

Children's Cancer Hospital - Aerosol Therapy
Webcast
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Peter Anderson, M.D.

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Andrew Schorr:

Hello, and thanks for joining us once again. This is Andrew Schorr with another edition of Patient Power on mdanderson.org. Thank you so much for joining us. It's incredibly satisfying for me to have this opportunity to talk with researchers and clinicians who keep moving things forward in cancer care as they do at M.D. Anderson and then a lot of people who are just so inspiring as they find out they're diagnosed with cancer, end up going to M.D. Anderson, and often participate in clinical trials or innovative care hoping for the best for them. I like to say, and I guess I get one too, is so many of us who have gone through cancer care get a purple heart for what we go through, but what's nice about it is, if you have been to M.D. Anderson, you know that everybody is incredibly upbeat, and it's a very positive place.

Well, somebody who is at that positive place right now is just 19 years old. That's Jennifer who happens to live in Houston, and imagine Jennifer's story. Jennifer was thinking about graduation from high school and then was to attend Texas A&M, and then things weren't going so well. Jennifer joins us today, we will set the stage, from her room at M.D. Anderson where just two days ago she had surgery on her lung, and we're going to explain what we're dealing with, with her, and then innovative therapy that is helping Jennifer and other children, in particular, with aerosol therapy for cancer care, and we'll meet Jennifer's doctor. But first I want you to hear just a little bit of Jennifer's story. So, first of all Jennifer, thanks for doing this and from your hospital room, we really appreciate it.

Jennifer:

No problem.

Andrew Schorr:

So, Jennifer, let's see if I get this right. Nineteen years old, softball player, active, and you started to have pain in your hip? Is that right?

Jennifer:

Right.

Andrew Schorr:

And so naturally, and I have a son who's on the cross country team, you think, well it's a sports thing.

Jennifer:

Yes.

Andrew Schorr:

And so you go to sports doctors, and sometimes you will wind up with physical therapy, and so in January/February of 2007 that's what you were doing, right?

Jennifer:

Yes. I was going to all different kinds of physical therapy places all over Houston, and it was just like nothing was getting better. So that's when my mom thought something was really wrong.

Andrew Schorr:

Well, let's mention your mom, Mary, is a nurse.

Jennifer:

Yes.

Andrew Schorr:

So, her radar was kind of up when her older daughter was not getting well. So, you had an MRI, and that was of even more concern, right?

Jennifer:

Yes, because my symptoms were such that I could not lie still. I was limping around and stuff. So, with MRIs you have to lie there for forever, and so I was crying lying on the MRI bed. It was bad.

Andrew Schorr:

It was painful in your hip?

Jennifer:

Yes.

Andrew Schorr:

Okay. So, you end up being referred to an oncologist.

Jennifer:

Right.

Andrew Schorr:

Let's meet the doctor. You call him "Dr. Andy."

Jennifer:

"Doc Andy."

Andrew Schorr:

"Doc Andy" is Dr. Peter Anderson. He is a professor at M.D. Anderson, and he's a pediatric oncologist. Thank you so much for joining us Dr. Anderson.

Peter Anderson:

Glad to.

Andrew Schorr:

So, this young woman comes to you. She has had workups by other doctors, and now she has had this MRI, and she has the pain in her bone. What does it turn out to be?

Peter Anderson:

This turned out to be a rare tumor, a bone forming tumor, called osteosarcoma. There are only about 1,000 cases a year in the U.S., but when they happen it requires quite a bit of different therapy to have the best outcome, not only surgery but chemotherapy as well.

Andrew Schorr:

Now, she had a tumor in her hip and her femur and then maybe some spots of cancer that had spread to her lungs?

Peter Anderson:

Correct, and once you have it outside of the primary site in the bone, it becomes much more serious because then you're dealing with recurrence in these other places. So, Jennifer living in Houston was in the right place at the right time to start getting additional therapy to these other areas.

Andrew Schorr:

Jennifer, were you familiar with M.D. Anderson at all, and did you know it was the number one cancer center in the country?

Jennifer:

Well, I mean, I knew of M.D. Anderson, but I was pretty pumped when I heard it was the number one cancer center. I mean, I couldn't even imagine being from, say, New Jersey. I met a lady from New Jersey yesterday who was down here for her first time. I couldn't imagine coming down here and staying here for a year. I mean, I like my house.

