everything, so thank you, Dr Ratan.

I'm also cat lover just you know so Jon doesn't feel alone here.

But I just want to start out by saying, as Jon said, you know, this is my personal experience, I know that this is not the same for everyone, you know a lot of things might be the same, but of course everyone's going to have a different journey.

And as Dr Ratan pointed out like depending on your specific case, this just might be very different so just want to say that off the top.

This term "compassionate use" just meant something so different to me before just a couple of years ago, I mean when I heard the word "compassionate use", I'm thinking people who are terminally ill.

And not someone like me who's you know just having a lot of pain and like

You know issues in my daily life with my disease, but I just didn't think that I even came close to that, so it was very enlightening to understand that this program is

For people like us too who have very few options available to in our for our treatment. So I've had this I've had my tumors for 15 years and

I had surgery way back in the day and had a near immediate recurrence and then have been dealing with those subsequent tumors ever since. But about two years ago, I had a new tumor pop up so that was definitely considered you know significant new growth

And I was having a lot of mobility issues and pain and it was it was very debilitating and started to deeply affect my life.

And so I started to think of what options were at my disposal, because I'd already tried, a number of chemotherapies that just did not work at all.

I tried doing nothing for a while. I'd tried all sorts of different things and I knew there were only few options left on the table.

And I had actually been in a clinical trial five years previously, and so I know firsthand what Jon's experiencing.

And I did very well on that trial with that therapy and did have to leave the trial due to some side effects but

I was always hoping that one day soon that drug would be available again and that I would maybe be able to take it again.

And so I heard about expanded access with that drug that I benefited from and thought, "am I even possibly a candidate for this?", and so I spoke with my care team

And this was in just December of last year, we started this process and

You know, I was evaluated and we just talked about everything I tried to have what was left on the table and they agreed that they thought this made sense to pursue for me in my position.

And I was getting to the point where I like couldn't walk and I was crawling up the stairs I mean I was I'm definitely having some severe issues so

I was grateful to hear that this was a possibility, you know no guarantee you when they start the process, there is no guarantee you just kind of wait and see what will happen.

And so i'm really glad that

Dr. Ratan's here to explain all the things that happened that were kind of in a black box, for me, because as a patient you don't get to know every single detail and, obviously, a lot is going on behind the scenes, but I was just told basically we're going to start this process,

it's going to take yeah probably one to two months, but you know between getting the FDA approval and the drug company approval and all of these things.

And so I just waited but I was you know hopeful and I found out, I think, probably in early February that I was that it was approved by the drug company, and then there were some more steps to take.

And so I actually switched doctors in this period, and I do, you know, have to wonder if that was kind of helpful for me, because the initial doctor I was seeing

was actually the principal investigators, like the manager of that trial for that therapy at my institution and so he was very familiar with this drug, very familiar with the drug company.

Not to say that someone who isn't the PI could certainly do this, but I just think that might have been a little bit in my in my favor.

And then I switched doctors, but this it was luckily a seamless process, they handed over the process to my new physician because

my previous physician was leaving and we just continued on and it didn't seem luckily my case didn't seem to affect the timeline. And so I think about

early March was when I finally got the a Okay, I had signed my consent.

Since I had been a in a clinical trial before I kind of knew some of the way things were going to go, you sit with this giant document, they go through every page, and tell you everything about it.

I had worked very closely with my physician and I don't know if this is a standard thing or not, but I've worked very closely with my physician to to write the protocol for my own little one person in clinical trial, which is how they kept describing it.

And that was amazing! I just thought that's the most involved in my care I've been in a long time, so we decided on how flexible we'd be with dosing, what kind of breaks, I could take etc and it it really

made me feel so good about what I was entering into and so that that was extremely comforting and the only hang up that I ran into, and I think this is really important, because I didn't expect it, is that my insurance company rejected

paying for anything that I needed that had to do with me being on this drug, initially. And my team was very shocked by that.

was very shocked by that, but then again, not so much because it's an insurance company and they're always a pain, but

My doctors submitted an appeal and they asked for it to be an urgent appeal, because I had already waited months for this to all come together.

