SUCCESS IN CNS DRUG DEVELOPMENT – INNOVATION IN RARE DISEASES

Corporate presentation
May 2017

Newron
Pharmaceuticals

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Investment Highlights

1. Diversified Portfolio of Innovative CNS Product Candidates
2. Xadago® - Commercialized in 12 European Countries, US launch announced for July 2017
3. Sarizotan for Rett Syndrome in Late Stage Development
4. Evenamide - a Novel Mechanism / Treatment Paradigm for Schizophrenia
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 for Parkinson's disease (PD); approved for commercialization in the US, launch upcoming (July)

Newron receives milestone and royalty payments from sales of safinamide in PD

Sarizotan

Developing Sarizotan for Rett syndrome, an orphan disease, in a potentially pivotal trial ongoing

Newron will commercialize Sarizotan for Rett syndrome

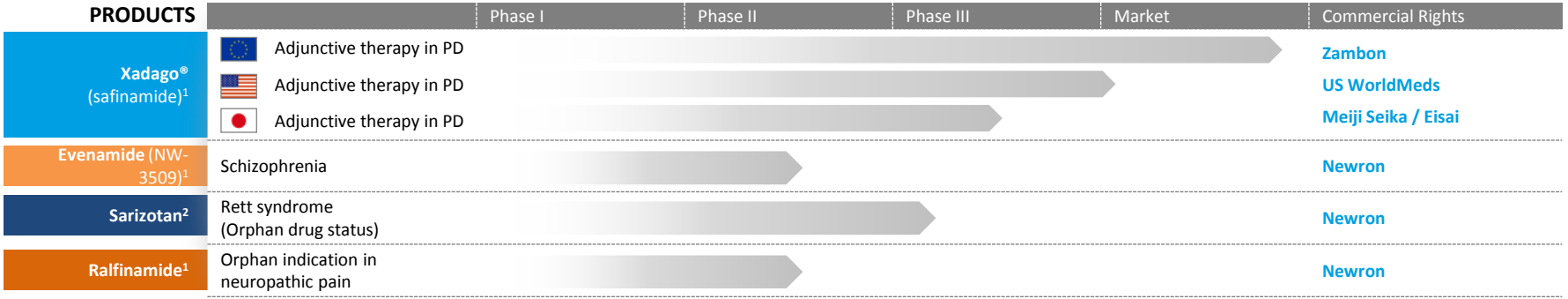
Evenamide

…(NW-3509) Phase IIa trial results met study objectives of good tolerability, safety, and preliminary evidence of efficacy

Ready for confirmatory efficacy / safety study by Newron or in conjunction with a partner

… INNOVATION
in rare diseases

Innovative Clinical Pipeline with Multiple Near Term Catalysts



Expected Milestones

Xadago®:
further EU launches expected;
US launch expected July 2017

Evenamide:
start of confirmatory efficacy / safety study alone or with a partner

Sarizotan:
potentially pivotal study commenced July 2016; results 2018; commercialization 2019

Ongoing search for strategically relevant assets to in-license

1 Safinamide, NW-3509 and Ralfinamide all developed from Newron's ion channel based research
2 Sarizotan was licensed from Merck KGaA

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

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

A progressing disorder, no cure available yet

- PD 2nd 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:
Parkinson's Disease – Global Drug Forecast and Market Analysis – Event-Driven Update -GlobalData, June 2015
Parkinson's Disease Foundation: Statistics on Parkinson's
Treatment of Advanced Parkinson's Disease, Varanese et al., 2010, NCBI

Significant Commercial Opportunity in Safinamide (Xadago®)

US / Canada

VALEO PHARMA®

US WorldMeds

FDA-Approved in March 2017
Launch expected in July 2017

EU

Zambon

Launched in Germany, UK, Italy, Spain and other EU territories, plus Switzerland

Latin America

Japan / Asia

meiji Eisai

Confirmatory Phase II/III and long-term Phase III studies initiated

Australia/New Zealand

Seqirus
A CSL Company

Partner to submit application for regulatory approval

- 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)

7 TO 10 million world wide

- **20** to **30** percent in early stage
- **70** to **80** percent in mid to late stage
- **>$4 Billion** worldwide market

