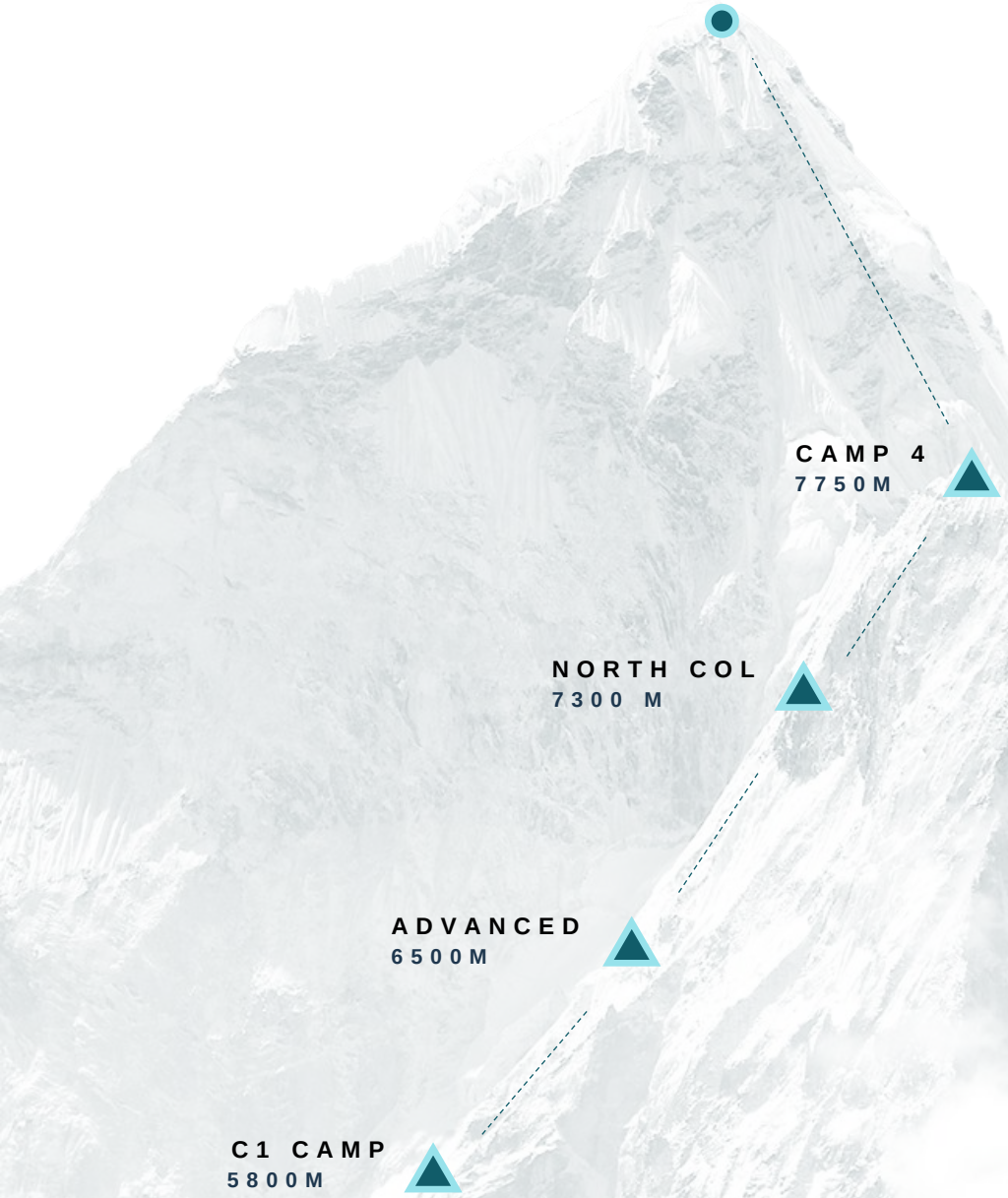

A Phase 1 Study to Evaluate the Safety and Tolerability of an Investigational ASO (CMP-CPS-001) for the Treatment of Urea Cycle Disorders

Yuri Maricich, Chief Medical Officer

CAMP4 Therapeutics Corp.

ASGCT Conference, May 16, 2025

Forward-Looking Statements

This presentation contains forward-looking statements that are based on the beliefs and assumptions and on information currently available to CAMP4's management. All statements other than statements of historical fact contained in this presentation are forward-looking statements. Forward-looking statements include information concerning the initiation, timing, progress and results of preclinical and clinical trials of CAMP4's product candidates, the timing or likelihood of regulatory filings and approvals for any of its product candidates, and estimates regarding CAMP4's expenses, future revenues, and future capital requirements. In some cases, you can identify forward-looking statements because they contain words such as "may," "might," "will," "would," "shall," "should," "expects," "plans," "anticipates," "could," "intends," "target," "projects," "contemplates," "believes," "estimates," "looks," "seeks," "predicts," "potential," "ongoing," or "continue" or the negative of these words or other similar terms or expressions that concern our expectations, strategy, plans or intentions, although not all forward-looking statements are accompanied by such words. Forward-looking statements involve known and unknown risks, uncertainties and other factors that may cause CAMP4's actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. This information was factually accurate on the date it was published. CAMP4 assumes no duty to update the information to reflect subsequent developments, except as required by law.

