Office of Medicaid
BOARD OF HEARINGS

Appellant Name and Address:

[redacted]

Appeal Decision:	Denied	**Appeal Number:**	2303376
Decision Date:	6/2/2023	**Hearing Date:**	5/23/2023
Hearing Officer:	Thomas J. Goode	**Aid Pending:**	No

Appearances for Appellant:

[redacted]

Appearances for Commonwealth Care Alliance:
Cassandra Horne, Appeals & Grievances Manager; Jeremiah Mancuso, Clinical RN Appeals Nurse Reviewer; Kaley Ann Emery, Appeals Supervisor; David Mello, MD, CCA Medical Director

The Commonwealth of Massachusetts
Executive Office of Health and Human Services
Office of Medicaid
Board of Hearings
100 Hancock Street, Quincy, Massachusetts 02171

APPEAL DECISION

Appeal Decision:	Denied	**Issue:**	Prior Authorization
Decision Date:	6/2/2023	**Hearing Date:**	05/23/2023
CCA's Reps.:	Cassandra Horne, et. al.	**Appellant's Rep.:**	Appellant, et. al.
Hearing Location:	Remote		

Authority

This hearing was conducted pursuant to Massachusetts General Laws Chapter 118E, Chapter 30A, and the rules and regulations promulgated thereunder.

Jurisdiction

Through a notice dated April 22, 2023, and following a first-level expedited internal appeal, Commonwealth Care Alliance (CCA), a MassHealth Integrated Care Organization (ICO), notified Appellant that it had upheld a denial of Appellant's request for the prescription medication Actemra injection (tocilizumab) because CCA determined the request did not meet CCA pharmacy policy, CCA medical necessity guidelines, or MassHealth medical necessity or pharmacy guidelines (130 CMR 508.007, 406.000 *et seq*. 450.204 and Exhibit 1). Appellant filed this appeal in a timely manner on April 26, 2023 (130 CMR 508.007, 610.015 and Exhibit 2). Denial of a prior authorization request by a MassHealth Integrated Care Organization is valid grounds for appeal (130 CMR 508.007, 610.032(B)).

Action Taken by Commonwealth Care Alliance

Following a first-level expedited internal appeal, Commonwealth Care Alliance (CCA), a MassHealth Integrated Care Organization (ICO), notified Appellant that it had upheld a denial of Appellant's request for the prescription medication Actemra injection (tocilizumab).

Issue

The appeal issue is whether Commonwealth Care Alliance (CCA), a MassHealth Integrated Care Organization (ICO), correctly denied Appellant's request for the prescription medication Actemra injection (tocilizumab).

Summary of Evidence

Appellant appeared telephonically with his mother. Appellant's neurologist also appeared and testified telephonically. CCA was represented telephonically by its Manager of Member Appeals & Grievances, an Appeals Supervisor, a Clinical Appeals Nurse, and a CCA Medical Director. The CCA representatives testified that Appellant has been enrolled in CCA One Care Plan since October 1, 2017. A prior authorization request for Actemra injection (tocilizumab)(hereinafter "Actemra") was submitted by Appellant's neurologist, Dr. Rebecca Gillani, to be administered over 13 visits at Massachusetts General Hospital between [redacted] 2023 and [redacted] 2024. The request was reviewed and denied on [redacted] 2023 by Dr. David Mello, CCA Medical Director. An expedited appeal request was submitted to CCA on April 20, 2023, reviewed by a CCA Medical Director and denied by notice issued on April 22, 2023 (Exhibit 1).

