

Clinical Trials in CAH: So Many Starting, but Not So Many Finishing

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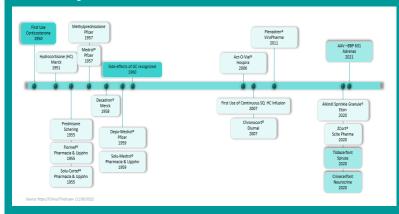
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Background: Since the introduction of hydrocortisone in 1951, fludrocortisone in 1955, and various synthetic glucocorticoids throughout the 1950's, there have been very few therapeutic advances in the treatment of congenital adrenal hyperplasia (CAH) until recently.

Therapeutic Advances in CAH 1950-2022



Objectives: To better understand research trends in CAH, we sought to:

- (1) Compare the number of registered pediatric and adult CAH trials (non-interventional = NIT and interventional = IT)
- (2) Determine the completion rate of both pediatric and adult NIT and IT CAH trials between 2000-2009 and 2010-2019

Methods: We identified all registered trials in ClinicalTrials.gov with start dates between January 1, 2000, and December 31, 2019, listing CAH as the target condition. We then determined the numbers of new pediatric and adult NIT and IT CAH trials, and their completion rates, between 2000-2009 and 2010-2019, based on clinicaltrial.gov designation through November 30, 2022. Trials that began after December 2019 were intentionally omitted to avoid confounding effects from SARS-CoV-2 infection.

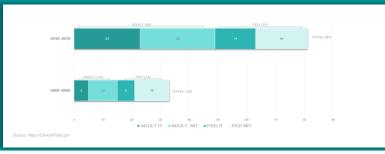
RESULTS (I): CAH Trials Map 2000-2009 vs. 2010-2019



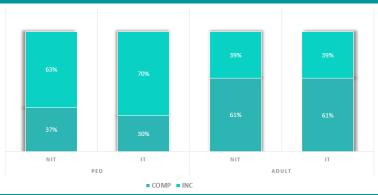
RESULTS (II): CAH Ped vs. Adult IT Initiated between 2000-2019



RESULTS (III): CAH Clinical Trials Initiated Between 2000-2009 vs. 2010-2019



RESULTS (IV): CAH NIT/IT Completion Rates 2000-2019



RESULTS (V): CAH Trial Stats NIT/IT2000-2019

CAH NIT/IT INITIATED (INIT) AND COMPLETED (COMP) 2000-2019						
YEARS	PEDIATRIC CAH TRIALS					
	NIT INIT	NIT COMP	%COMP	IT INIT	IT COMP	%COMP
2000-2009	12	6	50%	6	4	66.7%
2010-2019	18	5	27.8%	14	2	14.3%
2000-2019	30	11	36.7%	20	6	30%
YEARS	ADULT CAH TRIALS					
	NIT INIT	NIT COMP	%COMP	IT INIT	IT COMP	%COMP
2000-2009	10	7	70.0%	5	5	100%
2010-2019	26	15	57.7%	23	12	52.2%
2000-2019	36	22	61.1%	28	17	60.7%

Conclusions: The number of both pediatric and adult CAH clinical trials (both interventional and non-interventional) has increased dramatically decade-by-decade since 2000. However, the number of trials that are completed is less than one-third in pediatrics and less than one-half in adults. In all groups, the rate of CAH trial completion has fallen comparing 2000-2009 to 2010-2019. These findings underscore an outpacing of CAH clinical trial options to patient participation and highlight the need for flexible and accessible protocols as well as creative strategies to maximize study recruitment and retention.