Objectives: To perform a review of outcome measures to assess response to treatment in AAV which will help advance methodology for clinical trials in this disease.
Methods: As part of an ongoing international project to develop response criteria, we performed a scoping review to assess the tools used as outcome measures in randomized clinical trials (RCTs) of AAV. Medline, Cochrane Central, and ClinicalTrials.gov were searched from inception until November 2018 to identify RCTs enrolling patients with granulomatosis with polyangiitis and/or microscopic polyangiitis.
Results: Among the 24 RCTs included in the review (Figure 1), various versions of the Birmingham Vasculitis Activity Score (BVAS) were the most widely used instruments for disease assessment. BVAS was almost always reduced to a dichotomous variable (0 or > 0) providing distinction between remission and active disease. Reduction in BVAS and/or achievement of BVAS=0 represented a main study outcome (primary or secondary endpoint) in 20/24 (83%) RCTs; 6 of these trials used the BVAS/WG. Damage, mainly assessed by the Vasculitis Damage Index (VDI), was an outcome for 14/24 (58%) RCTs. Physician global assessment and patient-reported outcomes (PROs) [measures of health-related quality of life (HRQoL) and/or patient global assessment] were assessed in 7 (29%) and 14 (58%) RCTs, respectively. Assessment of renal function or activity was a major outcome or specifically included in definitions of remission/relapse in 23/24 (96%) RCTs. Timing for outcome measure assessment differed substantially, with baseline, 6 months (15/20 RCTs), and 12 months (14/20) after enrollment being the most common time points for reporting BVAS and VDI. Assessment of disease state occurred as early as 1-4 weeks after enrollment.
Conclusion: Outcome measures used in RCTs of AAV include the repeated use of vasculitis specific tools to assess disease state, but with heterogeneity in the definitions for remission/relapse and timing of assessment. Intermediate states of disease activity are currently poorly defined or evaluated. Furthermore, other important outcomes in AAV, including PROs, damage measures, and global assessments are often not included as primary or confirmatory secondary outcomes in RCTs in AAV. This review highlights the need for more homogeneous outcome assessment in RCTs for AAV and the current lack of a composite measure that integrates various endpoints relevant to physicians and patients.

Figure 1. Overview of outcomes assessed in randomized controlled trials of ANCA-associated vasculitis

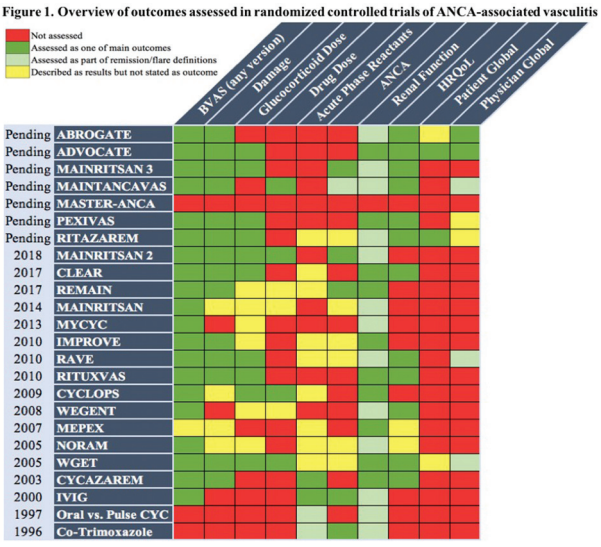

Figure 1. Overview of outcomes assessed in randomized controlled trials of ANCA-associated vasculitis

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SAT0668

"WHEN YOU READ THIS, YOU REALLY FEEL OLD!" PERSPECTIVES OF YOUNG PEOPLE WITH INFLAMMATORY ARTHRITIS ON PATIENT REPORTED OUTCOME MEASURES FROM A EUROPEAN QUALITATIVE STUDY

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Background: Although patient-reported outcome measures (PROMs) are extensively used in clinical practice and research, it is unclear whether the most commonly used instruments adequately cover the perspective of young people with chronic inflammatory arthritis.
Objectives: To investigate whether the aspects important to young people with inflammatory arthritis are sufficiently covered by the PROMs that are widely used in clinical practice and research.
Methods: A qualitative, multicentre focus group interview study was conducted in Austria, Croatia, Italy and the Netherlands in order to inform a EULAR-funded taskforce. Three groups of young people (aged 18-35 years) with either (1) rheumatoid arthritis (RA), juvenile idiopathic arthritis (JIA) and Still's disease, (2) psoriatic arthritis (PsA), or (3) axial spondyloarthritis (SpA) were interviewed at each centre. The interview guide was based on the WHO International Classification of Functioning, Disability and Health (ICF) to comprehensively cover all aspects of functioning in daily life. It also included questions on the perspectives and views of the participants on selected PROMs (Pain scales, Patient Global Assessment [PGA], FACIT Fatigue Scale, The Health Assessment Questionnaire [HAQ]/Bath Ankylosing Spondylitis Functional Index [BASFI], and The 36-Item Short Form Health Survey [SF-36]). All interviews were conducted by trained local investigators, audio-recorded, transcribed verbatim, and analysed using a modified form of 'meaning condensation'. During a face-to-face meeting of the task-force members, the concepts were reformulated and organized into a scheme of higher and lower-level concepts.
Results: Thematic saturation was reached after 12 focus groups with 53 participants (21 with RA/JIA/Still's, 15 with SpA, 17 with PsA; 72% female, mean age 28, SD±5), resulting in 18 hours and 22 minutes of recorded time and 269 pages of transcript. The analysis revealed aspects of functioning in daily life important to young people with inflammatory arthritis which were mentioned in all countries. Furthermore, 55 concepts emerged with regard to PROMs and were summarized into seven higher-level concepts. The table depicts these higher-level concepts including quotes from the interviews.
Conclusion: The evaluation of young patients' perspectives should probably reach beyond the topics/aspects covered in the most commonly used PROMs. Accordingly, tailoring the assessments to specific needs of young people should be considered.
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SAT0669 DISCRIMINANT ABILITY OF THE PARENT VERSION OF THE JUVENILE ARTHRITIS DISEASE ACTIVITY SCORE IN A LARGE MULTINATION COHORT OF PATIENTS WITH JUVENILE IDIOPATHIC ARTHRITIS

