



# SUCCESS IN CNS DRUG DEVELOPMENT – INNOVATION IN RARE DISEASES

Corporate Presentation  
September 2017



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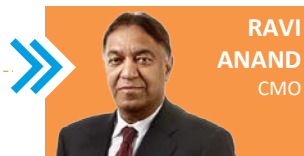
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# Newron Leadership Team



**STEFAN WEBER**  
CEO

- 30 years of experience
- Previously worked at: Lohmann Group, Girindus and Biofrontera



**RAVI ANAND**  
CMO

- >30 years of experience
- Previously worked at: Roche (CH), Sandoz (US), Novartis and Organon (NL)



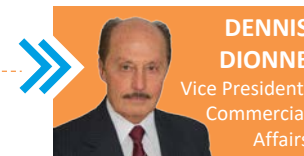
**ROBERTO GALLI**  
Vice President  
Finance

- 20 years of experience
- Previously worked at: Coopers & Lybrand and PricewaterhouseCoopers



**MARCO CAREMI**  
EVP Business  
Development

- >35 years of experience
- Previously worked at: Schwarz Pharma and Schering-Plough



**DENNIS DIONNE**  
Vice President,  
Commercial  
Affairs

- >26 years of experience
- Previously worked at: Novartis and Johnson & Johnson



## Non-Executive Chairman of the Board of Directors

### ULRICH KÖSTLIN:

Former Executive at Bayer Schering Pharma AG



## STEPHEN GRAHAM Executive Director, Clinical Development

- 30 years of experience
- Previously worked at: Boots Pharmaceuticals, Sandoz/Novartis and Forest Laboratories/ Forest Research Institute



## Company Highlights



1. **Diversified Portfolio of Innovative CNS Product Candidates**
2. **Xadago® - Commercialized in 12 European Countries, launched in US in July 2017**
3. **Sarizotan for Rett Syndrome in Late Stage Development**
  - Pivotal Phase III data expected QIII/2018
4. **Evenamide - a Novel Mechanism / Treatment Paradigm for Schizophrenia**
  - Phase IIa results met tolerability, safety and preliminary efficacy objectives
5. **Multiple Catalysts on the Horizon**
6. **Management Team with Proven Track Record**

# Successful Track Record in CNS Product Development

## NOVEL CNS PRODUCT CANDIDATES

### Xadago® (safinamide)

Commercialized in 12 European markets and the US for Parkinson's disease ("PD")



Newron receives milestone and royalty payments from sales of safinamide in PD  
– €36m received to date

### Sarizotan

Undergoing potentially pivotal development for Rett syndrome – an orphan disease



Newron will commercialize Sarizotan for Rett syndrome

### Evenamide (NW-3509)

Phase IIa trial results met study objectives of good tolerability, safety, and preliminary evidence of efficacy



Ready for confirmatory efficacy / safety study

... INNOVATION  
in rare diseases



# Innovative Clinical Pipeline with Multiple Near Term Catalysts

PRODUCTS		Phase I	Phase II	Phase III	Market	Commercial Rights	
<b>Xadago®</b> (safinamide) <sup>1</sup>	Adjunctive therapy in PD	[Progress bar]					<b>Zambon</b>
	Adjunctive therapy in PD	[Progress bar]					<b>US WorldMeds</b>
	Adjunctive therapy in PD	[Progress bar]					<b>Meiji Seika / Eisai</b>
	Levodopa Induced Dyskinesia (PD LID)	[Progress bar]					<b>Zambon</b>
<b>Sarizotan<sup>2</sup></b>	Rett syndrome (Orphan drug status)	[Progress bar]					<b>Newron</b>
<b>Evenamide (NW-3509)<sup>1</sup></b>	Schizophrenia / TRS	[Progress bar]					<b>Newron</b>
<b>Ralfinamide<sup>1</sup></b>	Orphan indication in neuropathic pain	[Progress bar]					<b>Newron</b>

## Expected Milestones

### Xadago®:

Further EU launches expected  
 Study in patients with Levodopa Induced Dyskinesia (PD LID) expected to start in 2018

### Sarizotan:

Potentially pivotal study commenced July 2016; results expected QIII/2018; own commercialization

### Evenamide:

Start of confirmatory efficacy / safety study; potentially pivotal PIIB/III results expected 2019

