

Axovant Sciences (AXON - \$12.62)

Initiation of Coverage – Currently More Risk than Reward?

We are initiating coverage of Axovant Sciences with a Sell rating and a \$7.50 price target. AXON caught everyone's attention in June 2015 with one of the largest biotech IPOs (\$334.5M net raise) during the most recent biotech bull market with a 5HT6-antagonist intepirdine (RVT-101) to treat mild to moderate Alzheimer's disease (AD) that was bought by AXON from GlaxoSmithKline (GSK) for \$5M upfront. Although AXON's valuation has since shrunk from ~\$2.9B to ~\$1.3B, we believe the stock could still be overvalued due to valuations of similar competitors, the remarkable history of failures (~99% failure rate) in the space - highlighted by the recent discontinuation and failures of Pfizer's and Lundbeck's 5HT6 receptor antagonists – and the lack of short term impact of the recent additions to the AXON pipeline. We see AXON as a binary AD story in a field plagued by disappointments and believe the current ~\$1.3B valuation may not adequately reflect the risk in the story at this point. We are initiating with a Sell rating and a \$7.50 price target.

- **Valuation appears to price in the reward over the risk.** AXON raised \$334.5M in, one of the largest biotech IPOs in recent memory and it valued the company at \$2.9B. While the valuation is now ~\$1.3B we believe this may price in more of the potential reward for success than the risk of potential hiccups. AXON's comps are around ~\$300M each, and there have been 100 AD failures from 1998-2011. There may be more risk yet to the AXON story.
- **Competitor read-throughs cloud an already murky field.** Two significant competitors to intepirdine (PFE's PF-05212377 and LUN.CO's idalopirdine) with similar activity (5HT6-antagonist) have so far failed to hit their clinical endpoints. This could be due to receptor occupancy (RO) and activity discrepancy between the various drugs, but we would caution that the potential read through to AXON's intepirdine could be real.
- **Growing pipeline diversification adds complexity.** AXON has recently substantially grown its pipeline by adding three products targeting additional indications in dementia. We believe the diversification could come at the cost of losing focus on the main asset (in our opinion) of intepirdine for AD.
- **Initiate with a Sell rating, \$7.50 PT.** Our PT is based on a sum-of-the-parts with intepirdine: \$5/share; nelotanserin: \$1/share, cash (end-'18) and tech value (including RVT 103 & 104): \$1.50/share.

Adj-Earnings Estimates: (per share)

| March FY | 1Q | 2Q | 3Q | 4Q | FY | P/E |
|--------------|---------|---------|--------|--------|--------|-----|
| FY18E | (0.33) | (0.35) | (0.37) | (0.40) | (1.45) | NA |
| FY17E | (0.27)A | (0.35)A | (0.28) | (0.30) | (1.20) | NA |
| FY16A | (0.11) | (0.10) | (0.25) | (0.21) | (0.64) | NA |
| FY15A | - | - | - | - | (0.80) | NA |

Source: Laidlaw & Company estimates

Healthcare / Biotechnology

Ticker: **AXON**
Rating: **Sell**
Price Target: **\$7.50**

Trading Data:

| | |
|---------------------------|-----------|
| Last Price (12/19/2016) | \$12.62 |
| 52-Week High (12/30/2015) | \$19.70 |
| 52-Week Low (02/09/2016) | \$8.86 |
| Market Cap. (MM) | \$1,251.4 |
| Shares Out. (MM) | 99.2 |

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Key Reasons to Sell Axovant Sciences

