

Pediatric Rheumatology Research Priorities

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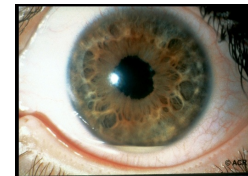
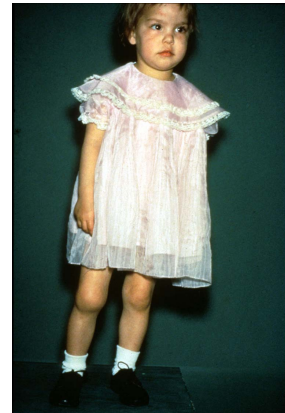
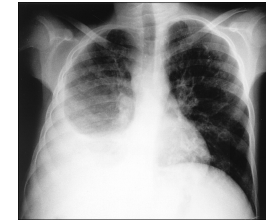
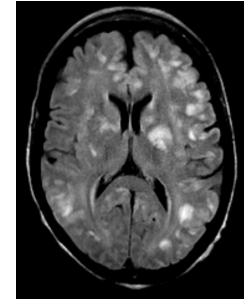
President, Childhood Arthritis and Rheumatology Research Alliance

Disclosures

- President, Childhood Arthritis and Rheumatology Research Alliance
- Professor of Pediatrics, University of California San Francisco
- Director, UCSF Child and Adolescent Chronic Illness Center
- Member, Arthritis Foundation Northern California Board and Medical & Scientific Advisory Committee

Pediatric Rheumatic Diseases

- Juvenile Idiopathic Arthritis
- Systemic Lupus Erythematosus
- Juvenile Dermatomyositis
- Vasculitis (Kawasaki, etc)
- Scleroderma
- Miscellaneous inflammatory conditions
- COVID-associated Multi-System Inflammatory Syndrome of Childhood (MIS-C)



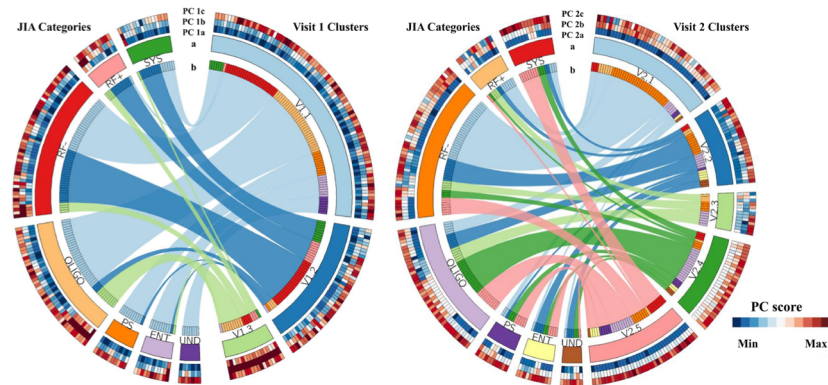
Juvenile Idiopathic Arthritis (JIA)

Many sub-types (ILAR criteria)

Sub-type	Peak Age (yrs)	Female to male ratio	Arthritis pattern	Extra-articular findings
Systemic	none	1:1	Any #, small & large	Fever, rash, serositis, MAS
Oligo-persistent	<6	5:1	≤ 4, small & med, asymmetric, not hip	uveitis
Oligo-extended	<6	5:1	≥ 4 <u>after</u> 6 mos, small & med, asymmetric, not hip	uveitis
Poly RF +	9-12	3:1	≥ 5, symmetric	Mild fever
Poly RF -	6-7	3:1	≥ 5, symmetric	Mild fever
Psoriatic	7-10	2:1	Dactylitis	Nail pits, onycholysis
ERA	9-12	1:9	Axial & peripheral, sacro-iliac	Acute uveitis

