TURNING ANECDOTES INTO EVIDENCE
TREND Health Initiatives connect patients and caregivers with leading specialists to collectively evaluate the effectiveness of specific therapies, diet, or other environmental interventions.
Exploring nutrition therapy as intervention for Prader-Willi Syndrome

Beth Zupec-Kania, consultant nutritionist for the Charlie Foundation followed and advised group of children with PWS in order to explore the effectiveness and benefits of the ketogenic and related diets.
- Physician approval required
- Baseline labs required
- Private discussion group for peer support
- Weekly webinars with nutritionist
- Starting ratios based on participant’s current reported diet
- Glucose & ketones monitored throughout
- Data collected on TREND
## PWS Diet Initiative

### Diet Initiative I

<table>
<thead>
<tr>
<th># Applications</th>
<th># Enrolled</th>
<th># Transitioned MKD</th>
<th># Completed 6 months</th>
</tr>
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<tbody>
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<td>57</td>
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## PWS Diet Initiative

### Diet Initiative II

<table>
<thead>
<tr>
<th># Applications</th>
<th># Enrolled</th>
<th># Transitioned SF/WF</th>
<th># Transitioned MKD</th>
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<tbody>
<tr>
<td>33</td>
<td>14</td>
<td>14</td>
<td>13</td>
</tr>
</tbody>
</table>
PWS Diet Initiative

Reported Benefits

- Hunger/Satiety
- Cognition/Development
- Behavior
- Energy
- Body Composition
- Quality of Life
Reported Negative Experiences

- Sleep disturbances
- GI issues
- Low energy
- Food prep difficult
Issues Noted on Baseline Labs

- Depressed ferritin levels
- Elevated lipids
- Depressed Free Carnitine levels
- Depressed CO2 levels
- Depressed Vitamin D-25-OH
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- Depressed ferritin levels
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- Depressed Vitamin D- 25-OH
For more information visit:
Capturing patient experience with Pitolisant

TREND Community partnered with The Chion Foundation to facilitate requests to FDA for personal importation of a drug approved in Europe with a superior safety profile to Provigil and specifically active against cataplexy. TREND is capturing the patient experiences using Pitolisant to share with stakeholders.
Update

- 4 children have received Pitolisant through personal importation
- 3 remain on drug and 1 has stopped
Pitolisant Initiative

Reported Benefits

- Wakefulness during the day
- Sleeping better and longer
- Increased processing speed
- Improved attention
For more information visit:
chionfoundation.org
Exploring smartwatch technology for children with special needs

20 families in the PWS and Williams Syndrome communities will beta-test an icon-based smartwatch by Joy called Octopus to explore the potential of this wearable technology to help children with special needs overcome the challenge of managing daily routines.
Sleep Initiative

Exploring sleep and wakefulness in rare disease

We will be collecting sleep studies and conducting surveys to explore sleep and wakefulness in PWS and other rare diseases. The data will be shared with Bioprojet (developed Pitolisant) and sleep scientists.
The Friedreich’s Ataxia Research Alliance (FARA) must provide FDA with patient perspective data at their Patient-focused Drug Development Meeting in June, 2017.

TREND Community partnered with FARA to perform an analysis of the community’s Facebook group data to provide community’s perspectives on the questions posed by FDA.
Want to Join?

interested@trend.community