- **Competitor market caps and the long history of failure in the AD space makes us concerned about \$1B+ market caps at this stage.** We believe that even at AXON's current levels, investors aren't appreciating the discrepancy between them and their peer group in the space. VTV therapeutics (VTVT) is at ~\$180M cap with an AD candidate in Phase 3 and T2D in Phase 2. Lundbeck (LUN.CO) had a valuation of ~\$7.9B prior to the failure of its Phase 3 5HT6/2a antagonist for AD, while recording ~\$2.15B in sales in 2015. As recently as January 2016 Acorda Therapeutics (ACOR) made a tender offer for Biotie Therapies for ~\$363M for that company's Phase 3 Parkinson's Disease (PD) asset and ac5HT₆/5HT_{2A} antagonist in Phase 2 for PD and Phase 1 for AD. Additionally the AD field is replete with failures of multiple drugs testing all manner of AD hypothesis. Independent studies have shown the failure rate for Phase 2 and Phase 3 AD drugs to be in the daunting 96%+ range over a 10-year period. We agree that AXON is somewhat different – they are looking to supplement an already approved AD treatment (Aricept) rather than a de-novo stand-alone treatment, but this shocking failure rate still gives us pause.
- **Difficult to ignore the Pfizer, Lundbeck, and most recently Lilly failures.** While Pfizer (PFE) discontinued their Phase 2 trial for PF-05212377 (a 5HT6 antagonist similar to AXON's) in Oct'15, PFE only updated the NIH's ClinicalTrials.gov in February 2016 – sending AXON's share price down ~25%. In September 2016 Lundbeck (LUN.CO) also had a 5HT6/2a antagonist failure, this time in Phase 3. This caused AXON to decline ~12%. After both failures AXON (correctly) noted that lower receptor occupancy (RO), a lack of 5HT_{2a}, and potentially the wrong patient populations could have caused PFE's discontinuation. There are real differences between AXON's intepirdine and LUN.CO's 5HT6/2a antagonist as well, including RO and trial drop-out rates, but we believe at AXON's current valuation this may be a case of ignoring what seems to be yet another real signal of risk in the 5HT6 antagonist mechanism. Lilly's (LLY) failure was in the amyloid beta plaque hypothesis, a different mechanism of activity entirely, but it is a high profile failure in what is the most popular, most invested-in AD hypothesis. If companies on the scale of PFE, LUN.CO, and even LLY can fail in such large & expensive trials (LLY for the 3rd time!) it does raise the question of whether pharma - or even science in general - has a grasp on what the underlying cause of AD is, much less how to successfully (or even partially) treat it.
- **Diversification of Dementia Pipeline could dilute focus from key intepirdine asset.** AXON has recently announced many significant expansions to their pipeline (see clinical timeline chart later in this report), we believe the uncertainty in the space continues to place the majority of the value at AXON on the upcoming intepirdine upcoming Phase 3 MINDSET data in 2H17. While AXON's new asset nelotanserin (5HT_{2a} inverse agonist) is slightly de-risked by the recent approval of Acadia's (ACAD) pimavanserin (another 5HT_{2A} inverse. agonist), we believe investors continue to place the majority of value intepirdine as other programs remain in early Phase 2 or Phase 1 proof-of-concept stages.

Figure 1: Upcoming Potential Catalysts

| Event | Expected Timing (CY) |
|--|----------------------|
| Intepirdine for AD MINDSET phase 3 data | 2H17 |
| Intepirdine for DLB HEADWAY phase 2b data | 2H17 |
| Intepirdine for Gait and Balance impairments phase 2 data | 2H17 |
| Nelotanserin for VH in LBD phase 2 data interim data/full data | 1Q17/3Q17 |
| Nelotanserin for RBD in DLB | 2H17 |
| RVT-103 for AD phase 2 | 2H17 |
| RVT-104 for LBD | 2H17 |

Source: Company reports; Laidlaw and Company estimates

Valuation

Figure 2: Sum-of-the-Parts Analysis

| Sum-of-the-parts valuation | | |
|---------------------------------|----------------------|--------------------|
| Segment | Valuation (000's) | Per share value |
| Intepirdine US sales | \$440,442 | \$4.00 |
| Intepirdine EU royalties | \$92,927 | \$1.00 |
| Nelotanserin | \$126,017 | \$1.00 |
| RVT-103 & 104 | \$41,735 | \$0.25 |
| Cash (end of '18E) and tech | \$144,161 | \$1.25 |
| Total | \$845,284 | \$7.50 |
| 2018 fully diluted shares (000) | | 107,110 |

Source: Company reports; Laidlaw and Company estimates

Company Description

Axovant Sciences (AXON) was founded in 2014 as a wholly-owned subsidiary of Roivant Sciences, Inc. (RSL). AXON is a clinical-stage biopharmaceutical company developing novel therapeutics for the treatment of dementia including Alzheimer's Disease (AD), Lewy Body Dementia (LBD) and a subset of LBD known as Dementia with Lewy Bodies (DLB), and Parkinson's Disease Dementia (PDD). Their near-term focus is to develop their lead product, intepirdine (previously RVT-101), a selective 5-HT₆ receptor antagonist for the treatment of Alzheimer's disease and dementia with Lewy bodies. Additionally AXON has recently started development on nelotanserin, their second product candidate, which is a selective 5-HT_{2A} receptor inverse agonist for the treatment of visual hallucinations in patients with Lewy body dementia and REM behavior disorder (RBD) in patients with DLB. Longer term AXON plans to develop a broader pipeline of product candidates to address a broad range of cognitive, behavioral, and functional aspects of dementia.

