BRINGING PRECISION MEDICINE TO THE BRAIN: UNLEASHING BIG DATA TO ANSWER ALS

At the Leading Edge
Never underestimate the power of observation

“The islands possess their own species … these species having the same general habits, occupying analogous situations, and obviously filling the same place in the natural economy of this archipelago, that strikes me with wonder.”

—Charles Darwin, journal written on the *Beagle*, 1831–1835
Darwin’s finches
“To make perfect the most difficult of all the arts, the art of observation ---
To call to aid the science of experimentation ---
To cultivate the reasoning faculty, so as to know true from false --
These are our methods”

William Osler, 1892
Medical advances are approaching the asymptote

- Human factors
- System factors
The Opportunities
Triple, Simultaneous Revolutions

- Connectivity
- Measurement
- Information/Analysis
All patients with the same diagnosis are not the same—Subgroups need distinct approaches to management
Types of filters in medicine

• Timing
• Phenotype
• Measurement
  – Immunological
  – Genomic – Genes and their expressed products
  – Biochemical
• Perturbation and observation
Human clinicians....

**Strengths**
- Empathy and caring
- Expertise and knowledge
- Intuition
- Experience
- WONDER

**Limitations**
- Subtle patterns
- Complex patterns
- Large numbers
- Unusual events below a critical frequency
- Variety
- Integrating and remembering trajectory over time
- Easily tired and bored
• **JH focus**: Precision Medicine Centers of Excellence (PMCOEs) - Integrate clinical care and discovery, to maximize recognition and management of subgroups

• Enable collaborations and partnerships *(APL, BSPH, WSE)*

• *Create platform* to enable learning to occur across the health system, and for tools to be easily applied (JH-IT and APL partnership)

• *Build and disseminate new tools* using innovative measurements and analyses to improve health and healthcare at more affordable costs