

Barbara Van Hare - DTRF 2022 Patient Meeting Webinar #1

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Jeanne Whiting: We'll go on to our next speaker now. This is going to be Barbara Van Hare who is with the Rare Cancer Research Foundation. We partner with this foundation in a very important program for donating tissue for research. Before Barbara gives the details of this, all you have to do if you're gonna have surgery, as you've learned, surgery is a lot more infrequent now in desmoid tumors, given all the things we've seen about. It is no longer the first line recommended treatment depending on the location of the tumor. And this active surveillance approach has become the predominant way of starting watching these tumors.

But we still need we still need tissue for research and it is becoming harder and harder to gather. So if you know you're going to have a surgery, there is a simple way to go online and consent to have your tissue donated to research, and Barbara's gonna talk about that. Thank you for being here, Barbara.

Barbara Van Hare: Thank you so much. Let me share my screen here. Can everyone see it?

Okay, well, I'm Barbara Van Hare and I'm with the Rare Cancer Research Foundation and our initiative Pattern.org. I'm the Director of Partnerships and I wanna talk to you for just a few minutes about the power of tissue donation which we try to make it fairly easy through our platform, Pattern.org.

And the Rare Cancer Research Foundation is a nonprofit. We were founded by a rare cancer patient who not only was frustrated after finding all the barriers to

his particular cancer journey and that the treatments hadn't really changed in his case for his disease in over 25 years. So when he was diagnosed in 2012, he started working on a foundation that could help bring down the barriers for each and every rare cancer, and now also for rare diseases.

So, the Rare Cancer Research Foundation launched Pattern.org, and basically its goal is to enable patients to directly donate fresh as well as stored tumor tissue. But right now we're really working on the fresh tumor tissue and medical data to

Jeanne Whiting: Barbara, could you just explain what fresh tissue is?

Barbara Van Hare: I will. Fresh tissue means that it's coming directly from surgery. It's not stored in a biobank. Literally the surgeon or the pathologist will take the tissue excess tissue that's not needed for a patient's care and take some of that and put it in a vial that we have sent in that has kind of some growth media so that the tissue can stay alive.

And then we send that to a lab overnight. So it's there the next morning and it's used for creating models so that they can better study the disease. And I'll talk about that in just a minute in a little more detail. And so basically Pattern allows patients to connect with scientists, empowering the patients to really partner in the research and help to improve outcomes because really patients are at the center of everything that we do, everything the doctors do, and so bringing the patient as part of the research process, that tissue is so valuable to help the researchers learn about the disease and how to find new treatments for it. So each one of you as patients, and also as if your caregivers for a patient, you have the power to make a difference. And so the importance of tissue donation is really to help advance rare cancers and rare disease research.

First of all, the name where it's a rare disease or a rare cancer means there aren't that many patients. And particularly when you look at all of the institutions out there, there are limited patients that may be seen at any given institution. When you have a surgery, usually some of your tissue is stored at that hospital.

A lot of hospitals though don't store tissue and they may actually throw it away, or ones that do store it may not fully utilize all that tissues there, and so it may sit in a biobank for years and in some cases never be used. Because rare diseases are rare, researchers don't have access to that tissue because it's rare.

And also historically, most institutions don't readily share tissues that they get from their patients. This fresh tissue, as we've talked about, is used to create models, things called like cell lines or organoids, or in some cases they can put

the tissue into like a small rodent. And that's how scientists learn about the disease, how it grows, what's causing the disease.

And then further another challenge is a lot of researchers, once they learn about a disease, or make models, they don't share them until after maybe they've published a paper. And in some cases, that can take years before a paper is published. So literally, you can help speed up this process to hopefully find new learnings and treatments through donating excess tissue.

As I mentioned, disease models help understand the biology. It can help test and identify ways to diagnose and prevent diseases, and also identify new therapies and hopefully cures. So how does Pattern work? Basically, patients learn about research studies for their disease through things like this webinar, through social media.

We work closely with DTRF as well as other research and advocacy organizations. Once a patient comes to Pattern, they put in basic information such as the type of the disease. If they're having surgery where they're having surgery. And then we match that patient with a study that's in need of that particular tissue.

Once the patient has gone online, put in this information, they're matched then actually online, the patient can sign the consent. Immediately after the patient signs the consent, we're notified and we start working on contacting that patient. It may be surgeon, it may be oncologist, it may end up being the pathologist will work with that institution and care team will send in a kit that has everything that the doctor needs to put the tissue as well as some blood into the kit and then we'll arrange for it to be all overnighted at our cost to the institution where it's going.

So where, what is Pattern's role versus the researcher? Sometimes people are a little confused on what we do versus what the researchers do. Basically, we are a biologistics, we aid in the biologistics.

The patient can consent online. We're the ones that coordinate that tissue donation. We're the ones that send in the kits. We get it to the institution. We also will have patients sign a medical record release so that we can get the medical records. We de-identify that information so that the researcher and others don't know anything about the patient except for de-identified data.