Ongoing search for strategically relevant assets to in-license

<sup>1</sup> Safinamide, Evenamide and Ralfinamide all developed from Newron's ion channel based research

<sup>2</sup> Sarizotan was licensed from Merck KGaA

# Xadago®: 1st New Chemical Entity Approved in US or Europe in a Decade for Parkinson's Disease



A progressing disorder, no cure available yet

- Parkinson's Disease: 2<sup>nd</sup> most common chronic progressive neurodegenerative disorder in the elderly
- Affecting 1-2% of individuals aged ≥ 65 years worldwide



Fast and sustained efficacy, well tolerated

MID- TO LATE-STAGE PD PATIENTS –  
add-on to L-Dopa dopamine replacement

- Significant improvement of
  - ON Time/OFF Time – regulatory endpoint
  - UPDRS II – activities of daily living
  - UPDRS III – motor function
  - CGI (clinical global impression) – severity and improvement
- Additional ON Time without any increase in any dyskinesia



Sources:

- 7 Parkinson's Disease – Global Drug Forecast and Market Analysis – Event-Driven Update – GlobalData, June 2015
- 7 Parkinson's Disease Foundation: Statistics on Parkinson's Treatment of Advanced Parkinson's Disease, Varanese et al., 2010, NCBI



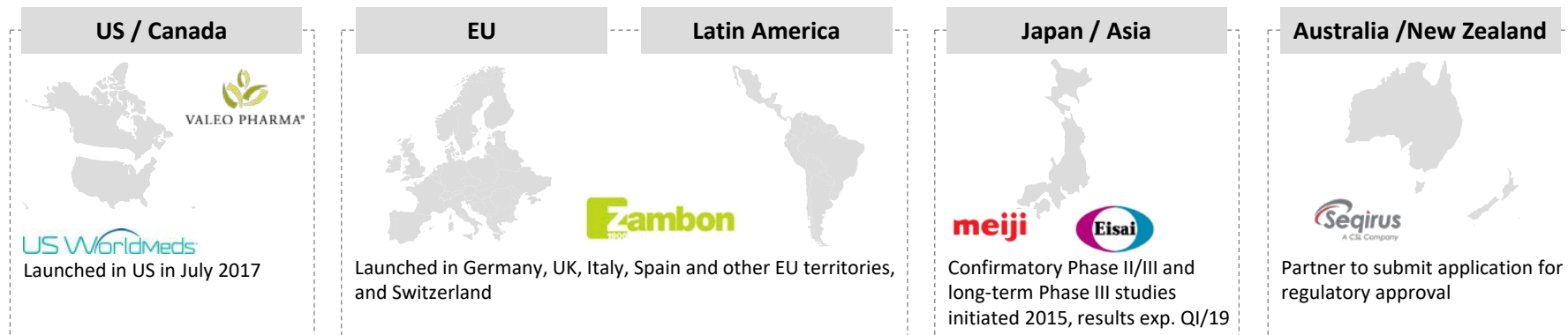
## Xadago®: Label Expansion Study in Patients with Levodopa Induced Dyskinesia (PD LID)

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- Newron and its partner Zambon, together with academic and regulatory experts, are in the process of designing a potentially pivotal efficacy study to evaluate the effects of Xadago® (safinamide) in patients with levodopa induced dyskinesia (PD LID)
- Discussions with the EU and US regulators on study design, based upon previously reported clinical and pre-clinical data for PD LID (>200 patients, 2 year treatment duration); expected to be finalized by Q1/2018
- The study is expected to start in 2018, with data read-out expected in 2019



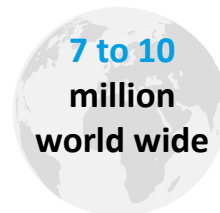
# Significant Commercial Opportunity in Xadago® (Safinamide)



➤ Milestone and royalty revenues to Newron since 2012

➤ Long period of market exclusivity (patent life: 2029 in EU, 2031 in the US)

➤ Peak sales potential up to \$700m+ (analyst estimates)



