Help advance CF Research

Gateway to Weight Gain Study

Kalydeco (Ivacaftor) treatment of patients with cystic fibrosis and gating mutations resulted in clinically significant improvements in weight and body mass index, in addition to improved pulmonary function.

We are conducting a study to determine the mechanism(s) for weight gain in patients for whom Kalydeco treatment is initiated for clinical purposes. This study will assess in detail change in energy balance, weight, muscle, fat, and lung function in subjects ≥6 years old with any of the gating mutations before treatment and after three months. All subjects will be seen at the Children's Hospital of Philadelphia Clinical Translational Research Center.

Inclusion Criteria

- Cystic fibrosis with one or two gating mutations
  (G551D, G178R, S549N, S549R, G551S, G1244E, S1251N, S1255P, G1349D)
- Age: 6 years and older
- Kalydeco treatment planned for clinical care
- In usual state of good health

Exclusion Criteria

- FEV_1 < 40% predicted
- Non-CF related illness affecting nutritional status or growth
- Pregnancy or lactation

Study procedures will require two, 2-3 day visits and consist of: interviews, blood draws, assessment of weight, height, total and resting energy expenditure, stool and urine collection, dietary intake, stool losses, body composition, muscle strength, and pulmonary function tests.

All travel to Philadelphia, housing and study procedures are paid by the study. Subjects are also compensated for their time and effort.

If you have a potentially eligible subject please contact us to learn more:

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Help lead the way.