Emory University
Consent to be a Research Subject

Title: A study to explore the safety and tolerability of Acthar in patients with Amyotrophic Lateral Sclerosis

Principal Investigator: Jaffar Khan, MD

Sponsor: Questcor Pharmaceuticals, INC.

Introduction:
You are being asked to be in a medical research study. This form is designed to tell you everything you need to think about before you decide to consent (agree) to be in the study or not to be in the study. It is entirely your choice. If you decide to take part, you can change your mind later on and withdraw from the research study. The decision to join or not join the research study will not cause you to lose any medical benefits. If you decide not to take part in this study, your doctor will continue to treat you.

Before making your decision:
- Please carefully read this form or have it read to you
- Please listen to the study doctor or study staff explain the study to you
- Please ask questions about anything that is not clear

You can take a copy of this consent form, to keep. Feel free to take your time thinking about whether you would like to participate. You may wish to discuss your decision with family or friends. Do not sign this consent form unless you have had a chance to ask questions and get answers that make sense to you. By signing this form you will not give up any legal rights.

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 may search this Web site at any time.

Study Overview:
You are being asked to take part in a research study of a drug called H.P. Acthar® Gel (Repository Corticotropin Injection) (“Acthar”). Acthar is currently approved by the Food and Drug Administration for the treatment of infantile spasms in infants and children under 2 years of age and for the treatment of exacerbations (worsening or increase in severity of disease) of multiple sclerosis in adults. Acthar may be used for the following disorders and diseases: rheumatic; collagen; dermatologic; allergic states; ophthalmic; respiratory; and conditions marked by swelling. Note: Acthar contains proteins derived from pigs and should not be used by people with sensitivity to these proteins.
The purpose of this research study is to test how safe, tolerable, and effective Acthar is in the treatment of ALS. Acthar has not been approved by the Food and Drug Administration for use in people with ALS; therefore it is considered experimental in this research study. This study will take place over a period of about 15 months.

The study will focus on testing the safety of different doses of Acthar in patients with ALS and will include about 40 subjects at about 20 centers in the United States. The study will be open-label and will include an 8-week treatment period. Open-label means that you know what drug you are taking. All subjects will receive the study drug, Acthar. After the 8-week treatment period, you will have the option to take part in the open-label extension period where you will take Acthar for an additional 28 weeks plus a 3-week treatment taper and 1-week follow-up period. If you agree to continue in the optional open-label extension period, you will continue to take the same dose of Acthar as before.

If you decide to be in this study (including in the optional open-label extension period), your participation will last about 10 months. This will include about 10 office visits and 4 times where you will be contacted by telephone. If you are unable to travel to the clinic, you will need to discuss this with your study doctor. You may have the option to have healthcare services come to your home to perform study procedures for selected study visits. Limited visit procedures will be performed by a qualified home healthcare provider.

You will be randomly assigned to one of four possible study drug groups: Acthar dose of 16 units (0.2 mL/0.04 teaspoon) daily, Acthar dose of 24 units (0.3 mL/0.06 teaspoon) daily, Acthar dose of 56 units (0.7 mL/0.14 teaspoon) twice weekly or Acthar dose of 80 units (1.0 mL/0.20 teaspoon) twice weekly. There will be a maximum of 10 subjects in each dose group. The group that you are placed in is decided by chance. Think of it as drawing numbers out of a hat. There is a 25% chance that you will receive one of the four doses. Towards the end of the study, you will gradually decrease the number of times you take the study drug for a period of 3 weeks before stopping the study drug and will complete end-of-study visit procedures.

During the 8-week treatment period the sponsor will be monitoring all adverse (unpleasant) side effects for each subject. Based on this safety evaluation, a dose group may be closed if it is no longer considered safe and tolerable for study subjects. Your study doctor will inform you if the dose group to which you are assigned is closed. At that time or if you decide to leave the study early, you will gradually decrease the number of times you take the study drug for a period of 3 weeks before stopping the study drug and will complete end-of-study visit procedures. If a dose group is closed, no additional subjects will be enrolled in that group.

**Procedures:**
If you agree to take part in this study, you will first sign this Subject Informed Consent Form before any study-related procedures are performed. During the study, you will undergo some or all of the following procedures at specific scheduled visits.

