MANAGED CARE ISSUES IN JUVENILE RHEUMATOID ARTHRITIS*

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ABSTRACT

The lack of consensus guidelines for the treatment of juvenile arthritis (JA) has contributed to managed care challenges faced by patients, providers, and insurers. Clinical studies demonstrating the optimal role of biologics for JA are insufficient, and high cost often limits their availability. Managed care providers and healthcare professionals should develop strategies to improve clinical outcomes, enhance patient and caregiver quality of life, and provide optimal education. (Adv Stud Pharm. 2008;5(6):184-190)

Despite being a relatively common pediatric disease and unlike adult rheumatoid arthritis, the economic impact of juvenile arthritis (JA) is not fully described. In 1992, the most recent US description, the economic impact of JA was found to be substantial, with $285 million in direct costs or $7905 per child annually. Family cost has been estimated at $1524 per year, which represents 5% of mean family income. The mean extra school cost associated with special services was $1449 per 9 months. Higher active joint count is associated with greater total direct medical costs because costs for medications, specialist and allied healthcare professional visits, and diagnostic tests are substantially higher.

DISEASE BURDEN

In one study of 215 patients assessed an average of 17 years after JA onset, direct costs contributed to more than 50% of the total healthcare burden (direct plus indirect costs). Although variation existed among different JA subgroups, patients with active disease incurred more than 90% of the total costs. Impairment in function and quality of life (QOL) were reported as 39% in patients in remission and 60% in those with active disease. Overall, 9% of patients needed help with daily living activities and 24% needed help engaging in their entire daily routines.

Dr Miller: In individual patients, the physical and emotional burden of illness can be more dramatic than demonstrated in this study. Impairment of function, particularly regarding remission, is largely related to physical mobility. Physical ability and emotional impairment, including anxiety and depression, are independent of remission or disease severity.

Dr Rich: Current economic impact may differ from the 1992 study. It is expected that direct costs would be substantially higher with better diagnosis and treatment of JA, particularly with the advent of biologics.

The study also demonstrated the emotional aspect of JA. In patients with active disease, impairment of global health and QOL were 90% and 80%, respectively. These patients also considered themselves to be

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burdened physically (almost 90%), emotionally (56%), and professionally (64%).

A long-term follow-up study of adults with JA examined social function, relationships, and sexual activity. Fewer patients were in stable relationships as compared to their siblings (42.8% and 55.3%, respectively). A detrimental effect on body image was observed in 50.7% of patients. In patients who were not sexually active, body image accounted for 67% of reported problems. Disease-related sexual dysfunction was experienced by 58.3% of patients.

**Dr Penna:** Managed care tends to focus on direct costs versus indirect costs because employers, who are the ultimate purchasers of health insurance, request documentation about direct benefits and safety of treatment options.

**Dr Miller:** Employers are also asking why variances in treatment occur, or more simply, employers want quality control when managing cost.

**Dr Rich:** When evaluating managed care, employers tend to focus on diseases with greater notoriety or prevalence, such as attention deficient hyperactivity disorder or cancer.

An educational needs assessment of 50 children with JA and their families showed differing needs of patients, siblings, and parents (Table 1). Psychological/emotional stress was the most difficult aspect of JA for parents and siblings whereas limited capacity was most difficult for patients. Pain was most concerning to patients. All groups associated JA with pain most commonly. However, patients and siblings associated JA with medications whereas parents associated JA with crippling and deformity.

**HEALTHCARE CHALLENGES**

Treatment challenges for JA include multidisciplinary healthcare coordination, cost of biologics, and medication adherence affected by adverse events (AEs) or regimen complexity. Specifically, the high cost of biologics may restrict or prevent patient access due to managed care requirements or denials. Additionally, insurance benefit design or copayment structures may cause financial stress on caregivers.

**Dr Penna:** Another challenge is the lack of relatively recent data that can be provided to managed care as evidence of disease impact on function and QOL. Furthermore, managed care coverage of new drugs may be slow, often citing safety concerns and historic market withdrawals to defend this delay.

