

A stylized graphic of a human brain composed of a network of white dots connected by thin white lines, set against a light blue and green background. The brain is positioned on the right side of the slide, partially overlapping a white rectangular area.

Ovid Therapeutics Corporate Overview

JUNE 2021

(NASDAQ: OVID)

Disclaimers and Forward-Looking Statements

This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as “may,” “will,” “believe,” “expect,” “plan,” “anticipate” and similar expressions (as well as other words or expressions referencing future events or circumstances) are intended to identify forward-looking statements. Forward-looking statements contained in this presentation may include statements regarding the progress, timing, development of the Company’s product candidates and pipeline programs; scope of clinical trials and the reporting of clinical data; the potential clinical benefit of the Company’s product candidates and pipeline programs; regulatory development; the success of any licensing or partnering opportunities; the potential commercialization of product candidates and pipeline programs; the potential value of the 2021 royalty, license and termination agreement with Takeda; the success of Takeda’s trials in soticlestat and the potential commercialization of soticlestat and the Company’s expectations regarding its operating expenses, and use of its cash, cash equivalents and short-term investments to the development the Company’s pipeline and pursue business development opportunities. Each of these forward-looking statements involves risks and uncertainties.

These statements are based on the Company’s current expectations and projections made by management and are not guarantees of future performance. Therefore, actual events, outcomes and results may differ materially from what is expressed or forecast in such forward-looking statements. Factors that may cause actual results to differ materially from these forward-looking statements include the fact that initial data from clinical trials may not be indicative, and are not guarantees, of the final results of the clinical trials and are subject to the risk that one or more clinical outcomes may materially change as patient enrollment continues and or more patient data becomes available; Takeda’s ability to successfully complete clinical development of, obtain regulatory approval for and, if approved successfully commercialize Soticlestat; and uncertainties in the development and regulatory approval. process. Additional risks that could cause actual results to differ materially from those in the forward-looking statements are discussed in the Company’s filings with the U.S. Securities and Exchange Commission, including the "Risk Factors" sections contained therein. Such risks may be amplified by the COVID-19 pandemic and its potential impact on the Company’s business and the global economy. Except as otherwise required under federal securities laws, we do not have any intention or obligation to update or revise any forward-looking statements, whether as a result of new information, future events, changes in assumptions or otherwise.

The trademarks included in this presentation are the property of the owners thereof and are used for reference purposes only.

Clear Vision Enabled by a Strong Foundation

The Right Team, Experience and Track-Record

+









Strong Balance Sheet



Building the Next Major CNS Company

On a mission to build a unique company that delivers first-in-class therapeutics for rare disorders of the CNS

Pipeline Focused on Advancing Treatment of Rare Diseases

PRODUCT CANDIDATE	INDICATION / TARGET	RESEARCH	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	DEVELOPMENT / COMMERCIAL RESPONSIBILITY
Soticlestat CH24H inhibitor	Dravet Syndrome	Phase 3 Planned Initiation Mid-2021					
	Lennox-Gastaut Syndrome	Phase 3 Planned Initiation Mid-2021					
OV329 GABA aminotransferase inhibitor	Seizures Associated with Tuberous Sclerosis Complex and Infantile Spasms						
OV882 Short hairpin RNA therapy <i>Collaborator: UCONN</i>	Angelman Syndrome						
OV815 Gene modulation therapy <i>Collaborator: Columbia Univ.</i>	KIF1A and other non-disclosed targets						
OV825 Gene modulation therapy <i>Collaborator: Columbia Univ.</i>	HNRNPH2 PPP2R5D						
OV835 Gene modulation therapy <i>Collaborator: Columbia Univ.</i>	PPP2R5D						
 <p><i>Anticipate filing three INDs in three years, beginning 1H 2022</i></p>							

Up to \$856M+ Agreement with Takeda (soticlestat)

Potential Non-Dilutive Cash Stream to Fund Pipeline Development Zero Financial Obligations for Soticlestat Moving Forward

