Pioneering precision medicine in epigenetics

ORYZON

CORPORATE PRESENTATION

Sept-2021

ORY:SM / ORY.MC

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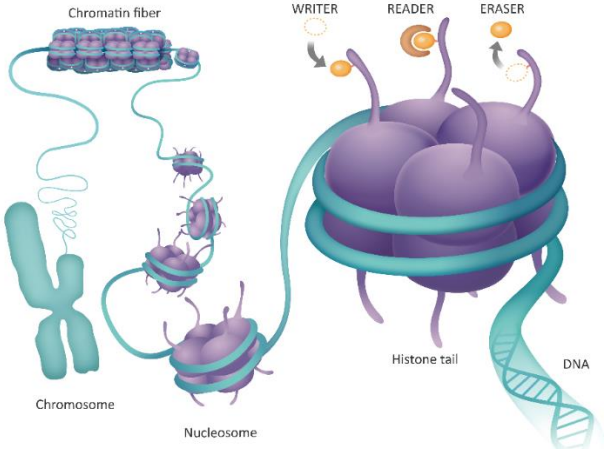
Oryzon: Epigenetic Champion Developing New Therapies in Oncology and CNS



Histones are modified by epigenetic enzymes resulting on the unfolding or folding of chromatin and allowing or silencing gene expression

- Leading & growing epigenetic platform with an expanding pipeline
- Developing highly potent and selective LSD1 inhibitors with best-in-class potential
- Two programs with positive Phase IIa data in AML and SCLC, and in aggression/agitation in CNS disorders
- Potential for expedited development in 2L AML and 1L ED-SCLC, and Kabuki Syndrome

Potentially Best-in-Class LSD1i Addressing Unmet Medical Needs in Oncology and CNS

ONCOLOGY

Iadademstat (ORY-1001)
MoA: Pro-Differentiation
Anti-cancer stemness

- Potential first & best-in-class LSD1i in Oncology
- Initially developed for AML and SCLC
- 4 Phase I/IIa clinical trials: 100+ patients dosed
- Robust and consistent responses in AML (80%+ ORRs)
- Two new Phase Ib/II trials with registrational potential
  - FRIDA, a study in 2L in AML
  - STELLAR, a study in 1L in ED-SCLC

CNS

Vafidemstat (ORY-2001)
MoA: Prosynaptic
Anti-neuroinflammatory

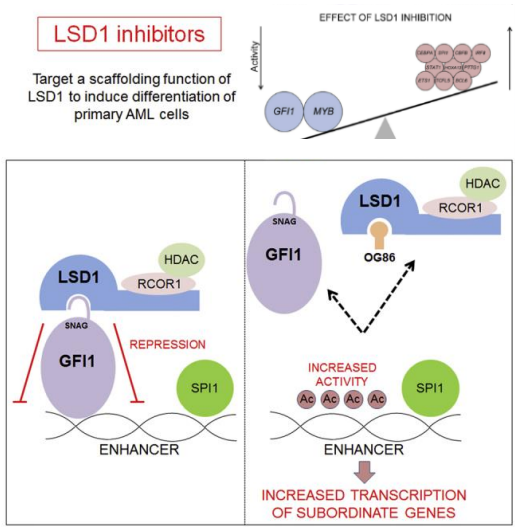
- Potential first-in-class LSD1i in CNS
- 8 Phase I/II clinical trials: 300+ subjects dosed
- Positive Phase IIa data in agitation and aggression in a basket trial in multiple psychiatric disorders
- Two new ongoing Phase IIb studies in 2021 in BPD and SCZ
- New HOPE Phase I/II trial in Kabuki syndrome has potential to support registration

Deep Pipeline with Multiple Upcoming Milestones

INDICATION	STUDY	PRECLINICAL / PHASE I / PHASE IIA / PHASE IIB / PHASE III	Next anticipated milestones
IADADEMSTAT (ORY-1001) - selective LSD1 inhibitor			
AML (Elderly Unfit)	ALICE (Combo w azacitidine)	ongoing (fully enrolled)	ASH2021 /EHA2022 updates ASH2022 Final Data
AML (2L)	FRIDA (Combo w gilteritinib)	in preparation	IND 2H2021 /FPI 1H2022 Data updates 2022
ED-SCLC (1L)	STELLAR (Combo w ICI)	in preparation	IND 1H2022 /FPI 1H2022 Data updates 2022
NETs (2L)	NET Basket (Combo w other agents)	in preparation	IND 1H2022 /FPI 1H2022 Data updates 2022
VAFIDEMSTAT (ORY-2001) - CNS optimized LSD1 inhibitor			
Borderline Personality Disorder	PORTICO	recruiting	US-FPI2021 Data updates 2022
Schizophrenia (Negative symptoms & Cognition)	EVOLUTION	recruiting	EU-FPI2021 Data updates 2022
Kabuki Syndrome	HOPE	in preparation	IND 1H2022 /FPI 1H2022 Data updates 2022
SetD1A Compass related SCZ		in preparation	IND 1H2022 / FPI2022
OTHER PROGRAMS			
COVID-19 Prevention of ARDS	ESCAPE	study finalized	ECCMID2021
Aggression in AD	New Study (Continuation of REIMAGINE-AD)	under study	

