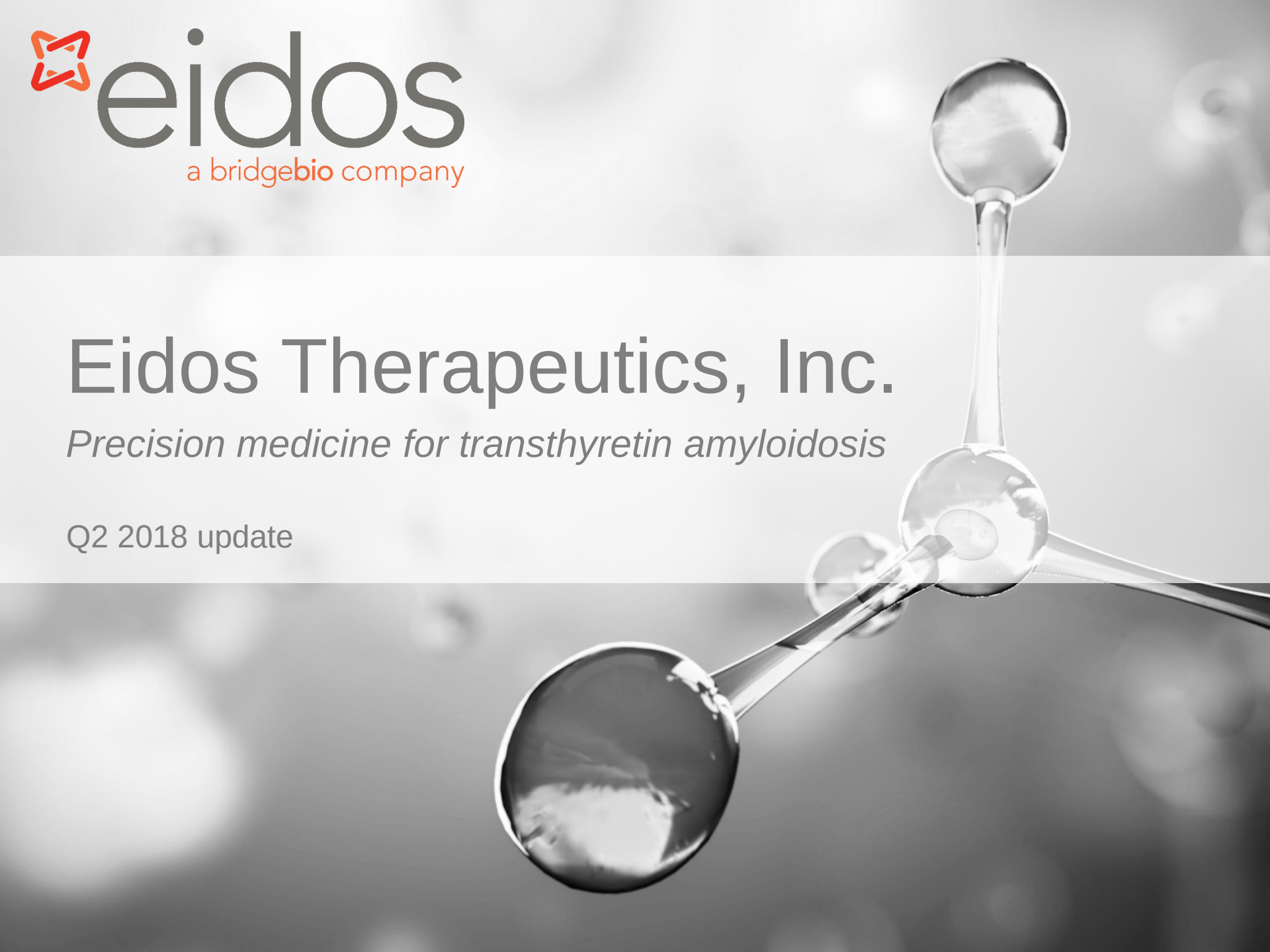
eidos
a bridgebio company

Eidos Therapeutics, Inc.

