Newron Pharmaceuticals - Developing novel treatments for CNS diseases and pain (SIX: NWRN)

Stefan Weber
CEO

Jefferies 2016 Healthcare Conference
New York, June 7, 2016

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Newron Pharmaceuticals: Developing treatments for patients with debilitating Central Nervous System diseases

- **Parkinson's disease: Xadago®** (safinamide)
  - Marketing Authorization in the EU and Switzerland in 2015
  - Launched in 8 European markets
  - US: Response to FDA CRL letter submitted; meeting planned
- **Schizophrenia: Evenamide®** (NW-3509)
  - Phase II placebo-controlled study in schizophrenic patients ongoing
- **Rett syndrome: Sarizotan**
  - Phase III potentially pivotal study approved by FDA and EMA
  - Study start in Q3/2016

Innovative Therapies for CNS Disease and Pain

Products	Preclinical	Phase I	Phase II	Phase III	Market	Commercial Rights
Xadago® (safinamide)[1]						
Adjunctive therapy in PD						Zambon
Adjunctive therapy in PD						US Worldmeds
Adjunctive therapy in PD						Meiji Seika
Evenamide (NW-3509)[1]						Newron
Schizophrenia						
Sarizotan[2]						Newron
Rett syndrome (Orphan drug status)						
Ralfinamide[1]						Newron
Orphan indication in neuropathic pain						

[1] Safinamide, NW-3509 and Ralfinamide all developed from Newron's ion channel based research

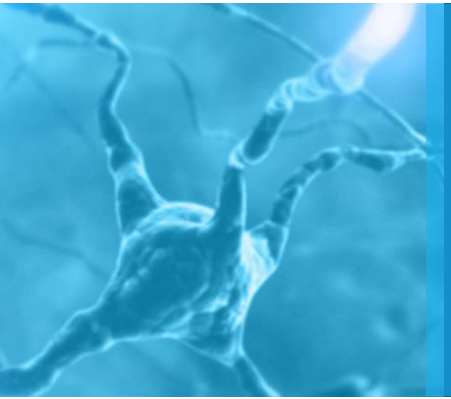
[2] Sarizotan was licensed from Merck Germany

Achievements in Past 12 Months

- **Xadago® (safinamide)**
  - Launched in 7 EU countries and Switzerland by Zambon
  - CRL received on March 29, 2016 (PDUFA date)
  - Response submitted in May, 2016, meeting planned
  - Phase III study initiated in Japan (Meiji Seika Pharma)

- **Sarizotan**
  - ODD for the treatment of patients with Rett syndrome in EU and U.S.
  - IND approved May 2016
  - International Phase III potentially pivotal study planned in patients with Rett syndrome

- **Evenamide (NW-3509)**
  - US Phase II study started and ongoing in patients with positive symptoms of schizophrenia



Xadago® (safinamide): First NCE Approved for PD in a Decade

Products	Preclinical	Phase I	Phase II	Phase III	Market	Commercial Rights

Safinamide

- Adjunctive therapy in PD
- Adjunctive therapy in P
- Adjunctive therapy in PD

Zambon
US WorldMeds
Meiji Seika

Xadago® (safinamide): Treatment of patients with PD

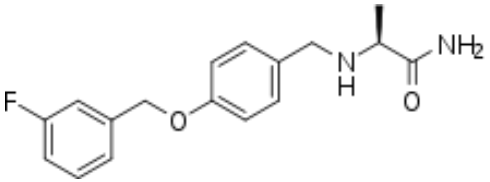
- Parkinson's disease (PD) affects 7 -10 million people worldwide
- Degenerative disorder of the CNS impairing patients' motor skills, cognitive processes, behaviour and speech
- Standard of treatment is with levodopa, with or without additional of dopamine agonists
- Treatment with L-dopa leads to motor fluctuations (ON or OFF-time) and involuntary movements known as L-dopa-Induced Dyskinesia (LID)

Xadago® (safinamide): First NCE Approved for PD in a Decade

First PD therapy working through dual mechanism

Once daily oral adjunctive therapy for patients with PD

- Alpha-amino amide derivative, high solubility and bioavailability
- Early onset of action and benefits seen in patients for over 2 years
- Significant increase in “Responder Rates” compared with standard of care
- Current PD treatments only enhance dopaminergic function
- Xadago (safinamide)
  - ✓ Enhances dopaminergic function
  - ✓ Reduces glutamatergic release (with potential to reduce dyskinesia)