IADADEMSTAT
A Phase II LSD1 inhibitor in Oncology

Iadademstat: Potentially Best-in-Class Small Molecule LSD1 Inhibitor for Oncology



In leukemia, LSD1 is a building-block of some multiprotein complexes that impairs normal differentiation. LSD1 inhibition collapses this complex and allows blasts to continue normal differentiation

LSD1 plays a key role in numerous cancers; elevated levels often correlate with more aggressive forms or poor prognosis

Iadademstat is a potent and highly selective, investigational oral LSD1i with best-in-class potential

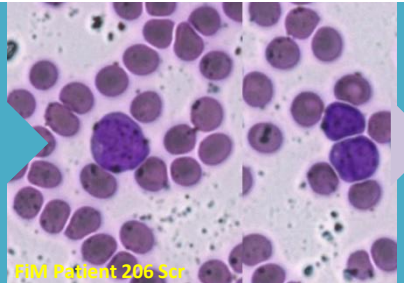
Encouraging signals of clinical efficacy in Phase IIa trials in elderly/unfit AML patients and in ED-SCLC

Advancing Phase Ib/II trials with registrational potential in 2L AML and 1L ED-SCLC; initiation in 1H 2022

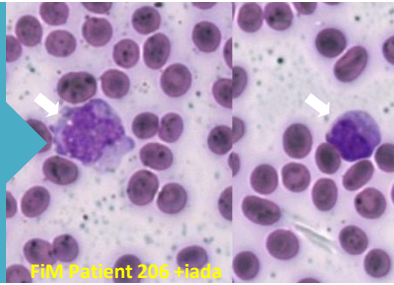
Iadademstat MoA and Antileukemic Activity Observed in AML

AML is the most common type of acute leukemia in adults

LSD1 interacts with GFI1/1B causing a block in leukemia cell differentiation



LSD1 inhibition modulates the GFI1/1B axis to induce terminal differentiation and control leukemia stem cell proliferation



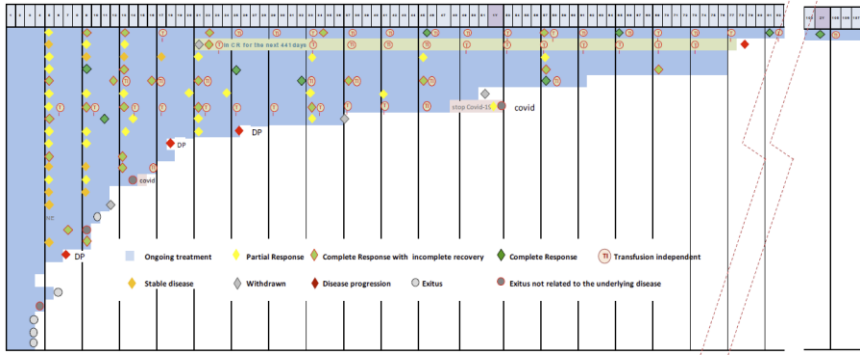
- LSD1 is **crucial for the function and maintenance of leukemic stem cells**, a subset of malignant cells believed to be the underlying cause for relapses
- Iadademstat **reduces leukemic stem cell capacity** and proliferation and **induces leukemic blast differentiation**
- Iadademstat has shown preclinical efficacy and safety in combination with standard of care in AML

- Clinical Status:
  - Completed FiM Phase I (41 patients) → Generally well tolerated with antileukemic activity (1CRi) and with reductions in blood and bone marrow blast percentages and induction of blast differentiation
  - Ongoing Phase IIa (ALICE) (36 patients) → Encouraging signals of clinical activity (83% ORR) and generally well tolerated
    - Final data anticipated at ASH 2022

Iadademstat AML Phase IIa ALICE Trial Overview

Trial Design / Overview

- Open label, multicenter, single arm study
- Newly diagnosed elderly or unfit AML patients
- Iadademstat in combination with azacitidine
- 36 patients (fully enrolled)
- Preliminary data presented at EHA 2021 with 18 evaluable patients



Evolution of Trial Efficacy / Patient Responses

Results Highlights

- **83% ORR** (15 out of 18 evaluable patients):
  - **67% CR/CRi** (10/18): 5 CRs, of which 3 are MRD negative, and 5 CRi (3 still evolving); 5 PRs
- Median Time to Response (TTR) of 29 days
- **Lasting responses**:
  - **6 out of the 10 CR/CRi patients (60%) had lasting responses (≥ 6 months)**
  - 5 patients with responses > 1 year
  - Longest EFS response of 2+ years
- 50% transfusion independence in CR/CRi patients
- The combo was generally well tolerated
  - No QTc prolongation; no neuronal, hepatic, renal or any other organ toxicity

Public report presented at (data correspond to May 24th cut off)

EHA2021 VIRTUAL

https://www.oryzon.com/sites/default/files/events/20210611_EHA2021_poster_1.pdf

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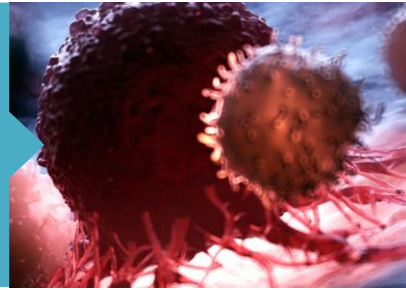
Iadademstat is a Therapeutic Approach for SCLC With a Well-Defined MoA