Precision medicine for transthyretin amyloidosis

Q2 2018 update

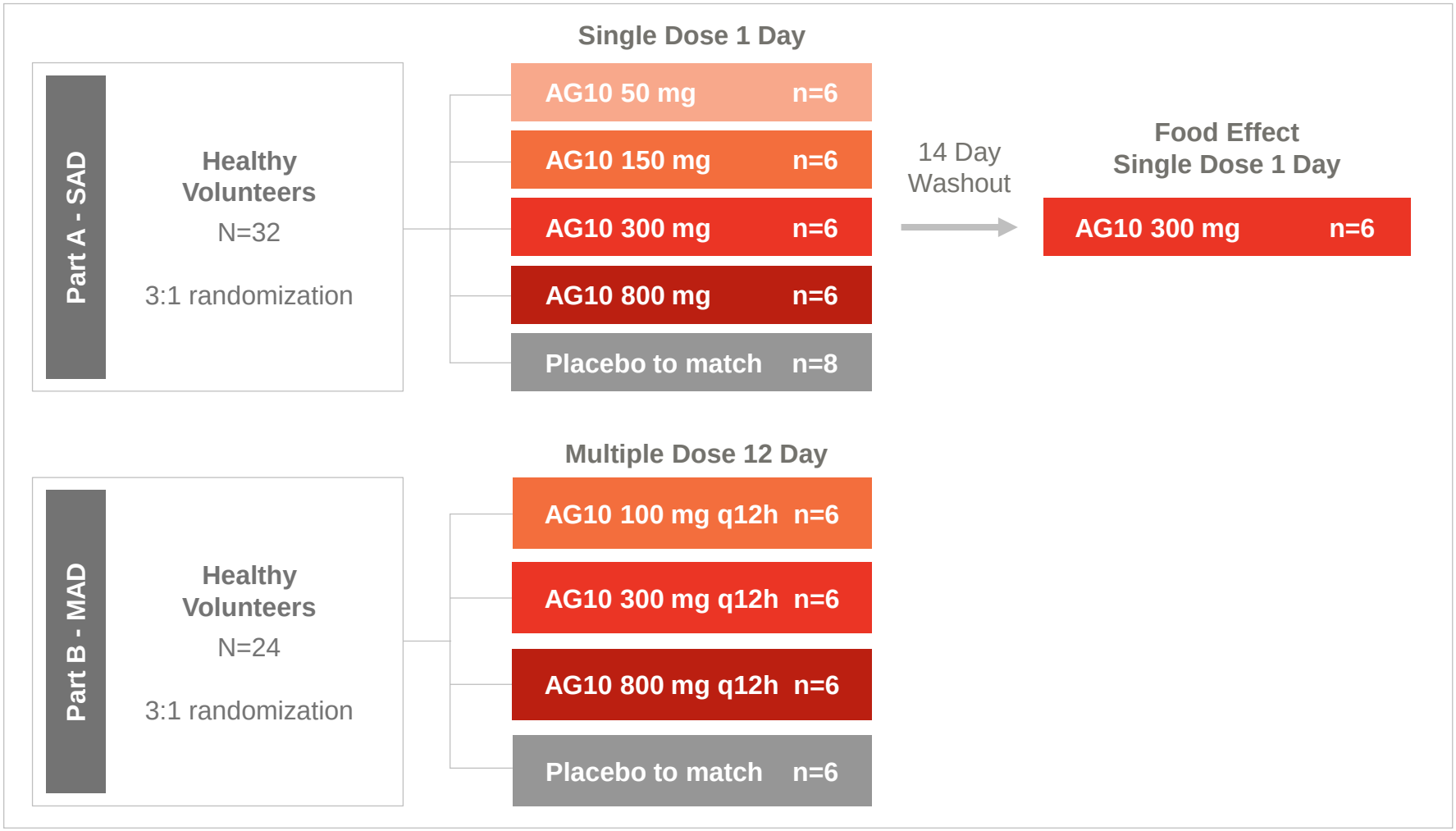


Q2 accomplishments and upcoming milestones

- Initiated and completed enrollment in Phase 2 study of AG10 in symptomatic ATTR-CM patients
- Completed Series B preferred stock financing raising $64 million
- Completed Initial Public Offering, with total gross proceeds of $122 million including exercise of underwriters' option to purchase additional shares, from the sale of 7.2 million shares of common stock
- Complete data from Phase 1 study of AG10 in healthy volunteers to be presented in poster presentation at Heart Failure Society of America 22nd Annual Scientific Meeting (September 15-18)
- Top-line results from ongoing Phase 2 study of AG10 in symptomatic ATTR-CM patients to be announced by the end of 2018

First-in-human study included SAD, MAD, and food effect components

AG10-001 Phase 1 clinical trial study design



AG10 was well-tolerated without any serious adverse events in our Phase 1 study

Summary of Phase 1 safety findings

Number of patients experiencing treatment emergent adverse events (%)

