



NPC Newborn Screening Clinical Roundtable

May 23, 2019

Bethesda, Maryland



HOW BENEFICIAL IS EARLY INTERVENTION FOR PATIENTS WITH NPC?

- A. Very beneficial
- B. Somewhat beneficial
- C. Not at all beneficial
- D. Undecided



HOW IMPORTANT IS MODALITY WHEN DETERMINING WHEN TREATMENT IS STARTED?

- A. Very important
- B. Somewhat important
- C. Not important
- D. Undecided



WHEN SHOULD TREATMENT DIRECTED AT VISCERAL SYMPTOMS BE INITIATED?

- A. Before symptoms are evident
- B. Only if evidence of disease, although disease may not be clinically compromising
- C. Only when disease becomes clinically compromising



WHEN SHOULD TREATMENT THAT IS DIRECTED AT CNS SYMPTOMS BE INITIATED?

- A. Before symptoms are evident
- B. Only when developmental delay is identified
- C. Only when neurological symptoms or regression appear



**IS THERE AN IMPORTANT QUESTION WE ARE NOT
ASKING?**