Dr. Mello testified that Appellant's medical history is notable for seizure disorder, optic neuritis, transverse myelitis, spastic hemiplegic cerebral palsy, and demyelinating disease of the central nervous system. Appellant was admitted to the hospital in [redacted] 2023 with Myelin Oligodendrocyte Glycoprotein-Immunoglobulin Associated Disorder (hereinafter "MOGAD"), with left eye optic neuritis, longitudinally extensive transverse myelitis (LETM) of the spinal cord with sensory symptoms of the arm, trunk and leg. During the hospital course Appellant was treated with intravenous Solu-Medrol for 5 days but poor vision continued, and plasmapheresis was attempted. Appellant had a seizure after the second session of plasmapheresis, and the seizure medication Keppra was increased, and he was discharged to complete plasmapheresis as an outpatient. At the third session of plasmapheresis, Appellant had another brief seizure, after which the provider submitted a prior authorization request for Actemra indicating that recent case studies show it may be effective in treating MOGAD. The request was denied because the treatment of the indicated diagnosis is not listed as being FDA approved by the Medicare approved Micromedex compendia. The request was reviewed by a board-certified neurologist at MCMC, an accredited independent review organization, who reviewed the 3 case studies submitted by the requesting provider and concluded that the medical literature is insufficient to support the use of Actemra to treat Appellant's condition (Exhibit 4, pp. 156-159). Dr. Mello testified that the studies show that Actemra may be an effective treatment for MOGAD in the future but are too small to show the safety and efficacy of the medication to treat MOGAD.

The prior authorization request was denied based on the CCA medical necessity guidelines which incorporate both Medicare and MassHealth guidance, as the request is not in accordance with

accepted standards of medical practice, and is not clinically appropriate in terms of type, frequency and duration, or considered effective for treatment of Appellant's specific illness or disease. Dr. Mello added that the determination is also supported by MassHealth guidelines which state that the indication for this specific medication requires an FDA indication or a non-FDA approved indication on a Medicare accepted compendia such as Micromedex (Exhibit 8). The CCA representatives testified that Actemra is on the MassHealth Drug List and requires prior authorization, and must be prescribed specifically for FDA-approved indications, or for non-FDA approved diagnoses, which are reviewed based on current medical evidence and clinical trials (Exhibit 7).

Dr. Gillani identified herself as an expert in Multiple sclerosis and MOGAD, and as an active member of MOGAD research efforts at Massachusetts General Hosptal. Dr. Gillani testified that MOGAD is a very rare demyelinating disease similar to Multiple sclerosis but rarer, and that presents differently from Multiple sclerosis and requires different treatments. She stated that the antibody to make this diagnosis was discovered in 2007 and became widely available for testing in late 2017 and early 2018. She added that MOGAD is a newly discovered disease which involves newly developed treatments. She testified that although clinical trials have begun which will hopefully guide treatment of patients in the next few years, there are currently no FDA approved treatments because MOGAD is a rare disease, and there has been insufficient time to complete clinical trials. In the interim, she relied on case reports submitted with the prior authorization request and her expertise to guide treatment decisions. She described Appellant's case as difficult due to multiple relapses of MOGAD occurring since she began treating him in 2018. When Appellant first developed symptoms of MOGAD, he had 5 different spells of optic neuritis, which is inflammation of the optic nerve resulting in loss of vision. She testified that a number of different treatments were tried, and Appellant was stabilized on Rituximab and Prednisone. Treatment with CellCept was ineffective; and treatment with IVIG (Intravenous immunoglobulin) resulted in aseptic meningitis and pulmonary emboli which could have been a fatal complication. Appellant was stable until [redacted] 2023 when he developed a new and severe episode of optic neuritis, and for the first time a transverse myelitis. Appellant's vision did improve with intravenous Solu-Medrol, and he required treatment with plasma exchange. The plasma exchange sessions were complicated by seizure activity as Appellant has had a seizure disorder since childhood.

Dr. Gillani testified that Appellant is currently stabilized on 20 mg of Prednisone daily, and added that in order to keep Appellant in remission on steroids, he would have to remain on 20 mg daily or more, which will lead to detrimental side effects including vasculopathy and cardiovascular disease, hyperlipidemia, diabetes, weight gain, bone loss, insomnia and many other side effects. Appellant's use of steroids has already been complicated by bilateral Avascular Necrosis (AVN) of the hip, and he underwent total hip replacement on the right in [redacted] 2019, and the left in [redacted] 2021. Appellant is diagnosed with hyperlipidemia, hypertension, osteoporosis, and weight gain due to steroids. Dr. Gillani testified that while steroids are effective at keeping Appellant's autoimmune disease in remission, the side effects from the chronic steroids will soon be worse than the disease. Dr. Gillani testified that the short-term treatment goal is to be able to wean

Appellant off steroids without inducing a relapse, while still maintaining remission. The longer-term goal is to keep Appellant in remission without having to use steroids, which might be accomplished with continued use of Actemra. Dr. Gillani testified that Appellant has already trialed and failed most of the medications used to treat MOGAD. She added that anti-IL-6 therapies including Actemra are promising in the treatment of MOGAD, and the case reports submitted with the prior authorization request show at the level of case reports, that Actemra seems to be effective in reducing the rate of relapse in patients with MOGAD. She stated that Actemra is the best available option to treat Appellant to decrease steroid use.