Your study doctor will also provide you with additional training on the proper technique for injection of the study drug, dosing instructions, and the completion of the study drug diary card that will be provided at the baseline visit. You (or your caregiver) will be expected to inject the study drug under your skin and record this injection in the provided study drug diary card each time you do.

**Visit 1 - Screening**

*Visit duration: about 2 hours*

Before entering the study you will have tests and procedures done to determine if you are able to take part in this study. The screening tests will be done within 4 weeks before you receive your first dose of study drug. Having these tests performed does not guarantee that you will be able to take part in this study. Your participation in this study will depend on the results of your lab tests, study guidelines, and the study doctor’s judgment. The following procedures will be performed:

- Sign the informed consent after all your questions have been answered if you agree to take part in the study
- You will be assigned a unique identification number that will be used to identify you on all study and laboratory documents throughout the study.
- Provide demographic information (including date of birth, gender, race and ethnicity), medical history (your health and any illnesses), and medication history (prescription and non-prescription treatments, including over-the-counter medicine, vitamins, or herbal treatments).
- Complete physical exam (height, weight, lungs, heart, abdomen and extremities), vital signs (body temperature, respiratory rate, heart rate and blood pressure), and ECG (measures the electrical activity of your heart);
- Blood test (22.5 mL or about 1.5 tablespoons) by inserting a needle into a vein and urine sample for safety. It is preferred that you are fasting for at least 8 hours prior to the blood draw. This means you should not eat or drink anything but water for at least 8 hours before the visit;
  - You will be tested for hepatitis B, C, and tuberculosis. If you test positive for any of these tests, you cannot take part in this study. We are required to notify state health authorities of positive results. You will be given a copy of your test results and will be referred to your primary care physician for follow-up. If you do not have a primary care physician, you may be referred to a community health clinic. If you do not want to be tested, you should not take part in this research study.
- For females of childbearing potential (you are still getting your period), a serum (blood) pregnancy test will be completed. The results of the pregnancy test must be negative to continue in the study;
- Review dosing instructions with you to make sure you understand dosing procedures;
• Spirometry will be performed to measure your breathing (the amount of air that can be
exhaled slowly after full inhalation);
• Study staff will assess your physical function.

If the study doctor determines that you can be in the study, you will then return for the Baseline
Visit. For the next 8 weeks, you will self-administer (or your caregiver will administer) your assigned
study drug dose. You will receive instructions and a dosing schedule to tell you when you need to do
this.

Visit 2 - Baseline
Visit duration: about 3 hours

The following procedures will be performed prior to dosing:

• Complete physical exam, vital signs, and ECG;
• Update medical history (as needed);
• Review any medications (prescription and over-the-counter) you are taking;
• Blood test (16.5 mL or about 1 tablespoon) and urine sample for safety lab testing. It is
preferred that you are fasting for at least 8 hours prior to the blood draw. This means you
should not eat or drink anything but water for at least 8 hours before the visit;
• For females of childbearing potential, a urine pregnancy test will be completed. The results of
the pregnancy test must be negative to continue in the study;
• Additional blood samples may be drawn for pharmacokinetic testing (analyzing how your
body breaks down and eliminates the drug);
• Quality-of-life, physical function, behavior and suicide risk will be assessed through ALS
assessment tools;
• Spirometry will be performed to measure your breathing;
• Handheld dynamometry will be performed to measure muscle strength in your hands, arms
and legs;
• Review dosing instructions to make sure you understand proper dosing procedures and the
completion of the study drug diary card.

After the pre-dose procedures are completed, you will be randomly assigned to one of the four study
drug groups: Acthar dose of 16 units (0.2 mL/0.04 teaspoon) daily, Acthar dose of 24 units (0.3
mL/0.06 teaspoon) daily, Acthar dose of 56 units (0.7 mL/0.14 teaspoon) twice weekly or Acthar dose
of 80 units (1.0 mL/0.20 teaspoon) twice weekly.

