

Access Through Partnership – A Stakeholder Dialogue

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Abstract

A major barrier to optimal care in Parkinson's disease (PD) is the many years it takes for patients to gain access to new therapies and the best neurological services. A new treatment has to overcome many hurdles to become a clinically and cost-effective new therapy for patients, for example, gaining regulatory approval and reimbursement. Sharing opinions between the industry, neurologists, patients and other stakeholders about the benefits and risks of new treatments is important in influencing this process. The complex chain of events that currently characterises the development of new treatments for PD could be enhanced and accelerated by group discussions and collaborative care – right from the outset of the development process. Developing the process in this way would optimise patients' receipt of the best treatments in a timely manner.

Keywords

Parkinson's disease, access to new therapies, shared decision-making, regulatory approval, patient advocacy

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With an ageing European population comes an ever increasing societal burden of brain diseases. These illnesses, if managed sub-optimally, cause terrible distress and consume a major proportion of the budgets of healthcare systems. Societal changes also contribute to the growing requirements for the care of those with brain disease. For example, women were traditionally the care-givers in the family structure whose caring was freely given, but their role has changed. Thus, there is an increasing number of people with neurological illness and a decreasing number of care-givers. In addition, the longer we live, the more diseases we acquire.¹ Most patients with brain disease are likely to be taking several different types of drugs.

There is also migration across Europe on a larger scale than ever before, but not one country has developed a culturally-competent care programme. In addition, access to medication is very variable: in some countries, there is access to medication and a reimbursement system, while in others, there is access but no reimbursement, or no access to medication at all (data from the National Institute for Health and Clinical Excellence²). Furthermore, the rules for access and reimbursement are not transparent and vary from country to country, sometimes even between different regions within one country. After Europe-wide approval, reimbursement often takes years to obtain and

is sometimes not achieved at all, and this is generally a longer process than regulatory approval. Thus, access to Parkinson's disease (PD) healthcare professionals and medication needs to be improved.

In summary, there is a need to develop strategies to optimise the quality of life of people with PD, but a major obstacle to optimal care in PD is the length of time it takes for patients to have access to new treatments and the best neurological services. This differs considerably across Europe, but is many years wherever the person lives. Many complex barriers have to be overcome in the process a new treatment goes through from the laboratory bench to regulatory approval, reimbursement, and becoming a clinically and cost-effective new therapy for patients. The barriers to access and the role of partnership between stakeholders in overcoming some of these barriers are discussed here in the context of a hypothetical scenario.

A Hypothetical Scenario

Imagine a fictional country where an imaginary new potential treatment for PD, called Doparestore, has been developed by a fictional company called SimuloPharma. Doparestore must pass a process of review by an imaginary regulator, the Agency for Review of Therapeutic Advances (ARTa), and then by the health technology assessor (HTA), called the

Drug Assessment and Value Executive (DAVE). The hypothetical scenario described below highlights the complex issues that must be considered before people with PD can gain access to the new treatment.

When the informed person with PD finds out about the clinical results of Doparestore, he or she wants access to this drug. Their thoughts may follow like this, “I’ve been doing some research on the Internet, and I’ve read about this new treatment, Doparestore made by SimuloPharma. I’ve read that this drug could slow the progression of my disease, perhaps even stop the dreadful dyskinesia that I sometimes experience. And some of the other effects of the condition, it could make them better as well. So I don’t want to just sit back and do nothing. This medication might make a world of difference to me. Can I have this drug now?”

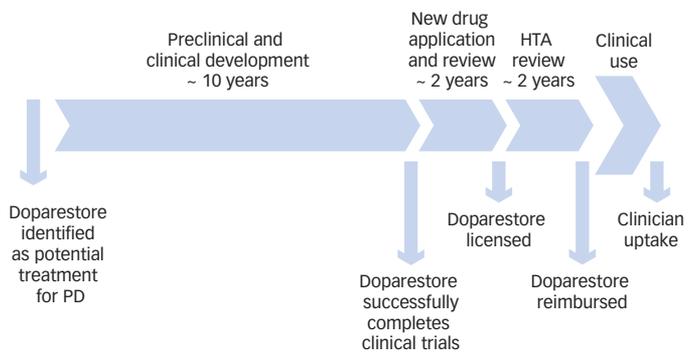
SimuloPharma wants people with PD to have access to Doparestore too! Generally, it takes 10–13 years to bring a new medication to the market (see *Figure 1*), and drugs such as Doparestore will have cost hundreds of millions of euros to develop, if one includes the failed drug candidates over that time. This is because industry is tightly bound by strict regulation to assess the efficacy and safety of each drug thoroughly before it reaches patients. Once SimuloPharma is “reasonably confident in the efficacy, safety and quality of Doparestore”, and thinks that it has established “an acceptable risk-benefit ratio”, it gives the drug to the independent regulatory body, ARThA, which will have to go through hundreds of thousands of pages of information to make sure that it agrees with SimuloPharma’s assessment of the drug.

