



3Q 2021
CORPORATE UPDATE

November 2021

Forward-looking statements

This presentation may contain “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995 relating to our business, operations, and financial conditions, including but not limited to express or implied statements regarding the current beliefs, expectations and assumptions regarding the future of our business, future plans and strategies, our development plans, our preclinical and clinical results and other future conditions. Words such as, but not limited to, “look forward to,” “believe,” “expect,” “anticipate,” “estimate,” “intend,” “plan,” “would,” “should” and “could,” and similar expressions or words, identify forward-looking statements. Any forward-looking statements in this presentation are based on management’s current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this presentation, including, without limitation, risks relating to: (i) the success and timing of our ongoing clinical trials, (ii) the success and timing of our product development activities and initiating clinical trials, (iii) the success and timing of our collaboration partners’ ongoing and planned clinical trials, (iv) our ability to obtain and maintain regulatory approval of any of our product candidates, (v) our plans to research, discover and develop additional product candidates, (vi) our ability to enter into collaborations for the development of new product candidates, (vii) our ability to establish manufacturing capabilities, and our and our collaboration partners’ abilities to manufacture our product candidates and scale production, (viii) our ability to meet any specific milestones set forth herein, and (ix) uncertainties and assumptions regarding the impact of the COVID-19 pandemic on our business, operations, clinical trials, supply chain, strategy, goals and anticipated timelines. New risks and uncertainties may emerge from time to time, and it is not possible to predict all risks and uncertainties. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise. Although we believe the expectations reflected in such forward-looking statements are reasonable, we can give no assurance that such expectations will prove to be correct. Accordingly, readers are cautioned not to place undue reliance on these forward-looking statements.

For further information regarding the risks, uncertainties and other factors that may cause differences between our expectations and actual results, you should review the “Risk Factors” section of our Annual Report on Form 10-K filed for the period ended December 31, 2020, our Quarterly Reports on Form 10-Q and other subsequent filings with the Securities and Exchange Commission.

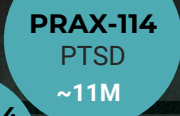


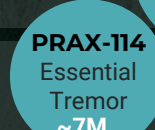

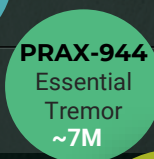
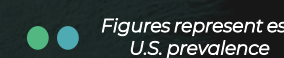
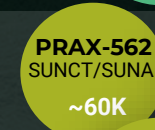
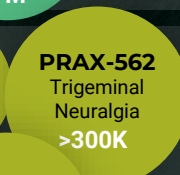





Certain information contained in this presentation relates to or is based on studies, publications, surveys and other data obtained from third-party sources and our own internal estimates and research. While we believe these third-party sources to be reliable as of the date of this presentation, we have not independently verified, and make no representation as to the adequacy, fairness, accuracy or completeness of, any information obtained from third-party sources. In addition, all of the market data included in this presentation involves a number of assumptions and limitations, and there can be no guarantee as to the accuracy or reliability of such assumptions. Finally, while we believe our own internal research is reliable, such research has not been verified by any independent source.

Leveraging genetics to efficiently translate insights into therapies repeatedly

- 01 **Targets identified through genetics**
- 02 **Translational tools to inform development**
- 03 **Efficient, rigorous clinical development paths to PoC**
- 04 **Patient-guided development strategies**



Broad portfolio of highly differentiated programs across multiple CNS disorders

FOCUS AREA	MECHANISM OF ACTION	PROGRAM	DISCOVERY	PRECLINICAL	PHASE 1	PHASE 2	REGISTRATIONAL ENABLING
PSYCHIATRY	GABA_A receptor PAM <small>GABRG2/A1</small>	PRAX-114 <i>Small molecule</i>				 	
MOVEMENT DISORDERS	GABA_A receptor PAM <small>GABRG2/A1</small>	PRAX-114 <i>Small molecule</i>					
	T-type calcium channel blocker <small>CACNA1G</small>	PRAX-944 <i>Small molecule</i>				 	
RARE DISEASES	Persistent sodium current blocker <small>SCN8A</small>	PRAX-562 <i>Small molecule</i>				 	
	Potassium channel T1 blocker <small>KCNT1</small>	KCNT1 INHIBITOR <i>Small molecule</i>					
	Nav1.2 downregulation <small>SCN2A</small>	PRAX-222* <i>Antisense Oligonucleotide</i>					
	Nav1.2 upregulation <small>SCN2A</small>	SCN2A-LOF** <i>Antisense Oligonucleotide</i>					

* PRAX-222 is a collaboration with Ionis Pharmaceuticals, and RogCon Inc; Ionis is eligible to receive double-digit royalties on net product sales worldwide.