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Rett Syndrome: Severe Neuro-developmental Orphan Disease with No Specific 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 July 2016

EFFECTS OF 14-DAY TREATMENT WITH SARIZOTAN IN RTT FEMALE MICE ($MECP2^{R168X/+}$)

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
- Will enroll minimally 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
- Centres 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
- Study enrolling
- Expected completion 2018

Sarizotan Market Opportunity Commercialization by Newron

Initiation of a Health Economic Outcome Research Study (HEOR)
→ ”burden of illness”

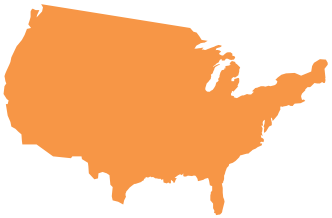
- Fostering partnership and collaborations with Rett advocacy, thought leaders & governing payers
- Global survey to quantify the ways in which patient “respiratory breathing abnormalities” affect daily life
- Meets Health Technology Assessment (HTA) requirements, including European Network of countries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

US 16,000 patients



Orphan exclusivity
7.5 years post approval

EU 20,000 patients

Orphan exclusivity
12 years post approval

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

No Effective Treatment that Reduces Burden of Schizophrenia in Last 20 Years

- Onset of disease occurs in early adulthood affecting 1% of the population worldwide
  - Need for life-long treatment
- 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
  - Typicals (e.g. haloperidol) worsen negative symptoms and cause neurological side effects
  - Efficacy of typicals and atypicals limited and wanes over 18 months; 60-70% of patients switch but without additional benefit
  - No effect on suicidality

Source: FiercePharma, 2011

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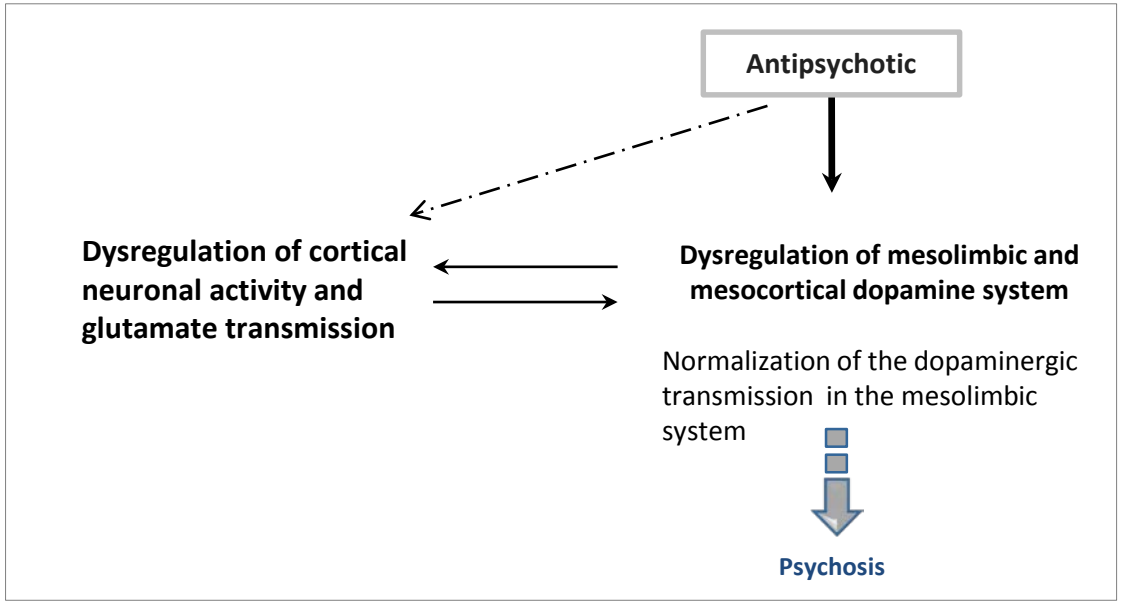
Unmet Medical Need with current antipsychotics

- Current antipsychotic drugs target the dysregulation of mesolimbic and mesocortical dopamine systems
- Reduced NMDAR activity on inhibitory neurons leads to disinhibition of glutamate neurons, increasing synaptic activity of glutamate especially in the prefrontal cortex
- **Abnormal cortical glutamatergic tone is not affected by existing drugs**



