

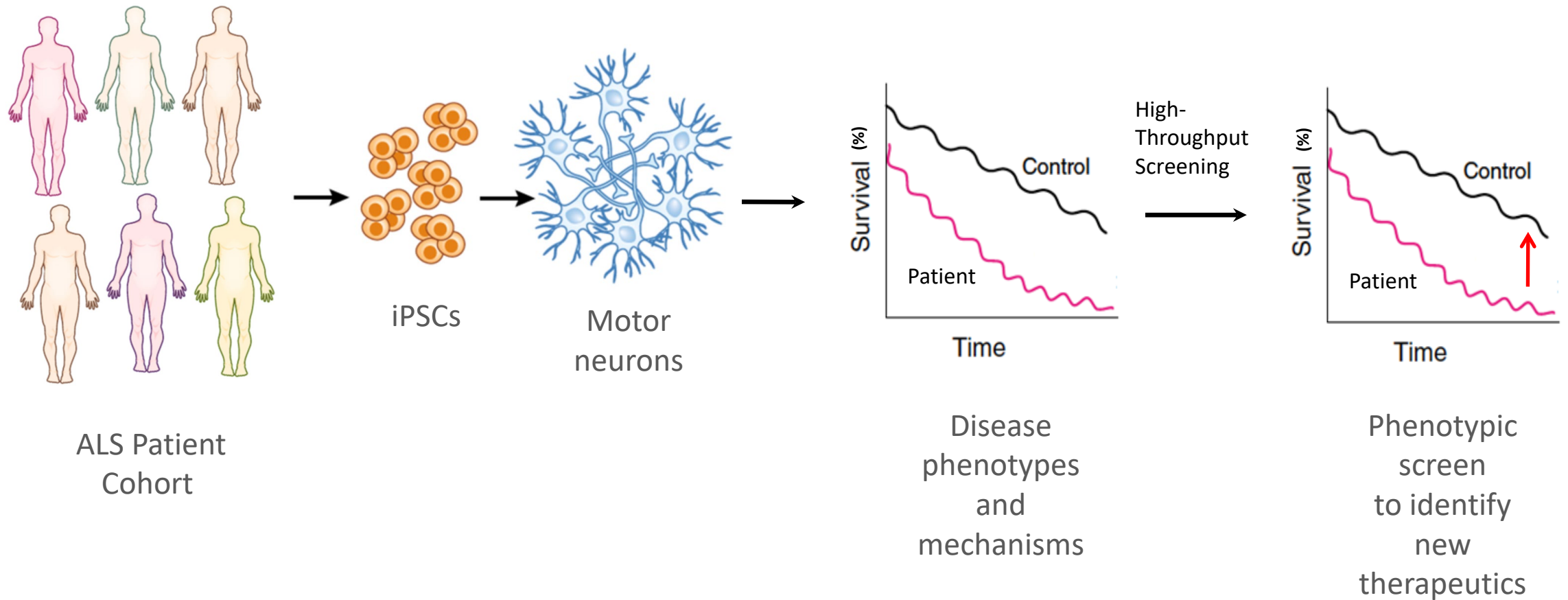
NAS ALS Panel

Justin Ichida

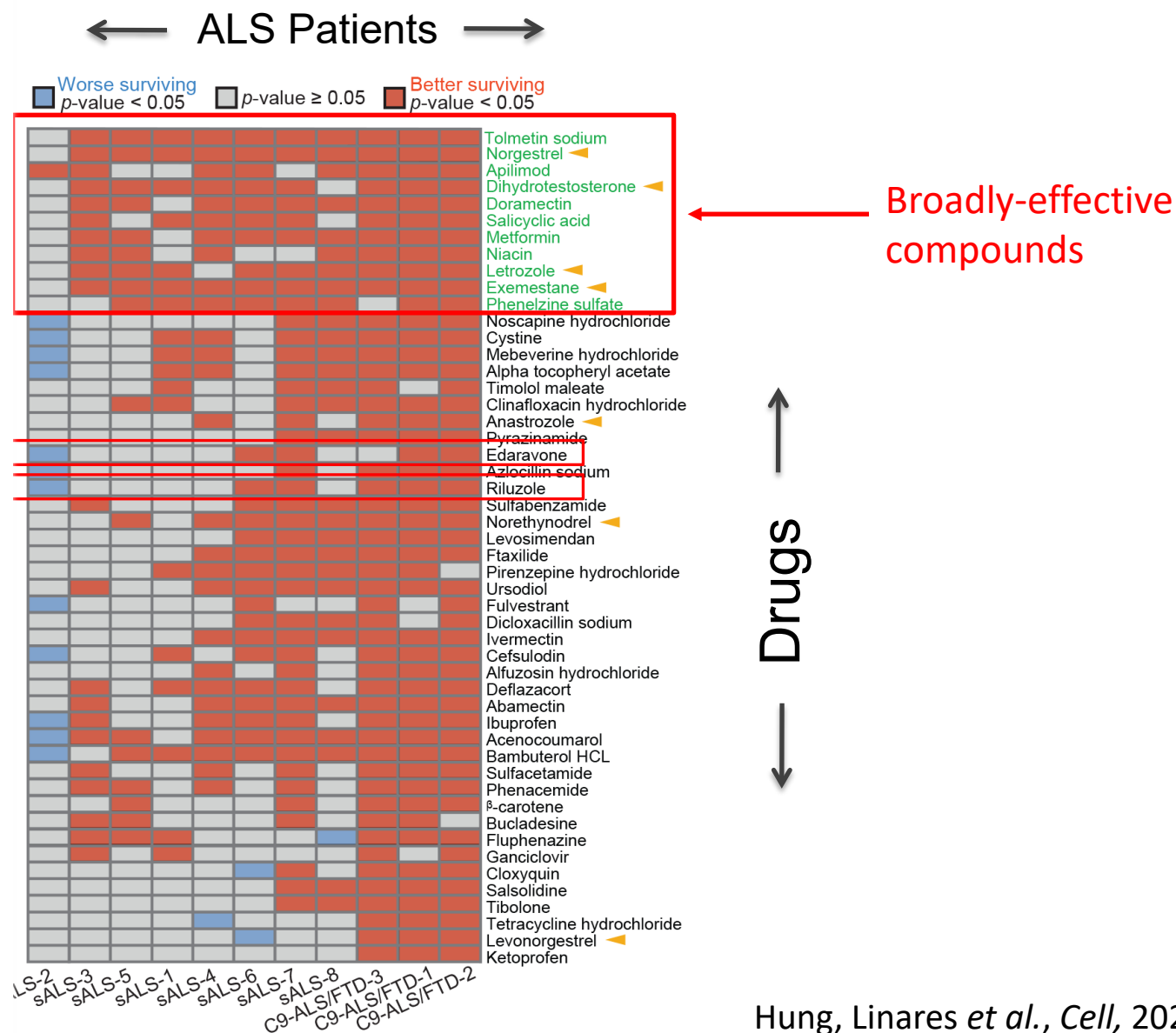
John Douglas French Alzheimer's Foundation Endowed Professor
Associate Professor of Stem Cell Biology and Regenerative Medicine
USC Keck School of Medicine
New York Stem Cell Foundation-Robertson Investigator

Co-founder of AcuraStem, Inc. and Modulo Bio
SAB member of Spinogenix, Vesalius Therapeutics
Employee of BioMarin Pharmaceutical, Inc.

Establishing a patient-specific approach to ALS



2000 compound screen: only rare targets rescue ALS motor neuron degeneration broadly

