



## A Patient's Progress- Access, Innovation and a Future of Reform

### EVENT TRANSCRIPT

Jonathan Wilcox: You'll have to forgive me, but I've always wanted to say this, "I'm a man who needs no formal introduction." My name is Jonathan Wilcox, I am the policy director and the co-founder of Patients Rising. Good morning. You are looking live at the music box in San Diego, California, which calls itself, grandly, "America's Finest City" and it's here in December, it's 70 degrees outside, it's a cloudless sky. I was looking at the bay and the brilliant blue water and I had to wonder the timeless questions of, "Why would anybody live here? Why would anybody ever want to live here?"

We are going to make some beautiful tunes and harmony here at the Music Box today. It's thanks to because of our sponsors, [00:00:54], Russel Myers Squibb, Celgene, Genentech, and Women's Magazine. Now as we begin, a few words from a few friends.

What was missing from that video? What was missing, what was missing? What does anybody say?

Speaker 2: The audio.

Jonathan Wilcox: The audio? Well, it had sound. I heard sound, so there was sound.

Speaker 3: The voice, it was voiceless.

Jonathan Wilcox: The voice, it was voiceless. It had sound, it had sight, and it had sense, but as you can see, or almost here, without the patient voice, we have almost nothing. We lose almost everything. We've founded Patients Rising for that purpose. Really, for four reasons: To stand for patients, to advocate for their needs, to fight for their access to the medication they need and deserve, and to tell the truth about healthcare. One truth I want to share quickly, on a personal note, is there is a certain completeness to this day because in many ways, Patients Rising was born of ash.

In San Francisco in 2014... Orlando was great last year too, but in San Francisco in 2014, we're preparing what we thought would be our last panel discussion. A totally different place with a previous team. It centered upon what was then called, very quaintly, "The Value Versus Cost Discussion." Preparing the preliminary ways we were going to talk about this, we heard from the perspective panel. They are good people and how they wanted to proceed and they said, "Price controls, frameworks, half ways, institutional control." Anything missing from that? Innovation, not there. Regulatory form, another time. Patients at the forefront, well, once we manage costs. It didn't feel right. It didn't feel right and we asked, "Is there anyone one else out there?"

Since miracles do happen, we were introduced to an entirely new world of people and professionals and allies and ideas and ways to fight. We signed up. It was a world of new and challenging policies and the limitless possibilities of medical innovation. Putting patients and the center, the forefront, and always first. When a crossroads path was presented to us, like the two roads that diverged in, 'Frost Yellow Wood,' my favorite poem, "We took the one less traveled by" and that has made all the difference to us, it's because of you. Thank you, thank you, thank you, thank you, thank you.

I want to introduce a video of a extraordinary man who's not here today. His name is Bob Tufts, he is known to rave sports fans. He's one of the... He's a major league baseball pitcher, but he throws gas now and hard ones. I can't think of a man who's fighting more for patients in the terms of living and embodying the words, "My life if worth it." Please listen to a few words from our friend, Bob Tufts.

Bob Tufts:

(Video) When I get diagnosed, can I got back to working on Wall Street again? Probably not. I had to come out of cancer and create my own third career. I thank Yeshiva for now employing me full time after all that's buried. I love this. The way I teach. I interact with my students. They like me, I like them. I look forward to doing this for another 10 or 15 years. I've never been sick a day in my life. I played major league baseball, I had worked on Wall Street, I hadn't missed a single day. Then all of the sudden, I had started getting ill and I saw a local Oncologist who gave me the dreaded words, "You have cancer, multiple myeloma." I wasn't really scared, it was just a shock to get it.

Having worked in baseball and sports information and wall street, I was interested in the numbers. My cases said, "Well, your numbers are rather aggressive and going up. If the first treatment we give you doesn't work, you're going to be in trouble." Fortunately, the medication they gave me, which was a novel therapy, did work. Within a month and a half, I was in normal bands for proteins, hemoglobin, and everything. I'm very grateful that medicine came on. In the process I've been able to teach, I've probably instructed close to 1500 students. I've seen so much value come from medication through me and go back to society. These kids, many are very good Stem students, many are good

IT students. They are going to be making a difference someday somehow. I'm glad that I'm able to push them just a few millimeters down the road.

Jonathan Wilcox: I mentioned before that in a lot of ways, we were born ash and we have so much connection to the Hematology space. One of the reasons is, with all patients, but one of the reasons is because I've met so many extraordinary people attached to extraordinary treatments and extraordinary advances because for the disease in multiple myeloma, there are extraordinary treatments and far sided miracles that are happening, but there is to day, no cure. These patients have a special challenge and a special awareness. I want to bring one of them today. He is a clinical professor at Meritus, University of Missouri, Kansas City School of Dentistry. An extraordinary voice of value, please welcome Dr. John Killam.

Have a seat doctor.

Dr. John : Good Morning Jonathan.

Jonathan Wilcox: When I first spoke to you... You live in Kansas City. I think you had just come back from a support group and advocacy forum. You came to St. Louis and you did the same. You brought the powerful word the other day I talked to you. My gosh, you are doing one in Orange County, it's a few miles up the road from here. One thing you said to me the first time I met you is, "That patients are connected in that way." Multiple myeloma patients, as I just said especially, we talked about it and called it the "Golden Thread." These patients are connected in ways that are, that I'm only now learning about, but you've made so tangible.

Dr. John : I want to refer back as I start, to the video that was just shown. The gentleman who was a baseball player and who is a colleague professor. I spent the last 25 years of my professional career at the University of Missouri Kansas City School of Dentistry as a college professor. The things he said about talking to the students, motivating the students, giving them that little light or that little push that helps them move forward, is really central to my thoughts about what I do as a myeloma patient. You mention that I have an opportunity to speak with people at support groups. I have the opportunity to mentor patients on the phone. I have the opportunity to speak to people personally.

