Childhood Arthritis and Rheumatology Research Alliance

CARRA

2019 ANNUAL SCIENTIFIC MEETING

SAVE THE DATE

LOUISVILLE, KENTUCKY

APRIL 10-14
MARRIOTT LOUISVILLE DOWNTOWN

We hope to see you there!
Meeting at ACR
6:30 - 7:30 PM

ROBERT FUHLBRIGGE, MD, PHD
PRESIDENT, CARRA, INC.
Reminders
CARRA Highlights
Meeting our Patients’ Needs: Two Important Initiatives
Q&A
Download the CARRA Meeting App

About CARRA

CARRA and other Pediatric Sessions
  • Links to maps of locations

Arthritis Foundation Related Sessions
CARRA-Related Abstracts: Sunday

- **Kappa-Deleting Recombination Excision Circles (KREC) in B Cells and Serum B Cell Activating Factor (BAFF): Possible Aids in Predicting Juvenile Dermatomyositis Response to Rituximab (Poster)** (Amer Khojah, Victoria Hans, Wilfredo Marin, Gabrielle A. Morgan and Lauren M. Pachman)*

- **Immunomodulatory Medication Use for Youth with Newly-Diagnosed Systemic Lupus Erythematosus (Poster)** (Alaina M. Davis, Marisa S. Klein-Gitelman, Jennifer Faerber, Hannah Katcoff, Zuleyha Cidav, David Mandell and Andrea M. Knight)*

- **Steroid Use in Pediatric Proliferative Lupus Nephritis (Poster)** (Nathalie Chalhoub, Jianghong Deng, Natasha M. Ruth, Stacy P. Ardoin, Mileka Gilbert, Theresa Hennard, Linda Hiraki, Paul T. Jensen, Andrea M. Knight, Rebecca Kunder, Laura Lewandowski, Siok Hoon Lily Lim, Angela Merritt, Sonia Iqbal Savani, Mary Beth Son, Emily von Scheven, Scott E. Wenderfer and Hermine I. Brunner)


- **Pro-Inflammatory High Density Lipoprotein Function Is Associated with Accelerated Carotid Intimal Medial Thickness Progression in Childhood-Onset SLE** (Jennifer C. Cooper, Stacy P. Ardoin, Emily von Scheven, Laura E. Schanberg, Brian Skaggs and Maureen A. McMahon)

- **The Association of Short-Term Ultraviolet Radiation Exposure and Calcinosis in Juvenile Dermatomyositis in the Childhood Arthritis & Rheumatology Research Alliance Registry** (Jessica Neely, Craig S. Long, Hugh Sturrock and Susan Kim)

- **Stopping Medicines for Inactive Juvenile Idiopathic Arthritis: What Do Patients and Families Consider?** (Daniel B. Horton, Jomaira Salas, Aleksandra Wec, Timothy Beukelman, Alexis Boneparth, Jaime Guzman, Ky Haverkamp, Melanie Kohlheim, Melissa L. Mannion, Nandini Moorthy, Elizabeth Stringer, Lori Tucker, Sarah Ringold and Marsha Rosenthal)*

*Projects funded via a CARRA-Arthritis Foundation Grant*
CARRA-Related Abstracts: Monday

- **Impact of Juvenile Localized Scleroderma on Longitudinal Quality of Life (Poster)**
  Vidya Sivaraman, Kathryn S. Torok, Melissa Moore-Clingenpeel, Fatma Dedeoglu, Polly Ferguson, Elena Pope, Thomas G. Mason II, Mara L Becker, Gloria Higgins, C. Egla Rabinovich, Sandy Hong, Maria Ibarra, Ronald M. Laxer, Robert Fuhlbrigge14, Katie Stewart, Marilynn Punaro and Suzanne Li

- **Research Priorities for Addressing Mental Health Needs of Pediatric Patients with Rheumatologic Disease (Poster)** (Tamar Rubinstein, Lindsay Waqar, Jennifer Woo, Ekemini Ogbu, W. Blaine Lapin, Lawrence Ng, Erin Treemarcki and Andrea M. Knight)