Dr. Mello testified that the studies submitted are small and would be more authoritative if a larger cohort was used to confirm findings. He added that the studies lack placebo-controlled clinical trials to assess the efficacy and adverse effects of the medication. Dr. Gillani agreed that better quality evidence is needed to help guide treatment of patients diagnosed with MOGAD, but the evidence does not exist today because MOGAD is a newly recognized disorder and there hasn't been sufficient time to complete clinical trials. She stated that there are ongoing clinical trials of Actemra and medications that work in a similar way to Actemra that will hopefully produce evidence to guide treatment of MOGAD in the future. She added that the studies submitted are very encouraging that Actemra is a beneficial treatment for patients with MOGAD. She pointed to the study by Ringelstein, specifically to a diagram of 14 patients showing a reduction in attacks of MOGAD after starting Actemra versus other medications previously trialed (Exhibit 4, p. 42, Figure 1). Dr. Gillani stated that the studies are sufficient evidence of medical necessity because physicians treating rare diseases must make decisions on treatment options based on information that is available and which allow the best medical decision for a patient's wellbeing. She added that in treating Appellant's disease she does not have the luxury of waiting 2 or 3 years for the results of a clinical trial. Dr. Gillani stated that she would not recommend the use of an immunosuppressive medication without taking the risks very seriously, and in Appellant's case, treatment with Actemra is the best option, with no other good alternatives available.

Findings of Fact

Based on a preponderance of the evidence, I find the following:

1. Appellant has been enrolled in CCA One Care Plan since October 1, 2017.

2. A prior authorization request for Actemra injection was submitted by Appellant's neurologist to be administered over 13 visits at Massachusetts General Hospital between [redacted] 2023 and [redacted] 2024.

3. The request was reviewed and denied by CCA on [redacted] 2023. An expedited appeal request was submitted to CCA on April 20, 2023, reviewed by a CCA Medical Director and following a first-level internal appeal, denied by notice issued on April 22, 2023.

4. Appellant's medical history is notable for seizure disorder, optic neuritis, transverse myelitis, spastic hemiplegic cerebral palsy, and demyelinating disease of the central nervous system.

5. Appellant was admitted to a hospital in [redacted] 2023 with Myelin Oligodendrocyte Glycoprotein-Immunoglobulin Associated Disorder ("MOGAD"), with left eye optic neuritis, longitudinally extensive transverse myelitis (LETM) of the spinal cord with sensory symptoms of the arm, trunk and leg. During the hospital course Appellant was treated with intravenous Solu-Medrol for 5 days but poor vision continued, and plasmapheresis was attempted. Appellant had a seizure after the second session of plasmapheresis, and the seizure medication Keppra was increased. He was discharged to complete plasmapheresis as an outpatient. At the third session of plasmapheresis, Appellant had another brief seizure, after which the provider submitted a prior authorization request for Actemra indicating that recent case studies show it may be effective in treating MOGAD.

6. The prior authorization request was denied because the treatment of the indicated diagnosis is not listed as FDA approved by the Medicare approved Micromedex compendia, MassHealth medical necessity criteria, and CCA medical necessity guidelines.

7. The request was reviewed by a board-certified neurologist at MCMC, an accredited independent review organization, who reviewed the 3 case studies submitted by the requesting provider and concluded that the medical literature shows insufficient evidence to support the use of Actemra to treat Appellant's condition.

8. Actemra is on the MassHealth Drug List and requires prior authorization.

9. MOGAD is a very rare demyelinating disease similar to Multiple sclerosis but rarer, presents differently from Multiple sclerosis, and requires different treatments. The antibody to make this diagnosis was discovered in 2007 and became widely available for testing in late 2017 and early 2018.

