INFORMED CONSENT FORM

Official title: A Phase 1-2 Multi-Center Study to Assess the Efficacy and Safety of Abiraterone Acetate as Adjunctive Therapy in Pre-Pubescent Children with Classic 21-Hydroxylase Deficiency

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Children's Medical Center

CONSENT TO PARTICIPATE IN RESEARCH

Title of Research: A Phase 1-2 Multi-Center Study to Assess the Efficacy and Safety of Abiraterone Acetate as Adjunctive Therapy in Pre-Pubescent Children with Classic 21-Hydroxylase Deficiency: Phase I

Sponsor: UT Southwestern Medical Center
Funding Agency: National Institutes of Health

Study Doctors: Perrin White, MD Soumya Adhikari, MD
Ming Yang, MD

You may call these study doctors or research personnel during regular office hours and after hours at 214-456-5959.

Note: If you are a parent or guardian of a minor and have been asked to read and sign this form, the "you" in this document refers to the minor.

Instructions:
Please read this consent form carefully and take your time making a decision about whether to participate. As the researchers discuss this consent form with you, please ask him/her to explain any words or information that you do not clearly understand. The purpose of the study, risks, inconveniences, discomforts, and other important information about the study are listed below. If you decide to participate, you will be given a copy of this form to keep.

Why is this study being done?

People who are diagnosed with congenital adrenal hyperplasia (CAH) can have high levels of male sex hormones called androgens in their blood and must take medication daily to keep the body from making too much of these hormones. This study is Phase 1 of a study being done to find out whether an FDA-approved drug called abiraterone acetate in combination with standard medications (hydrocortisone and fludrocortisone) can treat CAH better or more safely in children than standard medications alone. In this Phase 1 study, the researchers will learn the lowest dose of abiraterone acetate needed to combine with standard medication for the treatment of children with CAH to keep androgens in the blood within normal levels. The study will determine if the drug is well tolerated in children with CAH for the one week that each subject is taking the drug. This phase of the study will not provide treatment and is being done only to find the best dose to use for Phase 2 of the study. You will be invited to participate in Phase 2, the treatment study, if you qualify.

Why is this considered research?

This is a research study because abiraterone acetate has not been approved by the U.S. Food and Drug Administration (FDA) for the treatment of CAH, or for the treatment of any condition in children

- Abiraterone acetate in combination with prednisone is indicated for the treatment of patients with metastatic castration-resistant prostate cancer (adult men with prostate cancer that has spread to other parts of the body). The researchers are interested in learning if taking

abiraterone acetate in combination with standard medication for CAH will result in normal levels of androgens in the blood and less reduction in adult height and less excess weight gain in people treated for CAH. Abiraterone acetate has not previously been given to children.

The following definitions may help you understand this study:

- Standard medical care means the regular care you would receive from your personal doctor if you choose not to participate in this research.
- Researchers means the study doctor and research personnel at the University of Texas Southwestern Medical Center at Dallas and its affiliated hospitals.

Why am I being asked to take part in this research study?

You are being asked to take part in this study because you weigh at least 12 kg (26 lbs) and are a female 2-9 years old or a male 2-10 years old and have been diagnosed with CAH, and you are currently treated for CAH using standard of care medications (hydrocortisone and fludrocortisone).

Do I have to take part in this research study?

No. You have the right to choose whether you want to take part in this research study.
If you decide to participate and later change your mind, you are free to stop participation at any time.

If you decide not to take part in this research study, it will not change your legal rights or the quality of health care that you receive at this center.

How many people will take part in this study?

Up to 36 children will be enrolled in this study at up to 4 sites across the United States. Up to 15 children will be enrolled in this study at Children's Medical Center Dallas,

What is involved in the study?

If you volunteer to take part in this research study, you will be asked to sign this consent form and will have the following tests and procedures. Some of the procedures may be part of your standard medical care, but others are being done solely for the purpose of this study. You will probably have 9 study visits (6 clinic visits and 3 telephone call visits) over up to 74 days. However, you may have up to 13 visits (up to 8 clinic visits and up to 5 telephone calls) over 81 days if you need to have Optional Visits and Phone Visits 2A and 4A (see below).