They decided that they were not going to be urgent about it and they were going to take their sweet time and take up to four weeks to review it.

And so that was frustrating obviously. It's kind of like a running running running and then have to stop and kind of put everything on pause but luckily within I believe it was about three weeks, we got the okay.

And I was able to move forward, and so, and all that means is because the you know the drug companies paying for the drug, it's not like your insurance company has to pay for it, but they were

Having an issue with the idea of paying for all of the things that come with it so like Jon was saying in his clinical trial, you know that

they're extra MRIs or if there's extra blood work or whatever some of it might be paid for by the drug company, but a lot of it would be paid by insurance companies same thing with with this situation, and so

They just thought well she's going to go on this, you know experimental drug she's going to have issues we're going to have to pay for all the stuff and we don't want to.

So, having a care team that is really ready to go to bat for you in case that comes up, in my case, they were fantastic and it worked out.

But just want to put that out there, something that could could happen. And then really the first few months were very much like a clinical trial in the sense that I had frequent check-ins blood work.

I did have three month MRIs and we're still doing three months MRIs at this point, since I just started earlier this year, but you know, possibly by next year, we may change that it may be less frequent.

And also because it is a drug that is not you know widely available, you are only given so much when you first are given the drug and, at least for the first you know few months into it, so you might get one bottle.

Which means that you would have to physically go back and get the bottle or maybe they would mail it to you, depending on where you are.

And then, after a certain period, which I think for me, was the summer, I was able to get you know, three months worth of the drug because they had seen I was doing okay, we had adjusted dosage we were kind of in a

Good space, and so I I was able to kind of be a little more free in that sense. And I'll you know come back again to get another three months worth or so.

Let me just see here feel like I might be forgetting something.

They do yeah I was just going to mention real quick that you do have a diary as well, which is similar to a clinical trial and it's not as critical I feel like as the clinical when you're in a bigger clinical trial, but they do want you to obviously record your experience.

Jeanne Whiting: Thanks so much, Amanda and Dr Ratan. Very, very helpful before moving to questions, I just wanted to introduce Amanda.

She's worked for the Foundation for years as a volunteer and now she's marketing and social media director for us so every post you get from DTRF has Amanda behind it, and she does such great

Things so

We're thankful to you for what you do for DTRF and for sharing your story. Maneesh, let's move into questions.

Maneesh Kumar (DTRF): Sure, the first question is for you, Dr. Ratan.

You mentioned that this is an FDA pathway, so the assumption is that this is US only. Are you aware of a similar pathway in other countries, specifically in Canada?

Ravin Ratan: Yes, so I guess my my knowledge of the Canadian regulations is somewhat limited, although I would bet dollars to doughnuts that there is such a pathway available now if it's now

I doubt I can't speak to any specific drug because a company has to choose to make it available in that country um but yeah I mean did such a pathway I'm certain exists, I just I apologize for not having more details on that.

Maneesh Kumar (DTRF): That's okay. Would you suggest, going to the company's website to find out, or maybe the countries like equivalent of the FDA website? To try to do get more information?

Ravin Ratan: I guess my my suggestion would be that is certainly looking at company websites and so forth, is is good and a lot of patients have great patient-facing information.

I think your best option honestly is to seek consultation with someone who takes care of a lot of patients with desmoids right because they're going to have some sense of what's available and what's not.

You know, for some of those drugs that are commonly accessed in the compassionate access base, they'll likely know sort of what that's

what's involved um so you know I guess my bias is clear, but I think that you're treating physician is probably your best resource, especially if they take care of a lot of patients with desmoids.

Maneesh Kumar (DTRF): Great and then, I have a question for Amanda.

You mentioned that it took a couple of months from the time where you put in the application and then it was approved. Can you tell us more about during those couple of months, how you were feeling? Were you on a different

treatment during those months or

No treatment?

Amanda Hoffman: I wasn't and and that was difficult, I mean, and I think you know, depending on what situation you're in that's going to be more difficult.