10. There are currently no FDA approved treatments for MOGAD because it is a rare disease. Although clinical trials have begun which will hopefully guide treatment of patients in the next few years, there has not been sufficient time to do clinical trials.

11. Appellant has experienced multiple relapses of MOGAD since 2018.

12. When Appellant first developed symptoms of MOGAD he had 5 different spells of optic neuritis, which is inflammation of the optic nerve resulting in loss of vision. A number of different treatments were tried, and Appellant was stabilized on Rituximab and Prednisone.

13. Treatment with CellCept was ineffective, and IVIG resulted in aseptic meningitis and pulmonary emboli which could have been a fatal complication.

14. Appellant was stable until [redacted] 2023 when he developed a new severe episode of optic neuritis and for the first time a transverse myelitis. His vision did improve with intravenous Solu-Medrol.

15. Appellant required treatment with plasma exchange with improvement in his vision. The plasma exchange sessions were complicated by seizure activity as Appellant has had a seizure disorder since childhood.

16. Appellant is currently stabilized on 20 mg of Prednisone daily. To keep Appellant in remission on steroids, he would have to remain on 20 mg daily or more, which can lead to detrimental side effects including vasculopathy and cardiovascular disease, hyperlipidemia, diabetes, weight gain, bone loss, insomnia and many other side effects.

17. Appellant's use of steroids has been complicated by bilateral AVN of the hip, and he underwent total hip replacement on the right in [redacted] 2019, and the left in [redacted] 2021.

18. Appellant is diagnosed with hyperlipidemia, hypertension, osteoporosis, and weight gain due to steroids.

19. Appellant has trialed and failed most of the medications used to treat MOGAD.

20. The case studies submitted are small and lack placebo-controlled clinical trials to assess the efficacy and adverse effects of Actemra to treat MOGAD.

Analysis and Conclusions of Law

Appellant is a MassHealth member enrolled in Commonwealth Care Alliance One Care Plan, which is a health plan that contracts with the Commonwealth of Massachusetts Medicaid program (MassHealth) to provide benefits to members who are: age 21 through 64 at the time of enrollment, eligible for MassHealth Standard or CommonHealth, enrolled in Medicare Parts A and B and eligible for Part D, do not have access to other public or private health insurance that meets basic benefit level requirements, live in the CCA One Care service area, and agree to receive all covered medical, behavioral health, and long-term services and supports through CCA (Exhibit 6, p. 11). Pursuant to 130 CMR 508.007(C), when a MassHealth member chooses to enroll in an Integrated Care Organization (ICO), the ICO will deliver the member's primary care and will authorize, arrange, integrate, and coordinate the provision of all covered services for the member. As such, CCA is responsible for authorizing all covered services for Appellant, including pharmacy services in accordance with its medical necessity guidelines and MassHealth

regulations (130 CMR 508.007(A)-(C)). As MassHealth's agent, CCA is required to follow MassHealth regulations. Members enrolled in a managed care contractor have a right to request a fair hearing as further described in 130 CMR 610.032(B) provided the member has exhausted all remedies available through the managed care contractor's internal appeals process (130 CMR 508.010(B)). Appellant exhausted the internal appeal process through CCA, and thus is entitled to a fair hearing pursuant to the above regulations.

At issue is a prior authorization request for Actemra injection (tocilizumab) submitted by Appellant's neurologist, to be administered over 13 visits at Massachusetts General Hospital between [redacted] 2023 and [redacted] 2024. The request was reviewed and denied by CCA on [redacted] 2023. An expedited appeal request was submitted to CCA on April 20, 2023, reviewed by a CCA Medical Director and following a first-level internal appeal, denied by notice issued on April 22, 2023 (Exhibit 1). Appellant has the burden of proving by a preponderance of the evidence the invalidity of the determination by the MassHealth agency or the ICO contracting with MassHealth.[1]

Service Limitations:

(1) MassHealth covers drugs that are not explicitly excluded under 130 CMR 406.413(B). The limitations and exclusions in 130 CMR 406.413(B) do not apply to medically necessary drug therapy for MassHealth Standard and CommonHealth enrollees under age 21. The MassHealth Drug List specifies those drugs that are payable under MassHealth. Any drug that does not appear on the MassHealth Drug List requires prior authorization, as set forth in 130 CMR 406.000. The MassHealth Drug List can be viewed online at www.mass.gov/druglist, and copies may be obtained upon request. *See* 130 CMR 450.303: *Prior Authorization*.