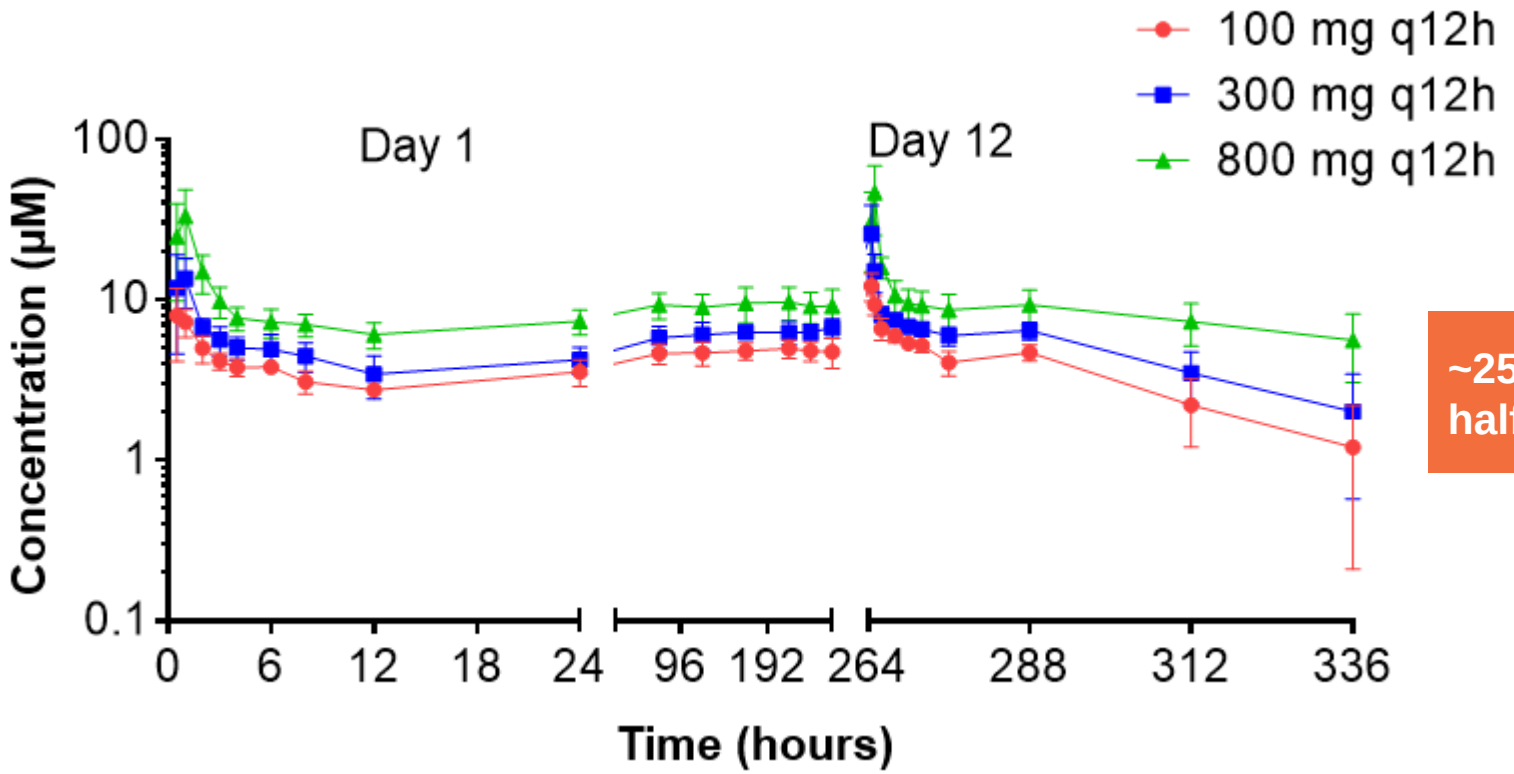
	Single ascending dose					Multiple ascending dose (q12h)			
	Placebo (n = 8)	50 mg (n = 6)	150 mg (n = 6)	300 mg[1] (n = 6)	800 mg (n = 6)	Placebo (n = 6)	100 mg (n = 6)	300 mg (n = 6)	800 mg (n = 6)
SAEs	0 (0%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)
AEs	2 (25%)	3 (50%)	2 (33%)	1 (17%)	1 (17%)	3 (50%)	2 (33%)	5 (83%)	1 (17%)

- Most AEs were reported by single subjects in both the SAD and MAD parts, and all were mild to moderate in intensity
- Only AEs that occurred in more than one subject were dry mouth, generalized headache, upper respiratory infection, and dizziness, all of which occurred in two separate subjects
- No AEs were reported as “probable” with regards to relationship to AG10

1 Adverse event in the fed component; no subjects in the fasted component experienced treatment emergent SAEs or AEs
Source: Data on file