**Dr Rich:** Managed care formularies can place moratoriums on coverage of new drugs to allow the availability of postmarketing surveillance data.

Copayment structures are shifting to a tier system, with biologics typically in the fourth or fifth tier. For biologics, copayments may be as high as 25% with out-of-pocket maximums established.

**Dr Myers:** For young families with several children, high copayments associated with biologics, which are typically only one of several medications prescribed, can be staggering.

**Dr Penna:** High copayments may force families in financial hardship to choose paying for necessities over receiving optimal therapy.

<table>
<thead>
<tr>
<th>What is the most difficult thing about having arthritis?</th>
<th>Parents, % (n = 76)</th>
<th>Children with JA, % (n = 41)</th>
<th>Siblings, % (n = 33)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Psychological/emotional stress</td>
<td>72</td>
<td>51</td>
<td>42</td>
</tr>
<tr>
<td>Family relationships</td>
<td>43</td>
<td>–</td>
<td>33</td>
</tr>
<tr>
<td>Fear about future</td>
<td>21</td>
<td>–</td>
<td>–</td>
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<tr>
<td>Regimen compliance</td>
<td>13</td>
<td>10</td>
<td>9</td>
</tr>
<tr>
<td>Limited capacity</td>
<td>–</td>
<td>61</td>
<td>39</td>
</tr>
<tr>
<td>Swollen joints/fever</td>
<td>–</td>
<td>17</td>
<td>–</td>
</tr>
<tr>
<td>Pain</td>
<td>–</td>
<td>41</td>
<td>–</td>
</tr>
</tbody>
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<table>
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<tr>
<th>When you think of arthritis, what do you think of?</th>
<th>Parents, % (n = 74)</th>
<th>Children with JA, % (n = 38)</th>
<th>Siblings, % (n = 34)</th>
</tr>
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<tbody>
<tr>
<td>Pain</td>
<td>76</td>
<td>71</td>
<td>50</td>
</tr>
<tr>
<td>Swelling/stiffness</td>
<td>74</td>
<td>53</td>
<td>44</td>
</tr>
<tr>
<td>Crippling/deformity</td>
<td>35</td>
<td>–</td>
<td>–</td>
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<tr>
<td>Emotions</td>
<td>28</td>
<td>21</td>
<td>20</td>
</tr>
<tr>
<td>Old age</td>
<td>12</td>
<td>16</td>
<td>15</td>
</tr>
<tr>
<td>Hospital/surgery</td>
<td>9</td>
<td>21</td>
<td>15</td>
</tr>
<tr>
<td>Medication</td>
<td>–</td>
<td>18</td>
<td>12</td>
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JA = juvenile arthritis.
**Dr Rich:** Employers may offer catastrophic plans instead of full coverage plans, which increases financial burden on families. Another concern is that patients on biologics may reach lifetime maximum benefits.

**MULTIDISCIPLINARY TREATMENT APPROACH**

The American College of Rheumatology (ACR) and Association of Rheumatology Health Professionals support the multidisciplinary care of patients with rheumatic and musculoskeletal diseases. Effective management and optimal outcomes require access to a diverse group of healthcare professionals. A coordinated program of disease management provides medical management, education, physical and occupational therapy, counseling and vocational support, and possibly surgery. The team needs to consistently assess response to therapy and modify treatment plans as needed.

**ROLE OF BIOLOGICS**

Biologics are generally third- or fourth-line agents in certain types of JA, after nonsteroidal anti-inflammatory drugs (NSAIDs) and/or intraarticular corticosteroids, and disease-modifying antirheumatic drugs (DMARDs), such as methotrexate (MTX). Biologics are shown effective for JA, but safety concerns exist. Pediatric patients may be more susceptible to serious AEs, and clinical evidence is not available for all biologics. This lack of robust long-term safety data prevents the resolution of a definitive risk-benefit ratio.

Etanercept has been approved by the US Food and Drug Administration (FDA) for JA longer than other biologics and has been shown to be well tolerated overall in patients nonresponsive to MTX. Abatacept and adalimumab were US FDA-approved for JA in 2008, and both agents are well tolerated.

