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

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Abstract

Objective: To improve the quality and participation in pediatric rheumatology research, patient-prioritized studies should be emphasized. We collaborated with United States based pediatric rheumatology advocacy organizations to survey patients and caregivers of children with rheumatic diseases to identify what research topics were most important to them.

Methods: We conducted web-based surveys and focus groups (FG) of patients and caregivers of children with juvenile myositis (JM), juvenile arthritis (JA), and childhood-onset systemic lupus erythematosus (cSLE). Surveys were emailed to listservs and posted to social media sites of JM, JA, and cSLE patient advocacy organizations. An initial survey asked open-ended questions about patient/caregiver research preferences. Responses were further characterized through FGs. A final ranking survey asked respondents to rank from a list of research themes the seven most important to them.

Results: There were 365 (JM), 44 (JA), and 32 (cSLE) respondents to the final ranking survey. The top research priority for JM was finding new treatments, and for JA and cSLE was understanding genetic/environmental etiology. The three prioritized research

themes common across all disease groups were medication side effects, disease flare and disease etiology.

Conclusions: Patient-centered research prioritization is recognized as valuable in conducting high-quality research, yet there is a paucity of data describing patient/family preferences, especially in pediatrics. We used multimodal methodologies to assess current patient/caregiver research priorities to help frame the agenda for the pediatric rheumatology research community. Patients and caregivers from all surveyed disease groups prioritized the study of medication side effects, disease flares, and disease etiology.

Introduction: Juvenile arthritis (JA), childhood systemic lupus erythematosus (cSLE) and juvenile myositis (JM) are three of the most prevalent rheumatic diseases of childhood(1–3). There is no cure for these diseases and they often require long-term and sometimes complex medication regimens, and can be associated with functional disability(4,5). Patients and caregivers are invested stakeholders in these diseases

given the lifelong impact on the patient's quality of life. However, researchers rarely engage patients and/or caregivers to assess their research priorities in pediatric rheumatic disease and other chronic diseases of childhood. Odgers, et al. conducted a systematic review of studies assessing stakeholders'—defined as patients, caregivers/families, and healthcare providers— research priorities in chronic diseases of childhood, and found only 83 such studies in the medical literature(6). The review found that only 20 studies included caregivers'/families' research priorities and only four included children's priorities—none of these were related to pediatric rheumatic diseases. More recently, studies from the United Kingdom (UK) and Australia have engaged patients with pediatric rheumatic diseases in setting research priorities,(7,8) but there has been a clear scarcity in these studies. Understanding patient and caregiver research priorities would likely increase patient participation in research, help influence treatment approaches, and improve care. Our goal for this study was to survey patients and caregivers of patients with JM, JA and cSLE to identify what research topics were most important to them. To conduct this survey, we collaborated

with three patient advocacy organizations in the United States (US)—Cure JM, Arthritis Foundation (AF) and Lupus Foundation of America (LFA).

Materials and methods: The study was developed in collaboration with Patients, Advocates and Rheumatology Teams Network for Research and Service (PARTNERS)(9). PARTNERS is supported by Patient-Centered Outcomes Research Institute (PCORI), a US government-sponsored institution aimed at promoting patient-centered outcomes research(10). PARTNERS brings together five leading institutions in rheumatology including Cure JM, AF, LFA, Childhood Arthritis and Rheumatology Research Alliance (CARRA) and Pediatric Rheumatology Care and Outcomes Improvement Network (PR-COIN) to help conduct research on childhood rheumatic diseases with the patient's and parent's voice at the center(9). A PARTNERS Research Committee, with representation from each affiliated organization, parent stakeholders, and rheumatology researchers, worked with the lead investigator (CKC), who developed survey questions and FG guides with input and feedback from the committee. Cure JM, AF, and LFA are all

pediatric rheumatology organizations in the US. CARRA and PR-COIN are mainly US organizations, though do include a few international centers. The approach was modified from published methodology(11,12). The surveys were programmed using Research Electronic Data Capture (REDCap)(13) and Qualtrics software (Copyright © Qualtrics, Provo, UT, USA).