(2) The MassHealth agency does not pay for the following types of drugs, or drug therapies or non-drug products without prior authorization:

(a) immunizing biologicals and tubercular (TB) drugs that are supplied to the provider free of charge through local boards of public health or through the Massachusetts Department of Public Health (DPH); and

(b) any drug, drug therapy, or non-drug product designated in the MassHealth Drug List as requiring prior authorization.

(3) The MassHealth agency does not pay for any drug prescribed for other than the FDA-approved indications as listed in the package insert, except as the MassHealth agency determines to be consistent with current medical evidence.

(4) The MassHealth agency does not pay for any drugs that are provided as a component of a more comprehensive service for which a single rate of pay is established in accordance with 130 CMR 450.307: *Unacceptable Billing Practices*.

[1] See Fisch v. Board of Registration in Med., 437 Mass. 128, 131 (2002) (burden is on appellant to demonstrate the invalidity of an administrative determination).

(130 CMR 406.413 (C)) (emphasis added)

130 CMR 450.204: Medical Necessity

The MassHealth agency will not pay a provider for services that are not medically necessary and may impose sanctions on a provider for providing or prescribing a service or for admitting a member to an inpatient facility where such service or admission is not medically necessary.

(A) A service is "medically necessary" if:

(1) it is reasonably calculated to prevent, diagnose, prevent the worsening of, alleviate, correct, or cure conditions in the member that endanger life, cause suffering or pain, cause physical deformity or malfunction, threaten to cause or to aggravate a handicap, or result in illness or infirmity; and

(2) there is no other medical service or site of service, comparable in effect, available, and suitable for the member requesting the service, that is more conservative or less costly to the MassHealth agency. Services that are less costly to the MassHealth agency include, but are not limited to, health care reasonably known by the provider, or identified by the MassHealth agency pursuant to a prior-authorization request, to be available to the member through sources described in 130 CMR 450.317(C), 503.007, or 517.007.

(B) Medically necessary services must be of a quality that meets professionally recognized standards of health care, and must be substantiated by records including evidence of such medical necessity and quality. A provider must make those records, including medical records, available to the MassHealth agency upon request. (See 42 U.S.C. 1396a(a)(30) and 42 CFR 440.230 and 440.260.)

(C) A provider's opinion or clinical determination that a service is not medically necessary does not constitute an action by the MassHealth agency.

(D) Additional requirements about the medical necessity of acute inpatient hospital admissions are contained in 130 CMR 415.414.

(130 CMR 450.204(A)-(D)).

The MassHealth Drug List specifies the drugs that are payable by MassHealth and designates which drugs require prior authorization.[2] Any drug that does not appear on the MassHealth Drug List

[2] According to the MassHealth Drug List, drugs may require PA for a variety of reasons. MassHealth determines the PA status of drugs on the List on the basis of the following. MassHealth program requirements; and ongoing evaluation of the drugs' utilization, therapeutic efficacy, safety, and cost. Drugs are evaluated first on safety and effectiveness, and second on cost. Some drugs require PA because MassHealth and the Drug Utilization Review Board have concluded that there are more cost-effective alternatives. With regard to all such drugs, MassHealth also has concluded that the more costly drugs have no significant clinically meaningful therapeutic advantage in terms of safety, therapeutic efficacy, or

requires prior authorization. The MassHealth agency evaluates the prior authorization status of drugs on an ongoing basis and updates the MassHealth Drug List accordingly (130 CMR 406.422(E)). MassHealth requires prior authorization for the prescription medication Actemra and its generic tocilizumab (See Exhibit 7, p. 6). Therapeutic uses for Actemra that are FDA-approved, and non-FDA-approved uses for Actemra are listed in the MassHealth Drug list and do not include treatment of Myelin Oligodendrocyte Glycoprotein-Immunoglobulin Associated Disorder (MOGAD), which is not listed in the Evaluation Criteria for Approval of Actemra.[3,4,5] Because Appellant's prior

