Patient Group Assets Across the R&D Continuum

- Translational tools (assays, cell & animal models, bio-samples, biomarkers, etc.)
- Natural history database, pt interviews & KOLs = trial design incl. relevant endpoints, power calculations, selection of subjects, sites, procedures, consent forms
- FDA guidance; benefit-risk eval.
- Accompany sponsor to pre-IND

- Well educated, motivated pts help retention
- Well designed protocols reduce amendments
- Help support pt costs
- Serve on DSMBs
- Assist in any sponsor consideration of adapting trial
- Accompany to after-p2/3 mtgs

- Communications support
- Provide feedback from pt. community re results
- Website/newsletter/blog, social media articles
- Co-present results
- Work w/payers on reimbursement
- Assist w/ post-market surveillance initiatives

Pre-Discovery → Pre-Clinical → Phase 1 → Phase 2/3 → FDA review & approval → PAS/ Outcomes

- Fund basic science
- Characterize disease: mech. of damage & action
- Partner with NIH
- Provide data on unmet need and therapeutic burden
- Educate/motivate pt community

- Clinical Infrastructure incl. Network of sites, clinicians, staff that know pts & disease
- Pt Registry for rapid recruitment
- Help support pt costs
- Serve on DSMB

- Accompany to after-p2/3 mtgs
- Serve on FDA advisory committees
- Provide testimony at FDA hearings