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Daniel Tardiff PhD: CMP-CPS-001 Urea Cycle Disorders Preclinical, Abstract #337

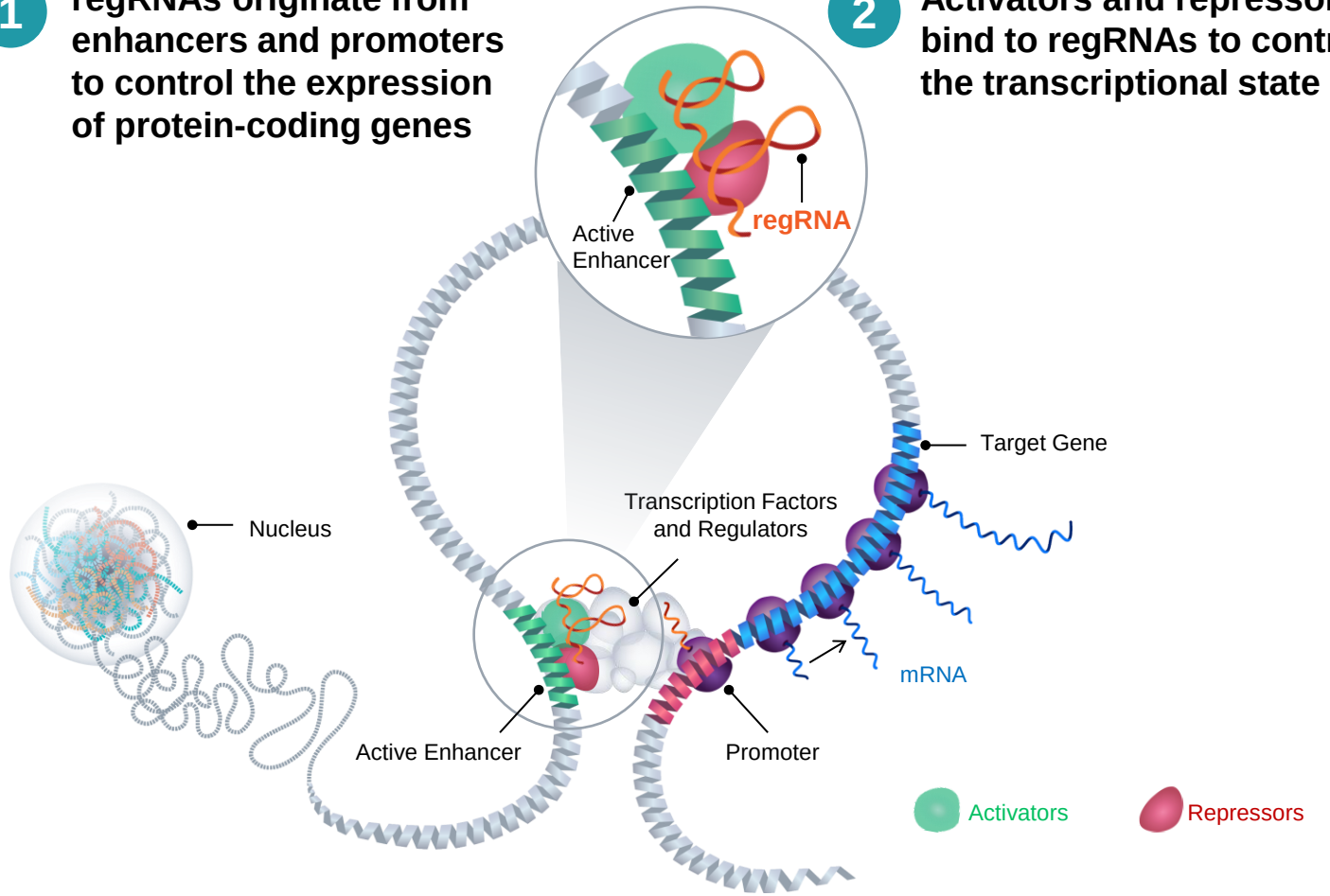
CAMP4 is pioneering a new class of RNA medicines to increase targeted gene expression

Program	Indication	Target	Discovery & Preclinical	Phase 1	Phase 2	Phase 3	Anticipated Milestones	Commercial Rights
Metabolic diseases								
CMP-CPS-001	Urea Cycle Disorders	CPS1					Phase 1 MAD data in Q4'25	CAMP4
CMP-CPS-001 Expansion	Urea Cycle Disorders-OTC heterozygotes	CPS1						CAMP4
CNS diseases								
CMP-SYNGAP-01	SYNGAP1-related Disorders	SYNGAP1					Initiation of GLP tox studies in '25	CAMP4
New Named Program	Genetically defined Parkinson's disease (PD) and sporadic PD	GBA1						CAMP4
New Discovery Programs	CNS & Metabolic	Numerous	Active discovery and development of multiple programs utilizing our RAP Platform™					CAMP4
Collaborations								
Strategic research collaboration leveraging CAMP4's RAP Platform advancing novel therapeutics that increase protein levels by targeting regRNA sequences for two genetic targets.								BIOMARIN

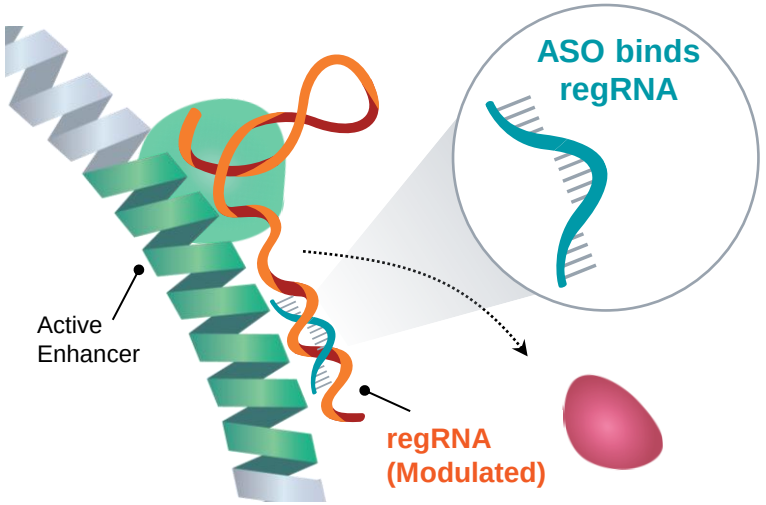
regRNAs play a central role in the regulation of every gene's expression

1 **regRNAs originate from enhancers and promoters to control the expression of protein-coding genes**

2 **Activators and repressors bind to regRNAs to control the transcriptional state**



3 **ASOs disrupt the interactions between repressors and regRNAs enabling increases in gene expression**



Increased mRNA expression

Addresses root cause of **haploinsufficient or partial loss-of-function diseases by** returning **targeted protein levels** to within a healthy range

Source: Sharp et al. (2022) RNA 28: 52-57; Henninger et al. (2021) Cell 184:207-225; Sartorelli & Lauberth (2020) Nat. Struct. Mol. Biol. 27: 521-8; Oksuz et al. (2023) Molecular Cell 83: 2449-63.