The ACR, not unexpectedly, states that all patients with serious rheumatic disease must have biologics available when clinically appropriate. Furthermore, the ACR discourages the restriction of biologics through non-evidence-based guidelines or through criteria outside of the patient-physician relationship. Cost-based substitution among biologics and managed care mandates based on cost are also discouraged. The ACR states that burdensome prior authorization requirements or tiered level copayment structures based on preferred drug status are not justifiable. In the absence of validated clinical guidelines, rheumatologists should be determining optimal therapy.

In April 2008, the ACR released a request for proposals for the development of evidence-based recommendations for the treatment of JA. The anticipated manuscript submission deadline to the journal *Arthritis Care & Research* is October/November 2009. Funding is approved to keep the guideline updated through a mechanism of review at defined intervals.

**Dr Rich:** Managed care tends to reserve prior authorization requirements for expensive therapies because operational costs are high, requiring electronic databases and extra staff.

**Dr Myers:** Managed care should not restrict use to a particular biologic agent. However, it is appropriate for managed care to control costs by requiring less expensive therapies initially, such as NSAIDs and DMARDs, except in cases of aggressive disease.

**Dr Rich:** One managed care challenge is the lack of a consensus definition of aggressive disease.

**Dr Miller:** Perhaps managed care and the ACR should collaborate to develop evidence-based consensus guidelines.

**Dr Penna:** Ironically, the evidence-based medicine that managed care demands for management of costs could ultimately oblige coverage of expensive medications. Historically, managed care recognizes consensus guidelines for disease management.

**Dr Swims:** The Veteran Affairs (VA) Criteria for Use of biologics may be a useful resource in the absence of JA consensus guidelines.

**Dr Busbey:** The VA Criteria for Use are developed in partnership with specialists, such as rheumatologists.

**MANAGED CARE CHALLENGES**

Although consensus guidelines can greatly improve clinical outcomes, treatment costs and formulary considerations are often not factored into recommendations, which is a challenge for managed care. Managed care also struggles with differing practice habits. Academic medical centers and urban practitioners may tailor consensus guidelines based on experience whereas rural practitioners may be unfamiliar with guidelines.

Goals for managed care providers include controlling costs, ensuring appropriate use of expensive agents (ie, biologics), and enhancing adherence to ensure optimal clinical outcomes. To control costs, managed care imposes several limitations with med-
ication coverage, including prior authorization, patient selection criteria, quantity limits, and early refill restrictions. Managed care typically involves internal Pharmacy and Therapeutics (P&T) Committees and physician subspecialties when formulating policies and programs. Managed care may offer disease state management programs to optimize clinical outcomes. Disease state management programs tend to be multifactorial, with disciplines such as case management included.

Managed care confronts several challenges regarding biologics for JA. These challenges may include appropriate patient selection and drug use, safety and efficacy associated with off-label use, formulary acceptance of selected agents among available biologics, and the lack of formal training of P&T Committee members concerning pharmacology, pharmacodynamics, and pharmacokinetics of biologics. When assessing the cost effectiveness of biologics for JA, the literature is inadequate and data are often extrapolated from limited data in adult studies. Adult pharmacoeconomic studies are often based on complex models comparing multiple regimens, in which cost seems directly proportional to goals of therapy.

**Dr Penna:** Public perception is that controlling cost is managed care’s primary concern. However, although cost is important, managed care also focuses on improving patient care.

**Dr Rich:** Traditionally, the silo effect was observed in managed care’s oversight of healthcare economics, where goals differed for various departments. For instance, drug costs were handled by pharmacy directors, treatment costs were handled by physicians, and hospital discharges were handled by case managers. Today, this strategy is changing, and managed care recognizes that avoiding hospitalization can control total cost of therapy, even if more expensive therapies would be required.

**Dr Myers:** Although hospital and physician costs are a large proportion of total healthcare expenditure, patients tend to focus on pharmaceutical costs because their expense is highest for medications.