- **Engaging Patients and Parents to Improve Mental Health for Youth with Rheumatologic Disease (Poster)**
  (Oluwatunmise Fawole, Michelle Vickery, Lauren Faust, Tamar Rubinstein, Julia Harris, Aimee O. Hersh, Karen Onel, Erica Lawson, Emily von Scheven, Kaveh Ardalan, Esi Morgan, Anne Paul, Judith Barlin, R. Paola Daly, Mitali Dave, Shannon Malloy, Shari Hume, Suzanne Schrandt, Laura C. Marrow, Angela Chapson, Donna Napoli, Michael Napoli, Miranda Moyer, Rachel Adamski, Vincent Del Gaizo, Martha Rodriguez and Andrea M. Knight)*

- **Screening Youth with Lupus for Depression and Anxiety in Pediatric Rheumatology Clinics (ACR Concurrent Abstract Session)**
  (Tamar Rubinstein, Marija Dionizovik-Dimanovski, Chelsey Smith, Raphael Kraus, Jordan T. Jones, Julia Harris, Martha Rodriguez, Lauren Faust, Beth Rutstein, Rebecca Puplava, Melissa Tesher, Alaina M. Davis, Karen Onel, Sangeeta Sule, Emily von Scheven and Andrea M. Knight)*

* Projects funded via a CARRA-Arthritis Foundation Grant
CARRA-Related Abstracts: Tues. and Wed.

• Tuesday
  • The Childhood Arthritis and Rheumatology Research Alliance Start Time Optimization of Biologic Therapy in Polyarticular JIA Study: Updated Report of Baseline Patient Characteristics and Treatment Choices (Poster) (Sarah Ringold, George A. Tomlinson, Pamela F. Weiss, Laura E. Schanberg, Mary Ellen Riordan, Anne C. Dennos, Vincent Del Gaizo, Katherine Murphy, Brian M. Feldman and Yukiko Kimura)

• Wednesday
  • Differences in Healthcare Transition Views, Practices, and Barriers Among North American Pediatric Rheumatology Providers from 2010 to 2018 (ACR Concurrent Abstract Session) (Kiana Johnson, Cuoghi Edens, Peter Chira, Aimee O. Hersh, Y. Ingrid Goh, Joyce Hui-Yuen, Rebecca E. Sadun, Nora G. Singer, Lynn R. Spiegel, Jennifer N. Stinson, Patience H. White and Erica Lawson)
CARRA has formed a National Team for the Arthritis Foundation!

• Walk teams
• Jingle Bell Run teams
• Start a new team or link your existing team to CARRA’s Team
• See AF table at the Reception
• Talk to CARRA’s Vincent Del Gaizo or the Arthritis Foundation’s Martha Snyderman
CARRA Registry and Research Updates
CARRA Registry Enrollment

Target: >10,000, followed for >10y

Status: 6,762 enrolled (Oct 12, 2018)
CARRA Registry

71 sites (63 activated)
6762 enrolled
• 6106 JIA
• 327 SLE & Related Conditions
• 42 JDM/JPM

Scleroderma enrollment launching soon at 17 SCORE sites

150 new JIA biosamples collected
• JDM sample collection to begin soon

Totals as of October 10, 2017
Large-Scale CARRA CTP CER Studies In-Progress

**STOP-JIA**

CARRA pJIA CTPs: Comparing timing of starting biologics in new onset pJIA

Funded by PCORI

- Co-PIs: Kimura, Ringold

**Enrollment completed:** 402/400 enrolled

131 biosamples collected (ongoing) for translational studies

- Precision Decisions to STOP-JIA
- PROMOTe

**FROST**

CARRA sJIA CTPs: Biologic vs Non-biologic treatment in new onset sJIA

- Biologic (IL-1 or IL-6) CTPs
- Non-biologic (Steroids or MTX) CTPs

Funded by Genentech

- Co-PIs: Kimura, Beukelman, Nigrovic

40 of 200 enrolled, 13 samples collected (four-year study)

