H. P. Acthar Gel

Description

H. P. Acthar Gel (corticotropin; ACTH)

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

Effectiveness of H.P. 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 H.P. Acthar Gel or prednisone. The study compared the number of patients in each group who were treatment responders. Acthar had a significantly higher response rate compared to prednisone. Thirteen of 15 patients (86.7%) responded to Acthar Gel monotherapy as compared to 4 of 14 patients (28.6%) given prednisone. Afterwards the prednisone nonresponders were given the choice to receive H.P. Acthar Gel treatment. Seven of 8 patients (87.5%) responded to H.P Acthar Gel after not responding to prednisone (2).

Studies have also shown that patients with nephrotic syndrome have had successful outcomes with Acthar Gel after failing other therapies (3,4). The Bomback, et al study demonstrated that ACTH gel produced remission rates approaching 80% (3). Also the Rauen, et al study concluded ACTH treatment produced a lasting remission with few side effects (5). Ponticelli, et al performed a study which compared the combination therapy of methylprednisolone with a cytotoxic agent and
monotherapy of ACTH. The findings showed monotherapy ACTH was as effective for nephrotic patients as the combination of methylprednisolone and a cytotoxic agent (6).

Filippini, et al produced a study of the use of H.P. Acthar Gel for multiple sclerosis in two randomized, double-blind trials. ACTH showed a protective effect against progression and stabilization of the disease (7). Thompson, et al's trial showed marked improvement in patients with acute relapse of MS after the use of ACTH (8). The study from Hauser, et al determined that combination therapy with cyclophosphamide and ACTH stabilized patients’ progressive MS (9).

Approved indications that are not supported by the clinical literature have been excluded from prior approval criteria.

Regulatory Status
FDA-approved indications: Acthar gel is an adrenocorticotropic hormone (ACTH) which is indicated for:
(2)
- Treatment of infantile spasms in infants and children under 2 years of age
- Treatment of exacerbations of multiple sclerosis in adults under 18 years of age
- Treatment of nephrotic syndrome without uremia of the idiopathic type or that due to lupus erythematosus to induce a diuresis or a remission.
- H.P. Acthar Gel may be used for the following disorders and diseases: rheumatic; collagen; dermatologic; allergic states; ophthalmic; and respiratory.

H.P. Acthar Gel should never be given intravenously.

Administration of live or live attenuated vaccines is contraindicated in patients receiving immunosuppressive doses of H.P. Acthar Gel.

H.P. 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.

H.P. Acthar Gel is contraindicated in children under 2 years of age with suspected congenital infections.

Related Policies
**Policy**

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

H.P. Acthar Gel may be considered **medically necessary** for the treatment of infantile seizures, exacerbations of multiple sclerosis, and nephrotic syndrome. It is 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)
2. Exacerbations of multiple sclerosis (in adults ≥18 years of age)
   a. Tried and failed corticosteroid therapy
3. Nephrotic syndrome
   a. Tried and failed corticosteroid therapy

**AND** not given intravenously

**Prior – Approval Renewal Requirements**

Same as above

**Policy Guidelines**

**Pre - PA Allowance**

None

**Prior - Approval Limits**

Duration 6 months

**Prior – Approval Renewal Limits**

Duration 6 months
Rationale

H.P. 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.

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

References

## Policy History

<table>
<thead>
<tr>
<th>Date</th>
<th>Action</th>
<th>Reason</th>
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<tbody>
<tr>
<td>April 2011</td>
<td>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.</td>
<td>(4)</td>
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<tr>
<td>May 2012</td>
<td>Updated criteria to include FDA indication for nephrotic syndrome</td>
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<tr>
<td>March 2013</td>
<td>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.</td>
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### Keywords

Annual editorial review and reference update

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This policy was approved by the FEP Pharmacy and Medical Policy Committee on June 6, 2013 and is effective July 1, 2013

**Signature on File**

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