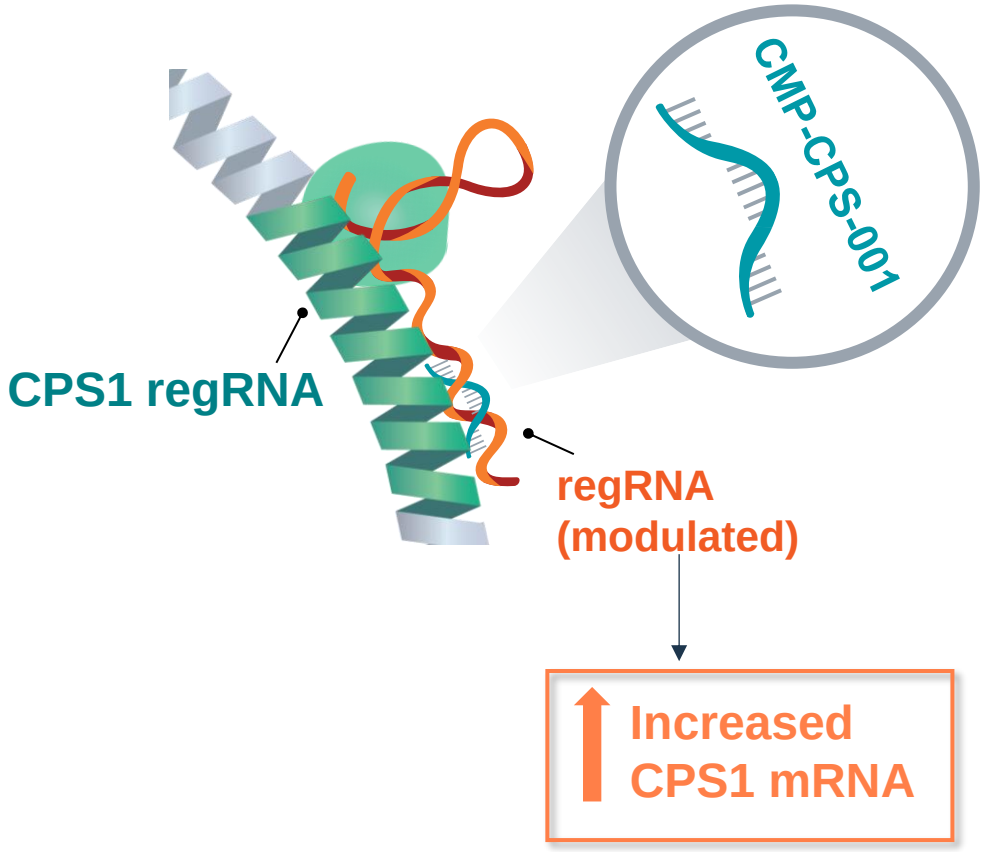
CMP-CPS-001 is designed to increase expression of CPS1, thereby potentially addressing more than 90% of patients with late onset UCDs

CMP-CPS-001

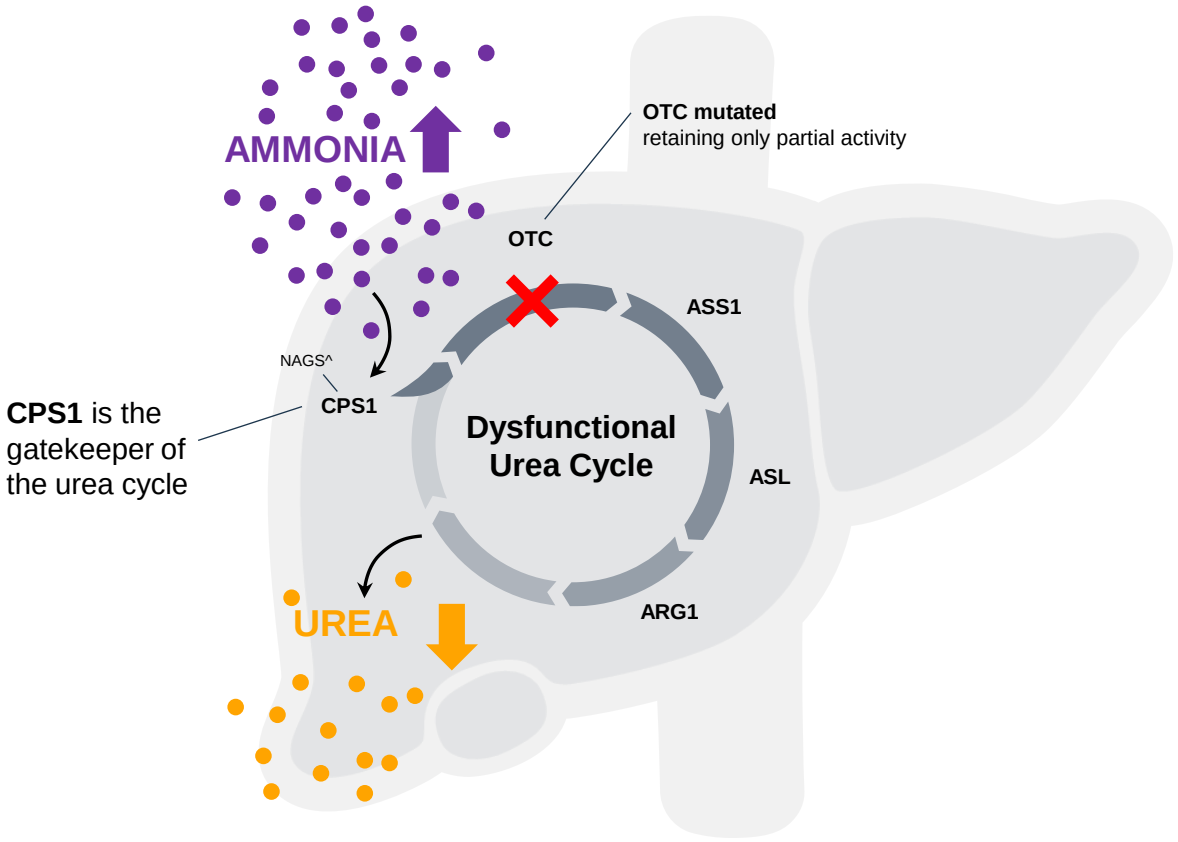
is a GalNAc-conjugated ASO that targets a CPS1 regulatory RNA to upregulate CPS1 expression

