What are we trying to achieve with basic research?
The road from bench to bedside

- Basic Research: How does nature work? What causes disease?
- Translational Research: How can biological knowledge be used to treat disease?
- Clinical Research: Are these treatment strategies safe and effective in humans?
Biological systems (specifically the nervous system) are highly complex and nonlinear

How we would like life to be

How life really is
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**
- **First In Human Trials**

- **9/2014 First meeting with McGinn Family**

- **2015-2027**
  - 2015: First meeting with McGinn Family
  - 2016: Establish model systems in the lab
  - 2017: Testing Therapeutics in model systems
  - 2018: Natural History Study in USA
  - 2019: Outcome Measure Studies in USA, Netherlands, Finland
  - 2020-2027: First In Human Trials