** SCN2A-LOF is a collaboration with The Florey Institute; collaboration includes 2 additional ASOs with undisclosed targets

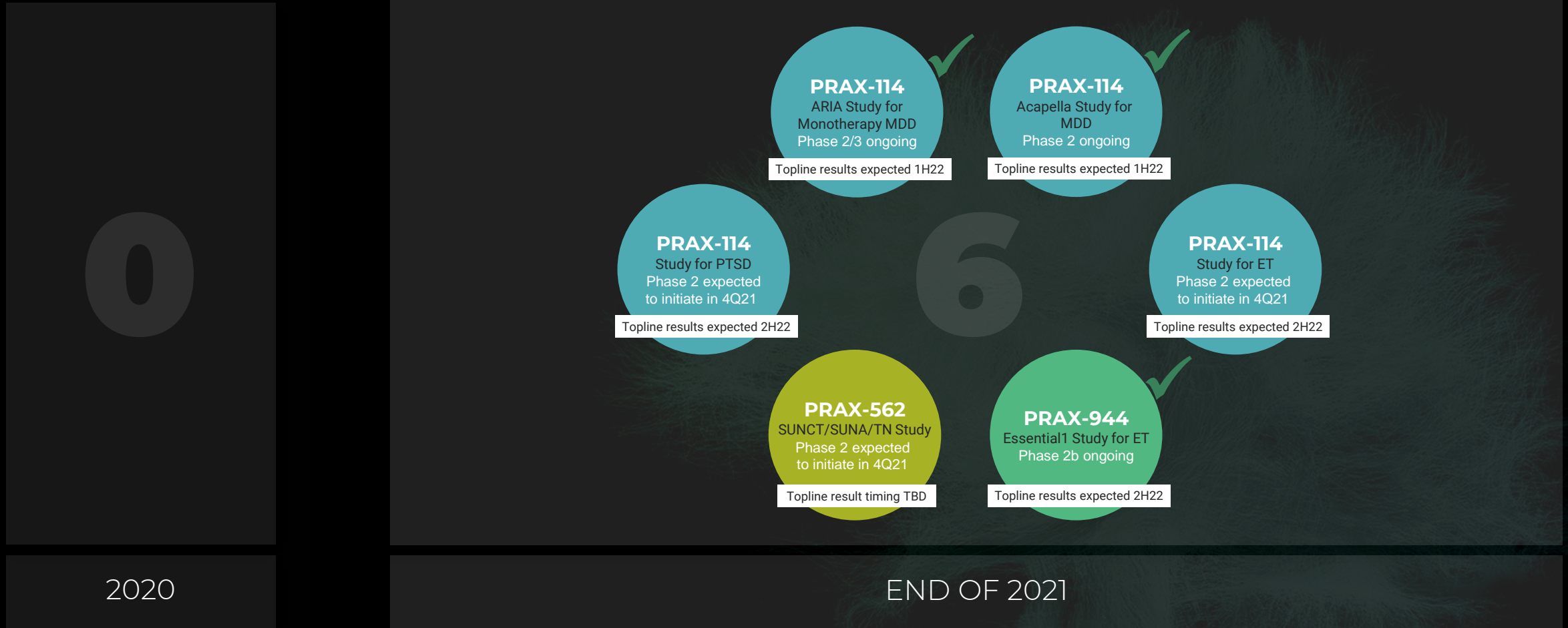
+ Phase 2b trial in women with menopausal & mood symptoms

PRAX-114 Phase 2 trials for ET and PTSD, PRAX-944 Phase 2 trial for PD and PRAX-562 trials for SUNCT/SUNA/TN and for DEEs have not initiated

Prevalence based on internal estimates

Six placebo-controlled trials across three clinical programs by end of 2021

PIPELINE MATURING TOWARD LATER STAGE



2020

END OF 2021

Substantial potential for value creation across the portfolio

MULTIPLE POTENTIAL VALUE-CREATING MILESTONES EXPECTED WITHIN THE NEXT 12+ MONTHS

PROGRAM	INDICATION	Q4 2021	Q1 2022	Q2 2022	Q3 2022	Q4 2022
PRAX-114	MDD		Phase 2/3 Aria Study Topline			
	PMD*		Phase 2 Acapella Study Topline			
	PTSD	Initiate Phase 2 Trial			Phase 2 Topline	
	ET	Initiate Phase 2 Trial			Phase 2 Topline	
PRAX-944	ET	Phase 2a High Dose Preliminary OL			Phase 2b Essential1 Study Topline	
	PD		Initiate Phase 2 Trial			
PRAX-562	SUNCT/SUNA/TN	Initiate Phase 2 Trial		Phase 1 Topline ASSR Biomarker		
	DEEs			Initiate Phase 2 Trial		
Preclinical	PRAX-222		Initiate Phase 1/2 SCN2A-DEE Trial			
	KCNT1	Development Candidate Nominated				

