

**AMERICAN COLLEGE RHEUMATOLOGY
POSITION STATEMENT**

SUBJECT: Patient Access to Biologics

PRESENTED BY: Committee on Rheumatologic Care

FOR DISTRIBUTION TO: Members of the American College of Rheumatology
Medical Societies
Members of Congress
Health Care Organizations/Third Party Carriers
Insurance Companies and Commissioners
Pharmacy Benefit Managers
Managed Care Entities
Other interested parties

POSITIONS

1. Biologics are expensive but vitally important therapeutic options for patients with rheumatic diseases. Given their effectiveness and potential to reduce long-term disability, patients should have affordable access to biologic therapy without undue delay.
2. The documentation for medical necessity should include the diagnosis, rationale for choice of treatment, and response to treatment. Access to biologics should not be tied to disease activity measures used exclusively in research trials that are not a part of routine clinical practice.
3. Reimbursement for biologic therapy in rheumatic diseases should be fair and equal and take into account FDA labeling and peer-reviewed literature.
4. The ACR opposes step edits, fail-first policies, tiering, forced switching, or excessive coinsurance for biologics.
5. Policies regarding the location of the administration of biologics should promote the highest standards of safety and allow patients to obtain their treatments in physician offices or a medical facility with specially trained practitioners overseeing their infusion.
6. The choice of biologic is a complex decision that is made between the patient and the rheumatology provider. Policies should be based on the best interests of the patient and allow for grandfathering of patients whose disease is well controlled on stable therapy.

BACKGROUND

Biologics are mainstay therapies in the treatment of many rheumatic conditions. The ACR provides guidelines on the use of biologics in the treatment of many rheumatic conditions, and the ARHP publishes medication quick guides that include basic information on biologics. The use of these biologics requires an understanding of their mechanisms of action, unique toxicities, proper screening and monitoring measures, and contraindications which may be found in these resources. Within this position paper, the ACR presents the principles for model biologic access based on these clinical standards of practice.

DOCUMENTATION AND DISEASE ACTIVITY MEASURES

The overarching goal of biologic therapies is to treat to a target of low disease activity or full clinical remission. Thus, it is common practice to include simple disease activity measurements and statements about achieving remission in the medical record. In fact, documenting the status of these goals has become a mainstay in physician reporting for quality of care. Early, aggressive, treat-to-target therapy is the recommended approach for rheumatoid arthritis and emerging data suggest the benefits of this approach for other rheumatic conditions as well. The biologics are often necessary when other disease modifying anti-rheumatic drugs (DMARDs) are either ineffective or not tolerated by the patient. In addition, biologics tend to work rapidly and may achieve control of the disease more quickly which is important when facing a brief window of opportunity for maximum therapeutic efficacy. Delayed treatment fails to prevent reduced mobility, daily function, performance at work, disability, and other complications of rheumatic disease. Therefore, providers require timely access to these medications to provide the best outcome for their patients. Processes for approval of biologics, such as prior authorizations, should not delay medically necessary treatment with biologics.

Biologic agents are used for moderate and severe rheumatic disease states and are approved based on specific disease states and clinical criteria. When a provider evaluates a patient, he or she uses an integrated history, physical exam, laboratory values, and imaging studies to determine the degree of disease activity. There are also commonly used disease activity measures that may be calculated and documented separately (ex. Health Assessment Questionnaire (HAQ), Routine Assessment of Patient Index Data (RAPID), Clinical Disease Activity Index (CDAI)). In addition, there are formal measures of disease activity utilized during clinical trials but these are not used in routine clinical practice. For example, the Psoriasis Area Severity Index (PASI), Bath Ankylosing Spondylitis Disease Activity Index (BASDAI), and Systemic Lupus Erythematosus Disease Activity Index (SLEDAI) scores define parameters for research purposes but do not incorporate complex factors that influence therapeutic decisions. These tools are neither sensitive nor specific for factors that influence choices between biologics. They are not intended to determine medical necessity for a drug. They often fail to adequately assess an individual's response to treatment. A patient may reach important goals but these may be accompanied by only small numerical changes in these measures. Thus, these tools are not used in routine practice to determine therapy and must not be required for approval of a biologic.