The following procedures will be performed after you are assigned to your dose group:

• Study drug will be provided. You or your caregiver will administer the first dose while in the
clinic to demonstrate proper injection technique under the supervision of the study
personnel;
• Review adverse (unpleasant) side effects;
• Study drug diary card will be provided and you will receive instructions about how to
complete the diary card; and
• Blood samples may be drawn for pharmacokinetic testing (analyzing how your body breaks down and eliminates the drug).

You will remain at the clinic for at least one hour after the study drug is injected. You will be monitored for signs of an allergic reaction or adverse (unpleasant) side effects.

**Visit 3, Week 2 – Treatment Period**

*Visit duration: about 1 hour*

You will be asked to return to the clinic and the following procedures will be performed:

- Limited physical exam and vital signs (weight, lungs, heart, abdomen and extremities. Edema (swelling) will also be evaluated);
- Review any medications (prescription and over-the-counter) you are taking;
- Blood test (10.5 mL or approx. 0.7 tablespoons) and urine sample for safety laboratory testing. It is preferred that you are fasting for at least 8 hours prior to the blood draw. This means you should not eat or drink anything but water for at least 8 hours before the visit;
- Assess for suicide risk;
- Review adverse (unpleasant) side effects; and
- Review study drug diary card.

**Visit 4, Week 4 – Treatment Period**

*Visit duration: about 1.5 hours*

You will be asked to return to the clinic and the following procedures will be performed:

- Limited physical exam, vital signs, and ECG;
- Review any medications (prescription and over-the-counter) you are taking;
- Blood test (16.5 mL or approx. 1.1 tablespoons) and urine sample for safety lab testing. It is preferred that you are fasting for at least 8 hours prior to the blood draw. This means you should not eat or drink anything but water for at least 8 hours before the visit;
- Additional blood samples may be drawn for pharmacokinetic testing (to study how much of the drug is in your body);
- Quality-of-life, physical function and suicide risk will be assessed;
- Spirometry will be performed to measure your breathing;
- Handheld dynamometry will be performed to measure muscle strength;
- Review adverse (unpleasant) side effects;
- Study staff will review the study drug diary card and will return it to you for completion before the next visit;
- You will be asked to bring all of your study drug vials to the visit and the study doctor and/or study staff will review for accountability; and
- Study drug will be provided.

**Week 6 – Telephone Contact - Treatment Period**

*Visit duration: about 0.25 hours*
The study staff will contact you via telephone to perform the following:

- Assess for safety, including symptoms of hyperglycemia (high blood sugar), hypertension (high blood pressure), infection, and edema (swelling);
- Review any medications (prescription and over-the-counter) you are taking; and
- Review adverse (unpleasant) side effects.

Based on the telephone contact, the study staff may ask that you return to the clinic or schedule a home visit for additional evaluations.

**Visit 5, Week 8 - Treatment Period**

*Visit duration: about 1.5 hours*

You will be asked to return to the clinic and the following procedures will be performed:

- Limited physical exam and vital signs;
- Review any medications (prescription and over-the-counter) you are taking;
- Blood test (16.5 mL or approx. 1.1 tablespoons) and urine sample for safety lab testing. It is preferred that you are fasting for at least 8 hours prior to the blood draw. This means you should not eat or drink anything but water for at least 8 hours before the visit;
- Additional blood samples may be drawn for pharmacokinetic testing;
- Quality-of-life, physical function and suicide risk will be assessed;
- Spirometry will be performed to measure your breathing;
- Handheld dynamometry will be performed to measure muscle strength;
- Review adverse (unpleasant) side effects;
- Study staff will review the study drug diary card and will return it to you for completion before the next visit;
- You will be asked to bring all of your study drug vials to the visit and the study doctor and/or study staff will review for accountability; and
- Study drug will be provided.

**Treatment Taper and Follow-up Period/Begin Optional Open-Label Extension Period**

After Visit 5, Week 8, you will have the choice to either continue receiving the study drug for an additional 28 weeks or discontinue any further study drug dosing. If you decide to discontinue dosing, you will gradually decrease the number of times you (or your caregiver will) administer the study drug over the next three weeks. This period is called the treatment taper. You will receive dosing instructions from the study doctor and/or study staff before you begin tapering off the study drug. If you choose to be in the optional open-label extension, you will be given an addendum to this consent form to sign. You will continue to take your study drug at the same dose as previously directed by the study doctor in the same manner.