20% to 30% in early stage

70% to 80% percent in mid to late stage

Approximately \$4 Billion worldwide market

# Rett Syndrome: Severe Neuro-developmental Orphan Disease with No Approved Treatment Options

- 95-97% of patients have spontaneous mutations in the X-linked MeCP2 gene
- Disease manifests almost exclusively in females with one affected X-chromosome
- Normal development until 6-18 months of age, then loss of skills and ability for social interaction
- Respiratory abnormalities, motor and severe intellectual impairment, sleep abnormalities and seizures in most patients (70-90%)
- 25% of sudden deaths in RTT may be due to cardio-respiratory abnormalities
- Focus on symptom management
- Estimated 36,000 patients in US and EU combined



# Sarizotan: Targeting Respiratory Disturbances in Rett Syndrome Patients

- First RTT drug candidate targeting respiratory disturbances as primary efficacy outcome
- Deficits in serotonergic transmission due to the MeCP2 mutation in the mid-brain nucleus underlie the respiratory abnormalities in MeCP2 deficit mice
- Sarizotan, a full agonist at the serotonergic 5HT1A receptor, has demonstrated dramatic improvement of respiration in genetic (MeCP2) mouse model of RTT
- Development path/regulatory requirements for approval agreed upon with FDA/EMA/HPB; clear commercialization strategy
- Orphan drug designation in EU and US
- Potentially pivotal STARS study initiated 2016

EFFECTS OF ACUTE ADMINISTRATION WITH SARIZOTAN IN RTT FEMALE MICE (MECP2<sup>R168X/+</sup>). BENEFIT PERSISTS IN LONG LASTING TREATMENTS (14-DAYS-MECP2<sup>R168X/+</sup>)

Apnea in MeCP2-deficient mice



Apnea in MeCP2-deficient mice treated with Sarizotan 5.0 mg/kg



## STARS: First International Phase III, Potentially Pivotal, Study in RTT



- International, randomized, double blind, placebo-controlled, 6 months treatment study under US IND
- Centers of excellence in the United States, Italy, UK, Australia and India
- Study protocol designed in accordance with regulatory authorities in the United States, Europe and Canada
- Will enroll at least 129 RTT patients, 6 years or older who experience at least 10 apnea episodes of >10 sec/ hour as verified by a validated device over at least 3 hours of recording time while patient is awake and at home
- Primary endpoint: percent reduction in number of objectively defined clinically significant (>10 sec) apnea episodes over an extended period of time
- Study enrolling
- Expected completion of study 2018, with top line results expected QIII / 2018

# Sarizotan Market Opportunity and Commercialization Strategy by Newron

## Initiation of a Health Economic Outcome Research Study (HEOR)

→ "burden of illness"

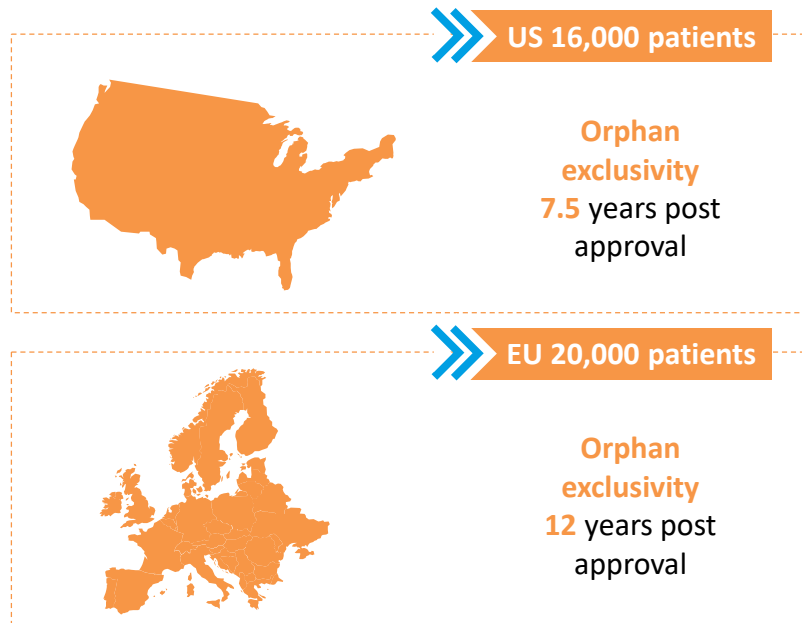
- Fostering partnership and collaborations with Rett advocacy, thought leaders & governing payers
- Global survey to quantify the ways in which patient "respiratory breathing abnormalities" affect daily life
- Meets Health Technology Assessment (HTA) requirements, including European Network of countries requiring information for treatment access