2021

Upfront Payment

- Received \$196M at closing (1Q '21)
- All financial obligations to Takeda are terminated

Est. 2023/2024

Regulatory Milestones

- Regulatory milestones
- Takeda initially funding two comprehensive pivotal trials (LGS and Dravet)

Est. 2024 and later

Commercial Milestones/Royalties

- Commercial sales milestones post approval
- Tiered double-digit royalties up to 20% on global soticlestat sales (all indications)

Up to \$660M in Combined Regulatory and Commercial Milestones



Captures significant value



Provides non-dilutive funding



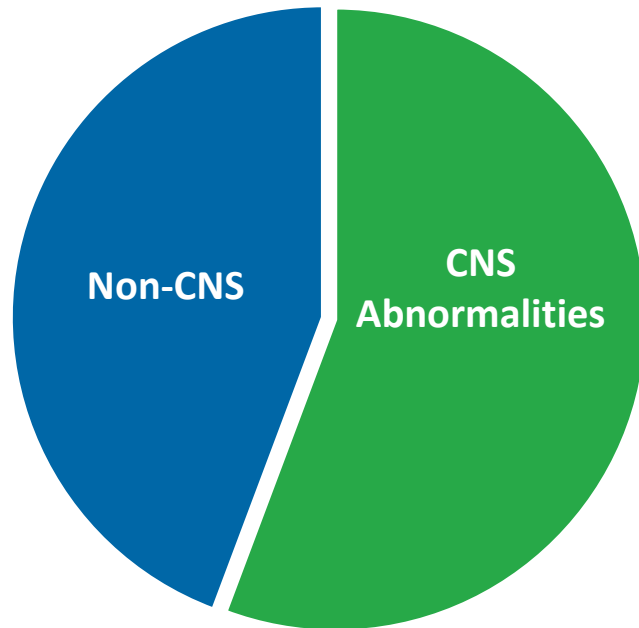
Enables investment in next-gen pipeline



Facilitates BD activities

Significant opportunity in CNS therapeutics

~7,000 RARE DISEASES



HISTORICAL CNS DRUG DEVELOPMENT CHALLENGES

- Incomplete understanding of disease biology
- Poor predictive value of animal models
- Lack of reliable biomarkers and difficult to measure endpoints
- Blood-brain-barrier preventing therapeutics from reaching the brain
- Patient population variability and need for large trials

Our rare disease expertise coupled with recent scientific advances are helping overcome historical CNS challenges

Source: Lee C. et al (2020); L.E.K. research and analysis

Recent major scientific advances are driving a new wave of CNS therapeutics

MULTIMODAL NEUROIMAGING HEADWAYS

More precise brain imaging enables better disease understanding and patient outcomes:

- Supported discovery of Parkinson's subtypes
- Earlier identification of high-risk TIA patients
- Biomarkers to better measure therapeutic efficacy

APPROACHES TO INTERACT WITH DNA / RNA

Recent successes in genetic medicine paves the path for next-gen therapies:

- Next-gen DNA and RNA editing tech
- Immune system modulation
- Understanding of functional genomics

BBB CROSSING ENABLED

Advances in BBB-crossing approaches can drive future growth of neuroscience therapeutics:






- Novel delivery approaches
- Targeting of therapies to specific cell types
- Decrease the time and risk associated with new CNS directed therapies

Advance Next-Generation Neuroscience Pipeline

STRATEGY

- Focus on ecosystem of rare neurological diseases with **high unmet need**
- Develop novel **first-in-class / best-in-class** therapeutics
- Create a **customized disease approach** leveraging deep understanding of underlying pathology
- Collaborate with the **patient community, KOLs**, and other **external innovators** to accelerate development