Knowledge Gap

biologic understanding & classification



Rezaei et al, Rheumatology 2020

Correlation

1	0.96	0.88	0.33	0.42	0.27	0.58	POligo
0.96	1	0.89	0.39	0.52	0.27	0.76	EOligo
0.88	0.89	1	0.35	0.61	0.22	0.72	RFnegpoly
0.33	0.39	0.35	1	0.45	0.12	0.22	RFpospoly
0.42	0.52	0.61	0.45	1	0.13	0.56	sJIA
0.27	0.27	0.22	0.12	0.13	1	0.44	ERA
0.58	0.76	0.72	0.22	0.56	0.44	1	jPsA
POligo	EOligo	RFnegpoly	RFpospoly	sJIA	ERA	jPsA	

Hinks A et al, Ann Rheum Dis 2017

Need to transition to biologic-based JIA classification

Epidemiology

- ~300, 000 children in North American have chronic arthritis
- Gender differences
 - Female > Male
 - 5:1 oligoarticular JIA
 - 3:1 polyarticular JIA
 - 1:1 systemic JIA
 - Male > Female
 - ERA
- Race/ethnicity differences
 - More common in Caucasians (in general)

Need better epidemiology by geographic region, race and ethnicity

Knowledge gap

Understanding etiology

- Genetic predisposition
 - HLA loci associations
 - Cytokine expression
- Altered immune responses
- Environmental triggers
 - Infection
 - Maternal smoking
 - Breastfeeding
 - Vitamin D/sun exposure