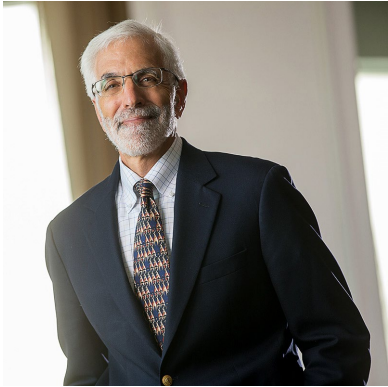


Hung, Linares *et al.*, *Cell*, 2023

Linares, Li *et al.*, *Cell Stem Cell*, 2023

The “ALS” Harrington Discovery Institute Therapeutics Development Team

Lead Advisor



Steve Brenner, PhD

Former VP of
Chemical and
Protein Technology
at Bristol-Myers
Squibb

DNA Drug Expert



Siew Ho, PhD

Former Head of
Nucleic Acid
Research at
Bristol-Myers
Squibb

Chemistry Expert



William Murray, PhD

Former VP of
Chemistry at
J&J

VP of Therapeutics Development, HDI



Diana Wetmore,
PhD

Project Manager



Simon MazzaLunn,
PhD

**ALS mouse
drug test**

**Short tail:
Placebo**

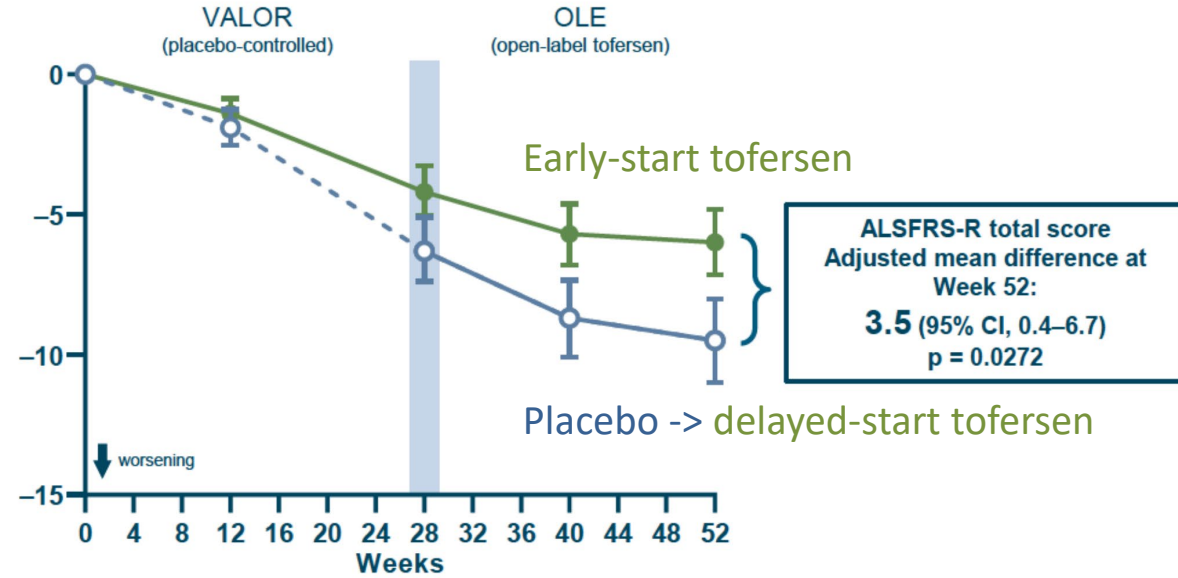
**Long tail:
Drug**



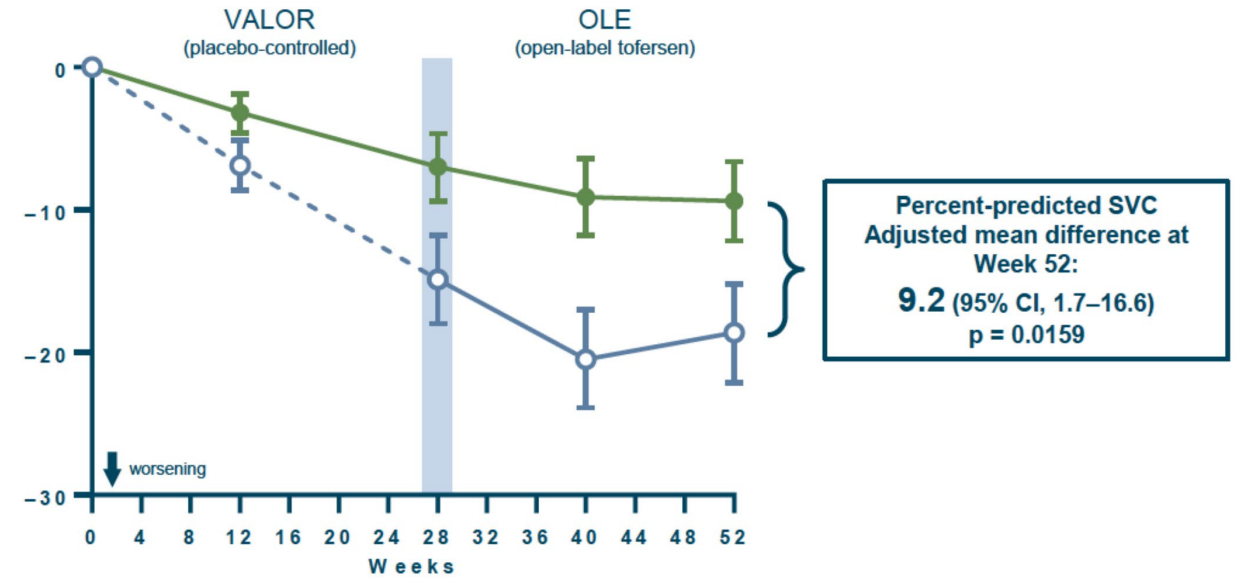
- 2018**
- Harrington Discovery Institute Rare Disease Grant Awarded