Step 1) Open-ended survey: A link to an electronic survey (created using REDCap) was emailed to members of Cure JM, AF and LFA patient/family listservs and posted on their respective social media sites. The survey links were sent in November 2016, January 2017, and March 2017 for Cure JM, AF, and LFA, respectively. Parents, patients 13 years of age or older and other caregivers were invited to participate. The first question of the survey asked if the respondent was 13 years or older and if the respondent answered "no" to this question the survey closed. The survey link included three open-ended questions to assess what concerns they found most important. These open-ended questions were: 1) What concerns do you have about your/your child's health and wellbeing? What keeps you up at night? 2) Which specific questions or problems do you

wish you/your child's doctors could fix? 3) In addition to finding a cure, what specific areas would you like research to focus on in the next five years? Respondents were asked to type their responses into an open-text data field. These descriptive responses were reviewed and categorized by CKC. Common themes were identified from the open-ended survey for each of the patient advocacy communities individually (Cure JM, AF, LFA).

Step 2) Focus Groups (FGs): These themes were further characterized through two FGs for parents of those with JM and JA. For JM, a FG was held in February 2017 at the Cure JM National Conference in Austin, Texas. Parents were notified of the FG prior to the conference and were able to sign-up the same day. Participation in the Step 1 survey was not an exclusion or inclusion criteria for the FG. The FG was facilitated by CKC. It was audio-recorded and transcribed to supplement notes taken during the session. For JA, a FG was held in June 2017 at the University of Minnesota (UMN). The group was led by a UMN professional community engagement facilitator. Parents were notified of the FG by email sent by the AF Minnesota Chapter and via flyers given to patients at the UMN pediatric rheumatology clinic. This session was audio-recorded and

CKC was present throughout the FG and took notes. Due to lack of resources, we did not conduct a FG for cSLE.

Step 3) Ranking survey: A final ranking survey was created (using Qualtrics software) based upon the themes identified via the open-ended survey and FGs. This ranking survey was emailed to the Cure JM, AF, and LFA listservs and a link was posted on their respective social media sites. Survey respondents were asked to rank the seven themes most important to them. Responses were weighted such that for each individual's rank, the first-ranked theme was scored 7 points, the second-ranked theme was scored 6 points, etc., and the seventh ranked theme was scored 1 point. Each theme's score was added across all respondents to give a final score.

We did not exclude respondents from the first survey or participants in the focus groups from responding to the final ranking survey. Therefore it is possible that the same individual completed the first survey, participated in the focus group, and completed the

final survey. Response rates could not be calculated from any of the surveys because the number of potential respondents from social media sites is unknown. However, based on number of email addresses included in the Cure JM, AF, and LFA listservs respectively, the maximum reach for each organization was approximately 2,500, 16,590, and 86. This project was deemed as not human subjects research by the UMN institutional review board (IRB). The study was approved by Duke Clinical Research Institute IRB (Pro00074584).

Results:

Step 1) Open-ended Survey (Table 1)

There were 138 (77% parents), 57 (93% parents) and 47 (55% parents) respondents to the open-ended survey by JM, JA, and cSLE disease groups, respectively. All research themes for each disease group can be seen in Table 1. For JM, the three most frequently stated themes were long-term effects of medications, long-term effects on overall health, and triggers for disease flare. For JA, the three most frequently stated

themes were long-term effects of medications, long-term effects on overall health, and discovery of new treatments. Two additional commonly reported themes were managing pain and understanding disease etiology. For cSLE, the three most frequently reported themes were long-term health, effects of the disease on organ systems, and desire for a cure.

Step 2) FG:

Juvenile myositis: Nine parents of children with JM participated in the FG. We did not collect demographic data from participants, however, the FG included parents of children with who were diagnosed less than one year prior and parents of children with longstanding disease. The goal of the FG was to gain additional qualitative insight into the three most common themes that were identified based on the open-ended survey. Patient quotes from the FGs are in Table 2. In regards to long-term health, parents identified several specific concerns. These included organ-specific concerns including cardiac, ocular, musculoskeletal, cutaneous, and mental health. They also discussed

concerns about growth and development, endurance, puberty, fertility, and future pregnancies. Secondly, the group was asked to expand upon concerns of long-term effects of medications. The greatest discussed concern included long-term effects of corticosteroid use. Other more general concerns about medications included cancer and infectious risks from medications; the effects of medications on organs, and the desire for development or testing of new biologic medications. Third, the group was asked to expand upon the concerns of triggers for disease flare. Specific concerns included identifying the role of environmental exposures on disease flare. They also discussed wanting a better way to predict when a flare will occur, to identify biomarkers for flare, to predict the severity and/or duration of a flare, and the best ways to treat flares.

