Worldwide Clinical Trials

In Their Own Words: Living with IPF

The Voice of the IPF Patient

Overview

At Worldwide Clinical Trials, the health and welfare of the patients, their families, and caregivers are at the core of our efforts. As a result, we conducted qualitative interviews with patients and caregivers affected by idiopathic pulmonary fibrosis (IPF) to help inform our IPF research and trial design. This patient-centric approach allows us to incorporate their collective voices and experiences into our overall strategy.

IPF Diagnosis

We interviewed seven patients and caregivers affected by IPF; these patients varied in age, sex, location, sociodemographic status, and date of diagnosis to ensure that we had a representative collective voice.

Questions & Responses

What Symptoms Do You Experience That Have the Most Impact on Your Day-to-Day Lives?

Respondents provided a wide range of responses when we asked about symptoms that varied depending on the patient's circumstances and the stage of their disease, amongst others. However, the consistent themes that emerged were surrounding the high level of fatigue experienced, feelings of breathlessness, and a persistent cough. These themes are generally consistent with other patient-focused research in IPF; however, another consistent theme that emerged was the need to plan for activities in their daily lives. Patients and their caregivers spoke about how this disease has stripped them of any spontaneity, especially those patients on supplemental oxygen; it caused them to lead very insular lives with their social circles dwindling and some not even able to leave the house.

"I can't even talk with my wife anymore because I get out of breath talking."

"It's destroyed my quality of life."

"When I am sicker, I can't even climb the stairs."

What Daily Activity Limitations Do You Experience?

Patients and their caregivers talked consistently about how much IPF has changed their daily lives and the significant impact on many of their hobbies and daily tasks. One patient mentioned they had been passionate about singing their whole life but can rarely do so due to getting breathless. Again, patients on supplementary oxygen therapy mentioned how this can cause them considerable anxiety if they need to travel outside the house. Several patients also commented on how they had become very reliant on their care partner, even those with a previously high level of independence.

"I have a lot of anxiety when going out and taking public transport. I am always checking to make sure I have enough oxygen and planning when I need to head home."

"I now need help getting dressed and undressed."

"I used to go antiquing with my buddy, but I can't do this anymore. I don't have the energy."

"I can't leave the house without my army of inhalers, and I have to pack a little to-go bag in case my oxygen drops. It's continual stress."

What Medications Do You Take & What Are the Side Effects?

Most patients spoke about the use of anti-fibrotic medications such as pirfenidone and nintedanib and the impact that these medications had on their condition. Many of these patients have tried both medicines before finding the right fit. There was a difference between patients in their sentiments towards taking these medications, with some understanding that these side effects were acceptable given the greater need for their underlying condition. However, others were more reluctant to take these medications.

"My appetite has gone, but I need to have protein, so have been having a lot of protein shakes instead of meals as it's easier for me."

"Both me and my wife now volunteer for an IPF support group and often speak to patients hesitant to take these medications because of the side effects. If you have cancer, you wouldn't not have chemo because of the side effects, so why is this any different?"

"I have been taking Ofev for about six months. It has affected my liver and kidneys, my LFTs [liver function tests] are well out of whack, and I have GI issues. I have learned to accept that I am not going to feel great."

"I got really sick on nintedanib; I stopped it after ten months and was really upset with my physician."

"It's a topic every time on my monthly Zoom meetings with other patients who don't tolerate very well with nintedanib."

What Is Your Perspective on Ideal Treatments?

As there is currently no cure for IPF, we wanted to understand patients' views on future directions for treatments specific to the disease and any symptoms consequently manifested. Responses varied depending on the stage of the disease and whether this was a patient or a caregiver. Both groups frequently mentioned that early treatment and intervention would be key, as well as the ability to stop the disease in its tracks — an interesting topic that came up around halting the progression of the disease was doing so when lung function was still high, as stopping the disease with reduced lung function would not improve their quality of life (QoL). Respondents also noted that any potential side effects of new treatments would be significant, learning from the side effects of both nintedanib and pirfenidone that these play a fundamental role in patient decision-making. Finally, patients and caregivers told us about preferred routes of administration, with an oral tablet being highly preferred, followed by inhaled medications, and lastly, intravenously given medications.