AXON completed their IPO in June 2015, raising \$334.5M. AXON is 75.6% owned by RSL.

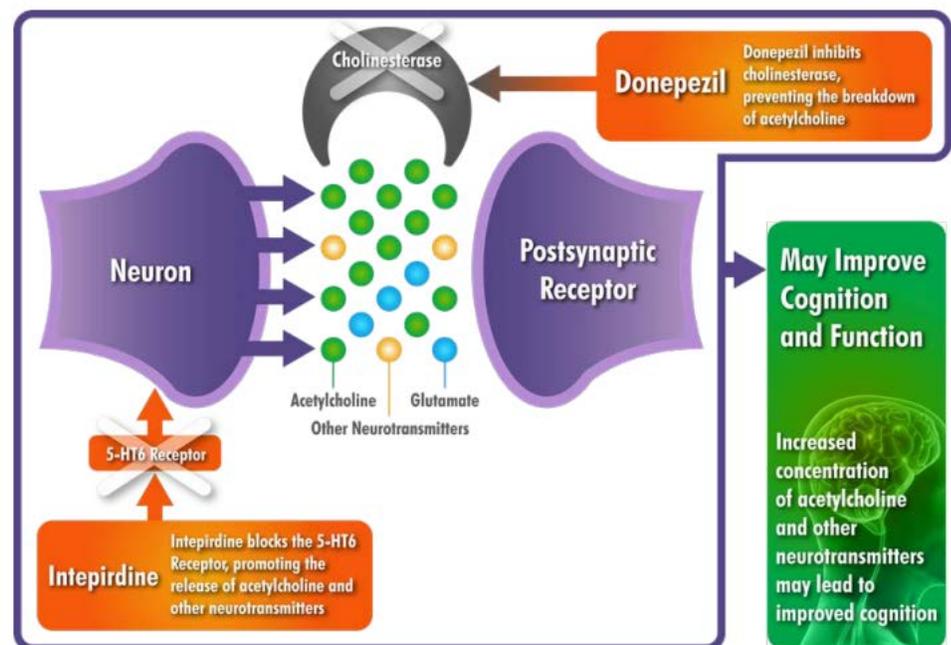
Intepirdine (RVT-101)

Intepirdine for Alzheimer's Disease

AXON's lead product candidate intepirdine (RVT-101) is currently being developed for the treatment of mild-to-moderate Alzheimer's disease (AD) and Dementia with Lewy Bodies (DLB). AXON acquired the worldwide rights to intepirdine from GlaxoSmithKline (GSK) under an asset purchase agreement entered into in December 2014 where AXON paid a remarkably conservative \$5M up-front, then \$5M in June 2016. AXON will pay an additional \$70M in approval milestones for US, EU & Japanese approvals, a 12.5% sales royalty and a one-time \$85M payment should global intepirdine sales reach \$1.2B.

Intepirdine is an orally administered potent antagonist of the 5-HT₆ receptor. By antagonizing the 5-HT₆ receptor, intepirdine promotes the release of key neurotransmitters including acetylcholine. These neurotransmitters are believed to be critical for alertness, memory, thought and judgment, which are the key components of cognition and function that are impaired in patients with dementia. AXON believes that intepirdine's action as a 5-HT₆ receptor antagonist supports its use in combination with cholinesterase inhibitors. While cholinesterase inhibitors help prevent the breakdown of acetylcholine, 5-HT₆ receptor antagonists promote the release of acetylcholine. Therefore, when used in combination with one another, they believe that 5-HT₆ receptor antagonists and cholinesterase inhibitors may increase the concentration of acetylcholine through complementary mechanisms. 5-HT₆ receptors are primarily localized to the central nervous system (CNS), particularly in regions of the brain that modulate cognition.