Effectiveness of current anti-psychotics in treating schizophrenia

- Findings From 3 Major Non-Commercial (CATIE , CUTLASS, and EUFEST) studies reveal significant dissatisfaction with all current antipsychotics:
  - Approximately 74% (range:64-82%) 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)
  - No differences between treatments (except clozapine) in extent of improvement in psychopathology as measured by PANSS, CGI, QLSS

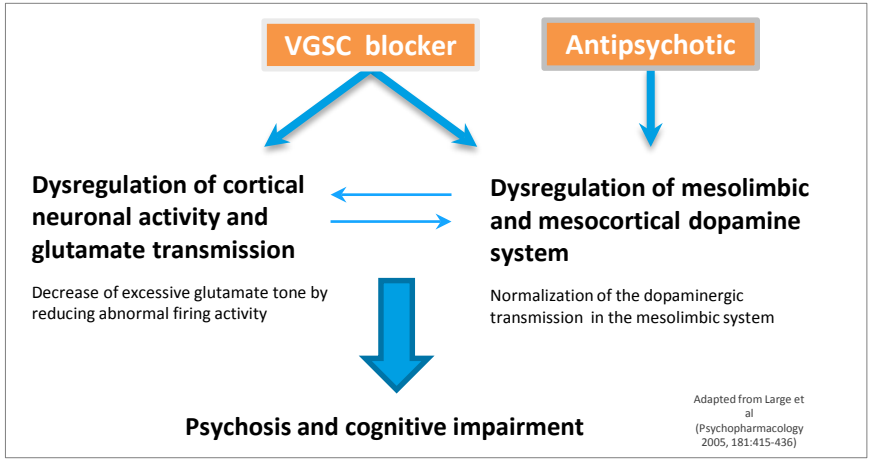
POSSIBLE REASONS INCLUDE:

- All these drugs have same/ similar mechanism of action, e.g. 5HT2/D2 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

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



Evenamide: Novel MOA to Benefit Poorly Responding Schizophrenia Patients

- First-in-class voltage-gated sodium channel (VGSC) blocker for add-on treatment in schizophrenia, schizo-affective and bipolar disorders
  - Small molecule, orally available, rapid onset of action, high availability in the brain
- Unique mechanism of action (MoA):
  - Selectively blocks VGSCs in a voltage- and use-dependent manner – no effect on dopaminergic, serotonergic, histaminergic neurotransmission
  - Modulates sustained repetitive firing without impairment of normal neuronal excitability
  - Reduces stimulated glutamate release
- Benefit shown in models of positive symptoms, aggression, cognition (schizophrenia), negative symptoms, mania, depression, obsessive behavior

- IND approval from FDA as ADD-ON TO ANTIPSYCHOTICS for patients with schizophrenia
  - Improvement of symptoms in patients worsening on standard treatment they had benefited from
- Well-tolerated in Phase I study
  - Exposure increased with dose; exposure achieved overlaps with plasma levels in animals at doses proven to be efficacious
- Phase IIa data in early 2017:
  - Consistent evidence of efficacy, good tolerability and safety
- Composition of matter – USPTO, 2013 - patent life 2028 plus extension

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Unique MOA Demonstrated

NW-3509, a selective Voltage-Gated Sodium Channel (VGSC) Blocker shows no effect on >130 CNS receptors, enyzmes, 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} (µM)

25

K_{inact} (µM)

0.4

High frequency firing

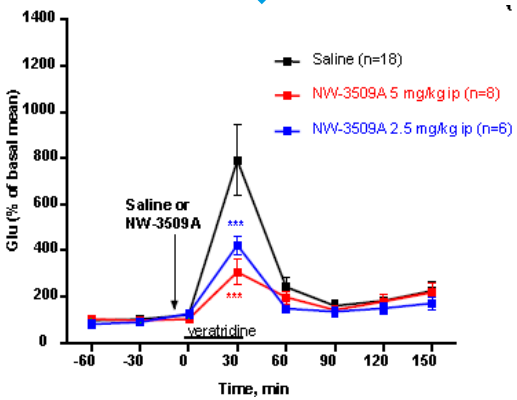
Control

NW-3509 1µM

Low frequency firing

Control

NW-3509 1µM



Evenamide is active in a wide range of schizophrenia and psychosis animal models as monotherapy and as add-on to antipsychotics

