

What can patients do? A lot!

- Research process is long and unpredictable
 - Delays and resetting of timelines is very common (expected)
 - Setbacks = knowledge, not failure
- Many things can happen in parallel
 - Small investments at critical junctures can have big pay-offs
- Patients have special knowledge of their disease
 - Registries, natural history studies
 - Data quality and interoperability are important
 - Educate and bring together the community
- Scientific meetings are not just for scientists
 - Meet the researchers
 - Family “tracks” within meetings
 - Participate in research agenda setting process
- Share your stories – they matter and people will listen
- Rare Diseases Are Not Rare
 - 30 million people in the US with a rare disease, 350 million worldwide
 - Join with other groups – there is power in numbers