Disease course and outcome

improvements in disability and mortality

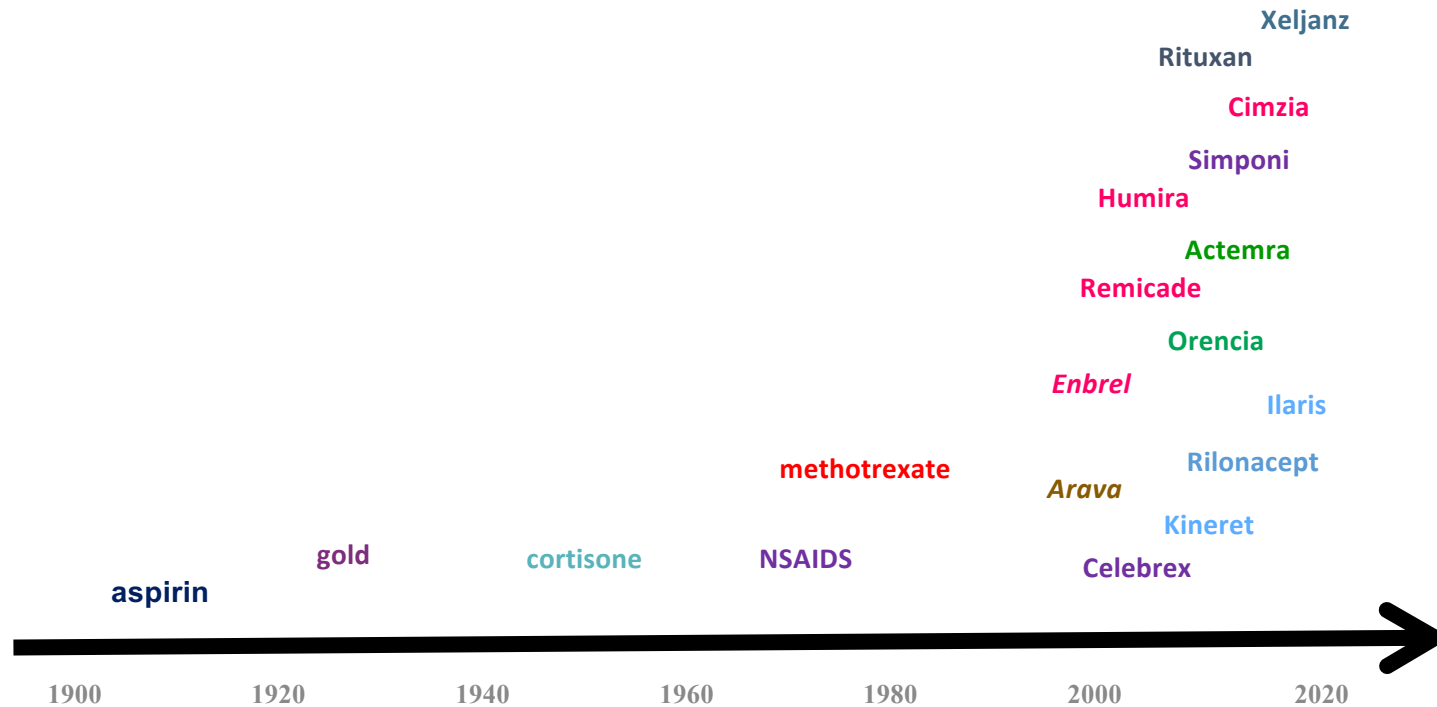


*Photos from Arthritis Foundation JA conference
1990 to present*

Now need to focus on prevention, reduction of morbidity across a lifetime,
and CURE

Advances in drug development

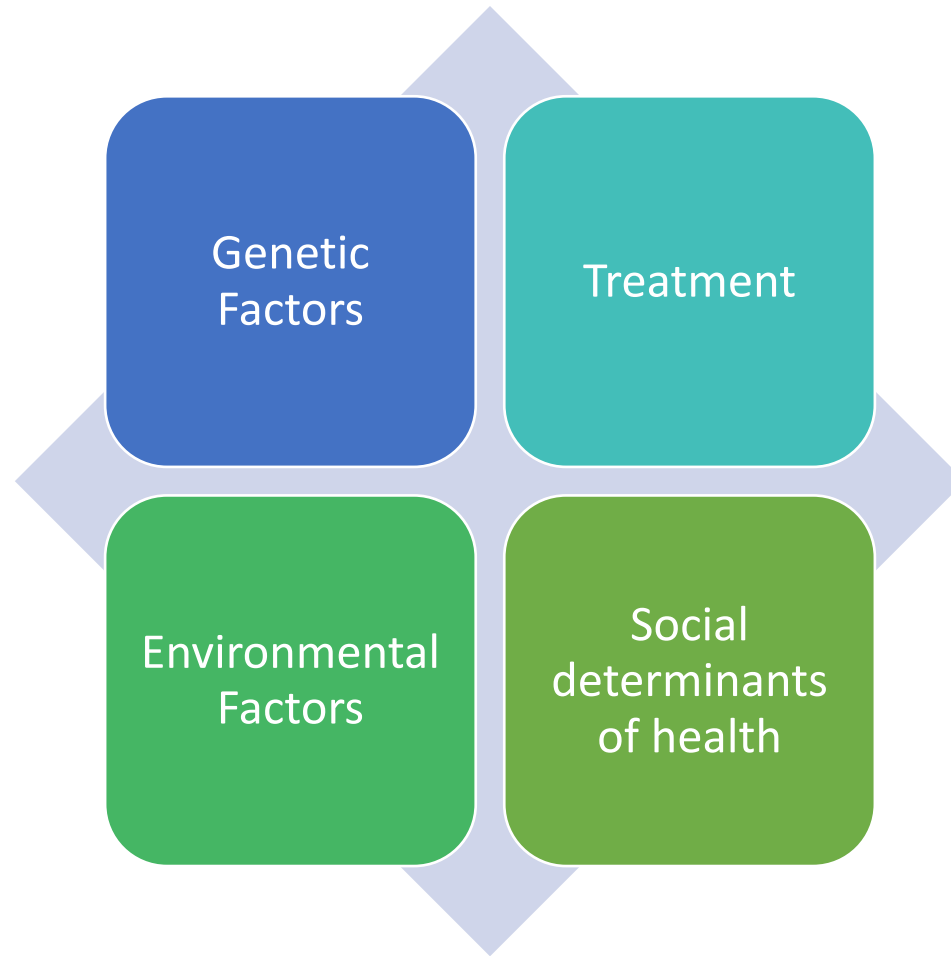
yet many patients do not respond



Need new medications, better understanding of how to use existing medications, which medication works for which patient, and when to safely stop medications