CPS1

is the gatekeeper of the urea cycle



Urea Cycle Disorders are caused by deficiency in one or more urea cycle enzymes or transporters, causing ammonia to accumulate



Disease background

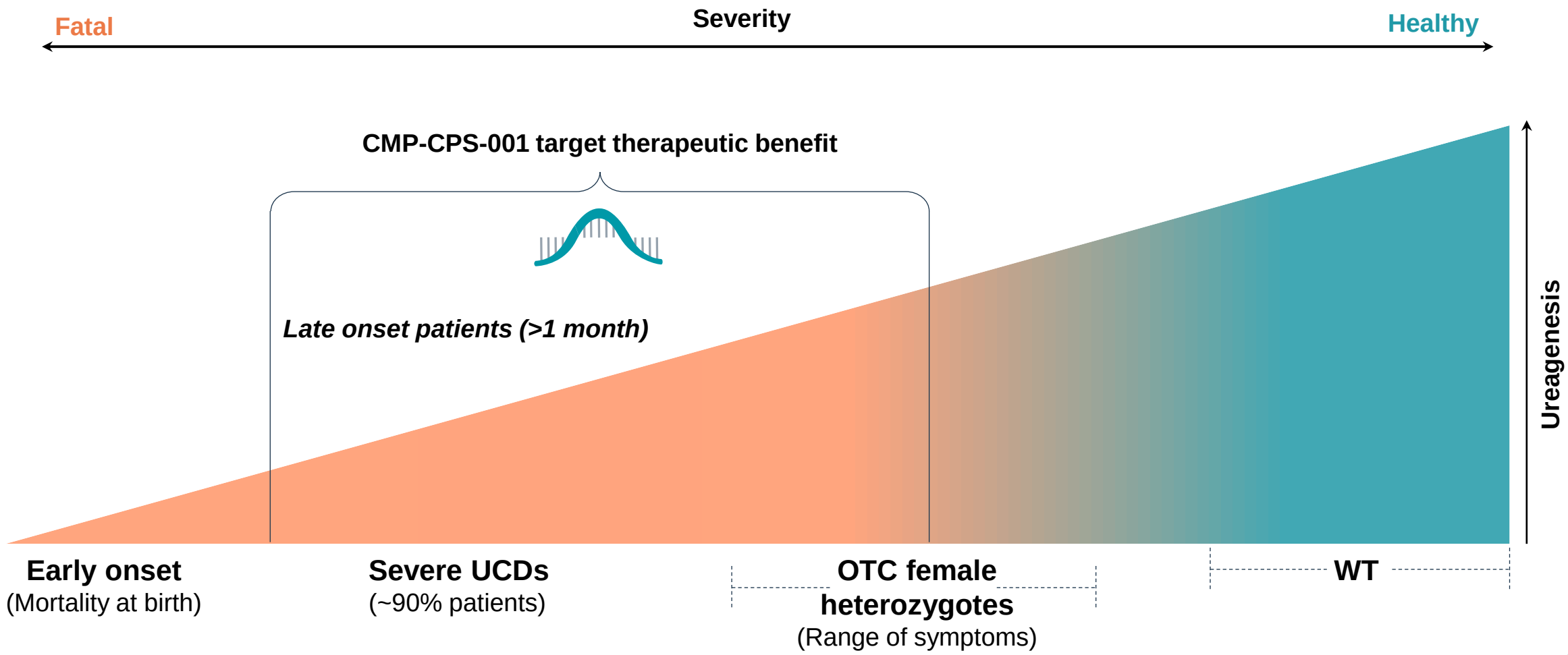
- UCDs are a group of 8 rare genetic diseases responsible for elimination of noxious metabolite ammonia
- Enzyme/transporter deficiency causes suboptimal ureagenesis (conversion of ammonia to urea)
  - Seizures, encephalopathy, coma, and death can occur in severe cases
  - Most prevalent late onset* deficiencies account for 90% of patients (OTC 60%, ASL 16%, ASS1 14%)

Current standard of care

- Symptomatic treatments, no disease-modifying therapy available
  - Burdensome (3-4 pills / day), strict diet
  - Constant risk of hyperammonemic crises can be triggered by lapse in medications or diet

- **Most prevalent UCD, OTC deficiency, is depicted in figure**
- Neonatal patients have severe symptom onset before the first month of life, with enzyme levels of less than 5% of normal; Late onset patients have severe symptoms after one month of life

Clinical observations and prior studies have shown that modest increases in ureagenesis can significantly reduce disease severity



Sources: OTC deficiency prevalence sourced from CAMP4; Diagnosis rate informed by CAMP4 diagnosis rate assumption and extrapolations; "Gyato et al. (2004) Ann. Neurol. 55(1):80-6, McCullough et al. (2000) Am. J. Med. Genet. 93(4):313-9, Scharre et al. (2022) Ann. Clin. Transl. Neurol. 9(11):1715-26, Sen et al. (2024) Mol. Genet. Genomic Med. 12(4):e2443, Sprouse et al. (2014) Mol. Genet. Metab. 113(1-2):136-41.

