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Systemic therapy in juvenile localized scleroderma

Juvenile localized scleroderma, also known as morphea, is an rare condition, it occurs around 1 in 5000 children, where the skin and the tissues underneath the skin like fat tissue, muscle, tendons, and bone, get inflamed and the skin become thickened and harder. To treat this condition, it's important to have a team of specialists, pediatric rheumatologist (a doctor who specializes in treating kids with joint and autoimmune diseases) and pediatric dermatologist (pediatric skin specialist).

Before starting treatments, we need to understand how active the disease is. We recommend using a tool called LoSCAT to assess the amount and intensity of skin involvement and also look for any signs of most common extracutaneous (not involving the skin) involvement as joint and eyes (white uveitis). This assessment needs to be repeated every 3 to 6 months to judge the treatment effect and assess activity. In the assessment it is sometimes difficult to differentiate between damage and activity of the disease. A main issue is to diagnose the condition quickly and early in the disease course to be able to start treatment before any damage occurs.

Our main goal is to achieve the state of "inactive disease". We suggest a "treat to target" strategy, it means stepwise escalating treatment till reaching inactive disease, it means no tolerance for active disease. We suggest considering a patient to be in remission while on medication if they have been free of active disease for at least 12 months, and our ultimate aim is to achieve remission without the need for medication.

Systemic therapy (treatment that affects the entire body) is needed for all patients, where the lesion crosses a joint and/or any cosmetic change can occur. All systemic therapy has an anti-inflammatory effect, with the aim to stop the inflammatory process.

The first line treatment is a classical disease modifying agents Methotrexate, in a dose (15mg/m^2 body surface /week, oral or subcutaneously). If someone can't tolerate Methorexate , if we still need a medication to keep the disease asleep, we stop Methotexate and switch mycophenolate mofetil. This medicine is orally taken every day. We don't suggest using hydroxychloroquine, cyclosporine, or anti-TNF as a treatment option.

To intensify the treatment effect we apply Methotrexate or Mycophenolate in combination or a combination with more effective anti-inflammatory drug, a biologic DMARD (Tocilizumab or Abatacept both subcutaneously) or targeted synthetic DMARDs(tsDMARDs) . JAK inhibitors, tsDMARDs, are a different category of oral medications, "oral biologics", may be beneficial if the bDMARDs are ineffective. It is not clear yet, which specific JAK inhibitor is most effective for juvenile localized scleroderma. In extremely severe cases, we might consider using Rituximab after the previous treatment options.

For patients with facial involvement, we suggest autologous fat transplantation to correct the cosmetic changes as early as possible independent of background therapy. Autologous fat transplantation means, that fat cells are subtracted from the upper leg and after a preparation reinjected in the lesion in the face to have anti-inflammatory effect and volume filling effect. This treatment can significantly boost self-esteem/body image and improve the quality of life.

it is an open question the duration of inactive disease on medication before we are considering tapering or stopping the medication. Currently we suggest waiting for at least 24 months of inactive disease on medication before making any changes. If it has been challenging to achieve remission, we recommend reducing the medication dose rather than stopping it. In the future, we hope that more data will give us better guidance regarding more effective control of the disease and the best way when and how wean or discontinue medication.

Ivan Foeldvari [1], ***Edoardo Marrani*** [2]
[1]*Hamburger Zentrum für Kinder- und Jugendrheumatologie, Am Schön Klinik Eilbek, Dehnhaide 120, 22081 Hamburg, Germany*
[2]*Rheumatology Unit, AOU Meyer IRCCS, ERN ReCONNET center, Viale Gaetano Pieraccini 24, 50139 Firenze, Italy*

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Ivan Foeldvari, Edoardo Marrani
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