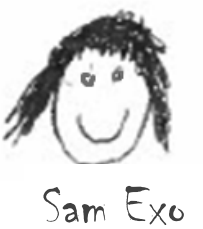


SamExo:
An External Pilot Study to Test the Feasibility of a Randomised Controlled Trial comparing Eye Muscle Surgery against Active Monitoring for Childhood Intermittent Distance Exotropia [X(T)].

Funded by the NIHR HTA Programme

Summary of Results for Participants

August 2013

Background

The main aim of the SamExo 'rehearsal' study was to find out whether parents would be willing to enrol children into a trial in which the treatment of squint would be decided randomly by a computer. The need for randomisation is important here because of a lack of evidence regarding the effectiveness and ideal timing of childhood squint surgery. Recruitment to studies involving surgery and children are particularly difficult, so it was sensible to conduct a rehearsal trial to find out if a full-size trial would be workable. We wanted to test whether recruitment to such a trial was feasible and whether participants stayed in the group to which they were randomly allocated or whether many dropped out before the trial ended. Of equal importance were interviews with parents about their reasons for agreeing, or refusing, to take part. We also wanted to compare outcomes of surgery vs. monitoring, and for this we examined clinical and 'quality of life' outcomes.

Results

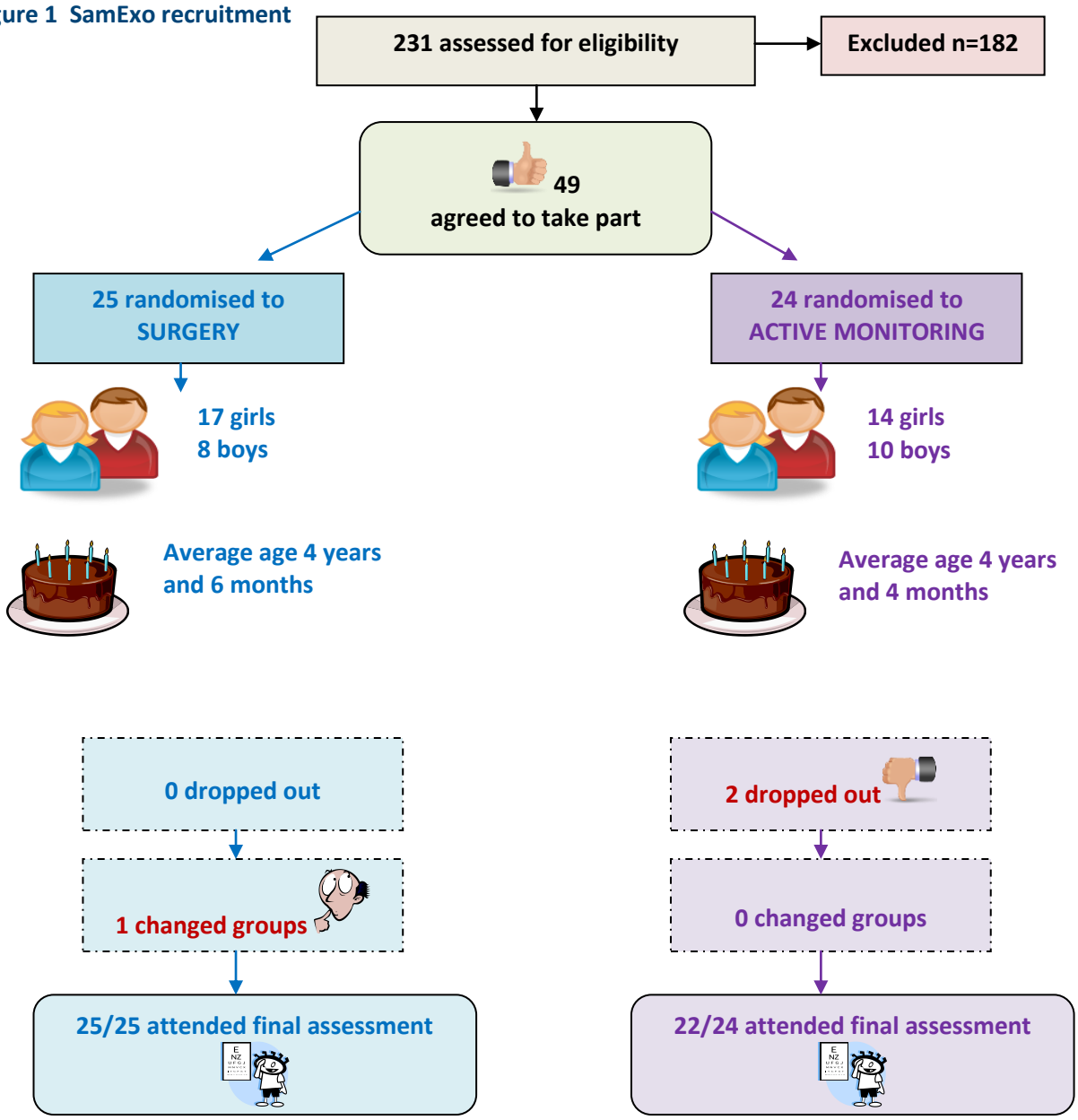
In this report we will describe the main results from the SamExo rehearsal trial. We hope some children themselves, as well as their parents, will be interested in these findings. The results can be divided into 3 main areas:

1. Recruitment success
2. Parents' views on taking part
3. Comparison of clinical and quality of life (QOL) outcomes in those who had surgery vs. those who did not

1. Recruitment success

In total, 49 parents decided that their child could be enrolled in the trial and therefore randomised to either have eye surgery right away or to 'wait and see' for 9 months before deciding whether they wanted to go ahead with an operation or other treatment, or to continue with observation only. Of these 49 children, 25 were randomised to the surgery group and 24 to the active monitoring group. Details of the age and genders of participants are provided in Figure 1. Participants were seen in clinic for follow-up assessments and monitoring at 3 months, 6 months and 9 months after recruitment (or 2 weeks, 3 months and 6 months after their operation if they were in the surgery group). By final assessment, only 2 families had dropped out of the study. So, we had an excellent retention rate with 47/49 children attending their final SamExo assessment (Figure 1).

Figure 1 SamExo recruitment



The final number of 49 recruited children was lower than we had hoped for, partly due to the fact that many children had a relatively mild squint and so were not quite eligible for inclusion in the trial. Also, as you will discover in the next section, many parents of those who were eligible did not like the idea of their child's treatment being decided 'by chance', although they would have loved to take part if they could have chosen which group their child would go into.

2. Parents' perceptions

48 parents kindly agreed to take part in a telephone interview with a university researcher who was not involved in their child's care. The purpose of these interviews was to get an idea as to why some parents agreed while others refused to take part in the trial.

 **Reasons for refusing to take part (34 parents):**	 **Reasons for agreeing to take part (14 parents):**
❖ Didn't want surgery *(felt child was too young; squint not severe enough; eyesight good)* ❖ Would rather wait and see *(may get better naturally or when child is older)* ❖ Lack of confidence in the effectiveness of surgery *(no 'miracle cure'; uncertainties regarding success rates or ideal age for surgery)* ❖ Felt the risks outweighed the benefits *(possible overcorrection of the squint; additional surgery; general anaesthetic)* ❖ Not comfortable with the idea of treatment chosen at random ❖ Distrust of the NHS ❖ Wanted surgery but didn't want to wait	❖ Felt surgery was inevitable *(and potentially waiting nine months was okay)* ❖ Reassured by the monitoring of those in the non-surgery group ❖ Felt the benefits outweighed the risks ❖ Felt that being in a trial would be beneficial in itself ❖ Reassured that surgery would be undertaken by 'a good doctor' ❖ 'Doing my bit' for research

Parents also gave us some useful ideas for improving studies like this in the future:

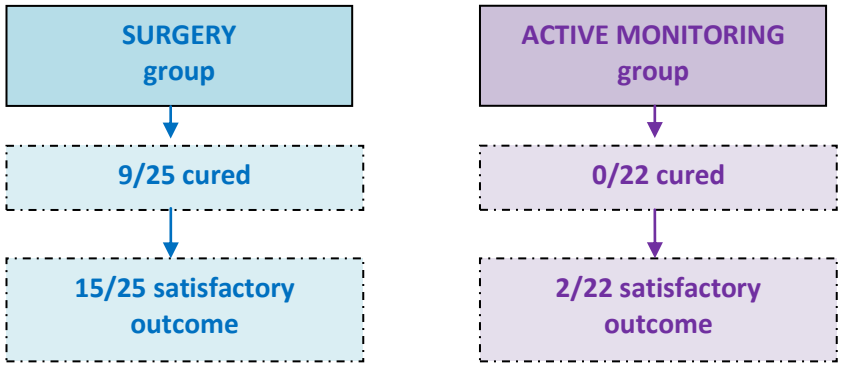
Parents' suggestions for improving the study 
❖ Provide more information *(eg, more detail about success rates; risks; costs to families)* ❖ Improve communication *(sensitivity in discussing the condition and trial at the same time)* ❖ Allow parents to choose groups ❖ Give them more time to make a decision

3. Clinical and quality of life outcomes

a) Clinical outcomes

The main clinical outcome was 'cure' rate - this was measured by a rating scale called the Newcastle Control Score (NCS). The NCS measures how well the squint is controlled and takes into account clinician and parental ratings. Scores range from 0 to 9, with 0 being 'no squint' or 'perfect control', and 9 being the worst possible score (indicating severe/constant squint/very poor control of the squint). The findings regarding cure rates and other key clinical outcomes are summarised in Figure 2, and described in more detail below that.

Figure 2 Clinical outcomes



We have to remember that this was a 'rehearsal' trial and that the number of participants was quite small. Nevertheless it was interesting to find that none of the children in the active monitoring group were 'cured' by the time of their 9-month outcome visit, whereas 9 children in the surgery group were cured at their 6-month post-surgery assessment. On the other hand, 3 children from the surgery group had constant overcorrections 6-months after their operation (meaning that the eye was now drifting inwards rather than outwards).

As well as 'cure' rates we also looked at how many children, whilst still having intermittent exotropia, had nevertheless improved in their ability to control it (as this may be considered by some people to be a satisfactory outcome). We defined a satisfactory outcome as having a NCS score no higher than 2, or having shown an improvement since recruitment by at least 3 points (remember the NCS ranges from 0 which is excellent to 9 which is the worst possible score). As can be seen in Figure 2 above, 15/25 children in the surgery group achieved a satisfactory outcome compared to only 2/22 in the active monitoring group.

b) Quality of life outcomes

The assessment of squint-related quality of life (QOL) was achieved by asking participants to complete the 'Intermittent Exotropia Questionnaire' (IXTQ). The IXTQ was especially developed by clinicians and researcher s in North America to measure parents' concerns as well as children's point of view. It includes a parental score (which reflects the anxiety and concerns of the parents themselves), a 'proxy' score (reflecting what the parents perceive their child to be concerned about), and a self-rated version which is completed by children themselves if they are old enough (5 years or over). We were particularly interested in changes in these QOL scores between recruitment and final assessment. The score ranges from 0 to 100 (0 indicates the worst/lowest outcome and 100 the best/highest outcome).

Parent scores

We found significant improvements in parents' average QOL scores within the surgery group, but no significant changes within the active monitoring group:

Surgery group		Active monitoring group	
Baseline QOL	Final QOL	Baseline QOL	Final QOL
52	↑74	53	48

Proxy scores

Although average proxy QOL scores improved in the surgery group, and deteriorated in the active monitoring group, these changes were generally insignificant:

Surgery group		Active monitoring group	
Baseline QOL	Final QOL	Baseline QOL	Final QOL
81	87	78	70

Child-rated scores

There were small improvements in child-rated scores for both treatment groups, but again these changes were not significant:

Surgery group		Active monitoring group	
Baseline QOL	Final QOL	Baseline QOL	Final QOL
73	77	74	81

Conclusions

Recruitment lower than anticipated:

- Many children had mild squints
- Parent preferences about treatment groups

Excellent retention rate:

- Only two parents withdrew from the study, and only one wanted to switch groups, which was very reassuring

Listening to parents:

- This is essential to future research and clinical practice

Outcomes of surgery:

- This was a rehearsal trial, therefore difficult to make definite conclusions
- However, SamExo provided encouraging info about squint surgery

Caution required:

- Children were assessed over 9-months with good outcomes in the surgery group but some risk of over-correction; however longer-term outcomes remain unclear

Future:

- More trials are required, and expected
- Experience from SamExo will prove INVALUABLE to future research and management of childhood squint