Target steady-state concentration achieved in MAD

Multiple ascending dose pharmacokinetics

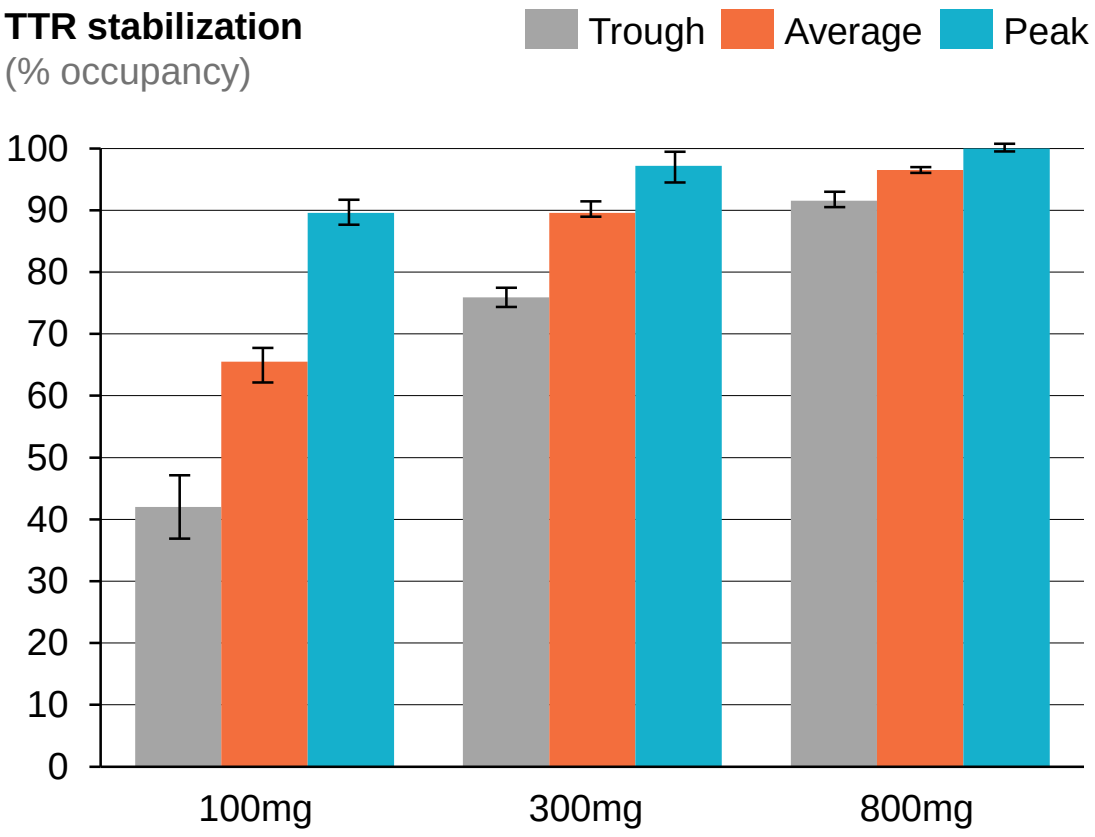


~25h terminal half life

Source: Data on file

Near-complete, sustained TTR stabilization in MAD

Steady-state stabilization in MAD cohorts by FPE assay

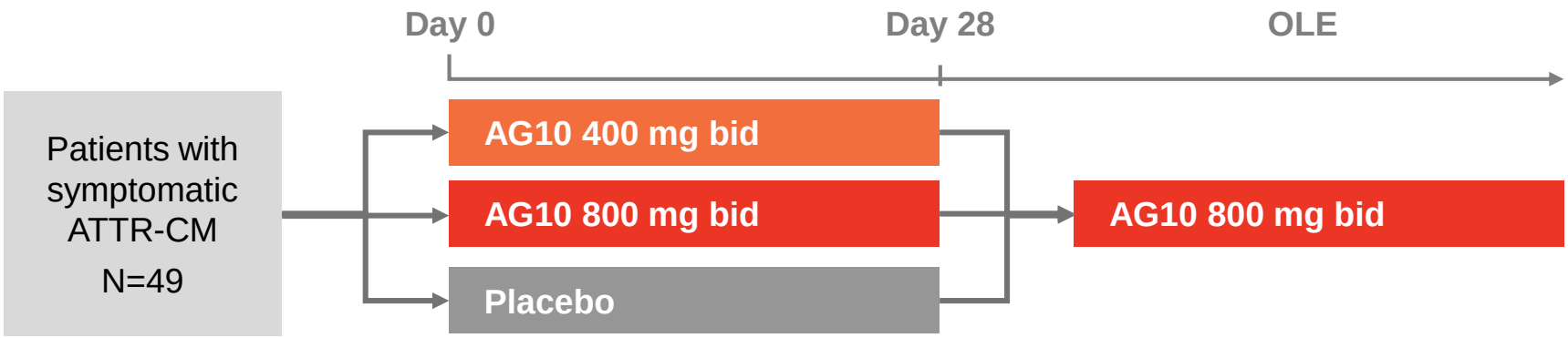


- **Clear dose-dependency**
- **Near-complete stabilization at 800 mg q12h**
  - @ Peak = 100%
  - Average = 96%
  - @ Trough = 92%
- **Full data to be presented at Heart Failure Society of America (General Poster Session I, September 15, 6:15-7:15pm)**