Overview of US UCD patients who may benefit from treatment with CMP-CPS-001

- Addressable population consists of diagnosed symptomatic patients
- US UCD severe population is estimated to be **2,300 patients out of 3,700 prevalent**
- Female OTC Heterozygotes represent an estimated **incremental 2,000 patients**
- US severe UCD + OTC heterozygotes expanded population is ~**4,300 addressable patients**
- An expanded patient population driven by increased identification of those needing disease-modifying treatment

Sources: Yilmaz et al. (2022) Life (Basel) 12(11): 1721; Buerger et al. (2019) J. Inherit. Metab. Dis. 42(2): 243-53; Posset et al. (2019) Ann. Neurol. 86(1): 116-28; Sen et al. (2024); Batshaw et al. (1986) J. Pediatr. 108(2): 236-41; Trinity Primary Research – N=6 OTCd KOLs, April 2025, Komodo Claims Data 2016-2024; Trinity Life Sciences analysis of Komodo Claims data shows ~3.1K unique female OTC heterozygotes with at least 2 instances of ICD-10 code E72.4 diagnosis from 2016-2024

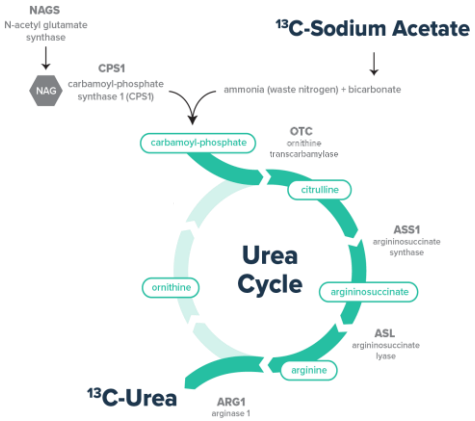
Preclinical evidence demonstrates that CMP-CPS-001 has the potential to treat UCDs by reducing ammonia and enhancing ureagenesis

Preclinical proof of concept

- **Human hepatocyte data:** Dose-dependent increase in CPS1 expression in normal and OTC-deficient human cells
- **Otc-deficient mice data:** 20-30% ↑ ureagenesis compared to baseline, leading to ~50% ↓ ammonia (wild-type levels); ~1 month duration of action
- **Humanized mouse data:** 20-30% ↑ ureagenesis compared to baseline, leading to ~70% ↓ ammonia; ↑ CPS1 + downstream enzymes
- **Non-human primate data:** ~40% ↑ ureagenesis

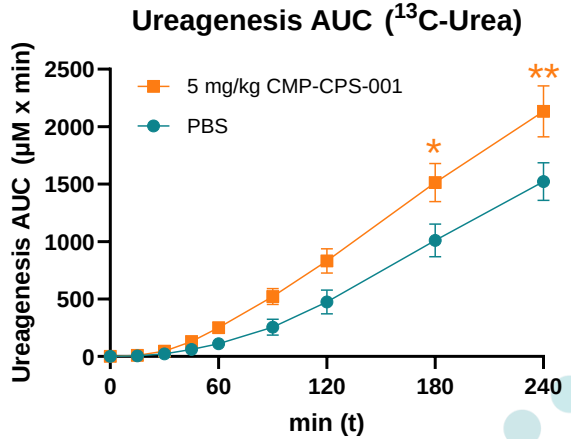
Compelling Non-Human Primate data

Urea Rate Test uses ^{13}C-labeled sodium acetate to measure ureagenesis



Urea cycle enzyme flux: NAGS, CPS1, OTC, ASS1, ASL, ASS1

CMP-CPS-001 increases ureagenesis in NHPs



Using ^{13}C-Sodium Acetate for ureagenesis in NHP. Data shown is one week after a second dose.

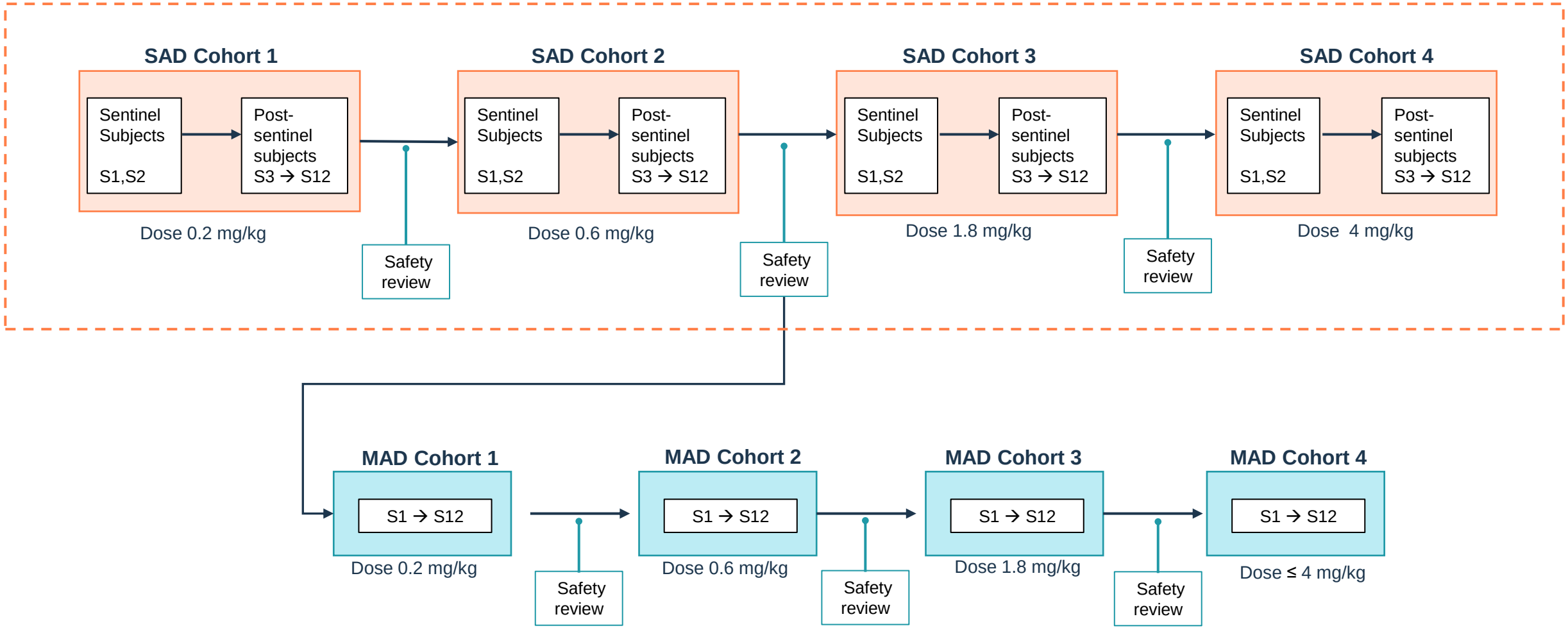
A phase 1 double-blind, placebo-controlled, single (SAD) and multiple ascending dose (MAD) study of CMP-CPS-001 in healthy volunteers