Efficacy and safety demonstrated as add-on to

- Dopamine agonists (early PD)
- L-dopa (mid to late stage PD)

Xadago® (safinamide) Offers Multiple Benefits to PD Patients

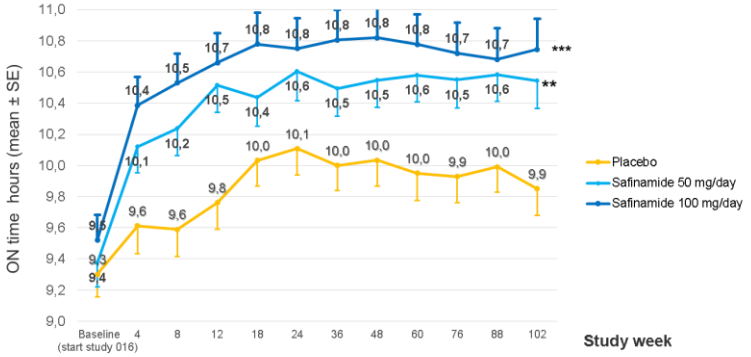
Early PD Patients – add to dopamine agonist

- Significant improvement of
  - UPDRS III - motor function, regulatory endpoint (mean change, responder rate)
  - Quality of life (PDQ-39, EQ5D)
- Reduction of number of interventions (first time use of L-dopa)
- Benefits seen after 6 and 18 months
- Delay levodopa

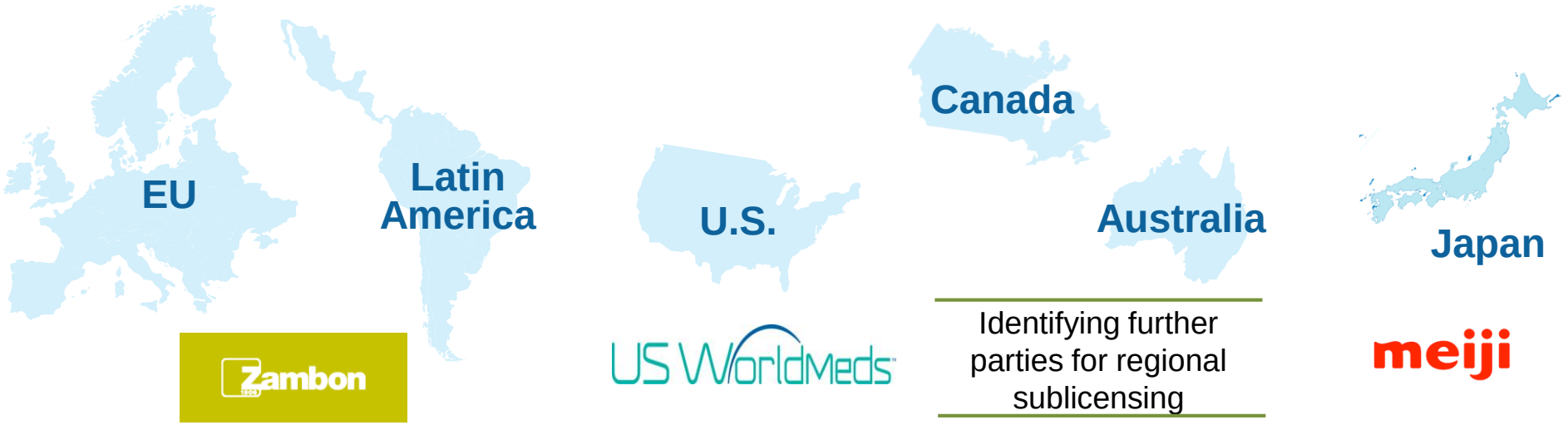
Mid- to late-stage PD Patients – add to dopamine replacement

- Significant improvement of
  - ON Time/OFF Time – regulatory endpoint
  - UPDRS II – activities of daily living/ UPDRS III – motor function
  - UPDRS IV – treatment complications
  - CGI (Clinical Global Impression) – Severity and Improvement
  - Increase in clinically significant response (ON time, OFF time, UPDRS III), response rates over standard of care
- Additional ON Time Without Any Increase In Any Dyskinesia
- Dyskinesia significantly improved
- Benefits seen after 6 and 24 months

