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Project Title: Studies of Growth Deficiency and Growth Hormone Treatment in Children with Osteogenesis Imperfecta Types III and IV

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Statistical Analysis Plan:

For each patient, they were seen at the NIH CC for a full year prior to treatment.

Baseline growth rate was set for each patient at NIH CC: At 1 yr prior and 6 months prior to treatment, as well as at t=0, patients length was measured on a stadiometer board by genetics and endocrine nurses trained to take accurate repetitive measurements. 10 measurements were taken and averaged for each date. The growth in cc from 1 year prior to treatment to treatment start in cm/yr was the baseline growth rate for that patient.

Treatment growth rate: Measurements taken on each patient at treatment time=0, 6 and 12 months were taken in the same way and a treatment year (cm/yr) growth rate was calculated.

Response to treatment: For each patient, their rate of growth during treatment was compared to the baseline growth rate. A positive response was scored if treatment growth rate was 50% or more increased over baseline rate. The percent of patients in the full treatment group with a positive linear growth rate response was scored as the % responders.