



Conducting research in vulnerable populations can be safe, beneficial and well received if infrastructure and staff experience are appropriate to patients needs

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Introduction

- Children with disabilities are one of the most marginalized and excluded groups in society. Developing partnerships and collaborative relationships with families and other agencies are an essential means of achieving better results for every child.
- Patients with complex health care needs, severely limited communication and mobility may be less likely to participate in research studies. They also may find continued participation in such studies challenging.
- Allan Herndon Dudley syndrome (AHDS), due to defective thyroid hormone transporter (MCT8), causes severe neurological and physical disability. A multicentre trial (the TRIAC trial) studying the efficacy of the thyroid hormone analogue TRIAC in this rare condition has just been completed.

Aim

To determine if the facilities, staff and infrastructure in our Clinical Research Facility (CRF) were appropriate for patients with severe intellectual and physical disability taking part in a clinical trial.

Methods

- Ten males with AHDS were recruited to the only UK site of the TRIAC trial (Cambridge). We conducted a participant survey in order to assess their experience of taking part in research. Participants had severe learning disability with communication difficulties, so their families were invited to complete the questionnaire
- As a brief overview, participants had one overnight visit at the CRF, where they had numerous measurements taken (including echocardiogram, energy expenditure, whole body DXA scan, blood and urine tests). TRIAC was then commenced and titrated according to their Thyroid Function Test results. Repeat blood tests were taken every 2-4 weeks while on the medication. Participants had another overnight visit to the CRF at the end of the trial.

Results

10 questionnaires were sent out to participants families and 8 responses were received.

Trial Enrolment

Q1	3 / 8 Strongly agreed that they found it easy to find out about the trial. 2 / 8 Tended to find it easy with a further 3 / 8 neither agreeing or disagreeing.
Q2	5 / 8 Families found out more about the Trial from their named local Consultant. 3 / 8 Families found the information about the trial from a 'closed' facebook group for families with children who have AHDS.
Q3	5 / 8 Families stated that they got most of their information about the condition from the closed facebook group / internet. 2 / 8 Families stated that they got most of their information from the Research team co-ordinating the Triac trial at Addenbrooke's Hospital. 1 / 8 Received most of their information from their named local Consultant.
Q4	8 / 8 Stated that their local care providers and teams were not able to provide them with any information about AHDS. 8 / 8 Stated that their local care providers and teams were not able to discuss what AHDS entails to the family.
Q5	100% said a telephone interview, to obtain a short history and usual care requirements for their child, was conducted prior to trial enrolment.
Q6	100% said they were given enough time to discuss their child at the initial telephone call.
Q7	100% said that the questions adequately covered the information about their child.

Experience at Addenbrooke's Hospital

Q1	8 / 8 Strongly agreed that ward appointments were arranged at a convenient day and time for their family.
Q2	7 / 8 Strongly agreed that it was easy to park at Addenbrooke's Hospital. 1 / 8 Tended to agree that it was easy to park at Addenbrooke's Hospital.
Q3	8 / 8 Strongly agreed that the room on the research ward was appropriate for their child's needs
Q4	2 / 8 Strongly agreed that the play / entertainment facilities were appropriate for their child's needs. 1 / 8 Tended to agree that the play / entertainment facilities were appropriate for their child's needs. 3 / 8 Neither agreed or disagreed that the play / entertainment facilities were appropriate for their child's needs. 2 / 8 Indicated 'Don't Know' if the play / entertainment facilities were appropriate for their child's needs.
Q5	6 / 8 Strongly agreed that the food and drinks provided were of good quality. 1 / 8 Tended to agree that the food and drinks provided were of good quality. 1 / 8 Neither agreed or disagreed that the food and drinks provided were of good quality.
Q6	8 / 8 Strongly agreed that the staff were attentive to the needs of their child.
Q7	Addenbrooke's Hospital values are 'Together - Safe, Kind and Excellent', and are at the heart of patient care, defining the way we work and behave. 8 / 8 Strongly agreed that these values are clear in all the procedures and tests completed, by all staff, on their child.
Q8	8 / 8 Strongly agreed that all staff explained each test and investigation in a way that they understood.
Q9	8 / 8 strongly agreed that they had a good relationship with the Triac trial research team.

Feedback of results

Q1. How did you receive feedback on your child's results

Received results by telephone	5 / 8
Received results by letter	7 / 8
Face to Face	6 / 8
Other - Please comment how	No comments

Q2. Would you prefer to receive these results in a different way

Prefer to receive results by telephone	2 / 8
Prefer to receive the results by letter	2 / 8
Face to Face	3 / 8
Other - Please comment how	Not applicable - 2 / 8 Encrypted E-mail - 1 / 8

Follow up and Improvement

Q1. When the Triac trial is completed worldwide would you like to know the results	100% agreed		
Q2. How would you like this information - Please tick all that apply			
A copy of the final scientific paper written on the trial	7 / 8		
A written letter on my child only	7 / 8		
Specific medical test results on my child only	8 / 8		
A 'lay summary' of the whole trial results	7 / 8		
Other (please comment)	No comments		
Q3. Have you or any member of your immediate family been involved in a clinical trial	7 / 8 replied No, 1 / 8 replied Yes		
Q4. Are you or your family member more likely or less likely to being involved in any research trial in the future	More Likely	Less Likely	Don't Know
	7 / 8	0 / 8	1 / 8
Q5. I was able to contact the research team when I needed to	8 / 8 Strongly agreed		
Q6. I had enough time to discuss my child with the research team	8 / 8 Strongly agreed		
Q7. Overall, was your experience of the trial at Addenbrooke's Hospital what you expected	5 / 8 replied Yes		3 / 8 replied No
	Example of comments: 'Absolutely and more...best treatment given so far' 'It is very rare we can meet people like this, which understand us, our life'		Example of comment: 'I expected it to be quite clinical and didn't think we would have such a positive, warm and friendly experience'

Parents comments

'The staff involved in the trial are absolutely amazing, second to none. They have treated us with the utmost respect and dignity. When the trial is over we are going to miss them very much'
'We felt like we were at home'
'It was a great experience. We learned so much more about AHDS'
'The ward could benefit from sensory equipment i.e. lights, music and textiles'
'All the people involved with my child were great with him. He does not like tests and they all comforted him and were patient and playful'
'All, from the domestic to the Consultants, were attentive - Nothing too much trouble'
'We had no real idea what to expect so we were very pleased at the level of care and professionalism shown to all of us at all times'
'It was amazing. The room was big enough with a separate toilet. I was happy and my son. We did not miss anything'
'It was more than I expected. A lot of hospital appointments can be draining. I never felt that at Addenbrooke's'
'Patients came first but they attended to the family members as well. Brilliant'
'We would definitely like to take part in further research'

Conclusions

- Feedback from the questionnaire on staff, infrastructure and organisation of the trial was universally positive.
- Feedback suggests that play and entertainment facilities could be improved - our team will work with staff on the CRF to obtain toys and games more suited to children with severe disabilities.
- It is possible to conduct research on vulnerable populations if infrastructure and staff experience are appropriate and targeted to the participants needs.
- Research participation can be a beneficial experience for children with severe disabilities and their families, and can encourage patients to engage in further research. For this reason, researchers should not be deterred from recruiting such individuals to clinical trials, especially since they are a group of patients who may most benefit from such research.