Long Term Duration of Effect ON Time (without troublesome dyskinesia) - Change from Baseline



Xadago® (safinamide): Commercial Opportunity



Zambon

US WorldMeds

Identifying further parties for regional sublicensing

meiji

Milestone and royalty revenues to Newron since 2012

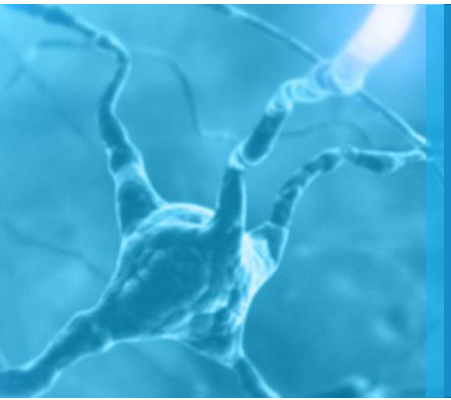
Long lasting market exclusivity (patent life: 2029 in EU, 2030 in the U.S.)

Peak sales potential $450m - $700m+ (*analyst estimates*)

Newron Pharmaceuticals

Milestones

- Xadago® (safinamide) has already been launched in (Germany, Switzerland, Italy, Spain, Belgium, Denmark, Sweden, UK)
- Further EU launches expected in 2016
- On US PDUFA date (March 29, 2016), Newron received Complete Response Letter
  - No need for additional efficacy or safety studies
  - Assessment of abuse liability and dependence/withdrawal effects needed
- Type A meeting with FDA/CSS to confirm Newron's response to CRL
- Xadago® (safinamide) has the potential to assist the more than one million Americans currently living with Parkinson's disease



Evenamide (NW-3509) – Voltage gated sodium channel blocker for Schizophrenia

Products	Preclinical	Phase I	Phase II	Phase III	Market	Commercial Rights
Evenamide (NW-3509) Schizophrenia						Newron

Evenamide (NW-3509) for treatment of symptoms of Schizophrenia

- Onset of disease occurs in teenage years in males, and 5 years later in females: need for lifelong treatment: affects 1% of the population worldwide*
- Disease characterized by hallucinations, delusions, paranoia and disorganised speech
- Progressive deterioration of cognition, behaviour, thought disorder, and presence of negative symptoms
- High rates of suicidality, suicide, multiple physical illnesses, and lower life expectancy (at least 10 years on average)
- No new effective treatment that reduces burden of disease in last 20 years: large market opportunity (anti-psychotic market >$23bn)

1. Kendler et al. Arch Gen Psychiatry 1996; 53: 1022–1031
2. World Health Organization. The World Health Report: 2001: Mental health: new understanding, new hope

Evenamide (NW-3509) acts by a new mechanism, improving treatment of Schizophrenia

- First in class voltage-gated sodium channel (VGSC) blocker for add-on treatment in schizophrenia, schizo-affective and bipolar disorders
- Novel small molecule, oral available, rapid onset of action, high availability in the brain
- Benefit shown in models of positive symptoms, aggression, cognition (schizophrenia), negative symptoms, mania, depression, obsessive behavior
- Potential to benefit patients showing inadequate response to current antipsychotics
- Phase I study completed
  - Drug was well tolerated
  - Exposure increased with dose
  - Exposure overlaps with exposure in animals at doses proven to be efficacious
- Phase II placebo-controlled study ongoing
- Composition of matter – USPTO, 2013 – Patent life 2028 plus extension

Unique MOA: selective Voltage-Gated Sodium Channel (VGSC) Blocker

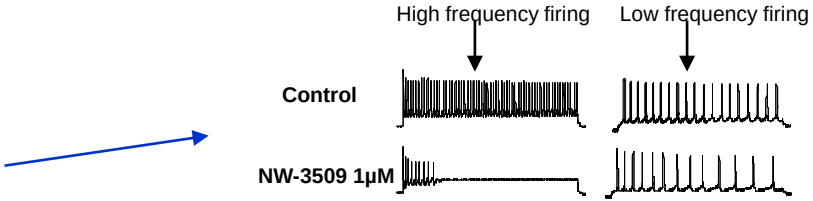
Evenamide (NW-3509)

