# Pediatric Rheumatology Research Priorities

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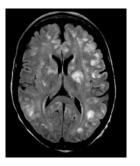
#### Disclosures

- 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)

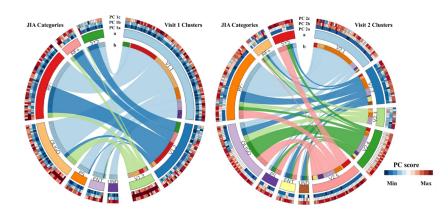
Many sub-types (ILAR criteria)

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

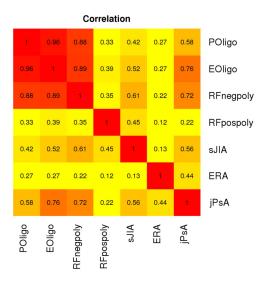


# Knowledge Gap

#### biologic understanding & classification



Rezaei et al, Rheumatology 2020



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









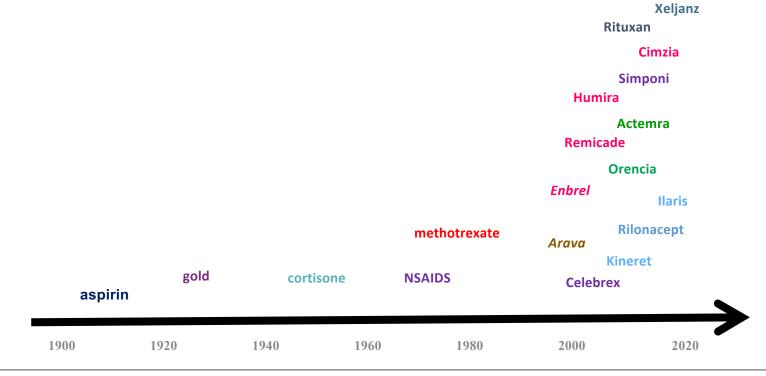
Photos from Arthritis Foundation JA conference 1990 to present

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



# Advances in drug development

yet many patients do not respond



Arthritis & Rheumatology

Ve. 72, No. 11, November 2003, pp 1945-1951
DO 2003, American College of Resourcing by

BRIEF REPORT

New Medications Are Needed for Children With Juvenile
Idiopathic Arthritis

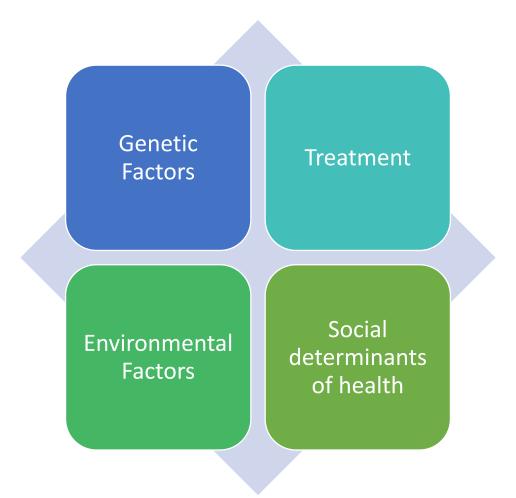
Hermine I. Brunner; Description Laura E. Schanberg, Yukiko Kimura, Anne Dennos, Dominic O. Co.,
Robert A. Colbert, Robert C. Fullbrigge, Ellen Goldmuntz, Daniel J. Kingsbury, Cathy Patty-Resk, Sandra Mintz, 1 Varson One, Use Sandra Mintz, 2 Varson One, Use Sandra Mintz, 1 Varson One, Use Sandra Mintz, 1 Varson One, Use Sandra Mintz, 2 Varson One, Use Sandra Mintz, 1 Varson One, Use Sandra Mintz, 2 V

