

# Azithromycin in patients with CF who are culture negative for *Pseudomonas aeruginosa* (AZ 0004 (Randomized))

## Summary

This study evaluated the effect of azithromycin in children with CF who are not infected with *Pseudomonas aeruginosa*. Earlier studies of azithromycin in people with CF who had chronic *P. aeruginosa* had resulted in the drug being recommended as a chronic treatment for these individuals. This study was conducted to find out whether azithromycin could provide benefits to CF patients who are not infected with *P. aeruginosa*. Participants in this study were randomized to receive either azithromycin or placebo three times a week for 24 weeks.

## Specimen Information

### Status: Specimens are Available

Specimens were collected at baseline and at 4 weeks and 24 weeks after treatment with 3 times weekly azithromycin.

| Visit # | Time From Baseline | Specimens Collected    |
|---------|--------------------|------------------------|
| 1       | +0 Days            | Lith-Hep Plasma, Serum |
| 3       | +4 Weeks           | Lith-Hep Plasma, Serum |
| 5       | +24 Weeks          | Lith-Hep Plasma, Serum |



## Study Design

|                         |                |
|-------------------------|----------------|
| Study Type?             | Interventional |
| Randomized Study?       | Yes            |
| Placebo Controlled?     | Yes            |
| Length of Participation | 24 Weeks       |
| Number of Study Visits? | 5              |



## Additional Information

|                |              |
|----------------|--------------|
| Phase?         | Phase Three  |
| Study Sponsor? | Rose, Lynn   |
| Study Drugs?   | Azithromycin |

## Eligibility

|                         |  |
|-------------------------|--|
| <b>Age</b>              | 6 Years to 18 Years  |
| <b>Mutation(s)</b>      | No Mutation Requirement  |
| <b>FEV1% Predicated</b> | 50 % or greater  |
| <b>PA Status</b>        | Negative   |
| <b>Other</b>            | Eligible subjects must be negative for P aeruginosa for one year prior to screening (documented by at least 2 negative cultures) |

## Study Results

### WHAT WE LEARNED:

Study results show that while there was no improvement in lung function in the treatment group compared to the placebo group; treatment with azithromycin did improve other measures including the rate of pulmonary exacerbations. Treatment with azithromycin did not increase the risk of adverse events.

### PRIMARY FINDINGS:

#### EFFECTIVENESS:

This study was conducted between February 2007 and July 2009. A total of 260 patients were randomized to receive either azithromycin (N=131) or placebo (N= 129); the vast majority of participants completed the study (126 in each treatment group). The researchers found that treatment with azithromycin for 24 weeks, compared with placebo, did not result in improved pulmonary function, as measured by the change in FEV1 (the volume of air that can be forced out in one second after taking a deep breath).

#### SAFETY:

Participants in the azithromycin group had no increased risk of adverse events, but had less cough and less productive cough compared with placebo participants.

### CITATION:

JAMA 2010;303(17):1707-1715, Chest 2012

For more information about the results of this study and where it was conducted, visit [ClinicalTrials.gov](http://ClinicalTrials.gov).