2022 LBSL International Patient Conference
"Information is Power"

Moser Center for Leukodystrophies
at Kennedy Krieger Institute
A Certified Leukodystrophy Care Network (LCN) Center of Excellence
Roadmap in LBSL – an ultra-rare disorder

- **Project Planning**
  - Establish model systems in the lab
  - Testing Therapeutics in model systems (anti-oxidants, small molecules, ASO, gene therapy)
  - Natural History Study in USA
  - Outcome Measure Studies in USA, Netherlands, Finland
  - Tox studies
  - FDA
  - First In Human Trials

- 9/2014 First meeting with McGinn Family
- 2015
- 2016
- 2017
- 2018
- 2019
- 2020
- 2021
- 2022
- 2023
- 2024
- 2025
- 2026
- 2027
WHY OBJECTIVITY MATTERS

These capsules are fabulous! When I look at the box, I stop coughing.
What is a clinical trial?

• Clinical studies are studies that involve research using human volunteers to add to medical knowledge

• Two kinds of clinical studies:
  • Observational study
  • Clinical Trial – assess the effect of an intervention