## Goals

- Identify gaps & unmet need for improving disease management
- Align economic & clinical outcomes
- Create awareness to breathing abnormality burden
- Optimize market uptake, access, reimbursement
- Build Newron leadership

## Rare pediatric disease voucher possibility

Sources:  
Rettsyndrome.org  
National Institute of Health – NINDS  
US Census Bureau, 2012  
Eurostat Census, 2011



Small team ~ 25-30 medical liaison managers required to commercialize sarizotan in US and Europe

# Schizophrenia Market Opportunity – No Effective Treatment that Reduces Burden of Disease in Last 20 Years

- Onset of disease occurs in early adulthood affecting 1% of the population worldwide
  - Need for life-long treatment
  - US diagnosed and treated approximately 2M
- Disease characterized by positive, negative, and cognitive symptoms:
  - Hallucinations, delusions, paranoia, hostility and irritability (positive)
  - Progressive deterioration of cognition and behavior & presence of negative symptoms
  - High rates of suicide, incarceration, multiple physical illnesses and lower life expectancy
- Efficacy of current treatment options insufficient
  - Typical (e.g. haloperidol) worsen negative symptoms and cause neurological side effects
  - Efficacy of typical and atypical limited and wanes over 18 months; 64-82% of patients switch but without additional benefit
  - No effect on suicidality
- Treatment-resistant schizophrenia (TRS)
  - 30% of patients after 3-5 years are TRS: only clozapine shows efficacy
  - 30-50% of these patients show resistance to clozapine



## Limited Effectiveness of Current Antipsychotics in Treating Schizophrenia

- Findings From 3 Major Non-Commercial (CATIE , CUTLASS, and EUFEST) studies reveal significant dissatisfaction with all current antipsychotics:
  - Approximately 74% of patients discontinue first or second generation antipsychotic medication (CATIE, CUTLASS) within 18 months due to inadequate efficacy/ intolerance
  - Median time to discontinuation ranges from 3.5 (ziprasidone)- 9.2 (olanzapine) months (CATIE)
  - Minimal marked differences between treatments (except clozapine) in extent of improvement in psychopathology as measured by PANSS, CGI, QLSS

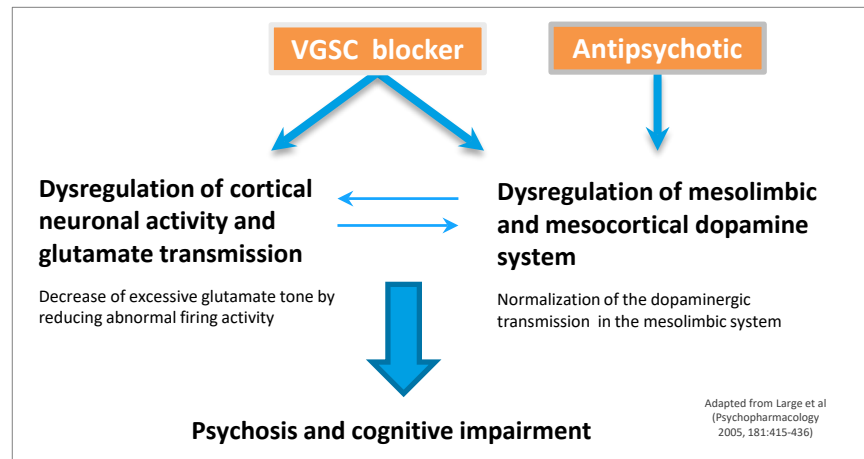
### POSSIBLE REASONS INCLUDE:

- All these drugs have same/ similar mechanism of action, e.g. 5HT<sub>2</sub>/D<sub>2</sub> antagonism with effects at other receptors of no relevance for efficacy
- Effective resolution of psychopathology requires effects on other targets / mechanisms
- Chronic blockade of dopaminergic receptors in mesolimbic structures may lead to upregulation of receptors and loss of efficacy/ worsening