Then the researcher wherever they are located, they're the ones working to create the models. They do the various genomic or, or have someplace else do

the genomic profiling and then one of the agreements that we're very insistent on for a researcher or an institution to work with us, they must make that data available and put it in the open domain so that other researchers worldwide will have access to it.

To date, Pattern has had patients consent, almost 400 patients consent, across 171 different institutions across the United States. We have collected 304 tumor samples actually from 107 institutions. In case you're wondering why the collections are less than the consents, many times patients will consent and then, prior to their surgery, maybe the doctor will try them on a different therapeutic option. And so, therefore surgery's not an option. Maybe for some other reason the surgery is canceled. So that's why we usually have between about 50% of the patients that actually consent we're able to collect the tissue.

We've collected from 42 states and Canada. We have not gotten fresh tissue itself out of Canada. We've gotten some fluid for some of the other diseases we work with, but we hope that by sometime next year we'll be able to get tissue out of Canada as well. And over 20% of the cases that we've collected comes from minorities. As far as actually what we've received from the desmoid community you all are a very engaged group and we wanna thank you so much for that.

We've had actually in the past about four years, 45 different patients have consented and they have been treated at 43 institutions. Of those patients, we've actually collected 18 samples. And again, these are fresh samples, not stored, so directly from surgery from 18 different institutions. Again, that number is slightly lower than our average because in the past few years, as all of you know, and as Dr. Pollack talked about, is there are some new treatment options where a surgery used to be the key one. As far as the research progress on desmoids thus far in the past we were sending them to the Broad Institute of MIT and Harvard in Cambridge, Mass. From that project, they were actually able to date be able to create two long-term cell line models.

A cell line is similar to a living tumor, but it's, I'm gonna say, grown in a tube or in a dish. That is, those two cell lines are undergoing CRISPR and drug screening right now at the Broad, and the actual models themselves have been sent to ATCC, which is a public biorepository, so that in the near future, those will be accessible to researchers worldwide. They can actually order them from there so they can do additional research on them. There are additional desmoid cell lines in process in the labs there at the Broad, and by mid next year there should be a priority list of drug targets and potential lead therapeutics hopefully with additional key learnings for desmoids.

However, as was already mentioned, we need more samples. The team that was working on the Broad on this project has literally moved across the street from the Broad [inaudible], so the same folks are working on the desmoids, as they did before. They're just in, literally across the street. So now we'll send the desmoid, the fresh desmoid samples to the Koch Institute. The work that's being done currently is being funded by a Department of Defense grant and would appreciate if you know of anybody having surgery, or if you're having surgery considering donate the excess tissue.

Some key information, as we've mentioned, is we utilize fresh tissue from resections. It is only excess tissue that we will take. If everything that the surgeon takes out is needed for your care, that is fine. We want the patient to be taken care of so we'll only get tissue if there's excess tissue. Whether you consent through us or don't, it does not affect your clinical care. The average sample size that we get is 0.5 centimeters cubed. And for model generation, we are asked this a lot, can we accept biopsy samples for the projects creating living models? [Audio Dropped] By this time next year I'll have some exciting information to tell you where we may be able to utilize some stored tissue.

You need to consent prior to the surgery because this is living tissue that we need. If you could give us up to a week even if it's a few days, we will do our best to coordinate with the hospital in order to get your tissue out. We do protect all the patient data. It's de-identified when it goes to the lab. All of the data that we get, including your medical records, are encrypted and stored in a HIPAA compliant data server. A patient can re, can revoke consent at any time, either before their tissue donation or even after they have donated.

There's no cost to the patient and currently the project that we're working on is for general research only. So no results are returned to the patient to help inform clinical care. But again, hopefully by this time next year, I'll have some positive news to share in that we're trying to work so that we can provide some value back to the patients who are donating their precious tissue.

So you as patients have the power to make a difference, and so please consider donating tissue if you have an upcoming surgery.

Jeanne Whiting: Thank you, Barbara. One question we had to clarify, can it only be tissue that is harvested within the United States, or can it come from other countries?

Barbara Van Hare: Right now it is just in the United States. We hope to be collecting tissue from Canada next year. The challenge we have with that is

there's some restrictions with Canada, with some of the media that's used to keep the tissue fresh, fresh and possible delays going through custom. So right now it is the US. At some point we would love to expand on that beyond the US and after Canada. But it kind of depends on when you're shipping fresh tissue there are a lot of customs and laws and regulations you have to work with.

Jeanne Whiting: Okay. Thank you. Thank you so much for your presentation. I just wanna say that there are some really important things that we as patients can do to help move toward cure in our own disease, right?

Whether it helps us now, whether it helps us long-term research, tissue is critical, and your tissue is like gold. You can leave that gold in a archive of some kind that might never be used, or you can share your gold at no harm to yourself, to the research community. Like Barbara mentioned, DTRF has been funding a project at the Broad Institute of MIT and Harvard.

You can donate to research in your disease, it's now moved over to MIT at these top research institutions through a very simple process. So thank you so much, Barbara, for your presentation, and thanks- if you want more information, all this is, on our website, on the homepage, or just write to us.