**Visit 6, Week 12 – Study Completion/Optional Open-Label Extension**
Visit duration: about 1.5 hours

You will be asked to return to the clinic and the following procedures will be performed:

- Complete physical exam, vital signs, and ECG;
- Review any medications (prescription and over-the-counter) you are taking;
- Blood test (12.5 mL or approx. 0.85 tablespoons) and urine sample for safety lab testing. It is preferred that you are fasting for at least 8 hours prior to the blood draw. This means you should not eat or drink anything but water for at least 8 hours before the visit;
- For females of childbearing potential, a urine pregnancy test will be completed. The results of the pregnancy test must be negative if you choose to continue in the Optional Open-Label Extension;
- Quality-of-life, physical function and suicide risk will be assessed;
- Spirometry will be performed to measure your breathing;
- Handheld dynamometry will be performed to measure muscle strength;
- Review adverse (unpleasant) side effects;
- Review study drug diary card;
- You will be asked to bring all of your study drug vials to the visit and the study doctor and/or study staff will review for accountability;
- Study drug and a study drug diary card will be provided if you choose to continue in the Optional Open-Label Extension.
- If you decide not to participate in the optional open-label extension, all study drug vials and the study drug diary card will be collected by the study doctor and/or study staff. This will be your last visit in the study.

The following describes visits in the Optional Open-Label Extension Period

Week 16 – Telephone Contact – Optional Open-Label Extension Period
Visit duration: about 0.25 hours

The study staff will contact you via telephone to perform the following:

- Assess for safety, including symptoms of hyperglycemia (high blood sugar), hypertension (high blood pressure), infection, and edema (swelling);
- Review any medications (prescription and over-the-counter) you are taking; and
- Review adverse (unpleasant) side effects.

Based on the telephone contact, the study staff may ask that you return to the clinic or schedule a home visit for additional evaluations.

Visit 7, Week 20 – Optional Open-Label Extension Period
Visit duration: about 1.5 hours

You will be asked to return to the clinic and the following procedures will be performed:
- Limited physical exam and vital signs;
- Review any medications (prescription and over-the-counter) you are taking;
- Blood test (12.5 mL or approx. 0.85 tablespoons) and urine sample for safety lab testing. It is preferred that you are fasting for at least 8 hours prior to the blood draw. This means you should not eat or drink anything but water for at least 8 hours before the visit;
- Quality-of-life, physical function and suicide risk will be assessed;
- Spirometry will be performed to measure your breathing;
- Handheld dynamometry will be performed to measure muscle strength;
- Review adverse (unpleasant) side effects;
- Study staff will review the study drug diary card and will return it to you for completion before the next visit;
- You will be asked to bring all of your study drug vials to the visit and the study doctor and/or study staff will review for accountability; and
- Study drug will be provided.

**Week 24 – Telephone Contact – Optional Open-Label Extension Period**

*Visit duration: about 0.25 hours*

The study staff will contact you via telephone to perform the following:

- Assess for safety, including symptoms of hyperglycemia (high blood sugar), hypertension (high blood pressure), infection, and edema (swelling);
- Review any medications (prescription and over-the-counter) you are taking; and
- Review adverse (unpleasant) side effects.

Based on the telephone contact, the study staff may ask that you return to the clinic or schedule a home visit for additional evaluations.

**Visit 8, Week 28 – Optional Open-Label Extension Period**

*Visit duration: approximately 1.5 hours*

You will be asked to return to the clinic and the following procedures will be performed:

- Limited physical exam, vital signs, and ECG;
- Review any medications (prescription and over-the-counter) you are taking;
- Blood test (12.5 mL or approx. 0.85 tablespoons) and urine sample for safety laboratory testing. It is preferred that you are fasting for at least 8 hours prior to the blood draw. This means you should not eat or drink anything but water for at least 8 hours before the visit;
- For females of childbearing potential, a urine pregnancy test will be completed. The results of the pregnancy test must be negative if you choose to continue your participation in the Optional Open-Label Extension;
- Quality-of-life, physical function and suicide risk will be assessed;
- Spirometry will be performed to measure your breathing;
- Handheld dynamometry will be performed to measure muscle strength;
- Review adverse (unpleasant) side effects;
- Study staff will review the study drug diary card and will return it to you for completion before the next visit;
- You will be asked to bring all of your study drug vials to the visit and the study doctor and/or study staff will review for accountability; and
- Study drug will be provided.