# Evenamide (NW-3509)'s novel MoA: Synergistic with Marketed Antipsychotics

- Evenamide has the potential to target the abnormal neuronal activity and glutamate transmission in patients with schizophrenia
- Evenamide may add to or synergize with antipsychotic drugs to bring about a combined therapeutic effect on glutamate and dopamine systems and modulate these major neurotransmitter systems that have been associated with positive symptoms in schizophrenia
  - Effects seen in combination with haloperidol, risperidone and aripiprazole
- Composition of matter – USPTO, 2013 - patent life 2028 plus extension

## Voltage-Gated Sodium Channels (VGSC) blockers may act Synergistically with antipsychotics in schizophrenia therapy





# Unique MOA Demonstrated

Evenamide, a selective Voltage-Gated Sodium Channel (VGSC) Blocker, shows no effect on >130 CNS receptors, enzymes, transporters, etc

Selectively blocks VGSCs in a voltage-and use-dependent manner

Modulates sustained repetitive firing without inducing impairment of the normal neuronal excitability

Inhibits Glutamate Release

Inhibition of native sodium channels expressed in rat cortical neurons

$K_{rest}$  ( $\mu\text{M}$ )

25

$K_{inact}$  ( $\mu\text{M}$ )

0.4

High frequency firing

Control



Evenamide 1 $\mu\text{M}$

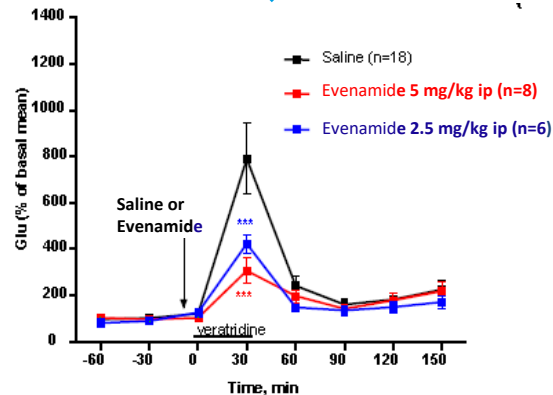


Low frequency firing

Control



Evenamide 1 $\mu\text{M}$



# Evenamide is Active in a Wide Range of Schizophrenia and Psychiatric Animal Models as a Monotherapy and as an Add-On to Existing Antipsychotics

		Monotherapy	Add-On
<b>Information Processing Deficit</b>	Pre-pulse inhibition (PPI) disrupted by dopamine activation (amphetamine -rat)	✓	✓
	Pre-pulse inhibition (PPI) disrupted by NMDA antagonists (MK-801, PCP, -rat)	✓	
	Pre-pulse inhibition (PPI) disrupted by natural stimuli (sleep deprivation -rat)	✓	
	Pre-pulse inhibition spontaneous deficit (C57 mice)	✓*	✓
	<i>Pre-pulse inhibition (PPI) disrupted by Ketamine in rat (ongoing)</i>	✓	
<b>Negative Symptoms</b>	PCP-induced deficit in Social Interaction in the rat	✓	✓
	<i>Saccharin preference test (anhedonia) in prenatal poly:IC exposed mice (ongoing)</i>	✓	
	<i>Three-chamber sociability test in prenatal poly:IC exposed mice (ongoing)</i>	✓	
	<i>Forced swimming test (avolition) in prenatal poly:IC exposed mice (ongoing)</i>	✓	
<b>Psychosis and Mania</b>	Amphetamine induced hyperactivity in mice	✓	✓
	Amphetamine plus Chlordiazepoxide induced hyperactivity in mice	✓	✓
<b>Cognitive Impairment</b>	Novel object recognition in the rat: short term scopolamine impairment	✓	
	Novel object recognition in the rat: long term 24 hr natural forgetting	✓	
<b>Impulse Control and Mood Symptoms</b>	Resident–Intruder test in mice (Impulsivity)	✓	
	Tail suspension test in mice (Depression)	✓	
	Marble burying test in mice (Obsessive Compulsive Disorders)	✓	