* Plans for upcoming PRAX-114 Phase 2b study in women with menopausal and mood symptoms to be disclosed by end of 2021

PRAX-114

GABA_A Receptor PAM

*PSYCHIATRY &
MOVEMENT DISORDERS*

Depression
Post-traumatic Stress Disorder
Essential Tremor

KEY UPCOMING MILESTONES

1H 2022

Ph 2/3 Monotherapy MDD Aria Study Topline

1H 2022

Ph 2 MDD Dose-Ranging Acapella Study
Topline

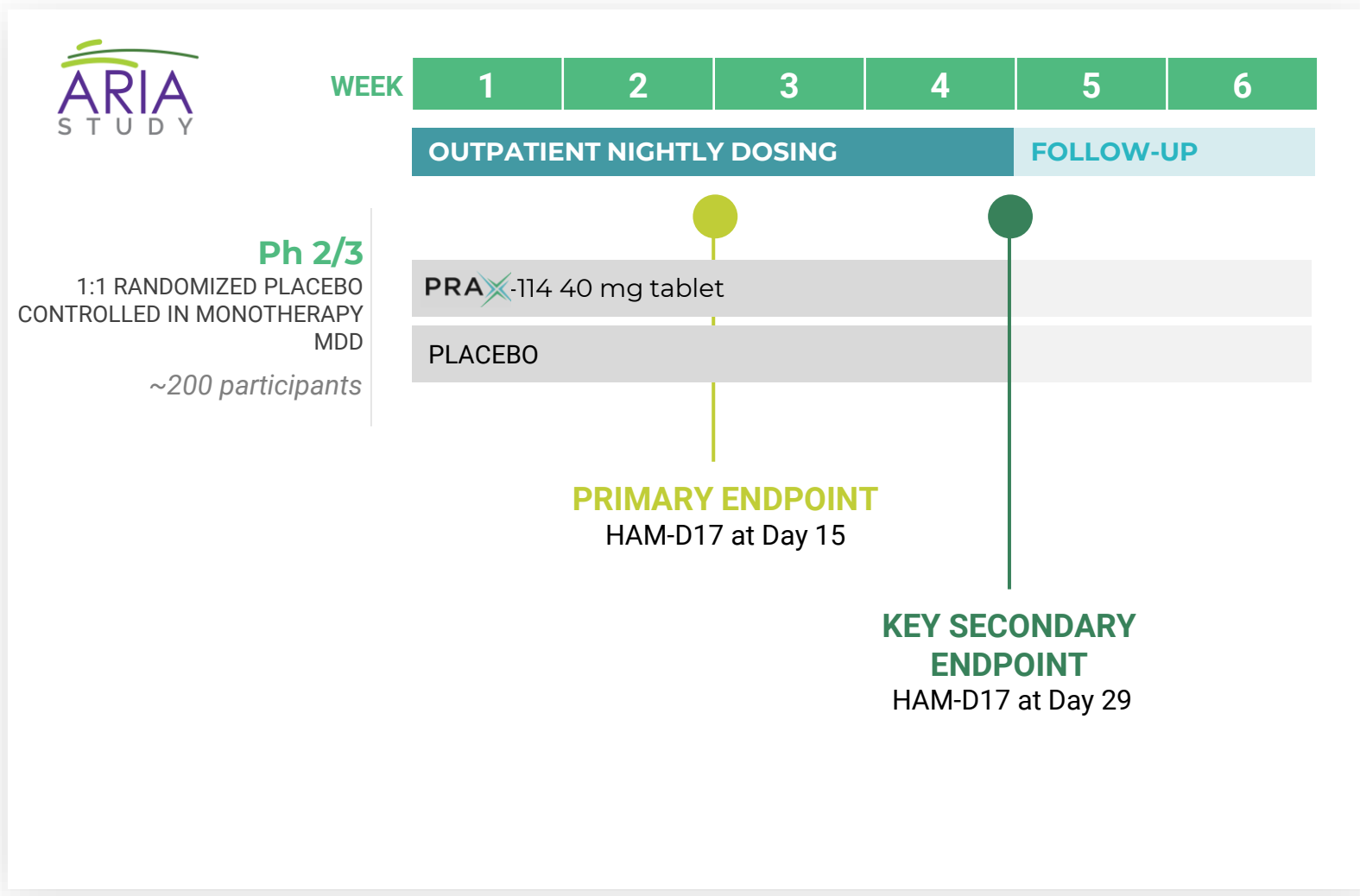
2H 2022

Ph 2 PTSD Topline

2H 2022

Ph 2 ET Topline

