

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

FDA



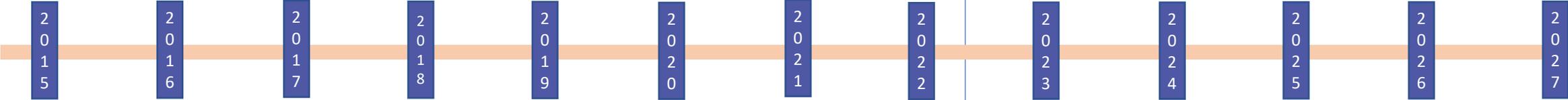
9/2014 First meeting with McGinn Family



Tox studies

FDA

First In Human Trials



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 involves research using human volunteers to add to medical knowledge
- Two kinds of clinical studies:
 - Observational study
 - Clinical Trial – assess the effect of an intervention