Throughout my entire myeloma journey, I have felt there are some down times, there are some times that we spend in that tunnel of treatment that are so intense that you just don't see the future on that day, but the really important word is to know that once you pass through that tunnel, there's light, there's hope, there's brightness. I have had more incredible opportunities to interact with people as patients and to interact with people as professionals. I've had the opportunity to know you as a result of having myeloma. If I didn't have this disease, I would have gone ahead. I worked full time throughout the time of the day of diagnosis until the time I retired on my 70th birthday in 2012.

Throughout that time, my life became richer because of the people that I had knew and got to know through myeloma.

Jonathan Wilcox: I remember you use the words the first time I met you, you said, "You were grateful to be a patient in that way." You had gratitude for things that you had learned and experienced. I thought that, I've heard that more and more, but in your way, you're sharing that with patients and bringing the gratitude out of them.

Dr. John : Yes, I really do feel, today, I feel grateful. I will admit, there were days I didn't feel grateful. I felt that my life was out of control. My life had taken a downturn, but because of my family and because of the support of the people that I worked with, I moved beyond that time frame. Just last week as we were preparing for Thanksgiving and I was thinking about my kids coming home for Thanksgiving, I realized that it was exactly 9 years ago, the day before Thanksgiving that I shared with my family that I had multiple myeloma. This Thanksgiving was a great anniversary for us and a time to celebrate.

You have to take it beyond your family. You have to realize that there are people out there who don't have the family support that I have that maybe don't have the access to the care that I got. My university insurance was incredible and I do have to thank the university and the support of my university. Getting that care opportunity out to other people, getting the message out to other people, that depending on your socio economic background, there are ways that we can help you through this disease. I can assure you personally that the outcome of the process is very positive. It's been fun the last few years.

Jonathan Wilcox: One thing that we talked about when we were joined in St. Louis was that, for the first time we got in depth about the question of what I describe as, value frameworks and these are common in the industry conversation. You broke it down in a way, you just said, "Access." You've brought such a... You've always said, "A human touch and the patient as a human being, as a personal entity." To lose side of that, even in any other issue form, even in any other discussion was something that it was to lose almost everything. When you said when you talked to patients and you talked about value frameworks, you said that it had essentially forgotten some part of the humanity and personality and in that ways, it had to be changed.

Dr. John : Thank you for listening. Thank you for reminding me of some of my thoughts. Truly, in my mind, I remember our discussion that day. Having the opportunity to work with the care providers, for me to work with my doctors and the people who are providing the medical care for me and having that personal interaction with those doctors. I have an incredible team and they know me well and I know them well. I'm a personality where I reach out to them in many different directions. Having that interaction with those people, I think is not only helped

me, but I know it's helped other members of our support group who are treated by that same treatment facility.

The doctors have become overtime that I have been involved with this, they have even stepped out of their treatment facility and come to our support groups and begun to interact with us a little bit socially. I realize you have to have a medical and patient relationship, but it's grown just beyond walking into a clinic and having an appointment. I've really become involved with their work and they're becoming involved in ours. I think that's what's important for a lot of patients. You have to maintain that ability to interact with your doctors and not have a lot of outside influences that may influence the treatment modalities that are presented to you as a patient.

Jonathan Wilcox: One thing also, just on one of my notes here, is when you said you talked to patients, and you described, you never stopped working. That's a tribute to your spirit and your stamina and what you bring to it. I was blown away. You said also, you were able to keep working too. In talking about it, you said that the therapies and innovation had come to a place where you could be fully treated as a lifetime patient, but live your life and work professional. When you brought that message to the support groups, some people may have not been aware of that. You said, "That way you open the door." When you say that, you see people responding, what does it mean to you?

Dr. John : The timeframe that I think about that in response to your question is, soon after my first transplant, I had set the goal in my mind that I was going to be at a national meeting in Washington DC on the 60th day post transplant. The tape worked and we got me there. Soon after my transplant, I started having my wife drive me to work a couple hours each day. I was working at a healthcare facility, a dental school, where we treat a lot of patients every day. Fortunately for me, my office was a little bit secluded and I put a sign on my front door that said, "Virtual hugs and kisses are great, but I can't touch you." Every one of my friends would stop by daily and wave and as I said, I worked from the day I was diagnosed off for a few weeks after my first transplant, four years of medication supportive remission. Second transplant, I didn't go back to work after the second transplant because I retired my transplant. My transplant was in October and I retired in December.

As I said, my university and my professional support family were there to help me make it through that entire time. People get down, but you get up every morning and you go and you get out there and follow the responsibilities. Just like the man said in the video, he's had more opportunities to interact with students and help people following his myeloma diagnosis. Similar for me, I use to teach, I use to interact with students. I still go to work one day a week, but the rest of the time, I'm out interacting with patients and hopefully making a difference in their outlet for the future.

Jonathan Wilcox: Let me ask you this, when I talked to you, you were going to Orange County, which is 80 miles from here to see a support group. I know that it's a beautiful thing that you do, but why do you do it? Why go there from here to open that door, walk into that room and share your experience?

Dr. John : Selfishly, I get a lot back. I get a lot of positive energy that comes back to me, but I think that's the least important of the reasons. It's my opportunity to assure patients that throughout their treatment sequence, that there is that light at the end of the tunnel. There is a future. Give them knowledge because we all know that knowledge is very empowering. I have a little bit of knowledge to share that a lot of them don't know or a lot of them do know. I think it's also important to give them the fact that there is that bright future out there and that we continue to move forward. We get up everyday and we go out and we have a great time.

Jonathan Wilcox: Moving forward, end every day. We are grateful that you are here in San Diego, but what brought you here to San Diego?

Dr. John : The first reason I came was for the ash meeting. I had the opportunity to participate as a patient at the ash meeting.

Jonathan Wilcox: Fortunate as well.

Dr. John : It's again, even at the ash meeting, I'm meeting people, I'm talking with people, I'm learning things and I'm gaining information that I can take back and share with others. Just as I did at the support group in Santa Ana. An interesting thing that happened there is I had been phone mentoring a lady who lives in San Juan Capistrano and I had not met her personally. I had invited her to come up to the support group. Actually, she had joined the support group a month earlier. She came and it was just, now I know the person. She reacted, "The reason I'm here and sitting in the front row is because now I want to know you as a person." It really is making, I continue to make personal ties with people with this disease.