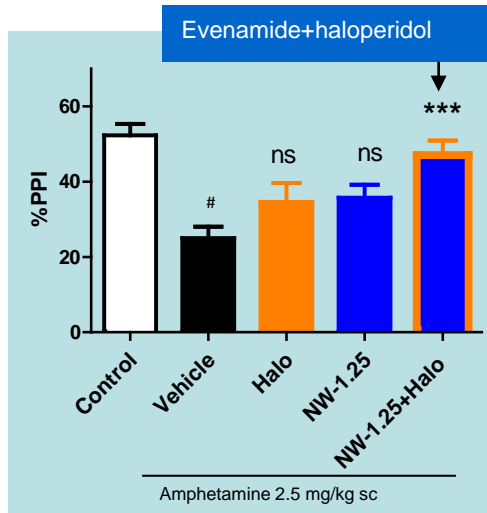
\*Trend  
Blank cells = not evaluated



# Add-on: Evenamide augments the effect of typical and atypical antipsychotics in positive symptom models

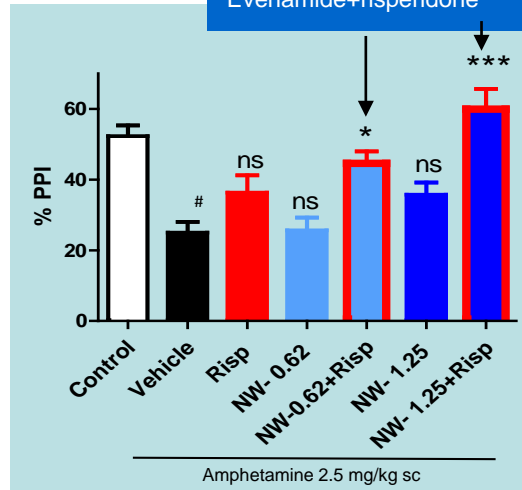
Add-on with non-active dose of **haloperidol**  
MED 1.25 mg/kg *po* (+haloperidol 0.05mg/kg *ip*)

## Amphetamine-induced PPI deficit



Add-on with non-active dose of **risperidone**  
MED 0.62 mg/kg *po* (+risperidone 0.05 mg/kg *ip*)

## Evenamide+risperidone



Tukey's multiple comparison test \* $p < 0.05$ , \*\*\* $p < 0.001$  vs Vehicle+Amp (n=6-18 rats per group)

## Add-on activity showed in other models

- ✓ Pre-pulse inhibition spontaneous deficit (C57 mice)
- ✓ Amphetamine hyperactivity in mice
- ✓ Amphetamine plus Chlordiazepoxide induced hyperactivity in mice
- ✓ PCP- induced deficit in Social Interaction in the rat

## Summary Phase IIa - Clinical Validation of a Novel Treatment Concept



- Evenamide as add-on treatment
  - For patients with schizophrenia on stable and adequate dose of standard therapy, experiencing break-through symptoms
- Double-blind, placebo-controlled, randomized, 4-week in/outpatient study in US and India in 89 patients receiving Evenamide 15-25 mg/ twice daily or placebo, in addition to their current antipsychotic
- Endpoints: Symptoms of schizophrenia, as assessed by
  - Positive and Negative Syndrome Scale (PANSS),
  - Strauss-Carpenter Level of Functioning scale,
  - Clinical Global Impression - Change from baseline (CGI-C) and CGI - Severity of illness (CGI-S)
- Detailed results presented at 16th International Congress on Schizophrenia Research March 25, 2017
- Evenamide met study objectives of good tolerability, and safety
- Evenamide demonstrated consistent evidence of efficacy on key measures
  - Primary measure: Significant improvement on PANSS positive (mean change and responders)
  - Near Significant increase in CGI-C responders
  - No side-effects that are associated with dopamine-blocking antipsychotics
  - Greater improvement on all efficacy measures at every time point compared to standard of care
- Ready for confirmatory efficacy / safety study or partnering

## Evenamide – Study 002 in patients with chronic schizophrenia

- 4-week, placebo-controlled, add-on study of evenamide (15-25mg BID/day) study in patients on stable doses of aripiprazole or risperidone showing signs of worsening;
- Analyses indicate significance/trends in favor of evenamide for PANSS positive scale total ( $p=0.0459$ ; ANCOVA-LOCF), and the proportion of patients improved (Fisher's exact test) on PANSS positive scale ( $p=0.0043$ ) and CGI-C ( $p=0.0855$ )
- Results indicate greater improvement in patients who are  $< 32$  yrs with  $< 10$  yrs of disease
- Results support hypothesis that evenamide's glutamatergic mechanism will improve symptoms of psychosis in patients not responding to D2/5HT2 blockade of standard antipsychotics
- Physiological modelling predicted that mean plasma concentrations of  $>20$  ng/ml would be efficacious: this was confirmed in this study at doses of 15-25 mg bid
- Scientific Advisory Board meeting (July 12, 2017);
  - US and EU schizophrenia experts reviewed preclinical, safety and efficacy data
  - Fully endorsed above conclusions: unique mechanism needs exploration in other indications
  - Strongly recommend study in clozapine failures
- Additional Advisory Board meetings planned