Source: Data on file

Ongoing Phase 2 study is fully enrolled, top line data expected in Q4 2018

Randomized, double-blind, placebo controlled, multi-center study of AG10 in ATTR-CM patients



- **Inclusion criteria:** At least one prior hospitalization for heart failure or clinical evidence of heart failure, confirmed ATTR by scan or biopsy (mutant or wild type)
- **Primary endpoint:** safety and tolerability
- **Secondary endpoints:** Pharmacokinetics, TTR stabilization, TTR concentration
- **OLE:** safety and tolerability, cardiac biomarkers including NT-proBNP and troponin I, echocardiographic measurements including LV wall thickness, global longitudinal strain

- **Top-line results from randomized portion expected by the end of 2018**
- **Ongoing results from OLE expected to yield clinical proof-of-concept**

We will be looking at three measures of TTR stabilization as proof-of-mechanism

In vivo assay

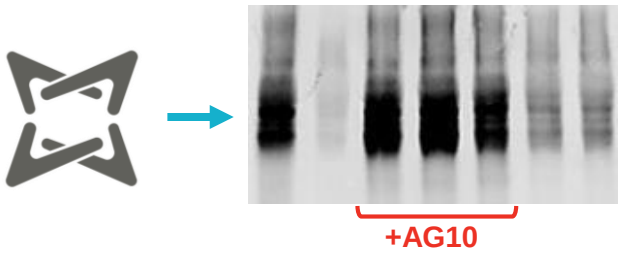
Plasma TTR concentration

- TTR circulates at 4-7 µM in blood with a half-life of approximately two days
- Circulating TTR declines in ATTR patients and is a prognostic indicator of progression
- Increased circulating TTR concentrations are correlated with improved clinical outcomes
- In vivo biomarker of TTR stabilization

Ex vivo assays

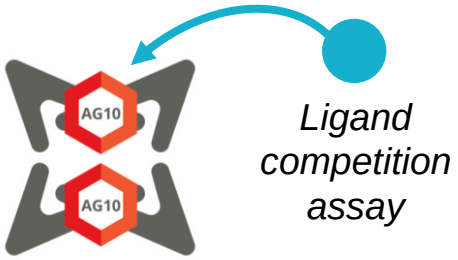
Western blot

- TTR incubated under accelerated destabilizing conditions
- Remaining, stabilized TTR tetramer measured by immunoblotting



Fluorescent probe exclusion (FPE)

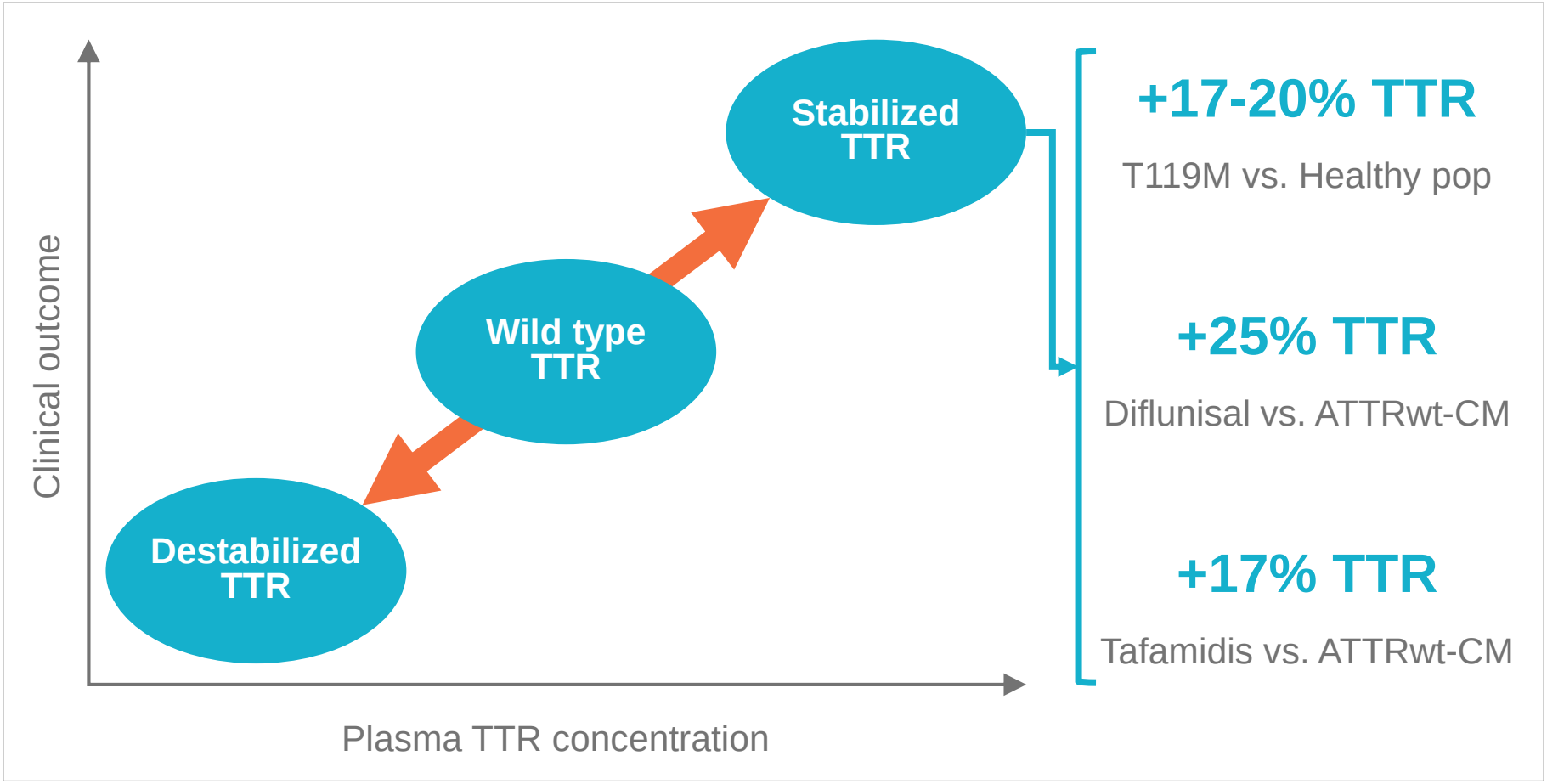
- TTR in plasma/serum incubated with covalent probe
- Fluorescent conjugate measured over time



