

# Research opportunities for children and young people with MSK\* conditions

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This leaflet contains information on current and future research studies taking place across the United Kingdom (UK) into \*musculoskeletal conditions. For updates or additions to this leaflet, please email [csgconsumers@outlook.com](mailto:csgconsumers@outlook.com).

Only brief summaries are provided, and not all inclusion and exclusion criteria are listed. If you or your child are interested in any of these research studies, please speak to your rheumatology team!



**If you are interested in a research study (even if it isn't at your usual hospital), contact your rheumatology team!**



This leaflet has been developed and is regularly updated by members of the NIHR Clinical Research Network: Children/Versus Arthritis Paediatric Rheumatology Clinical Studies Group.

**This document has been designed to raise awareness of current and future research into (but not limited to) the following childhood-onset MSK and associated conditions:**

- **Juvenile idiopathic arthritis (JIA)**
- **Enthesis-related arthritis (ERA)**
- **Psoriatic arthritis (JpsA)**
- **Systemic arthritis**
- **Autoinflammatory diseases**
- **Noninflammatory musculoskeletal pain**
- **Joint hypermobility syndrome (JHS)**
- **Ehlers-Danlos syndrome (EDS)**
- **Chronic widespread pain syndrome**
- **Fibromyalgia**
- **Complex regional pain syndrome**
- **Juvenile systemic lupus erythematosus (jSLE)**
- **Scleroderma/systemic sclerosis (SSc)**
- **Juvenile dermatomyositis (JDM)**
- **Sjogren's syndrome**
- **Vasculitis**
- **Kawasaki disease (KD)**
- **Behçet's Disease**
- **Mixed connective tissue disease**
- **Undifferentiated connective tissue disease**
- **Antiphospholipid syndrome (APS)**
- **Macrophage activation syndrome (MAS)**
- **Uveitis**

## **Current and future studies:**

*Some studies have an acronym and are in alphabetical order based on msk condition. For each study, there is a short description, a website link and email address (if available) and the name of the researcher looking after the study. The condition the research is aimed at is also summarised; this should not be considered inclusion or exclusion criteria. Please discuss this with your specialist medical team.*

If you are interested in a research study (even if it isn't at your usual hospital), contact your rheumatology team!



### ADJUST – Adalimumab in JIA – associated uveitis stopping trial

**Description:**

Children and young people (2 years and older) with a diagnosis of juvenile idiopathic arthritis-associated uveitis who are currently taking adalimumab (Humira®) or adalimumab biosimilars and are interested in stopping their medication may be eligible for this 12-month observational study.

**Condition:**

**JIA and uveitis**

**Locations:**

Bristol, Liverpool, London (GOSH), Newcastle, Sheffield, Southampton

**Chief investigator:**

Professor Athimalaipet Ramanan

**More information:**

[www.clinicaltrials.gov/ct2/show/NCT03816397](http://www.clinicaltrials.gov/ct2/show/NCT03816397)  
[ADJUST@ucsf.edu](mailto:ADJUST@ucsf.edu)

### BEAN

**Description:**

Phase 3 Study of Apremilast in Children with Mouth Ulcers from Behçet's Disease (BEAN)

**Condition:**

**Behçet's Disease**

**Locations:**

Liverpool and Birmingham

**Chief investigator:**

Clare Pain

**More information:**

<https://clinicaltrials.gov/ct2/show/NCT04528082>

### Baricitinib\*\* in systemic JIA

**Description:**

An interventional study looking at how well baricitinib, an oral Janus Kinase (JAK) inhibitor medicine, works for children and young people with systemic JIA.

**Condition:**

**Systemic JIA**

**Locations:**

Bristol, Liverpool, London (GOSH), Sheffield

**Chief investigator:**

Professor Athimalaipet Ramanan

**More information:**

[www.clinicaltrials.gov/ct2/show/study/NCT04088396](http://www.clinicaltrials.gov/ct2/show/study/NCT04088396)