Information processing deficit	• 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) • *Pre-pulse inhibition (PPI) disrupted by Ketamine in rat (ongoing)*
Psychosis and Mania	• Amphetamine hyperactivity in mice • Amphetamine plus Chlordiazepoxide induced hyperactivity in mice
Cognitive impairment	• Novel object recognition in the rat: short term scopolamine impairment • Novel object recognition in the rat: long term 24 hr natural forgetting
Disruption of Impulse control and Mood symptoms	• Resident –Intruder test in mice (Impulsivity) • Tail suspension test in mice (Depression) • Marble burying test in mice (Obsessive Compulsive Disorders)
Negative symptoms	• PCP- induced deficit in Social Interaction in the rat • *Saccharin preference test (anhedonia) in prenatal poly:IC exposed mice (ongoing)* • *Three-chamber sociability test in prenatal poly:IC exposed mice (ongoing)* • *Forced swimming test (avolition) in prenatal poly:IC exposed mice (ongoing)*

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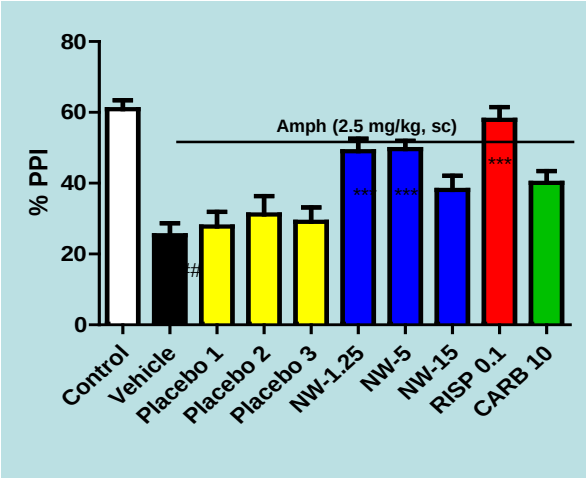
Monotherapy: Evenamide antagonizes amphetamine and MK-801-induced PPI deficits

PPI deficit induced by **amphetamine**

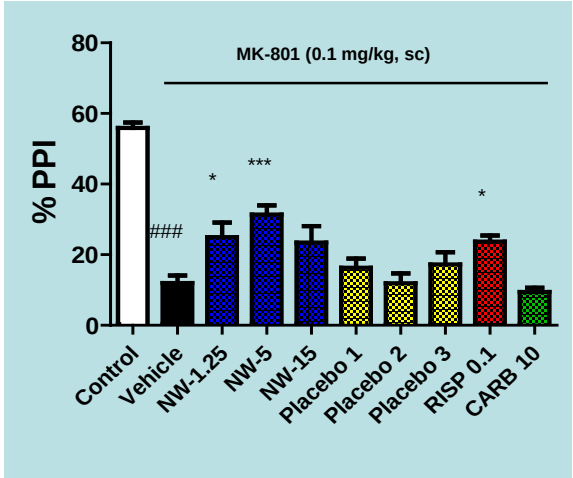
- evenamide minimal effective dose: 1.25 mg/kg *po*



*Tukey's test ***p<0.001 vs vehicle+ Amph; ###p<0.001 vs control (n=23-24 rats per group).*

PPI deficit induced by **MK-801**

- evenamide minimal effective dose: 1.25 mg/kg *po*



*Tukey's test *p<0.05, ***p<0.0001 vs vehicle+MK-801; ###p<0.001 vs control (n=27-47 rats per group)*