Juvenile arthritis: Four parents of children with JA participated in this FG. All the parents were Caucasian, one parent was male, one parent had a child newly diagnosed with JA and three parents had children with longstanding JA. In this FG, parents were first

asked to expand upon their concerns about long-term health related to JA. Parents described the following specific concerns: effects of long-term medication use, long-term joint health, risk of developing other autoimmune diseases, risk of developing uveitis and how to treat it, and concerns about the health of patients' future offspring. Parents were next asked to expand upon medication-related concerns. Parents discussed their desire to have treatments tailored for their individual child so that their children would not have to be treated with several different medications before finding a treatment that works best. When asked to expand upon concerns of pain management, the parents expressed their desire for holistic treatment for their children, beyond treatment of arthritis. They wanted their children to have support coping with the disease and with pain, including social and emotional support and the development of life skills.

Step 3) Ranking survey (Table 3):

A ranking survey was conducted among constituents of the three advocacy groups that asked respondents to rank order by priority research topics generated from the themes identified via the open-ended survey and FGs. There were 365 (75% parents), 44 (89% parents) and 32 (3% parents, 97% patients) respondents to the open-ended survey by JM, JA, and cSLE disease groups, respectively (Table 3). For JM, the top priorities identified were a desire for new treatments, identifying triggers of, treatment for and prevention of flares, and medication side effects. For JA, the top priorities identified were genetic/environmental etiology, personalized medicine, and medication side effects. For cSLE, the top priorities were disease etiology, quality of life, and medication side effects. The three prioritized research themes common across all disease groups were medication side effects, concerns related to disease flare, and disease etiology (Table 3).

Discussion

Our study was the first in the US to partner with advocacy organizations' social media sites and listservs to ask patients with pediatric rheumatic diseases and their caregivers what types of research were most important to them. The three prioritized research themes common across all disease groups concerned medication side effects, disease flare, and disease etiology. Given that most pediatric patients with JM, JA, and cSLE will require chronic treatment with medications into adulthood, it is not surprising parents and patients have concerns about the short- and long-term effects of these medications. Importantly, the current CARRA Registry, started in 2015, includes disease and treatment data on JM, JA, and SLE with plans for ten year follow-up of registrants, and will eventually allow study of many of these long-term medication-related questions over time(14). Patients also desired to have a better understanding of disease etiology. A considerable amount of research exists in the genetic etiology of JM, JIA and adult SLE, however it is likely that patients are unaware of these studies(15–17). The results of genetic studies are often complex, and it may be difficult for clinicians to interpret these findings for patients. Therefore, we may need to focus on methods to better disseminate

study results to patients and their families. There is a relative gap in the literature in regards to the causes of disease flare in JM, JIA, and cSLE, thus this may be an area to target for future investigation(18–21). It is important to note that some of the topics prioritized by patients/families did not fall within areas of traditional biomedical research, but addressed concerns related to health care delivery and awareness. These are important topics that should not be ignored by funding agencies or researchers as they are daily concerns of patients and families outside of traditional research.

Patient-centered research prioritization is increasingly recognized as valuable in conducting high-quality research, yet there is a paucity of literature describing patient/family preferences, especially in pediatrics(6). The implementation of patient priorities has previously been utilized in adult and pediatric rheumatology as exemplified by Outcome Measures in Rheumatology (OMERACT); however, these studies have focused on prioritization of patient-centered outcomes to measure in clinical trials rather than identifying patient-prioritized research areas(22–25). More recently, Parsons, et al. from

the UK conducted 13 FGs consisting of children and young adults with a variety of rheumatic diseases (n = 63), ages 11-24, to assess their research priorities(7). Interestingly, in contrast to our study, youth in this study did not prioritize the desire for new treatment options, as they felt their treatments worked well for them. This contrast supports the concept that these types of studies should be disease- and culture-specific to best assess the needs of unique communities. An Australian study conducted structured interviews and FGs consisting of children and young adults with SLE (n = 26) to assess their research and healthcare priorities(8). Similar to our findings, this study found that youth with SLE desired research focused on improving the psychological burden of the disease. A Dutch pediatric rheumatology group recently published a protocol to create a research agenda with parents and patients of children with JIA, based upon the James Lind Alliance (JLA) method(11). As our study approach was a modification of the JLA protocol, it will be interesting to compare our findings with the Dutch study in the future. An advantage of surveying groups using listservs and social media sites is the ability to reach more patients/families than those who receive

treatment at the academic centers where most research is conducted. When patients are surveyed through social media sites of patient foundations, it allows opportunity of getting input from patients from diverse backgrounds who are treated at a variety of clinics(16–18).

