

# A double imperative



*Mary Baker*

**Q&A** *What is your perspective on the difficulties that the pharmaceutical industry can face when trying to bring a new drug for Parkinson's Disease (PD) to market?*

Parkinson's Disease patients are not happy about the existing clinical trials. When a drug is ready for clinical trials, industry and clinicians seek patients to participate in the trial and for all the scientific reasons, they exclude conditions such as dementia, osteoporosis, diabetes, and hypertension, seeking the pure, idiopathic, Parkinson's Disease patient.

But, because the age of onset of PD is about 59 to 60, and at the age of 60, people usually have about three diseases beginning to move on them, it means that when all this money is spent to get these trials in place, the person that they're testing is actually a rare, unreal person. So when the drugs come onto the market, they almost have to start all over again with the neurologists who've got patients with Parkinson's plus hypertension, plus osteoporosis, plus diabetes, plus etc.

The point we want to make is that it is going to become increasingly difficult for the industry to find these pure, idiopathic Parkinson's Disease patients because as the world is living longer, there are going to be more patients with PD, and more patients presenting with more than one illness.

We understand that there has to be a procedure, but we question this procedure. This comes from our desire to work alongside the industry, which we think is facing increasing challenges. Regulatory bodies are constantly raising the hurdles for industry to leap, quite understandably being obsessed with safety and increasingly, threats of litigation, and this limits innovation. We want to try to bring together the customers – the patients – with industry, clinicians, regulatory, plus the Commission and hopefully the FDA, to totally re-look at the procedure which, frankly, is now 50 years old and may be ripe for improvement to meet the challenges of today's world.

**Q&A** *What is the role of patients or their advocates in this evaluation?*

We have not really – really – listened to the patients in their Patient Reported Outcomes. “We must put the patient at the center. It must be patient-led. It must be patient-driven.” But it isn't. There's still very little credence given to the voice of the patient. I don't think that patients are experts about everything, but there's one thing that they are expert at, and that's living with this disease. Doctors are experts in understanding the disease and the disease process, but patients are the experts of “living with.” And therefore, when they do report back that “xyz is better” or that “abc is worse,” they should be listened to, because it helps clinicians to focus on better management of the illness and better use of the health system resources.

**Q&A** *How have you seen the industry respond to this sort of approach?*

I've found that many senior people within the pharmaceutical industry believe this. They're all very, very nervous about changing the process

because these big companies are like massive oil tankers; they can't turn quickly, and they need to face these challenges by working together. It's by working in partnership, with mutual respect, that we can improve current practice.

**Q&A** *Are you talking about personalized medicine for Parkinson's patients?*

It could be the ultimate goal. But before you even get the medication, the patients need to be involved further “up the river” in the production of a medicine and agree on the endpoints of what it is that would improve their quality of life. A lot of this, with the very best of intentions, is done through perceptions. They might see a tremor and focus on it. Whereas, perhaps, if you talk to patients, they might say, “Well, the tremor's all right, but I wish you'd do something about clearing my thought processes.” We're very good at treating what we see, so PD has for years been seen solely as a movement disorder because that is what James Parkinson described in his essay on the shaking palsy in 1817, with the patient presenting with slowness of movement, poverty of movement, and the pill-rolling tremor. That's what the doctor sees, sitting at his desk as the patient comes through the doorway into the consulting room.

When the EPDA and the WHO initiated a global survey of 2000



patients living in the six wealthiest healthcare systems, they found that, in a pie chart about the quality of life, only 17.2% was about the movement disorder symptoms and the prescribed medicines. Forty-one percent was the feelings of depression, not clinical depression but worry for the future. After that, was how well the diagnosis was given and understood. Behind that – and this is what really surprised the neurologists – came pain, sleep, bowels, bladders, sexual dysfunction. Things that were never discussed in neurological clinics...much of the quality of life is below the belt!

It began to throw a different light on this illness. It's not just a movement disorder. When we presented this at a Vancouver conference, a lot of neurologists were very surprised and interested, but when I presented the findings at the PDS UK AGM, one of the patients, a delightful chap, said, “Mary, how much did it cost you to find all that out? We could have all told you that!” The patients already knew what really mattered to them.

**Q&A** *Where and how might we improve our ability to listen?*

I've sat in clinics, even with key opinion leaders in neurology, and heard them say, “How are you? You look a lot better.” Well, that's a wonderful way to prevent further discussion. “Not depressed, are you? Bowels okay? Bladder okay? No sexual problems, are there? Now let me see you walk.” The patient gets up and walks, and then the clinician treats the walking, but there's a lot gone under the bridge that they've now missed. I have the highest regard for our neurological colleagues, but there was something they were missing, and we found it only



by asking and listening to what the patients are saying.

Finally, you weave into this that none of us can afford these health systems now, so we've got to trim the ships. Let's at least get an accurate treatment of the problem, not our perception of it.

We have a double imperative to be more focused on listening to the patient...to improve care, and to reduce the economic burden of the treatment.

**Q&A** *Can you articulate any changes that could get the patient's voice heard more "up river"?*

I think they need to be involved considerably earlier than they are. I remember when I joined the society, the pharma representatives came 'round and asked me if I liked how the pills were wrapped up. Do you like the blister pack? Well, both my parents had PD, and I can remember my mother having to bisect the pills; she soon found out that rather than taking one pill every four hours, it was better for her to take half a pill every two hours, so she used to cut them up. I and others reported this back to the product managers and eventually led to the little cutting

line on the pills. All these little things matter a great deal to the families living with Parkinson's Disease.

But really there's a lot more. Patients are intelligent, and the industry need not be frightened of them. If industry can involve them, and the patient can be better educated about how they can contribute to their treatment – if they were really, really listened to, there's an awful lot that could be improved.

For instance, there was a nice little study done about the wearing off of medication. Patients would take their medication, and if it was for four hours, the last hour was actually pretty poor because it was "wearing off" and they were looking forward to taking the next pill. When we did the survey about this phenomenon, only 29% of the doctors said they had seen it. The rest had not. When we surveyed the patients, 56% of them had experienced the feeling but hadn't discussed it with their doctor.

On further investigation, we found out that many patients get dressed up to see the doctor, because they're going to be examined, and they over-medicate so that they

feel and look good. Therefore, the doctors are missing it because they're not seeing real life; they're seeing how a patient goes off to perform in front of the doctor. So you can't blame the doctors for not picking up on it because the patient's behavior prevents the doctors from thinking upon it. It's the unscrambling of all these little things that come together to blur the real picture.

How do you feed this back in? It matters not only to the patient but to the economics of our healthcare systems now. We can't afford to waste time or medication any more. It's a double reason for getting this right. ■

• **MARY BAKER** serves  
• as President, European  
• Parkinson's Disease Association  
• (EPDA), UK. Mary will also  
• serve as chair for "What Does  
• the User Consider Value?  
• Including the Patients' Voice  
• in Assessments," Session 3 of  
• Theme 12, "Health Economics  
• and Health Technology  
• Assessments – Supporting  
• Sustainable, Socially Acceptable  
• and Equitable Access to  
• Innovation." She shared her  
• thoughts about the patients  
• for whom she advocates and  
• their perspective on drug  
• development and clinical trials.



**Mary Baker**