SCLC is very aggressive and represents 15% of all lung cancers

LSD1i acts in SCLC by enhancing Hes1 and Notch1 pathways

LSD1i synergizes with ICIs and boosts the host immune system



- Iadademstat produces **complete and durable tumor regression** in different **chemoresistant SCLC PDX models**
- Preclinically, Iadademstat is **efficacious in combos with platinum/etoposide and other agents such as Immune Checkpoint Inhibitors (ICIs)**
- Oryzon has identified and patented **biomarkers** that differentiate SCLC tumors by their sensitiveness to LSD1i

- Clinical Status:
  - Phase I study (NCT02913443) (18 patients) → RP2D in monotherapy
  - Phase IIa (CLEPSIDRA) (14 patients) → Signs of clinical activity & Generally well tolerated in monotherapy

Iadademstat SCLC Phase IIa CLEPSIDRA Trial Overview

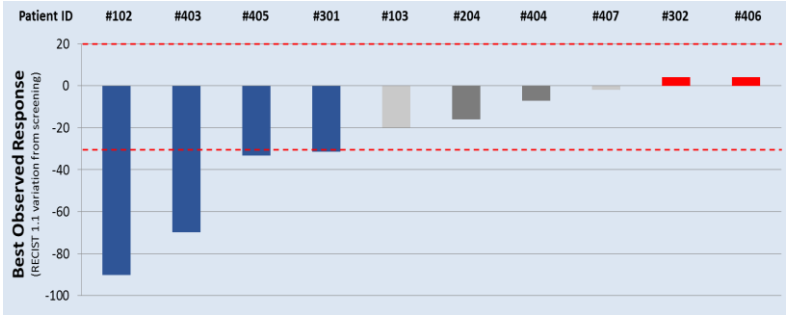
Trial Design / Overview

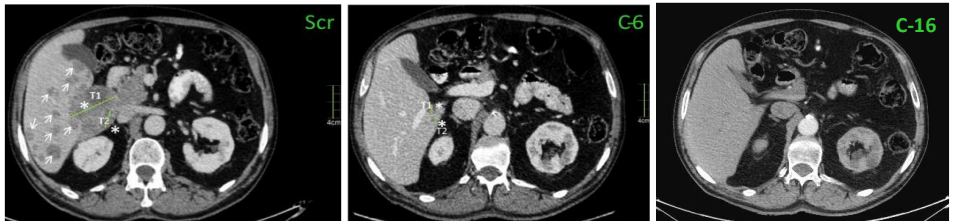
- Open label, multicenter, single-arm study
- Biomarker selected, platinum sensitive 2L ED-SCLC patients
- Iadademstat in combination with 4-6 cycles carboplatin/etoposide (21 d/cycle). After chemo, iada could be administered alone
- 14 patients enrolled; 12 biomarker-positive patients; 10 evaluable patients
- Data presented at ESMO 2020

Results Highlights

- Platinum/etoposide in combo with iadademstat showed hematotoxicity
- Iada alone was generally well tolerated. Toxicity disappeared when treated with iada alone
- Activity signals were encouraging with **40% OR** and **mean DoR of 4.5 months** compared to SoC 15-35% OR
- **60% clinical benefit rate** (6/10 evaluable patients): 4PRs + 2 long-term SD
- One patient had 15 cycles in monotherapy, with a total tumor size reduction of 90% and a relative tumor size reduction during iadademstat monotherapy of 53%

2020 ESMO Conference Data Release





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Orphan Drug Designation granted for AML in US and EU

- **Registrational strategy:** Potential for expedited development in AML and SCLC in two indications
  - 2L in AML with FLT3 mutations
  - 1L in ED-SCLC

  } *Patient populations with high medical need & Significant market opportunity*

- Additional trial in NETs (collaborative) in prep and other trials in study

Next Steps: Phase Ib/II FRIDA 2L AML Trial with registrational potential

FRIDA, a Phase Ib/II in FLT3 mut+ R/R AML patients combining iadademstat and gilteritinib

FRIDA: FLT3 mutated Relapsed/Refractory AML treatment with IaDAdemstat

- 2L AML is an underserved population: 50% of AML patients relapse
- FLT3 is the most common mutation in AML (30%) and patients have adverse prognosis. 2L R/R FLT3 mutated patients are now treated with gilteritinib, yet it remains a subpopulation with high medical need
- Strong rationale: preclinical synergies between iadademstat and gilteritinib
- Open label, multicenter (around 20 sites in US). 120 patients to be included
- Phase Ib objectives are to evaluate safety/tolerability, and to determine the RP2D and MTD
- Phase II objective is to evaluate efficacy of the drug at the RP2D
- IND submission 2H2021 / FPI 1H2022

FRIDA study could potentially support an application for accelerated approval if a significant clinical benefit in the population is demonstrated over the efficacy of gilteritinib monotherapy as determined by a matched contemporary synthetic control study