Figure 3: Intepirdine + Aricept (Donepezil) Proposed Mechanism of Action



Source: Company Documents

AD, the most common form of dementia, is a progressive neurodegenerative disorder that results in significant impairments in cognition, function and behavior. More than five million Americans are living with AD and along with other dementias, it is projected to cost the nation ~\$236B in 2016. It is also believed that 70% to 90% of AD patients are at or above 65 years old and are classified as mild-to-moderate AD patients (Alzheimer's Association, 2016). No new chemical entity has been approved by the FDA for the treatment of AD since 2003.

AXON plans to develop intepirdine for use in combination with donepezil (Aricept). Aricept by Eisai and Pfizer, is the most commonly used cholinesterase inhibitor. Cholinesterase inhibitors are the current standard of care for the treatment of AD, and the only class of drugs approved by the FDA for the treatment of patients with mild AD. Based on preclinical and clinical data collected to date, AXON believes the intepirdine and Aricept combination could work additively or synergistically to increase the concentration of acetylcholine for improved cognition and function. Although AXON believes intepirdine could be the best-in-class 5-HT₆ receptor antagonist for AD because of its safety, tolerability and efficacy profile for up to 48 weeks as observed in a 684-subject randomized placebo-controlled Phase 2b trial completed by GSK in 2011, we note that its miss at 36 weeks could point to weak efficacy. However, we do agree with AXON that intepirdine has a number of favorable properties, such as once daily dosing, a low potential for drug interactions, and an ability to be administered with or without food.

Before getting into the GSK's data, here is a table of acronyms that could be helpful to refer to throughout the text.

Figure 4: AD Acronyms and Descriptions

| Domain/Scale | Description |
|--|--|
| Cognition | Memory, orientation, language, praxis, etc. |
| Mini-Mental State Exam (MMSE) | 30-pt. scale (higher scores better) Clinician administered patient evaluation Mostly used for eligibility screening and dementia staging |
| Alzheimer's Disease Assessment Scale-cognitive subscale (ADAS-cog) | 70-pt. scale (higher scores worse) Clinician administered patient evaluation Standard cognitive outcome measure in mild-moderate AD |
| Severe Impairment Battery (SIB) | 100-pt. scale (higher scores better) Clinician administered patient evaluation Cognitive outcome measure used in moderate-severe AD |
| Global Change | Summary outcome assessment from baseline to endpoint |
| Clinical Global Impression of Change (CGI-C) | 7-pt. scale (1 = very much improved, 4 = no change, 7 = very much worse) Clinician rated, based on patient +/- informant interview |
| Clinician's Interview-Based Impression of Change Plus Caregiver Input (CIBIC-Plus) | 7-pt. scale (1 = very much improved, 4 = no change, 7 = very much worse) Clinician rated (with caregiver input), based on semi-structured interview covering cognition, behavior, function |
| Global Deterioration Scale (GDS) | 7-pt. scale (1 = no decline, 7 = very severe decline) Clinician rated based on cognitive change only |
| Function | Activities of daily living (basic and instrumental) |
| Alzheimer's Disease Cooperative Study Activities of Daily Living (ADCS-ADL) | 54-pt. scale (higher scores better) Informant rated interview of 27 basic and instrumental ADL's used in mild – moderate AD; a subgroup of 19 validated items has been used in moderate-severe AD |
| Disability Assessment for Dementia (DAD) | 100-pt. scale (higher scores better) Informant rated interview of 17 basic and 23 instrumental ADL's; initiation, organization, and planning distinguished |
| Bristol Activities of Daily Living Scale (Bristol ADL) | 60-pt. scale (higher scores worse) Informant rated interview of 20 items (10 ADL's, 10 IADL's) each rated on a 0–3 pt. Scale |
| Behavior | Mood, behavior, personality alterations, etc. |
| Neuropsychiatric Inventory (NPI) | 144-pt. scale (higher scores worse) Informant interview of 12 symptom domains rated on a 12-pt. scale based on Frequency (0–4) x Severity (0–3) |
| Behavioral symptoms in Alzheimer's disease (BEHAVE-AD) | 75-pt. scale (higher scores worse) Informant interview of 25 behavioral symptoms rated on a 0–3 pt. Scale |