**Week 32 – Telephone Contact – Optional Open-Label Extension Period**

*Visit duration: about 0.25 hours*

The study staff will contact you via telephone to perform the following:

- Assess for safety, including symptoms of hyperglycemia (high blood sugar), hypertension (high blood pressure), infection, and edema (swelling);
- Review any medications (prescription and over-the-counter) you are taking; and
- Review adverse (unpleasant) side effects.

Based on the telephone contact, the study staff may ask that you return to the clinic or schedule a home visit for additional evaluations.

**Visit 9, Week 36 – Optional Open-Label Extension Period**

*Visit duration: approximately 1.5 hours*

You will be asked to return to the clinic and the following procedures will be performed:

- Limited physical exam and vital signs;
- Review any medications (prescription and over-the-counter) you are taking;
- Blood test (12.5 mL or approx. 0.85 tablespoons) and urine sample for safety laboratory testing. It is preferred that you are fasting for at least 8 hours prior to the blood draw. This means you should not eat or drink anything but water for at least 8 hours before the visit;
- Quality-of-life, physical function and suicide risk will be assessed;
- Spirometry will be performed to measure your breathing;
- Handheld dynamometry will be performed to measure muscle strength;
- Review adverse (unpleasant) side effects;
- Study staff will review the study drug diary card and will return it to you for completion before the next visit;
- You will be asked to bring all of your study drug vials to the visit and the study doctor and/or study staff will review for accountability;
- Study drug will be provided; and
- Study staff will provide instructions to begin the treatment taper over the next few weeks.

**Treatment Taper and Follow-up Period**
After Visit 9, Week 36, or if you discontinue study drug dosing early, you will gradually decrease the number of times you (or your caregiver will) administer the study drug over the next three weeks. You will receive dosing instructions from the study doctor and/or study staff before you begin.

**End of Study - Visit 10, Week 40**

*Visit duration: about 1.5 hours*

You will be asked to return to the clinic and the following procedures will be performed:

- Complete physical exam, vital signs, and ECG;
- Review any medications (prescription and over-the-counter) you are taking;
- Blood test (12.5 mL or approx. 0.85 tablespoons) and urine sample for safety lab testing. It is preferred that you are fasting for at least 8 hours prior to the blood draw. This means you should not eat or drink anything but water for at least 8 hours before the visit;
- For females of childbearing potential, a urine pregnancy test will be completed;
- Quality-of-life, physical function and suicide risk will be assessed;
- Spirometry will be performed to measure your breathing;
- Handheld dynamometry will be performed to measure muscle strength;
- Review adverse (unpleasant) side effects;
- Review study drug diary card;
- You will be asked to bring all of your study drug vials to the visit and the study doctor and/or study staff will review for accountability; and
- All study drug vials and the study drug diary card will be collected by the study doctor and/or study staff at the visit.

**Blood Volumes**

Some of the blood drawn during the study will be tested immediately. Some blood will be stored for future testing by the sponsor.

**Stored Blood Samples**

As part of the study, the sponsor will store some of the blood samples collected from you for future testing. Any future research that uses your blood samples will be focused on tests to conduct new medical research and develop proposals for new medical products or therapies in the future. The samples and any results from future testing will become the property of the sponsor. However, you will always have the right to request that your samples be destroyed at any time by writing to the study doctor. Should you withdraw consent or authorization at any time; all results collected prior to your withdrawal will remain the property of the sponsor. The samples will not be used for genetic research nor will they be made to last indefinitely (through cloning). The sponsor may store the samples for any amount of time and they may be destroyed without notifying you. There are no direct benefits to you from the collection and storage of these samples. Samples will never be sold for financial gain.
Subject Responsibilities

While taking part in this research study, you will need to:

- Keep all scheduled appointments.
- You must tell your study doctor if you have had any vaccinations within 30 days prior to receiving study drug.
- Notify your study doctor about any medications you are taking and if you plan to have surgery or any other medical treatment or procedure.
- Keep a daily diary to record when you take the study drug.
- Take the study drug as directed at about the same time each day.