PROGRAMS

PRODUCT CANDIDATE	MOA	LEAD INDICATION / TARGET	RESEARCH	PRECLINICAL
OV329	GABA aminotransferase inhibitor	Seizures Associated with Tuberous Sclerosis Complex and Infantile Spasms		
OV882	Short hairpin RNA therapy	Angelman Syndrome		
OV815	Genetic/molecular approach	KIF1A Associated Neurological Disorders (KAND)		
OV825	Gene modulation therapy	HNRNPH2		
OV835	Gene modulation therapy	PPP2R5D		

Anticipate filing three INDs in three years, beginning 1H 2022

OV329: Highly Potent Inhibitor of GABA Aminotransferase

Asset overview

OV329

- **Mechanism:** Highly potent GABA aminotransferase (GABA-AT) oral small molecule inhibitor
- **Development status:** IND enabling studies are underway, IND expected 1H 2022

Indication(s) overview

Refractory epilepsies

Tuberous sclerosis complex

- Affects 1 in 6K individuals (~50K patients in U.S.); epilepsy present in ~85% of TSC patients
- Current treatment options include vigabatrin, everolimus, and surgery
- **Significant unmet need:** Most patients resistant to current therapy

Infantile spasms

- 2-3.5 cases per 10K births in U.S.
- Current treatment options include ACTH and vigabatrin
- **Significant unmet need:** Significant side effects associated with standard of care

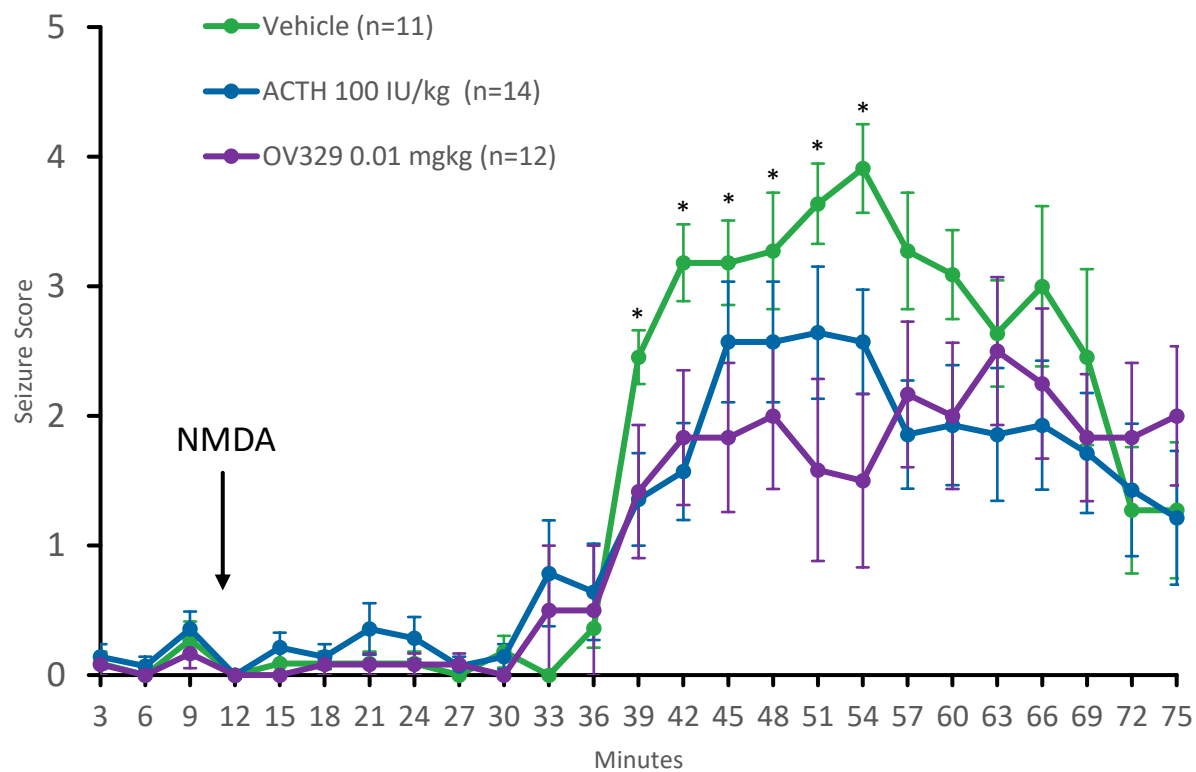
Opportunity and OV329

- **Opportunity:** Create a superior product based on a validated mechanism
- **OV329:** Potential best-in-class