"If I could have kept my lung function around 75-80%, everything would have been more manageable."

"Stopping the disease needs to be done when lung function is still there and definitely before oxygen therapy is started."

"Treatment with minimal risk."

"It would suppress the cough. The cough is the most bothersome thing. It would also have to be a medication that was an extended release which would last. All these medications I am using don't last; it's so short acting."

How Do You Understand IPF & Learn About Your Diagnosis?

As IPF is a rare condition, patients' knowledge and understanding of being newly diagnosed and what this means for them is a key foundation on their disease journey. All patients and their caregivers commented that they went home and searched online for information about the disease, with varying levels of success. Many patients were signposted to or found themselves patient support groups for IPF, which they additionally used both for information and support. One patient who had been living with the disease for several years commented on how poor the information was at the time of his diagnosis, but this has improved considerably in recent years. Yet, there is still a gap in patients' knowledge of the disease and ease of access to this information.

"The information online was awful."

"We were sent to a support group through the hospital – they also send all the information we need on clinical trials."

"I almost cried in my doctors office, especially when he told me it's irreversible. It's like getting hit in the head with a sledgehammer."

"It's like any other disease — it was all in medical framework and medical terminology."

"I went on Google and did a 'horrible' Google search."

What is Your Clinical Trials Experience & Any Associated Burden?

Most patients we interviewed had clinical trial experience, and we asked targeted questions about the burden clinical trials place on patients based on the assessments used, length of participation or number of visits, and travel requirements. During the questions about clinical trials, it was clear that patients and their caregivers experienced some frustrations during their participation.

"I was disappointed about the lack of feedback. It's important to know whether my participation made a difference. I am now a patient research ambassador [at his hospital] ensuring the patients receive proper acknowledgement and updates about trials they are involved in."

"I did a trial two years ago, and I haven't had any information about it since I left."

"I can't say if it benefitted me as I don't know if I got the drug or the placebo."

A patient who had done two trials not related to IPF mentioned how he wasn't excited about looking into them for IPF: "Problem is I have had such a bad experience with the other trials. They were so cumbersome."

What Are Your Perspectives on IPF Assessments & Endpoints?

We often hear concerns from the rare disease community about the freuqnecy of assessments used in clinical trials. Our goal in these interviews was to learn about this topic specifically within the IPF community and which assessments patients considered to be part of their normal standard of care. We asked for their views on PFT assessments, high-resolution computerized tomography (HRCT), and the use of questionnaires, both paper-based and electronically via a tablet or smartphone.

Spirometry

"At times it feels like you are going to suffocate inside this box as its very hard to breathe."

"I need time to recover after doing them before I go home, so I sit and have a coffee."

"I initially enjoyed them but grew to hate them as my disease progressed due to the fatigue and coughing."

"I think PFTs [pulmonary function tests] are made to frustrate people, I really do."

HRCT

"I would get anxiety if I needed to do it monthly."

"The first time I got stressed, but it doesn't stress me out anymore."

"I would prefer a CT over spirometry; they are less physically demanding."

"I hope the clinical trial would pay a lot of money for doing that many scans."

"They don't bother me as bad (compared to spirometry) but it would be a pain to have to go in and do them."

Questionnaires

"They should be done at home and shouldn't take longer than 15 minutes."

"I have used them before. I think most of them are redundant."

"A lot of times when I recorded it on the tablet, it didn't go through. I get a call the next day saying it hadn't gone through. Well, it did. This happened nine times."

"It would be a chore. You don't really want to it, but you know you have to."

"Anything longer than 30 minutes would be a no."

