CLINICAL TRIALS TRANSFORMATION INITIATIVE

Tool 1. Patient Group Organizational Expertise and Assets Evaluation Tool

The highlighted cells indicate the phase(s) of the clinical trial continuum where the activity is most likely to occur.

	Discovery	Preclinical	Phase 1-3	Regulatory Review	Postapproval
Input regarding interest of research question to patient community					
Providing data on unmet need & therapeutic burden					
Fundraising and direct funding for research to identify target molecules					
Facilitating collaboration with NIH					
Characterizing the disease & relevant mechanisms of action					
Helping define study's eligibility criteria					
Providing translational tools (assays, cell & animal models, biosamples, biomarkers, etc.)					
Natural history database & patient registry support					
Input on meaningful clinical endpoints/patient-reported outcomes					
Assistance re informed consent form					
Working with FDA re benefit-risk and draft guidance					
Accompanying sponsor to Pre-IND FDA meeting to advocate for study					
Fundraising and direct funding for research, trial operations support					
Assistance in selecting & recruiting optimum clinical sites					
Clinical infrastructure support					
Helping educate/motivate patient community & recruit for trials					
Providing patient feedback on participant experience					
Serving on Data and Safety Monitoring Board					
Input for any trial adaptations or modifications					
Accompanying sponsor to milestone meetings (e.g., after phase 2 & 3)					
Providing public testimony at the FDA Advisory Committee & other FDA hearings					
Preparing submission for new born screening when appropriate					
Serving on postmarket surveillance initiatives					
Helping return study results to participants					
Co-presenting results					
Publications/communications regarding results					
Feedback on how patient community views results					
Working with payers regarding reimbursement					

To learn more about CTTI's Patient Groups & Clinical Trials work, please visit https://ctti-clinicaltrials.org/our-work/patient-engagement/patients-groups-clinical-trials/