For some people than others right um I was doing my best to manage my pain with some you know kind of things to manage my symptoms but

I was in kind of rough shape, so it was

A fingers crossed situation and just and waiting and hoping, I know that would obviously wouldn't be the case for some people, and actually maybe Dr Ratan can mention this. I if I had needed to do something in that period I'm not sure what the rule would be

In that case, but I personally didn't have to be on anything and just kind of toughed it out.

Ravin Ratan: I mean you're writing the protocol, and so I think, in theory, you could be on something I think the challenge that often comes is that

The reason that you're seeking compassionate access is there isn't necessarily a clear something to be on.

And I think that's the larger frustration and the larger challenge right is that you're waiting for a period of time, this is not always a fast process. It rarely is for for unless you're in the life-threatening situation, in which case that act, by the way, can sometimes be sped up.

So I think that's the challenge right is identifying something to be on when you're seeking compassionate access because there may not be an alternative readily apparent.

Maneesh Kumar (DTRF): And then also during that process, Dr Ratan, in your presentation, you mentioned is a lot of different regulations you have to do company, FDA,

So this question is actually for Amanda. How much communication did you get about that process from your physician or were you just kind of waiting until the end?

Amanda Hoffman: Yeah not not a whole lot and then the physician who initiated the process did

You know, after we decided okay we're going to go forward with this, you know he called me a couple weeks into it to just ask me some more questions. Because we had just started

To have a patient-doctor relationship. I was seeing someone else before that, who left, so I went through a lot of transitions but

But you know we just kind of I just gave him my history, basically, and that was kind of the only part that I feel like I

was able to take part in other than that so much is going on behind the scenes, and you know I would get updates, which was nice, I had a you know

The team would keep me posted and just send me a note or give me a call and say hey, this is still going on we're just waiting for this or whatever, but

yeah it is just kind of being patient, and you know I'd say you know if things I have talked to other patients who this process too much, much, much longer and I don't obviously know the details of why but

As Dr Ratan mentioned, it's an incredibly labor-intensive process there's a lot going on behind the scenes, depending on what your institution or your sarcoma center has the resources for

You know I mean, I imagine, this could be this could very pretty widely and and I think it makes sense I don't want to say that you as a patient are completely

at the mercy of this that you can call and check in you know I think that's Okay, and I think I did you know.

Maybe now and then to just say hey just curious about an update and like not to be annoying but just to say hey I kind of want to know where we are, I think that's totally fair, you know you have to advocate for yourself in that way.

Ravin Ratan: It's a struggle, sometimes to know what to tell patients, I mean I think it's limited utility for most people to hear that their application is currently with Bob at the IRB office.

That doesn't mean anything.

And so I think you're always welcome to reach out and say hey Is this still cooking and we're happy to tell you that it is right, but

yeah you know that that is a challenge right because waiting is waiting is tough, especially when you have something that you may have symptoms from that you have struggled with for some time.

So it's never it's never bothered to sort of reassure someone that things are moving, but you may not get real time updates about sort of where it is moving through the bureaucracy.

Maneesh Kumar (DTRF): Maybe you can comment on that duration of time is one or two months pretty typical or what's the range that might be expected?

Ravin Ratan: There's just different bottlenecks right so speaking to compassionate access generally right if you are in a situation where you need to get a drug

In a week or two to prevent you from dying is there sometimes a way to make this move really fast? The answer is yes, right and there are

lethal conditions for which that is done. Now, in the desmoid space is that what you should expect? No right, I think that the

The bottlenecks can vary, they can be institutional, based on how long IRB takes to turn it

Around. The FDA actually surprising You may be surprised here tends not to hold on to these for too long, it can take a couple of weeks, sometimes but it's usually pretty quick.

But then, you know the company has their process, they have to approve it, there really a medical review there.

And so you know I would not, I would not expect this to be a rapid way to get a treatment for your desmoid. But the compassionate access pathway and specific circumstances can move faster.

Amanda Hoffman: Something, Maneesh, sorry I just thought of something that might be important for some people.