One patient shared their frustrations with the amount of paperwork needed to participate in the clinical trial process. **"Keep it more simplified! For one clinical trial I applied to, they called me back, then sent me 44 pages to review and sign off, then when I got to the study site they gave me the same papers, it took me four hours in my first visit."**

Travel

In most countries, IPF treatment occurs at a specialized clinic. Scientifically, this results in high-quality data, but from a patient burden perspective, patients often have to travel long distances to a potential clinical research site. To better understand this challenge, we asked patients how far they would be willing to travel, possible barriers to travel, and what types of support would be most useful.

As for types of support, patients mentioned reimbursement as a necessary component in clinical trials to ensure they do not have to pay out-of-pocket expenses. Finally, patients on supplemental oxygen therapy raised concerns about the duration of any required travel and limitations to ambulatory oxygen as a barrier to their trial participation.

"I have no issues with travel as long as I am not out of pocket."

"I can travel anywhere within a 50-mile radius. If I have to go to two or three different sites in a day, this is more difficult. Definitely provide transportation."

"I'd be happy to travel ten hours but would need help with expenses and hotel costs. Concierge travel would be awesome and something I would be interested in."

"I travel 400kms three times a year for medical care."

What Are Some of Your Barriers to Trial Participation?

As many of the patients had taken part in clinical trials, we could gain first-hand insights into what challenges they faced in their experience and potential future barriers they would want to be alleviated for any future clinical trial participation. The sentiments here are consistent with themes previously shared in this report. Finances and travel were key decision-makers. Again, patients shared concerns about how the trial would fit around their supplemental oxygen use, and finally, multiple patients shared concerns about the lack of communication.

"I wouldn't want to travel alone; I would want my partner to come with me."

"You need to make patients feel special and valued."

"Concierge services would make me happier to travel further, but would be even better if ambulance travel could be offered."

"If you could be paid while doing it, it would be a bonus."

"There needs to be more information about clinical trials. Current databases are often outdated and not user-friendly."

Capturing What Matters Most — The Participant Experience

The perspectives of patients and caregivers in IPF clinical trials are crucial to optimizing research and improving the lives of those affected. The psychological and behavioral impacts of IPF, such as lung function decline, limited mobility, and undesirable side effects, are among the most challenging components of the disease, significantly impacting patients' QoL. In the face of adversity, patients and caregivers are still motivated by the hope that treatment options will get better and ideally reach a point that can halt and reverse lung damage from fibrosis. To make IPF clinical trials more aligned with the needs and hopes of those impacted, trial design can take several key actions:

1. **Patient-Informed Clinical Endpoint Selection:** Ensure protocols capture clinical endpoints that genuinely matter to those participating while tempering the overall participation burden. These protocol considerations should use the minimal required frequency for PFTs and HRCTs and use focused and relevant questionnaires. Additionally, participants want feedback during and after the trial, making it vital to create a structure that fosters open communication.
2. **Minimize Participant Burden:** As daily life is already challenging, and patients require substantial preparation to leave the house, any elements of trial design that lower the burden will significantly increase enrollment. For example, providing physical and financial support for transportation, minimizing the number and length of questionnaires, and overall site visitation requirements collectively go a long way in helping those with IPF participate.
3. **Elimination Over Mitigation:** Those with IPF experience a significant downturn in their quality of life, sometimes rapidly. While delaying or slowing disease progression is helpful, patients understandably want treatments that work to halt and reverse lung fibrosis progression, to more fully function and return to their hobbies, friends, and loved ones.

By making study participation more accessible to more patients, we can recruit clinical trials more easily and ensure higher-quality data to develop novel and effective medical interventions.

Worldwide Clinical Trials

About Worldwide Clinical Trials

Worldwide Clinical Trials (Worldwide) is a leading full-service global contract research organization (CRO) that works in partnership with biotechnology and pharmaceutical companies to create customized solutions that advance new medications – from discovery to reality.

Anchored in our company's scientific heritage, we are therapeutically focused on cardiovascular, metabolic, neuroscience, oncology, and rare diseases. Our deep therapeutic knowledge enables us to develop flexible plans and quickly solve problems for our customers.

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