**\*\*Baricitinib is available as a tablet and liquid (suspension)!**

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<b>BILAG BR</b> (Long-term safety of new treatments in the management of SLE)	
<b>Description:</b> The purpose of the research study is to assess whether newer biological treatments available to treat lupus have any side effects when used long-term in 'real life' not revealed during shorter-term clinical trials. These side effects, if any, will be compared to those seen with standard treatments for lupus such as azathioprine, mycophenolate mofetil or cyclophosphamide.	<b>Condition:</b> <b>JSLE</b>
<b>Locations:</b> For patients aged <18 years this study is currently open in Manchester	<b>Chief investigator:</b> Professor Ian Bruce
<b>More information:</b> <a href="https://sites.manchester.ac.uk/bilag">https://sites.manchester.ac.uk/bilag</a>	

<b>EMERALD</b>	
<b>Description:</b> A trial to evaluate efficacy, safety and tolerability of emapalumab in children and adults with macrophage activation syndrome (MAS).  The ongoing EMERALD study is designed to address the unmet need for efficacious and safe therapies for the treatment of MAS, particularly for patients who are refractory to high-dose GCs. At the time of writing it is closed to recruitment for patients with Still's disease associated MAS, but remains open to recruitment for MAS caused by SLE.	<b>Condition(s):</b> <b>MAS</b>  Systemic lupus erythematosus (SLE)
<b>Locations:</b> Great Ormond Street Childrens Hospital (GOSH)	<b>Chief investigator:</b> SOBI/Professor Paul Brogan (PI for GOSH)
<b>More information:</b> <a href="https://hra.nhs.uk">EMERALD - Health Research Authority (hra.nhs.uk)</a>	

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### International cohort study of systemic scleroderma

<b>Description:</b> An observational study with the aim of standardising care of children with juvenile systemic sclerosis.	<b>Condition:</b> <b>Juvenile systemic scleroderma</b>
<b>Locations:</b> International. UK centre: Liverpool, Birmingham, Leeds and other to open	<b>Chief investigator:</b> Dr Ivan Foeldvari (Hamburg) Dr Clare Pain (UK lead)
<b>More information:</b> <a href="https://www.hra.nhs.uk/planning-and-improving-research/application-summaries/research-summaries/international-cohort-study-of-juvenile-systemic-sclerosis/">https://www.hra.nhs.uk/planning-and-improving-research/application-summaries/research-summaries/international-cohort-study-of-juvenile-systemic-sclerosis/</a>	

### UK JIA BIOLOGICS REGISTER

<b>Description:</b> An observational study looking at how well biologics, biosimilars, and other advanced therapies (i.e. JAK inhibitors) work, and the long-term safety of these in children and young people..	<b>Condition:</b> <b>JIA</b>
<b>Locations:</b> Manchester. All UK sites invited to participate.	<b>Chief investigator:</b> Professor Kimme Hyrich
<b>More information:</b> <a href="https://www.rheumatology.org.uk/practice-quality/registers/juvenile-idiopathic-arthritis">https://www.rheumatology.org.uk/practice-quality/registers/juvenile-idiopathic-arthritis</a> email: <a href="mailto:Katy.Mowbray@manchester.ac.uk">Katy.Mowbray@manchester.ac.uk</a>	

### JIA VIP RESEARCH PANEL

<b>Description:</b> A longitudinal patient and parent panel survey for Juvenile Idiopathic Arthritis, developed and managed by Juvenile Arthritis Research. The Panel contributes to current JIA research in two ways: <ol style="list-style-type: none"> <li>1. By enabling people to take part in high quality research through surveys and other formats; and</li> <li>2. By supporting PPIE (Patient and Public Involvement and Engagement), helping co-develop research and enabling the voice of patients and parents to be heard.</li> </ol>	<b>Condition:</b> <b>JIA</b>
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Patients with JIA and their families can join the JIA VIP Research Panel at any time.	
<b>Locations:</b> Across the UK	<b>Chief investigator:</b> Richard Beesley
<b>More information:</b> <a href="http://www.jarproject.org/vip">www.jarproject.org/vip</a>	