The ACR recommends clinicians follow the AMA and CPT guidelines for documentation. The treating provider must clearly indicate in the medical record the diagnosis of the rheumatic condition. Where necessary, previous treatment failures for lack of efficacy or poor tolerability,

or contraindications to other medications (such as DMARDs) need to be adequately documented in the medical record.

MEDICAL DECISION MAKING FOR CHOICE OF BIOLOGIC

The decision to choose one biologic over another requires careful clinical evaluation and consideration by a physician and patient. Patient factors that strongly influence this choice include but are not limited to an individual patient's age, gender, diagnosis and comorbid conditions, concomitant medications, specific organ manifestations, antibody status, disease severity and burden, physical or psychological abilities, access to transportation, and ability to tolerate a particular route of administration. For example, susceptibility to infection, heart or lung conditions, malignancy, and other disease manifestations may drive the choice between agents. Thus, both individual patient characteristics and differences in disease states for rheumatic populations will determine the choice of biologic. This class of drugs is complex, the rheumatic diseases are complex, and the choice of treatment may be complex. Of note, the complex medical decision making and subsequent risks associated with these medications fall on the physician and the patient. Therefore, insurance plans must leave the clinical decision making for medical necessity to the provider and should not determine the treatment of the patient, nor should they mandate use of one therapy over another based on cost alone.

Presently, there is a paucity of peer-reviewed literature addressing the comparative efficacy and safety of biologics. Given the safety concerns with this class of drugs and lack of evidence for clinical superiority or safety of one versus another, access and coverage for biologics should remain fair and equal. Forcing a stable patient to switch to another biologic medication for the sake of cost control needlessly disrupts continuity of care and puts patients at significant risk for loss of disease control (see Reggia et al.) and potentially life-threatening complications.

In contrast, there is a large body of research demonstrating that each of these drugs is unique in terms of their molecular structure, immunogenicity, mechanism of action, safety and efficacy. Between classes of biologics, there are enormous differences in therapeutic pathways and FDA indications. Even within the most commonly used class of biologics, the TNF inhibitors, differences in responses and adverse events are commonly observed. Again, individual patient considerations, overlapping medical and immune conditions, safety and other considerations will drive the clinician and patient's decision for appropriate therapy. While some biologics may have similar mechanisms of action, this does not confer equivalent adherence, tolerability, or safety profiles. Moreover, all biologics can differ in time to remission, need for concurrent oral DMARD therapy, frequency of administration, and frequency of infusion and injection site reactions.

The influence of anti-drug antibodies and immunogenicity also influence the choice of a biologic. Some patients develop drug-specific antibodies that influence the efficacy of subsequent therapies. Also, inadvertent drug holidays, class switching, and retreatment after cessation of a drug increase the risk of disease relapse, drug resistance, and serious reactions. Therefore, forced switches in biologic therapy due to formulary changes may harm patients and lead to disease relapse.

Any biologic formulary policy must be supported by high quality research and remain in accordance with best clinical practices. It must also make exceptions for patient characteristics and current status (including remission status). Policies related to biologic choices must include a “grandfather” provision that allows stable patients to continue on their current treatment at affordable prices.

AFFORDABLE ACCESS TO BIOLOGICS

The ACR recognizes that biologics are costly medications and rheumatology providers must consider this choice carefully. Given the high value of this class of drug in achieving disease remission and improvements in overall patient wellness, employer health plans, other payers, and pharmacy benefit managers must allow affordable coverage options for biologics. Importantly, the cost of the drug is not the only financial consideration. A growing body of evidence indicates that by slowing disease progression these medications may reduce costly disease-related complications including adverse outcomes related to cardiovascular disease, metabolic syndrome, and expensive procedures and surgeries. Early use of biologics in rheumatic conditions also reduces costs by preventing missed work, improving work performance, and avoiding long-term disability. Although the ACR recognizes that biologic costs are a factor in health care delivery, it believes that restricting access not only adversely affects patients’ health but impacts important public health outcomes as well.