You know I had I had been on this drug previously I knew that it was beneficial to me. I'm sure there are a lot of people

who are looking into this who are looking at a drug they've never tried before, and so I think just taking that into consideration as well, that if you if you don't know

What it's going to happen, just like factor that into the stress or the mental weight of it all, because I I knew what I was getting into right I knew what was coming if I finally got access to that to that drug and

And I don't know in what way, you know how much or how little the fact that I had been on the drug and did well might have helped me in my case or not I really don't know that that's

That that was part of it, but I just wanted to put that out there, that I kind of knew what I was getting into.

Maneesh Kumar (DTRF): Yeah that's that's a great point. I think one of the common things from this session in the clinical trials one was that this is still experimental. It's still an experimental drug.

So if you haven't had it before you don't know how it's gonna affect you. That might affect people different, so I think one of the keys here and is really having these conversations with your

Care team or your providers, and if that means going to the Desmoidian or you know, Google to get some more information patient empowerment I think is important.

Jeanne Whiting: Okay, well, thank you so much, very enlightening really appreciate it, Dr Ratan and Amanda, thanks so much for being with us today.

We have one more presentation to go. This will be from Dr Joe Germino, he is with the Bayer Corporation and Bayer is a manufacturer of sorafenib but also known as nexavar

So Bayer is not approved by the FDA in desmoid tumors. It is approved in other diseases, but so then we get to the question of how can patients get prescription assistance from us pharmaceutical companies. So Dr Germino, I'll turn it over to you.

Joe Germino: Thank you very much, Jeanne, and thank you, DTRF for inviting me, I think

One our goals, I think of all physicians whether we work in industry or we're in academia, or we're in private practice,

is to try to make sure patients get what they need, and if they have a desmoid tumor I've taken care of desmoid tumor patients in my past before I joined Bayer

And I know it's it's it's a very challenging disease and anything we can do to help patients is important.

And what I'm going to tell you today I'll show you some information from Bayer, but I think all the major pharmaceutical companies have very similar processes, so what I tell you will be very similar to another drug for another company.

First of all, Dr Ratan talked about unmarketed drugs, so the compassionate use but I'm going to tell you about is how to get access to marketed drugs.

And as as Jeanne said, there are two types of conditions with marketed drugs, those that are approved-

So, for example, nexavar is approved in kidney cancer. Most insurance companies will cover the drug for kidney cancer without a question, but if it's not approved by the FDA that's called off-label use. And off-label use

Very often insurance companies won't pay for. Unless there's an NCCN designation if it's a tumor. So if you have an NCCN designation as sorafenib does, for example, and they're all drugs as well,

Most insurance companies, they may have to be pushed, the doctor may have to write a letter but most insurance companies will cover off label use if it's NCCN listed so that's an important consideration.

But now let's assume it's not NCCN listed for whatever reason. How do you go about asking for a drug? Maybe it's an a class that

You would expect it to work. So if you want to think about it, I've got I want to show you the slide now. I'm going to show you some slides now but

This information I've given to the DTRF people, so you can get it from them, I think they're going to give it to you when you when I when I

When I finish. Okay, can you all see my my screen now?

Jeanne Whiting: Not yet.

Joe Germino: I know we went through this so I better not screw it up right?

Lynne Hernandez (DTRF): There's a little box at the bottom of your screen.

Joe Germino: Yes, I hit that I don't see.

Sorry guys.

This is why i'm in industry.

Lynne Hernandez (DTRF): Take your time.

I'll posted in the chat too, Joe.

Joe Germino: Okay, now we got my video.

Share screen, there we go.

Maneesh Kumar (DTRF): And then you'll have to select which screen, you want to share.

Joe Germino: Perfect, but only have one but yeah.

Is it showing up now?

Maneesh Kumar (DTRF): We see a sign

out screen on there. Okay.

Joe Germino: God I don't know how did I do this before. I think you just

Maneesh Kumar (DTRF): pulled up and just the wrong tab.

Joe Germino: Okay, let me start sharing trying to.

Share screen. OK, think I got it.

Now, you should be able to see.

Maneesh Kumar (DTRF): Yes, so now we see the application for the patient assistance.

