### Patient Group Assets Across the R&D Continuum

<table>
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<th>Phase</th>
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| **Pre-Discovery** | • Fund basic science  
• Characterize disease: mech. of damage & action  
• Partner with NIH  
• Provide data on unmet need and therapeutic burden  
• Educate/motivate pt community | | | | |  |
| **Pre-Clinical** | | | | | | |
| **Phase 1** | | | | | | |
| **Phase 2/3** | | | | | | |
| **FDA review & approval** | | | | | | |
| **PAS/Outcomes** | | | | | | |

#### Pre-Discovery
- **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

#### Pre-Clinical
- 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

#### Phase 1
- Clinical Infrastructure incl. Network of sites, clinicians, staff that know pts & disease
- Pt Registry for rapid recruitment
- Help support pt costs
- Serve on DSMB

#### Phase 2/3
- 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

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