Source: Hanson, J.L.S. et al. Circ Heart Fail 2018 11:e004000

Plasma TTR concentrations may be predictive of clinical benefit in ATTR patients

Relationship between circulating TTR concentration and clinical outcome



Source: Hornstrup, L.S. et al. Arterioscler Thromb Vasc Biol 2013 33:1441-1447; Hanson, J.L.S. et al. Circ Heart Fail 2018 11:e004000. Tafamidis Food and Drug Administration Center for Drug Evaluation and Research Advisory Committee Meeting background package

Our Phase 3 design will incorporate learnings from Pfizer's ATTR-ACT trial and our internal program

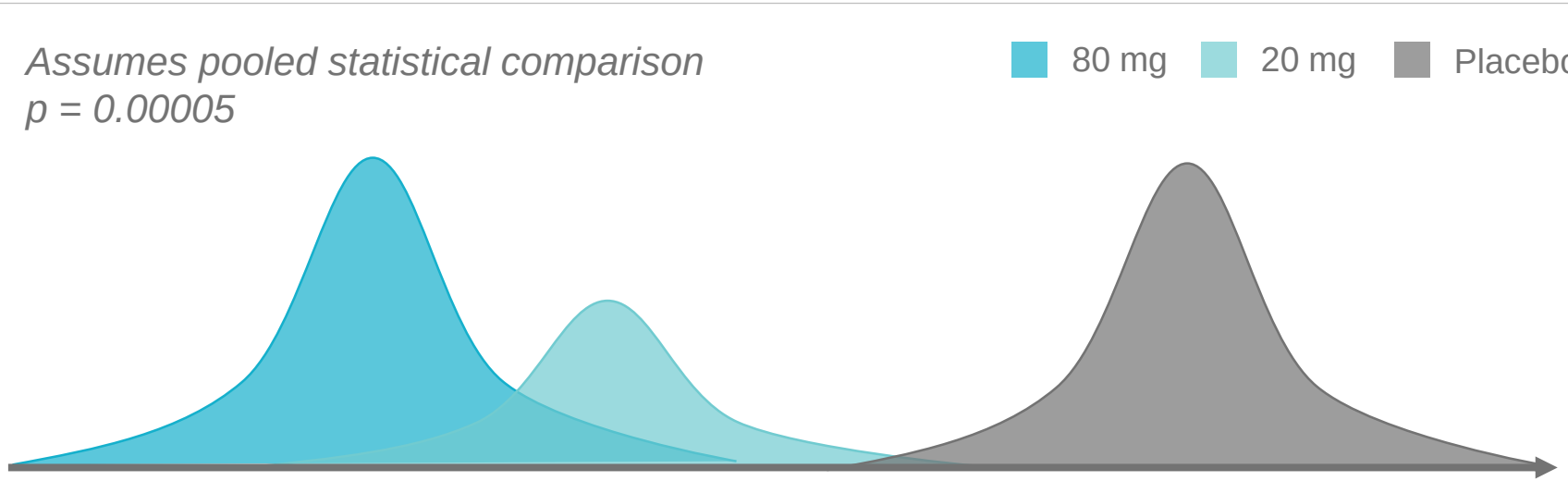
NON-EXHAUSTIVE

Data sources	Implications for AG10 Phase 3
ATTR-ACT readout	
▪ Safety	› Refinement of eligibility criteria