I have two families, my blood family and I have my myeloma family. I guess I have a third family, my friend family or my heart family. I definitely feel my interaction with the myeloma patients that I know, that's a third family that I carry with me every day of my life. I gain strength from them and I hope I can give back to them.

Jonathan Wilcox: An extraordinary voice of value. Everybody please thank Dr. John Killam. Thank you, John.

Dr. John : Thank you.

Jonathan Wilcox: Dr. Killam represents the heartfelt sharing and the connection and the personal touch that I have personally, I would not have believed, but I have seen personally touch people in ways that I thought were unimaginable. Along that

journey of people who need other support and other issues are the ones who are facing barriers. Not just of their own health, but other challenges and other ways. They need a golden thread too. I want to bring forward then a great friend and a great partner. From the need alliance, the alliance for the adoption of innovation of medicine, the legal, Stacy Worthy. This is a person you don't want to mess with too much, I just want to let you know.

Stacy, the first time I met you, you were preparing to take on something that I hadn't heard about before. It was called, "Non Medical Switching." Non medical switching, as an example of the things that happen that I hadn't heard about, that patients hadn't heard about, some who even had it happen to them. This to me, catalyzed in my own sense, these barriers are real. These patients experiences that are trouble, are real. Tell me about it.

Stacy Worthy: Non-medical switching refers to a practice of insurers when you have a stable patient who has over the years, tried several different medications that didn't work. Finally, they have one that works for them. They don't have as much side affects and they are doing well and then after they've signed up for the health insurance plan, the insurer essentially forces them to switch medications, to a different medication. By either dropping their current medication from the coverage all together or by making out of pocket costs so expensive that they can no longer afford their medication.

Jonathan Wilcox: Let me stop you. You just said, you're on a treatment path, you're receiving your medications. It's change, switched to something else. Tuesday this, Wednesday, another, no notice, no phone call, text, email, Instagram, Pinterest, it's not there. You're saying this is happening?

Stacy Worthy: Yes. It's a bait and switch tactic essentially. A lot of times, the physician isn't even informed, so they don't even know their patient is forced to take a different medication. We are not talking about switching from a brand to generic. It's an entirely different treatment. This can violate several consumer protection laws currently in place, a contract law. You're on a plan and then your medication is changed, so that's a violation of the contract. We are seeing it take place now.

Jonathan Wilcox: When I got my car to come down here, I tell you, I signed up for the full size and I got a small SUV. That was a switch I could live with even though they didn't tell me. You're saying, two points then, you mentioned the legal aspects and responsibility aspects, but you're saying these patients, besides having their rights violated, they're having their treatment path violated. They suffered adverse health effects?

Stacy Worthy: Yes. This is a thing that we want patients to speak up against because it's an interference with the physician patient relationship. It's taking their choice away. We've created a website called, [Coveragerights.org](http://Coveragerights.org), which you have pamphlets available to you which informs patients what they can do if they are

switched. If they go through other policies that other insurers are imposing such as step therapy, which requires you to try and fill on a cheaper medication sometimes inferior with adverse events before they can take the medication that was prescribed to them. This website will inform you how to appeal. If you don't win the appeal, then how you can request a independent reviewer or review organization to have a second opinion. Then file a complaint with your insurance commissioner if that doesn't work.

Jonathan Wilcox: What I want to ask you then, is you mentioned step therapy, which patients tell me they call, "Fail first," in the sense of you... A doctor may conclude, he or she may conclude, that their patient needs this best medication for treatment for toxicity in light. Administratively, they are told, "Well, try something else." Not in the opinion that it will not succeed as well, but you try it and if it doesn't work, IE, you get maybe sicker, then maybe try the thing that was recommended. That practically just calls step therapy, or a darkly fail first by the patient community is one that has been in states, prohibited by I think 40 states. Different states at a time when maybe the country seems very divided. Can the same thing happen for non-medical switching?

Stacy Worthy: Yeah. It's not necessarily prohibited, but there are restrictions in place so that it's not implied in a discriminatory manor. Currently, there are some consumer protection laws, but they don't go far enough. If you have a plan and maybe you enroll in a new plan, there's no guarantee that when you sign up for the new plan, you're still going to have coverage of that medication. It is very important for states to enact those laws. We are starting to see those laws be introduced, but they haven't passed yet, really strong language. One of the ways we are trying to influence state lawmakers and legislators is through a survey that we are conducting.

Jonathan Wilcox: Well, tell me all about it.

Stacy Worthy: Sure. The survey, which will come out in 2 weeks, we've interviewed and pulled about 1,000 different people throughout the country. We've excluded individuals who are in the health industry, media, or marketing so it doesn't skewer results.

Jonathan Wilcox: A thousand individuals have been interviewed?

Stacy Worthy: Yes.

Jonathan Wilcox: The survey asks them a series of questions?

Stacy Worthy: It does. It gets to how to value their medications and their healthcare. It goes over a couple different buckets of questions including, what their expectations are when they sign up for healthcare. Do they believe that their particular condition is going to be covered? Do they believe that their medication is going to be covered? Then we go into accessibility. If you have a plan, do you know

where you can access that plan? Do you know where to go online? Once you have access, do you understand what it's trying to tell you. Do you understand of certain barriers that are in place? Then from there, it goes into, how do you face those barriers? A lot of these barriers can be unethical and as I said, in some instances, illegal. We want to get at how often it's occurring. We can use that data to inform policy makers so they can either enforce laws currently in place, or they can enact new laws such as the non-medical switching laws to increase protections.

The last part of it goes into how they personally value their medications. We've asked questions such as, "Would you be okay switching from your current medication to a cheaper medication to save money even though you could have potential adverse events?" Also, "What's more important to you? Saving up money for your mobile data plan, or to save for your co-pays?" Things like that. We anticipate that we will get a lot of very mixed results, but we intend to show with those results that life is full of trade offs and that patients should be the ones who are making those decisions about what they want to pay for. Whether it be that novel medication that saves their lives or something else, but an insurer or a institution, shouldn't be making that decision for them.