<u>Group Assignment</u>

If the researchers believe you can take part in this study, you will be assigned to the study medication dose group currently enrolling or the next group if the current group is full. Up to 3 groups of 8 participants are planned to receive a different dose of study medication starting with the lowest dose first. Each group will receive double the dose of the previous group until the dose that keeps androgens near normal levels is found. Planned doses are approximately 2 and 4 mg/kg/day. We will tell you which group you are assigned to. Doses within this range have been tested in a small group of adult women with CAH and the medication was well tolerated. We may stop the study after only one or two groups have been tested if the study drug works to control androgen levels in the blood with one of the lower dose levels. If the highest dose planned does not work and the drug has not caused problems, an additional group testing a higher dose may be added.

<u>Study Medication/Intervention</u>

If you decide to participate in this study, you will have a lead-in period which could last up to 30 days. During the lead-in period you will not be taking the study medication. The study doctor will decrease

your standard hydrocortisone dose based on your body size as part of the research study. After your hydrocortisone dose is adjusted, you will get blood lab tests to see if you still qualify to participate in the study. If you still qualify for the study, you will begin the 7-day treatment period and will take:

- Abiraterone acetate (study medication) each day by mouth. The study medication will be in tablets which may be swallowed or crushed in a small amount of water. The researchers will provide instructions with additional suggestions.

Procedures and Evaluations during the Research

Clinic Visit 1 - Screening Procedures (about 3 hours)
To help decide if you qualify to continue in this study, the researchers will ask you questions about your health, including medications you take and any surgical procedures you have had.

You may also have to fill out certain forms and have the following exams, tests or procedures:

- Physical exam and medical history
- Demographic information (age, sex, ethnic origin)
- Vital signs; to include height, weight, temperature, blood pressure, heart rate
- Electrocardiogram (EKG), a tracing of the electrical activity of the heart
- Urinalysis
- Eye examination for cataracts (clouding of the lens of the eye). If this is not done it Visit 1, it may be scheduled separately at any time before, or at, Clinic Visit 2.
- Skeletal age assessment (by wrist x-ray)
- Registration in central study database
- At this and every visit medications you are taking and any recent illness and health events will be reviewed.
- Blood tests (up to 3-4 teaspoons of blood will be drawn);
  - hematology panel, metabolic panel, chemistry, lipids, liver function, hormones, plasma renin activity
  - samples to freeze and bank for future determination of abiraterone levels. All banked samples will be destroyed at study conclusion (up to 5 years from now)
  - genetic test to confirm diagnosis of CAH (if not already available or if information needed for the study is missing). We will also ask your parents for a blood sample to help identify the type of CAH you have.

- What is genetic testing?

Genetic tests look for naturally occurring differences in a person's genes, or the effects of specific genes. These differences could indicate an increased chance of getting a disease or condition. Genetic testing includes gene tests (DNA testing) and sometimes biochemical tests (protein testing) if it relates to a specific gene.

In gene tests, DNA in cells taken from a person's blood, body fluids or tissues is examined for differences. The differences can be relatively large - a piece of a chromosome, or even an entire chromosome, missing or added. Sometimes the change is very small - as little as one extra, missing or altered chemical within the DNA strand.

- What is DNA?

DNA means deoxyribonucleic acid. DNA is the substance in our cells which contains information we

inherited from our parents and other family members. Your DNA contains “genes” which predict things like physical characteristics (eye color, hair color, height, etc.) and may also be a factor in whether you develop or are at risk of developing certain illnesses or disorders.

The genetic testing being done for this study is to learn about the type of CAH you have. All samples collected for genetic testing will be destroyed at the end of the study and not retained for future use. It is up to you whether to allow this testing. If you do not wish to have this genetic testing, you will not be able to participate in this study because the researchers need to know which form of CAH you have to know if you qualify for this study.

Your samples for genetic testing will be analyzed by an independent laboratory. Your genetic samples will be securely stored and accessible only to study team members or lab personnel processing and shipping the samples going to Quest Laboratory for analysis.

Phone Visit 1 – Lead-in Period (about 30 minutes)
When your lab results are available the study team will contact you to review the results. If you qualify to stay in the study, you will be instructed to reduce your daily hydrocortisone dose to a target amount based on your body size. This target dose is at the lower end of the dose range used to treat CAH. You will also be given instructions about your next visit and be asked to come to the clinic or to the lab for a blood draw in about 2 weeks.