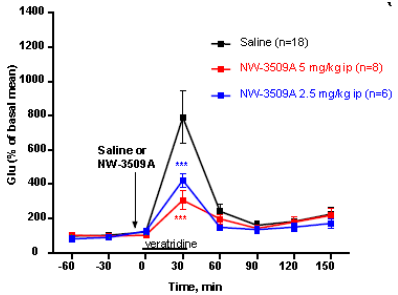
Selectively blocks VGSCs in a voltage-and use-dependent manner

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

Inhibits Glutamate Release

Inhibition of naive sodium channels expressed in rat cortical neurons	
K_{rest} (μM)	K_{inact} (μM)
25	0.4

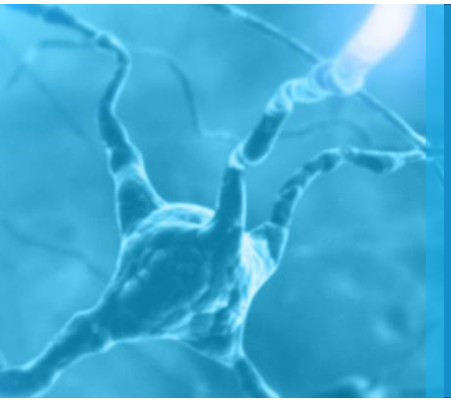




Milestones

Phase II placebo-controlled study with Evenamide (NW-3509) as add-on in positive symptoms of schizophrenia

- NW-3509 as add-on treatment to patients with 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 minimally 90 patients receiving Evenamide (NW-3509) 15-25 mg/daily (given BID) or placebo
- Selection Criteria:
  - Current diagnosis of schizophrenia in accordance with DSM-5
  - PANSS (Total) < 80; CGI-S rating of mildly, moderately, or moderately severely ill
  - Excludes patients with hallucinatory behavior, excitement, delusions, suspiciousness/persecution and hostility
  - Endpoints: Symptoms of schizophrenia, as assessed by
    - Positive and Negative Syndrome Scale (PANSS)
    - Clinical Global Impression - Change from baseline (CGI-C) and CGI - Severity of illness (CGI-S)
- Enrollment started: Jan 2016, results expected by end 2016

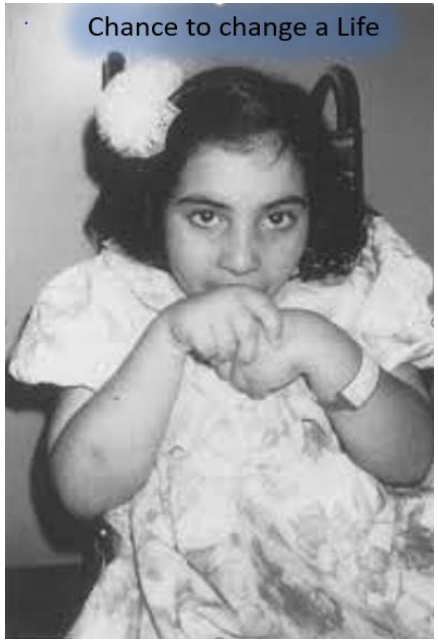


Sarizotan – Targeting respiratory disturbances in Rett syndrome

Products	Preclinical	Phase I	Phase II	Phase III	Market	Commercial Rights
Sarizotan Rett syndrome						Newron

Sarizotan for treatment of patients with Rett Syndrome

- Severe neurodevelopmental disorder primarily affecting females (1:10,000)
- Mutations in X-linked methyl CpG-binding protein 2 in majority of patients
- Causes severe disability, reduces life expectancy
- Normal development until 6-18 months of age, then lose fine motor skills, ability for social interaction, encounter cardiorespiratory dysregulation
- 60% survival at 37 years (vs. appr. 98%)
- Approx 70% of patients demonstrate respiratory abnormalities e.g. apnea, hyperventilation, respiratory dysrrhythmia
- 25% of sudden deaths in Rett linked to cardio-respiratory abnormalities
- Medication needed for breathing irregularities, motor difficulties, seizures' control (anti-convulsant)
- No specific treatment approved for Rett Syndrome; focus on symptom management



