

5.30.010

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<b>Section:</b>	Prescription Drugs	<b>Effective Date:</b>	January 1, 2023
<b>Subsection:</b>	Endocrine and Metabolic Drugs	<b>Original Policy Date:</b>	June 9, 2011
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**Last Review Date:** December 2, 2022

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## Acthar Gel

### Description

#### Acthar Gel (corticotropin; ACTH)

##### Background

According to the US Food and Drug Administration, Acthar gel (repository corticotropin injection, ACTH) was approved for marketing in 1952. Since that time Acthar gel has shown to produce positive therapeutic outcomes in disease states such as infantile spasms, nephrotic syndrome and multiple sclerosis (1).

Effectiveness of Acthar Gel (ACTH) for treatment of infantile spasms was shown in a single blinded clinical trial in which patients received either a 2 week course of treatment with Acthar Gel or prednisone. The study compared the number of patients in each group who were treatment responders (1).

Studies have also shown that patients with nephrotic syndrome have had successful outcomes with Acthar Gel after failing other therapies. ACTH treatment produced a lasting remission with few side effects. Findings showed monotherapy ACTH was as effective for nephrotic patients as the combination therapy of methylprednisolone and a cytotoxic agent (1-2).

Studies of the use of Acthar Gel for multiple sclerosis have showed a protective effect against progression, stabilization of the disease, and also marked improvement in patients with acute relapse of MS after the use of ACTH (1-3).

##### Regulatory Status

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FDA-approved indications: Acthar gel is an adrenocorticotrophic hormone (ACTH) which is indicated for: (1)

1. Treatment of infantile spasms in infants and children under 2 years of age
2. Treatment of exacerbations of multiple sclerosis in adults over 18 years of age
3. Treatment of nephrotic syndrome without uremia of the idiopathic type or that due to lupus erythematosus to induce a diuresis or a remission
4. Acthar Gel may be used for the following disorders and diseases: rheumatic; collagen; dermatologic; allergic states; ophthalmic; respiratory; and edematous state

Acthar Gel should never be given intravenously. Administration of live or live attenuated vaccines is contraindicated in patients receiving immunosuppressive doses of Acthar Gel (1).

Acthar Gel is contraindicated in patients with scleroderma, osteoporosis, systemic fungal infections, ocular herpes simplex, recent surgery, history of or the presence of a peptic ulcer, congestive heart failure, uncontrolled hypertension, primary adrenocortical insufficiency, adrenocortical hyperfunction or sensitivity to proteins of porcine origin (1).

Patients with nephrotic syndrome (NS) show a combination of clinical and laboratory features of renal diseases characterized by heavy proteinuria, hypoalbuminemia, and peripheral edema, with hyperlipidemia also frequently seen. Nephrotic-range proteinuria is the loss of 3 grams or more per day of protein into the urine or on a single spot urine collection, the presence of 2 g of protein per gram of urine creatinine. Nephrotic syndrome is the combination of nephrotic-range proteinuria with a low serum albumin level and edema (4-5).

An exacerbation of MS (also known as a relapse, attack or flare-up) causes new symptoms or the worsening of old symptoms. It can be very mild, or severe enough to interfere with a person's ability to function at home and at work. No two exacerbations are alike, and symptoms vary from person to person and from one exacerbation to another. To be a true exacerbation, the attack must last at least 24 hours and be separated from the previous attack by at least 30 days (6).

Acthar Gel is contraindicated in children less than 2 years of age with suspected congenital infections (1).

### **Related policies**

Ampyra, Aubagio, Cortrophin Gel, Gilenya, Kesimpta, Lemtrada, Mavenclad, Mayzent, MS Injectables, Ocrevus, Ponvory, Tecfidera, Tysabri, Zeposia

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## Policy

*This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.*

Acthar Gel may be considered **medically necessary** in patients with infantile seizures, exacerbations of multiple sclerosis, or nephrotic syndrome and if the conditions indicated below are met.