**Dr Rich:** For the managed care risk model of expenditure, hospitalization and physician costs are typically fixed whereas pharmaceutical cost is variable. Therefore, pharmaceutical cost becomes a key determinant of managed care plans.

**Dr Penna:** Pharmaceutical costs are generally rising faster than other medical costs, particularly with new technologies, such as biologics. If pharmaceutical cures were discovered, other medical costs would be avoided, and managed care prohibitions on expensive therapies would lessen.

Appropriate patient selection may be the most useful mechanism of controlling costs. If therapy criteria were coordinated, cost could be controlled while improving clinical outcomes. However, companion diagnostic costs could increase.

**Dr Miller:** For disease states with smaller patient populations and less available funding for pharmaceutical research, the US FDA recognizes that off-label use may be unavoidable.

**Dr Rich:** Although the US FDA recognizes the high cost of manufacturing biologics, the US FDA is also considering processes that could reduce production costs and allow the availability of less expensive alternatives.

**Dr Penna:** In reference to P&T Committee member training, another challenge is the relative lack of biologic therapy experts. P&T Committees will often use consultants when considering new therapies. However, rheumatologists and immunology pharmacists are often not readily available.

**Dr Rich:** To combat this issue, many managed care providers are requiring the submission of Academy of Managed Care Pharmacy dossiers that provide clinical trial summaries and pharmacoconomic analysis.

**Dr Penna:** The intangibility of indirect healthcare cost savings increases managed care’s reluctance to accept new therapies. Electronic medical records can integrate pharmacy data, medical data, and clinical outcomes more accurately, which could provide better justification for expensive therapies.

**Dr Miller:** Does managed care retrospectively analyze the impact of formulary decisions to add or reject medications?

**Dr Penna:** For more common disease states, this type of analysis may occur.

**Dr Rich:** However, this analysis is selective, often not assessing factors, such as patient productivity and employment absenteeism. Managed care tends to base coverage and premiums on medical cost analyses.

**Dr Myers:** When expensive therapies are added and premiums increase, employers may pass this cost onto employees.

**Dr Penna:** Ultimately, healthcare costs associated with managed care decisions are passed onto the public.
MEDICATION ADHERENCE

Medication adherence is a common problem encountered by patients with JA and their clinicians. Adherence appears to be symptom-driven and more prevalent in families with lower socioeconomic status. Improving medication adherence requires a multidisciplinary healthcare approach. Educating patients and caregivers, keeping therapies simple and conducive to lifestyles, and providing social and psychological support are important strategies for improving adherence. Teaching caregivers positive reinforcement strategies can promote adherence.

**Dr Penna:** Nonadherence is an immense struggle for healthcare and pharmaceutical companies who could be strategic allies to improve adherence. Reporting nonadherence is controversial because patients may believe that their privacy is invaded, and physicians may feel forced to intervene.

**Dr Miller:** With pediatric patients, physicians tend to be more willing to intervene because nonadherence may be due to caregiver negligence. Behavioral strategies by institutions could augment medication adherence. When adherence is improved, an economic benefit might be demonstrated.

**Dr Rich:** Adherence may be better for injectable versus oral medications in pediatric patients because parents typically administer injections.

**Dr Myers:** Parents should transition this responsibility to self-administration and should monitor adherence before their children leave home for college.

Nonadherence seems to be more common when symptoms lessen. When symptoms return, adherence should be questioned.

**Dr Penna:** Should managed care penalize the patient when nonadherence is discovered?

**Dr Myers:** Therapy should not be refused based on nonadherence. However, it may be appropriate to restrict more expensive or higher-tier medications until adherence improves.

**Dr Busbey:** VA medical centers use this approach of denying higher-tier medications, such as injectable antiretrovirals, if nonadherence is confirmed.

**Dr Rich:** This approach could happen because some managed care plans currently penalize patients for unhealthy behaviors, such as smoking or being obese.

