Abiraterone in CAH

Why is this study being done?

Patients with the severe, classic form of congenital adrenal hyperplasia (CAH) due to 21-hydroxylase deficiency are unable to synthesize cortisol (stress hormone) or aldosterone (salt retaining hormone) normally. These deficiencies can be treated with hydrocortisone or fludrocortisone, respectively. In patients who are not getting enough hydrocortisone, the adrenal gland secretes large amounts of androgens (male sex hormones). Excess androgens can cause the growth plates in long bones to close too early, leading to short adult height. Controlling androgen levels may require relatively high doses of hydrocortisone that can themselves slow down growth. If androgen synthesis could be blocked in prepubertal children, this might allow us to use lower doses of hydrocortisone and eventually result in CAH patients being taller as adults.

Who can participate in this study?

To take part in this study, you must:

• Have classic CAH due to 21-hydroxylase deficiency
• Be 2 to 8 (girls) or 2 to 9 (boys) years old
• Be taking both hydrocortisone and fludrocortisone
• Not be in puberty

What will volunteers be asked to do?

• Have a physical exam, blood and urine tests, X-ray of the hand, electrocardiogram (ECG) and eye exam
• Take abiraterone acetate powder daily by mouth for 7 days.
• Attend 6 (but up to 8) study visits in person and 3 (but up to 5) telephone calls during the 11 week study

Volunteers will receive, at no cost, study-related:

• Close monitoring of their CAH
• Direct access to doctors or nurses to answer questions
• Compensation for study visits

Where will this study take place?

There are 4 study centers nationwide.
South Central: The main site is in Dallas, Texas, at UT Southwestern Medical Center/ Children's Medical Center Dallas.
East Coast: National Institutes of Health, Bethesda, Maryland
North Central: University of Michigan, Ann Arbor, Michigan
West Coast: Childrens Hospital Los Angeles, Los Angeles, California

What does abiraterone have to do with CAH?

• This study is being done to determine whether an investigational drug called abiraterone acetate, which blocks androgen synthesis, is safe to use in children with CAH, and to determine the lowest dose which works to reduce androgen levels to normal.
• This is a research study because although abiraterone has been approved by the U.S. Food and Drug Administration (FDA) for other indications, it has not been approved by the FDA for the treatment of CAH.
For more information on the Abiraterone in CAH study, please contact us:

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