Joe Germino: Yes, Okay, so in our website, there are websites, where you can apply and essentially

there's a couple of a couple of conditions, one is your insurance company has to deny, so if you get a rejection letter from the insurance company, that's fine. You don't necessarily have to appeal, but if you get a rejection, that's important that's part of the process.

The other thing to consider, and this is why they need to get the information, is the federal government has certain rules about how we can provide drugs to patients for free.

So if if you don't qualify because of the rules, then you don't qualify. If you do qualify, we have to know that, and so they asked a lot of financial information and that's tended to the back of the form.

And one of the things that can delay the process is if the information is not complete, so when you get the application, make sure you fill it out completely okay.

If your if your if your insurance company won't cover it, or if they do cover it, but the copay's too high, in in in if you have commercial insurance,

Most companies have a zero dollar copay, so you don't pay anything beyond what the insurance company pays. If you're on medicare or any other governmental assistance that is not legal, so there is a copay and the copay can be substantial.

If you can also then request copay assistance from the company, in which case the company will give you the drug for free. Won't charge medicare, won't charge anything governmental

governmental organizations, but you have to qualify for the income, the income levels as we, as we talked about.

Now what i've been told by the lawyers is, they are not allowed to consider the cost of the drug in your application. So let's say you have a drug that's going to cost you $10,000. That's a lot of money, most people couldn't afford $10,000 a month for 12 months.

But you might be able to afford $10,000 for a month. If you do not qualify,

And you buy one month of drug free, you can reapply and now the cost of what you spent on that month's drug can be factored into your eligibility criteria.

So if you get denied firsthand first application, do not assume you'll never get approved again because your financial situation will change as you outlay more money for medical care and part of the cost of the drug is is part of your medical care okay.

And so, how do you apply, there is a part your doctor has to fill out and again it has to be completed. If it's not completed,

it's going to take more time it's going to get kicked back to the doctor. It might get if it was from Bayer, and it might get kicked to me, and then I have to contact the doctor and the doctor would have to resubmit the information.

And then the bottom, this is, this is our there's a fax and there's a mail where you can mail the application to and the application form is farther down. So

I hope this is clear if anybody has any questions I'd be happy to try to answer them.

Thank you, thank you for your attention and I wish all of you the best.

Lynne Hernandez (DTRF): Thanks, Dr. Germino.

Jeanne Whiting: Would you like to handle the questions now?

Joe Germino: Sure if they're not hard.

Maneesh Kumar (DTRF): Sure I'll just remind anybody if they have questions to post them in the Q&A, not into the chat and the Q&A is much easier for us to follow.

Jeanne Whiting: But just to clarify, Joe, this form is right on your website.

Joe Germino: That's correct, that's correct.

So you can download it.

Jeanne Whiting: But it has to be snail mailed. You can't email it in, you can't, if you don't have a fax machine which most people don't anymore.

Joe Germino: Um you know I don't know what the I'm sure there's a website, I'm sure there's an email, but I don't know what it is. If you if you want I'll try to find out, and then I can email to you, and you can let people know.

Jeanne Whiting: Just wasn't on the form there.

Joe Germino: yeah I know I it's

it's

I'm sure that what they want to do is make sure it goes to the right person and because

People change it may, it may get lost.

Jeanne Whiting: Do they acknowledge receipt if you do mail it? Do they acknowledge somehow that they've received it?

Joe Germino: I believe they do, on the other hand

If if, for example, you mail something to Bayer and you don't hear,

You can certainly contact the Medical Communications department, the Medical Affairs Department, and they will track it down. Usually they'll send me an email and then I'll track it down, but we will find out where it is the process and let you know.

Jeanne Whiting: Okay, thank you.

Maneesh Kumar (DTRF): Just, just to clarify this

This is for drugs that are approved or marketed just not approved for desmoid?

Joe Germino: Correct correct.

Maneesh Kumar (DTRF): Okay.

Joe Germino: It's called off label use. Okay.

Maneesh Kumar (DTRF): So then, this is a question I guess for that and also for perhaps Dr Ratan.