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Background: The assessment of disease activity plays a pivotal role in the management of children with juvenile idiopathic arthritis (JIA). Most recent recommendations require that parents' and children's perception is incorporated in the evaluation of the disease course and of effectiveness of therapeutic interventions. A new disease activity tool, named parent Juvenile Arthritis Disease Activity Score (parJADAS) and based only on parent-centered outcome measures, is currently under development (1).

Objectives: To demonstrate, in a large multinational dataset of JIA patients, the discriminant ability of the parJADAS.

Methods: The parJADAS includes 4 measures: 1) parent assessment of disease activity; 2) assessment of pain intensity; 3) proxy assessment of joint disease; 4) assessment of morning stiffness (MS). Disease activity and pain are assessed on a 21-numbered circle VAS (0 = best and 10 = worst). The active joint count is based on the count of any swollen or painful joint, irrespective of its type, up to a maximum of 10 joints. MS duration is assessed on a Likert scale, ranging from no MS (0 points) to > 2 hours of MS (10 points). Validation was conducted on a dataset of 8,656 children with JIA from 49 countries, enrolled in the study of Epidemiology, treatment and Outcome of Childhood Arthritis (2), who had all the variables included in the parJADAS available. Discriminant ability was evaluated by comparing parJADAS levels (median, [IQR]) among patients with inactive disease (ID), low disease activity (LDA), moderate disease activity (MDA), and high disease activity (HDA) according to the cJADAS10; patients in remission, continued activity, or flare according to the attending physician; patients whose parents were satisfied or not with current disease state. To assess the possible influence of the articular and extra-articular damage on the parJADAS, the levels of the score in patients with or without damage (Juvenile Arthritis Damage Index > 0) were compared. For this analysis, only subjects in inactive disease and with at least 2 years of disease course were considered (n = 2,423).

Results: The levels of parJADAS in patients in ID, LDA, MDA, and HDA were 0.0 [0.0, 1.0], 3.0 [1.0, 6.0], 6.0 [2.0, 11.5], an 14.5 [8.5, 21.0], respectively (Kruskal-Wallis test, p < 0.001). The levels of par JADAS in patients in remission, continued activity, or flare according to the attending physician were 0.5 [0.0, 3.5], 9.0 [3.5, 17.0], 12.0 [5.5, 20.0], respectively (Kruskal-Wallis test, p < 0.001). Median parJADAS in patients whose parents were satisfied or not satisfied with disease course is 1.5 [0.0, 7.0] and 13.0 [6.6, 20.5], respectively (Mann-Whitney test p < 0.001). ParJADAS was not different in JIA patients in remission with or without damage measured with the JADI (Mann-Whitney test p = 0.08)

Conclusion: The parJADAS showed excellent discriminate ability in a large multinational cohort. The score did not show to be relevantly influenced by disease damage in JIA patients in remission.

Table. Overview of higher-level concepts and related quotes

Higher-level concepts	Quotes
Relevant issues for young people are not covered by widely used PROMs	*My appearance has never been brought up for discussion, but it impacts my teaching, my sex life, [...].* (male, 30, PsA)
Information, transparency and clarity regarding the purpose of PROMs are often missing	*Honestly, I have often thought that we should fill all those questionnaires to keep us quiet and to avoid that we will freak out!* (female, 27, PsA) *I do not understand the meaning. Why does the doctor care if I can wash my hair? Who will wash it instead? It is a stupid question!* (female, 27, PsA)
The individual life situation of a patient adds essential importance to the results of PROMs	*In my opinion it is problematic to estimate disease activity for today. With my medication, or without? At the moment, I am feeling fine, but it won't be like that without any medication, I guess. And that makes scoring a bit difficult.* (female, 27, PsA)
The scoring on a rating scale sometimes differs from the current health situation	*I am just putting my line anywhere and think – that's fine. (female, 27, PsA)* *I always score very low, like a 1, 2, or 3. which might look very harmless to the doctor. I often ask myself whether I should score worse, to get recognized.* (male, 30, PsA)
Certain PROMs were seen as outdated	*When you read this, you really feel old! (female, 21, RA)* *I think 'working on your computer' or 'typing' or something could be included. I mean how often do we still use a pen and pencil all day long? It should be a little more up to date.* (female, 25, PsA)
PROMs focusing on symptoms and physical function only, do not comprehensively cover patients' experiences	*I think it is also important what you [as an individual] need. Not only the physical part, but also the mental part, so how are you feeling. I think that is important too.* (male, 22, SpA)
The use of new technologies for data acquisition was suggested by some young patients	*Sometimes I do not want to answer with a whole story. [...] but I can also do that questionnaire [HAQ] digitally at my hospital. That is nice!* (female, 25, PsA)

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