Audit of a rare disease clinical trial support service

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Introduction

Patient retention in rare disease clinical trials is crucial when considering the limited patient numbers available.

It has been reported that 18% of patients randomised into a clinical trial end up withdrawing.[1]

This number is predicted to be even higher in rare disease clinical trials.

There is considerable burden to the patient/their family to take part in a clinical trial.

Common reasons cited for why patients withdraw include:

- Financial constraints
- Location of trial
- Personal/family matters
- Condition not improving
- Side effects

When surveyed about their experiences of clinical trials, 38% of patients who withdrew from a trial reported the process of attending site visits stressful, compared with just 16% of those who completed the trial.[2]

Using a specialised trials support service, may help to reduce the burden of study visits to the patient, which in turn, may improve retention.

Aims

To determine if commissioning a specialised clinical trial support service impacts on study retention rates in rare disease studies.

Methods

An audit of a rare disease clinical trial support service was conducted in September 2019.

The audit considered numbers of patients recruited and numbers of patients who withdrew from a trial.

Five clinical trials, involving 51 sites worldwide were audited (Figure 1).

Results

The service supported **166 patients**, across **five clinical trials**: CLN-2 (Batten's disease); MPS II (Hunter's disease); MPS IIIA (Sanfilippo Type A); MPS IIIB (Sanfilippo Type B); and Fabry disease.

The level of support provided varied at an individual level, depending on the needs of the patient (and their family) and included: making travel arrangements, booking accommodation, reimbursement of any expenses and relocation to attend a trial in another country (Table 1).

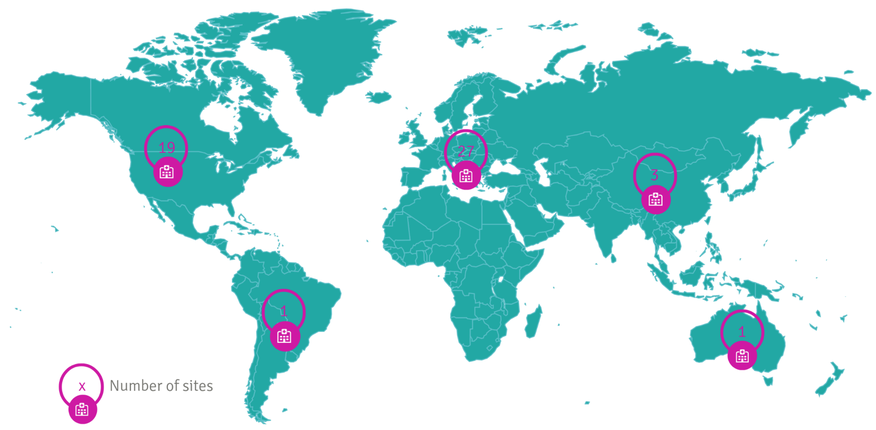

Figure 1. Clinical trial sites by continent

All patients had access to a 24 hour helpline, with access to an interpreter, if required.

Table 1. Support provided to patients attending clinical trials

Arrange travel	Book accommodation	Organise reimbursement	Coordinate relocation
Airport assistance	Suitable and safe for the patient's needs	Prompt reimbursement of expenses	Accommodation and furnishings
Priority boarding	Lift access	Different reimbursement options offered	VISAs
Expedited security	Onsite restaurants or dining in the nearby area		Daily allowance
Wheelchair spaces	As close to hospital as possible		
Companion seats	Understanding and supportive staff		
Station assistance			
Child seats in taxis			
Wheelchair accessible taxis			
Pre-booked parking			

Supporting evidence for entry into other countries

24/7 helpline, 365 days a year, with access to interpreters

The number of patients per trial ranged from 5 to 95 (Table 2).

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Table 2. Number of sites, patients recruited and withdrawn

	Number of sites	Patients recruited	Patients withdrawn
Trial I	3	21	1
Trial II	7	23	5
Trial III	4	22	3
Trial IV	35	95	8
Trial V	2	5	1

The **lowest withdrawal rate** was **4.8%**; the **highest** was **21.7%**; with a **mean** of **13.7%** (**median** of **13.6%**) across the trials audited (Figure 2).

Reasons for withdrawal were not disclosed to the service, so cannot be commented on here.

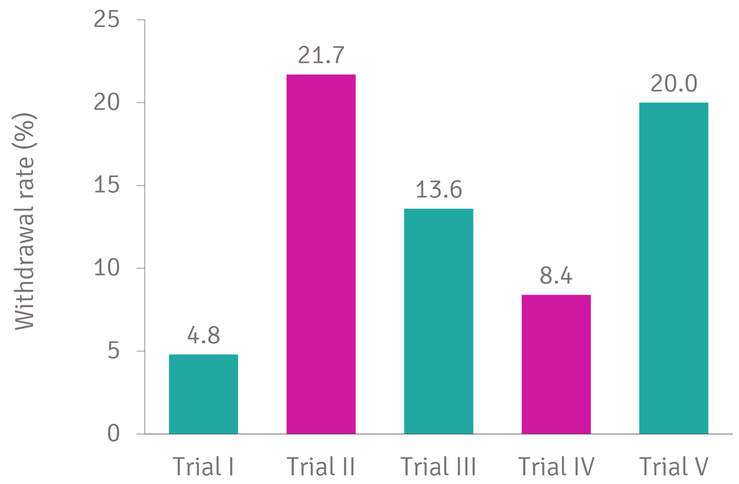

Figure 2. Trial withdrawal rates

Conclusions

Overall, this audit demonstrated a lower trial withdrawal rate in patients supported by a specialised clinical trial support service to attend site visits or relocate to take part in a clinical trial, than reported in the literature.

An additional option to the services detailed here is the provision of home study visits.

Improving the patient experience, by reducing the burden associated with taking part in a clinical trial may contribute to improved retention rates and should be considered when setting up a new rare disease study.

References

1. ADVARRA FORTE (2020) Retention in clinical trials: Keeping patients on protocols. Available from: https://forteresearch.com/news/infographic/infographic-retention-in-clinical-trials-keeping-patients-on-protocols/ (Accessed January 2020)

2. CISCRP (2013) Report on ineligible participants and those who terminate participation early. Available from: http://www.medavante-prophase.com/wp-content/uploads/2018/09/2013_ciscrp_study_ineligible_participants_and_those_who_drop_out.pdf (Accessed January 2020)