PRAX-114 monotherapy MDD Phase 2/3 Aria Study topline data expected 1H 2022



PHASE 2/3

First of two registrational trials for monotherapy MDD

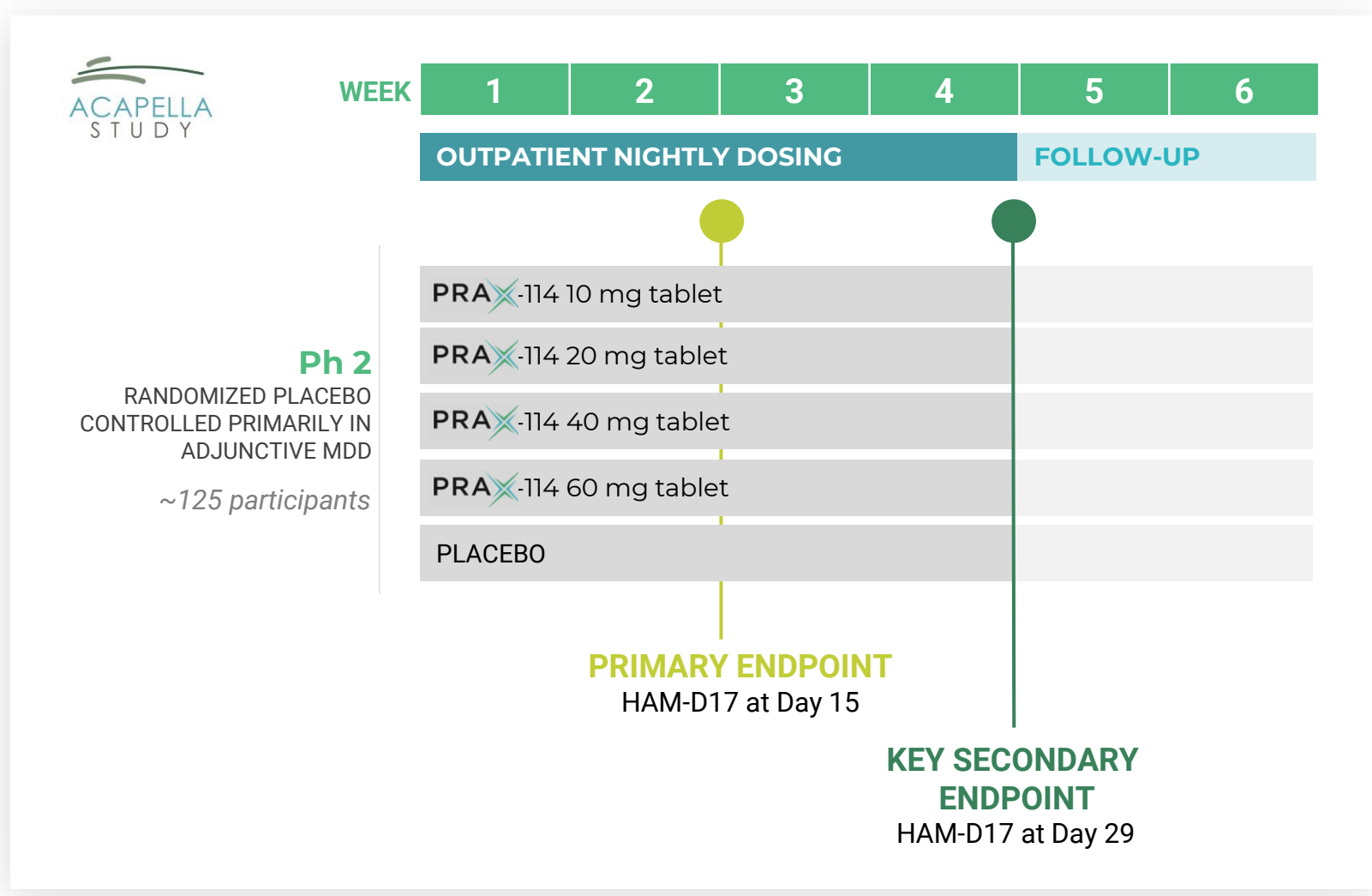
KEY INCLUSION CRITERIA

- Ages 18-65
- HAM-D17 \geq 23
- At least one prior episode of MDD

KEY EXCLUSION CRITERIA

- Treatment-resistant depression
- Current antidepressant treatment

PRAX-114 MDD Phase 2 Acapella Study topline data expected 1H 2022



PHASE 2

Dose-ranging study to evaluate safety and efficacy of PRAX-114 at doses of 10, 20, 40 and 60 mg