* Source: Tuberous Sclerosis Alliance; Pellock JM, et al. Epilepsia (2010)

OV329 Appears Active in Models of Drug-Resistant Seizures

Orally delivered OV329 as effective as injectable SOC in infantile spasms model



Model described by Shi *et al.*, 2014 (PMID 26600368)

OV882: Potential Disease-Modifying Genetic Therapy for Angelman Syndrome (AS)

Asset overview

OV882

- **Mechanism:** Short hairpin RNA that interacts with non-coding RNA to inhibit the silencing of paternal UBE3A gene
- **Development status:** POC* activity confirmed in vitro; currently undergoing pre-clinical validation
- In collaboration with Connecticut Autism Language Lab under Associate Professor Stormy Chamberlain



Indication overview

Angelman syndrome

- Affects 1 in 15K individuals
- Characterized by developmental delay, ataxia, sleep disorder, seizures, and speech impairments
- Current treatment options are symptomatic (e.g., anti-seizure)
- **Significant unmet need:** No specific treatments available which target the neuropathophysiology of Angelman syndrome
 - ASOs** are being investigated by others; approach may have challenges

Opportunity and OV882

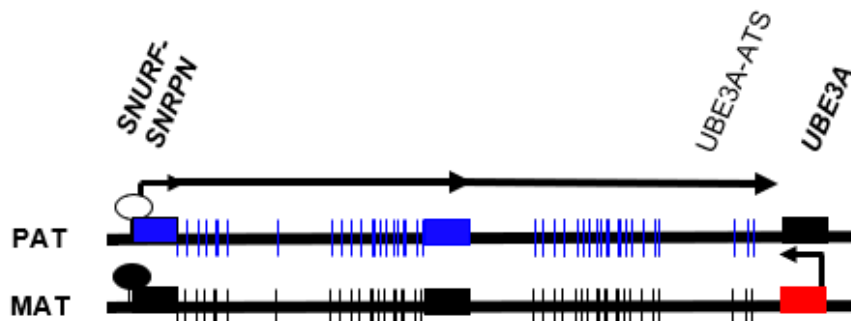
- **Opportunity:** Create potential disease modifying treatment for high unmet need indication
- **OV882:** Targets the mechanism of silencing without affecting the gene, minimizes off-target effects, and potentially increases treatment duration compared to ASOs

Source: Foundation for Angelman Syndrome Therapeutics (FAST)
* Proof of Concept (POC), ** Antisense oligonucleotides

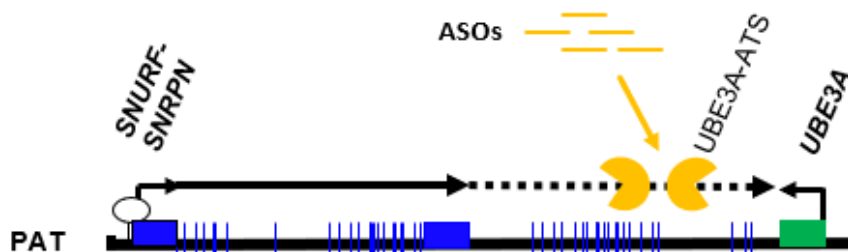
OV882 Approach to the Treatment of Angelman Syndrome

