

Treat-to-target for children with Lupus



An initial step towards target-based treatment for children with Lupus

INTRODUCTION

Systemic lupus erythematosus (often called Lupus or shortened to SLE) is an autoimmune disease. It typically starts in women between the ages of 15 and 45, but it can also start in childhood.

Symptoms can vary, but Lupus often causes tiredness, joint pain, and skin changes that are sensitive to sunlight. Lupus can also affect a person's inner organs, such as their kidneys, lungs, or brain. Compared to people who develop Lupus as adults, children and teenagers with the disease often have higher disease activity and more widespread or severe organ involvement. They can also need more medication than adults.

The aim of treatment is to reduce symptoms, and prevent long-term damage. Healthcare professionals often use targets or goals to work out if a patient is responding to treatment. Both remission and low disease activity (shortened to LDA) have been proposed as targets. Remission is when there are no signs and symptoms of active disease. Achieving remission or LDA is important, since these states have been associated with a lower probability of flares, damage, cardiovascular events, and death. LDA or remission are therefore also linked to better overall health-related quality of life, as well as less hospitalisation and lower healthcare costs.

A treat-to-target approach (T2T) is increasingly being used in other chronic rheumatic diseases. In a T2T strategy, treatment is adjusted or escalated until a specific predefined goal is achieved.

WHAT DID THE AUTHORS HOPE TO FIND?

The authors wanted to develop a set of principles to help guide the development of a T2T approach for children with Lupus.

HOW WAS THE PROJECT CONDUCTED?

This project was run by a group of healthcare professions as well as patients and parent representatives. The healthcare professionals included specialists in paediatric rheumatology, paediatric nephrology, adult rheumatology.

The authors looked at published evidence in the medical literature to collect information on T2T in children with Lupus. The experts then completed two online surveys to gather their opinions and develop some principles that could be used to help doctors use a T2T approach. They agreed on four overarching principles and fourteen points-to-consider. These final principles and points-to-consider were reviewed and endorsed on behalf of PReS – the Paediatric Rheumatology European Society.

WHAT ARE THE MAIN PRINCIPLES?

All treatment targets and strategies should be decided through shared decision-making with patients and their caregivers. But one of the main principles is that achieving full disease remission should be the main T2T goal for children with Lupus. Low disease activity can be used as an alternative goal when remission is not possible. Management should also aim to maintain the target in the long-term.

Doctors should aim to prevent flare and organ damage, and minimise the use of glucocorticoid (steroid) treatments. Additionally, they should address factors that affect a child's quality of life. This includes fatigue, pain, mental health, side effects from medication, and challenges that can affect a child's schooling.

The authors also developed a research agenda to help develop future T2T strategies.

ARE THESE FINDINGS NEW?

Yes. At present, children with Lupus are not managed with a T2T approach. This new paper is the work of leading experts who have come together for the first time to summarise existing evidence and opinion on a T2T approach for these patients.

More work is needed to validate targets and develop trials to test the approach. But previous research in adults with Lupus shows that reaching targets reduces the risk of severe flare and damage, improves quality of

life, and has cost benefits. These principles and points-to-consider are an initial step towards developing a T2T approach for children with Lupus.

WHAT ARE THE LIMITATIONS OF THE PROJECT?

The main limitation is that the principles and points-to-consider are based on published evidence, which is limited in some areas. To fill the gaps, the authors relied on expert opinion, and/or review of evidence in adults with Lupus, instead of children.

As new evidence emerges, these principles may need to be revised.

WHAT DO THE AUTHORS PLAN ON DOING WITH THIS INFORMATION?

The authors hope this guidance will provide a helpful framework for paediatric rheumatologists to follow. The research agenda should also help to strengthen the T2T approach and inform future clinical trials.

Ultimately, a trial is needed to see if this approach can improve clinical outcomes and reduce the risk of organ damage, whilst being safe and cost-effective. Work to support this is now underway.

In addition, studies focusing on T2T processes are planned to improve understanding of how T2T can be achieved within the healthcare setting, whilst recognising the increased demands for clinicians, health services, and families.

WHAT DOES THIS MEAN FOR ME?

If you or your child has Lupus, this guidance provides a framework for using treatment targets in their care. Since 2019, EULAR recommendations for adult Lupus have promoted this kind of approach. But until now there have been no similar T2T guidelines for children with Lupus.

You may not have come across the idea of targets for treatment before. Previous research in the UK showed that families had not discussed treatment targets before. But when asked, most families expressed a preference for T2T over standard care.

If you are interested in this approach you could discuss targets with your healthcare team. This will help you to find out more about how they can help to structure monitoring and influence treatment changes.

If you have any concerns about your disease or its treatment, you should speak to your doctor.

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