

Modern Care for Congenital Adrenal Hyperplasia: New Horizons in Management

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Abstract

Congenital adrenal hyperplasia (CAH), most commonly caused by 21-hydroxylase deficiency, is an autosomal recessive disorder characterized by impaired cortisol and aldosterone synthesis with androgen excess. Conventional management with lifelong glucocorticoid and mineralocorticoid replacement often fails to prevent adrenal crises and long-term complications while exposing patients to adverse effects of supraphysiologic steroid doses.

Recent advances are reshaping management strategies. Modified-release and continuous subcutaneous hydrocortisone formulations provide more physiologic cortisol replacement, improving hormonal control and quality of life. Novel pharmacologic agents such as crinecerfont, a corticotropin-releasing factor receptor antagonist and the first FDA-approved therapy for classic CAH, and investigational tildacerfont reduce ACTH-driven androgen production, thereby lowering glucocorticoid requirements. Future approaches include melanocortin 2 receptor (MC2R) antagonists, which block ACTH action directly at the adrenal cortex, offering a targeted means of suppressing androgen excess. Additional innovations include micro-dosed pediatric hydrocortisone granules, androgen receptor antagonists, and experimental gene and cell therapies with curative potential.

Beyond pharmacological treatment, modern care emphasizes fertility preservation, cardiometabolic health, psychological support, and shared decision-making regarding genital surgery. These evolving strategies represent a paradigm shift from traditional hormone replacement toward individualized, outcome-focused care, offering renewed prospects for improved growth, fertility, and quality of life in patients with CAH. [*J Assoc Clin Endocrinol Diabetol Bangladesh*, 2025;4(Suppl 1): S21]

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