	Objectives	Endpoints
Primary	Evaluate **safety** and **tolerability** of single and multiple doses of CMP-CPS-001 administered subcutaneously (SC) to HVs	• Adverse Events • Vital signs • ECG • Clinical laboratory assessment • Physical examination
Secondary	To characterize the **plasma PK, urinary excretion**, and **ureagenesis** after single and multiple doses of CMP-CPS-001	• Pharmacokinetic parameters and urinary excretion • Ureagenesis rate test determinations using isotope-labeled urea quantification

Study Design	• 96 subjects randomized 3:1 CMP-CPS-001 or placebo • **SAD**: 4 cohorts (N=12); single SC dose; 0.2, 0.6, 1.2, or 4.0 mg/kg • **MAD**: 4 cohorts (N=12); 3 SC doses every 28 days; 0.2, 0.6, 1.2, or 4.0 mg/kg
Key Criteria	• Healthy male or female adults 18 to < 65 years* • BMI ≥18 and ≤32 kg/m^2 and ≤110kg • No significant disease or disorder that would hinder participation in the study or pose a risk to subject in trial • No clinically relevant illness or infection within 7 days before the first dose of study drug

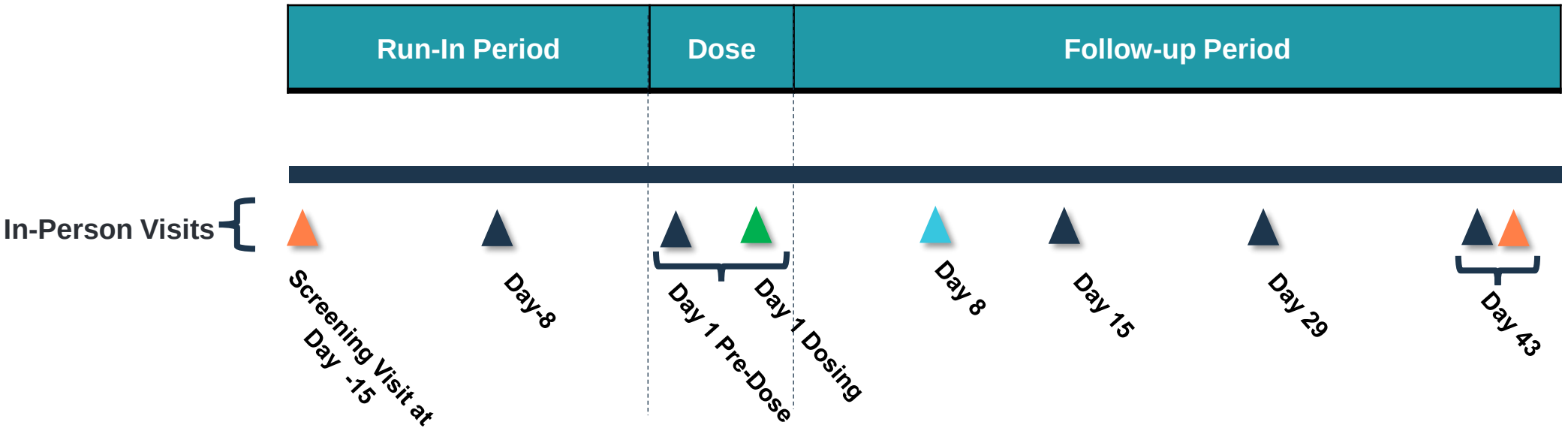
*Per protocol version 5.0

CPS-101 SAD and MAD cohort schematics



S = Subject

Summary of schedule of assessments for SAD cohorts



- Screening, End of Study Visits
- Dosing + 24hr observation (discharge on Day 2)
- Safety laboratory tests and Triplicate ECGs
- Ureagenesis Assay (participants will be admitted the night before for standard meal and fast)

*Additional safety labs and triplicate ECGs performed on Day 14, 28, and 43.

CPS-101 SAD Demographics and Disposition

Demographics

Parameter	(N=48)
Age in years	
Median (range)	20 (18, 55)
Sex	
Female	28 (58.3%)
Male	20 (41.7%)
Race & Ethnicity	
White	32 (66.7%)
Asian/Other	16 (33.3%)
Hispanic or Latino	11 (22.9%)
Not Hispanic or Latino	37 (77.1%)
BMI	
Median (range)	24 kg/m^2 (18.1, 31.9)

Disposition

Enrollment: 48 subjects enrolled into 4 cohorts and received CMP-CPS-001

- 42 completed
- 6 discontinued post-dosing:
  - lost to follow-up, COVID, personal reasons, etc.

Summary of Safety Findings in SAD (N=48)

- CMP-CPS-001 was generally safe and well tolerated with no evidence of a maximum tolerated dose
- All TEAEs were Grade 1 or Grade 2.
- No serious or severe AEs or TEAEs
- No participants discontinued study drug due to an AE
- No trends of concern including across liver function tests, platelets or immunogenicity
- Single participant in SAD Cohort 2 with concomitant viral illness and lymphopenia and GGT elevation 62 at screening had asymptomatic ALT 136 (ratio of 3.8X ULN) and GGT 178 (5X ULN, 2.87XScreening) at D15, normal bilirubin, which self-resolved.

		Cohort 1 0.2 mg/kg or PBO N=12	Cohort 2 0.6 mg/kg or PBO N=12	Cohort 3 1.8 mg/kg or PBO N=12	Cohort 4 4.0 mg/kg or PBO N=12	Overall N=48
Total Number of TEAEs		8	16	6	10	40
Participants with at Least One TEAE		6 (50.0%)	6 (50.0%)	4 (33.3%)	6 (50.0%)	22 (45.8%)
Participants with at least one TEAE by Maximum Severity	**Grade 1 Mild**	4 (33.3%)	3 (25.0%)	3 (25.0%)	3 (25.0%)	13 (27.1%)
	Grade 2 Moderate	2 (16.7%)	3 (25.0%)	1 (8.3%)	3 (25.0%)	9 (18.8%)
Participants with at Least One treatment-related TEAE		1 (8.3%)	3 (25.0%)	1 (8.3%)	3 (25.0%)	8 (16.7%)
Participants with at Least One TEAE of Site Reaction		0	1	0	3	4
Participants with at least one TEAE of Flu-Like Symptoms		0	0	0	0	0

ALT= Alanine Transaminase, AE = Adverse Event, GGT= Gamma-Glutamyl Transferase, PBO= Placebo, SAD = Single Ascending Dose, SAE = Serious Adverse Event, TEAE = Treatment Emergent Adverse Event, ULN= Upper Limit Normal.