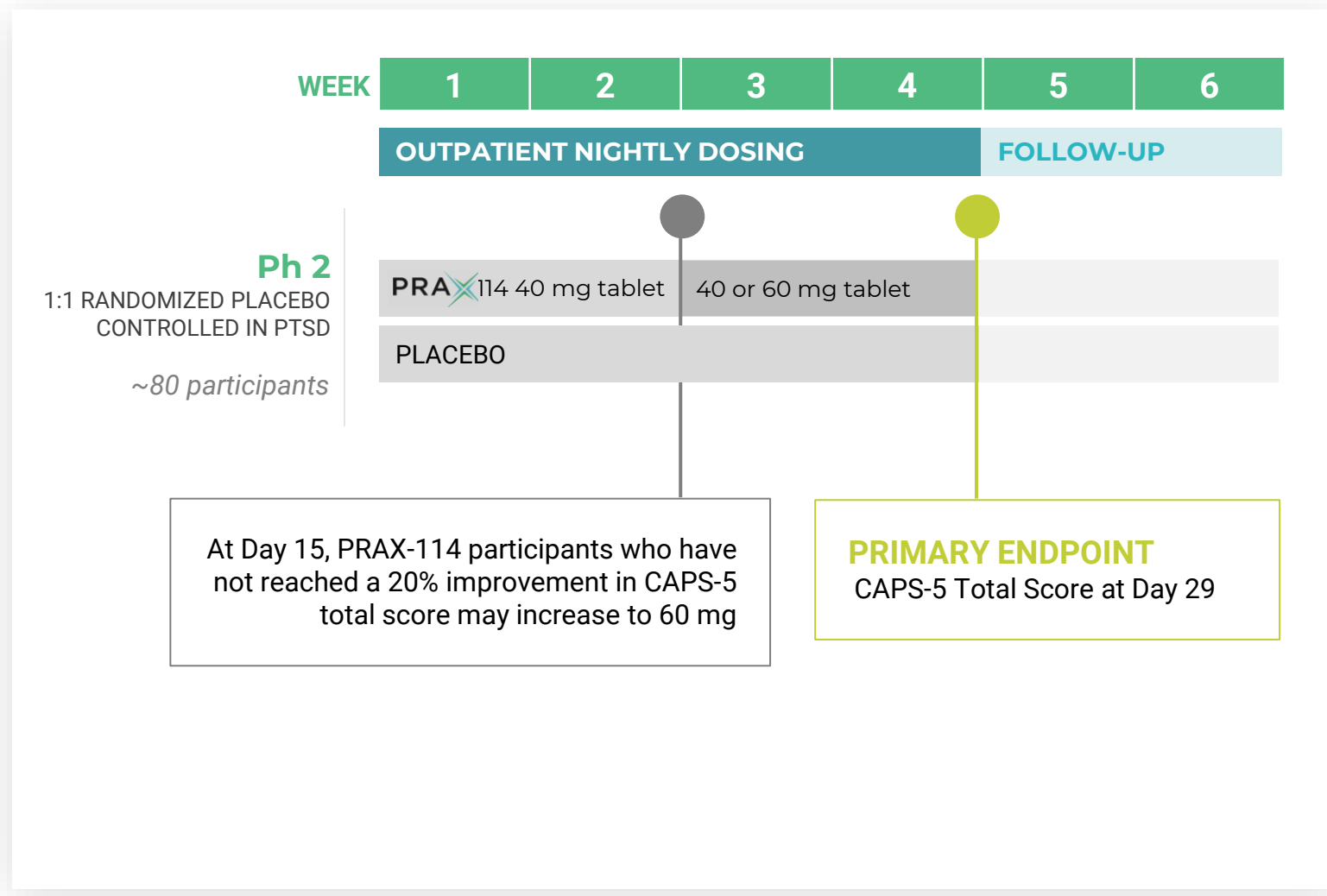
KEY INCLUSION CRITERIA

- Ages 18-65
- HAM-D17 \geq 23
- At least one prior episode of MDD
- Inadequate response to treatment in current episode of at least 12 weeks

KEY EXCLUSION CRITERIA

- Treatment-resistant depression

PRAX-114 PTSD Phase 2 study expected to initiate in 4Q21



TOPLINE DATA EXPECTED 2H22

To evaluate safety, tolerability and efficacy of PRAX-114 for treatment of adults with PTSD

