Making a Difference: Leaders in Advocacy From the MPN Alliance Australia Share Their Story

Nathalie Cook
MPN Patient Advocate

Ken Young
MPN Patient Advocate

Andrew Schorr:
Hello and welcome to Patient Power. I'm Andrew Schorr, and we are just back from visiting New Zealand and Australia. And along the way, met some wonderful people living with one of the conditions I have, an MPN; for me, myelofibrosis. And we were very struck by the fact that people sometimes have to really go to bat to connect with a knowledgeable specialist and ideally get the treatment they need and deserve, and have it paid for by the government.

Well, joining me now are two Australians from Melbourne who've really done that battle, both living with polycythemia vera. So, there's Ken Young. Ken, welcome to Patient Power.

Ken Young:
Hi and thank you, Andrew.
Andrew Schorr:
Thank you. And then also Nathalie Cook. Nathalie, welcome to Patient Power.

Nathalie Cook:
Thank you, Andrew. It's great to be here.

Andrew Schorr:
So, Nathalie, let's start with you. Tell me and us a little bit about your polycythemia vera story; how it started with ET, I think, and then how you have really fought for getting the treatment that would be right for you.

Nathalie Cook:
Thanks, Andrew. Yes, I was diagnosed with ET in 2008 and then by 2010, we realized that I actually had polycythemia vera. And so then I had to make treatment decisions. And the two treatments available were either hydroxyurea (Hydrea), or hydroxycarbamide, and the original form of interferon, interferon alfa-2a (Roferon). I read up on as many papers as I could get my hands on to help me make the decision on which treatment and I decided initially to start hydroxyurea to try to get my blood counts down, but long-term, I wanted to use interferon because I liked the research that had shown that it could

improve long-term outcomes.

In Australia at that time, there was only the old form of interferon, the Roferon that was available on our Pharmaceutical Benefits Scheme, which is the government-funded medications scheme. And the newer form, Pegasys or pegylated interferon, was only available off-label and it was very expensive, so it was out of reach of most patients.

So, I started calling the drug company Roche, who are the producers of interferon, of peginterferon alfa-2a (Pegasys) and Roferon, to ask about their plans to apply for a PBS listing of Pegasys. And they told me they had no plans and they said there were no clinical trials being done on that for MPN patients in Australia and there was really no interest. And it was also not approved by our government agency called the Therapeutic Goods Administration. And that's the first point in Australia that a drug has to be approved for a particular indication before the PBS can go through the approval process. So, there were several hurdles that no one was looking into.

So, I started Roferon, and I got all the typical side effects; flu-like symptoms, and aches and pains, and headaches, et cetera, and nausea, and even hair loss, which was very distressing. And I continued to call Roche and I told them about the side effects I was having. So, I started Roferon in early 2011 and regular calls and emails to Roche. And then in 2012 towards the end, they offered me Pegasys on compassionate grounds.

So, I called my hematologist and he arranged for me to get that. And initially, it was on for one year. And then during the course of that year, I continued to update Roche and told them about how better I was doing on Pegasys compared to Roferon and thanked them for it. And then they continued that access sort of on a year-by-year basis.

Andrew Schorr:
You were your own clinical trial.

Nathalie Cook:
Yes, exactly. Exactly. And then I started contacting my local politician, federal member, and I actually met with him in Canberra and asked him if he could help. And he asked me to write him a letter that he would give to the health minister. And then the health minister wrote back to me and basically said thanks for your interest, but it's not possible.

Then I had the opportunity because I'd been doing a lot of voluntary work with the Leukaemia Foundation – I'm also a dietician, so I've presented as a dietician at patient conferences and write articles for the MPN News, et cetera. And I was invited to a rare cancers forum at Parliament House in 2016. And at that forum, I got to hear a lecture by Professor Andrew Wilson, who is the Chair of the Pharmaceutical Benefits Advisory Committee. And he spoke about the importance of hearing the patient's perspective in making drug decisions.

And after his lecture, I actually got the opportunity to speak with him and I told him my story. And I asked him how can we get Pegasys on the PBS because I said Roferon, which is the same drug, is on the PBS, so why don't you expand your listing to include the newer form of the same drug that you've already approved previously. And I asked if I could write directly to him. He said yes and he wished me luck.

So, I went home and I wrote a submission to him. I wrote it like a scientific report and had about two pages of references at the end. And I spoke about my personal story, but also the evidence had references to the evidence that supported use of interferon in MPN. And I got three physicians in Melbourne to review my submission before I sent it in; two hematologists and an infectious diseases physician who's a friend of mine.

Then I sent my submission in and I got a letter back saying yes, the PBAC, the Pharmaceutical Benefits Advisory Committee, agreed that there was a need for interferon-based therapies in MPN, however, they hadn't had a submission from Roche. And they asked me to continue lobbying to Roche, which I continued to do. Then the PBAC would get with Roche over the next year. And after it was tabled at three PBAC meetings, it was finally approved for listing.

And then the next step was for the health minister to sign off on it, which then happened. And then in July last year, I was asked to attend the announcement by the health minister at Peter MacCallum. Ken also came with me to that announcement, which was wonderful. And then 1st of August, 2018, it was on the PBS.

Andrew Schorr:
Okay. So, let's make sense of this for people. So, basically, you beating the band and connecting with the right people, some of it by happenstance at the conference where you met one of the head officials, and putting the evidence together, and lobbying the drug company while you were lobbying the government, you were able to get approval and now payment for a less toxic therapy that's effective for MPN patients. Did I get it right?

Nathalie Cook:
Absolutely. That's correct. Absolutely.

Andrew Schorr:
So, Ken.

Ken Young:
Yes?