Jonathan Wilcox: Can I say about the survey... You mentioned of course, that we will receive data information from the patient, but at the same time, you've described that it will actually inform as well as receive, in a sense in discussing these aspects with the patients of population in the survey, that at the same time, you found out what they feel, what they believe and what they want, but you said how much they don't know. I'm interested in that.

Stacy Worthy: A lot of patients who get put through these processes, such as step therapy, non-medical switching, they don't really know it's even occurring to them. What we are trying to do here is just raise awareness, so once they understand what's going on, then they can mobilize, they can speak up, they can talk to their doctors, ask for help and really be their own advocate. They can find their voice, so to speak.

Jonathan Wilcox: Last point, be your own best advocate. You work on... This data you said... When we advocate, you said patients believe that patients should want this, people actually said to us then, "How do you know? Prove it." You're saying this survey is going to be, you will take that in your hand and you will say, "This will prove it."

Stacy Worthy: Yeah, absolutely. We are hoping that we will be able to go to state lawmakers and say, "This is what's happening and you need to do something about it."

Jonathan Wilcox: Finally, a question for you, what does a future of reform look like?

Stacy Worthy: A future of reform I would say, is one where there is very strong patient protections in place where insurers can't use these discriminatory, unethical

measures to put their costs savings ahead of the patients voice and the patients treatment where they are interfering with that physician patient relationship. That patients are informed enough to take an active role in their healthcare.

- Jonathan Wilcox: One of my very favorite voices of value, please welcome and thank Stacy Worthy.
- Bob Tufts: (Video) The only way we are going to get out of this problem is through innovation. We need to basically adapt, go forward, and basically take today's patient who lives only 1 to 3 years longer who like my case, myeloma, the amazing improvement. The average patient with diagnosis lives 7 to 10 years longer. That's only in a ten year period. We need to do that with every single disease and we will be able to someday.
- Joanne Smith: (Video) Because of the medication I'm just able to be a healthier, happier, young Grandma. I'm able to do the things I enjoy doing, gardening and photography and taking hikes and being with the grandchild and doing day trips and things my quality of life is so much better. I'm happier.
- Bob Tufts: (Video) Every day we go from a lung cancer patient being a 1 to 3 month live to a 1 to 3 year live, to a 10 year live, to a cure.
- Jeff Julian: (Video) What immunotherapy tries to do is varnish your body's strength and your body's immune system. That type of approach of using your body's immune system to fight it, is genius. It is an incredible feeling to know that it worked that well.
- Bob Tufts: (Video) One novel therapy leads to another novel therapy, which will lead to another. I've seen it in my home of 1 to 3 years, to 3 to 5, and now it's 7 to 10. Five years survivorship has rocketed my myeloma. I want every disease to be able to experience that same thing. Through innovation and if we don't destroy innovation in the process here, we will get there.
- Jonathan Wilcox: We call that video, "Innovation." One of the practitioners of it, I think it will explain, that's what we are seeing, he is the get family professor of cancer and chair of the department of medicine, distinguished male investigator with the mayo clinic, please welcome Dr. Rafael Garcia.
- Dr. Rafael: Thank you, thank you.
- Jonathan Wilcox: After I saw you on panels and as a writer and a commentator, but I guess you're also positioned as well. One of the things that came through in your writing, it was so extraordinary that I committed to memory and then I thought I would forget it, so I wrote it down. You said that, "Patients and patient organizations should be wary of the consequence of inappropriate use of value frameworks. Everyone should be asking for value in healthcare. This value can only be uncovered by asking the right questions. Those interested in the well being and

betterment of patients, should be hopeful as well as skeptical." What did you mean by that?

Dr. Rafael: I think the word, 'value,' is a little bit of a euphemism in certain circumstances to talk about withholding care. We all can agree on value. Value is what we do in life in every realm of human activity. Value is currently being used in medicine, sometimes with an aspiration. Sometimes that aspiration is not well intended. Sometimes it's restricting dollars for a payer. Sometimes it's an aspiration that's misunderstood by colleagues who think we spend too much on certain things. We have to look at value for what value is. When we think about value, we think about costs, we think about gains. We think about gains not only in life, but in people returning to a economical activity in society. I'm very, very concerned when the word, 'value' is being used right now, particularly, 'value frameworks,' that it's really a euphemism for rationing. That's absolutely terrible. I'm completely opposed to that.

Jonathan Wilcox: Let me ask you, how did the word, 'value,' which you said is a human value, and all that that entails, you say has become a tool of restriction and a understanding of how to dehumanize individuals and get more in economics?

Dr. Rafael: I think it stems from this triangle that has been thrown into medicine where physicians should be responding to patients. That is a fiduciary responsibility for a physician. Somewhere along the line, someone inserted a third point, which is society. There should physicians and policy makers that look at society. When physicians, practitioners, clinicians, are sitting in front of a patient, their only objective is that patient. That's the only thing that should matter to them.

Let me give you an example. At the American Society of Clinical Oncology a couple of years ago, they presented a study of Melanoma. One of the most important studies in cancer. It was in the [inaudible 00:35:05]. The commentary that fold, you would imagine was somewhat celebratory that now more Melanoma patients were going to live longer. The comment was focused on the expense that those medications would incur for the treatment of melanoma. That would have been fair, if that comment came together with value, but no, it was exclusively about costs. That's why, one of my blind spots, I'm not an economists that lives on economic terms and descriptions to folks like, Dr. Goldberg, but as a physician, I see a complete breakage of that fiduciary responsibility that physicians should have for their patients.

Jonathan Wilcox: You're treating patients, you're seeing them, your hands are in their hands, their family in yours, and yet you say a line has been put between it and broken. How do we return to the purity of your first practice and why you got into medicine? Celebrate that and accept it and make it stronger.