KEY INCLUSION CRITERIA

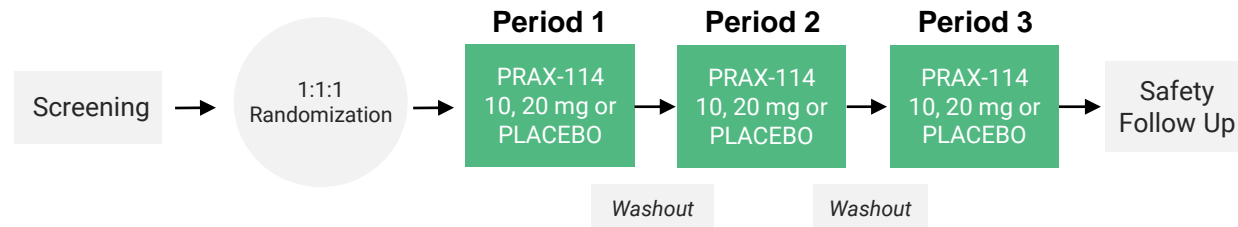
- Ages 18-65
- CAPS-5 ≥ 30
- PTSD diagnosis with duration of >6 months

PRAX-114 ET Phase 2 study expected to initiate in 4Q21

STUDY DESIGN

Randomized, double-blind, placebo-controlled, cross-over study

~15 participants



TOPLINE DATA EXPECTED 2H22

To evaluate safety, tolerability, PK and efficacy of daytime dosing of PRAX-114 for treatment of adults with ET

Participants will receive a single daily dose in each period followed by a washout between periods of at least 3 days

