BONUS: Baby Observational and Nutrition Study (BONUS-IP-11, BONUS10K0)

Summary

This observational study was conducted to gain a better understanding of the factors that interfere with good growth in infants with CF. Researchers measured both weight gain and linear growth in addition to other measurements. Participants were enrolled soon after diagnosis of CF (by the time they were 3 months old) and were followed until they were 12 months old.

Specimen Information

Status: Specimens are Available

Babies are enrolled within 3.5 months of birth. Blood samples will be collected at enrollment (1 to 3.5 months of age), and then at 6 months and 12 months of age. Urine and stool will be collected 1 month after enrollment (unless the baby is already 3 months old at enrollment). A buccal swab for DNA will be collected at enrollment. Throughout the study, any "extra" OP swabs will also be saved and banked at Colorado Children's Hospital.

Visit #	Time From Baseline	Specimens Collected
1	+1 Months	Buccal DNA, Lith-Hep Plasma
2	+2 Months	Stool, Urine
3	+3 Months	Stool, Urine
4	+4 Months	Stool, Urine
5	+5 Months	Stool, Urine
6	+6 Months	Lith-Hep Plasma, Stool, Urine
8	+8 Months	Stool, Urine
9	+10 Months	Stool, Urine
10	+12 Months	Lith-Hep Plasma, Stool, Urine

Study Type?	Observational
Randomized Study?	No
Placebo Controlled?	No
Length of Participation	12 Months
Number of Study Visits?	9

Additional Information

Phase?	Phase Two
Study Sponsor?	Ramsey, Bonnie
Study Drugs?	N/A

Eligibility

Age	Less than 15 Weeks
Mutation(s)	No Mutation Requirement
FEV1% Predicated	No FEV1 Limit
PA Status	Not Applicable
Other	Babies with CF will be identified via newborn screening and enrolled before 3.5 months of age.

Study Results

WHAT WE LEARNED:

This study collected data on newborn infants with CF diagnosed by newborn screening. The study showed that, in general, infants with CF continue to have modestly lower body weight than healthy infants early in life but achieve normal body weight by 12 months of age. However, infants with CF have modestly lower body length than healthy infants and this difference persists to at least 12 months of age.

PRIMARY FINDINGS:

EFFECTIVENESS:

This study was conducted between December 2012 and May 2014. The study enrolled 231 infants and 16 withdrew from the study. The primary reason for withdrawing was due to a family decision. A total of 93% of the participants enrolled completed follow-up through 12 months. At birth and during the first 6 months of age, CF infants' weight lagged behind the average weight infants, as defined by the World Health Organization (WHO). However, by 12 months, CF infants saw an increase in weight and caught up with healthy infants. Additionally, researchers observed that infants caught up in weight by one year regardless of feeding type (formula vs. breast feeding). Statistical analysis showed that length for age in CF infants was significantly below average. CF infants in the BONUS study had shorter lengths than the WHO average values for healthy infants at 3, 6, and 12 months of age ($p<0.001$). Weight and length from this cohort of CF infants were significantly improved ($p<0.0001$) when compared to a historical 1994-1995 CFF Patient Registry cohort, and were higher but not significantly different than a 2004-2005 CFF Patient Registry cohort. Originally the study had planned to include a sub-study to evaluate an infant formulation of pancreatic enzymes; this sub-study did not enroll and there are no results to report.

SAFETY:

As this was an observational study, no safety measures were applicable.

CITATION:

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For more information about the results of this study and where it was conducted, visit ClinicalTrials.gov.