The Effect of Lipoic Acid Natural Supplement on Cystine Stone Formation

Cystinuria is an orphan disease for which there are no effective treatments and causes severe, debilitating, recurrent kidney stones. In this clinical trial we aim to perform a randomized double-blind, placebo-controlled clinical trial with lipoic acid, to treat patients with cystinuria and evaluate its effect on their blood, urine, and stones. Lipoic acid is a naturally occurring compound that is safe in humans and has demonstrated remarkable effects in a mouse model for cystinuria. When the supplement was given to mice that had cystinuria, almost all of the mice stopped forming new stones. If the effect of the supplement in cystinuric mice is also observed in humans, even if somewhat blunted, it has the potential to dramatically change the way cystinuria is managed, a disease for which no new therapeutics have been developed in over 30 years. Specifically, the supplement may reduce the burden of medications and the number of stone events with related hospitalizations and procedures, as well as helping preserve long-term kidney function. However, this is the first study in humans, and the results in mice may not be found in humans.

This clinical trial is funded through an FDA and NIH collaborative effort. The purpose of this study is to find out what effects, good and/or bad, a natural supplement has on you and your urine cystine level. You will be given either the natural supplement or inactive substance depending on a random assignment, to take once daily by mouth. The supplement may reduce the risk that stones may return or grow. You will be "randomized" into one of the study groups described below. Randomization means that you are put into a group by chance. A computer program will place you in one of the groups. Neither you nor your doctor can choose the group you will be in. You will have an equal chance of being placed in any group. During your clinic visits, you will see the doctor like you would normally. You will have kidney imaging, urine and blood testing and collection of passed kidney stones as is normal for standard practice. We will collect leftover blood and urine samples for further analysis outside of your routine care, and no additional blood draw or urine collection will be needed. You will be in this study for a total of 5 years, of which the first 3 years will include the study
supplementation. The purpose of the last 2 years of monitoring will be to observe for any long-term benefits or risks to the supplement. All study procedures will take place at UCSF, Parnassus Campus.

16 visits will be required over the course of five years; one visit every four months. The costs of all routine visits, treatments, and tests will be billed to you or your insurance carrier. The study will only pay for the CT scan at the Visit 10 (at 3 years) and the supplement or inert substitute you are given to take. Insurance companies and other carriers sometimes refuse to pay the costs of treatment when individuals are participating in research. If this happens in your case, you will be billed for the care your insurance will not cover. Financial counselors are available through the hospital accounting department to discuss this with you.

If you have any questions or would like to get further information, please contact:
Victoria Hogue
Stoller and Chi Laboratories
Research Data Analyst/Clinical Research Coordinator
Victoria.Hogue@ucsf.edu [1]