Next Steps: Phase Ib/II STELLAR 1L ED-SCLC Trial with registrational potential

STELLAR, a randomized Phase Ib/II study of iadademstat plus a checkpoint inhibitor in patients with treatment naïve metastatic small cell lung carcinoma

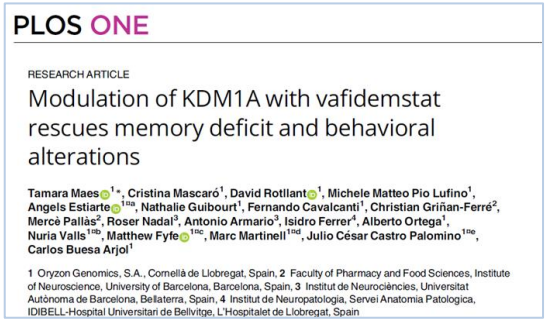
STELLAR: Synergistic Treatment with Epigenetics in front line small celL Lung cAnceR

- High unmet medical need and a relative low bar for improving efficacy due to the modest efficacy improvements shown in the IMPower-133, CASPIAN and Keynote-604 trials
- Strong rationale for combination: preclinical proof of strong synergies between iadademstat and ICI
- Randomized, multicenter (15-20 sites in US). 120 patients to be included
- Phase Ib objectives are to evaluate safety/tolerability, and to determine the RP2D and MTD in combination with ICIs
- Phase II objective is to evaluate efficacy of the drug measured as PFS in randomized trial against an ICI-only arm
- IND submission 1H2022 / FPI 1H2022

STELLAR can potentially support an application for approval if a significant clinical benefit in the population is demonstrated over the efficacy of ICI+CbEt

VAFIDEMSTAT
A Phase II LSD1 inhibitor for CNS diseases

Vafidemstat: “Neuron-resetting” LSD1 Inhibitor in Phase II for Multifactorial and Monogenic CNS Disorders



PLOS ONE

RESEARCH ARTICLE

Modulation of KDM1A with vafidemstat rescues memory deficit and behavioral alterations

Tamara Maes[1]*, Cristina Mascaró[1], David Rotllant[1], Michele Matteo Pio Lufino[1], Angels Estiarte[1ne], Nathalie Guibourt[1], Fernando Cavalcanti[1], Christian Griñan-Ferré[2], Mercè Pallàs[2], Roser Nadal[3], Antonio Armario[3], Isidro Ferrer[4], Alberto Ortega[1], Nuria Valls[1ne], Matthew Fyfe[1nc], Marc Martinell[1nd], Julio César Castro Palomino[1ne], Carlos Buesa Arjol[1]

1 Oryzon Genomics, S.A., Cornellà de Llobregat, Spain, 2 Faculty of Pharmacy and Food Sciences, Institute of Neuroscience, University of Barcelona, Barcelona, Spain, 3 Institut de Neurociències, Universitat Autònoma de Barcelona, Bellaterra, Spain, 4 Institut de Neuropatologia, Servei Anatomia Patologica, IDIBELL-Hospital Universitari de Bellvitge, L'Hospitalet de Llobregat, Spain

CNS Drugs
https://doi.org/10.1007/s40263-021-00797-x

ORIGINAL RESEARCH ARTICLE

First-in-Human Randomized Trial to Assess Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of the KDM1A Inhibitor Vafidemstat

Rosa María Antonijoan[1,2] · Juan Manuel Ferrero-Cafiero[1] · Jimena Coimbra[1] · Montse Puntes[1] · Joan Martínez-Colomer[1] · María Isabel Arévalo[3] · Cristina Mascaró[3] · Cesar Molinero[3] · Carlos Buesa[3] · Tamara Maes[3]

Accepted: 12 February 2021

Oryzon is the first and only company pursuing LSD1i in CNS diseases

Vafidemstat is an investigational LSD1 inhibitor with high BBB penetration, optimized for the CNS

Vafidemstat has been administered to more than 300 patients across the multiple Phase I and II clinical trials completed or ongoing, and has shown to be well tolerated

Patients showed significant reduction in Aggression and Agitation in BPD, ADHD, ASD and AD; Good tolerability profile

Multiple value-driving catalysts with Phase IIb trials ongoing in BPD and SCZ, and an IND submission for Kabuki Syndrome expected by 1H2022

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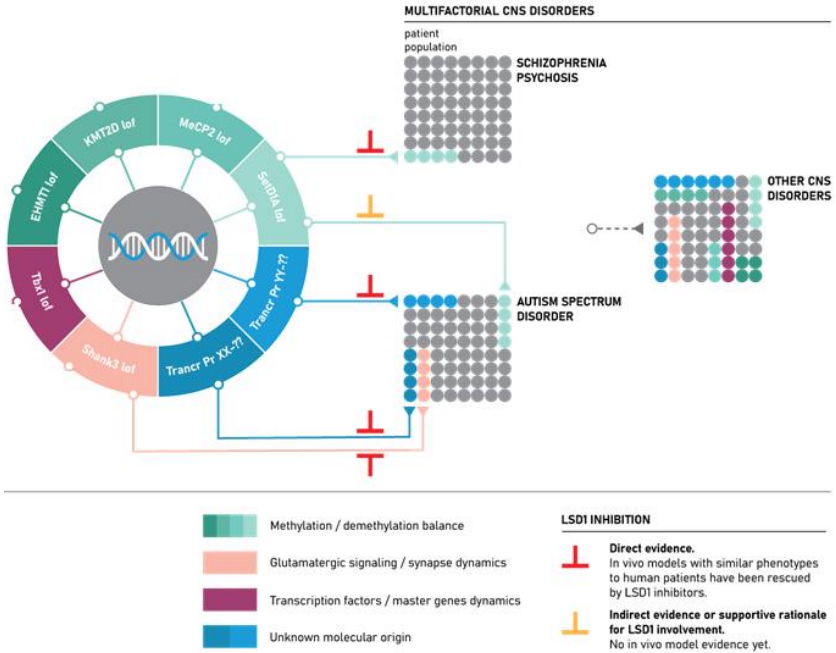
LSD1 Inhibition and Multifactorial CNS Disorders