RNAI APPROACHES TO TREATING ANGELMAN SYNDROME

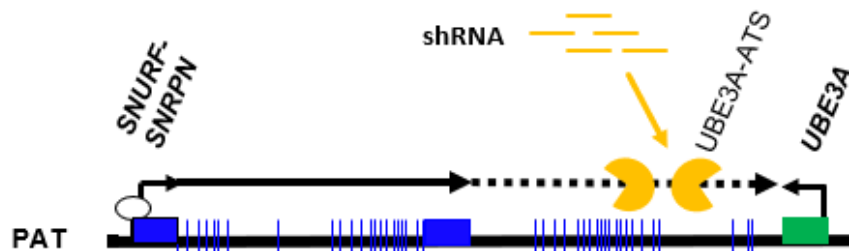
DISEASE STATE



ASO APPROACH



OV882 shRNA APPROACH

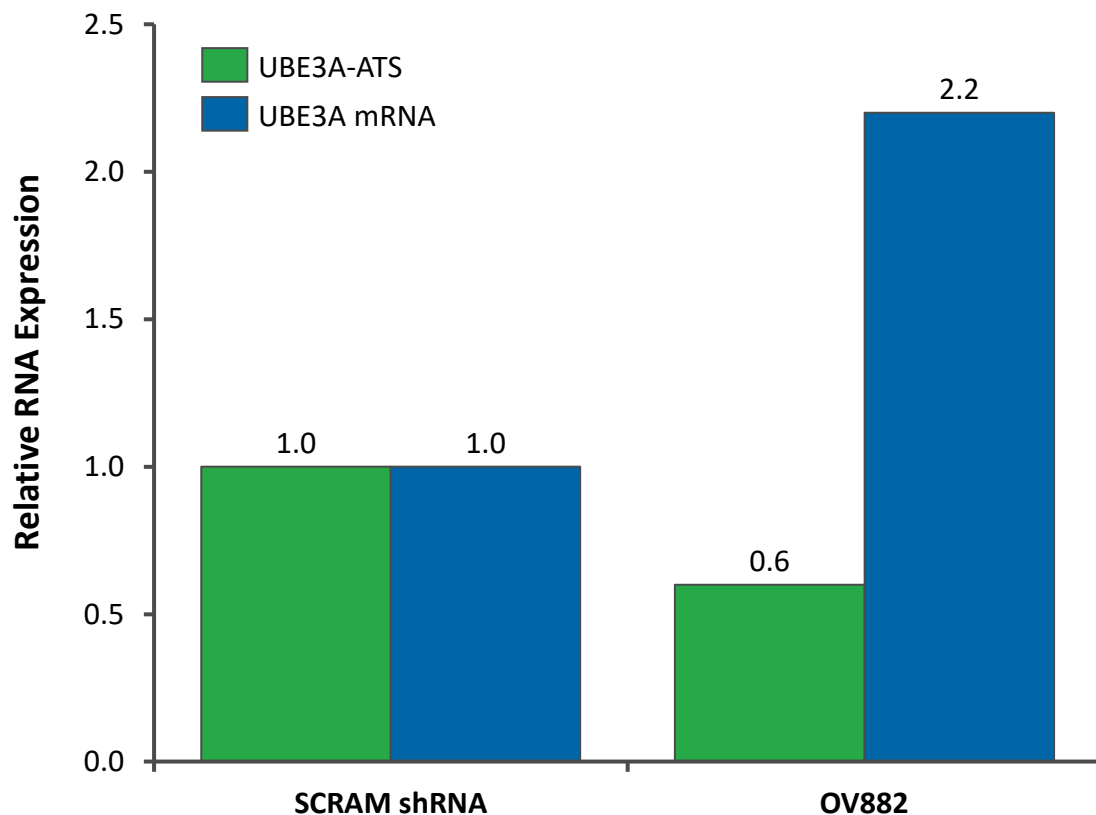


DESCRIPTION OF APPROACH BENEFITS AND DRAWBACKS

- Angelman syndrome is caused by a mutation in the maternal copy of the UBE3A gene and silencing of the paternal copy
 - Silencing is mediated by a non-coding RNA sequence whose expression blocks transcription of the paternal UBE3A gene
-
- Mechanism may cause undesirable off-target effects
 - Requires redosing on approximately quarterly timescale
 - Requires chemical modification of ASO
-
- + Exclusively silences UBE3A-ATS and unsilences UBE3A
 - + Minimizes off-target effects
 - + Potential for longer lasting effects