Majority of TEAEs were mild or moderate and not related to study drug

		Cohort 1 0.2 mg/kg or PBO (N=12)	Cohort 2 0.6 mg/kg or PBO (N=12)	Cohort 3 1.8 mg/kg or PBO (N=12)	Cohort 44.0 mg/kg or PBO (N=12)	Overall (N=48)
Participants with at least one TEAE by Relationship	***Related***	1 (8.3%)	3 (25.0%)	1 (8.3%)	3 (25.0%)	8 (16.7%)
	Not Related	5 (41.7%)	3 (25.0%)	3 (25.0%)	3 (25.0)	14 (29.2%)

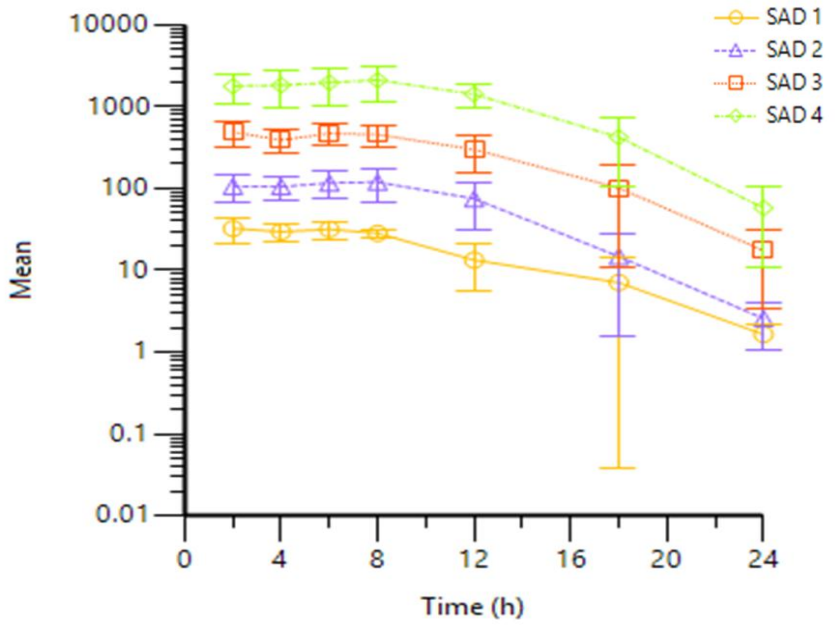
Event (preferred terms)	Number of Participants	Total Number of reported TEAEs*	Number of Related TEAEs
Headache	6	6	1
Nausea	4	4	2
Injection site reaction	4	4	4
Upper respiratory tract infection	2	2	0
Viral upper respiratory tract infection	2	2	0
Hypoglycaemia	2	2	2
LFT derangement (ALT, GGT)	1	2	2

*No participants experienced more than one of a given TEAE so the number of participants reporting an AE and the Total Number of Reported TEAEs were the same.

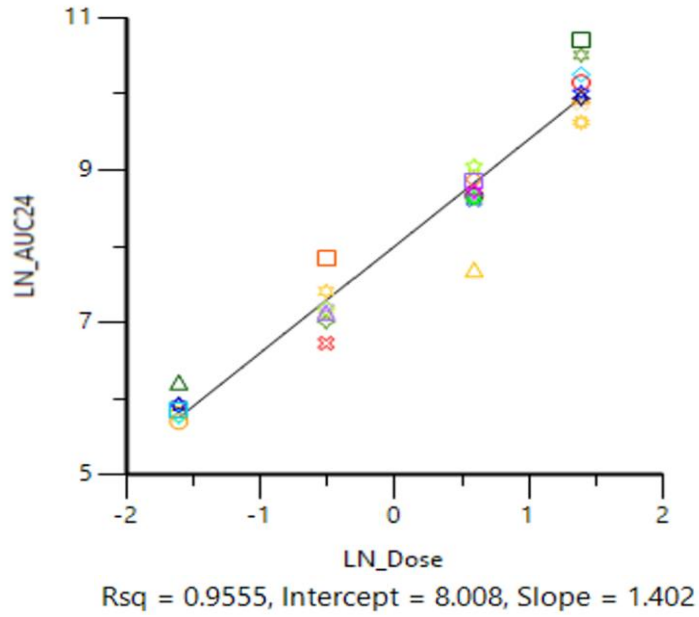
CMP-CPS-001: SAD Plasma PK Summary

- Dose-dependent increase in exposure (Cmax & AUC) with clear separation between groups
- Low-to-Moderate variability in key PK parameters at all dose levels
- Modestly greater than dose-proportional increase in exposure (Cmax and AUC0-24) that increased with dose
- Biphasic elimination distribution and elimination profiles with T1/2 of approximately 2.5-3 hours

Group means (ng/mL)



Dose-proportionality



Female OTC patients- *an underrecognized yet severe population*- could enable additional proof-of-concept

Disease Snapshot

~1:2 male to female diagnosed prevalence ratio; females are characterized by a broad spectrum of **clinical variability** from asymptomatic to severely symptomatic

55% of identified females are symptomatic, experiencing **hyperammonemia crisis and are burdened by chronic neurocognitive symptoms**

75% of females are identified through family screening; metabolic MDs believe **many females remain undiagnosed**

30-80% of diagnosed symptomatic females are managed with ammonia scavengers

- OTC deficiency is X-linked; female heterozygotes carry one mutant copy of the gene
- Expanding into OTC heterozygotes expected to enable us to **measure ureagenesis** in a population with reduced urea cycle function
- **Potential opportunity to measure ammonia** via protein-challenge which can produce a transient hyperammonemic state