Clinic Visit 2 – Lead-in Period (about 40 minutes and can be repeated once)
You will be instructed to go to the clinic or Quest lab before taking your morning dose of CAH medications (hydrocortisone and fludrocortisone) and have a blood draw between 8:00 and 10:00 AM (up to 1/2 teaspoon of blood will be drawn).

Phone Visit 2 – Lead-in Period (about 30 minutes and can be repeated once) When your lab results are available the study team will contact you to review the results. In particular, your level of a particular androgen, androstenedione, must be elevated to at least one and one half (1.5) times the upper limit of normal. If you qualify to stay in the study, you will be given instructions about your next visit to the clinic to begin the 7 day Treatment Period.

If your androstenedione level is not that high, you will be given instructions to repeat Clinic Visit 2 and Phone Visit 2 (the repeat visits are termed Clinic and Phone Visits 2A).

At the time of the next Phone Visit (2A) if your androstenedione level is more than times the upper limit of normal you will be given instructions about your next visit to the clinic to begin the 7-day Treatment Period. If your androstenedione level is less than this level, you will be instructed to resume your standard of care CAH medications as prescribed prior to participation in the study. Your participation in the study will end at this time because you do not qualify to continue participation.

Clinic Visit 3 – Treatment Period Day 1 FASTING VISIT (about 5 hours)
You will be instructed to go to the clinic before eating and before taking your morning dose of CAH medications (hydrocortisone and fludrocortisone). You will come to the clinic between 8:00 and 10:00 AM and have the following procedures:

- Vital signs; to include height, weight, temperature, blood pressure, heart rate
- An intravenous catheter (IV) will be placed to allow for several blood draws without an additional needle stick.

- Blood tests (up to 1 1/2 teaspoons of blood will be drawn).
- First dose of study medication will be given.
- 30 minutes after taking study medication, you will be given a low fat breakfast or snack.
- 1, 2 and 4 hours after taking the study medication you will have a blood draw (up to 1/2 teaspoon of blood).
- Following the last blood draw your daily dose of hydrocortisone and fludrocortisone will be given using your home supply of these medications.
- The IV will then be removed.
- You will be given a diary with instructions for completing it.
- You will be given a supply of study medication and instructions on how to take the medication at home. The study medication will be taken on an empty stomach each morning.
- In total, 4-5 teaspoons of blood will be taken

Clinic Visit 4-Treatment Period Day 2-FASTING VISIT (about 1 hour)
You will be instructed to go to the clinic before eating and before taking your morning dose of CAH medications (study medication, hydrocortisone and fludrocortisone). You will come to the clinic between 8:00 and 10:00 AM and have the following procedures:

- Blood tests (up to 1 1/2 teaspoons of blood will be drawn).
- Dose of study medication will be given.
- Your daily dose of hydrocortisone and fludrocortisone will be given.
- 30 minutes after taking study medication, you will be given a low fat breakfast or snack.

If you become aware before Clinic Visit 5 that you have missed any dose of study medicine, please inform the study doctors as soon as possible. You may be asked to repeat Clinic Visit 4 (the repeat visit is Visit 4A) and the results of the lab tests will be reviewed with you in an additional Telephone Visit 4A. You may be asked to restart the 7-day treatment period before Clinic Visit 5.

Clinic Visit 5 – Treatment Period Day 8 END OF TREATMENT VISIT (about 2 hours)
You will be instructed to go to the clinic before eating and before taking your morning dose of CAH medications (hydrocortisone and fludrocortisone). You will come to the clinic between 8:00 and 10:00 AM and have the following procedures:

- You will be asked to return the diary you have been keeping.
- Vital signs; to include height, weight, temperature, blood pressure, heart rate
- Physical examination
- Blood tests (about 2-3 teaspoons of blood will be drawn.)
- Electrocardiogram (EKG), a tracing of the electrical activity of the heart
- Your daily dose of hydrocortisone and fludrocortisone will be given.
- You will be instructed to resume the doses of hydrocortisone that you were on prior to starting the study.
- You will be instructed to return to the clinic or lab for the next visit in about 2 weeks.

Clinic Visit 6 – Follow-up Period (about 40 minutes)
You will be instructed to go to the clinic or Quest lab before taking your morning dose of CAH medications (hydrocortisone and fludrocortisone) and have a blood draw between 8:00 and 10:00 AM. (about 3 teaspoons of blood will be drawn). You will also provide a urine sample for urinalysis.