▪ Magnitude of treatment effect	› Size/duration of trial, choice of comparator
▪ Treatment lag	› Duration of study
▪ Biomarkers	› Secondary endpoints
▪ Sub-population comparisons	› Inclusion/exclusion, stratification
▪ **Dose response** ▪ **Mutant vs. wild-type**	→ **Not powered to detect a significant difference**
AG10 ATTR-CM Phase 2	› Safety, dose selection
End of Phase 2 FDA meeting	› Trial design finalization

ATTR-ACT trial not powered to detect difference between 20 mg and 80 mg groups

Illustrative distributions of clinical response between ATTR-ACT groups[1]

Assumes pooled statistical comparison
p = 0.00005



16%

Power to detect significant dose response

15-30%

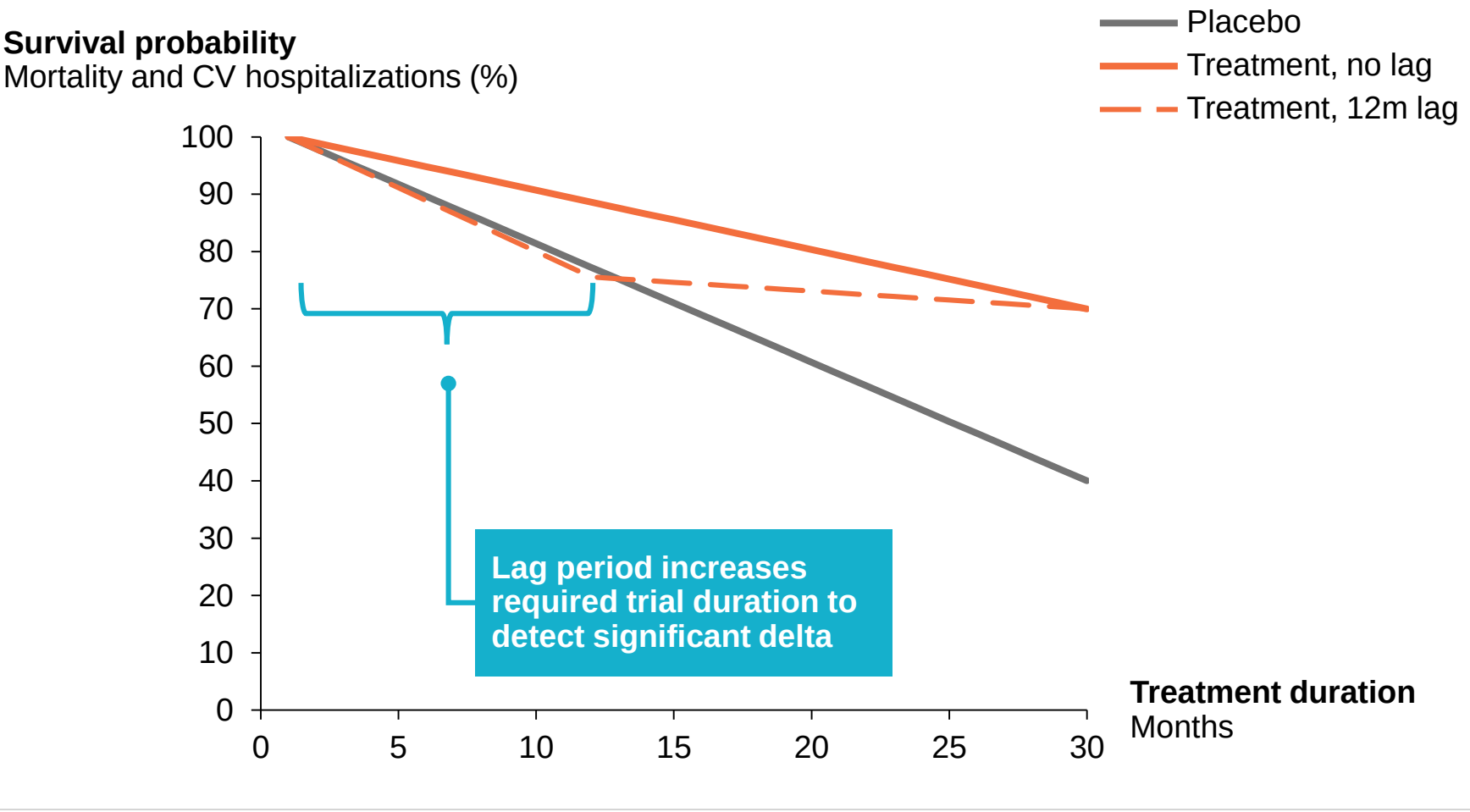
Probability of 20 mg appearing numerically superior to 80 mg