Collecting home PROs using smart phones and tablets

Biosample collection funded by Systemic JIA Foundation
Additional CARRA Studies

• PEPR
  • PRO validation in JIA and Lupus
  • Enrollment complete: 451/450 enrolled

• PROMOTE
  • MTX pharmacogenomics predictors of MTX response in poly JIA

• Ana-STILLS
  • Systemic JIA product registration trial (anakinra)

• LIMIT JIA
  • RCT of abatacept to prevent JIA extension and/or uveitis
  • Funded by PCORI
Biosample Repositories & Collection

CARRA Biorepositories established
- US: Cincinnati Children’s
- Canada: Toronto Hospital for Sick Children

Biosample Collections:
- STOP-JIA (Precision Decisions)
  - Biomarkers to predict response in new untreated poly JIA
- FROST
  - Optimal first line tx for systemic JIA patients
- PROMOTe
  - MTX pharmacogenomics predictors of MTX response in poly JIA
- SCORE
  - Biomarkers in localized scleroderma
- JDM/JPM
  - Sample collections starting soon
## CARRA-Arthritis Foundation Intramural Grants

<table>
<thead>
<tr>
<th>Grant Type</th>
<th>Max Award Value</th>
<th>Number Awarded to Date</th>
<th>Total $ Awarded to Date</th>
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<tr>
<td>Small Grants</td>
<td>$25k</td>
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<td>Large Grants</td>
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<td>Senior Fellow Grants</td>
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<tr>
<td>Biostatistics/Data Analysis Support Grants</td>
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<td>Publication Support Grants ((NEW))</td>
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<tr>
<td><strong>Total</strong></td>
<td></td>
<td></td>
<td><strong>$1,148,574</strong></td>
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1<sup>st</sup> grants awarded in June 2016
CARRA Registry Associate and Intern Positions

- Registry Associate (15% effort)
  - Mary Beth Son
  - Past Registry Intern
  - Oversight and mentorship of current Registry Interns

- Registry Internship (25% effort)
  - Two 2nd year interns (CJ Inman, Marinka Twilt)
  - Two 1st year interns (Colleen Correll, Natalie Shiff)
  - Registry operations and investigator support
  - Registry-based research projects

- Informatics Internship
  - Jon Hausmann
  - Informatics infrastructure
  - Use of social media to engage participants and improve long term follow up
Early Investigator Development/ Travel Awards

- PReS travel awards
  - 2 per year to present abstracts and interact with international colleagues
- Utrecht Summer School
  - 2 per year to attend translational research course
- Registry Analysis Manuscript RFA’s
  - 2 awarded in 2018 (Schiff, Rumsey)
  - 2019: RFA for new registry analysis proposals coming soon
CARRA-AF Coordinator Pilot Awards – 4/1/2018 – 3/30/2019

• $25,000 one-year grant to support research coordinator effort for the Registry

• Goal to support **long-term sustainability** for a CARRA registry coordinator at sites

• Funding provided directly from the Arthritis Foundation to the sites
CARRA-Arthritis Foundation Registry Coordinator Pilot Award Program

8 Pilot Sites (from West to East):
Randall Children’s Hosp (Portland OR)
Texas Children’s Hosp/Baylor (Houston TX)
Children's National Med. Ctr. (Washington DC)
Univ. of Iowa Hosp (Iowa City IA)
St. Louis Children’s Hosp (St. Louis MO)
Riley Children’s Hosp (Indianapolis ID)
Shands Children’s Hosp (Gainesville FL)
Levine Children’s Hosp (Charlotte NC)
CARRA in 2018: A Non-Profit Governed by a Board of Directors

- Board of Directors
  - Finance Committee
  - Research and Registry Oversight Committee (RROC)
  - Elections Committee
  - CARRA Executive Committee
  - Ethics Committee
  - Audit Committee
  - Board Development & Governance Committee
  - External Advisory Council
- CARRA Steering Committee
- DCRI
  - Clinical and Data Coordinating Center
    - Site Management
    - Contracts
    - Data Management
    - Regulatory Compliance
    - Safety Reporting
    - Call Center
- 71 Registry Sites
  (63 activated)
- 550+ Members
- 140+ Sites

