How to Discuss Your Options, Including Clinical Trials, with your Care Team: Oley Webinar

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Disclosures

- **Site Principal Investigator/ Research Support for Clinical Trials:**
  - Teduglutide ® - Takeda Pharmaceuticals
  - Apraglutide ® - VectivBio
  - Thrive Trial – Protara Therapeutics
Goals of Discussion

- Intestinal Failure, Adaptation & Rehabilitation
- Multidisciplinary Approach
- Individualized Plan of Care
- Clinical Trials
Intestinal Failure
Intestinal Failure (IF)

- Inability of the small intestine to maintain adequate nutrient, fluid, and electrolyte absorption

- A requirement for continuous parenteral nutrition for at least 60 to 90 days.

Etiology of Pediatric IF

Prevalence: 2.5 per 10,000 live births

n = 272 Pediatric Intestinal Failure patients from 14 pediatric medical centers

- NEC
- Gastrochisis
- Intestinal Atresia
- Volvulus
- Long Segment Hirschsprung
- CIPO
- Tufting or MVID
- Other

Squires R et al. 2012
Wales P et al. 2017
Etiology of Adult IF

Indications

Adults

- Retransplant: 7%
- Other: 9%
- Tumor: 14%
- Motility Disorder: 12%
- Short Gut: 64%
- Trauma: 7%
- Volvulus: 7%
- Ischemia: 23%
- Crohn's: 11%
- Other Short Gut: 10%

Intestinal Transplant Registry Report © 2015

- Intestinal Rehabilitation and Transplant Association
INTESTINAL ADAPTATION

- Enteral autonomy from PN
- Starts after resection & can continue for years

Slow but steady wins the race.
INTESTINAL ADAPTATION

Compensates for loss of intestinal surface area & is characterized by functional & morphological changes

- Increase in villous height and crypt depth
- Elongation and dilation of remnant bowel
- ↑ Enterocyte proliferation & ↓ apoptosis

The Journey

Intestinal Failure

Parenteral Nutrition Support

Enteral Feeding Regimen

Medical Therapy Glutamine GH/GLP

TPN Alterations (Lipids)

Enteral Autonomy

Autologous Reconstructive Surgery

Intestinal Transplantation

Fistula Management
Stricture/adhesion Management
Ostomy Management
Lengthening Procedure (S.T.E.P)
Goals for Intestinal Rehabilitation

- Enhance absorption of enteral calories
- Reduce parenteral nutrition (PN)
- Minimize PN related complications
  - CLABSIs, IFALD, vascular thromboses
- Improve disease management and quality of life

Tappenden, K. JPEN 2014: 38: 23S-31S.
Intestinal Rehabilitation Team

Patient

Nursing

Registered Dietician

Stomal Therapy Nurses, Mental health experts, Pharmacists

GI

Surgery

Social Work
### Multidisciplinary Approach and Progressive Improvement in IF Outcomes

<table>
<thead>
<tr>
<th>Site</th>
<th>Date</th>
<th>n</th>
<th>Survival</th>
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<tbody>
<tr>
<td>Reports in English literature</td>
<td>Prior to 1972</td>
<td>50</td>
<td>54%</td>
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<tr>
<td>Los Angeles, CA</td>
<td>1977-1984</td>
<td>13</td>
<td>69%</td>
</tr>
<tr>
<td>Boston, MA</td>
<td>1986-1998</td>
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<td>70%</td>
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<tr>
<td>Ann Arbor, MI</td>
<td>1997-2003</td>
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<td>73%</td>
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<tr>
<td>PIFCon (14 U.S. Centers)</td>
<td>2000-2004</td>
<td>272</td>
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<tr>
<td>Seattle, WA</td>
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<td>62</td>
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<tr>
<td>Boston, MA</td>
<td>2002-2014</td>
<td>313</td>
<td>94%</td>
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Clinical Trials
What is a Clinical Trial?

- Research studies performed in people that are often aimed at evaluating an intervention.
- Often aimed to find out if a new treatment is safe and effective (relative to standard treatment)
- Clinical trials may test ways to find a disease early, before there are symptoms.
- Others test ways to prevent a health-related problem.
- A clinical trial may also look at how to make life better for people living with diseases or chronic health problems.
Phases of Clinical Trials

- Clinical trials advance through four phases to test a treatment, find the appropriate dosage & look for potential side effects.

- A new medication or intervention often may be tested first in animals or in the laboratory. If the results are favorable → the FDA may authorize the study to be conducted in humans.

- If, after the first three phases of study, researchers find the intervention to be safe and effective, the FDA can approve it for clinical use and continue to monitor its effects.
Why Consider a Clinical Trial?

- Prior treatments did not work.
- Participants may find out about new treatments before they are widely available.
- Trials are a way to play a more active role in one’s own health care.
- Help researchers learn more about certain health problem/ help future generation.
How to Find a Clinical Trial?

- Ask you healthcare providers

New from NIH MedlinePlus Magazine

Find clinical studies on the updated ClinicalTrials.gov

05/02/2022 02:10 PM EDT

ClinicalTrials.gov beta site is now available. Interested in learning about clinical trials?

Want to find research that’s happening on a specific health topic? On ClinicalTrials.gov you can do all that, and more! It's an online database from the National Library of Medicine that has current, up-to-date information about over 400,000 research studies from all over the world. ClinicalTrials.gov is free to use and doesn’t collect any personal information. Anyone can access it online from

Read the full article
What is the next step after finding a clinical trial?

- Contact the clinical trial study coordinator
- Set up a screening appointment to see if you qualify to participate
  - Inclusion/Exclusion Criteria
- Let your health-care team know that you are considering joining a clinical trial
  - Your team may want to talk to the research team about your health to make sure the study is safe for you and to coordinate your care while you are in the study
What happens in a clinical trial?

- Study staff explain the trial in detail and gather more information about you.
- You are screened to determine if you qualify for the trial.
- Once you have had all your questions answered and agree to participate, you sign an informed consent.
- Schedule a first “baseline” visit.
- You may be randomized to treatment or control group.
- You may need follow-up visits at the research site at regularly scheduled times.
  - At these visits, the research team may collect information about the efficacy of the intervention and your safety and well-being.
Questions to ask before starting a clinical trial

- What is this study trying to find out?
- What treatment or tests will I have? Will I have access to the test or lab results?
- What are the chances I will get the experimental treatment or the placebo?
- What are the possible risks, side effects, and benefits of the study treatment compared with my current treatment?
- How will I know if the treatment is working?
- How long will the clinical trial last?
Additional questions to ask before starting a clinical trial

- Where will the study take place?
- Will I have to stay in the hospital?
- Will I be provided with a way to get to the study site if I need it (rideshare service etc...)?
- How often will I need to be seen at the clinical trial site?
- Can any of my visits be done virtually?
- Is there a closer site or clinical trial to me?
- How will the study affect my everyday life?
- What steps ensure my privacy?
Thank you to our amazing patients & their families and to The Oley Foundation!