**BACKGROUND**

**Rare Disease Clinical Trial Challenges**
- Few eligible subjects
- Wide geographic dispersal of prospective subjects
- Few centers and investigators with disease expertise and research experience
- Limited funding and resources to support rare-disease trial
- No pre-existing guidelines for establishment of sites new to research

**TRIAL DESIGN**
- Juvenile Batten disease is a rare, fatal inherited pediatric neurodegenerative disorder.
- Randomized, double-blind, crossover, 22-week clinical trial to assess safety and tolerability of mycophenolate mofetil.

**Challenges:**
- Minimize repeat study visits
- Minimize travel burden
- Manage costs

**Solutions:**
- Combine single-center and multi-center infrastructure
- Central site: University of Rochester (UR) - screening, consent, drug dispensing, and major safety and tolerability measurements
- Local site: local physician is sub-investigator for safety visits

**DISCUSSION**
- The Clinical Research Coordinator (CRC) plays a key role in the clinical trial process.
- CRC conducts many site startup and study procedures
- Local sites, without CRCs, have prolonged site initiation times (Fig. 1).
- The novel enrollment process (Fig. 2) facilitates
- Most timely site start-up possible
- Compliance with all federal, ICH, OHRP regulations
- Essential clinical research training for new investigators.

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