Source: NIH, 2006

In terms of monotherapy (intepirdine or Aricept vs. placebo), GSK conducted three Phase 2 studies that AXON believes support intepirdine's favorable tolerability profile but leaves some questions open on efficacy, in our opinion. In fact, GSK's '603 (intepirdine and Aricept monotherapy) study showed statistically significant results on the primary analysis of Clinician's Interview-Based Impression of Change Plus Caregiver Input (CIBIC+) with $p=0.049$ and significant improvement in Mini-Mental State Exam (MMSE) treatment versus placebo ($p=0.044$) in the Aricept arm alone. Both intepirdine arms (15mg and 35mg) showed no statistically significant results for any of the endpoints

measured. Two other monotherapy studies by GSK, the '242 and the '865 studies, showed confounded interpretation of results in the sense that the placebo group improved relative to baseline in both studies and Aricept (positive control) failed to show statistical significance vs. placebo on Alzheimer's Disease Assessment Scale-cognitive subscale (ADAS-cog). Although AXON believes modest monotherapy effect is consistent with mechanism of action and preclinical findings, we view the lack of efficacy as a potential reason for such favorable tolerability. See Figure 5 below for a summary of GSK's monotherapy clinical trial results.

Figure 5: GSK's '603 Monotherapy Intepirdine & Aricept – Little Efficacy Shown

| Monotherapy vs placebo (2011 data) | | | |
|------------------------------------|----------------|----------------|---------------------|
| End Point | RVT-101 (15mg) | RVT-101 (35mg) | Donepezil (Aricept) |
| 1° ADAS-COG | N.S. | N.S. | N.S. |
| 1° CIBC+ | N.S. | N.S. | p=0.049 |
| 2° ADCS-ADL | N.S. | N.S. | N.S. |
| 2° RBANS | N.S. | N.S. | N.S. |
| 2° MMSE | N.S. | N.S. | p=0.044 |
| 2° CSDD | N.S. | N.S. | N.S. |

N.S. = not-significant; RVT-101 now called intepirdine;

Source: *Alzheimer's & Dementia*, 2015

Prior to AXON acquiring intepirdine in December 2014, GSK conducted 13 clinical trials for intepirdine involving over 1,250 individuals, which included healthy subjects as well as subjects with mild-to-moderate AD. In GSK's '603 combination Phase 2b trial (n=684) with mild-to-moderate AD, subjects who received 35 mg intepirdine + Aricept showed statistical significance vs. donepezil (p-value = 0.012 @ 24 weeks for the co-primary endpoint of ADAS-COG. Statistically significant improvements in cognition were also observed at 12 and 48 weeks but not at 36 weeks following initiation of treatment, which we believe is peculiar and may explain what we consider somewhat mixed efficacy on the cognition standpoint. In addition, the combination drug hit one of the key secondary endpoints for patients who received 35mg intepirdine + Aricept, achieving statistical significance (p= 0.033) vs. the Aricept-only group @ 24 weeks as measured by the Alzheimer's Disease Cooperative Study Activities of Daily Living (ADCS-ADL). ADAS-COG evaluates the ability to perform a list of daily activities and is the indicator of choice to measure function. The ADCS-ADL scale is evaluated based on information obtained from the subject's caregiver. See Figure 6 below for a summary of the combination therapy trial results.

The second pre-specified co-primary endpoint of GSK's Phase 2b trial was Clinical Dementia Rating Sum of Boxes (CDR-SB), which is a composite scale with certain components that evaluate cognition and other components that assess function, at 24 weeks. While the 35mg intepirdine dose group achieved statistically significant improvement in the CDR-SB at 12 weeks (not a prespecified endpoint) it was not superior at 24 weeks and further time points. It

was numerically superior at later time points, but not significantly so. AXON has chosen the two endpoints that hit in the Phase 2 trial: ADAS-COG (cognition) and ADCS-ADL (function) as the endpoints they’re going after in the Phase 3 MINDSET trial. These endpoints have been confirmed by their Special Protocol Assessment (SPA) agreement with the FDA, but it does seem noteworthy to us that Namenda and Aricept used different combinations of endpoints for function and cognition measures. Namenda, approved in 2003, for the treatment of moderate to severe AD patients used ADCS-ADL for function and Severe Impairment Battery (SIB) for cognition while Aricept (approved in 1996) for mild to moderate AD, opted for Clinician’s Interview-Based Impression of Change Plus Caregiver Input (CIBIC+) for function. Positively, intepirdine’s adverse events (AE) profile was clean, but this could be an artifact of the somewhat mixed efficacy seen in the Phase 2 trial. See Figure 6 below for a summary of these clinical trial results.