- LSD1 is expressed in the developing and adult nervous system
- LSD1 plays a critical role in neurogenesis, in neuronal differentiation, axonal navigation and regulating expression of key genes
- Regulation of methylation has emerged as a top pathway significantly correlated with adult psychiatric disorders
- LSD1 is the most abundant KDM in the cortex and controls gene expression through methylation and scaffolding
- Detailed topographic analysis indicates that LSD1 interacts with enhancers and promoters of important CNS genes, including some confirmed CNS disease risk genes, controlling their expression
- There is significant evidence of additional pathways where LSD1 could have an impact on multifactorial CNS disorders



LSD1 Inhibition can compensate complex phenotypes caused by Multifactorial CNS Disorders

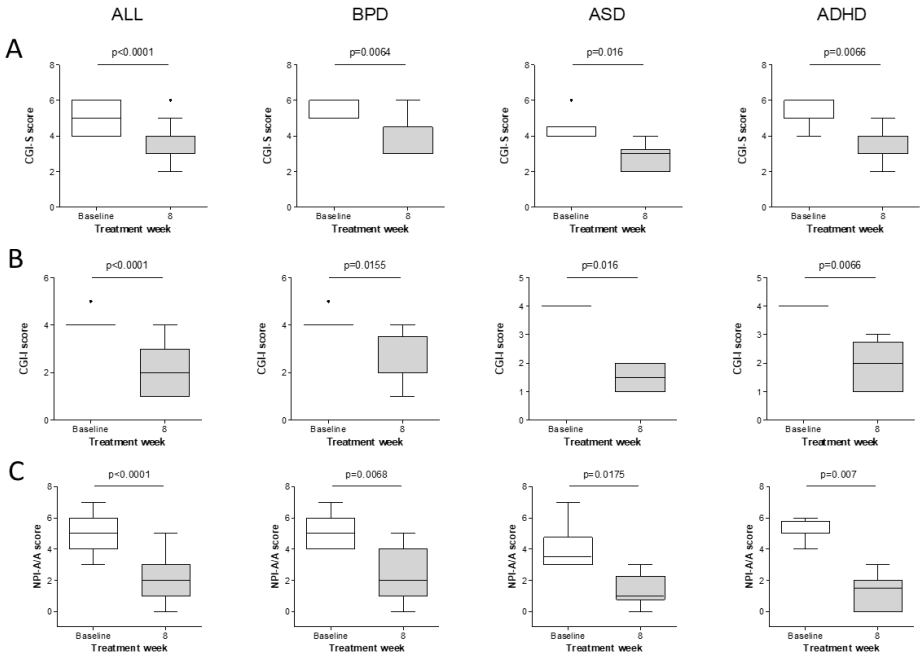
Vafidemstat: Aggression / Agitation Phase IIa REIMAGINE Trial Overview

Trial Design / Overview

- Open label, single-center, Phase IIa study
- Adult patients with BPD, ADHD and ASD with significant agitation and aggression
- Received vafidemstat 1.2mg/day, 5 days per week for 8 weeks
- 30 patients enrolled (12 BPD, 11 ADHD, 7 ASD)

Results Highlights

- Observed statistically significant improvements in aggression on the aggregated data as well as for each of the three disease groups, BPD, ASD and ADHD, independently
- Vafidemstat resulted in a 64% reduction of aggressiveness measured by the neuropsychiatric inventory - agitation and aggression ("NPI-A/A") scale
- Aggressiveness reduction was observed in 96% patients treated with vafidemstat for a period of two months
- Improvements also observed in overall patient functioning, particularly in BPD patients



Effect of vafidemstat on aggression assessed by the CGI-S (A), CGI-I (B) and NPI-A/A (C) scales. Baseline and end of treatment (week 8) data are presented as Tukey whisker plots with outliers.