Phone Visit 6 – End of Study (about 30 minutes)
When your lab results are available the study team will contact you to review the results. The doses

you are taking of your CAH medications (hydrocortisone and fludrocortisone) will be reviewed and adjusted if needed. This is the final study visit.

Even though the researchers are not looking at your blood test results to find or treat a new medical problem, you will be told if they notice something unusual. You and your regular doctor can decide together whether to follow up with more tests or treatment.

How long can I expect to be in this study?
You will participate in the study for up to 81 days.

You can choose to stop participating for any reason at any time. However, if you decide to stop participating in the study, we encourage you to tell the researchers. You may be asked if you are willing to come for a final Clinic Visit and complete some study termination tests. You will be instructed to resume the hydrocortisone and fludrocortisone doses you were taking at the beginning of the study.

What are the risks of the study?
Study Procedure/Intervention
There are risks associated with participating in a research study. One risk is that you may receive a treatment that does not improve your disease or may actually worsen your disease. Another risk is the development of side effects.

More than 1900 adult male subjects with prostate cancer have been treated with the study medication (abiraterone acetate) in clinical studies, either alone or combined with other treatments. Currently, the study medication is approved for treatment of advanced prostate cancer that has spread to other parts of the body. At the same time, the study medication is still an investigational drug for other uses. Not all the potential side effects in humans are known. Because abiraterone acetate has not yet been tested in children, at this time the risks in children are not known. In addition to the possible side effects listed below, there is also the possibility that you may experience uncommon or unexpected side effects.

While you participate in this study you will be followed carefully for any side effects that may result from taking the study medication. Side effects may be mild or very serious. In some cases, side effects can be long lasting, or may never go away. Alternatively, some side effects may be temporary and/or may resolve when the study treatment is stopped. It is not possible to tell whether you will experience side effects or if you experience side effects, how mild or severe they might become.

You should tell your study doctor immediately about any side effects, problems or unusual experiences that occur while taking part in this study. Sometimes there are other medications that your doctor can give you to make the side effects better or make you more comfortable and this may decrease the chance that the side effects continue or become worse. If severe side effects do develop, you and your doctor may decide it is in your best interest to stop taking part in the study.

The known risks of this medication come from how it is tolerated in men being treated for prostate cancer. Most of the known risks are the result of how treatment with the study medication affects the adrenal glands. Because you have CAH, you do not have normally functioning adrenal glands and these risks may or may not apply to you. Please discuss with the study doctor symptoms you should report to the study team.

If you have a liver function disorder or heart disease you should not take this medication.

In men without CAH who are being treated for prostate cancer the most common side effects are:

Frequent (Chance of more than 20% that this will happen in men with prostate cancer)

- Low blood potassium (hypokalemia) - Potassium is a mineral that helps regulate heart rate/function, fluid balance in the body and is needed for adequate body function. (Symptoms of hypokalemia may include weakness, constipation, muscle pain or cramps.)
- High blood pressure

Very Common (Chance of 10-20% that this will happen)

- Swelling of the legs as a result of the body keeping too much fluid

Common (Chance of 5-10% that this will happen)

- Uncomfortable feeling in upper belly, indigestion
- Presence of blood in the urine
- Abnormal liver tests that may indicate liver injury/damage
- Urinary tract infection
- Fractures (broken bones)

Less Common (Chance 1-5% that this will happen)

- High blood levels of fat molecules called triglycerides
- Chest pain
- A fast and irregular heartbeat
- Fast heartbeats

Uncommon (Less than <0.1% chance that this will happen)

- Loss of function of adrenal glands
- Heart Failure: Weakness of the heart that leads to a buildup of fluid in the lungs and surrounding body tissues
- Changes in the rhythm of the heart
- An Abnormal finding on the ECG
- Loss of strength of bones
- Muscle weakness and/or muscle pain

Unknown

- Swelling and irritation of the lung
- Acute liver failure
- Breakdown of muscle tissue
- Rapid or irregular heart rate related to feeling faint or lightheaded

While taking this medication it is important that it be taken on an empty stomach because taking it with food increases the amount available in the blood. No food should be consumed for 2 hours prior to taking the drug or for 30 minutes after taking it. You will be provided information about what to bring to the clinic with you for a low-fat breakfast you will be able to eat 30 minutes after taking the drug and you will be instructed to take the medication the same way at home.