Figure 6: Glaxo’s ‘603 Combination w/Aricept – Missed Primary, Mixed On Secondary’s

| Combo vs. placebo + Donepezil | | |
|-------------------------------|--------------------------|--|
| End Point | RVT-101 (15mg)+Donepezil | RVT-101 (35mg)+Donepezil |
| 1° ADAS-COG | N.S. | 24w: p=0.012; 36w: p=N.S; 48w: p=0.024 |
| 1° CDR-SB | N.S. | N.S. |
| 2° ADCS-ADL | N.S. | 24w: p= 0.033; 36w: No data; 48w: N.S |
| 2° RBANS | N.S. | N.S. |
| 2° MMSE | N.S. | N.S. |

N.S. = not-significant; RVT-101 now called intepirdine;

Source: *Alzheimer's and Dementia*, 2015

AXON’s Phase 3 MINDSET study began in October 2015 and is a 1,150 person, global, multi-center, double-blind, placebo-controlled confirmatory Phase 3 clinical study of intepirdine for the treatment of patients with mild-to-moderate AD. AXON expects enrollment to be completed by the end of 2016 with final data read-out in 2H17. If the results prove to be positive, AXON plans to file an NDA in 2H17.

Figure 7: MINDSET Study Design

| Phase 3: Intepirdine (RVT-101) in Subjects with Mild to Moderate Alzheimer’s Disease on Donepezil: MINDSET Study | |
|--|--|
| Aim | Confirm a demonstrated treatment effect of intepirdine (RVT-101) as an adjunctive therapy to donepezil for the treatment of subjects with Alzheimer’s disease. |
| Design | phase 3 randomized, double-blind, parralel assignment safety/efficacy |
| Dosing | intepirdine (once daily 35mg tablet) adjunct to 5mg or 10mg Aricept (donepezil) vs placebo adjunct to 5mg or 10mg donepezil |
| Endpoints | 1) change from baseline on ADAS-Cog-11 and ADCS-ADL after 24 weeks; 2) CIBIC+, NPI, EQ-5D-VAS, occurrence of all AEs, ECGs and routine lab. Assessments during 24 weeks. |
| Patients | n=1150; MMSE 10-15 (<=30%); MMSE 16-20 (<=60%); MMSE 21-26 (<=30) |
| Results | Trial initiated 1Q16 and data expected 2H17 |

Source: *Company and Laidlaw estimates*

We believe that the majority of value attributed to AXON by the Street comes down to the MINDSET Phase 3 read-out in 2H17. Although we do believe there is a reasonable chance that the data hits statistical significance on the endpoints chosen based on the one primary endpoint and one secondary endpoint that hit in the GSK Phase 2 trial; we simply believe that at the current \$1B+ valuation investors haven't priced in enough of a risk premium for the very real possibility that MINDSET misses. The AD field has a long and ignominious history of promising compounds with good Phase 2 data only to be dashed by the Scylla and Charybdis of Phase 3. Recently two 5HT6 antagonists (Pfizer's and Lundbeck's) have recently been discontinued or failed, respectively. A 2014 study in the Journal of Alzheimer's Research & Therapy looked at 413 trials over a ten year period from 2002-2012 and depressingly found a 99.6% failure rate over that time, concluding:

"The Clinicaltrials.gov database demonstrates that relatively few clinical trials are undertaken for AD therapeutics, considering the magnitude of the problem. The success rate for advancing from one phase to another is low, and the number of compounds progressing to regulatory review is among the lowest found in any therapeutic area."