So if you get a drug on this off- label, use are you able to take a break from it

and get it back again.

Joe Germino: Yes, you are, you can take a holiday, you can you can stop, and then, if you decided something, because I mean honestly for most cancer patients, it's not usually an issue for desmoid patients because it's a different type of tumor.

That is certainly a possibility and again, you can reapply or get the drug back so it's not a problem. Okay.

Maneesh Kumar (DTRF): So for Dr Ratan is it a similar thing if you're getting it on compassionate use now, instead of off- label?

Ravin Ratan: So I think that's often going to be governed by the by the rules, the sponsor sets forth and so more of your, so I think that

You know what what Dr Germino was talking about is access to a therapy, which may or may not be FDA approved

But as a function of being listed in the NCCN guidelines can be considered a relatively standard there.

There's a lot of latitude there to use it how you want to. Getting access to the drug isn't so much the issue that we're talking about assisting with it. It's paying for it, which can be a barrier right.

Compassion access is different it's not about the payment the drug is most often provided for free. It's

Can you get access to this drug because you can't go to a pharmacy and pick it up and no commercial pharmacies going to mail it to you, so there's sort of

different issues. Now say for compassionate access, there is more flexibility than standard clinical trials, but less flexibility than using a drug off label with copay or

Payment assistance.

Joe Germino: And I just

I would reiterate that

Having worked at a pharmaceutical company, you know getting access to a drug if it's very early in development is very, very difficult because they may not know what the right dose is.

They may not know enough safety, and so it is very, very difficult if it hasn't progressed at least past phase 1 so

And yeah and that's why going to a health care professional knows about desmoid and knows what's available you don't waste your time looking for access to a drug, which you have no hope of getting access because it's just too early in development.

Maneesh Kumar (DTRF): And then Ravin and Joe you both mentioned and NCCN guidelines, maybe Joe, you can start what you'd be able to explain what those are to our audience?

Joe Germino: Yeah sure these so for for for tumors, NCCN has a number of different tumor types and there's over 25 and I don't know if the 50 whatever but

Maneesh Kumar (DTRF): desmoid... Can you define NCCN as well?

Joe Germino: It's a group of I think 30-35 institutions that have come together and formed this this cooperative group

And most of their their different committees like there's a sarcoma committee, and they are drawn from people in these academic institutions

And they look at the data for various trials even early trials and they basically assign a category of level of evidence, Level one meaning like a clinical trial was positive,

And Level 2, Level 3, and as long as there's a reasonable amount of evidence that the drug may be beneficial and not terribly harmful, they'll give it a 2A or 2B, and then the insurance companies, will try to cover it.

Ravin Ratan: I guess just to expand on that, I mean so yeah so the National Comprehensive Cancer Network guidelines right. So these are basically meant to define

A standard of care for most tumor types right so there's most cancers out there will have either a guideline of their own, or a subset and desmoids are included in the soft tissue sarcoma guidelines.

And so the purpose is one to describe what appropriate treatment is so if you're a doctor practicing the community wants to get a sense of hey what's the right thing to do, for a patient with diagnosis X.

The NCCN guidelines are sort of the standard place to start certainly in the United States.

And so I think that is that sort of one of their key functions.

But then they have the added benefit of if something is described on there something that you might do

It sort of elevates it as a reasonable therapy for a disease so it's not just someone making something up - not that we would do that, but you get the idea, and so I think that's that's sort of how these are how these are viewed.

Joe Germino: And most most most payers most insurance companies that I've dealt with in my job, have said they will pay if it's NCCN- listed.

I don't know if they actually follow through on that and how many hoops you have to jump through but, but they will pay for it.

Jeanne Whiting: Could I just ask a question? I don't know if you can answer, feel free to decline, how much does sorafenib cost per month, if you don't get coverage?

Joe Germino: Okay, so good question. I'm a doctor and, like most doctors, don't know really exactly what things cost but I've been told it's probably on the order

Of $18,000 a month, which is an enormous amount of money for the standard dose. Now for desmoid it's it's it's not it's not the standard dose but um

But it's still a significant amount of money, and I would say 90% of the population 95

Unless you're Jeff Bezos or something you could not afford paying for the drug out of your pocket if the insurance company said no, and so, most people

will qualify for free drug if their insurance company will not cover it. Although, as I said, you have to meet federal guidelines for coverage.