Knowledge gap

Understanding determinants of outcome



Growth and Development

Children are not just little adults



- Different diseases
- Different manifestations
- Impact on growth/development
- Different drug safety profile
- School issues
- Children come with parents

The power of partnership: CARRA & Arthritis Foundation



Childhood Arthritis and Rheumatology Research Alliance



- Investigator-led research network 501(c)(3)
- >575 members
- >175 clinical site

The CARRA Registry

- 21CFR11 compliant
- Electronic Data Capture
- 71 sites
- Physician and patient-reported data
- >10,00 patients enrolled



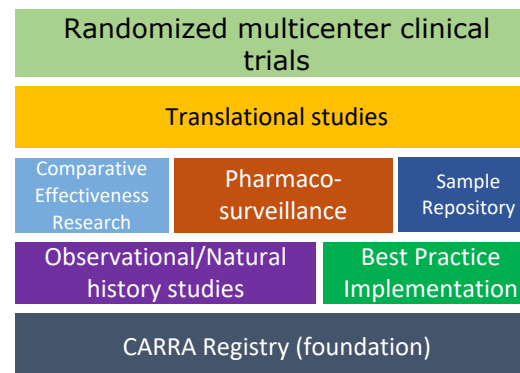
Patient Engagement



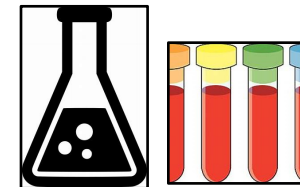
Disease Committees & Workgroups

- JIA
- Lupus
- Dermatomyositis
- Pain
- Rare Diseases
- Cross-Cutting

Research Portfolio

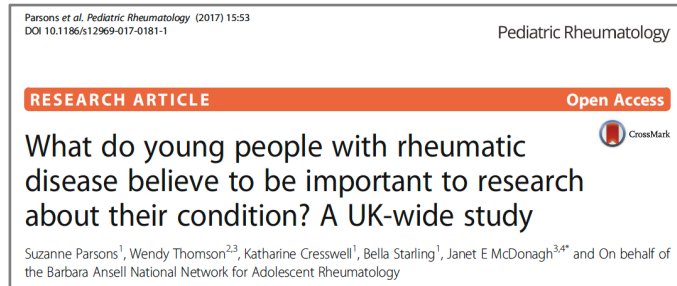


Bio-repository Translational Research



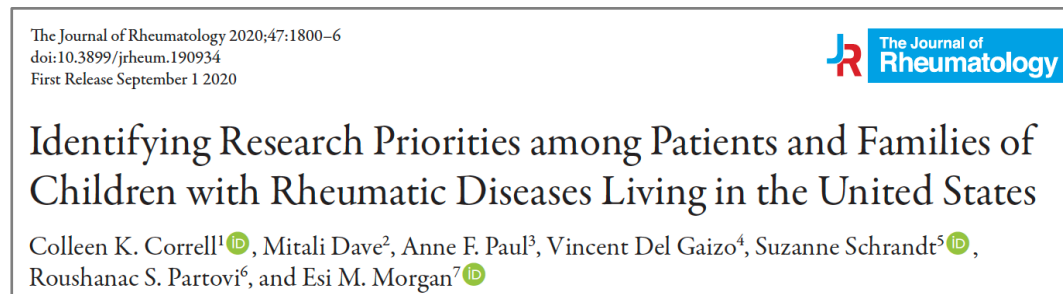
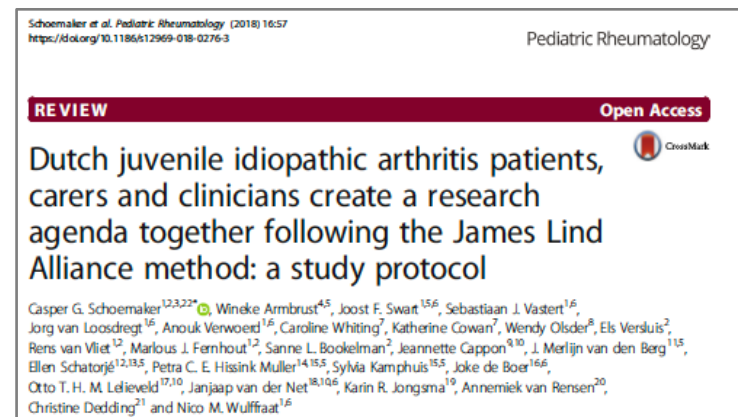
Childhood Arthritis and Rheumatology Research Alliance