PFE's PF-05212377 discontinuation was updated on Clinicaltrials.gov on February 2, 2016. At the time, many defended AXON explaining that the ~25% AXON price decline wasn't warranted due to lack of potency of PFE's drug (<70% receptor occupancy - RO), too short of a trial design (12 weeks) and a too severe study population (included patients with neuropsychiatric comorbidities, which are excluded in MINDSET). Another difference in the PFE trial was the use of ADAS-COG13 (more suited for early AD) as opposed to ADAS-COG11 for AXON, which may be more suited for mild-moderate patients. Eli Lilly's recent failure on their third Phase 3 trial, EXPEDITION3 in mild AD patients, used ADAS-COG14, which just adds to the complexity and general misunderstanding of the space, in our opinion. Although we agree PFE's discontinuation shouldn't necessarily invalidate all 5HT6 receptor antagonists, it adds some risk to the MINDSET study. In terms of potency/receptor occupancy (RO), GSK studies seem to indicate RO for intepirdine was >90% for both the 15mg and 35mg arm with dose dependent binding of 5HT2a receptor at ~58% for the 35mg dose (J Nucl. Med, 2015). This would seem to indicate a potential importance of 5HT2a receptor inverse agonist since combination study of intepirdine (15mg) and Aricept showed no functional or cognitive effect vs. Aricept and placebo.



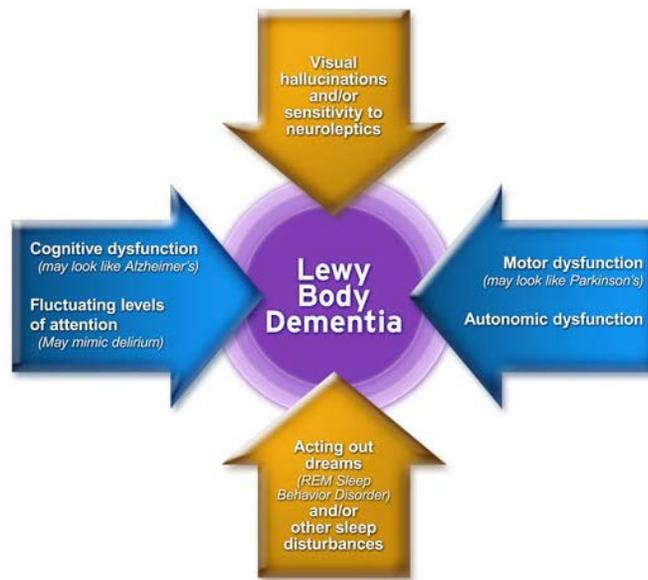
<http://www.drugsdb.com>

This should have helped Lundbeck's idalopirdine which is a 5HT₆/5HT_{2a} antagonist (similar to AXON's intepirdine). However when idalopirdine failed this past September in the STARSHINE Phase 3 trial AXON's stock once again fell ~12%. Idalopirdine failed to hit statistical significance on its primary endpoint ADAS-COG as well as its secondary endpoints in its first Phase 3 trial, STARSHINE. AXON noted the important differences between STARSHINE and MINDSET, mostly related to the variation between LUN.CO's Phase 2 and Phase 3 dosage used and RO. After using a 30mg dose TID (3x/day) in its Phase 2 (LADDER), LUN.CO used a 60mg QD (1x/day) dose in STARSHINE due to liver toxicity concerns in LADDER. In addition, we agree that it is important to note the half-life of idalopirdine is only 10-12 hours while intepirdine's ranges from 30-40 hours. To AXON's advantage, MINDSET will be similarly designed to its Phase 2 and will use the same dose that hit the endpoints of interest. While RO seems to be a valid reason for inability to hit its endpoint, LUN.CO, at the 2016 Alzheimer's Association International Conference (AAIC), clarified that they opted for the 60mg dose because it had similar RO to the 90mg dose (80%-85%). We believe this adds risk to the MINDSET. We do find noteworthy that LUN.CO did see efficacy trends and is still completing two other Phase 3 trials, STARBEAM and STARBRIGHT, which are expected to read-out ahead of MINDSET in 1H17. We believe that AD remains a remarkably misunderstood field and although PFE's and LUN.CO's studies are different, we believe their respective discontinuations and failures do add a real level of risk to AXON's MINDSET Phase 3 trial hitting positive data in 2H17.

