



**Niemann-Pick Type C Clinical Roundtable**  
***Gaining Consensus Regarding Initiation of Treatment for Patients Positively Identified with NPC***  
***Following Newborn Screening***

**Roundtable Discussion Topics**

Below is a listing of a set of discussion topics Dr. Berry-Kravis pose in the afternoon session.

1. Do we have evidence that early treatment is beneficial? If not, what do we need in order to provide this evidence? How will we get it?
2. When should treatment be initiated?
  - As soon as there is a confirmed diagnosis (newborn period in the case of NBS)?
  - As soon as we see any developmental symptoms?
  - As soon as we see any neurological abnormalities?
  - When we see slowing of developmental progress?
  - When we see regression?
3. Do we need to treat before any symptoms appear?
4. Do we view developmental delay with ongoing progress as being NPC-affected?
5. If developmental rate is normal initially, how much change in rate should take place before initiating treatment? Do we need to replicate a change with several repeat tests?
6. What are the best ways to monitor developmental change and neurological function?
7. Are there different ways to monitor younger and older children for neurological symptoms? If so, what are they?