Dr. Rafael: We as a profession, have to strive to do that better. Again, I think there are reasons to be concerned. The world, there is scarcity that is surrounding us and there are limited resources. Within the realms of what we can use, I think we as

a profession need to remain focused on doing the best for our patients and not bring those third party considerations from whatever perspective they come into that decision making process. Ms. Stacy made a great example. People will say, "Well maybe you should try this first." I will give you an example in Myeloma, we moved from a medication called, Thalidomide, which by the way is still used around the world and is an affective medicine in countries that don't have the resources to do that. It's nowhere close to what we use now which is, Lenalidomide, which is really a standard of care for patients. People might start using sometimes poisonous language, like, "Well, it's not really that different. We haven't seen the overall survival in this trial." I know if I'm a patient, I know very well which one I want to get.

Jonathan Wilcox: Getting back into clinical judgement, I saw you at a conference in the Economist, you were saying, "It's time to return to putting the patient at the forefront and maintaining the doctor patient relationship to where the doctor can really prescribe in a responsible way given medical knowledge of the treatment." I saw some people kind of shake their heads like they never heard it put that way. I'm interested to see as you advocate, here at Ash and other places, are you winning the fight to get people to put the doctor patient relationship at sacrosanct, or do you think that it's going the other way?

Dr. Rafael: Hematologist are pathologic optimists.

Jonathan Wilcox: Hematologists are pathologic optimists.

Dr. Rafael: We have to hope for a better future.

Jonathan Wilcox: I knew there was a reason why we liked you.

Dr. Rafael: We like it. We've been sort of flirting for many, many decades with cures and better treatments for the disease. I think many of my colleagues aspire for a better future. Without contacts and self describing myself as an optimist, I think we are starting to make a dent against the pendulum swinging towards this way where societal considerations would appear to prevail and be more important to the individual considerations for the patient. It is an effort that will take may to speak up and having forms like this as well as publications, but we still have a lot of work to do of course.

Jonathan Wilcox: You also said at the Economist event and I wrote it down, you said, "Misunderstandings of the context of value are used to negate so much of the progress that has occurred only recently in the treatment of Myeloma." What you're saying, in the times that you've been practicing, you have seen an extraordinary innovation progress and patients saved in a way that sometimes we don't hear much about.

Dr. Rafael: Without a doubt. In fact, at this meeting, we are presenting a poster where we will look at survival from Myeloma patients over the last several years. You see

the survival perks coming like that. The survival is clearly there. I think if some of the frameworks are put into place, I think we are putting a hand brake on the speed to that curb and how that curb is rising. I have seen tremendous gain. It's not unusual for me now to see patients who are out 10 years from their diagnosis, not everyone, but 10 years from their diagnosis, leading a clear, a good life. Sometimes close to a normal life. I think that's incredibly gratifying. There's a saying Spanish that says, "So much swimming to drown right at the edge." I think we are right at the edge with Myeloma. I think we are right at the edge with many other cancers and our progress will continue to come. We can't just sit down and pretend that what we have today in 2016 is the best there is. Today's best is still not good enough. We still need to advance medical research, innovation, etc.

Jonathan Wilcox: That's being the incurable optimists. Let me ask you one other thing, when you've said, and I do think people respond to this when you hear about it more, you talked about the patient experience when you began and what it was like with patients then and they didn't live as long. Now you've seen that change. My first point is, are you a different practitioner from the time that you were treating patients and they weren't living as long to the time you've seen this progress and make it you even more of an optimists? How did you keep the optimism and what drives you now?

Dr. Rafael: That's for sure. When I started in Myeloma, that was close to 20 years ago, you were the last in line to get picked up. Everyone wanted to look at Lymphoma, Leukemia, those were really exciting areas. Myeloma was [inaudible 00:40:23] so there was a lot of handholding. I was incredibly fortunate to have the right mentors and obviously without foresight of what was to come, to have participated in this field. The ability that we have, 11 new drugs approved by the FDA for the treatment of the disease. The ability to reduce to complete remissions, we look at life expectancies that exceed sometimes 10 years. Of course, that makes you more of an optimists. That makes your visits more rewarding. There's a whole series of things that happened with this, so we have patients that come to us for seventh line therapy. They have received seventh line therapy. Interestingly, it's one of the past things is they come up to you and say, "Well, what's next? Something else is going to work." They have been accustomed to a series of treatments that can't control the disease.

Jonathan Wilcox: I want to go back to the point, I just want to reiterate, is when you began, Myeloma was a field in which was very attractive to some people starting out. You said you had the right mentors. My point is, you're saying that the treatments weren't there, you weren't seeing a lot of affective treatments and patients were in a way, not being treated as effectively. You went into it and stuck with it. I always thought that was one of the most extraordinary stories. Starting your career and being in medicine, it had to be hard. You said you had the right mentors to inspire you to take this leap in a field that was not very developed in a time when the patient reward was not much there.

Dr. Rafael: Absolutely. I think I could use other examples for hematology colleagues in the audience, the only thing we did was hand holding. This is a condition related to the Myeloma. We used the same treatments. I remember just essentially supporting people as they went down a path that ultimately lead to their demise. Now, with this modern treatment, it's not unusual that we have very, very long term survivors with that. You're mindset changes in such a way. At the end of the day, I think we are as compassionate, we are as careful, hopefully as educated as we were back then, but what we have are tools. Which is now what makes a difference in how approach as patients.

Jonathan Wilcox: One more big question. You have, your practice has lived to see patients, patients have lived to see, you're an incurable optimists, let me draw upon that. What does a future of reform look like to you? Practitionally and personally.

Dr. Rafael: Well, I hope the reform of the future recognizes that we have a tremendous potential. I just had the opportunity to give a talk down in Mexico, sort of like a tech talk forum that they have and the title was, "We can cure cancer if we allow ourselves." I don't pretend that that's now or maybe even 5 years. I'm sure 10 to 15 years we are going to see big changes. Maybe in 30 years we are going to see dramatic changes. To that affect, I did so a picture of our cancer center. I said, "This is a beautiful building, but I actually hate it because I can't imagine people looking 50 years from now looking back and saying, "Can you imagine these guys in 2016. They had cancer hospitals. Is that possible? They have tuberculosis hospitals."" Fifty years ago, that was the norm and we potentially could be looking at that.

