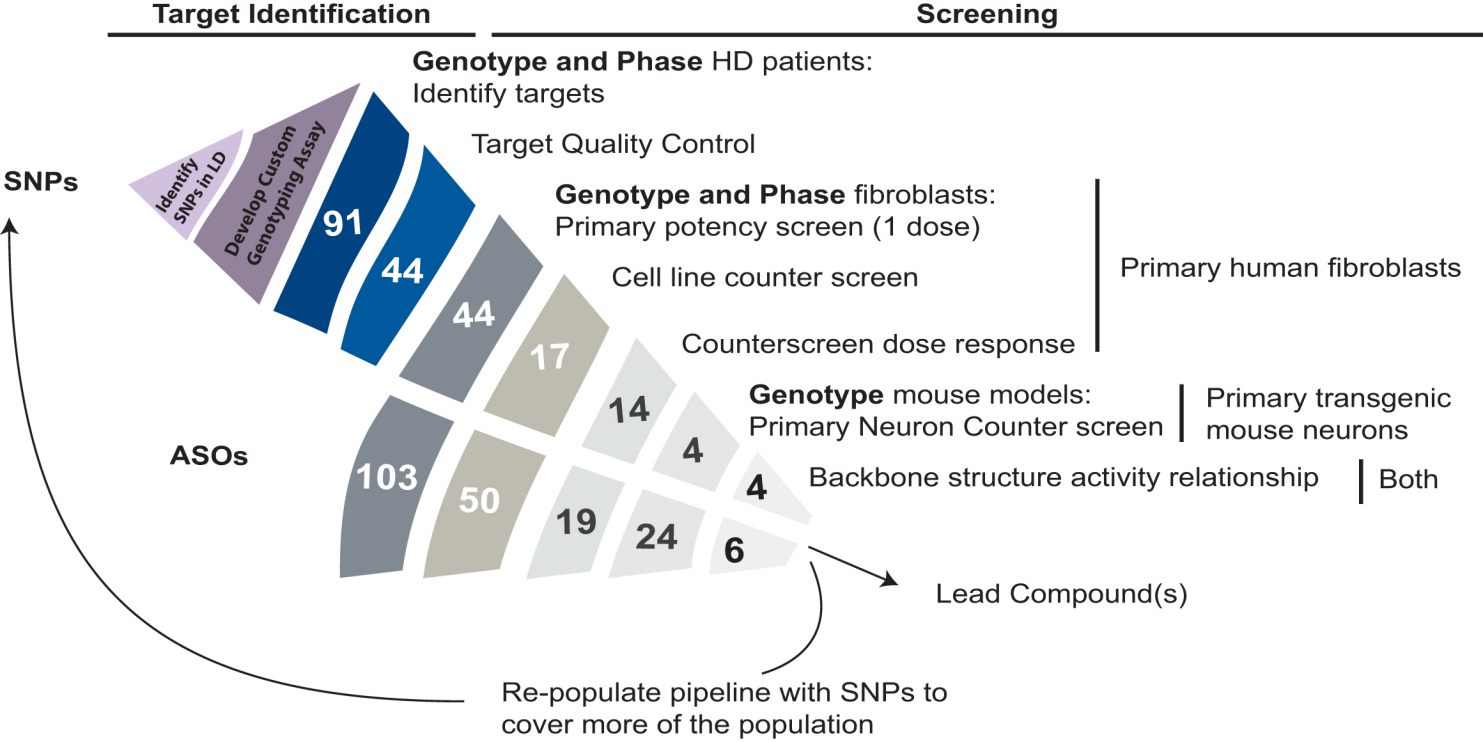
Target Identification
Screening
SNPs
ASOs
Identify SNPs in LD
Develop Custom Genotyping Assay
91
44
44
17
14
4
4
103
50
19
24
6
Genotype and Phase HD patients:
Identify targets
Target Quality Control
Genotype and Phase fibroblasts:
Primary potency screen (1 dose)
Cell line counter screen
Counterscreen dose response
Primary human fibroblasts
Genotype mouse models:
Primary Neuron Counter screen
Primary transgenic mouse neurons
Backbone structure activity relationship
Both
Lead Compound(s)
Re-populate pipeline with SNPs to cover more of the population