

Infant Study of Inhaled Saline in Cystic Fibrosis (ISIS002)***Study # NA_00017484******Peter J. Mogayzel, Jr., M.D., Ph.D. Principal Investigator*****Study Background:**

People with cystic fibrosis (CF) often have thick mucus in the airways of the lungs that is hard to cough up. The mucus builds up and eventually leads to chronic cough and lung infections. Research has shown that even young children with CF may have thickened mucus in the lungs.

Inhaling a concentrated salt solution, called hypertonic saline (HS), may help to thin the mucus in the lungs. Thinning the mucus can make it easier to cough up. This helps to clear the lungs and improve lung health. Research studies about the safety and effectiveness of inhaled HS have been done in adults and children with CF 6 years of age and older. Patients in these studies took HS for up to a year. HS appears to be a safe treatment in these age groups. The main side effects were cough, throat irritation, and wheezing. The use of HS improved lung function and quality of life. It also decreased the need for antibiotics for acute respiratory infections. HS is now routinely used by many CF patients over 6 years of age.

HS appears to be safe in infants with CF based on several studies, but its effectiveness has never been studied in infants. Because HS treats a very early step in the chain of events that leads to chronic lung problems in people with CF, it may be particularly helpful when started early in life.

Study Purpose:

This research study will evaluate the safety and effectiveness of HS compared to a salt solution of much lower concentration, isotonic saline (IS). HS or IS will be given twice a day for a year to infants who are between 4 and 16 months of age at enrollment. This is the first study in infants with CF of a maintenance therapy that has been shown to be safe and effective in older children and adults with CF. The information from this study will help CF doctors learn more using HS in infants.

Who Can Participate?

About 150 children with CF will take part in this research study in 18 CF clinics across North America. To take part, your child must have CF, be at least 4 months of age but not older than 16 months, and be safe to undergo infant lung function testing. In addition, you, as the child's parent/guardian, must be willing to administer the nebulized treatments twice daily at home for 48 weeks.

What is Involved?

Taking part in this study is voluntary and will not affect your child's care in the CF clinic. If you agree to allow your child to take part in this study:

FACT SHEET

- ❑ Your child will be randomized to receive either HS or IS. This means that your child will be assigned to receive HS or IS by chance (like the flip of a coin). You and your doctors won't know which treatment your child is receiving.
- ❑ You will be asked to administer treatments to your child twice each day using a nebulizer that will be provided for you.
- ❑ Your child will be in this study for up to 52 weeks. There will be 7 study visits in addition to your child's regular clinic visits. Whenever possible, the visits will be scheduled as part of your child's CF Clinic visits.
- ❑ At 3 of the study visits, your child will have an Infant Pulmonary Function Test that will require him/her to be sedated. The results of these tests will be provided to you and your doctors.
- ❑ Information regarding your child's medicines, height and weight, respiratory culture results, and lung function test results will be collected during the study.
- ❑ Between study visits, you will receive a phone call from a clinical research coordinator.
- ❑ You will be asked to fill out a brief questionnaire about your child's symptoms about every 6 weeks during the study.

For more information about this study, please contact:

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