Story 2 : Why I decided to join a trial

I am 34 years old and was diagnosed with Pompe disease just over 2 years ago. I have probably been suffering since I was around 13 years old, but never understood why I found certain things difficult, so I just put it down to being unfit. My sister started to experience the same difficulties and went for testing so it prompted me to do the same. I had my testing completed at Royal London Hospital but then transferred to Addenbrookes in Cambridge because they have a specialist unit and a specialized team.

My sister had heard about clinical trials and told me that they may want to speak to me about doing one. As soon as my sister told me I started to worry. A trial sounds really scary, and my perception of what a trial is and what it entails at that moment in time was so fabricated. I know that now, but at the time all I could imagine was the worst.
In my first meeting at Addenbrookes I was nervous but the staff are so knowledgeable that it put my mind at rest.

My doctor and nurse explained the trial briefly in my first visit, but they didn't overwhelm me with information. I was then asked to return for baseline tests, either way if I started on the trial or not, they needed my baseline results. We were given a more detailed explanation of the trial and what I was to expect. My doctor explained it to me like this:- Imagine the treatment that is available at the moment called Myozyme is a key and it enters the body looking for the correct padlock. Well, the investigational drug enters the body with 12 keys. So, they are aiming to prove that this drug is more effective in getting to the correct place to do the job it is required to do.

The trial I am on had specific requirements. The participant has to be treatment naïve. My lung function and muscle strength tests need to fall below a specific level. Once we found out I was eligible for the trial I took a few weeks to decide what I was going to do. I weighed up the pros and cons. No matter what I decided, I am going to start treatment, so either way I will be having treatment, but if I decide to do the trial I could be receiving a better drug than what is approved and on the market right now.

On this trial, I am blinded for the first year, not knowing which drug I am receiving, but the remaining 2 years it is Open Label, so I will definitely be receiving the trial drug. Therefore, worst ways I have 2 years of the investigational drug, or best ways I have 3 years of the investigational drug. I will be in hospital for every treatment, so if anything did go wrong I am in safe hands, surrounding by professionals.

The hospital is just over an hour away from our home, but my travel expenses are covered by the trial, as are any food or drink I wish to have whilst at hospital. At Addenbrookes there is a new Clinical Research Ward which is just like being in a private hospital and all rooms are side rooms, so you have your own privacy. Every 3 months I am required to come into hospital for Lung Function tests and strength test and on 2 occasions I have to stay at the hospital overnight to give a blood sample 10 hours after my infusion finishes.

By taking part in a trial, if this drug is approved by the FDA, then I will be helping my sister and my friends around the world as well as myself.

Once I had all the information, I realized that this is the 3rd phase section of this drug, so the risks are at their lowest. I asked my partner and family what they thought, but ultimately they said I am the one that needed to decide, and when talking to others I was advised that after knowing all of the facts I then need to go with my gut. I was also made aware that you can pull out of a trial at any point, but this has an effect on a study and its results, especially one that is for a rare disease as the amount of participants is very minimal, so I wanted to be extra sure before starting.

Once I had made my decision I had to wait for about 3 months before we could start, due to the fact that the study was only just about ready to accept participants and my hospital needed to be approved for the trial. Again my doctor and nurse ensured we understood fully and answered any questions we had before signing the consent forms.

I now have just 1 year remaining on my 3 year trial. My 2 years has flown by, my 2 weekly visit has simply become a part of my life. The research team are so kind, professional and compassionate, they are only a small team of about 10 and so they have taken their time to learn about this rare disease and have watched me improve and are genuinely excited to be a part of my journey. My specialist nurse is so supportive, no matter what questions I have she always makes time to listen and answer. There are a few things that take time in the trial such as letting my nurse know when I am feeling ill, if I take any paracetamol or if I injure myself.

For the first 18 months I was travelling to hospital every 3 months for lung function and physio tests, but now I only need to go every 6 months. My nurse organized it so that I could complete both in the same day to save me having to make 2 trips. I have a very small amount of paperwork to fill in every 3 months such as questionnaires and I also have an ECG and a checkup with the Doctor.

My team has spoken to me about what happens once I have competed my 3 year trial. Because I was one of the first on this trial, I will be finished before they have collected enough data for this study. They hope for me to continue with the trial drug as part of a study for further investigations.

I am very hopeful about the future because scientists are doing so much research and therefore require us to participate where possible to enable these studies. I would not hesitate to take part in another study as my experience has been such a positive one due to the drug benefitting me and the smooth running of the trial thanks to all those involved.