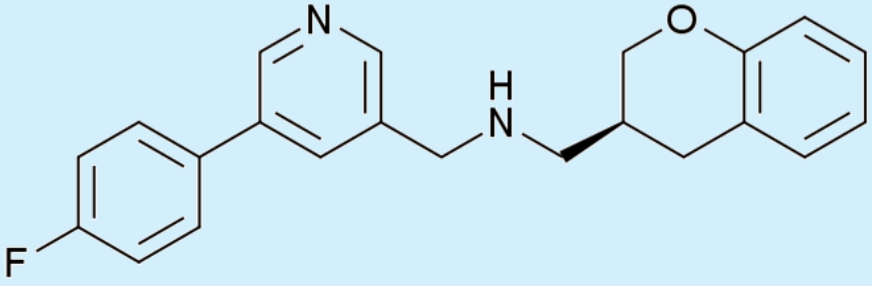
Sarizotan has Potential to Treat Respiratory & Associated Symptoms

- Aminomethyl chromane derivative; new chemical entity
- Breathing disturbance in Rett syndrome postulated to involve neuronal hyperactivity in the brainstem (Raphe nucleus, Kölliker-Fuse nucleus, Bötzinger complex)
- Dramatic effect demonstrated on respiration in null mutant MeCP2 mouse model of Rett syndrome
- Potential additional benefits in other core features of Rett syndrome

Behavior

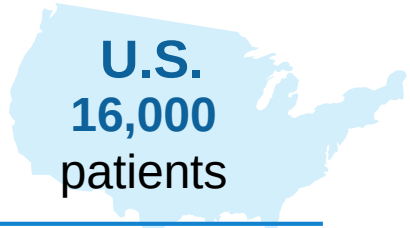
Cognition

Neurological deficits



Strategy for Regulatory Approval and Commercialization

- EU (Germany, Spain, UK) Health Authorities accepted proposed CMC/preclinical/clinical safety data package, agreed to single pivotal study: Q2/2015
- Similar agreements with Canadian (TPD) and U.S. (FDA): Q2-3/2015
- Orphan Drug Designations obtained in EU and U.S.: July 2015
- Efforts ongoing to extend treatment to younger patients: Interaction with EU Pediatric Development Committee Q3/2015
- Advocacy relationships being developed; Rett foundations for potential funding/co-sponsorship of activities
- ‘Rare Pediatric Disease’ voucher possibility



Orphan exclusivity
7.5 years post approval

Orphan exclusivity
12 years post approval

Sarizotan: Next Steps

- First ever Phase III potentially pivotal study in Rett syndrome (US IND); start in Q3/2016:
  - Double-blind, randomized placebo-controlled, 28 week, multi-center design in approx. 120 patients
  - Primary endpoint: Reduction in number of objectively defined apnea episodes
- Initiation of Global Burden of Illness Caregiver Outreach Program
- Partnering within the Rett community to solicit the views of caregivers in support of the unmet medical need (impact on health-related or general quality of life & resource utilization)
- Health Economic Outcome Research study (HEOR) → “Burden of Illness” will support Health Technology Assessment (HTA); meets European Network of countries requirements to support pricing & treatment access

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Milestones / News Flow

2016 / 2017

Xadago® (safinamide) in PD

- Market approval/launch
  - EU next launches: HY 2/2016
  - U.S. Meeting with CSS: **Q3/**2016

Evenamide (NW-3509) in Schizophrenia

- Phase II results **Q4/**2016
- License transaction

Sarizotan in Rett syndrome

- Phase III pot. Pivotal efficacy study initiate: **Q3/**2016
- Results from Phase III pot. pivotal study: 2017
- Commercialization by Newron: 2018

Financial Snapshot

Shares Outstanding
14,432,911

Market Cap
240 Million CHF

52-week
High: 31.35 CHF
Low: 15.00 CHF

Liquidity of Stock
42,000/day
average trading volume (6mth)

Analyst Coverage
- Charles Duncan, Piper Jaffray
- Bob Pooler, Valuation Lab
- Samir Devani, Rx Securities
- Susie Jana, Edison

Key Shareholders
- Investor AB
- Zambon
- Aviva
- JPMorgan AM
- Swisscanto
- Polar Capital
- Sphera Global Healthcare
- Nyenburgh
- Abingworth

Newron Pharmaceuticals: Highlights

- Innovative treatments for patients with debilitating CNS diseases with unmet medical needs
- Material revenues from Parkinson's disease drug
- Pipeline opportunities
  - First therapy to be approved for patients with Rett Syndrome – to be commercialized by Newron
  - First add-on therapy to patients with positive symptoms of schizophrenia
- Experienced management team – CNS specialists
- Solid financial position

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