Andrew Schorr:

Right, right. And your family and friends can come see you.

Jennifer:

Yes.

Andrew Schorr:

So, there you were and finishing high school, and I am very focused on that because my son is doing that this year, and so you were to have the prom and graduation, but as Dr. Anderson was saying, you were starting very aggressive care, and that would be chemotherapy and radiation. So, were you doing that while you were trying to go the prom and graduate, and were you able to do all that?

Jennifer:

Yes, actually I had prom May 5th and then I got my port put in the day after prom, that Monday. So, after prom I got my port put in, and then I got my chemotherapy. I got sick. I was in the hospital for a little bit, and then graduation was May 26th, and I had to speak at graduation and everything, and I did it. I hadn't lost my hair yet then or anything, so that was good.

Andrew Schorr:

What high school did you graduate from?

Jennifer:

Pearland High School.

Andrew Schorr:

Congratulations. Now, we should mention that the area where you have had treatment at M.D. Anderson, and where Dr. Anderson is a professor of pediatrics and a physician, is at the Children's Cancer Hospital, which is a hospital within a hospital, right Dr. Anderson?

Peter Anderson:

Yes. It's a very child-friendly environment, both in the hospital as well as the clinic. Although M.D. Anderson is a large place, it's kind of a base of operations for the children to get all the services that M.D. Anderson has to offer using a child-friendly environment, and also teenage and young adult friendly. We also have other special services for these age groups too.

Andrew Schorr:

Let's talk about Jennifer's care and one approach to delivering medicine that is helping her but also can help so many others, and that's what you call aerosol therapy. So, you've been involved in the research and the development of that, Dr. Anderson, with some other of your colleagues for many years. What is aerosol therapy? What's the advantage for kids like Jennifer or maybe even other kids who maybe don't need quite so many modalities coming into play?

Peter Anderson:

I think of aerosol therapy as really trying to give lung-specific therapy. If one of your major concerns is disease in the lungs, it makes a whole lot of sense to try to

get your drug to the lungs directly. The model of care for this for many years has really been cystic fibrosis, and look at the advances they've made. There's a wonderful book out there to read called *Better* by Atul Gawande, and what he does is he looks at how areas of medicine can continually improve and using Warren Warwick, M.D., as an example in one of the later chapters. In cystic fibrosis they will give antibiotics by aerosol, so the kids and young adults and adults who live a long time with this formally fatal disease will breathe in their drugs, and they'll also do this for enzymes that help the secretions. They kind of led the way in probably a decade or two before any other field did. In cancer we're trained to give drugs by pills or IVs, but then you wonder do you really need to treat the whole body and have all the side effects from treating the whole body? So I became interested in aerosol therapy in the late 1980s/early 1990s when my research at the University of Minnesota began to take a direction toward sarcoma, and also Dr. Kleinerman here took the same direction of research too, so I got to know her from way back when.

Andrew Schorr:

Now, as far as what aerosol therapy is; so, the idea is that somebody is just breathing it in. It's like a breathing treatment like you'd think of for asthma for example?

Peter Anderson:

Yes, exactly. There's not only small, little metered-dose inhalers that you would put in your pocket or purse, but there are small machines that you can put a liquid medicine in, and it will generate a mist that you breathe for anywhere from 5 to 15 minutes.

Andrew Schorr:

Right, I used to use a nebulizer. One of my kids has asthma, so when he was an infant, we used a nebulizer too. So, same idea?

Peter Anderson:

Exactly the same.

Andrew Schorr:

So, there you are. What sort of medicines are you able to deliver, and take in Jennifer's case, just to bring it back to her, so Jennifer was having kind of the full boat radiation and infused medicine, but also you wanted to be able to have her have medicine at home in a simple way without needles and all those kinds of things and reducing the side effects as well. So, would Jennifer have some nebulizer kind of device with certain cancer drugs at home?

Peter Anderson:

Yes. The drug she got was the GM-CSF, which is an immunostimulating drug. The reason we chose GM-CSF was back in the 1990s when they looked at how do you

get the immune system to recognize tumors and reject them, they tested a wide variety of natural immunostimulants that white cells made, and GM-CSF was the winner. So, what we did was we started giving patients a small dose and then see if they had side effects, and to our surprise unlike IV GM-CSF or GM-CSF under the skin, it did not give you a high white count in the blood or bone pain from stimulating the bone marrow everywhere. This was lung specific and very nontoxic, so it was very well tolerated. We went on to actually show in pulmonary function tests that it was safe. For patients like Jennifer where if there is an area that you'll be concerned about the most in the future it's a recurrence in the lungs. That is major surgery. She can tell you all about that today.