That can only be achieved with the right reform. Reform that recognizes the need for patients for access. There is a disparity that the United States is a cauldron of innovation. We pay a higher price, but guess what? We also have early access. I think whatever we are doing right now will be the legacy for the treatment for the future. I hope that reform is respectful of that and supports innovation in the United States.

Jonathan Wilcox: I heard that we will cure cancer if we allow ourselves.

Dr. Rafael: That was the title of the talk, yes.

Jonathan Wilcox: I hope, pray, that we can allow you and others. Please thank Dr. Rafael Garcia.

Dr. Rafael: Thank you, thank you very much. I appreciate it.

Bob Tufts: (Video) Where we are unfortunately is in the age of value being determined by people who don't see the patient. One of the groups I serve, which claims it's independent, which is funded by people who are insurance company based, they use quality adjusted life here. Big data unfortunately, turns people into little inhuman dots on a chart.

George Ackers: (Video) Are you a human being or are you a number on a paper? The bottom line, give the people the drugs that they need.

Eileen Brewer: (Video) My doctor is willing to have a conversation with me and try any of the options that are available. She is a great doctor and my insurance company says, "No." I don't make enough money to afford it on my own.

David Kottler: (Video) Somewhere down the line, there is somebody that is able to diminish my quality of life. It shouldn't be.

Eileen Brewer: (Video) To me, it's really frustrating and unacceptable.

George Ackers: (Video) Give me the drug that's there, that's going to work and we know it's going to work.

Bob Tufts: (Video) I just want the care to be delivered by a doctor, period. With no interference, no one coming in between.

George Ackers: Let's worry more about people instead of a bottom line.

Bob Tufts: If the patients and doctors have access and choice in care, we will get better medication, we will get results.

Jonathan Wilcox: These are the access barriers that we talked about. These barriers are real, the experiences are real. One of the people I think, who is making the case, other than from the economic standpoint, the co-founder of the Center of Medicine in the Public Interest, the most dangerous place to be is between him and the patient interest, please welcome Dr. Robert Goldberg. Cleared out of the way for you.

Dr. Goldberg: Pretend that I'm Jimmy Kimmel, okay?

Jonathan Wilcox: Ladies and gentleman, Jimmy Kimmel.

Dr. Goldberg: Thank you, thank you. Thank you Jonathan.

Jonathan Wilcox: Wait until you see how we wrap it up.

Dr. Goldberg: I have to say though, of all the introductions that I've had, this is the most recent.

Jonathan Wilcox: The most recent, yes. One of the things that come to me, when we met, you talked about as a healthcare economists, that can be considered a dry field. You told me and I observed, you've infused different issues. Hope, dignity, morality, to the cause into the science. How have you done it?

Dr. Goldberg: Well, healthcare economists are, it's a group of under achievers. I will tell you why. If you look at the value frameworks, the premises is a false premise. A premise is that medicines are too expensive, cancer drugs particularly are too expensive and they are going to blow up the economy. No healthcare economists, who call themselves an economists, would look at that statement and say, "That's true." It's just the opposite. It's because of the use of new medicines, that people are living longer, producing more, spending less time in the hospital.

I will give you an example of a multiple myeloma and leukemia. If we were treating those blood disorders with the same treatments we had in the 1990's, we would be spending 2 billion dollars more a year on treatment and the number of people dying in the hospital would increase by 200%. Why hasn't any healthcare economists, with the exception of me and a couple others stood up and said that. It's because the profession, so to speak, is geared towards looking at what's valuable to the payers and the system and not the interaction of innovation in economic growth and well being.

Let me make a final point which is, you talk about economics, Evan Smith, Wealth of Nations, talks about the role of capitalists and producing well being for everybody. Democratizing well being. That someone can live as well as the king of England through the use of the markets. Medicine does that better than any other enterprise in human history. Instead, we are set with a group that says, "We should ration excess to save money." I'm sure you will ask me some questions about that and I'm ready to answer.

Jonathan Wilcox: I love a good lead in from my guest. What you said to me, you said, "So many have failed the systematically explained. The social and economic and medical consequences of these policies of this rationing of this..." What are the consequences?

Dr. Goldberg: Does anyone know who ICER is? Raise your hands. Oh, that's pretty good. ICER is a group, it's a private group, funded by the insurance companies and the PBM's, they say they are the authoritative independent voice of what is defining the value of a drug and how much the health system can afford. The way they define it is as follows. Let's take myeloma for instance. They said that the new drugs on the market are not cost effective because they don't meet, they cost more than \$150,000 a year to keep someone alive. They look at and then they devalue... They take a life and they say, "It's only worth half of a healthy individual." They are discriminating against poor people-

Jonathan Wilcox: No, stop, stop. Stop right there. Worth half of healthy people?

Dr. Goldberg: In other words a quality, one is you're really, really healthy, and zero, obviously you're dead. They say that people with cancer, with myeloma and leukemia, their quality is about five or six, which means that, you can play the game and say, "Well, it's going to cost more than \$150,000 to keep somebody healthy who

is really sick." That is discrimination right there. Here's the other point, even when ICER said they wouldn't support step therapy, that was a shiny object.

Jonathan Wilcox: You don't seem satisfied.

Dr. Goldberg: I'm not satisfied because if you look into the report, they say that even at the discounted prices, which, by the way, the discounts, who do the discounts go to ladies and gentleman? Do they go to the patients? No. Those 40% discounts or rebates go to the insurance companies who are funding ICER. No mention of that. What happens to the patients is that even at those discounted prices, ICER says that only 28% of myeloma patients should receive these new drugs in second and third line settings. I did a little calculation, and in over five years, that would mean myeloma patients, there would be 220,000 myeloma patients who would be dead as a result of that. Over five years. Leukemia is the same thing. Lung cancer, there would be a 500,000 life years lost. As a result of rationing. That's never mentioned. I ask you and I ask them, is that value? Who's value is that? Is that the purpose of medicine? To cut costs and cut corners so that insurance companies doesn't go up against an artificial barrier on how much to spend on medicines.