NOTE: How often you take the study drug will depend on which group you are assigned to.

- Store the study drug in the refrigerator at all times throughout the study.
- Notify your study doctor if you are planning to stop taking the study drug as some side effects may occur if you stop taking the drug too quickly.
- Notify your study doctor or study staff if you are experiencing any side effects, even if you don’t think that they are important.
- For women of childbearing potential, you need to use a medically acceptable method of birth control as determined by your study doctor.
- Continue to take your other ALS medications unless changed by your study doctor.

If, for any reason, the study drug is stopped or you decide to discontinue your participation in the study, you will be asked to complete the treatment taper period. You will also be asked to return to the clinic to complete the study procedures required at either Visit 6 (if you withdraw from the study before Visit 5) or Visit 10 (if you withdraw from the study before Visit 9).

Study Drug Handling

The study drug (Acthar) is supplied in vials to be used for multiple doses. Please keep it out of the reach of children and store it in the refrigerator. Always allow the study drug to warm to room temperature before using it.

Risks and Discomforts:

All drugs can cause side effects in some people. You must tell the study doctor or study staff about any side effects you experience. If you do not tell the study doctor and study staff about the side effects, you may harm yourself by being in this study. Do not stop taking the study drug without first talking to your study doctor even if you are experiencing side effects.

Your condition may not improve and could even worsen if you take part in this study.

Possible Acthar risks

Acthar causes your body to make more steroids which are hormones that help your body to respond to stress. The known adverse (unpleasant) effects of Acthar are related to this increase in steroids.
Not all of these side effects have occurred with Acthar but they may occur based on known effects of other steroid drugs.

Risks and side effects related to the study drug include those which are:

**Likely:**
- Elevated blood pressure, salt and water retention
- Increase in blood sugar and decrease in blood potassium may occur. Acthar may worsen pre-existing diabetes (may increase blood sugar).
- Changes in mood and behavior such as irritability, depression, or trouble sleeping. If you note a significant change in level of anxiety, depression or mood swings, be sure to contact your study doctor.
- Increase appetite and weight gain. Acthar can stimulate the appetite and increase water retention. It is advisable to follow a low-salt and/or potassium rich diet and watch your caloric intake. Your study doctor will make specific dietary recommendations for you.

**Less Likely:**
- Increased risk of infections. Importantly, you will be screened for tuberculosis (TB) and hepatitis and if you test positive you will not be permitted to take part in this study. Acthar can lower your resistance to infection and make any infection that you get more difficult to treat. Contact your study researcher if you notice any signs of infection, such as sore throat, fever, coughing, or sneezing.
- Cushing’s syndrome (a rare condition caused when too much corticosteroid hormones are stored in the body) may occur as a result of too much steroids in the body. It causes an increase in upper body fat, rounded face and thinning of the skin. This is more common in patients who take Acthar for a long time and usually goes away when Acthar is stopped.
- There are potential risks with receiving vaccinations while using Acthar. You cannot receive live or live attenuated vaccines, such as FluMist® for influenza or chicken pox/shingles vaccines. You must tell your study researcher if you have had any vaccinations within 30 days prior to receiving study drug.
- Worsening of eye disorders such as cataracts or glaucoma.
- Decrease in the density of your bones which causes bones to weaken, causing fractures.
- Allergic reactions (for example, skin rash, swelling of the face, tongue, lips, and/or throat, and trouble breathing).
- Long-term use of Acthar may cause an increase in the size of the heart, but this condition usually goes away after Acthar is stopped.

**Rare, but Serious**
If you have certain medical conditions, Acthar may worsen pre-existing conditions, such as the following:

- Myasthenia gravis (muscle weakness; most commonly in facial muscles)
Acthar may mask symptoms of other diseases/disorders without altering the course of the other disease/disorder.