ARThA reviews the information SimuloPharma has provided about Doparestore and forms an opinion on the product. The primary goal of ARThA is to ensure public safety, so any new product has to respect certain standards in quality, efficacy and safety. All patient data have to be carefully checked by the regulator, and they could include documents running to several hundred pages for each one of the patients – up to 20,000 in total – involved in the clinical trials. Various criteria, such as those relating to the manufacturing process, also need to be met. The review process can take two years.

After ARThA has approved the drug, the HTA, DAVE, needs to determine whether Doparestore has benefits in terms of the quality of life of people with PD in real-world clinical practice. The HTA defines its role as follows, “Our healthcare system doesn’t have a limitless amount of money. So our job is to look at the drugs and evaluate whether these are medicines that we should be spending our limited money on”. From time to time, the clinical data may fail to demonstrate real-world benefits for patients or that the new treatment represents value for money, and the HTA will not recommend the medicine.

What can be done at this stage to help people with PD access the drug? A patient advocate could engage with DAVE to convince the organisation of the real-world benefits of the drug for people with PD. The patient advocate would argue, “Since taking Doparestore, patients are sleeping better. They are less depressed. Their bowel function and constipation are better, and many can actually manage to go to work. Many have found that they can return to their hobbies or that their sex life is improving. Overall, quality of life has improved for many people with PD, and this is very important for families. Patients have been making such strides forward since taking this new drug that their partners have been able to go back to work full-time.

Figure 1: Typical Timeline from Identification of a Drug Candidate to Clinician Uptake



HTA = health technology assessor; PD = Parkinson’s disease. Please note that Doparestore is a fictional drug.

The drug is allowing people to play their full roles in society again”. Such arguments and data on patient-reported outcomes can influence a good health technology assessment process and lead to positive decisions.

The outcome here is that Doparestore is finally recommended by DAVE, so people with PD should gain access to the drug – or will they? The informed PD patient now asks their doctor, “You have the drug there, Doparestore. It’s been approved. Can I have it?” The doctor, a conservative neurologist, is reluctant to prescribe this new drug. He or she wants to see long-term data, because there may be safety issues that have not become apparent in the relatively short-term clinical trials (where, for example, the patients’ age range and concomitant diseases or medications have been restricted). Their argument to the patient may be, “Are you sure you want to risk your life for this drug that has just come onto the market and has not been tried in a significant number of cases in real life?” The patient can open a dialogue with their doctor and may say, “But what about shared decision-making? I’m still young and I really feel that, apart from anything else, this drug is going to improve my sex life”. Communication, by the patient, about precisely what quality of life means to them as an individual is important and can help physicians in the decision-making process. Together, the informed PD patient and the conservative neurologist may decide that the patient should be started on Doparestore, as he or she is willing to accept that the drug may have unexpected adverse effects. Indeed, what many decision-makers who control access to treatments may not recognise is that patients are willing to take some risks for an improvement in their quality of life, and they are best placed to know what risks they are willing to accept.

The informed PD patient now has access to Doparestore, and he or she wants others to have access to the drug too. One way to do this would be to work together with the patient advocate and HTA to improve the regulation process, as discussed below.

The Role of Partnership to Improve Access to New Treatments

This hypothetical scenario highlights some very important issues. There is scope for industry to co-ordinate more with the HTAs and with patients. HTAs must develop universal and transparent systems and align their goals with the regulatory bodies. Patient advocates and people with PD need to understand how to work effectively within the system and appreciate that the regulatory body or HTA is not the

enemy. For example, people with PD, their care-givers and advocates can work together with industry to gather and communicate to the HTA the information it requires to recommend a treatment, such as real-world benefits to patients that are beyond the standard clinical trial endpoints. In addition, neurologists should understand that they can be involved in the health technology assessment process.

Conclusions

Patient advocacy groups do not have the power to effect change on their own. They need to partner with other key stakeholders, including the pharmaceutical and biotechnology industries, healthcare professionals, regulatory institutions and governmental policy-makers

to give people with PD a strong voice to communicate their needs and make their case effectively. Partnership provides shared information that allows patients to assess the benefits and risks of any new treatment, and open communication between the healthcare team and the informed patient allows joint decision-making and optimal outcome in terms of quality of life. Access to new treatments by people with PD is delayed by the sequential decision-making of regulatory institutions, HTAs, physicians and policy-makers. The possibility of these groups co-operating and making parallel joint decisions, while maintaining the precision of these decisions, can help all parties achieve the common goal of giving people with PD access to the treatment they need. ■

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