Jennifer:

Yes.

Peter Anderson:

And it's not something you would wish somebody to undergo two, three, or four times. So, if you can get the immune system in the lungs to be functioning as good as humanly possible, they might have a better chance of having fewer surgeries.

Andrew Schorr:

Now, Jennifer, you had surgery in the summer and then again now in the end of September. So, what Dr. Anderson is saying is he would like to avoid that or certainly for you too, avoid future surgeries.

Jennifer:

Yes.

Andrew Schorr:

You've had this aerosol delivery of cancer-fighting medicine. What do you think of that approach?

Jennifer:

I like it because I can do it at home, and it doesn't make me sick whereas with the other chemotherapies I have to carry around this huge backpack full of the medicine and a pump, but the aerosol thing; it's like I do it once in the morning and once in the night for a week on, and then I get a week off. It kills the cancer, or it doesn't kill it, but it helps kills the cancer; so, I like it.

Andrew Schorr:

Well, okay, that's quite a good testimonial. I imagine you have children of all ages now and their parents using this, Dr. Anderson.

Peter Anderson:

That's correct. I guess one of the things we try to do in the Children's Cancer Hospital is maintain as normal of a life as possible for these families who have lots

to do already and then can you avoid hospitalizations and do things as outpatients? This is a good example of an outpatient therapy.

Andrew Schorr:

Okay, we're going to take a short break, and when we come back we're going to understand this better and understand how it applies to different cancers. Jennifer, we want to hear from you and what you'll say to other young people who where it doesn't turn out to be a sports injury, but it turns out to be a cancer, and you're a great inspiration to all of us. So, we will be back with Jennifer and Dr. Peter Anderson when we continue. Stay tuned for more Patient Power brought to you by M.D. Anderson Cancer Center. We'll be right back.

Andrew Schorr:

This is Andrew Schorr as we continue our program on Patient Power on mdanderson.org. Thank you so much for being with us. We're visiting with folks who are either in or work in or hospitalized right now in the Children's Cancer Hospital at M.D. Anderson, a hospital within a hospital and a very special place devoted to young people. Jennifer is 19 years old and recovering from lung surgery there just a couple of days ago. She is from Houston and is being treated for osteosarcoma, and that has been in her bone in her hip and her femur and then also had spread to her lungs, and we're talking about aerosol therapy with her doctor, who is Dr. Peter Anderson. He is one of the researchers and clinicians who has helped develop it, and he is explaining how getting the cancer-fighting drug in a simple way directly to where you need it, in this case Jennifer's lungs and other children as well and adults that we'll talk about in a second, directly to the lung tumors, and as the folks at M.D. Anderson like to say, let kids be kids. So, Jennifer, you could have this aerosol therapy when you've been at home and then just go on with your life, right?

Jennifer:

Right, no doubt about it.

Andrew Schorr:

So, Dr. Anderson, in this case we have cancer that's spread to the lungs; somebody else might develop lung cancer; and also other ages, either much younger or an adult. Where does aerosol therapy fit in?

Peter Anderson:

The way I see it is each therapy has a different mechanism of action, and in some cases a therapy will be able to do everything like a curative surgery. In other cases it will take combinations of therapies. So, the aerosol therapy with immunostimulating drugs will probably work best with chemotherapy by increasing the number of white cells where they're needed in the lungs to try to get the best effect, not only killing the cancer cells but having the immune system clear them and be able to recognize them and prevent cancer from recurring in the future.

So, I'd say in combination with the immunotherapy, we're also developing some aerosol therapies that are chemotherapy that you breathe directly into the lungs. L9NC is an example of a protocol we recently did for that, and we're still looking at that for Ewing's sarcoma. In the future, we will probably be doing gemcitabine based on very exciting work that was first done in Dr. Koshkina's and Dr. Kleinerman's lab here showing that if animals with osteosarcoma inhaled gemcitabine, they had very few toxic effects, if any, and it was surprisingly effective, much more effective than IV. So, we've gone on to treat dogs with osteosarcoma, and those results look quite promising too. They're being done by Carlos Rodriguez, Ph.D., in California. If you don't know, dogs get osteosarcoma more commonly than people. These are like big dogs, so it's a natural model to try these new therapies.

