FALS Session 13 May, 2012
Familial ALS

- SOD1
- C9ORF72
- FUS/TLS
- TDP-43
- ?
Mutant- Copper/Zinc SOD1 mediated Toxicity (include genetics summary slide)

> 100 different mutations known

The ALS Association Research Department
Model Systems to Understand Disease Mechanisms
Zebrafish studies

Wim Robberecht
TARGET IDENTIFICATION
EARLY DISCOVERY
VALIDATED TARGETS
THERAPEUTIC DEVELOPMENT
PRE-CLINICAL STUDIES
CLINICAL TRIALS
CLINICAL MANAGEMENT

MODEL SYSTEMS
BIOMARKERS

TOOLS

Laboratory — bedside

SOD1 antisense trial—UCSD/Isis Pharmaceuticals