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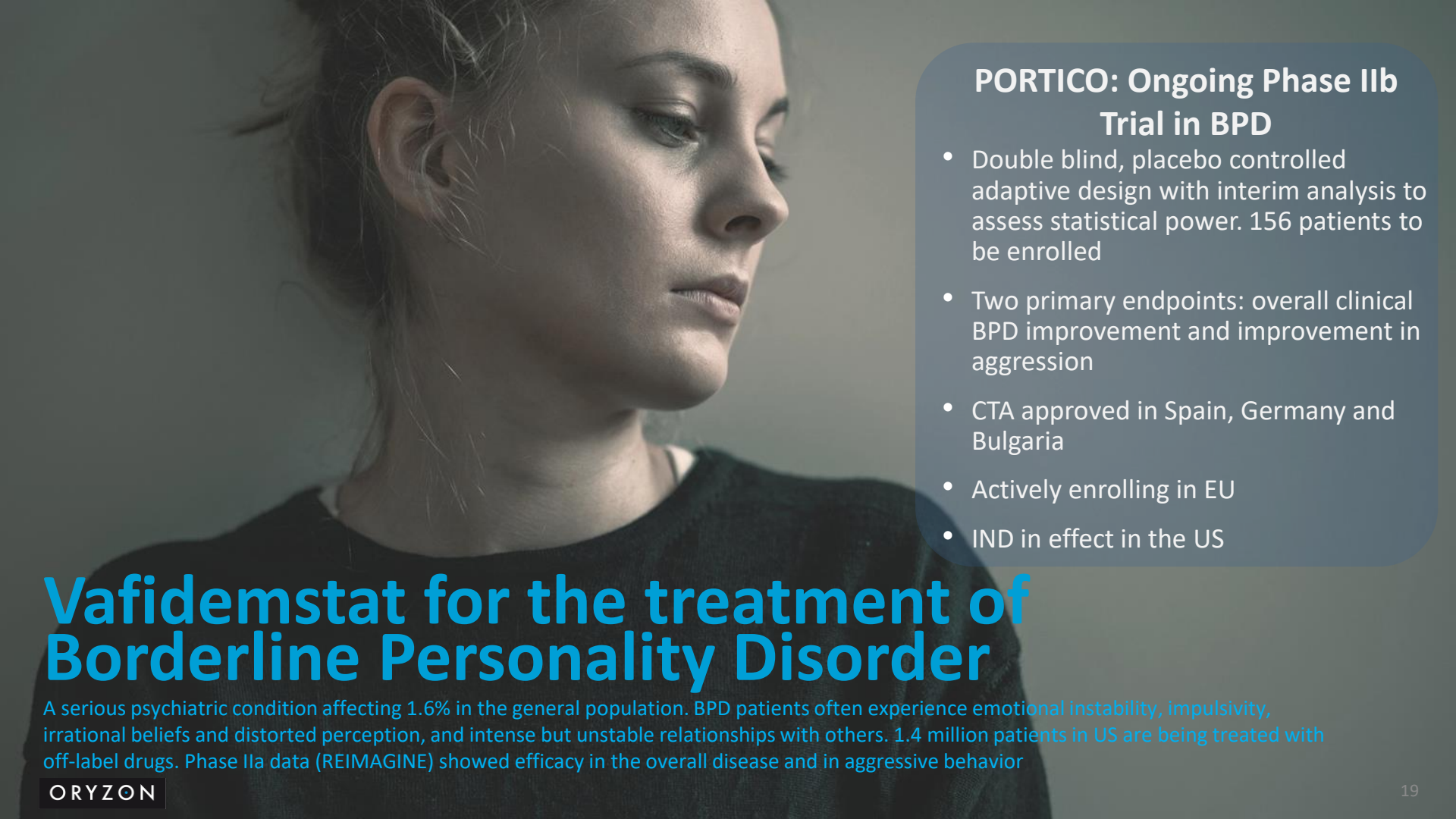

PORTICO: Ongoing Phase IIb Trial in BPD

- Double blind, placebo controlled adaptive design with interim analysis to assess statistical power. 156 patients to be enrolled
- Two primary endpoints: overall clinical BPD improvement and improvement in aggression
- CTA approved in Spain, Germany and Bulgaria
- Actively enrolling in EU
- IND in effect in the US

Vafidemstat for the treatment of Borderline Personality Disorder

A serious psychiatric condition affecting 1.6% in the general population. BPD patients often experience emotional instability, impulsivity, irrational beliefs and distorted perception, and intense but unstable relationships with others. 1.4 million patients in US are being treated with off-label drugs. Phase IIa data (REIMAGINE) showed efficacy in the overall disease and in aggressive behavior

EVOLUTION: Ongoing Phase IIb Trial in SCZ

- Double blind, placebo controlled adaptive design with interim analysis to assess statistical power. 100 patients to be enrolled in two arms
- 2 arms, vafidemstat as add-on to SoC. 6 months of treatment
- Primary endpoints: efficacy to address SCZ Negative and Cognitive Symptoms
- 6-10 sites. CTA approved in Spain
- Currently recruiting patients

Vafidemstat for the treatment of Schizophrenia

Prevalence of schizophrenia (SCZ) and related psychotic disorders in the US range between 0.25% and 0.64%.
No current approved treatments for the cognitive impairment or the negative symptoms of schizophrenia.
Abnormalities of GABAergic neurons believed to play a key role in the pathophysiology of schizophrenia. Vafidemstat upregulates GABAergic genes in PFC neurons in response to stress. LSD1i restores phenotypes in various SCZ mice models.