Andrew Schorr:

And what about adults?

Peter Anderson:

In adults, the way I see it is these bone cancers tend to occur not only in children and adolescents but also young adults and they would get the same kinds of therapies. For example, we had a woman come for surgery at M.D. Anderson. She was responding to the chemotherapy we had recommended and had very successful thoracic surgery and then went home. She has three children she is taking care of, but she can do the aerosol therapy in the morning and in the evening seven days on and seven days off and really maintain a very full and functional life, so it's been fun to hear how well she's doing.

Jennifer:

Yes, I just met a lady who has my same kind of cancer. She has sarcoma that spread to both lungs, and she got her surgery in August the day before me, and then she got her surgery in September the day before me, so we've been chatting.

Andrew Schorr:

Has she had the aerosol therapy, do you know?

Jennifer:

I'm not sure.

Andrew Schorr:

Dr. Anderson, you're saying for a woman like that; we don't know her exact situation; but then that might be something she'd do at home?

Peter Anderson:

That could be considered too, sure. Then I guess the other way you can look at the aerosol therapy is there's another drug called MTPPE, which was developed for

osteosarcoma and has recently been looked at by the FDA and probably looked at by the European regulatory agencies, and that might work in combination with the aerosol GM-CSF better than either alone because they both work on the same kind of cell, but you would have more cells from the aerosol therapy and more active ones.

Andrew Schorr:

Now that brings us to an important point for people who may be listening to this program all around the world. So, you have been devoting, you know, 20-some-odd years of your life researching this, developing this, Dr. Kleinerman and others you're working with now all, it's like a family, come together at M.D. Anderson. So, if someone has one of these very serious cancers, then they might benefit from being, for example, in I imagine one of your clinical trials that you have at M.D. Anderson as you explore new medicines that you can deliver in this way.

Peter Anderson:

Oh absolutely, and in fact a lot of times what we'll do for patients who don't live in Houston is they will come here for a consultation, get a plan put in place, and then be able to go home and carry it out. With the aerosol therapies, we've even developed technologies that a patient can go home and they can take a device that will monitor their lung function. It uses their telephone essentially to send pulmonary function tests back to us, and that way we can keep track almost better than if they lived in Houston. So, distance is not as big of a problem as you would think it is to participate in aerosol clinical trials because the patient gets their treatment at home whether they live in Houston, Austin, Dallas, or Seattle.

Andrew Schorr:

Wow, or wherever. So, Jennifer, you're listening to this as I am, and it probably confirms for you that you're in the right place.

Jennifer:

Right.

Andrew Schorr:

And so, a little bit about you, Jennifer. So, you're going to go on and have these therapies. What's your hope for the future?

Jennifer:

Well, Dr. Anderson just told me that I was going to have to get six more chemotherapies after this, so I'll probably do the chemo's and stay around here for I guess six more months and take some classes at a junior college in the spring, and then next fall, hopefully, I can go to Texas A&M and do what I was supposed to do this year.

Andrew Schorr:

Well, I sure hope so. My producer was even saying "Go Aggies" so let's hope you'll get to be an Aggie. You know, I just want to mention, I get to do a lot of these programs, many on cancer, but I do some that are not as well, and it was only a week or so ago that I interviewed a 19-year-old college student on a scholarship at a college, not in Texas, but at an outside college, and guess what? She has exactly what Dr. Anderson was talking about earlier, and that's cystic fibrosis. So not only is she able to run, but the college gave her a scholarship, and she has treatments that she does in her dorm room, and her friends help her and support her. So it may be, Dr. Anderson, as Jennifer goes on that hopefully there will be therapies she can just put in her pocket or do in her dorm room, and it becomes just a natural part of her life to continue fighting the cancer. Can we have that dream for her? Is that a fair one?

Peter Anderson:

Oh that's absolutely a fair one. Currently each drug has its set of side effects, but what we're trying to do is chronic therapy, and cystic fibrosis is the model that if you can provide a therapy that's tolerable for a long period of time, people would rather do that than be very, very sick with a lot of side effects, and the aerosol therapy is one way to achieve that.