Acthar gel may be considered **investigational** for all other indications.

## Prior-Approval Requirements

### Diagnoses

Patient must have **ONE** of the following:

1. Infantile spasms (in children < 2 years of age)
  - a. Prescribed by a neurologist
  - b. **NO** dual therapy with Cortrophin Gel (corticotropin)
  
2. Exacerbations of multiple sclerosis (in adults ≥18 years of age)
  - a. Prescribed by a neurologist
  - b. Used in combination with a maintenance MS therapy
  - c. Submission of medical records (e.g., chart notes, laboratory values) documenting **ONE** of the following:
    - i. FDA labeled contraindication to oral or parenteral glucocorticoid therapy
    - ii. An inadequate response or intolerance to a 1 month trial of oral or a 1 week trial of parenteral glucocorticoid therapy
  - d. **NO** dual therapy with Cortrophin Gel (corticotropin)
  - e. Patient **MUST** have tried the preferred product (Cortrophin Gel) unless the patient has a valid medical exception (e.g., inadequate treatment response, intolerance, contraindication)
  
3. Nephrotic syndrome
  - a. Prescribed by a nephrologist

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- b. Submission of medical records (e.g., chart notes, laboratory values) documenting an inadequate response or intolerance to a 1 month trial of **ONE** of the following:
  - i. Oral glucocorticoid therapy
  - ii. Immunosuppressant such as:
    - 1. Cyclophosphamide
    - 2. Cyclosporine
    - 3. Tacrolimus
    - 4. Mycophenolate mofetil
- c. Submission of medical records (e.g., chart notes, laboratory values) documenting baseline levels of protein in urine indicative of proteinuria and low levels of albumin in blood indicative of hypoalbuminemia
- d. **NO** dual therapy with Cortrophin Gel (corticotropin)
- e. Patient **MUST** have tried the preferred product (Cortrophin Gel) unless the patient has a valid medical exception (e.g., inadequate treatment response, intolerance, contraindication)

All approved requests are subject to review by a clinical specialist for final validation and coverage determination once all required documentation has been received. Current utilization, including samples, does not guarantee approval of coverage.

## Prior – Approval *Renewal* Requirements

### Diagnoses

Patient must have **ONE** of the following:

- 1. Infantile spasms (in children < 2 years of age)
  - a. Prescribed by a neurologist
  - b. **NO** dual therapy with Cortrophin Gel (corticotropin)
- 2. Exacerbations of multiple sclerosis (in adults ≥18 years of age)
  - a. Prescribed by a neurologist
  - b. Used in combination with a maintenance MS therapy
  - c. Submission of medical records (e.g., chart notes, laboratory values) documenting 30 day lapse since previous exacerbation
  - d. **NO** dual therapy with Cortrophin Gel (corticotropin)

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- e. Patient **MUST** have tried the preferred product (Cortrophin Gel) unless the patient has a valid medical exception (e.g., inadequate treatment response, intolerance, contraindication)
3. Nephrotic syndrome
- a. Prescribed by a nephrologist
  - b. Submission of medical records (e.g., chart notes, laboratory values) documenting a decrease in urine protein level and increase serum albumin level
  - c. **NO** dual therapy with Cortrophin Gel (corticotropin)
  - d. Patient **MUST** have tried the preferred product (Cortrophin Gel) unless the patient has a valid medical exception (e.g., inadequate treatment response, intolerance, contraindication)

All approved requests are subject to review by a clinical specialist for final validation and coverage determination once all required documentation has been received. Current utilization, including samples, does not guarantee approval of coverage.

## Policy Guidelines

### Pre - PA Allowance

None

### Prior - Approval Limits

#### Duration

Infantile spasms	1 month
Exacerbations of multiple sclerosis	1 month
Nephrotic syndrome	6 months

### Prior – Approval *Renewal* Limits

Same as above

## Rationale

### Summary

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Acthar Gel stimulates the release of endogenous cortisol. It is approved for a number of indications that are more generally treated with corticosteroids. Indications that are supported by published clinical literature are covered by the prior approval criteria (1-6).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Acthar Gel while maintaining optimal therapeutic outcomes.