---

clinical outcome compared to those less-costly drugs used to treat the same condition. Evaluation of a drug includes a thorough review by physicians and pharmacists using medical literature and consulting with specialists, other physicians, or both. References used may include AHFS Drug Information; Drug Facts and Comparisons, Micromedex; National Comprehensive Cancer Network (NCCN); literature from peer-reviewed medical journals; Drug Topics Red Book, Approved Drug Products with Therapeutic Equivalence Evaluations (also known as the "Orange Book"); the Massachusetts List of Interchangeable Drug Products, and manufacturers' product information. MassHealth may impose PA requirements in therapeutic classes in which it has designated a preferred product on the MassHealth Brand Name Preferred Over Generic Drug List or the MassHealth Supplemental Rebate/Preferred Drug List pursuant to the supplemental rebate agreement and preferred brand-name policies described above. The MassHealth Pharmacy Online Processing System (POPS) uses diagnosis codes from medical claims for some drug classes when processing claims at pharmacies. This means that a prescriber may not need to submit a paper PA form if a member's diagnosis in POPS meets the criteria for that drug. MassHealth uses technical software called Smart PA to link diagnosis codes from medical claims during pharmacy claims adjudication. Smart PA is used in the MHDL to identify drugs for which this process is currently available. For this reason, MassHealth requests pharmacies to submit all claims through POPS, as some drugs that are designated as requiring PA on the MHDL will process at the pharmacy without a paper PA submitted. In addition, if the limitations on covered drugs specified in 130 CMR 406.412(A) and 406.413(A) and (C) would result in inadequate treatment for a diagnosed medical condition, the prescriber may submit a written request, including written documentation of medical necessity, to MassHealth for prior authorization for an otherwise noncovered drug. See www.mass.gov/druglist.

[3] Therapeutic Uses: FDA-approved, for example:

- Cytokine release syndrome – Actemra
- Giant cell arteritis – Actemra
- Polyarticular juvenile idiopathic arthritis, moderate-to-severe – Actemra, Enbrel, Humira, Orencia, Simponi Aria, Xeljanz
- Rheumatoid arthritis, moderate-to-severe – Actemra, Avsola, Cimzia, Enbrel, Humira, Inflectra, Kevzara, Kineret, Olumiant, Orencia, Remicade, Renflexis, Rinvoq, Simponi, Simponi Aria, unbranded infliximab, Xeljanz, Xeljanz XR
- Systemic juvenile idiopathic arthritis (sJIA) – Actemra, Ilaris
- Systemic sclerosis-associated interstitial lung disease – Actemra

Non-FDA-approved, for example:

- Polymyalgia Rheumatica (PMR) – Actemra, Kevzara
- Scleritis – Actemra, Avsola, Humira, Inflectra, Remicade, Renflexis, unbranded infliximab
- Uveitis – Actemra, Avsola, Inflectra, Remicade, Renflexis, unbranded infliximab

[4] FDA Approved Uses for Actemra are also summarized under Micromedex:

authorization request for Actemra does not meet the prior authorization requirements for coverage of Actemra or its generic tocilizumab pursuant to MassHealth regulations, it would not be authorized by MassHealth for the treatment of MOGAD.

Commonwealth Care Alliance's Medical Necessity Guideline:

Medical necessity is a term that means health care services or products that a physician would provide to an individual member for the purpose of evaluating, diagnosing, or treating an illness or disease in a manner that is:

1. In accordance with generally accepted standards of medical practice
2. Clinically appropriate, in terms of type, frequency, extent, site, and duration and considered effective for the member's specific illness or disease
3. Not primarily for the convenience of the member, prescribing health care provider, or other health care providers

DECISION GUIDELINES:

Commonwealth Care Alliance (CCA) reviews determinations of medical necessity for services based on federal regulations and coverage criteria including National Coverage Determinations and applicable Local Coverage Determinations, applicable state regulations and coverage criteria, Change Healthcare InterQual® criteria, and CCA Medical Necessity Guidelines. In addition to these criteria, CCA Medical Directors evaluate requests for a specific health care service or product based on this Medical Necessity Guideline and in accordance with Medicare and relevant state Medicaid definitions of medical necessity:

1. CMS describes the "reasonable and necessary" standard for medical necessity in the CMS Program Integrity Manual, including that a service is appropriate, including the

---

- Covid-19, In hospitalized patients receiving systemic corticosteroids and require supplementation oxygen, non-invasive or invasive mechanical ventilation, or extracorporeal membrane oxygenation (ECMO)
- Cytokine release syndrome, Chimeric antigen receptor T-cell induced, severe or life-threatening disease
- Juvenile idiopathic arthritis, Polyarticular
- Lung disease with systemic sclerosis
- Rheumatoid arthritis (Moderate to Severe), In patients who had an inadequate response to disease modifying antirheumatic therapy
- Systemic onset juvenile chronic arthritis
- Temporal arteritis

Non-FDA Uses are:

- Rheumatoid arthritis (Modearte to Severe), With no previous treatment failure
- Thyroid eye disease (Moderate to Severe), Active

(See Exhibit 8)

[5] Evaluation Criteria for Approval of Actemra are also listed (See Exhibit 7, pp. 13-16).

duration and frequency that is considered appropriate for the item or service, in terms of whether it is: Furnished in accordance with accepted standards of medical practice for the diagnosis or treatment of the patient's condition or to improve the function of a malformed body member; Furnished in a setting appropriate to the patient's medical needs and condition; Ordered and furnished by qualified personnel; One that meets, but does not exceed, the patient's medical need; and At least as beneficial as an existing and available medically appropriate alternative.

AND

2. CMS defines medical necessity to only allow Services or Supplies that: are proper and needed for the diagnosis or treatment of your medical condition, are provided for the diagnosis, direct care, and treatment of your medical condition, meet the standards of good medical practice in the local area, and aren't mainly for the convenience of you or your doctor. Consistent with all CCA Medical Necessity Guidelines, CCA uses this MNG as a guide in making individualized coverage determinations. Requesting providers are advised that requests for healthcare services or products under this MNG should be accompanied by clear documentation of medical necessity. Supporting documentation should include justification that the request aligns with accepted standards of medical practice including: (1) Credible scientific evidence in reputable, peer-reviewed medical literature; (2) Physician or Health Care Provider Specialty Society Recommendations; and (3) Other relevant factors specific to the member.

(Exhibit 4, pp. 72-73).

In determining medical necessity, CCA also relied on Medicare Local Coverage Determinations (LCD) published by National Government Services to determine whether Actemra is safe and effective as an off-label use to treat MOGAD in Appellant. LCDs are decisions made by a Medicare Administrative Contractor (MAC) whether to cover a particular item or service in a MAC's jurisdiction (region) in accordance with section 1862(a)(1)(A) of the Social Security Act. MACs are Medicare contractors that develop LCDs and process Medicare claims. The MAC's decision is based on whether the service or item is considered reasonable and necessary.[6] In determining whether there is supportive clinical evidence for a particular use of a drug, the quality of the published evidence must be considered. Such consideration involves the assessment of the following study characteristics:

- The adequacy of the number of subjects;
- The response rate;

[6] See https://www.medicare.gov/search/medicare?keys=Local+Coverage+Determination

- The effect on key status and survival indications. That is, the effect on the patient's well-being and other responses to therapy that indicate effectiveness (e.g., reduction in mortality, morbidity, signs and symptoms);
- The appropriateness of the study design, that is, whether the experimental design in light of the drugs and conditions under investigation is appropriate to address the investigative question. (For example, in some clinical studies, it may be unnecessary or not feasible to use randomization, double blind trials, placebos, or crossover.); and
- The prevalence and life history of the disease when evaluating the adequacy of the number of subjects and the response rate.