- Registry Clinical/Interventional Study Teams
- Registry Pharmacosurveillance Study Team
- Registry Biorepository
- Registry Quality Improvement Program
- Registry Technology Program

Registry Operations Committee

Site Management
Contracts
Data Management
Regulatory Compliance
Safety Reporting
Call Center
CARRA Strategic Framework 2019–2021

**Membership**
- Increase the Involvement of Sites in Research
- Develop Investigators and Encourage Their Involvement in Research
- Ensure a Robust Leadership Pipeline

**Sustainability**
- Ensure Financial Stability and Relationship with Stakeholders

**Mission:** Conduct collaborative research to prevent, treat and cure pediatric rheumatic diseases.

**Research**
- Develop Translational Research and the Biosample Repository
- Emphasize Research from Bench to Bedside

**Partnerships**
- Emphasize International Research Collaborations
- Strengthen Strategic Alliances/Partnerships with North American Organizations
- Enhance Collaboration with Other Specialties
- Strengthen Partnership with the NIH
- Strengthen Relationship with Industry
Important Dates

January 8:
• Abstracts Due for the CARRA Annual Meeting
• Pediatric Residents Program Applications Due

February 3-4
• CARRA Registry Site PI and Coordinator Retreat—Orlando, FL
• Details to be provided to sites soon

February 15 (previously Mar 1):
• Small Grants, Fellows Grants Due

February 18:
• CARRA Registry Intern Applications Due

April 10-14:
• CARRA Annual Meeting- Louisville, KY
Meeting our Patients’ Needs:
Two Important Initiatives

YUKIKO KIMURA, MD
PAST PRESIDENT, CARRA, INC.
New Approaches to Evaluate the Efficacy and Safety of Therapeutics for Juvenile Idiopathic Arthritis (JIA)

Externally-led FDA Patient Focused Drug Development Meeting
New Approaches to Evaluate the Efficacy and Safety of Therapeutics for JIA

CARRA Sponsored Discussion (April 22, 2018)

Participants:
• Patients/parents
• Arthritis Foundation
• Clinicians/investigators/clinical trial experts
• Industry representatives
• Regulatory agencies (FDA and EMA)
The Problem

Need rapid authorization of new JIA medicines

Too few JIA patients

- Rare disease
  - Often well-controlled on currently available treatments

- Pts unwilling to enroll in trials as currently designed

- Need for better/more meaningful outcomes in trials

- Many new classes of medicines

- Many “me-too” medicines in same class
New Approaches to Evaluate the Efficacy and Safety of Therapeutics for JIA

Potential solutions

• Use of new and innovative trial designs
• Use of outcomes that are meaningful and informative
• Better use of registries to evaluate safety
• More open/frequent dialogue: investigators, patients/parents, industry, regulators

Manuscript coming soon!
Externally-led FDA Patient Focused Drug Development Meeting

Led by Arthritis Foundation and CARRA
August 2, 2018 (Washington DC)

Inform FDA and industry about Patient/Caregiver viewpoints
  • Symptoms that aren’t being addressed
  • Burden of disease and treatments
  • Getting new treatments to market quickly
  • Improving clinical trials in JIA: methods, outcomes, participation

Prior to meeting: focus groups, on-line surveys
At meeting: panelists, in-person and on-line participation
“Voice of the Patient” Report will be published
Panelists

Guy Eakin, PhD
  • Senior Vice President, Scientific Strategy for the Arthritis Foundation

Vincent Del Gaizo
  • Director of Strategic Partnerships & Patient Engagement for CARRA

Suzanne Schrandt
  • Director of Patient Engagement for the Arthritis Foundation

Corinne Pinter
  • Parent of a JIA Patient
Nick Kim Presenting at PFDD Meeting

Click here to play a video of Nick Kim Presenting at PFDD Meeting
Questions & Comments from the Audience
Tonight!
• 7:30-10pm
• 2nd Level, Prairie Room
• Hors d'oeuvres
• Open bar until 9pm, cash bar 9-10pm
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