# Evenamide – Applicability in Clozapine Resistant Treatment Resistant Schizophrenia (TRS), an Orphan Indication

- ~ 30%\* patients continue to be psychotic, with unresolved symptoms, such as delusions, hostility, grandiosity, and hallucinations, after they have completed treatment with at least two adequate trials antipsychotic medications at therapeutic doses from different chemical classes
- Outcomes/ service utilization data indicate treatment-resistant schizophrenia is a categorically different illness to treatment-responsive schizophrenia
- TRS is associated with some of the highest rates of hospitalization and costs to society - \$34B in Direct Healthcare costs in the United States
- Despite similarities; olanzapine and quetiapine do not show efficacy in TRS
- No drug other than clozapine has shown efficacy in these patients
- Clozapine is the only drug with an FDA indication for TRS and for reducing suicidal behavior
- 30% of (TRS) patients on clozapine do not respond adequately, or develop resistance to its effects
- NIMH/ FDA/ ECNP/ EMA have raised this as an issue of grave concern

\*Treatment-resistant schizophrenia: current insights on the pharmacogenomics of antipsychotics; John Lally, Fiona Gaughran and Sarah R Curran, Nov. 2016

# Evenamide – Applicability in Clozapine Resistant Treatment Resistant Schizophrenia (TRS), an Orphan Indication

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## Glutamate system in (TRS) patients

- Studies suggest treatment-resistance (TR) may be differentiated by abnormalities in brain glutamate concentrations not seen in treatment-responsive patients; as with the dopamine findings, this again suggests a categorical difference rather than one of severity
- Case studies, small placebo-controlled studies, meta-analyses, suggest benefit of lamotrigine as add-on treatment, in clozapine resistant TRS; however there is considerable variability among trials.
- The benefits of lamotrigine are most likely due to antagonism of glutamate release
- Evenamide has antagonized as mono-therapy, and as add-on treatment effects of MK-801, PCP (glutamate releasers) in animal models of psychosis
- Results with Evenamide in animal models of schizophrenia mimic effects of clozapine
  - This indication will qualify for an Orphan Designation
- Interaction with Health Authorities ongoing

# Orphan Drug Market Opportunity Of Evenamide

## Add On Therapy For Treatment Resistant Schizophrenia (TRS) Who Are NOT Responding Adequately To Clozapine

Description	Total	
Patients with Schizophrenia in US	2.4M	
TRS patients (20-50%) after 5-10 years	30%	600K
Current and past users of clozapine	168K	
Clozapine resistant schizophrenia (30%)	19K	

- Similar prevalence estimated for EU, Japan and Canada
- Physician prescribers (US) identified through National Registry
- Targeted launch to known clozapine prescribers only
- Upside for Physician expansion >40%

Sources:

National clozapine FDA mandated monitoring registry, 2016; Current perspectives in the treatment of resistant Schizophrenia, 2009 Oct-Dec; Schizophrenia Researcher – Meltzer et al, Mouchlianitis E, Bloomfield MA, Law V, et al., Treatment-resistant schizophrenia patients show elevated anterior cingulate cortex glutamate compared to treatment-responsive. Schizophr Bull.; Ayuso-Mateos, Jose Luis., Global burden of schizophrenia in the year 2000; World Health Organization for the prevalence of disease across US, Europe and Japan; BMC Psychiatry, 2017



## Advantages Of Pursuing Orphan Designation

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- Rapid interactions with health authorities including FDA, MHRA, HPB, and EMA
- Possibility for receiving earlier access to market through 'Expanded Access'/'Treatment IND' programs (possibility for Sponsor to receive limited reimbursement)
- Through above programs faster, more economical collection of safety data thus reducing cost and length of time required to satisfy requirements for NDA/MAA filing
- This strategy would allow earlier filing for main indication