KEY INCLUSION CRITERIA

Ages 18 or older
Diagnosis of moderate to severe ET
TETRAS UL score ≥ 10

PRAX-944

T-Type calcium
channel inhibitor

MOVEMENT DISORDERS

Essential Tremor
Parkinson's Disease

KEY UPCOMING MILESTONES

Q4 2021

Ph2a ET High Dose Cohort Preliminary OL

1H 2022

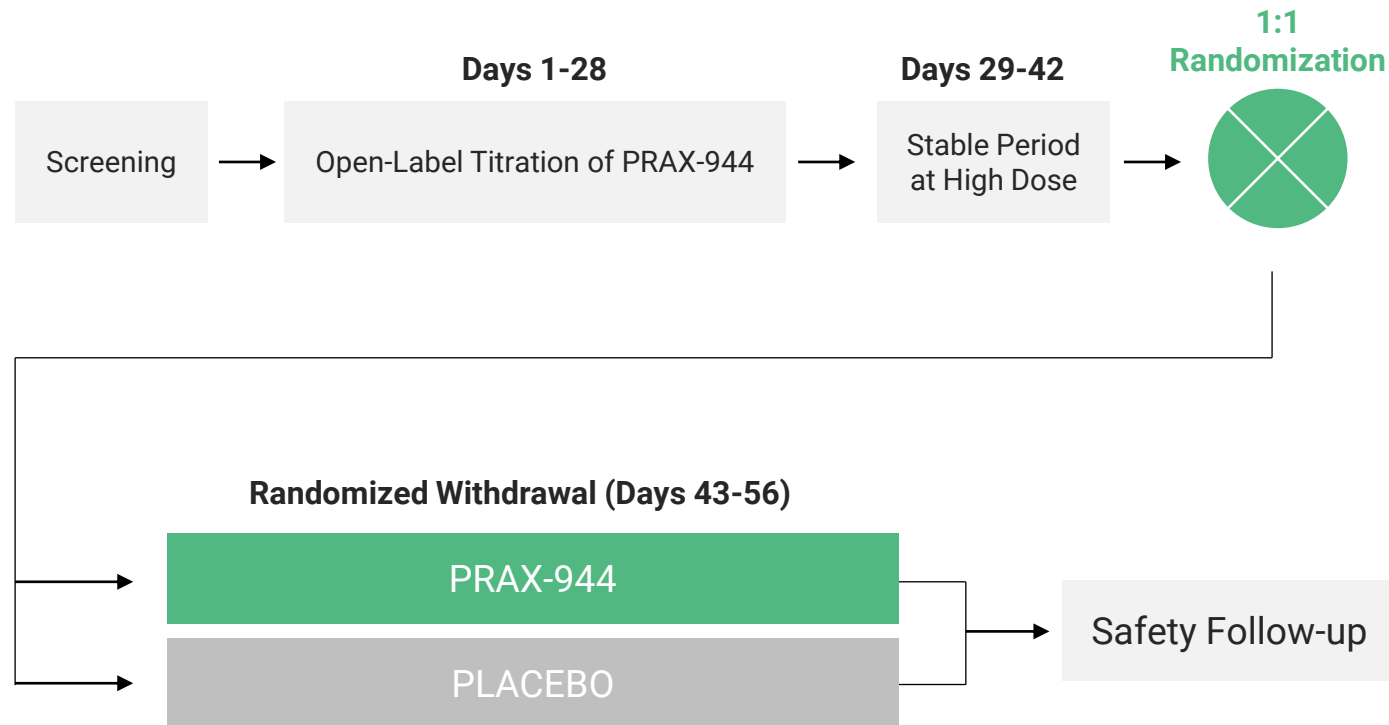
Initiate Ph2 PD Trial

2H 2022

Ph2b Essential1 Study Topline

PRAX-944 Phase 2a high dose cohort preliminary results expected in 4Q 2021

PART B: Open-Label Titration & Randomized Withdrawal Study Up to 120 mg



To evaluate safety, tolerability and efficacy of PRAX-944 in patients treated up to 120 mg per day

4Q21

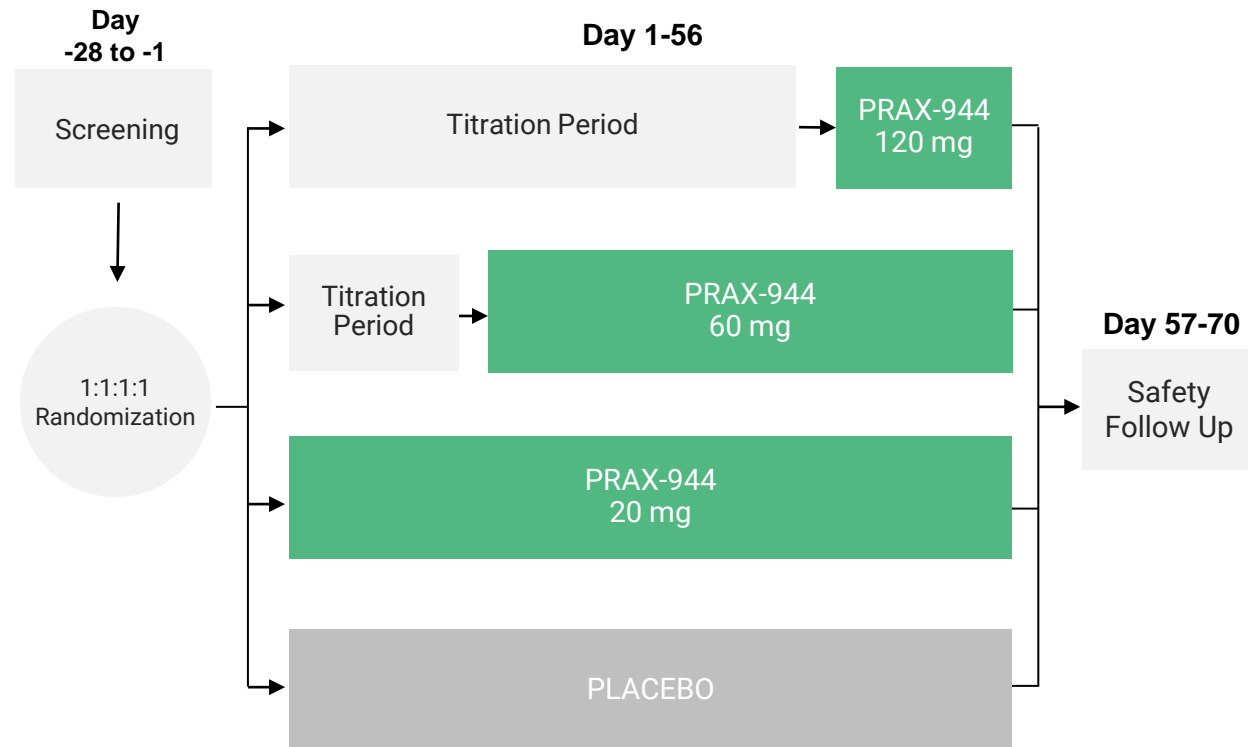
Preliminary open-label safety, tolerability and efficacy results

1H22

Complete open-label and placebo-controlled randomized withdrawal results

Enrollment has initiated for PRAX-944 ET Phase 2b Essential1 Study

Randomized, double-blind, placebo-controlled study in ~112 participants



TOPLINE DATA EXPECTED 2H22

Dose-ranging study to evaluate safety, tolerability and efficacy of PRAX-944 for treatment of adults with ET