Then you flip it around. Then you see what my friend, Bob Tufts is doing with his life. You see what Don Wright has been able to do. To see his grandchildren. That's what I mean about hope and dignity is that they are the ultimate product of innovation. If you don't have a future, you don't invest. If we don't have hope, then there is very little that human society begins to fall apart. We revert back to the pre-enlightenment days where our life was short, Brutus and nasty, and dependent upon the compassion of the king or the ed ix of the church.

Jonathan Wilcox: You're talking about infusing then and investing new concepts into consideration economically as well as socially. You said, "A long term measure of value that takes into account, individual differences and needs in a response and out comes that matter like productivity, quality of life, physical and emotional independence. Time spent with loved ones." In a sense, we consider these things priceless, but we quantify, but we also appreciate in this way. That has, you're here at Ash and other places, this is coming through.

Dr. Goldberg: Ears are plugged, December 8th, I'm working with Bio and Jade develop, we are having a program, Beyond Value Frameworks. How to define and measure value from a patient perspective. It's being done. We are going to get rid of quality, we are going to measure the value based on life years saved, productivity, well being. All these things as Pat Furlong says, of the parents who are fighting muscular dystrophy, "We can quantify the tears." I just wanted to read to you a quote from Bobby Kennedy who talks about this. He talked about the fact that our GDP measures a lot of things that don't reflect who we are in our real worth. Let me just say what he says that, the gross national product, you can use the term, health frameworks, does not allow for the health of our children.

The quality of their education or the joy of their play. This does not include the beauty of our poetry or the strength of our marriages, the intelligence of our public debate, or our integrity. It measures neither wised or courage, neither our wisdom or our learning, neither our compassion nor our devotion. It measures everything in short except that which makes life worth while. We can measure what makes life worthwhile. Just because the Value of Frameworks don't, doesn't mean that we shouldn't and we should. That's the way we reward innovation. By measuring how much value and how much ability we have to hope and feel that life is worth while.

Jonathan Wilcox: These principals are timeless. I was fortunate to attend an event yesterday, one of our sponsors Soul Gene and an artist and a philosopher, but he left me with something that was not complicated concept. There was beauty in simplicity. Hovel said, "We've blurred and lost the distinction between net worth and self worth." What you have said, is the self worth of patients and a society and whatever end that they touch, the economics of healthcare are as complicated and timeless as infinity as the science of healthcare as well.

Dr. Goldberg: I'm not just inventing something out of thin air. The whole field of development economics in third world developing countries, have done careful scientific measures on how interventions raise hope and raise aspirations and how important those aspirations are to development. Far more than the transfer of income. It's not just about the money. Economics is the conduit through which innovation makes us a better people and makes us hope and look forward to the future. We have to start measuring the innovations by those benchmarks and we can.

Jonathan Wilcox: I spoke before about our journey and how we got to this place and space and the people we met who led us on the path. I did not know about this world. I did not know about these ideas. Bob Goldberg is one of the people that I met, one of the first people. He was just an angry young man back then, but now he's a-

Dr. Goldberg: Successful angry old man.

Jonathan Wilcox: No, my point is that, you and other people showed that this fight is not worth having, but it's all of our fights.

Dr. Goldberg: It's all of our fights. There are things we can do. It's not just really against the system. There are changes we can make in how we value medicines. There are changes in the way that drugs are made accessible. It is unfair and it is wrong for PBM's and insurance companies to get 40%, 30% rebates, put it in their pockets and then turn around and tell patients, "Fail first and then we will give you the privilege of paying 30% of the retail price of a drug." How the hell did we get into this situation? That is so fundamentally wrong that if we don't change it in the next 2 to 3 years, shame on all of us.

Jonathan Wilcox: When I spoke, we put it in the way of, "That rarely, if ever, had so much economic analysis, clinical data, and public presentation been used so ineffectively that has opened the door." What does the future of reform, in a lot of ways we are talking about reform. In a lot of ways, these are new opportunities. What does the future of reform look like to you?

Dr. Goldberg: Well, there you go again. I think that there are three things we can look forward to. First of all, the inequity of cautionary and failed first will be addressed. It will be addressed. I think there's bipartisan support for it. I think there is bipartisan support for changing the regulations will make it very difficult for pharmaceutical companies who directly provide patients and their physicians and their health systems with the support to make a patient whole as opposed to rebates. Number two, I think you're going to see, there is a greater emphasis on generating real world evidence at the FDA level, which in turn, can be used to make the case for reimbursement going forward. You don't have a forth hurdle.

The third thing is, I really do think that we are on a... We have an opportunity to even accelerate the process of innovation even more quickly. Cell Gene for instance, is doing meta analysis, they are using real world evidence to get approvals for additional indications. Those things are pushing, are reducing the time that people have to wait for medicines. The recent approval of these immunotherapies for satellite instability in tumors. We are going to go... The fourth thing is, we are going to go past body parts and work on mechanisms and pathways. I think all of that will become an irresistible force to eliminate the barriers, which are literally costing peoples lives.

Jonathan Wilcox: One irresistible force to another, I'm going to turn him around and send him out into the world. Watch out and please thank Dr. Robert Goldberg.

Dr. Goldberg: Thank you, sir.

Speaker 15: (Video) When Don Wright set out on his milestone run, that blustery Sunday morning in Philadelphia, he carried his familiar message of inspiration.

Don Wright: (Video) There's life after diagnosis.

Speaker 15: (Video) From Duluth in 2003, to Philadelphia in 2016, Don with his wife and daughter, have spread that message across the United States and Canada. Of course, not everyone can run marathons. We can all appreciate the value of medical innovation.

Speaker 17: (Video) He calls it, "His miracle pill."

Speaker 18: (Video) My official diagnosis was May of 97'.