**Risk of abruptly stopping Acthar**

Treatment with Acthar must not be stopped suddenly. Suddenly stopping Acthar can result in adrenal insufficiency, which means your adrenal glands have stopped making hormones. Adrenal insufficiency can cause low blood sugar, low blood pressure and weaken the body’s ability to respond to stress. Acthar treatment should be decreased gradually over a period of weeks and then stopped. In this study, this is accomplished during the Study Drug Taper Period. Additional treatment with steroids may be necessary while Acthar dosage is gradually reduced.

**Blood draws/needle stick risks**

Risks associated with drawing blood from your arm include pain, bruising, bleeding at the site of the needle puncture, lightheadedness and, on rare occasions, infection.

**Electrocardiogram (ECG):**

The ECG test is a recording of the electrical activity of your heart. It is a painless procedure. The sticky pads placed on your skin may sometimes cause some discomfort such as redness or itching. If the skin under the patches needs to be shaved, irritation from shaving could also occur.

**Unforeseen risks**

When taking any new medication, some risks may be unforeseeable. It is important to tell your study doctor if you experience any side effects.

In addition to the risks or discomforts listed here, there may be other risks that are currently not known. All drugs have potential risk of an allergic reaction, which, if not treated promptly, could become life-threatening. Also, the risks or discomforts described may occur more often or be more severe than has been seen before.

Your safety will be closely monitored throughout the study.

The study drug must be taken only by the person for whom it has been prescribed. It also must be kept out of the reach of children or persons of limited capacity to read or understand.

**Reproductive Risks**

*If you are a woman:* to protect against possible side effects of the study drug, women who are pregnant or nursing a child may not take part in this study. If you become pregnant, there may be risks to you, the embryo, or fetus. These risks are not yet known. If you are a woman of childbearing ability, you and the study doctor must agree on a method of birth control to use throughout the study.
and for at least 30 days after the last dose of study drug. If you think that you have gotten pregnant during the study, you must tell the study doctor immediately. Pregnant women will be taken out of the study.

If you become pregnant during the study or within 30 days of discontinuing study drug, the study doctor will report the pregnancy to the sponsor or its representative.

You will be followed by the study doctor until completion of the pregnancy. At the completion of the pregnancy, the study doctor will document the outcome of your pregnancy.

Keep the study drug out of the reach of children or anyone else who may not be able to read or understand the label. Do not let anyone else take the study drug besides you.

**New Information**

It is possible that the researchers will learn something new during the study about the risks of being in it. If this happens, they will tell you about it. Then you can decide if you want to continue to be in this study or not. You may be asked to sign a new consent form that includes the new information if you decide to stay in the study.

**Benefits**

This study is not designed to benefit you directly. Your participation in the study may contribute to information about the study drug and may benefit other patients in the future.

**Compensation**

You will be offered a $50.00 gift card at each visit to help defray costs for travel and parking at the clinic.

**Other Treatment Outside this Study**

If you decide not to enter this study, there is care available to you outside of this research. You may receive your ongoing care at the Emory ALS Clinic. There may be other studies that you are eligible for. The study doctor will discuss these with you. You do not have to be in this study to be treated for ALS.

**Confidentiality**

Certain offices and people other than the researchers may look at your medical charts and study records. Government agencies and Emory employees overseeing proper study conduct may look at your study records. These include the Food and Drug Administration, the Office for Human Research Protections, Emory offices that are part of the Human Research Protection Program, and those that are involved in administration and billing. Questcor Pharmaceuticals, Inc. (study sponsor) and people or companies they use to carry out the study may also look at your study records. Emory will keep any research records we create private to the extent we are required to do so by law. A study number and your initials rather than your name will be used on study records wherever
possible. Your name and other facts that might point to you will not appear when we present this study or publish its results.

Study records can be opened by court order. They may also be produced in response to a subpoena or a request for production of documents.

Research Information Will Go Into the Medical Record:
If you are or have been an Emory Healthcare patient, you have an Emory Healthcare medical record. If you are not and have never been an Emory Healthcare patient, you do not have one. Please note that an Emory Healthcare medical record will be created if you have any services or procedures done by an Emory provider or facility for this study. Copies of the consent form and HIPAA patient form that you sign will be placed in your Emory Healthcare medical record.

