3 reasons ALS research struggles to find treatment answers

[T]he desire to give [ALS] patients hope has often outstripped good scientific sense. “Many drugs that have gone into ALS clinical trials shouldn’t have, because the preclinical data package didn’t support it,” says [ALS Therapy Department Institute CEO] Steve Perrin.

…

Progress has been hindered by three main challenges. First, the disease’s causal mechanisms are poorly understood.

…

Second, ALS is a highly heterogeneous disease in terms of origin (90 percent to 95 percent of cases are sporadic rather than inherited), initial symptoms (patients may report limb weakness or difficulty in speaking or swallowing), and speed of progression (some patients live months, others decades, after diagnosis). This has made it tricky to model the disease.

…

Finally, there are no quantitative biomarkers to track disease progression or serve as clinical endpoints for trials.

…

[H]owever, the explosion of biological and technical advances in the ALS therapy field—as well as growing connections between the players—have led to general optimism that future drug development might finally be able to avoid past pitfalls.
“ALS has been labeled incurable, but I think it will be curable with the right strategy,” says the University of Arkansas’s [pharmacologist Mahmoud] Kiaei. Steve Perrin agrees: “Many of the things that are in clinical development today are better shots on goal than they were a decade ago.”

Read full, original post: New ALS Therapies Move Closer to the Clinic