Scientific Priorities: topics in health & biology

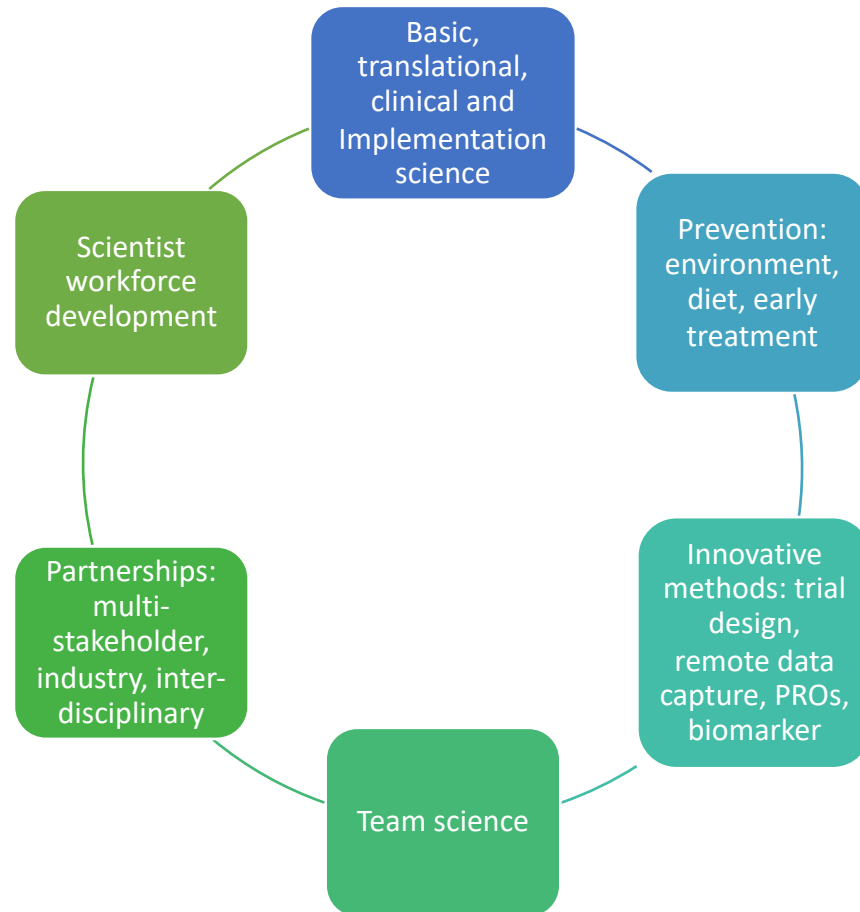


Improving musculoskeletal health for children and young people – A ‘call to action’

Helen E. Foster ^{a,*}, Christiaan Scott ^b, Carl J. Tiderius ^c,
Matthew B. Dobbs ^d, Members of the Paediatric Global
Musculoskeletal Task Force



Scientific Priorities: optimizing methods & building resources



Moving the Science of Team Science Forward Collaboration and Creativity

Kara L. Hall, PhD, Annie X. Feng, EdD, Richard P. Moser, PhD, Daniel Stokols, PhD, Brandie K. Taylor, MA

Questions