Sources: Yilmaz et al. (2022) , Buerger et al. (2019), Posset et al. (2019), Sen et al. (2024), Batshaw et al. (1986), Trinity Primary Research – N=6 OTCd KOLs, April 2025

Clinical Program Update: CMP-CPS-001 expansion into Ph 1b in OTC heterozygotes

SAD* HV - completed

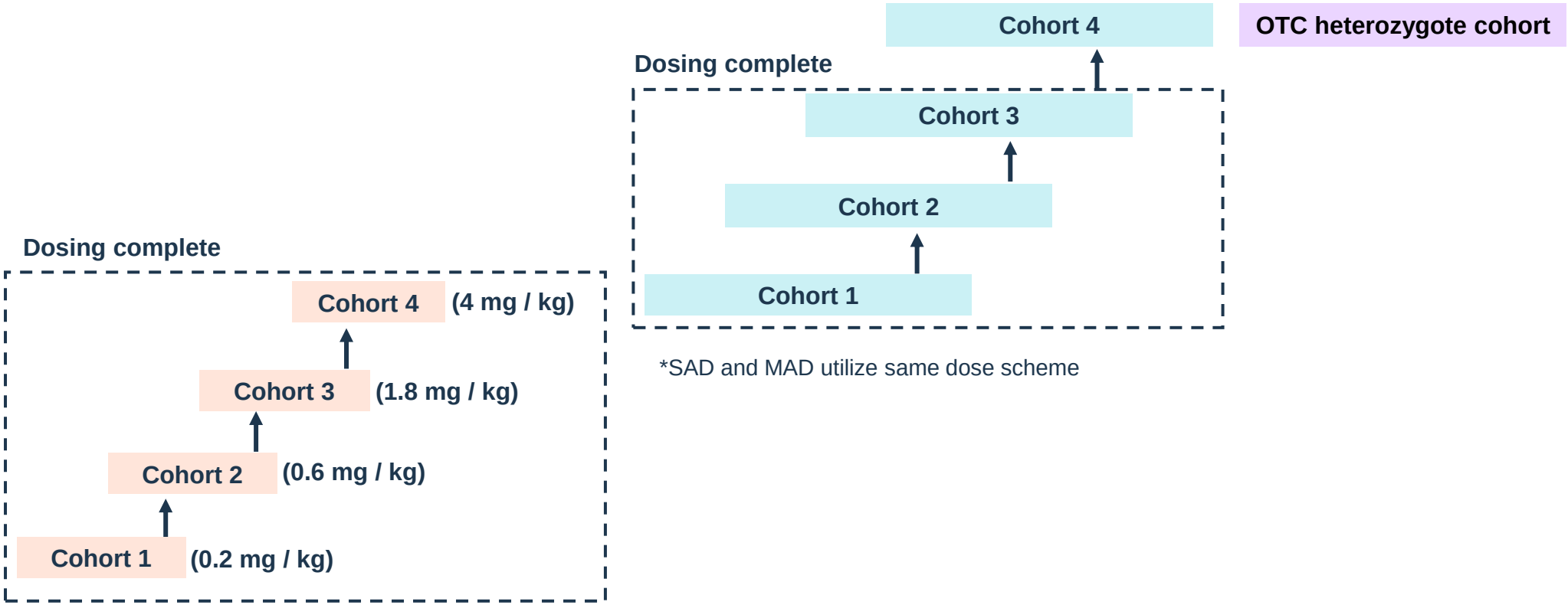
PRIMARY FOCUS: safety, inform MAD

MAD* HV - data expected in Q4 2025

PRIMARY FOCUS: safety and pharmacokinetics / pharmacodynamics (URT)

Anticipated OTC expansion

PRIMARY FOCUS: safety, pharmacokinetics / pharmacodynamics (URT) in OTC heterozygotes



*SAD and MAD utilize same dose scheme

*n=10- 12 participants per cohort, 3:1 randomization drug to placebo. MAD frequency is q28 days x 3 doses. Subcutaneous administration.

Ongoing trial has the potential to support a combined Phase 2/3 study

Data Elements

1 Safety

- Vitals
- Cardiac monitoring
- Liver function tests
- Immunogenicity

2 Pharmacokinetics

- Plasma and urine measurements
- Compare human pharmacokinetic behavior to pre-clinical data observations
- Observe that human PK achieves levels expected to demonstrate efficacy on ammonia and ureagenesis in animal studies

3 Ureagenesis rate test (PD)

- Ureagenesis measures rates of flux through the urea cycle
- Rates of ureagenesis correlate with reduction in ammonia (approvable endpoint)
- URT utilized by other programs to correspond with clinically meaningful dropping of low protein diet and scavenger (supportive care measures)

Key Endpoint(s) in one or more anticipated Phase 2 / 3 Trials:

- Ammonia (recognized approvable endpoint)
- Diet liberalization (Responder analysis)
- Nitrogen Scavenger reduction (Responder Analysis)
- Ureagenesis, Plasma glutamine
- Clinical events

Conclusions

- CPS-101 is a double-blinded, randomized single-ascending and multiple ascending dose clinical trial with SAD portion of 48 enrolled participants (3:1) with 36 randomized participants receiving study drug
- Overall single doses of CMP-CPS-001 from 0.2 to 4.0 mg/kg were safe and well tolerated, with no evidence of maximum tolerated dose
- Majority of TEAEs were mild or moderate and not related to study drug
  - All TEAEs Grade 1 or Grade 2
  - Top two most common TEAEs most were headache (6) followed by nausea (4)
  - No trends of concern across liver function tests, platelets or signs of immune reaction
- *Pharmacokinetics demonstrated*
  - A dose-dependent increase in exposure (Cmax & AUC) with clear separation between dose levels
  - Greater than dose-proportional increase in exposure (Cmax and AUC0-24)
- *Study Update and Expansion*
  - MAD Cohort 1 thru 3 dosing completed
  - Anticipated expansion into OTC heterozygotes to assess safety and CMP-CPS-001 effect on ureagenesis and ammonia (protein-challenge) in patients with evidence of reduced urea cycle function

Acknowledgements

We thank the investigators, healthcare providers, research staff, and patients who continue to work on our clinical studies.

Questions?

CAMP4™

Thank you