Emory Healthcare may create study information about you that can help Emory Healthcare take care of you. For example, the results of study tests or procedures. These study results will be placed in your Emory Healthcare medical record. Anyone who has access to your medical record will be able to have access to all the study information placed there. The confidentiality of the study information in your medical record will be protected by laws like the HIPAA Privacy Rule. On the other hand, some state and federal laws and rules may not protect the research information from disclosure.

Emory does not control results from tests and procedures done at other places, so these results would not be placed in your Emory Healthcare medical record. They will not likely be available to Emory Healthcare to help take care of you. Emory also does not have control over any other medical records that you may have with other healthcare providers. Emory will not send any test or procedure results from the study to these providers. If you decide to be in this study, it is up to you to let them know.

In Case of Injury
If you get ill or injured from being in the study, Emory would help you get medical treatment. You should let the study doctor know right away that you are ill or injured. If you believe you have become ill or injured from this research, you should contact Dr. Khan at 404-778-3807. You should also let any health care provider who treats you know that you are in a research study.

If you get ill or injured as the direct result of being in this study, then, depending on what insurance you may have, the sponsor may pay for some or all of the costs for your medical treatment of the illness or injury if it:

a. is not a medical condition that you had before you started the study;
b. is not the result of the natural progress of your disease or condition;
c. is not caused by your failure to follow the study plan; and
d. is not proved to be directly caused by the negligence of an Emory employee. “Negligence” is the failure to follow a standard duty of care.

If your case meets all four of these requirements and you are uninsured or have Medicare or Medicaid, then the sponsor will pay all of the costs of your medical treatment for the illness or injury. If your case meets all four of these requirements and you have private insurance, Emory will look at the claims for these costs to see if they can be sent to your insurer for payment. Your insurer may be told that you are in a research study and given information about your treatment.
You will have to pay for any costs that the sponsor or your insurer does not pay. The sponsor will pay for any of the costs that are not paid by your insurance provider. The sponsor will not pay for costs like co-payments that your insurer says you have to pay.

Emory has not set aside any money to pay you or to pay for your treatment if you get ill or injured from being in the study. The only exception to this policy is if it is proved that your injury or illness is directly caused by the negligence of Emory employee.

**Costs**

You do not have to pay for study drug, study visits conducted in the clinic or at your home, and tests that have to be done for the study. You will be responsible for the cost of any treatments you are taking to manage your disease, such as riluzole, muscle relaxants or pain relievers.

**Withdrawal from the Study**

Your study doctor, the sponsor company, or the FDA has the right to stop your participation in the study at any time, with or without your consent, for any of the following reasons: if you have an adverse effect from the study drugs, if you need a treatment not allowed in this study, if you do not keep appointments, if you do not take the study drug as instructed, if you become pregnant, or if the study is canceled by the FDA or the sponsor company. The sponsor may decide to stop the study and your access to the study under certain circumstances even if the study drug appears to be safe and effective.

You may withdraw from the research study at any time. Your decision will not affect any benefits to which you are entitled. For your safety, your study doctor will ask you to complete end-of-study visit procedures.

If you decide to withdraw before the end of the study, there may be risks associated with this decision. You should discuss potential risks of withdrawal with your study doctor.

If you withdraw or are removed from the study, for any reason, you must return all study drug given to you. This includes empty vials and any unused study drug.

The researchers and sponsor also have the right to stop your participation in this study without your consent if:

- They believe it is in your best interest;
- You were to object to any future changes that may be made in the study plan; or
- For any other reason.

**Contact Information**

Contact Principal Investigator, Dr. Jaffar Khan at 404-778-3807:

- if you have any questions about this study or your part in it,
- if you feel you have had a research-related injury or a bad reaction to the study drug, or
- if you have questions, concerns or complaints about the research
Contact the Emory Institutional Review Board at 404-712-0720 or 877-503-9797 or irb@emory.edu:

- if you have questions about your rights as a research participant.
- if you have questions, concerns or complaints about the research.
- You may also let the IRB know about your experience as a research participant through our Research Participant Survey at http://www.surveymonkey.com/s/6ZDMW75.