Andrew Schorr:

And it is changing, and as I told that young woman where, you know, people as you said now we have adults living with cystic fibrosis, and that's the model, and we want to do that with these cancers as well; I said to Erin, is her name, my dream is that you'll go jogging with your grandchildren. Well, the same thing for you, Jennifer, is we want you to be active and play softball with your grandchildren and whatever therapy that you may need along the way that it can be convenient and less toxic. It looks like if I've got it right, Dr. Anderson, what you're finding is with some of these therapies that did have toxicities if you infused them and they went all around the body, but you knew they were effective, if you deliver them right through an aerosol to the lungs, you don't have the toxicities, but you do have the effectiveness.

Peter Anderson:

Yes, and that's our goal is to develop drugs like that, and I think it will be a slow patient process, clinical trials always are, but there's nothing to indicate so far that we can't succeed with this in the long run.

Andrew Schorr:

Okay, if people want to get in touch with you or your department, should they just go on the M.D. Anderson web site (mdanderson.org) and go to the Children's Cancer Hospital area, and there will be information about this, or what would you recommend?

Peter Anderson:

That's an excellent way to do it. The pediatric access office information is there, and they often are able to provide information to patients and families about M.D. Anderson, how to obtain services here. The other way I think is sometimes having your doctor call too, so either one works. Certainly the way I see it is patients are becoming much more sophisticated, and often they will provide information to other patients and families, and there's just too much information for one person to know, myself included. So often trying to get a specialist who has a special, very specific interest in the disease that's being treated is the way to go, and M.D. Anderson is the model for that.

Andrew Schorr:

Yes, I would agree. I mean, I'm from Seattle, but I was treated for leukemia, chronic lymphocytic leukemia, at M.D. Anderson because that's where there were specialists, Dr. Keating and others, in my disease, and there's the match. Jennifer, you were fortunate. You just go across town where you have Dr. Anderson and others, and they're specialists in what you have. M.D. Anderson being as large as it is and is preeminent has subspecialists in so many cancers, so I always urge people; I say check whether M.D. Anderson has somebody who researches and treats what you have and whether they have clinical trials.

Dr. Peter Anderson, Professor of Pediatrics at the Children's Cancer Hospital at M.D. Anderson, I want to thank you for joining us, and then we're going to give the last word to Jennifer, but thank you for being her doc and the doctor for so many and advancing your research. We wish you all the best with that, sir.

Peter Anderson:

A pleasure, thank you.

Andrew Schorr:

And Jennifer, so you've gone, I'm sure your emotions have gone up-down, left-right through all this.

Jennifer:

Oh you have no idea.

Andrew Schorr:

So, there's a 19-year-old or younger listening somewhere, and they're told that what maybe they didn't think was serious turns out to be serious, and it's a cancer, and some of the things that you've been through lie ahead. This is your chance. What do you want to say to them?

Jennifer:

I would just say to not give up because, yes, when first get it, it blows your mind, like you think, I don't have cancer. You don't want to think that, but then it sets in, and that's when you get in your bad phases, and you're like, why me, but for me personally, it's making me a whole lot stronger. I have the best family and friends to support me. Just don't give up because once you get done with it you can tell people, dude I survived cancer. Once you get done with cancer, I mean, there's not much else that you can't get through.

Andrew Schorr:

Yes, you're going to climb Mount Everest or something like that.

Jennifer:

I know!

Andrew Schorr:

And I'm sure you'll always have a close connection with M.D. Anderson. They've been pretty good for you, haven't they?

Jennifer:

Yes, they have.

Andrew Schorr:

Yes. Jennifer, thank you for participating just a couple of days after surgery there and still in this hospital, and you've got a great mom, Mary, who really looked out for you, didn't she?

Jennifer:

Yes, she did.

Andrew Schorr:

Yes, well thank her for us too, and shake Dr. Anderson's hand for me. I want to thank you both for being with us. This is what we do on Patient Power every two weeks. Check out the mdanderson.org/patientpower web site. There are many other programs we've done in the past, all the transcripts and all the audio replays are there for you, and we've got a lot more coming, and we always invite your suggestions.

In two weeks, we're going to do a program with Dr. Karen Lu on genetics in cancer, and so everybody wonders, am I genetically set up where I'm at risk for a cancer and also what about others in my family? So, that's always a concern. We'll hear about that in two weeks, October 16th.



Thank you so much for being with us. I'm Andrew Schorr. As always, knowledge can be the best medicine of all. You've been listening to Patient Power brought to you by M.D. Anderson Cancer Center.

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