There were several limitations to this study. First, we were unable to calculate a true survey response rate because the survey links were posted to social media sites in which the number of patients/parents reached was unknown. However, it was important to our partnering advocacy foundations to make this survey accessible to all of their members through these sites. Moreover, there was a lower absolute number of responses from AF and LFA compared to Cure JM. We believe this may be because Cure JM is a well-organized and pediatric-specific organization that emphasizes grassroots fundraising and awareness efforts whereas AF and LFA reach largely adult populations as well as a subset of pediatric patients and other audiences. It is also possible that families of children with JM are highly engaged with the organization given

the relative rarity of JM compared to JA and cSLE. There was some variation in the conduct of this study among groups. For example, we did not conduct a cSLE FG due to lack of resources. The FGs were used to have patients elaborate upon and clarify the themes that were developed from the first survey, and therefore, the FGs did influence the development of the final survey. Since we did not conduct a cSLE FG, we may have missed important elaboration and/or clarification of research priority themes for this group. It may be important to conduct cSLE FGs in the future.

Another limitation of our study was that we did not collect demographic data from all respondents. We were, however, able to detect several important findings about the respondents in this survey. First, cSLE respondents were characteristically different from JM and JIA respondents in that the majority of cSLE respondents of the ranking survey were adult patients themselves with disease onset more than 15 years prior, rather than parents. The most likely reason that the majority of respondents were adult patients with SLE diagnosed in childhood is that the majority LFA's audience is comprised of adults with SLE(27). In the future, we will need to tailor approaches to

reach children with SLE and their parents. Nonetheless, the groups responded with some overlapping research priorities. Most patients were interested in knowing what caused their disease, what causes flares, what their prognosis will be, and how their medications will affect them.

Another limitation of this survey is that web-based surveying may unintentionally target populations skewed by income, literacy, age, access to technology, and leisure time(28). This sampling bias in representativeness of patients suffering from a condition may also occur in the conduct of FGs. In our study, the parents who attended the FGs may not have been fully representative to the greater population of patients with JM and JIA owing in part to the relatively small size and number of sessions conducted, and context in which they were held. For example, the families participating in the Cure JM Annual Conference in Austin, Texas, had the resources and desire to travel to a specialty meeting, and are likely a very engaged group of people with high level of health literacy compared to the general public. It is important to have engaged,

knowledgeable parents contributing to the discussion and elucidating priorities. However, we note the importance of inclusiveness to gain a more comprehensive viewpoint and generalizable conclusions. Ideally, multiple FGs would have been conducted for each disease group.

Another limitation to our study was that although we allowed children with rheumatic diseases who were age 13 and older to participate in the study, we did not include any pediatric patients in the survey design. If we did include youth and young adults in the design of our survey, we may have learned from them better web-based platforms from which to reach youth. Utilizing youth in survey design would be an important future step in assessing patient, rather than parent, research priorities. Another potential limitation from this study was that the same individuals who responded to the first survey could also respond to the final survey. Given the relatively small number of respondents, there could have been a sample bias.

In conclusion, patient-centered research prioritization is increasingly recognized as important in conducting high-quality research, yet there is a lack of literature describing patient/family preferences, especially in pediatrics. Here, we demonstrate a successful initial exercise from which we assessed patients/family research priorities from a sample recruited from US patient advocacy organizations listservs, in order to frame the patient-prioritized research agenda for the pediatric rheumatology research community. Our survey found that the top research priority among families of those with JM included finding new treatments. The top priority for those with JA and cSLE was studying the genetic and environmental etiology of their diseases. Although each group generated a unique list of seven research priorities (Table 3), all groups prioritized the following research topics: medication side effects, disease flares, and disease etiology. Future steps include replicating and expanding similar surveys and FGs in future years to account for shifts, new knowledge, or reaffirm, identified research priorities, with a focus on hearing from patients in addition to their parents.