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LSD1 and Precision Medicine in CNS

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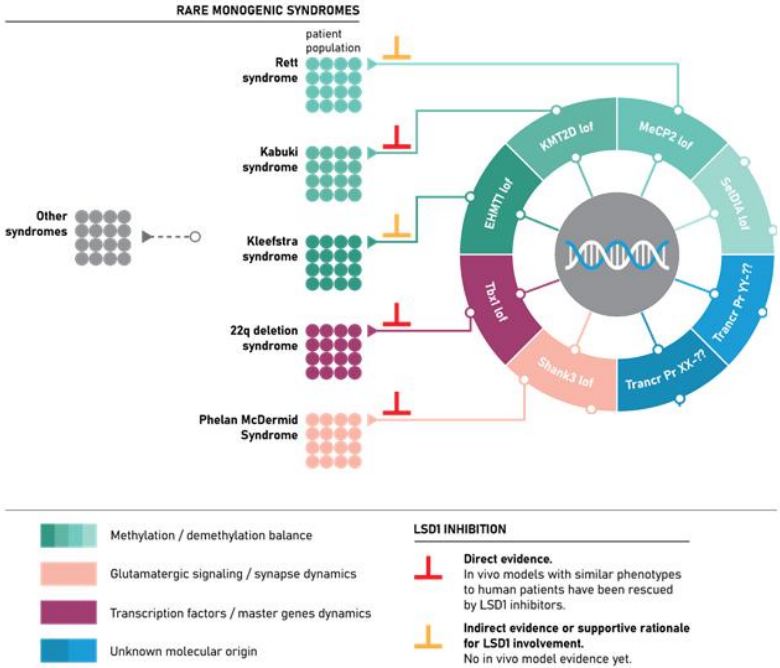
LSD1 Inhibition: Personalized Medicine in Rare Monogenic CNS Disorders

- Dysregulated methylation plays an important role in the onset of certain neurodevelopmental disorders
- Genetic single defects or loss of alleles can cause CNS disorders with complex phenotypes
- Preclinical data has shown that excessive LSD1 activity may be a key part of specific monogenic neurodevelopmental syndromes
- LSD1 inhibition can correct distinct independent deficiencies occurring upstream by resetting the appropriate transcriptional program

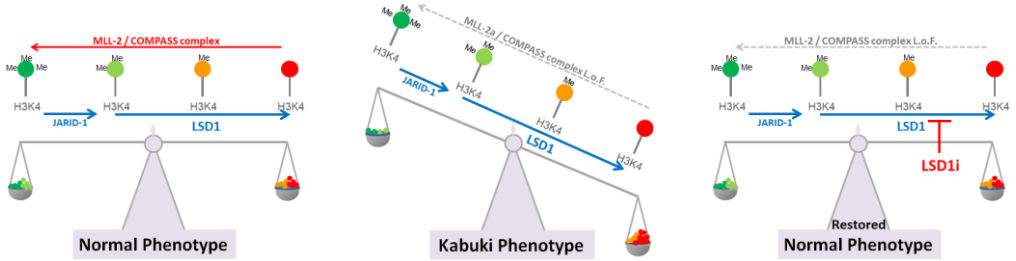


LSD1 Inhibition can compensate complex phenotypes caused by single gene deficiencies that are the cause of some rare neurodevelopmental syndromes

Methylation is involved in Kabuki Syndrome and LSD1 inhibition rescues phenotypes in genetic models

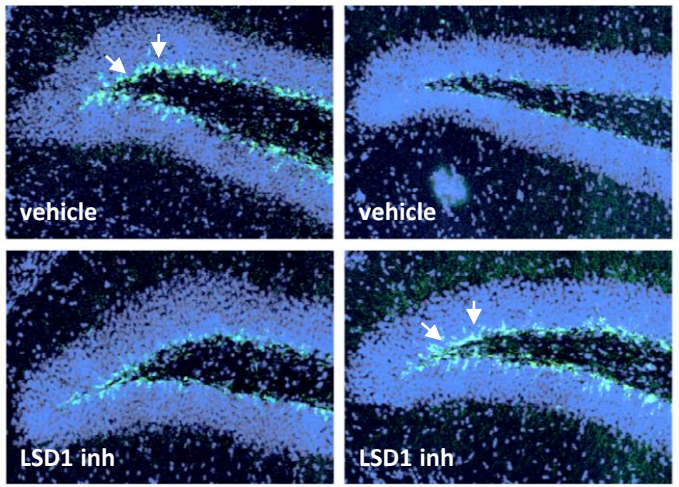
Kabuki syndrome is a congenital disorder characterized by intellectual disability, growth retardation, dysmorphic facial features and immune defects

Mutations of *MLL2 (KMT2D)* cause Kabuki syndrome in >70% of cases (known as KS type I)



- **Effects of LSD1i in the phenotype of KO Kabuki mice***
  - **LSD1i restores methylation balance in the hippocampus**
  - **LSD1i rescues adult neurogenesis and memory deficits**
  - **LSD1i restores normal neuronal morphology**
  - **LSD1i rescues global gene expression changes**
  - **LSD1i rescues the visuospatial learning and memory defects**
  - **LSD1i rescues immune defects**

LSD1i rescues neurogenesis defects in hippocampus of Kmt2d$^{+/\beta Geo}$ mice



*Modified from Zhang et al, Molecular Therapy: Methods & Clinical Development ,Vol. 20 , 779-791 (March 2021)