Psychological Stress

Some of the questions we will ask you as part of this study may make you feel uncomfortable. You

may refuse to answer any of the questions, take a break or stop your participation in this study at any time.

Loss of Confidentiality
Anytime information is collected; there is a potential risk for loss of confidentiality. Every effort will be made to keep your information confidential; however, this cannot be guaranteed.

Problems obtaining insurance or employment
A Federal Law called the Genetic Information Nondiscrimination Act of 2008 (GINA), generally makes it illegal for group and individual health insurers from using your genetic information to set insurance eligibility, premiums, or contribution amounts. They cannot request or require that you take a genetic test. In addition, employers with 15 or more employees may not use your genetic information to make decisions regarding hiring, firing, job assignments, or promotions, nor can they request, require, or purchase your genetic information. GINA does not protect you against genetic discrimination by companies that sell life, disability, or long-term care insurance.

Risks of Radiation – Diagnostic Test
This research study includes exposure to radiation from diagnostic tests in addition to that which you would receive from standard care. The additional radiation dose you will receive is about 2% of the average radiation dose from all sources (natural background radiation, and radon gas exposure) that a person in the United States receives each year.

Risks of Blood Drawing
Risks associated with drawing blood from your arm include minimal discomfort and/or bruising. Infection, excess bleeding, clotting, and/or fainting are also possible, although unlikely. The risk of drawing blood for the study is not higher than the risk of drawing blood for standard of care and when possible, blood needed for the study will be drawn with standard of care labs to minimize needle sticks.

You will have up to 18 teaspoons of blood drawn for the entire study. This amount is considered safe for children weighing more than 12 kg (26 lbs).

Other Risks
There may possibly be other side effects that are unknown at this time. If you are concerned about other, unknown side effects, please discuss this with the researchers.

If you become sick with a fever or vomiting while on the study, it is important that you contact the study doctor or your regular doctor for sick day care.

The study medication may cause harm to an unborn child. It is important that women, who are pregnant, might become pregnant or are breast feeding, should handle the study drug only with gloves.

The study medication must be kept in a secure, safe place where children cannot gain access to it.

How will risks be minimized or prevented?
Study team members are specially trained to provide safe care during your participation in the study. They will be in close contact with you throughout the study. You will have regular blood testing so that

the investigators will know how your body is handling the study medication, and to make sure that you are on adequate doses of your usual CAH medications (hydrocortisone and fludrocortisone). You will receive physical examinations and electrocardiograms (EKG) at the beginning and end of study drug treatment to ensure that there are no problems from retaining too much fluid in the body or from heart failure. The study doctors are pediatric endocrinologists with expertise in treating children with CAH. We will provide a wallet card including information about the study medication and contact information for the study doctor. This should be presented in any health emergency or when seeking healthcare treatments during the time you are participating in the study.

What will my responsibilities be during the study?

While you are part of this study, the researchers will follow you closely to determine whether there are problems that need medical care. It is your responsibility to do the following:

- Ask questions about anything you do not understand.
- Keep your appointments.
- Follow the researchers' instructions.
- Let the researchers know if your telephone number or address changes.
- Store study materials study medications, hydrocortisone and fludrocortisone in a secure place at home away from anyone who is unable to read and understand labels, especially children.
- The study medications are for you only. Do not share them with anyone.
- Tell the researchers before you take any new medication, even if it is prescribed by another doctor for a different medical problem or something purchased over the counter.
- Tell your regular doctor about your participation in this study.
- Carry information about abiraterone acetate in your purse or wallet.
- Report to the researchers any injury or illnesses while you are on study even if you do not think it is related.

If I agree to take part in this research study, will I be told of any new risks that may be found during the course of the study?

Yes. You will be told if any new information becomes available during the study that could cause you to change your mind about continuing to participate or that is important to your health or safety.

What should I do if I think I am having problems?

If you have unusual symptoms, pain, or any other problems while you are in the study, you should report them to the researchers right away. Telephone numbers where they can be reached are listed on the first page of this consent form.

If you have a sudden, serious problem, like difficulty breathing or severe pain, go to the nearest hospital emergency room, or call 911 (or the correct emergency telephone number in your area). Tell emergency personnel about any medications you are taking, including any medications you are taking for this study.

What are the possible benefits of this study?

