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).

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

Table 1. Support provided to patients attending clinical trials

<table>
<thead>
<tr>
<th>Service</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>Arrange travel</td>
<td>Airport assistance, Priority boarding, Expedited security, Wheelchair spaces, Companion seats, Station assistance, Child seat in taxis, Wheelchair accessible taxis, Pre-booked parking</td>
</tr>
<tr>
<td>Book accommodation</td>
<td>Suitable and safe for the patient’s needs, Lift access, Onsite restaurants or dining in the nearby area, As close to hospital as possible, Understanding and supportive staff</td>
</tr>
<tr>
<td>Organise reimbursement</td>
<td>Prompt reimbursement of expenses, Different reimbursement options offered</td>
</tr>
<tr>
<td>Coordinate relocation</td>
<td>Accommodation and furnishings, VISA, Daily allowance, Supporting evidence for entry into other countries, 24/7 helpline, 365 days a year, with access to interpreters</td>
</tr>
</tbody>
</table>

Table 2. Number of sites, patients recruited and withdrawn

<table>
<thead>
<tr>
<th>Number of sites</th>
<th>Patients recruited</th>
<th>Patients withdrawn</th>
</tr>
</thead>
<tbody>
<tr>
<td>Trial I</td>
<td>3</td>
<td>21</td>
</tr>
<tr>
<td>Trial II</td>
<td>7</td>
<td>23</td>
</tr>
<tr>
<td>Trial III</td>
<td>4</td>
<td>22</td>
</tr>
<tr>
<td>Trial IV</td>
<td>35</td>
<td>95</td>
</tr>
<tr>
<td>Trial V</td>
<td>2</td>
<td>5</td>
</tr>
</tbody>
</table>

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 1. Clinical trial sites by continent

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

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