We don't get to set well if the insurance company won't pay for it will will give it to you free. In my my my world, I would say yeah fine.

But that's not the way it works. And and that's why I said that if you are near qualification, but you just miss it if you if, you can get the drug for one month and then reapply you, may qualify then. So it's not just because you didn't qualify in your initial,

You won't ever qualify. That's not the way it works. It's it's really financial, it's really financial.

Maneesh Kumar (DTRF): Thank you, I think that will cover the general process.

But another follow up question if your insurance company says they'll pay for half of it, can you get prescription assistance for the other half, or is it an all or nothing?

Joe Germino: So um it's funny you should ask that because

I actually before this meeting sent it to mark Marshall colleagues, because they run the zero dollar copay program and they they they weren't sure, but I believe I believe if they only pay half

You will have zero dollar copay but if there's an issue

If there's an issue, I mean that's part of my job is, if there's an issue they can contact

Medical communication there's a medical communication site for all the drug companies. Contact them and say you're having trouble with you're getting the drug.

Can you talk to somebody in Medical Affairs and I know at Bayer, it'll come to me and I will do my best to resolve the issue, however, however, we can.

Maneesh Kumar (DTRF): Fantastic. Well thank you so much, Joe, and all of our panelists. I think we're at time so I'm going to hand it back over to Lynne to close us out.

Jeanne Whiting: Well I'll have a few announcements before.

I just want to thank Lynne and everybody who's made this meeting possible our presenters and everyone whose worked behind the scenes. This will be available this webinar will be available for you to watch again if you want, on our website correct, Lynne,

After the meeting, and it really it's an invaluable educational resource, so thank you all for helping us create this.

Just a couple of announcements, please remember to join our live RFA Virtual Challenge Ceremony which is tomorrow at 11am Eastern.

This is not just if you participated in fundraising you've just got to be there, and if you didn't, you still want to be there, cause this is fun and Lynne

and her team put so much into this and you'll just get so much enthusiasm for what we do to make all of our research possible and you'll feel part of the community.

So just attend for fun tomorrow if if not anything else, and thanks to everyone who raised money because we're going to learn about

reaching our goal, though I'm going to have Lynne explain share this story tomorrow. We're also hosting one more Desmoid Tumor Awareness Month event.

So all of September we've been holding these events and having a lot of posting, sharing our stories.

The big event that will be coming up will be a live Facebook and instagram broadcast on Wednesday, the 29th at 4:30pm. We will have one of our top desmoid experts, Dr Raphael Pollock from

Ohio State speaking on the question of categorization of desmoid tumors - cancer? benign? What's in the name? What's the impact of the name?

What you and your physician really should know about all the confusing terminology that is used to describe desmoid tumors and how it might impact your treatment and your journey.

So, again that's Wednesday the 29th at 430. It's still there's still time for you to be part of our awareness month by sharing your stories check out more information at

dtrf.org/awareness and I have to say I'm very active on instagram I have just loved opening instagram every morning. I don't follow a lot of people.

I follow my family members and a small group of others, but to have desmoid patient stories out there on instagram and it's just been wonderful. Thank you so much for

being part of that and sharing your story. It's just it's just great like it was last night when we all met in our networking event.

So we're going to send you a survey after the meeting. Why do we send a survey? Because we always want to approve what we offer to you. We work all year we actually will start in about a week planning for 2022 events. Our research workshop, our patient meetings, our interactive

connecting seminars that we hold throughout the year, not just once a year. So let us know, through the survey. Save the dates for 2022 for our annual patient meeting, it will be September 23rd, 24th and 25th will be our next

2022 DTRF events, and thank you it's been wonderful weekend. It's not over yet, come tomorrow to the ceremony, and thanks to everybody who made this possible. Have a great day, and great evening and great night wherever you may be located.

Bye bye.