[1] Assumes placebo mortality rate = 25%, placebo hospitalization rate = 50%, pooled treatment group mortality rate = 12.5%, pooled treatment group hospitalization rate = 35%, 10-33% larger response in 80 mg group than 20 mg group, population variability as observed in previous TTR stabilization trials

AG10 Phase 3 trial may be <30 months if a limited treatment lag is observed

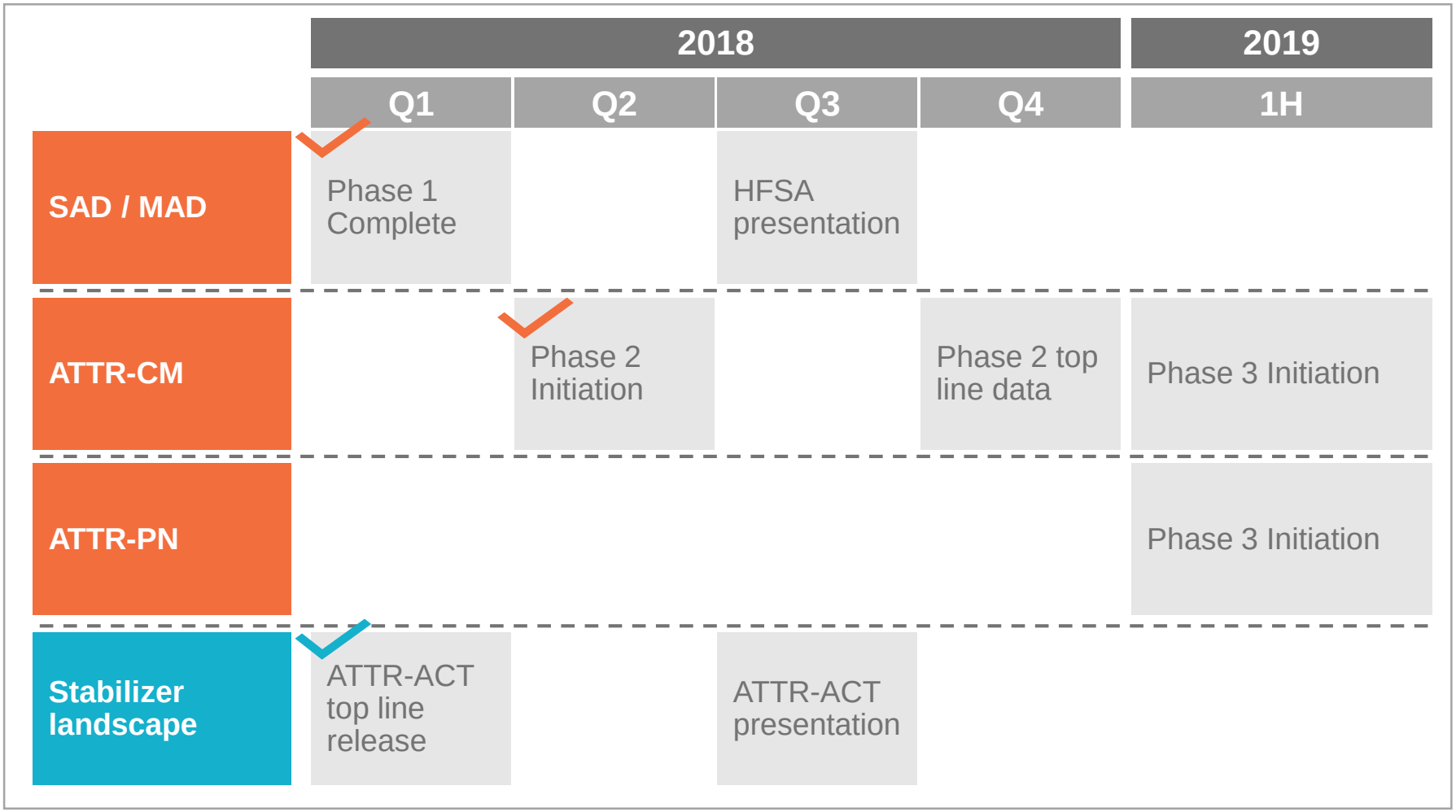
Potential primary endpoint Kaplan-Meier separation in ATTR-ACT

ILLUSTRATIVE



Eidos development timeline and milestones

Anticipated key milestones



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