If you agree to take part in this study, there will not be direct benefits to you. We hope the information learned from this study will benefit you or others with CAH in the future. Information gained from this research could lead to better treatments.

What options are available if I decide not to take part in this research study?

You do not have to participate in this research to receive care for your medical problem. Instead of

being in this study, you can continue standard of care treatment for CAH. Please talk to the researchers or your personal doctor about these options.

Will I be paid if I take part in this research study?

Yes. You will be given a stipend and a parking voucher for each study clinic visit to help with expenses related to participation in the study, up to a total of $500 if you attend all 6 clinic visits. The stipends will be as follows:

Visit	Stipend
1	$100
2	$50
3	$150
4	$50
5	$100
6	$50

You will also receive lunch vouchers to cover the cost of lunch at visit 3 for the subject and parent(s)/legal guardian(s). If you are enrolled in this study and you live more than 50 miles away from Children's Medical Center, the Sponsor may cover the cost of travel and/or hotel. There are no additional funds available from the research center to pay for lost time away from work and other activities, lost wages, or childcare expenses.

How will I be paid?

You will be issued a UT Southwestern Greenphire ClinCard, which can be used as a credit or debit card. You will also receive instructions on how to use the card. In order to receive study payments, your name, address, date of birth and Social Security Number (SSN) will be collected from you by the research staff. All information will be stored in a secure fashion and will be deleted from the UT Southwestern Greenphire ClinCard system once the study has been completed.

Important Information about Study Payments

1. Your SSN is needed in order to process your payments. Should you decide not to provide your SSN, your study participation payment will be decreased at the current IRS tax rate. Study payments are considered taxable income and are reportable to the IRS.
2. An IRS Form 1099 will be sent to you if your total payments are $600 or more in a calendar year.
3. Your payment information will not be shared with any third parties and will be kept completely confidential

This payment information will remain confidential unless you give your permission to share it with others, or if we are required by law to release it.

UT Southwestern, as a State agency, will not be able to make any payments to you for your participation in this research if the State Comptroller has issued a "hold" on all State payments to you. Such a "hold" could result from your failure to make child support payments or pay student loans, etc. If this happens, UT Southwestern will be able to pay you for your taking part in this research 1) after you have made the outstanding payments and 2) the State Comptroller has issued a release of the "hold."

Will my insurance provider or I be charged for the costs of any part of this research study?

No. Neither you, nor your insurance provider, will be charged for anything done only for this research study (i.e., the Screening Procedures, Experimental Procedures, or Monitoring/Follow-up Procedures

described above).

However, the standard medical care for your condition (care you would have received whether or not you were in this study) is your responsibility (or the responsibility of your insurance provider or governmental program). You will be charged, in the standard manner, for any procedures performed for your standard medical care.

What will happen if I am harmed as a result of taking part in this study?
It is important that you report any illness or injury to the research team listed at the top of this form immediately.

Compensation for an injury resulting from your participation in this research is not available from the University of Texas Southwestern Medical Center at Dallas, or Children's Medical Center.

You retain your legal rights during your participation in this research.

Can I stop taking part in this research study?
Yes. If you decide to participate and later change your mind, you are free to stop taking part in the research study at any time.

If you decide to stop taking part in this research study, it will not affect your relationship with the UT Southwestern staff or doctors. Whether you participate or not will have no effect on your legal rights or the quality of your health care.

Your doctor is a research investigator in this study. S/he is interested in both your medical care and the conduct of this research study. At any time, you may discuss your care with another doctor who is not part of this research study. You do not have to take part in any research study offered by your doctor.

If you decide to stop participating during the study, you should contact the study doctor for instructions about how to transition back to taking your standard of care medications.

If I agree to take part in this research study, can I be removed from the study without my consent?
Yes. The researchers may decide to take you off this study if:

- Your medical problem becomes worse.
- The researchers believe that participation in the research is no longer safe for you.
- The researchers believe that other treatment may be more helpful.
- The sponsor (UT Southwestern Medical Center) or the FDA stops the research for the safety of the participants.
- The sponsor cancels the research.
- You are unable to keep appointments or to follow the researcher's instructions.

Will my information be kept confidential?
Medical information collected during this study and the results of any test or procedure that may affect your medical care may be included in your medical record. The information included in your medical record will be available to health care providers and authorized persons including your insurance company.