Presently the cost of drugs is determined by pharmaceutical companies and may be negotiated (for example, between a manufacturer and a pharmacy benefit manager). Unfortunately, there is a lack of transparency in pricing in the eyes of the patients, providers, and the public. Pricing differences between companies or plans are not based on clinical decision making or standards of practice and are subject to change with tremendous frequency. While rebates and price fixing in particular contracts may reduce the cost of a drug for the plan, privately negotiated cost savings to the insurance company should not be allowed to undermine the important clinical considerations and decisions made by patients and providers when choosing a biologic. Essentially, plan savings should not override medical necessity or intrude on safe medical practice.

The ACR is concerned that patients are susceptible to adverse events that result from changes in these negotiations from year to year, inconsistencies between plans, and the dangers of third party negotiations driven by profit rather than by safe and sound medical practice. The increasing costs of these drugs must be addressed without placing the burden on sick patients. The ACR finds that step therapies, fail first, and tiering policies may disregard the appropriate clinical decisions made between providers and patients and may contradict the current standard of care and practice guidelines. Affordable access to these drugs, in the absence of excessive copayments, coinsurance, and other subversive financial restrictions, for patients who suffer from chronic, disabling conditions is a necessity. Clinical guidelines should drive these discussions and not the other way around. Furthermore, patients who receive biologic therapy and achieve an acceptable clinical response should be allowed to remain on that therapy.

ADMINISTERING BIOLOGICS IN MEDICAL SETTINGS

Biologics carry a high risk of dangerous adverse and allergic reactions, both at the point of care and remotely. As detailed in peer-reviewed research articles, ACR position papers on biologics, and FDA labeling, direct supervision of the infusion of biologics remains the standard of care for the administration of these medications. The administration of infusible biologics requires a safety checklist and detailed patient history and evaluation prior to their infusion by specially trained providers. Given the black box warnings for serious infusion reactions and infections, the safest location for the administration of these drugs remains a setting supervised by a physician. The clinical monitoring is best accomplished and risks are best mitigated when these drugs are infused in medical facilities rather than at a patient's residence. Given the level of care and required expertise, the position of the ACR is that proper administration of biologics should take place under the close supervision of a physician. Biologics should be given in a physician's office or medical facility whenever possible to ensure the highest standards of safety for patients. Financial matters related to potential cost savings of home infusions should not override the safety of the patients and standards of practice.

Biologics are currently administered and coded according to the CPT manual, and in accordance with this definition, these agents require direct physician supervision. Thus, not only does the ACR recognize the safest standards of practice, the AMA and CPT have defined the coding regulations requiring oversight by a physician. Again, managed plans and specialty pharmacies should comply with coding regulations set forth by these associations.

There may be rare circumstances in which home infusions could be medically necessary in order for a particular patient to have access to treatment with a biologic. In these highly unusual situations, the increased risk of a home infusion may be outweighed by the risks associated with a lack of access to biologic therapy at all. The ACR encourages providers in such unusual and difficult situations to make the best medical decision based on the individual needs of the patient. The ACR believes that home infusion for the sake of cost-cutting undermines patient safety. Home infusion of biologics is considered an unnecessary and dangerous risk to patients and violates our current clinical standards of practice.

In addition to the safety considerations and compliance issues, forcing patients to perform infusions at home inadvertently threatens to reduce access to these critical therapies. Specialty trained physicians are less likely to prescribe treatments that are not administered properly in the safest clinical setting for their patients.

EXECUTIVE SUMMARY

Given the high value of biologics and their tremendous positive impact on health outcomes, the safety concerns and complexity surrounding these agents, and the lack of evidence for clinical superiority or safety of one agent over another, access to and coverage of biologics should remain fair and equal according to the labeling and standard of care as described in peer-reviewed literature. Access to biologics should be affordable to the plans and to the patients. Step therapies, fail-first policies, tiering, and class switching requirements often create

unnecessary obstacles for patients and their physicians, delays in appropriate therapy, potentially dangerous outcomes for patients, and can undermine careful and collaborative decisions made by patients and providers. In the interest of patient safety, administration of biologics should take place in medical facilities rather than at home. All policies should grandfather patients on stable therapy in such a way that they can affordably continue effective biologic treatment.

RESOURCES

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Note: This position statement was previously titled “Model Biologics Access Policy”.