TREAT Trial
Patient Involvement

Terms of Reference

Project Summary

Research Title: Treating severe paediatric asthma; an open label randomised controlled trial comparing mepolizumab and omalizumab (TREAT trial)

Principle Investigator: Professor Sejal Saglani

Institution: Imperial College London

Funder: National Institute for Health Research (NIHR) – Efficacy and Mechanism Evaluation (EME)

Total Cost: £2,418,595.78

Duration: 1 May 2019 – 1 November 2024 (66 months / 5.5 years)

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Background and Research Aims

Led by Professor Sejal Saglani at Imperial College London, TREAT is an exciting new programme looking at new treatments for severe asthma in children.

Asthma is the most common lung condition affecting 5.4 million people in the UK, of which 1.1 million are children. The UK has among the highest prevalence rates of asthma symptoms in children worldwide, and a child is admitted to hospital every 20 minutes because of an asthma attack.

Approximately 2-5% of children with asthma have persistent symptoms, repeated hospital admissions with asthma attacks, school absences and poor quality of life despite being prescribed maximal doses of treatment. They use up half of all healthcare resources for asthma. Although it is recommended that such patients should be assessed in specialist clinics to establish why their asthma control is poor, there is currently no nationally agreed approach to their evaluation. A large number (60-70%) will have poor asthma control because they are not taking their asthma treatment regularly, but again there is no agreed way on how this should be identified. Those who continue to remain poorly controlled despite improved adherence and treatment have severe therapy resistant asthma.

Apart from very high dose steroids, which can result in severe side effects, there is currently only one medication that is licensed to use as an additional treatment in children with severe asthma.

Omalizumab is given as an injection in hospital but can only be used in about 60% of children with severe asthma because prescribing is limited by blood tests (called IgE) that show they are not too allergic. Of those in whom omalizumab can be tried, not all respond. Further increasing steroids leads to side effects and even very high doses do not benefit all children. Therefore, a significant number (those who are ineligible and those who do not respond to omalizumab) currently remain with troublesome asthma for which there are no new medicines to offer.

Mepolizumab is a drug that has been licenced in Europe for use in adults with severe asthma. It is also given as an injection and works by reducing the number of a specific type of cells in the lungs called eosinophils (a type of white blood cell). The safety and doses for children have been evaluated but no studies have assessed whether it helps children with severe asthma. Children with severe asthma have a lot of eosinophils in their lungs, so it is likely that mepolizumab will be beneficial.

Professor Saglani and her team will compare the effectiveness of each drug and is aiming to reduce the number of attacks by one-third compared to the previous year, as well as assess change in symptoms and the patient's and carers' quality of life. By the end of the study the team hopes to know whether mepolizumab is at least as good as omalizumab in reducing asthma attacks in children with severe asthma.

Asthma UK's role

Asthma UK is a co-applicant on this project, and our role is to ensure that the design and implementation is patient-centric. We will recruit and facilitate the Patient Advisory Group (PAG), and work with

Professor Saglani and the PAG to set up a paediatric severe asthma Patient and Public Involvement (PPI) network.

What is involved being part of TREAT?

As volunteers from Asthma UK you will be involved in the Patient Advisory Group, and a few of you might also be involved in the Trial Management Group

- **Patient Advisory Group (PAG)**

The Patient Advisory Group will be made up of 5 parent and child pairs, where the child has severe asthma and is between ages 6-16. As part of the PAG you will ensure that the trial addresses a relevant need, that all trial processes and procedures are acceptable to children (and parents) who may take part, as well as producing and reviewing patient facing documentation, and inputting on strategies for trial recruitment and retention, and dissemination of the results. The children will be able to share their thoughts and input into making sure the patient facing information is engaging and suitable.

- **Trial Management Group (TMG)**

The TMG will consist of 2 adult members of the PAG, and together with the Chief Investigator, Trial Manager and other key investigators will have responsibility for the day to day management of the study.