### References

1. Acthar gel [package insert]. Bedminster, NJ: Mallinckrodt Pharmaceuticals Inc.; October 2021.
2. Bomback AS, Tumlin JA, Baranski J, et al. Treatment of nephrotic syndrome with adrenocorticotrophic hormone (ACTH) gel. *Drug Des Devel Ther.* 2011; 14(5):147-53.
3. Filippini G, Brusaferrri F, et al. Corticosteroids or ACTH for acute exacerbations in multiple sclerosis. *Cochrane Database Syst Rev* 2000 CD001331.
4. Madan A, Mijovic-Das S, et al. Acthar gel in the treatment of nephrotic syndrome: a multicenter retrospective case series. *BMC Nephrology* (2016) 17:37.
5. Gupta K, Iskandar SS, Daeihagh P, et al. Distribution of pathologic findings in individuals with nephrotic proteinuria according to serum albumin. *Nephrol Dial Transplant.* 2008 May. 23(5):1595-9.
6. Sellebjerg F, Barnes D, Filippini G, et al. EFNS guidelines on treatment of multiple sclerosis relapses: report of an EFNS task force on treatment of multiple sclerosis. *European Journal of Neurology* 2005; 12:939-946.

### Policy History

Date	Action
April 2011	Updated criteria to mirror FDA indications for infantile spasms in infants and children less than 2 years of age and exacerbations of multiple sclerosis in adults.
May 2012	Updated criteria to include FDA indication for nephrotic syndrome
March 2013	Annual editorial review. Remove tried and failed corticosteroid from infantile spasms. Addition of the following to the criteria: Not intended for IV administration, patient must not have scleroderma, osteoporosis, systemic fungal infections, ocular herpes simplex, recent surgery, history of or the presence of a peptic ulcer, congestive heart failure, uncontrolled hypertension, or sensitivity to proteins or porcine origin. No administration of live or live attenuated vaccines with immunosuppressive doses of Acthar Gel. No congenital infections in children under 2 years of age. Revised limitations to 6 months in light of use for nephrotic syndrome and MS.

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June 2013	Annual editorial review and reference update
June 2014	Annual editorial review and reference update
December 2014	Annual editorial review and reference update
March 2015	Annual editorial review and reference update
May 2015	Addition of specialist and change of duration on approvals for MS and infantile spasms
June 2015	Annual editorial review and reference update
September 2015	Annual review
September 2016	Annual editorial review and reference update Addition of inadequate response to a 3 month trial of parenteral glucocorticoids therapy to MS and Nephrotic indications Addition of Inadequate response to a 3 month trial of interferon beta therapy Policy number change from 5.08.10 to 5.30.10
December 2016	Annual editorial review
June 2017	Added to Managed PA
December 2017	Annual review
February 2018	Rewording of the 3 month trial of glucocorticoids and the interferon beta requirements and the addition of oral glucocorticoids to MS and Nephrotic indications
March 2018	Annual review
October 2018	Change of initiation requirement to inadequate response, intolerance or contraindication to a 1 month trial of oral or 1 week trial of parenteral glucocorticoids. Change in nephrotic syndrome requirement to include oral glucocorticoids or an immunosuppressant trial
November 2018	Annual review
September 2019	Annual review and reference update
March 2020	Annual review
September 2020	Annual review
December 2020	Annual review
March 2021	Annual review and reference update
June 2021	Annual review and reference update
March 2022	Annual review and reference update
April 2022	Per FEP, addition of no dual therapy with Cortrophin Gel
June 2022	Annual review
December 2022	Annual review. Addition of requirement to t/f preferred product Cortrophin Gel for MS and Nephrotic Syndrome indications

## Keywords

**This policy was approved by the FEP® Pharmacy and Medical Policy Committee on December 2, 2022 and is effective on January 1, 2023.**