**Dr Saleh:** The question is whether financial penalties will change patient behaviors.

**Dr Penna:** Behavior may not be changed, but financial pressure will be placed more heavily on patients with inappropriate behaviors.

**Dr Myers:** JA is not self-induced, such as smoking and obesity, which requires a level of compassion.

TREATMENT RESPONSE

Assessment of JA treatment response is challenging. The Health Assessment Questionnaire (HAQ) and Disease Activity Scores are useful in clinical trials but are not routinely used in clinical practice. The ACR Pediatric 30 (Table 2) response criteria remain the only prospectively validated measure of JA disease activity. However, the ACR Pediatric 30 is also used primarily in clinical trials. It is less useful for quantifying response, tracking progress longitudinally, and describing disease state at a specific moment in time. Other tools for assessing health-related QOL include the Pediatric Quality of Life Inventory (PedsQL) and the Child Health Assessment Questionnaire (CHAQ).

**Dr Miller:** The ACR Pediatric 30 is well studied and validated but more commonly used in research versus practice. Electronic medical records could someday provide useful assessment of therapy response.

**Dr Rich:** Managed care could benefit from an understanding of what assessment tools are used in practice versus research.

**Dr Myers:** If using electronic medical records, the

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**Table 2. American College of Rheumatology Pediatric 30 Response Criteria**

- Physicians’ global assessment of overall disease activity
- Parent of patient global assessment of overall well-being
- Functional ability
- Number of joints with active arthritis
- Number of joints with limited range of motion
- Erythrocyte sedimentation rate

A minimum of 30% improvement from baseline in a minimum of 3 out of 6 components, with no more than 1 component worsening by >30%.

input data must be accurate and current.

Dr Swims: Inclusion of ACR assessment criteria into the VA Criteria for Use was debated because assessment can be time-consuming.

Dr Myers: The individual measurements of the ACR Pediatric 30 are not routinely performed at every visit or for every patient due to time constraints.

Dr Rich: If performed, more detail could be provided to managed care and approval would be more likely.

Dr Penna: ACR Pediatric 30 data upon the initial request for biologic therapy would be especially useful to managed care.

Dr Saleh: In adult rheumatic diseases, the HAQ score can be calculated in the clinic and provided to managed care.

Dr Rich: ACR Pediatric 30 may indicate QOL changes to family members and clinicians, regardless if required by managed care.

Dr Miller: The CHAQ and PedsQL are useful but serve slightly different functions. The CHAQ is a recall questionnaire regarding physical mobility. The PedsQL may be more comprehensive because it is a questionnaire to assess overall QOL in all children, healthy and those with acute or chronic illness. The PedsQL Rheumatology Module is used to assess mobility.

Dr Myers: Quality of the information from any of these questionnaires is dependent on caregivers’ understanding of the question.

CONCLUSIONS

Until more studies of biologics in patients with JA are available, managed care challenges will continue. Managed care providers need to be prudent when establishing cost controls related to biologics. Enhanced technology, disease state management programs, and medication therapy management may be useful strategies for controlling costs.

DISCUSSION

Dr Myers: Biologics are typically similarly priced, which prevents additional struggles associated with price discrepancies. Because of cost constraints, biologics cannot be used for every patient with JA but should be reserved for patients who would benefit most from therapy.

Dr Rich: Some physicians may request biologics prematurely citing efficacy benefits and not considering therapy costs.

Dr Miller: Specifically for JA, more studies comparing biologics and consensus guidelines would be valuable. Postmarketing surveillance would be valuable to managed care for determining optimal clinical outcomes.

Dr Rich: Decisions concerning when to discontinue therapy, when to re-evaluate patients, and how to define therapy success should be incorporated into consensus guidelines.

Dr Miller: Quality of care would improve with guidelines, and unexplained variances in care could be captured.

Dr Myers: Although managed care provisions seem burdensome at times, these patients are not struggling with cost issues to the extent of patients with catastrophic disease, who face high deductibles and copayments and may not be able to afford biologics.

Dr Penna: Managed care attempts to balance cost issues with ethical issues when formulating their requirements.

REFERENCES