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Table 1: Results of open-ended survey assessing concerns and research preferences from patients, families, and friends of those with pediatric rheumatic diseases

	Juvenile Myositis	**Juvenile Arthritis**	**cSLE**
Number of Respondents	138	57	47
Parent (%)	106 (77)	53 (93)	26 (55)
Patient (%)	15 (11)	2 (3.5)	15 (32)
Other caregiver (%)	17 (12)	2 (3.5)	6 (13)
Reported concerns	**Number of times concern reported***		
Symptom-related			
Pain management	5	16	5
Mental Health	4	11	2
Effects of the disease on organs	0	0	8
Calcinosis/rash	4	0	0
Uveitis	0	5	0
TMJ-related concerns	0	4	0
Fatigue	0	3	3
Gut health	0	3	0
Growth/weight concerns	0	3	0
Long-term outcomes			
Long-term health/prognosis	35	19	8
Chance of remission	8	3	0
Cure	7	6	7

Quality of life	0	6	4
Medication-related			
Use of alternative therapies	1	10	3
Current medication side effects	4	8	4
Medication administration concerns	0	6	0
Long-term medication side effects	35	21	3
Improved/tailored treatment plan	5	6	2
Healthcare Delivery			
Improving testing to measure disease activity	3	0	0
Discovery of new treatments	2	17	5
Improved awareness among healthcare and public	2	5	3
Medical expenses/Insurance costs**	5	6	4
Disease onset and flare			
Etiology	1	14	5
Triggers for flare	32	4	4
Risks from sun exposure	3	0	0
Prevention of disease onset/progression	0	2	0

TMJ = temporomandibular joint

*Concerns listed by only a single person were not included in the table

**We did not include medical expenses/insurance costs in this study as it was beyond the scope of this project

Table 2. Quotes from parents about their concerns they have about their children with rheumatic disease and their research priorities.

Parent of a child with JM	"I would like to see…medications with *less* (to no) side effects. We need drugs that help fight this disease, but also help our children lead a normal life. Prednisone is not normal."
Parent of a child with JM	"Our daughter was in remission from JDM for 14 years, now at 22 she is having a myositis recurrence. She was the picture of perfect health until last month. What happened?"
Parent of a child with JIA	"The mental is as hard as the physical. We need a manual, signals to watch out for."
Parent of a child with JIA*	"Obviously finding a cure! But finding a way to know which cytokines should be targeted for which person. Jumping from one TNF-inhibitor to another only to have them fail, and having to try other drugs makes a long painful process to find the right drug. I'm also interested in genetics! I would also like to know if there is a trigger that 'turns on' arthritis in someone's immune system."
Parent of a child with cSLE*	"When will he get a flare and will we be back in the hospital? Will he be able to do the work expected at college? Will he be able to hold down a job and support himself and a family?"

*Quoted from electronic survey response

Table 3: Results from final ranking survey. Basic demographics and top seven priorities from each group, ranked from highest to lowest priorities.

	Juvenile Myositis	Juvenile Arthritis	cSLE
Respondents	N (%)	N (%)	N (%)
Parent	274 (75)	39 (89)	1 (3)
Patient	37 (10)	3 (7)	31 (97)
Other	55 (15)	1 (2)	0 (0)
Total	365 (100)	44 (100)	32 (100)
Disease duration			
Less than 2 years	99 (27)	3 (7.5)	0 (0)
2-6 years	113 (31)	22 (55)	4 (13)
7-10 years	69 (19)	7 (17.5)	7 (22)
11-14 years	40 (11)	3 (7.5)	3 (9)
15 or more years	44 (12)	5 (12.5)	18 (56)

Top priorities	Juvenile Myositis*	Juvenile Arthritis	cSLE*
1	New Treatments	Genetic/ Environmental Etiology	Genetic/ Environmental Etiology
2	Flares (triggers, prevention, treatment)	Personalized medicine	Quality of life
3	Medication side effects	Medication side effects	Medication side effects
4	Standards to measure disease activity and/or remission	Growth and development	Pain management
5	Genetic/Environmental Etiology	Flares (triggers, prevention, treatment)	Long-term health/prognosis

6	JM complications (i.e. rash, calcinosis, lipodystrophy)	New Treatments	Fatigue
7	Risk of other autoimmune diseases	Pain management	Flares (triggers, prevention, treatment)

*Cure was the highest ranking priority. Our aim for this study was to identify research priorities other than cure and thus we eliminated these from the final results.

cSLE = childhood systemic lupus erythematosus