

## Story 1 : Unexpected turns in my trial experience

I was having Myozyme ERT infusions for about 6 years when my consultant asked me during my six monthly check-up whether I would be willing to join a trial starting soon. I had to have an assessment first to see whether I was eligible for it. It was a lot of tests for my breathing and lung function which had to be below a certain level otherwise I could not join the trial.

There was a lot of paperwork and it was hard to understand, it was very technical and not in layman terms. I spoke to my consultant and some things were explained. All my travelling costs would be paid and a meal in the evening as well.

Following the tests I felt that I did not want to join the trial because there was a big time commitment and it felt overwhelming. It meant I would have to stay late sometimes in the hospital, have a lot of extra tests and travel to the hospital every two weeks for all my infusions (when I had been having them at home until then). However, after long consideration and talking to other people I decided to start the trial despite my reservations. I felt a bit like a guinea-pig but I also felt that I was really doing something to try to help develop a new treatment that could help everyone with Pompe disease.

The commitment was massive. Every six weeks I had to spend 3 whole days (not nights) at the hospital having tests. This changed later on to every three months. I had MRI scans, muscle tests, ECG, walking tests, blood tests and urine tests. The commitment was massive.

I was very nervous having the very first infusion for the trial, there were new staff and different surroundings and the first infusion meant I had to stay one night in hospital as well. By about two months I got used to being on the trial and it was almost easier going to the hospital for infusions than at home. When having them at home you have to tidy up and get everything ready for the nurse to come. In hospital on the other hand, although travelling could be annoying, all I had to do was turn up and wait. Everything was done for you. As time went on I got used to the routine and it was fine.

In the eighteenth month I had a big shock. I went on Facebook one day to talk to a woman in America. She was also on the trial and she told me it had been stopped. It was stopping all over the world and the trial was finishing. That was the first I heard there was something not right. I looked at the company website and found that it was being stopped. I got in touch with the nurse doing my infusions and he confirmed this. They had been waiting for me to come in for my next infusion to tell me face to face. This was a very big shock indeed. I felt upset and angry and I cried. I had felt better on the trial and it all felt wasted time. All my time and travelling had no result and it all just ended with nothing. Of course, I realise that what is learnt from negative outcomes can be as important as that from positive outcomes.

After six weeks having no infusions, I re-started the Myozyme ERT and my home-care company started again too. The shock continued for a long time. I would rather have infusions in hospital, it suits me better. If another trial came up and it was offered to me I would consider it, but again I would have reservations. I would have a really good think about it.