OV882 Appears Active in AS Neuron Cell Model

shRNA-OV882 Effects on UBE3A-Antisense and UBE3A Expression (iPSC-AS neurons)



OV882 demonstrates activity in AS neuronal cell system:

- **>2x increase in UBE3A** mRNA expression when compared to SCRAM control
- Reduction in UBE3A-ATS expression **further demonstrates** the potential mechanism and efficacy of OV882

OV815: Potential Advanced Genetic Therapy for KAND and Other KIF-associated Diseases

Asset overview

OV815

- **Mechanism:** Genetic/molecular approach targeting KIF1A
- **Development status:** Currently in screening stage for aptamer and gene silencing technologies
- In collaboration with



Indication overview

KAND*

- ~200** patients worldwide with documented diagnoses; total number of affected patients likely in the thousands
- Broader kinesin superfamily opportunity
- Symptoms associated with KAND include hereditary spastic paraplegia, ataxia, epilepsy, hypotonia, autism, and ADHD
- Current treatment options are symptomatic
- **Significant unmet need:** No specific treatments available

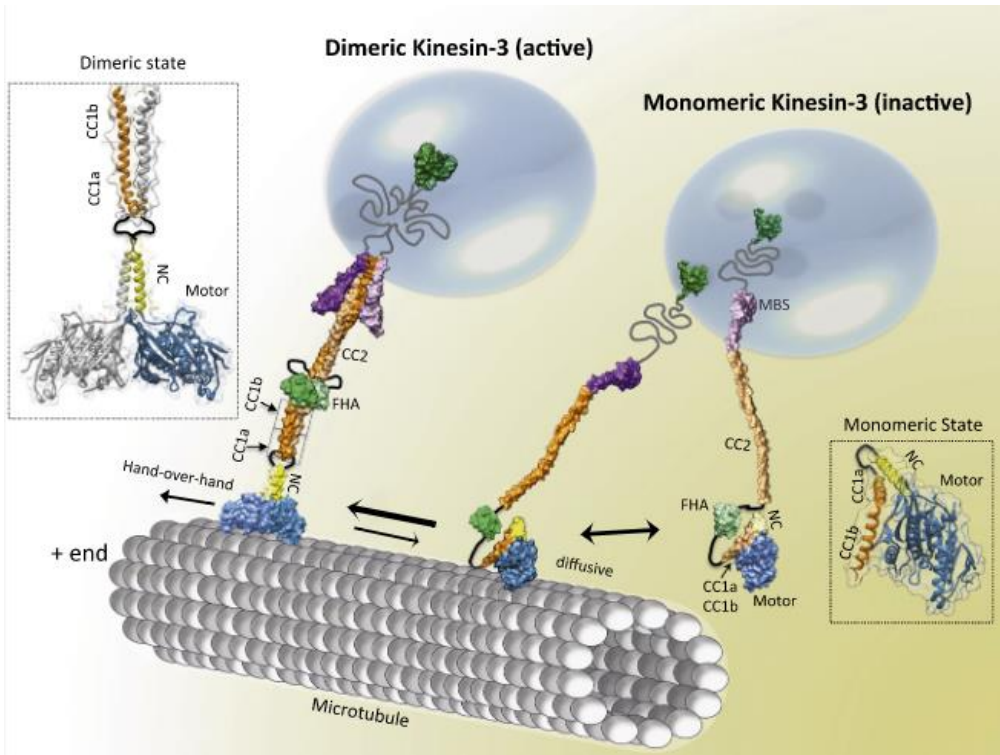
Opportunity and OV815

- **Opportunity:** Leverage knowledge gained from KIF1A to access the broader kinesin superfamily associated diseases

