

NASEM Workshop
Exploring Novel Clinical Trial Designs for Gene-Based Therapies
November 13, 2019

Patient and Family Perspectives

Ron Bartek
Co-Founder/President
Friedreich's Ataxia Research Alliance (FARA)

Patient & Family Concerns

- Full disclosure – In our disease group, no experience, yet, in gene therapy but on the doorstep so keen interest. About half dozen companies and three universities advancing GT programs in FA and grappling with trial-design issues.
- Some of same issues identified in two-day conference at NIH August 2018 and subject of an NCATS/Cures Acceleration Network Review Board program aimed at developing advanced technology solutions to be made available to all GT programs.
- Those issues of most serious concern to patients and their families, I believe, are:
 - Safety, of course, but willing to take on considerable risk
 - Because most likely to be one-shot therapy:
 - How to be sure first dose is potentially therapeutic – new paradigm vs. dose escalation.
 - Opportunities for additional dosing (e.g., different vector or different delivery vehicle, different route of administration, immune response suppression)?
 - Inclusiveness beginning early – new paradigm vs. demonstrate efficacy in adults; include older, later-stage patients AND younger, earlier-stage patients in early trials, especially considering treatment window. Pre-symptomatic patients?
 - Exclusion due to prior exposure to vector? Managed via immune suppression?

Patient & Family Concerns, cont'd

- Alternatives to placebo arm?
- How therapeutic will gene therapy be? Managing patient expectations.
- How long will it take to get to my disease? Current pace way too slow. Manufacturing is biggest impediment.
- Could NCATS and the FDA help significantly accelerate progress in gene-therapy product development, across the board, by leading a workshop on development of standardized clinical trial designs that would simultaneously address many of these issues for a large number of disease groups?
- Will gene therapy be accessible? Will pricing provide for sustainable access, sustainable innovation, sustainable reimbursement and a sustainable healthcare economy?