<b>KD-CAAP: Kawasaki Disease Coronary Artery Aneurysm Prevention trial</b>	
<b>Description:</b> This study will work out the best way to treat children and adolescents aged between 30 days and 15 years who have Kawasaki disease. This is a randomised trial, to work out if giving corticosteroids upfront (in addition to Intravenous Immunoglobulin (IVIG) and aspirin) to children and adolescents with Kawasaki disease across Europe reduces the rate of heart complications.	<b>Condition(s):</b> <b>Kawasaki Disease</b>
<b>Locations:</b> London (UCL). 52 hospitals across the UK and 11 European countries taking part.	<b>Chief investigator:</b> Professor Paul Brogan and Professor Despina Eleftheriou
<b>More information:</b> <a href="https://kdcaap.mrcctu.ucl.ac.uk/">https://kdcaap.mrcctu.ucl.ac.uk/</a> <b>Email:</b> <a href="mailto:mrcctu.kdcaap@ucl.ac.uk">mrcctu.kdcaap@ucl.ac.uk</a>	

<b>LoSQI – Cross cultural adaptation and validation of LoSQI</b>	
<b>Description:</b> An international study to translate and validate a patient reported outcome measure (LoSQI) in juvenile localised scleroderma and compare it to other outcomes to better understand the disease and how it impacts children and young people.	<b>Condition:</b> Juvenile localised scleroderma
<b>Locations:</b> Liverpool, Newcastle, Manchester and Nottingham.	<b>Chief investigator:</b> Clare Pain
<b>More information:</b> <a href="#">PRINTO - Paediatric Rheumatology International Trials Organization</a>	

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<b>Lupus Nephritis study</b>	
<b>Description:</b> A study to evaluate the efficacy and safety of Obinutuzumab in adolescents with active lupus nephritis.	<b>Condition:</b> <b>Lupus Nephritis</b>
<b>Locations:</b> International. UK centre: Liverpool.	<b>Chief investigator:</b> Hoffman La-Roche
<b>More information:</b> <a href="#">A Study to Evaluate the Efficacy, Safety, and Pharmacokinetics of Obinutuzumab in Adolescents With Active Class III or IV Lupus Nephritis - Full Text View - ClinicalTrials.gov</a>	

<b>NIHR bioresource study of juvenile systemic sclerosis</b>	
<b>Description:</b> The observational study compares children and adults with systemic sclerosis. The aim is to deepen understanding of this rare condition by establishing a UK database including genetic biomarkers.	<b>Condition:</b> <b>Juvenile systemic sclerosis</b>
<b>Locations:</b> Multiple including Leeds, Liverpool, Birmingham	<b>Chief investigator:</b> Dr Tania Amin
<b>More information:</b> <a href="#">web-summary-jsc-v1-27082019.pdf (nihr.ac.uk)</a>	

<b>Paediatric Sjögren syndrome cohort study and Repository (PaedSSCore)</b>	
<b>Description:</b> Study aimed at gathering epidemiological data and understanding long term outcomes in this rare disease looking to recruit patients younger than 18 when diagnosed with SS as either sole diagnosis or in association with other autoimmune conditions (does not have to be rheumatic). The study involves baseline and 6 month/time of flare routine clinical, laboratory and imaging data as well as a blood sample from sites which have the capacity to support this.	<b>Condition:</b> <b>Sjögren syndrome</b>
<b>Locations:</b> London, GOSH, Newcastle, Sheffield, Southampton, Sheffield, Cambridge, Ireland	<b>Chief investigator:</b> Dr Coziana Ciurtin
<b>More information:</b> <a href="https://clinicaltrials.gov/ct2/show/NCT04528082">https://clinicaltrials.gov/ct2/show/NCT04528082</a>	

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<b>PEAPOD</b>	
<b>Description:</b> Phase 3 Study of Apremilast in Children with Psoriatic JIA	<b>Condition:</b> <b>Psoriatic JIA</b>
<b>Locations:</b> Liverpool, Nottingham	<b>Chief investigator:</b> Kishore Warrior
<b>More information:</b> <a href="https://clinicaltrials.gov/ct2/show/study/NCT02011111">Apremilast Pediatric Study in Children With Active Juvenile Psoriatic Arthritis - Full Text View - ClinicalTrials.gov</a>	