Becoming a patient member of the PAG and the TMG will involve:

1. **Familiarising yourself with TREAT**

This includes keeping up to date with developments throughout the project by reading meeting minutes and any other documentation sent to you.

2. **Attending and participating in meetings**

You will be asked to attend yearly in-person meetings (parents and children) and monthly/quarterly teleconferences (parents only) to discuss developments and tasks, to communicate with researchers, discuss findings and results, and together make decisions about the next stages of the research programme.

3. **Maintaining confidentiality**

It is important that all paperwork and discussions relating to TREAT remain confidential. You are welcome and encouraged to discuss the project with fellow volunteers, however due to the sensitive nature of the discussions we ask that you keep detailed information about the research and findings confidential, including in written, verbal, online and social media communications. We ask that you also clarify this with your child.

4. **Expenses and Honorariums**

All reasonable travel and out of pocket expenses will be reimbursed, as approved by Asthma UK's Research Team and in line with our **Expense Claim Form**. In recognition of your contributions a small honorarium has been allocated to each parent per year. This will be provided through vouchers after every yearly meeting.

For further details on what to expect from being a patient volunteer on a research project, please consult our **Asthma UK Volunteer PPI Framework and Standards in Research.**

What will I contribute to the TREAT trial?

Throughout this study you will provide insights and a patient's perspective into this clinical research. You will be an active partner in running the trial, supporting trial set-up and informing design, producing participant information sheets in collaboration with the Investigators and clinical trials unit and assisting in strategies to maximise recruitment, retention and in dissemination. As patient representatives you will be actively involved in the management of the study, namely:

- Ensure the research is relevant to children with severe asthma and addresses key unmet needs of this population
- Ensure the methods and outcome measures proposed for the study are acceptable and sensitive to the situations of potential research participants
- Support efficient and cost-effective recruitment and retention of research participants
- Support ongoing and wider public engagement with and participation in research via the communication of activities and findings of this study
- Support the establishment of a wider PPI paediatric severe asthma network

How much time will I need to give?

Volunteering means giving up some of your free time to be involved in a project. You should make sure that

you are comfortable with the amount of commitment needed to be involved before you join.

- TREAT is a 5.5-year long project and you and your child should join under the expectation that you will be able to stay for the full duration. However, we appreciate that circumstances may change, so if you find that you and your child are no longer able to take part, please inform Asthma UK as soon as possible so that we can identify a suitable replacement for you.
- You are expected to attend and participate in relevant TREAT meetings. Meeting dates will be scheduled with plenty of notice. The **PAG** will meet once per year, for a full day meeting in London, for a total of 5-6 times for the project duration. Additionally, there will be a quarterly teleconference for the adult members of the group. The **TMG** is anticipated to meet a total of 19 times throughout the project: monthly for the first 6 months, then every 6 months until the end of the project. It is expected that these will be 1-hour conference calls. We ask that you inform Asthma UK at least a week in advance if you are not able to attend a meeting. Please see our **Absentee Policy** for further details.
- We appreciate that you will need to work around your own schedule, so you will be provided with at least 2 weeks to work on any given tasks. We expect that you will spend ~2 hours per month on home-based tasks. However, there may be extra-ordinary instances with a quick turn-around, but we recognise that not all volunteers may be able to respond to these.
- Researchers sometimes apply for no-cost extensions to their projects if their work falls behind schedule. These are usually up to 1 year and you will be consulted if TREAT plans on doing this.

What can I expect to get back?

Being involved in research can be a hugely rewarding experience. By joining TREAT as a patient expert, you will have a chance to influence and support a possible life-changing new treatment for children with severe asthma who do not respond to the current alternatives. You and your child will be supported throughout the project by Asthma UK and recognised and valued for your contributions, perspectives and feedback.

More questions?

We're here to help! If you have further questions, would like to talk more about the TREAT trial, or have identified any needs or areas of concern, please contact Caroline at Asthma UK on cwijnbladh@asthma.org.uk or Tel: 0207 786 4936