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Next Steps: Vafidemstat Phase I/II HOPE Trial in Kabuki Syndrome

HOPE, a new randomized Phase I/II study of vafidemstat in patients with Kabuki syndrome (KS)

Adaptive randomized clinical trial in KS to study immune status and CNS capabilities

- High unmet medical need
- Strong molecular rationale for LSD1 inhibition improving KS core features
- Randomized, double blinded, multi-arm, multicenter study
- IND submission 1H2022 / FPI 1H2022. 50-60 patients to be included
- KS occurs in 1/32,000 births. This represents a prevalence of 3,000 US patients younger than 25yrs

HOPE can potentially support an application for an accelerated approval if a significant clinical benefit in the population is demonstrated over placebo

Multiple Collaborations Exploring Vafidemstat in CNS Precision Medicine

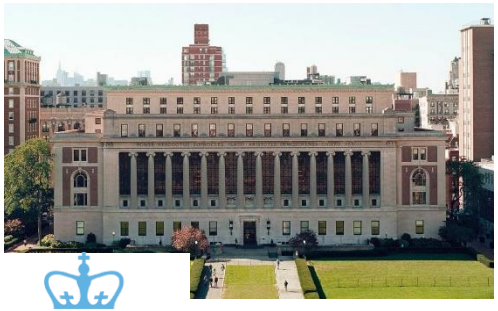
Collaborations with prominent institutions exploring the potential of LSD1 in CNS Precision Medicine

MLL-2 Kabuki Syndrome Type 1

JOHNS HOPKINS
SCHOOL of MEDICINE

- Kabuki Syndrome provides a clear molecular concept and preclinical evidence supports a potential accelerated approval application in 2023 following HOPE trial
- HOPE clinical trial in preparation with KKI-JH

SETD1A-related SCZ



COLUMBIA UNIVERSITY
IN THE CITY OF NEW YORK

- Subjects carrying these mutations develop schizophrenia and psychosis
- Patients identified in the US Amish community
- Ongoing psychometric study with 60 subjects to inform a future clinical trial
- Results expected in 2H21

SHANK3-related ASD

SaludMadrid Hospital Universitario La Paz
Hospital Carlos III
Hospital Cantoblanco

- Subjects carrying these mutations develop Phelan McDermid syndrome and ASD
- +200 Patients identified in Spain and LA
- Ongoing psychometric study with 40 subjects to inform a future clinical trial
- Results expected in 2H21

Numerous Near-term Milestones Expected

Iadademstat Milestones	
Event	**Timing**
IND submission for FRIDA trial	2H'21
IND submission for STELLAR trial	1H'22
IND submission for NET Basket Combo trial	1H'22
ALICE trial clinical update (AML,Elderly Unfit)	ASH'21
FPI IN FRIDA	1H'22
FPI IN STELLAR	1H'22
Final data for ALICE Phase II Trial	ASH'22
Update on FRIDA	EHA'22 and ASH'22
FPI in NET Basket Combo trial	1H'22
FRIDA / STELLAR / NET trials clinical update	EHA/ASCO'23 WCLC/ASH'23

Vafidemstat Milestones	
Event	**Timing**
US FPI in PORTICO trial (BPD)	2021
ASD/Shank3 update	ICA'21
SCZ/SetD1a update (Columbia University collab)	2H'21
MS data update	ECTRIMS'21
FPI in EVOLUTION trial (schizophrenia)	2H'21
IND submission for Kabuki syndrome (HOPE trial)	1H'22
FPI HOPE	1H'22
HOPE trial update	ESHG'22
IND submission / FPI for SETD1a related schizophrenia	2022
Initiate Phase IIb in AD aggression/agitation	2H'22

Potentially Best-in-Class LSD1i Addressing Unmet Medical Needs in Oncology And CNS

ONCOLOGY

Iadademstat (ORY-1001)
Differentiation
Anti-cancer stemness

- Potential first & best-in-class LSD1i in Oncology
- 4 Phase I/II clinical trials: 100+ patients dosed
- Robust and durable responses in AML (80%+ ORRs)
- Two Phase Ib/II trials with potential to support registration
  - FRIDA, a study in 2L in AML
  - STELLAR, a study in 1L in ED-SCLC

ORYZON

A unique dual EPIGENETIC proposition in ONCOLOGY and CNS

- A validated approach with multiple shots on goal
- 2 Phase II programs
- Differentiated pipeline of potential best-in-class LSD1 therapies
- Tolerability shown in 400+ subjects dosed
- Value-inflection updates in 2022
  - FIH for FRIDA trial in 2022
  - Final data for ALICE at ASH 22
  - HOPE Phase I/II trial in Kabuki syndrome has potential to support registration

CNS

Vafidemstat (ORY-2001)
Prosynaptic
Anti-neuroinflammatory

- Potential first-in-class LSD1i in CNS
- 8 Phase I/II clinical trials: 300+ subjects treated
- Reduced agitation and aggression in Phase II trials in multiple psychiatric disorders and AD
- Two ongoing Phase IIb studies in 2021 in BPD and SCZ
- Two new personalized medicine Phase II trials in 2022: in Kabuki syndrome and in SETD1a related schizophrenia