Andrew Schorr:
You've been around all this. Is this an example of the power of patients when we really start speaking out?

Ken Young:
I think the important thing is two steps of history back. Quite a few of us had been trying to crack the nut of how to get Pegasys listed, and it was so bureaucratic and so difficult to negotiate the pathways through. And what Nathalie did was having the real knowledge as well about how the system worked; building relationships with the PBAC, but also the drug company.

Previously, Pegasys was only listed for treatment with hepatitis C here in Australia. A lot of the drug companies weren't prepared – they didn't really have a market to bring forth some of these drugs. So, what the patient side of it was is we also used our community to lobby. There was a Senate inquiry that looked at access to drugs, so we mobilized, through social media and through email lists, submissions in the first instance to the Australian Senate in an inquiry.

So, it also was about the process of consciousness-raising amongst the patient community that we could actually actively take on these requirements. And I think it's just as much raising the awareness and saying you can do it.

I was diagnosed 20 years ago and probably never expected to see the changes that have happened in the MPN field since then. You know, the discovery in the early 2000s of the JAK2 mutations and then the development of the JAK2 inhibitor treatments. And that has opened it up and I think it's really, really important to understand the patients are our own best experts on our diseases. And we can be angry. A lot of us, we can still make a significant impact. And I think that's the important takeaway to the story, is to keep plugging away.

I know Nathalie has been at this for quite a while, and people before, and the community. But it's about keeping the faith, I suppose.

Andrew Schorr:
Right, right. Well, I think this is really a universal message. As you know, before Esther and I were in your city, we were in New Zealand, and we met with patients there. So, fewer patients, much smaller country, really no specialists, and also a government strapped with trying to pay for different medicines, and really not knowing about MPNs as well. So, in their country, ET and PV are not even listed or there's no registry keeping count of those people and whether they, in fact, are living with a cancer; myelofibrosis yes, but the other two, no.

So, a lot of, I think, what's going on in your country and I think can happen in others is to your credit, both of you, and with the MPN Alliance in Australia and the Leukaemia Foundation, is bringing people together so there can be 100 Nathalies or 1,000 Nathalies. And obviously, with the smarts that you have, Nathalie, of how to navigate the system, but with more impact; speaking to more senators, more government officials.

And it's so interesting and we're having the same thing in the U.S. Where the gentleman gave the speech about wanting to hear the voice of the patient and they're saying that in the U.S. as well, but they're often not hearing it or not effectively

listening. Where it's not coming through with clarity and I think that's what we can work on. But Nathalie, to your credit, you hit all the steps.

Nathalie Cook:
Thank you, Andrew.

Andrew Schorr:
And I think it benefited a lot of other patients. So, it really is about self-advocacy for patients in getting to the right healthcare team. And then it's community advocacy, a way to know you're not alone, but also to the community to have the options available to them.

And Nathalie, you should really be commended. So, how are you doing? How's your health these days?

Nathalie Cook:
Thank you, Andrew. I'm very well, thank you. And I've recently had a third bone marrow biopsy; the third I've had in the 10 years since my diagnosis. And it seems to be that the fibrosis that was developing has regressed. So, my hematologist said that looking at my bone marrow, she couldn't actually tell that I've got MPN now. This bone marrow biopsy looked like mild to moderate fibrosis six years ago, but the last one only showed very mild fibrosis. And she said she knows I have MPN because I'm JAK2 positive, but my bone marrow biopsy was looking really great and I'm feeling really well as well.

Andrew Schorr:
That's great. Ken, what about you? How are you doing?

Ken Young:
I'm traveling quite well. I'm actually not using pegylated interferon. Since 2006, I've been using Roferon and I'm, again, traveling well. I took the decision, even though it's available, when it's not broken, don't try and fix it.

Andrew Schorr:
Yeah. For Nathalie, it was broken.

Nathalie Cook:
Yes.

Ken Young:
Yes, for Nathalie, it was broken, but for myself, I'm traveling all right with the Roferon at the moment.

Andrew Schorr:
Okay. Well, I think the point is we're all gonna be different. We're gonna react to different medicines differently.

Ken Young:
Absolutely.

Nathalie Cook:
Yes.

Andrew Schorr:
And our journey may be different with an MPN. As science identifies more effective treatments, even for certain subsets, if we're in that subset, we wanna make sure it's available to us.

Ken Young:
Absolutely.

Nathalie Cook:
Yes.

Andrew Schorr:
So, we're all about advocacy and I really commend all the work that you do. The two of you as volunteers of the MPN Alliance Australia, the Leukaemia Foundation, that collaboration and working with doctors who, Nathalie, you said they

were helping you tell the story. And I'm really glad that both of you are doing well. And Nathalie, I hope that bone marrow biopsy, not that anybody wants to have them frequently, but they continue to look better and better.

Nathalie Cook:
Thank you, Andrew.

Andrew Schorr:
Okay. And Ken, it was great seeing you in person in Australia and all the great folks we got together, Nathalie couldn't make it that night, but really meeting some of you in person. And our commitment at Patient Power is to tell more of these stories. There's no shortage of American stories, but there are certainly Australian stories, New Zealand stories, European stories and lessons to be learned worldwide.

So, I want to thank you for sharing the story of advocacy and the progress you've made in Australia. Nathalie Cook, thanks for being with us.

Nathalie Cook:
Thank you very much, Andrew, for having me.

Andrew Schorr:
Okay. And Ken Young, thank you. All the best to you too.

Ken Young:
Great pleasure. Thank you very much.

Andrew Schorr:
Andrew Schorr recognizing that we patients, we have a worldwide story to tell and isn't it great that we can connect this way to bring us together, share stories, and really take action that can make a difference. Remember, knowledge can be the best medicine of all.