You should know that certain organizations that may look at and/or copy your medical records for research, quality assurance, and data analysis include:

- Representatives of government agencies, like the U.S. Food and Drug Administration (FDA), involved in keeping research safe for people; and
- The UT Southwestern Institutional Review Board.
- Sharp Clinical Services
- National Institutes of Health (NIH)

A description of this clinical trial will be available on http://www.ClinicalTrials.gov, as required by U.S. law. This Web site will not include information that can identify you. At most, the Web site will include a summary of the results. You can search this Web site at any time.

In addition to this consent form, you will be asked to sign an "Authorization for Use and Disclosure of Protected Health Information." This authorization will give more details about how your information will be used for this research study, and who may see and/or get copies of your information.

Who will be able to use or share my health information?
Children's Medical Center may use or share your health information with *Perrin C. White, MD* and staff at UT Southwestern Medical Center ("Researchers") for the purpose of this research study.

Will my protected health information be shared with someone other than the Researchers?
Yes, the Researchers may share your health information with others who may be working with the Researchers on the Research Project ("Recipients") for purposes directly related to the conduct of this research study or as required by law. These other people or entities include:

- *UT Southwestern Medical Center and Perrin C. White, MD.* The sponsor includes any people, entities, groups or companies working for or with the sponsor or owned by the sponsor. The sponsor will receive written reports about your participation in the research. The sponsor may look at your health information to assure the quality of the information used in the research.

- *Your treating Primary Care Physician or Endocrinologist*

- *NIH (National Institutes of Health)*

- *Quest Diagnostics Laboratory*

- *University of Michigan: Laboratory of Richard Auchus, MD*

- *The UT Southwestern Institutional Review Board (IRB).* This is a group of people who are responsible for assuring that the rights of participants in research are respected. Members and staff of the IRB at UT Southwestern may review the records of your participation in this research. A representative of the IRB may contact you for information about your experience with this research. If you do not want to answer their questions, you may refuse to do so.

- *Representatives of the Food and Drug Administration (FDA).* The FDA may oversee the Research Project to confirm compliance with laws and regulations. The FDA may photocopy your health information to verify information submitted to the FDA by the Sponsor.

- Representatives of domestic and foreign governmental and regulatory agencies may be granted direct access to your health information for oversight, compliance activities, and determination of approval for new medicines, devices, or procedures.

Medical information collected during this study and the results of any test or procedure that may affect your medical care may be included in your medical record. The information included in your medical record will be available to health care providers and authorized persons including your insurance company.

How will my health information be protected?
Whenever possible your health information will be kept confidential as required by law. Federal privacy laws may not apply to other institutions, companies or agencies collaborating with UT Southwestern on this research project. There is a risk that the Recipients could share your information with others without your permission. UT Southwestern cannot guarantee the confidentiality of your health information after it has been shared with the Recipients.

Why is my personal contact being used?
Your personal contact information is important for the UT Southwestern Medical Center research team to contact you during the study. However, your personal contact information will not be released without your permission.

What health information will be collected, used and shared (disclosed)?
The Researchers will collect information in your medical record about your medical history related to CAH including results of tests and procedures and all treatments including surgeries you have had. Information about your early development and results of tests and observations done for the study and the diaries you keep during the study will be collected.

Will my health information be used in a research report?
Yes, the research team may fill out a research report. (This is sometimes called “a case report”.) The research report will not include your name, address, or telephone or social security number. The research report may include your date of birth, initials, dates you received medical care and a tracking code. The research report will also include information the research team collects for the study.

Will my health information be used for other purposes?
Yes, the Researchers and Recipients may use your health information to create research data that does not identify you. Research data that does not identify you may be used and shared by the Researchers and Recipients in a publication about the results of the Research Project or for other research purposes not related to the Research Project.

Do I have to sign this authorization?
No, this authorization is voluntary. Your health care providers will continue to provide you with health care services even if you choose not to sign this authorization. However, if you choose not to sign this authorization, you cannot take part in this Research Project.