Notes: * KIF1A-Associated Neurological Disorder
Source: **KIF1A.org

OV815 Has Potential For Broader Applicability Within the Kinesin Superfamily

KIF1A (Kinesin 3 Family Member)



Source: Al-Bassam_2018_Malleable folding of coiled-coils regulates

Impact of KIF1A on neurotransmission

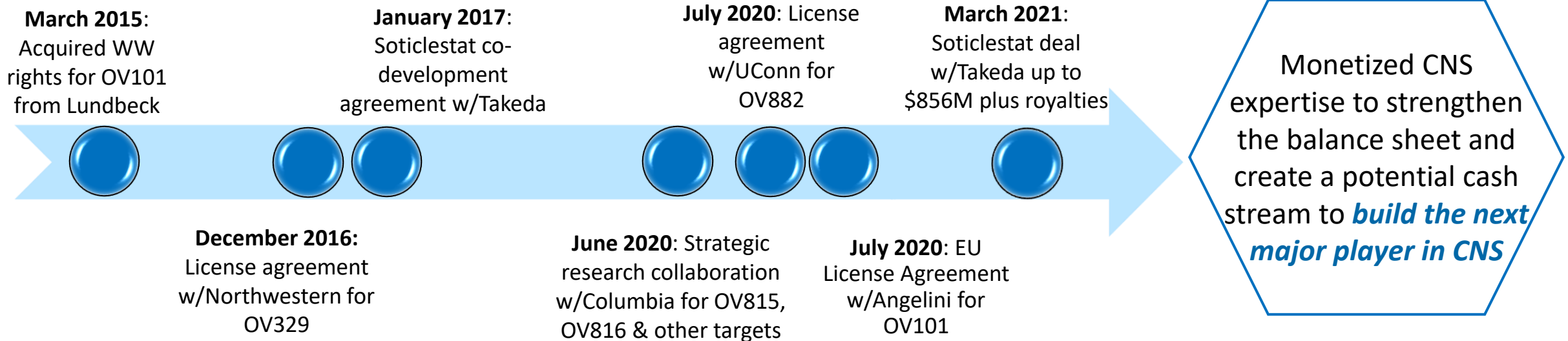
- KIF1A is a motor protein that transports cargo for neurons
- **Disruption of cargo transport impacts neurotransmission and leads to progressive neurologic deficits**

Initial opportunity

KAND

Additional opportunities in kinesin superfamily

Proven Business Development Strategy



Successful track record of identifying promising targets in hard-to-treat diseases

Targeting CNS Technology and Therapeutic Approaches

Technologies



CNS therapeutic enabling

- Supports company therapeutic area focus on genetic medicine



Blood-brain barrier assays / delivery systems

- BBB assays: assess penetration, evaluate targeting, and test in vitro
- Delivery systems: minimize immunogenicity, enable precision targeting, and address manufacturing issues



Proprietary and differentiated

- Strong IP position
- Unique delivery system or differentiated technology

Clinical Stage Targets



Neuroscience

- Rare neurological, neurometabolic, ophthalmic, seizure related, other CNS disorders



Actionable assets near IND or later

- Complementary to existing pipeline and strategy
- Leverage core capabilities in rare CNS diseases



Best- or first-in-class therapies

- Disease modifying therapy / potential to establish new standard of care

Strong Balance Sheet Supports Strategy

- **Strong balance sheet:**
 - **\$233M** in cash and cash equivalents as of 3/31/21
 - No further financial obligations for the development of soticlestat, significant downstream economics if soticlestat is approved
- **Expected 2Q 2021 - 4Q 2021 quarterly Op Ex of \$8M-\$10M¹**
- **69M shares of common stock outstanding²**