## Next Steps

### Meetings with European regulatory authorities to obtain feedback on plans for development of Evenamide

- Orphan Designation: 8 week, placebo-controlled, add-on therapy trial in Treatment Resistant Schizophrenia (TRS) not responding to clozapine (240 pts; **18 months to completion**)
- Pivotal Study: 6 week, placebo-controlled study to demonstrate efficacy and safety/tolerability of three fixed doses of Evenamide as add-on to antipsychotics in patients experiencing worsening of symptoms of schizophrenia (480 pts; **18 months to completion**)

### Meetings with FDA and HPB (Canada)

### Submissions relating to Orphan/Breakthrough/Fast track designation

- Briefing books/Meeting requests to be submitted in September
- Orphan designation decision expected by Q1 2018
- Feedback on protocols expected end November

# Multiple Catalysts on the Horizon

## Recent Accomplishments

March 2017	Encouraging PIIa data for Evenamide in Schizophrenia patients	✓
March 2017	FDA approves Xadago® for Parkinson's Disease patients	✓
April 2017	Xadago® launched in Portugal for Parkinson's Disease	✓
May 2017	Expansion of sarizotan STARS study to include patients under 13 years	✓
July 2017	Xadago® launched in US for Parkinson's Disease	✓

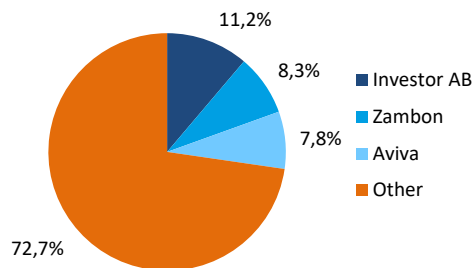
## Expected Milestones

Q1 2018	Potential Orphan Designation for Evenamide in Clozapine resistant TRS patients	
Mid-2018	Pivotal Phase III Rett Syndrome data for sarizotan	
2018	Initiate pivotal study for Xadago® in Parkinson's Disease patients with PD LID	
2018	Japan Phase II/III Xadago® results in Parkinson's Disease	
2019	Pivotal Phase IIb/III and Orphan Designation TRS Evenamide data	
2019	Potential approvals for sarizotan in Rett syndrome	
2019	Potential approvals for Xadago® in Japan	

# Company Snapshot

- Headquarters: Bresso/Milan, Italy
- Subsidiary: Morristown, NJ USA
- Listed on the Swiss Stock Exchange
- Market Capitalization: CHF 290m<sup>1</sup>

## Key Shareholding<sup>2</sup>



## Summary Financials

€'000	2015	2016	HY 2017
<b>Revenue</b>	<b>2,380</b>	<b>6,726</b>	<b>11,687</b>
Research & development expenses	(18,449)	(12,398)	(4,608)
General & administrative expenses	(8,278)	(9,140)	(4,448)
<b>Operating (loss)/profit</b>	<b>(24,400)</b>	<b>(15,325)</b>	<b>2,334</b>
<b>Net (loss)/profit</b>	<b>(22,816)</b>	<b>(15,237)</b>	<b>1,542</b>
Operating cash outflow	(12,862)	(19,583)	(1,487)
Financing activities	28,032	25,086	129
Investing activities	59	34	(42)
<b>Net change in cash</b>	<b>15,229</b>	<b>5,537</b>	<b>(1,400)</b>
<b>Closing net cash<sup>3</sup></b>	<b>40,931</b>	<b>46,468</b>	<b>45,068</b>
<b>Cash Position as of 30 Jun 2017<sup>3</sup></b>			<b>€45.1m</b>

1 Capital IQ as of 13 Sep, 2017  
 2 Company filings / As per SIX, Significant shareholders  
 3 Cash and available for sale financial assets

## Company Highlights



1. **Diversified Portfolio of Innovative CNS Product Candidates**
2. **Xadago® - Commercialized in 12 European Countries, launched in US in July 2017**
3. **Sarizotan for Rett Syndrome in Late Stage Development**
  - Pivotal Phase III data expected QIII/2018
4. **Evenamide - a Novel Mechanism / Treatment Paradigm for Schizophrenia**
  - Phase IIa results met tolerability, safety and preliminary efficacy objectives
5. **Multiple Catalysts on the Horizon**
6. **Management Team with Proven Track Record**