How long will my permission last?
This authorization has no expiration date. You may cancel this authorization at any time. If you decide to cancel this authorization, you will no longer be able to take part in the Research Project. The Researchers may still use and share the health information that they have already collected

before you canceled the authorization. To cancel this authorization, you must make this request in writing to: *Perrin C. White, MD, 5323 Harry Hines, Blvd., Dallas, TX 75390-9063, 214-648-9246*

Are there procedures I should follow after stopping participation in this research? Yes. If you or the researchers, or the sponsor (UT Southwestern Medical Center) stops your participation in the research, you may be asked to do the following:

- Let the researchers know immediately that you wish to withdraw from the research.
- Do not stop taking the study medication unless you have been instructed to because of a health-related emergency or you are following the instructions of the study doctor. You may need an adjustment in your standard of care medication to treat CAH when you stop taking the study medication. It is very important that your physician or the study doctor monitor you while medications are adjusted.
- Return to the research center for tests that may be needed for your safety.
- Return any unused study materials, including empty containers.
- Discuss your future medical care, if any, with the researchers and/or your personal doctor.

Whom do I call if I have questions or problems?
For questions about the study, contact Dr. Perrin White at 214-456-5959 during regular business hours, after hours and on weekends and holidays.

For questions about your rights as a research participant, contact the UT Southwestern Institutional Review Board (IRB) Office at 214-648-3060.

SIGNATURES:
YOU WILL BE GIVEN A COPY OF THIS CONSENT FORM TO KEEP.

If you agree to participate in this research and agree to the use of your protected health information in this research, sign this section. You will be given a copy of this form to keep. You do not waive any of your legal rights by signing this form.

SIGN THIS FORM ONLY IF THE FOLLOWING STATEMENTS ARE TRUE:

- You have read (or been read) the information provided above.
- Your questions have been answered to your satisfaction about the research and about the collection, use and sharing of your protected health information.
- You have freely decided to participate in this research, or you are voluntarily giving your consent for another person to participate in this study because you believe this person would want to take part if able to make the decision and you believe it is in this person's best interest.
- You understand that a copy of this signed consent document, information about this study, and the results of any test or procedure that may affect your medical care, may be included in your medical record. Information in your medical record will be available to health care providers and authorized persons including your insurance company.
- You authorize the collection, use and sharing of your protected health information (another person's protected health information) as described in this form.

By signing this document you are permitting UT Southwestern Medical Center to use and disclose health information about you for research purposes as described above.

_____ _____
Signature of Research Participant Date Time: AM/PM

For Legal Representatives of Research Participants (if applicable):

Printed Name of Legal Representative: _____
Relationship to Research Participant: _____

I certify that I have the legal authority under applicable law to make this Authorization on behalf of the Research Participant identified above. The basis for this legal authority is: _____.
(e.g. parent, legal guardian, person with legal power of attorney, etc.)

_____ _____
Signature of Legal Representative Date Time: AM/PM

Adult Signature Section

Printed Name of Participant	Signature of Participant	Date	Time AM PM
Printed Name of Person Obtaining Consent	Signature of Person Obtaining Consent	Date	Time AM PM

Signature Section (two parent signatures)

Printed Name of Participant	Signature of Participant giving Assent (*If incapable of signing, person obtaining consent should initial here*)	Date	Time AM PM
Printed Name of Parent 1 Giving Consent for Child	Signature of Parent 1 Giving Consent	Date	Time AM PM
Printed Name of Parent 2 Giving Consent for Child	Signature of Parent 2 Giving Consent (***Required unless:*** *deceased, unknown, incompetent, not readily available, or no longer has legal parental rights)*	Date	Time AM PM
Printed Name of Person Obtaining Consent	Signature of Person Obtaining Consent	Date	Time

PARENT CONSENT TO PROVIDE BLOOD TO CONFIRM FORM OF CAH:

Please sign below if you wish to provide a blood sample in order to allow the study team to determine the type of CAH your child has. The samples will be used only for the genetic test explained in this consent. Your test result will be compared with your child's sample to confirm classic CAH or another form of CAH.

Signature Section (two parent signatures)			
			AM PM
Printed Name of Participant	Signature of Participant giving Assent (*If incapable of signing, person obtaining consent should initial here*)	Date	Time AM PM
Printed Name of Parent 1 Giving Consent for Child	Signature of Parent 1 Giving Consent	Date	Time AM PM
Printed Name of Parent 2 Giving Consent for Child	Signature of Parent 2 Giving Consent (***Required unless:*** *deceased, unknown, incompetent, not readily available, or no longer has legal parental rights)*	Date	Time AM PM
Printed Name of Person Obtaining Consent	Signature of Person Obtaining Consent	Date	Time