<b>STAR-JIA</b> - Steroid Treatment Trial in JIA: A randomised trial to compare effectiveness, safety and cost-effectiveness of intravenous versus oral corticosteroid induction regimens for children and young people with juvenile idiopathic arthritis.	
<b>Funder:</b> National Institute for Health Research	<b>Sponsor:</b> Alder Hey Children's NHS Foundation Trust
<b>Description:</b> STAR-JIA aims to compare the effectiveness, safety and cost-effectiveness of intravenous versus oral corticosteroid treatment for children and young people with new onset polyarticular JIA. We aim to provide evidence that can be used to inform corticosteroid-use in the treatment of JIA based on the findings of our study and use our health economics analysis to inform NHS commissioning.	<b>Condition:</b> Juvenile Idiopathic Arthritis 
<b>Locations:</b> Liverpool, Bristol, Sheffield, Cardiff, Newcastle, Oxford, London, Norfolk, Norwich, Leicester, Manchester, Leeds, Wolverhampton, Stoke-on-Trent, Gloucester, Cheltenham, Cambridge, Belfast, Preston, Watford, Nottinghamshire, Brighton and Hove.	<b>Chief Investigator:</b> Dr Clare Pain <b>Co-Chief Investigator:</b> Professor Athimalaipet Ramanan
<b>More information:</b> Email: <a href="mailto:star-jia@liverpool.ac.uk">star-jia@liverpool.ac.uk</a> Animation: <a href="https://vimeo.com/828958543/d56df9eb2e?share=copy">https://vimeo.com/828958543/d56df9eb2e?share=copy</a>	



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<b>TURTLE</b> - A randomised controlled trial of Secukinumab versus Adalimumab for the treatment of Juvenile Idiopathic Arthritis (JIA) Associated Uveitis or chronic anterior uveitis.	
<p><b>Description:</b> This trial aims to recruit 50 participants with Juvenile Idiopathic Arthritis (JIA) Associated Uveitis or chronic anterior uveitis aged between 2-18 years of age. The trial will take place over two different stages. In the first stage of the trial, 10 Adalimumab refractory patients (patients who do not respond to anti-TNF therapy) will be included. If 3 participants out of these 10 participants respond to treatment (2-step improvement in SUN grade score after 12 weeks of treatment) then the trial will move on to the second stage. In the second stage, 40 participants will be randomised to receive either Secukinumab or Adalimumab.</p>	<p><b>Condition:</b> <b>JIA associated Uveitis</b></p>
<p><b>Locations:</b> Bristol, Liverpool, Newcastle, Nottingham, London, Norfolk / Norwich, Sheffield, Leicester, Manchester, Edinburgh, Cambridge, Nottingham,</p>	<p><b>Chief investigator:</b> Professor Athimalaipet Ramanan</p>
<p><b>More information:</b> <b>Email:</b> <a href="mailto:turtle.trial@liverpool.ac.uk">turtle.trial@liverpool.ac.uk</a></p>	

<b>UK JSLE Cohort - Juvenile Systemic Lupus Erythematosus Cohort Study and Repository</b>	
<p><b>Description:</b> A study collecting clinical information and biological specimens to learn how Lupus affects children and the treatments being prescribed. Samples are assisting in researching the causes and genetics of Lupus.</p>	<p><b>Condition:</b> <b>Childhood Lupus</b></p>
<p><b>Locations:</b> Liverpool, Birmingham, Bristol, Glasgow, Edinburgh, Aberdeen, London, Leeds, Nottingham, Newcastle, Oxford, Manchester, Somerset, Bradford, Southampton, Sheffield, Leicester and Cambridge.</p>	<p><b>Chief investigator:</b> Professor Michael Beresford</p>
<p><b>More information:</b> <a href="https://www.liverpool.ac.uk/translational-medicine/research/ukjsle/about/">https://www.liverpool.ac.uk/translational-medicine/research/ukjsle/about/</a></p>	

If you are interested in a particular study but it isn't taking place at your usual hospital, ask your rheumatology team to contact the research team looking after the study. You can show this summary to your rheumatology team for more information.

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## Glossary of terms

**Efficacy** - The ability to produce a desired or intended result.

**Interventional research studies** - Interventional research studies are where people are assigned to groups that receive one or more interventions or treatments (e.g. a medicine, an exercise, an online resource), or no intervention at all. Researchers then look at the effect of the intervention or treatment on health and wellbeing.

**Observational research studies** - Observational research studies are where researchers look at groups of people with similar health conditions or treatments and collect information about them and their condition(s).

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