 - Identified risks associated with modality and target
 - Redefined project scope
 - HDI Acceleration Funding Granted
 - Defined ASO project plan
- 2019**
- Generation of in vitro proof-of-concept in human derived induced motor neurons from both sporadic and familial ALS patients
 - Human ASOs designed
 - Primary screening of human ASOs completed
- 2020**
- ASO based toxicity risk mitigated
 - In vivo mouse proof-of-concept established
 - ASO technology in-licensed from USC into **AcuraStem, Inc.** for further development

Clinical Trials: length of trials, outcome measures, and surrogate endpoints

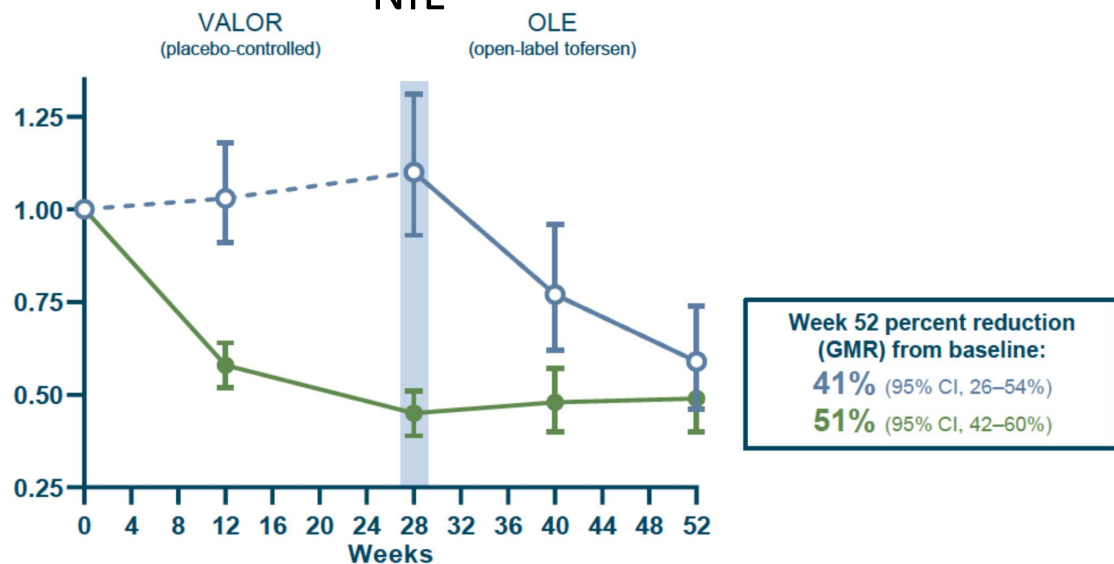
ALSFRS-R



SVC



NfL



ALS nerve cells

**A cancer drug candidate
prevents
ALS nerve cell death
but has poor exposure in
the brain**