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

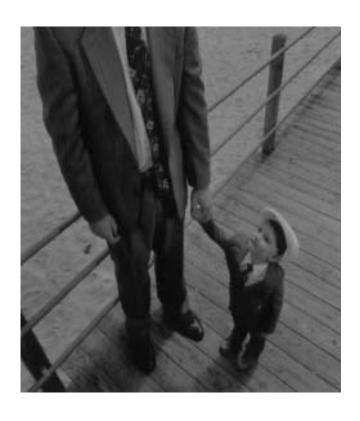
Understanding determinants of outcome





# 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



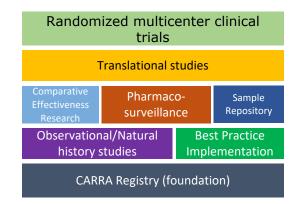
#### **Patient Engagement**



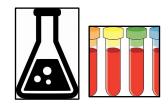
#### **Disease Committees & Workgroups**

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

#### **Research Portfolio**

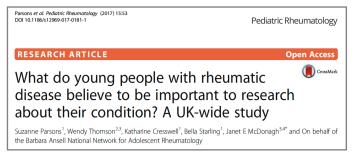


## Bio-repository Translational Research





# Scientific Priorities: topics in health & biology



The Journal of Rheumatology 2020;47:1687–95 doi:10.3899/jrheum.190361 First Release August 1 2020



Prioritized Agenda for Mental Health Research in Pediatric Rheumatology from the Childhood Arthritis and Rheumatology Research Alliance Mental Health Workgroup

Tamar B. Rubinstein<sup>1</sup>, Ekemini A. Ogbu<sup>2</sup>, Martha Rodriguez<sup>3</sup>, Lindsay Waqar<sup>4</sup>, Jennifer M.P. Woo<sup>5</sup>, Alaina M. Davis<sup>6</sup>, William Blaine Lapin<sup>7</sup>, Lawrence Ng<sup>8</sup>, Erin Treemarcki<sup>9</sup>, Emily von Scheven<sup>10</sup>, and Andrea M. Knight<sup>11</sup>, for the CARRA Mental Health Workgroup

Improving musculoskeletal health for children and young people — A 'call to action'

Helen E. Foster <sup>a, \*</sup>, Christiaan Scott <sup>b</sup>, Carl J. Tiderius <sup>c</sup>, Matthew B. Dobbs <sup>d</sup>, Members of the Paediatric Global Musculoskeletal Task Force



The Journal of Rheumatology 2020;47:1800–6 doi:10.3899/jrheum.190934 First Release September 1 2020



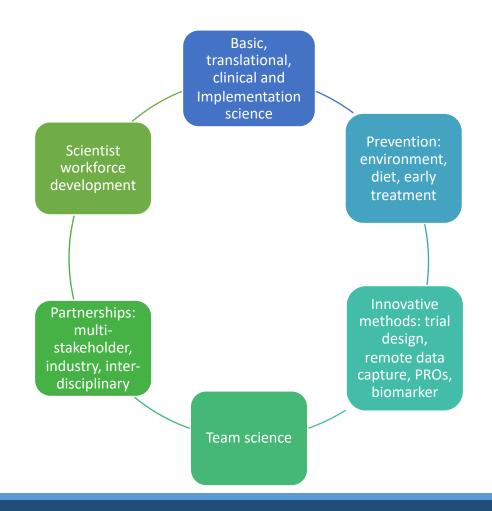
Identifying Research Priorities among Patients and Families of Children with Rheumatic Diseases Living in the United States

Colleen K. Correll<sup>1</sup>, Mitali Dave<sup>2</sup>, Anne F. Paul<sup>3</sup>, Vincent Del Gaizo<sup>4</sup>, Suzanne Schrandt<sup>5</sup>, Roushanac S. Partovi<sup>6</sup>, and Esi M. Morgan<sup>7</sup>





# Scientific Priorities: optimizing methods & building resources



American College

\*/\*RHEUMATOLOGY

\*\*Bytheumatology

\*\*With The 12 December 2018 pp 1976-1984

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\*\*Dot 16-1002/dr.461049

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#### 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

