

ENMC Workshop on Diagnostic criteria & Outcome Measures in PMM

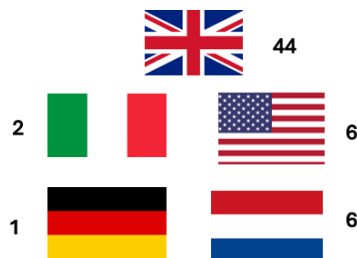
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Free online survey developed
Distributed by email to adults on Lily Foundation CRM and on social media
Further distributed by IMP to collect additional responses

77 expressed interest

71 consented to take part

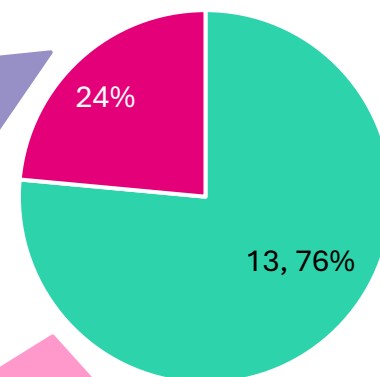


59 confirmed diagnosis of PMM given by a Dr

20 had taken part in a clinical trial for PMM

Do you think that new technology could help to track and report on these changes?

"Technology would help to log symptoms so doctors etc can progress further as everybody's disease symptoms are not necessarily typical."



I'm just not sure that wearable technology is reliable enough. And it is not a substitute for actually recording how one feels, physically, mentally and emotionally.

Are there particular factors associated with having a mitochondrial disease that make taking part in a clinical trial challenging?

"It can be very tiring and can demand a lot of mental and physical input".

Travel.
Finances.
Energy

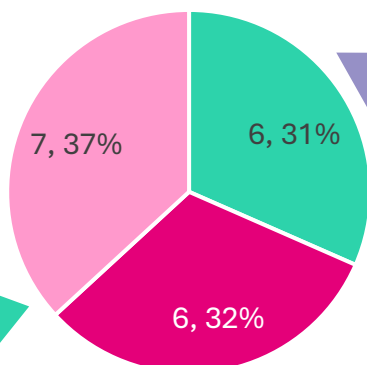


"Yes I would agree they are challenging and particularly tiring. Particularly acknowledging that a patient cannot travel without a rest. I have found that the timetabled day for a trial visit and all it entails hasn't always been easy (i.e. exercise test, bloods, paperwork done over 1 day when I'd have preferred to rest more in between each)".

"There are major factors for those of us who work. Time demand, travel demand, duration of trials, frequency of necessary visits all make participation challenging."

"I think there is work to do on clinical trial design and that bridges need to be built between researchers and patients as scientists are often removed from patients."

Did you notice an improvement in your symptoms whilst participating in the clinical trial?



"I had much more energy, I fatigued much less easily, I had less breathlessness, I was able to do very tiring activities that otherwise would have knocked me down more easily, I no longer had episodes of diplopia at night."

"It gave me a bit of my old self back. I could do more and felt better in lots of aspects."

