Growth Parameters in Infants With Cystic Fibrosis: A Comparative Registry Analysis

Michael S. Schechter,1 Suzanne Michel,2 Jerry Hall,3 Shufang Liu,*3 Rupal Khurmi,3 Mark Haupt3

1Children's Hospital of Richmond at Virginia Commonwealth University, Richmond, VA; 2Medical University of South Carolina, Charleston, SC; 3AbbVie Inc., North Chicago, IL

*With AbbVie at the time of the study

Presented at the SGNA (2016) Society of Gastroenterology Nurses and Associates - 43rd Annual Course • Seattle, WA • May 20 – 24, 2016

INTRODUCTION

• Early nutritional status is strongly associated with long-term pulmonary function, anthropometric measures, and survival in patients with cystic fibrosis (CF)1
• Recovery of birth weight Z-score within 2 years of diagnosis is associated with higher lung function and better chest radiographic scores at 6 years of age2
• Pancreatic insufficiency occurs in ~85% of patients with CF and can lead to fat malabsorption, malnutrition, and growth failure3
• Infants with pancreatic insufficiency should be given pancreatic enzyme replacement therapy (PERT), which may promote normal growth and weight gain but may not completely correct nutrient malabsorption4

OBJECTIVE

• To investigate anthropometric data in infants with CF who are or are not prescribed PERT using data from the CF Foundation patient registry

METHODS

• Retrospective analysis of CF Foundation patient registry data for infants born in 2010–2013
• Patient selection criteria are shown in Figure 1
• Patients were categorized by whether or not they were prescribed PERT by 3 months of age, a secondary sensitivity analysis categorized patients by initiation of PERT by 12 months of age
• Anthropometric measures were compared at birth, initial CF Center encounter, and final follow-up (12 months)
• Comparisons were made between groups using Wilcoxon, chi-square, and Kruskal-Wallis tests

RESULTS

Table 1. Patient Demographics and Clinical Characteristics

<table>
<thead>
<tr>
<th>Characteristic</th>
<th>No PERT (n=100)</th>
<th>PERT (n=750)</th>
<th>Total (n=850)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Male, n (%)</td>
<td>46 (46.0)</td>
<td>384 (51.3)</td>
<td>430 (50.6)</td>
</tr>
<tr>
<td>White, n (%)</td>
<td>89 (89.0)</td>
<td>798 (94.1)</td>
<td>887 (98.9)</td>
</tr>
<tr>
<td>Birthweight percentile, median (Q1, Q3)</td>
<td>41.9 (27.2, 62.4)</td>
<td>27.5 (10.7, 50.1)**</td>
<td>27.6 (11.8, 50.1)***</td>
</tr>
<tr>
<td>Initial weight percentile, median (Q1, Q3)</td>
<td>46.6 (22.5, 67.9)</td>
<td>15.4 (11.2, 39.4)**</td>
<td>18.3 (6.0, 39.9)***</td>
</tr>
<tr>
<td>Initial birth length percentile, median (Q1, Q3)</td>
<td>42.4 (26.6, 64.7)</td>
<td>21.6 (6.4, 43.5)**</td>
<td>27.5 (10.7, 50.1)**</td>
</tr>
<tr>
<td>No PERT (n=50)</td>
<td>0</td>
<td>49 (6.5)***</td>
<td>49 (6.5)***</td>
</tr>
<tr>
<td>No PERT (n=100)</td>
<td>0</td>
<td>7 (7.0)</td>
<td>7 (7.0)</td>
</tr>
<tr>
<td>G-tube, n (%)</td>
<td>276 (36.8)***</td>
<td>324 (38.1)</td>
<td>350 (41.2)**</td>
</tr>
<tr>
<td>Pseudomonas infection, n (%)</td>
<td>15.4 (5.1, 34.2)**</td>
<td>32.7 (41.9)</td>
<td>21.6 (6.4, 43.5)**</td>
</tr>
</tbody>
</table>

PATIENTS

• The PERT group included 750 patients, and the no-PERT group included 100 patients
• Median initial encounter age was 1 month; median age at final follow-up was 12 months
• Patients initiating PERT by 3 months of age had significantly smaller birthweight percentiles, and 1-month weight, length, and weight-for-length (W/L) percentiles, compared with the no-PERT group (Table 1)

CHANGES

• Median birth weight percentiles (Figure 2A) and birth length percentiles (Figure 2B) were significantly lower in PERT vs no-PERT patients
• Initial encounter median weight, length, and weight-for-length percentiles were significantly lower in PERT patients (Figure 2A–2C)
• Despite larger increases in median weight and length percentiles in the PERT group, significantly lower weight and length percentiles persisted at 12 months (Figure 2A and 2B)
• Median weight-for-length percentiles increased more in PERT patients and were similar to the no-PERT group at 12 months (Figure 2C)
• Similar results were noted when categorizing patients by PERT administration within 12 months (n=857) and by first CF Foundation encounter within 12 months for patients not on PERT (n=124; data not shown)

CONCLUSIONS AND DISCUSSION

• Infants with CF who receive PERT during the first year of life have lower anthropometric measures at birth and at the initial CF encounter compared with infants with CF who do not receive PERT
• By age 1 year, both PERT and no-PERT patients have similar, acceptable median weight-for-length percentiles; however, PERT infants have smaller height and weight percentiles
• Even no-PERT patients have a median weight below the 50th percentile
• Indication bias may contribute to these results, as initial presenting signs and symptoms may influence the decision to start PERT
• Further analysis is planned to understand growth patterns and the role of PERT dose in infants with CF

REFERENCES


ACKNOWLEDGMENTS

The authors would like to thank the Cystic Fibrosis Foundation for the use of CF Foundation Patient Registry data to conduct this study. Additionally, we would like to thank the patient care providers, and clinic coordinators at CF Centers throughout the United States for their contributions to the CF Foundation Patient Registry.

DISCLOSURES

The authors and AbbVie scientists designed the study and analyzed and interpreted the data. All authors contributed to the development of the content, all authors and AbbVie reviewed and approved the presentation; and the authors maintained control over the final content. Medical writing support was provided by Katherine Grossewitz, PhD, of Complete Publication Solutions, LLC, Horsham, PA. AbbVie funded the research and medical writing support. M Schechter receives research support from the Centers for Disease Control and Prevention, Novartis, and the Cystic Fibrosis Foundation and is a consultant for Genentech, Glide, Novartis, and Vertex. S Michel is a consultant to AbbVie’s marketing team regarding educational materials and a technical advisor to MVV Nutritionals, maker of a CF-specific vitamin product. J Hall, R Khurmi, and M Haupt are employees of AbbVie and may own AbbVie stocks and/or options. S Liu was an employee of AbbVie at the time of the study and may own AbbVie stock and/or stock options.