Activity as monotherapy demonstrated in other models

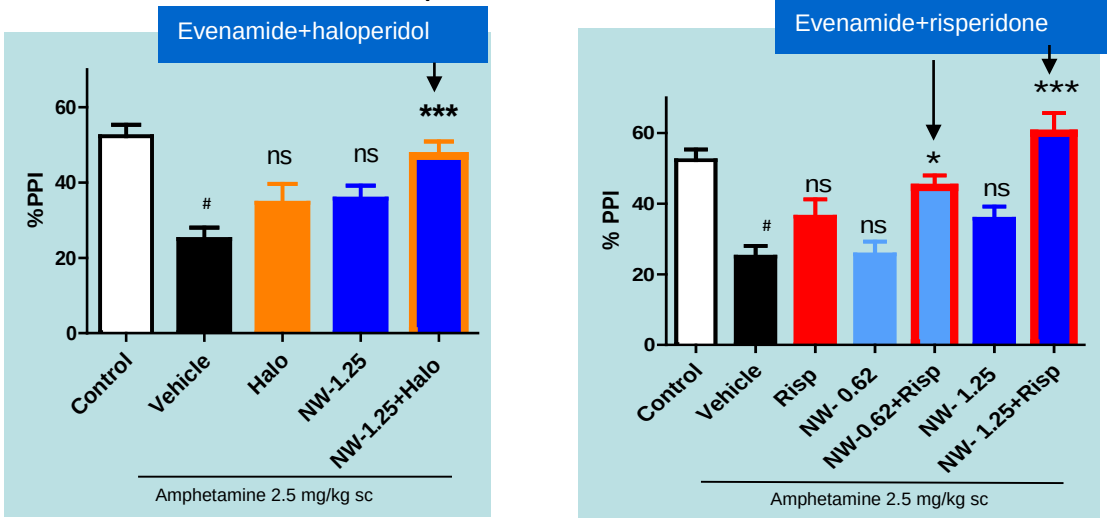
- ✓ PPI disrupted by sleep deprivation (rat)
- ✓ Pre-pulse inhibition spontaneous deficit (C57 mice)
- ✓ Amphetamine hyperactivity in mice
- ✓ Amphetamine plus Chlordiazepoxide induced hyperactivity in mice
- ✓ Novel object recognition in the rat: short term and natural forgetting
- ✓ Resident –Intruder test in mice
- ✓ Tail suspension test in mice
- ✓ Marble burying test in mice
- ✓ PCP- induced deficit in Social Interaction in the rat

Add-on: Evenamide augments the effect of typical and atypical antipsychotics

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

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

Amphetamine-induced PPI deficit



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

Evenamide – Study 02 in patients with chronic schizophrenia

DESIGN:

4-week, randomized, double-blind, placebo-controlled study evaluating the safety, tolerability, and preliminary evidence of efficacy of a dose range of NW-3509A 15 mg-20mg-25mg BID or placebo

Minimally 90 patients randomized in a 3:1 ratio to receive either NW-3509A or placebo
(amended to 1:1 late in the study)

Population:

Patients on risperidone (2 mg/day or higher) or aripiprazole (10 mg/day or higher) who are still symptomatic, despite ≥ **4 weeks** of treatment at a stable dose, and diagnosed ≥ 2 years ago; current symptoms present for at least one month

Total PANSS <80; Clinical Global Impression - Severity (CGI-S) rating of mildly, moderately, or moderately severely ill (score of 3, 4 or 5)

Patients with 1 or more core positive symptoms (hallucinations, delusions, excitement, suspiciousness/persecution and hostility) rated moderately severe or higher, or rating of moderate on more than 2 of these items, were excluded

Objectives:

Primary – Safety and tolerability

Secondary – Efficacy (PANSS positive, PANSS total, CGI-S and C, Level of Functioning [LOF])

Demographics and baseline characteristics

	Statistic	NW-3509 N=50	Placebo N=39	Total N=89
Age (Years)	Mean (SD)	43.5 (11.93)	44.4 (10.38)	43.9 (11.22)
Gender	Male [n (%)]	42 (84.0)	35 (89.7)	77 (86.5)
Weight (kg)	Mean (SD)	83.5 (17.37)	83.8 (19.76)	83.7 (18.35)
BMI (kg/m^2)	Mean (SD)	27.8 (5.27)	27.9 (5.12)	27.8 (5.17)
Duration of Current Episode of Schizophrenia (Months)	Mean (SD)	92.0 (115.8)	89.0 (127.5)	90.7 (120.4)
Number of Hospitalizations for Schizophrenia	Mean (SD)	3.3 (6.34)	2.5 (4.15)	2.9 (5.48)
PANSS Total Score	Mean (SD)	62.7 (6.51)	63.1 (8.60)	62.9 (7.42)
CGI-Severity	Mean (SD)	3.5 (0.50)	3.4 (0.50)	3.4 (0.50)
Concomitant Antipsychotic				
Risperidone	n (%)	40 (80.0)	29 (74.4)	69 (77.5)
Aripiprazole	n (%)	9 (18.0)	10 (25.6)	19 (21.3)