¹excluding non-cash and non-recurring expenses

²as of 5/6/21, on an as if converted basis

Leadership Team With Required Track Record



Jeremy Levin

DPhil, MB Bchir
Chairman, CEO



Amit Rakhit

MD, MBA
President, CMO



Jason Tardio

MBA
Chief Commercial Officer



Jeffrey Rona

Chief Business & Financial Officer



Thomas Perone

JD, MBA
GC, Corporate Secretary, and CCO



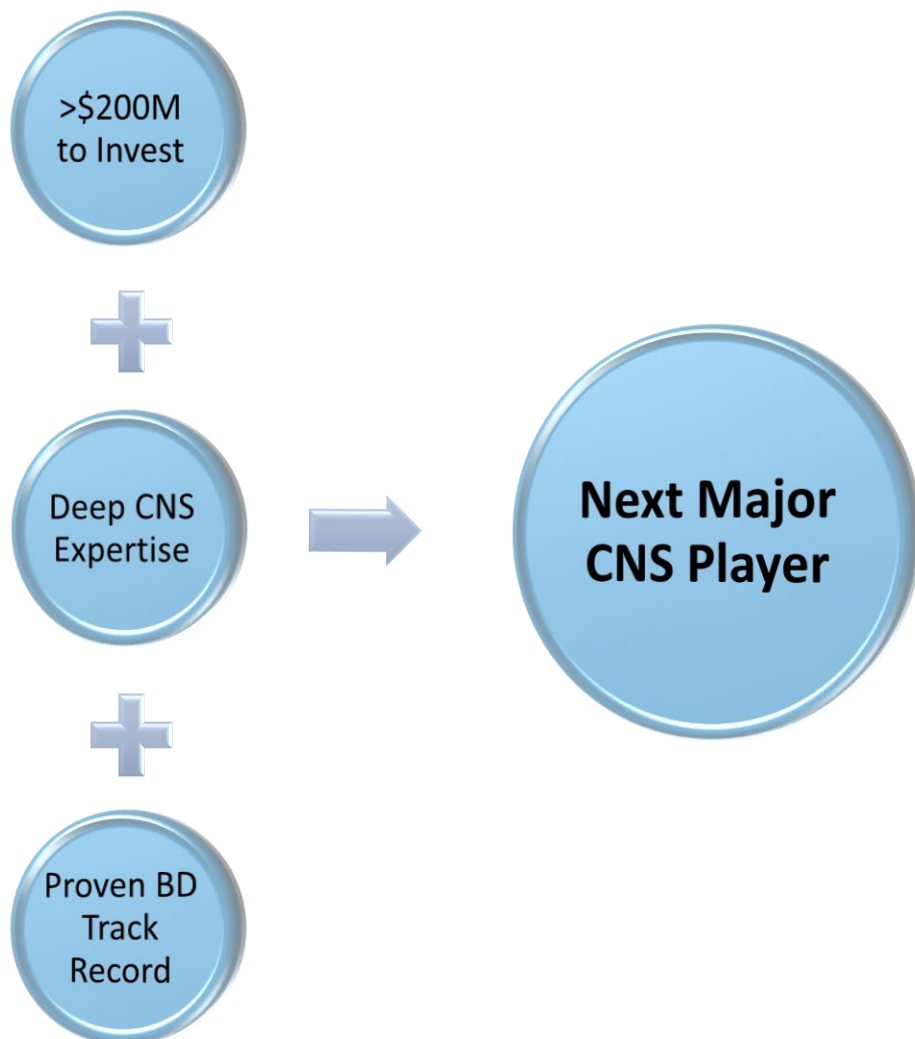
Claude Nicaise

MD
Head, Rare Disease Strategy

Deep experience across R&D continuum, BD, IND filings, approvals and launches



The Ingredients to Become a Major Player in Next CNS Wave



Prepared and capitalized to be a major player in the next CNS wave

- Tackle, hard to treat CNS diseases avoided by others
- Use technology to achieve a fundamental change
- Leverage strong academic relationships
- Invest smart and conduct R&D efficiently



Thank you

JUNE 2021

(NASDAQ: OVID)