- Speaker 15: (video) In fact, the day before the race, Don met Phil Falkowitz, one of the very first patients to take an even earlier treatment for multiple myeloma. The same cancer that Don has.
- Speaker 18: (video) It was a pioneering thing. It was a pioneering episode.
- Speaker 15: (Video)Fifteen years later, Phil still takes the medication and says he's doing well. So, void by his new treatments, Don set out to cross the finish line for the 100th time. As he did, Don carried his message with them.
- Speaker 19: (video) I read about this man in a newspaper and I was so proud of him and I was really wanting to give him his metal today and I finally got to do it.
- Don Wright: (Video) I'm going to be just fine. Everything's tired and it's suppose to be.
- Speaker 15: (Video) Of course a hug from his daughter, Sarah, and a kiss from Artist, his wife of 53 years.
- Speaker 20: (Video) Think of all the cancer patients that may get a drug they need because somehow in the intrinsic circles of this publicity, they get what they need. You really did it.
- Don Wright: I didn't, we did it. It feel wonderful, it feels wonderful. I expect to run more marathons. There will be a number 101.
- Jonathan Wilcox: The one, the only, the 100 man, please welcome, Don Wright. I remember the other night before the Philadelphia race, I turned to my wife and said, "How many marathons am I behind Don?" She said, "Still 99 honey." Now I'm 100 behind you and 101 I will have to accept that to you. Everybody says congratulations, but you've always been about sharing the key points, your journey, your experience, what innovation has meant and people who are watching. You've always made it very clear how you started. You took your medication as part of a clinical trial. Tell me about that.
- Don Wright: I'm reminded of the wisdom of my favorite philosopher, Eddie White, who said, "Don't believe your own publicity." You just saw the publicity. Of course, the 100 marathons are true, but what's important to me is not the marathons. What's important to me is my wife and daughter, they are here with me. The 13 years that we've had to love each other and the fact that I've lived to meet my grandchildren. That's the value to me. It's all possible because of three three things: Innovation, access, and reimbursement. The medications that I've taken down the road and that I'm taking now, are ones that were not even thought of at the time that I was diagnosed.
- Jonathan Wilcox: That's an interesting point, you talked about, I know got a clinical trial, what was said to you was that it gave you a 6 year head start.

Don Wright: That's right.

Jonathan Wilcox: As eraser, for patients who had to wait on formal approval. You have always said, patients need not just access, seek out clinical trials like you did.

Don Wright: Absolutely. I would recommend that to patients and for doctors to seek out trials to participate in too. That's part of the access point.

Jonathan Wilcox: Everybody's putting metals on you, what I'm going to do is, I don't want to undo the metal, if you can just hold that for a moment.

Don Wright: Oh, sure. Innovation is equally important and I think that what we must do as a nation and as a world, is make sure that we don't take away, that we preserve the incentives that the pharmaceutical companies have. They're the ones that do the heavy lifting and we have to make sure that they continue to have the incentive to innovate, to bring us the new drugs that we need.

Jonathan Wilcox: One thing comes to mind when watching the video. I'm going to believe book scenes, I've seen it. Innovation is a word that we embrace. I love that word, but it's not just medical innovation. It's continuing innovation you said. The medications that have allowed you to do this have been that tool, weren't even imagined even in the most farsighted researchers at the time. I guess I'm going to ask you, what would have happened to you, even after getting your miracle pill, a miracle as it was, what if it stopped there?

Don Wright: Myeloma evolves and myeloma mutates. The combination of medications that I'm on now will eventually stop working. We know that's the case.

Jonathan Wilcox: Even though you've been given a miracle, you've gone as far as the miracle can go. We need new ones?

Don Wright: That will happen. I've been thrilled to see what's coming down the road by walking around Ash and seeing the posters of the trials. Sometimes very early trials of things that are going on. I remember seeing one of them 3 or 4 years ago that really looked pretty good and now I'm taking it.

Jonathan Wilcox: There are a lot of complaints about coverage. I've heard about them today and policy, high out of pocket expenses. You've informed us that you're living proof that the system does work, it can work, it can help. Even if only for certain patients. Your experience is something that we should hear.

Don Wright: My insurance company has stepped up. I've never heard a peep from them. My doctor prescribes it and they pay it. Maybe there is other interactions that have gone on that I don't know about, but this is how it should be. This is just exactly the way insurance should work.

Jonathan Wilcox: A lot of people complain about the industry, there are a lot of controversies in this and they are not to be fully addressed today, but you've told me is that the industry can also be not just a material, a tool support, a lifeline of support. When your clinical trial ended, what did you do? Did you turn to somebody for help? What happened?

Don Wright: I wasn't sure what would happen next. I went to the manufacturer of the medication and they fixed it right away. Whoever they talked to, it was covered right away. That's something that I highly recommend to patients is the manufacturer of the medication can be a lot of help.

Jonathan Wilcox: This is my point. There is a term that used in my world, which is, "Patients need to be their own best advocates." One thing you've accepted is, you took it upon yourself, be a leader for yourself, reach out and access. You're living proof that it can happen. You've told other people this.

Don Wright: Yes, and I could give other examples. I think it's very important for the patient to understand what's happening and not to just entirely rely on the medical profession to take care of you.

Jonathan Wilcox: One last point. I was president, was that Marathon 93' in Washington DC. What I want to tell everybody here is I said, "How do you do it?" I ran to you for a little while and that was enough. My point is, I said, "How do you do it?" You talk to people, you converse with people, you reach out and I can tell you, that young people, old people, fast runners, slow runners, I just want to relate that I have seen it. You have reached out to touch them and I've seen them. They have reached back, reached out to touch you. I've seen it, I believe it. Marathon 101 is on it's way. This man is my favorite guest. Please thank and honor Don Wright.

Don Wright: Thank you.

Jonathan Wilcox: I will say a final word and thank everybody. I've never a patient just one voice and to me, I walk in the sacred steps of the patients and the fighters. They are really the ones that are here. I confess, even though, I'm a big talker and everything, but I've always felt vague, I feel vaguely smaller. Nobody ever made me feel this way, build me up in this hero community. I walk only in the sacred steps behind them. This is why we fight. This is why we do this. I think we are making a difference, but you're the ones who all make it possible for us, inspire us, make this all possible. Please thank, this is Dr. Robert Goldberg, Dr. Rafael Garcia, Don Wright. Please thank. Thank you very much.

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