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Patient disposition (randomized population)

Disposition	NW-3509A (N=50) n (%)	Placebo (N=39) n (%)	Total (N=89) n (%)
Randomized	50	39	89
Completed study	42 (84.0)	38 (97.4)	80 (89.9)
Discontinued study	8 (16.0)	1 (2.6)	9 (10.1)
Adverse event	2 (4.0)	0	2 (2.2)
Non-compliance with study drug	1 (2.0)	0	1 (1.1)
Withdrawal by subject	4 (8.0)	1 (2.6)	5 (5.6)
Other	1 (2.0)	0	1 (1.1)

Most frequent (>5% of patients in any treatment group) and important TEAEs (safety population)

Preferred Term	NW-3509 (N=50)		Placebo (N=39)		Total N=89
	n (%)	Mod.	n (%)	Mod.	n (%)
At least one Serious AE	***5 (10.0)***		***1 (2.6)***		***6 (6.7)***
At least one TEAE	***23 (46.0)***		***12 (30.8)***		***35 (39.3)***
Somnolence	8 (16.0)		5 (12.8)		13 (14.6)
Insomnia	5 (10.0)	1	1 (2.6)		6 (6.7)
Headache	3 (6.0)	2	0		3 (3.4)
Overdose	3 (6.0)		1 (2.6)		4 (4.5)
Dry mouth	3 (6.0)		2 (5.1)		5 (5.6)
Diarrhoea	0		2 (5.1)		2 (2.2)
Pain in extremity	0		3 (7.7)		3 (3.4)
Cold sweat	1 (2.0)		0		1 (1.1)
Hyperhidrosis	1 (2.0)		0		1 (1.1)

Mod. = AEs of moderate severity

PANSS positive scale total score: mean value, change from baseline, and statistical analyses (mitt population)

	Mean Value				Mean Change from Baseline			
	NW-3509 (N=48)		Placebo (N=39)		NW-3509 (N=48)		Placebo (N=39)	
Visit	n	Mean (SD)	n	Mean (SD)	n	Mean (SD)	n	Mean (SD)
Baseline	48	14.8 (2.77)	39	14.7 (2.81)	--	--	--	--
Day 8	46	13.7 (2.49)	38	14.9 (2.87)	46	-1.2 (1.59)	38	0.1 (1.84)
Day 15	44	13.5 (3.06)	38	14.3 (2.96)	44	-1.4 (2.43)	38	-0.5 (1.81)
Day 22	42	12.6 (3.41)	38	13.6 (3.23)	42	-2.3 (3.03)	38	-1.2 (2.61)
Day 28	47	13.0 (3.60)	39	14.0 (3.79)	47	-1.9 (3.15)	39	-0.7 (3.08)

	Change from Baseline				Difference: NW-3509 vs. Placebo		
	NW-3509 (N=48)		Placebo (N=39)				
Day 28	n	LS Mean (SE)	n	LS Mean (SE)	LS Mean (SE)	(95% CI)	p-value
MMRM	47	-2.06 (0.439)	39	-0.87 (0.478)	-1.19 (0.643)	(-2.47, 0.09)	**0.0678**
ANCOVA (LOCF)	48	-2.31 (0.445)	39	-1.03 (0.477)	-1.28 (0.632)	(-2.54, -0.02)	**0.0459**
ANCOVA (OC)	43	-2.51 (0.454)	38	-1.03 (0.475)	-1.48 (0.641)	(-2.76, -0.20)	**0.0237**

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PANSS positive scale: proportion of patients rated as improved[#] from baseline (M-ITT population)

Visit	NW-3509 n/n (%)	Placebo n/n (%)	p-value*
TOTAL	(N=48)	(N=39)	
Day 8	28/46 (60.9)	11/38 (28.9)	**0.0044**
Day 15	29/44 (65.9)	14/38 (36.8)	**0.0143**
Day 22	31/42 (73.8)	20/38 (52.6)	0.0638
Day 28/Endpoint	35/47 (74.5)	17/39 (43.6)	**0.0043**

