

WHAT WE'VE BUILT...

to make treatment research possible ::::

Patient Data

Natural history study, clinical data, patient reported priorities & outcomes, Rare-X registry data

Human Cell Models

Patient-derived WAC iPSC lines • neuronal models • multiple WAC variants

Animal Models

WAC disease models for studying biology and testing potential interventions in mice and zebrafish

Research Network

DESSH investigators, clinicians, industry collaborators, patient community ready to support studies