



ACTHAR GEL (corticotropin [ACTH])

RATIONALE FOR INCLUSION IN PA PROGRAM

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

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



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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).

Summary

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



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