[#]Improvement = PANSS Positive Score change from baseline less than 0 (reduction in score = improvement)

*p-value for Fisher's Exact chi-square test

CGI-C: PROPORTION OF PATIENTS RATED AS IMPROVED[#] FROM BASELINE (MITT POPULATION)

Visit	NW-3509 (N=50) n/n (%)	Placebo (N=39) n/n (%)	p-value*
Day 8	15/46 (32.6)	6/38 (15.8)	0.0845
Day 15	21/44 (47.7)	8/38 (21.1)	**0.0198***
Day 22	24/42 (57.1)	14/38 (36.8)	0.012
Day 28/Endpoint	26/46 (54.2)	14/39 (35.9)	0.0855**

*p-value <0.05, **p<0.1 for Fisher's Exact chi-square test

[#] Improvement = Rating of 1, 2 or 3 (very much, much or minimally improved, respectively)

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Summary of other efficacy results

Baseline Value and Mean Change from Baseline at Day 28								
	Baseline Value				Change from Baseline to Day 28			
	NW-3509 (N=48)		Placebo (N=39)		NW-3509 (N=48)		Placebo (N=39)	
Scale	**N**	**Mean (SD)**	**n**	**Mean (SD)**	**n**	**Mean (SD)**	**n**	**Mean (SD)**
PANSS Total	47	57.8 (9.66)	39	59.3 (10.81)	47	-5.1 (9.67)	39	-3.7 (9.65)
CGI-S	47	3.1 (0.68)	39	3.2 (0.77)	47	-0.3 (0.60)	39	-0.2 (0.74)
LOF Total	48	22.04 (3.608)	39	20.64 (4.533)	47	0.72 (3.321)	39	0.31 (3.130)

Proportion of Responders [n/n (%)] at Day 28					
Scale	**Responder Criterion**	**N**	**NW-3509**	**N**	**Placebo**
PANSS Positive	Change from baseline less than 0 (reduction in score = improvement)	50	35/47 (74.5)*	39	17/39 (43.6)
CGI-C	Rating of 1, 2 or 3 (very much, much or minimally improved, respectively)	50	26/46 (56.5)**	39	14/39 (35.9)

*p < 0.05 vs. placebo, **p<0.1 for Fisher's Exact chi-square test

CONCLUSIONS

- Analyses indicate significance/trends in favor of NW-3509 for PANSS positive scale total (p=0.051; 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 that patients who were younger (< 32 yrs) and earlier in the course of their disease (< 10 yrs) experienced greater improvement
- Results are consistent with hypothesis that NW-3509 add-on will improve symptoms of psychosis in patients who are not responding adequately to standard antipsychotic treatment
- Physiological modelling predicted that mean plasma concentrations of >40 ng/ml would be efficacious: this was confirmed in this study at doses of 15-25 mg bid

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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

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NEXT STEPS

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

Design and conduct of adequate and well-controlled study to demonstrate efficacy and safety/tolerability of fixed doses of Evenamide as add-on to antipsychotics in patients experiencing worsening of symptoms of schizophrenia

Global, 12-week study requiring approx. 360 patients randomized (1:1:1) to Evenamide (15 and 30 mg BID) or placebo

- Male and female (not of childbearing potential) outpatients; ages 18-55 yrs
- Diagnosis of schizophrenia (DSM-5) ≤ 6 yrs prior; current symptoms present ≤ 6 mo.
- PANSS total score >70; CGI-S – mildly ill or greater; score of 13 or higher on the following core symptoms of psychosis: hallucinatory behavior, delusions, suspicious/persecution, unusual thought content (on PANSS)
- Receiving a stable dose (> 4 weeks prior to screening) of an oral atypical antipsychotic (risperidone, olanzapine, lurasidone, ziprasidone, paliperidone, or aripiprazole)

Investment Highlights

1. Diversified Portfolio of Innovative CNS Product Candidates
2. Xadago® - Commercialized in 12 European Countries, US launch announced for July 2017
3. Sarizotan for Rett Syndrome in Late Stage Development
4. Evenamide - a Novel Mechanism / Treatment Paradigm for Schizophrenia
5. Multiple Catalysts on the Horizon
6. Management Team with Proven Track Record

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