(See Exhibit 4, p.63)

Three studies were submitted to CCA with the prior authorization request. The first is: Interleukin-6 Inhibition With Tocilizumab for Relapsing MOG-IgG Associated Disorder (MOGAD): A Case-Series and Review (Exhibit 4, pp. 27-33). The MCMC reviewing neurologist quoted the article's conclusion: "Tocilizumab is an IL-6 inhibitor that may be a promising therapeutic option for patients with relapsing MOGAD that has not responded to other immunotherapies. Our results support a key role for IL-6-related mechanisms in MOGAD disease activity. Its safety and tolerability profile, both in our own experience and based on its use for other FDA approved conditions, may even justify its use as a first line therapy in select patients. Further research is needed to establish the safety and efficacy of IL-6 inhibition in MOGAD" (Exhibit 4, pp. 156-157). The second study is: Off-Label Use of Tocilizumab in Neuromyelitis Optica Spectrum Disorders and MOG-Antibody-Associated Diseases: A Case-Series (Exhibit 4, pp. 34-37). The MCMC reviewing neurologist quoted the article's conclusion: "Our study also shows the potential efficacy of tocilizumab for MOGAD. Further studies with a larger cohort should be conducted to confirm these findings" (Exhibit 4, p. 157). The third study is: Interleukin-6 Receptor Blockade in Treatment-Refractory MOG-IgG-Associated Disease and Neuromyelitis Optica Spectrum Disorders (Exhibit 4, pp. 38-57). The MCMC reviewing neurologist quoted the article's conclusion: "In summary, the results of this metaanalysis showed that tocilizumab treatment has a beneficial effect and tolerable adverse events in NMOSD patients. However, more long-term trials and placebo-controlled clinical drug trials are required to assess efficacy and adverse effects of TCA treatment in NMOSD" (Exhibit 4, p. 157). The MCMC reviewing neurologist reviewed each article and concluded "there is insufficient evidence in the current medical literature to conclude that tocilizumab is safe and effective for this patient's condition. Tocilizumab is not medically necessary for this patient's condition" (Exhibit 4, p. 158).

Dr. Gillani and Dr. Mello agreed that the studies submitted are small, and that better evidence of the effectiveness of Actemra in treating MOGAD would be derived from a larger cohort used to confirm preliminary findings. The studies submitted lack placebo-controlled clinical trials to assess the efficacy and adverse effects of the medication. Moreover, each study concludes that further long-term study is needed with larger cohorts to confirm the initial evidence that Actemra is effective for the treatment of MOGAD. Dr. Gillani testified that although there are ongoing clinical

trials of Actemra and medications that work in a similar way to Actemra that will hopefully produce evidence to guide treatment of MOGAD in the future, better quality evidence is needed which does not exist today because MOGAD is a newly recognized disorder and there has not been sufficient time to do clinical trials to help guide treatment of patients diagnosed with MOGAD. Dr Gillani and Dr. Mello generally agreed that the case studies are too small to definitively show the efficacy of Actemra in treating MOGAD, which corroborates the conclusions of the MCMC board certified neurologist that there is insufficient evidence in the current medical literature to conclude that Actemra (tocilizumab) is safe and effective to treat Appellant's condition. Dr. Gillani's testimony that in Appellant's case, the studies are sufficient evidence of medical necessity because physicians treating rare diseases have to make decisions on treatment options based on information that is available and allows the best medical decision for a patient's wellbeing without waiting 2 or 3 years for the results of clinical trials, does not outweigh the medical evidence, which at this time is inadequate to show that Actemra meets CCA's Medical Necessity Guideline or MassHealth's medical necessity criteria. Therefore, I defer to Dr. Mello's medical testimony which is corroborated by the MCMC neurologist's summation of the case studies and conclude that there is insufficient evidence in the current medical literature to definitively conclude that Actemra (tocilizumab) is safe and effective to treat Appellant's diagnosis of MOGAD.

Accordingly, the appeal is DENIED.

Order for Commonwealth Care Alliance

None.

Notification of Your Right to Appeal to Court

If you disagree with this decision, you have the right to appeal to Court in accordance with Chapter 30A of the Massachusetts General Laws. To appeal, you must file a complaint with the Superior Court for the county where you reside, or Suffolk County Superior Court, within 30 days of your receipt of this decision.

Thomas J. Goode
Hearing Officer
Board of Hearings

cc:
Commonwealth Care Alliance, Attn: Cassandra Horne, 30 Winter Street, Boston, MA 02108
[redacted]