

Discussion and questions

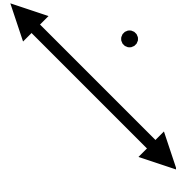
- Ethical issues of placebo controlled studies in type 1 SMA
- Use of palliative care in studies in type I SMA

Discussion and questions

- **Ethical issues of placebo controlled studies in type 1 SMA**
- Use of palliative care in studies in type I SMA

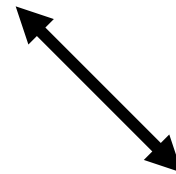
Placebo

- Provides natural history in the context of a trial
 - Limitations of natural history studies
- When is a single arm study appropriate?



Drug under investigation

- Convergence of multiple studies
- Recruitment challenges



Effective drug

- Impact on the field
 - Standard of care
 - Clinical Trial design

Discussion and questions

- Ethical issues of placebo controlled studies in type 1 SMA
- **Use of palliative care in studies in type I SMA**

SOC and Clinical Trials

Ethical quandary



- Allow parental decisions about palliative care
- Yet support the participant for sufficient time to respond to drug

Original Article

Spinal Muscular Atrophy Type I: Is It Ethical to Standardize Supportive Care Intervention in Clinical Trials?

Journal of Child Neurology
1-6

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Decision of parents in the context of a trial

Agreement of physicians

Palliative Care Reactive Measures Proactive measures

To implement:

– Respiratory measures

- Non-invasive ventilation
- Invasive ventilation
- Tracheostomy

– Nutritional measures

- Nasogastric tube
- Gastrostomy tube