KEY INCLUSION CRITERIA

Ages 18 or older
Diagnosis of ET for at least 3 years
TETRAS UL score ≥ 10

PRAX-562

Persistent Sodium
Channel Blocker

RARE DISEASES

Adult Cephalgias
Pediatric Epilepsies (DEEs)

KEY UPCOMING MILESTONES

Q4 2021

Initiate Ph 2 Adult Cephalgias Trial

1H 2022

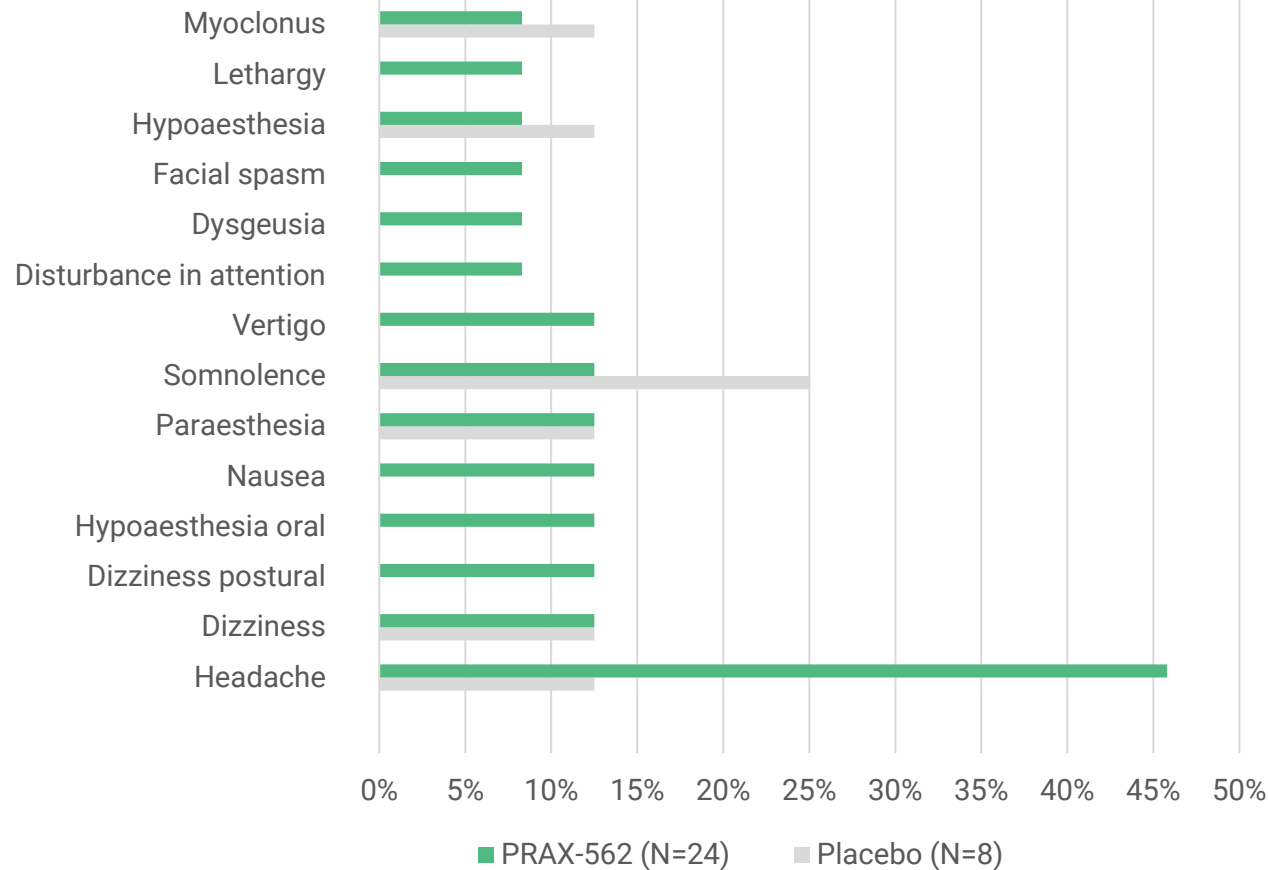
Topline Ph 1 ASSR Biomarker

1H 2022

Initiate Ph 2 DEE Trial

PRAX-562 well tolerated in Phase 1 healthy volunteer study

Percentage of treatment emergent adverse events (TEAEs) reported in ≥5% of PRAX-562 participants*



No clinically significant safety findings

All TEAEs mild to moderate in severity

No drug-related SAEs or severe AEs

PRAX-562 development strategy in rare cephalgias and pediatric epilepsies

OBJECTIVE

Identify PoC and safety in SUNCT/SUNA & Trigeminal Neuralgia while continuing efforts to expand to rare pediatric epilepsies