Intepirdine for Dementia with Lewy Bodies

AXON is also developing intepirdine to address other forms of dementia, such as dementia with Lewy bodies (DLB). DLB is a subset of Lewy body dementia (LBD), which affects approximately 1.4 million patients in the U.S that show distinctive features such as visual hallucinations, fluctuating cognition, attention and alertness, neuroleptic sensitivity, REM sleep behavior disorder (RBD), and movement disorder symptoms. DLB is a progressive neurodegenerative disorder which is pathologically characterized by the aggregation of alpha-synuclein and other proteins in the brain, known as Lewy bodies, causing disruption in cognition, function and behavior. DLB is the second most prevalent cause of neurodegenerative dementia in elderly patients. It has been estimated that DLB affects approximately 1.1M people in the US. DLB patients are often treated off-label with cholinesterase inhibitors.

Figure 8: The Lewy Body Dementia Spectrum



Source: Lewy Body Dementia Association, 2016

Cholinergic neurotransmission is thought to be even more dysfunctional in DLB than in Alzheimer's disease. This suggests that neurotransmitter-targeted therapies that work by increasing the inter-synaptic concentration of acetylcholine, much like intepirdine in AD, may also be effective in improving cognition and function in DLB patients. While cholinesterase inhibitors are not approved by the FDA or EMA for the treatment of DLB, Aricept was approved in September 2014 in Japan for DLB. In addition, intepirdine has antagonist activity against the 5-HT_{2A} receptor, which has been implicated in the pathophysiology of visual hallucinations and other behavioral disturbances affecting patients with DLB. AXON thinks intepirdine could be the first drug approved by the FDA and EMA for the treatment of DLB.

After AXON's IND in December 2015, they began a Phase 2b clinical trial of intepirdine, called the HEADWAY-DLB study, in patients with DLB in 1Q16.

Figure 9: HEADWAY-DLB Study Design

| Phase 2b: Intepirdine (RVT-101) in Subjects with Dementia With Lewy Bodies: The HEADWAY-DLB Study | |
|---|--|
| Aim | Evaluate the efficacy and safety of intepirdine (RVT-101) in patients with dementia with Lewy bodies. |
| Design | phase 2b, randomized, double-blind, parallel assignment, safety/efficacy study |
| Dosing | RVT-101(once daily 35mg tablet), RVT-101 (70mg) vs placebo |
| Endpoints | 1)change from baseline on CIBIC+ after 24 weeks, change from baseline on computerized cognitive battery, 2)change on clinical assessment of visual hallucinations after 24 weeks, occurrence of reported AEs and findings noted by investigators on clinical examination, electrocardiograms, and routine lab assessments after 24 weeks |
| Patients | n=240 |
| Results | Trial initiated in 1Q16 data expected 2H17 |

Source: Company and Laidlaw Estimates

AXON believes that if the data is positive, in combination with data from their studies in AD, this could serve as the basis for seeking approval of intepirdine for DLB. While this data is exciting, the more complicated patient population of DLB combined with dependence on the MINDSET study results make us view the risk of success quite elevated. We are interested in seeing the 70mg doses efficacy since the RO of 5HT_{2a} should be better, but uncertainty remains about the potency and safety, which also attributes a high level of risk to this trial.

Intepirdine for Gait and Balance Impairments

AXON began a Phase 2 study to evaluate the effects of intepirdine on gait and balance in patients with AD, DLB and Parkinson's disease dementia (PDD) in 3Q16. AXON plans to further investigate the observation of less falls seen in patients with intepirdine in the prior (n=684) study of intepirdine on a background of stable Aricept therapy. In fact, prior studies have shown the incidence of falls in studies of 5HT₆ (intepirdine '866 study with n=684 and Lundbeck's idalopirdine LADDER study with n=278), the percentage of patients reporting falls was ~1/3 of the Aricept alone group (2% vs. 6% in both cases). However we don't believe that using Lundbeck's (LUN.CO)'s data is credible as its 5HT₆ antagonist recently (9/22/16) failed its Phase 3 trial, sending AXON's shares down ~12%. While AXON believes LUN.CO's failure was caused by the lower dose used in their Phase 3 trial and shorter half-life, we believe the similarity in the mechanisms of action of both molecule raises the risk of AXON's trials not hitting statistical significance.

Figure 10: Gait and Balance Study Design

| Phase 2: Gait and Balance study | |
|--|---|
| Aim | Evaluate risk of falls in MINDSET and HEADWAY-DLB studies |
| Design | Double-blind, randomized placebo-controlled, crossover study in subjects diagnosed with AD, DLB, or PDD with a history of defined gait impairment and on stable background therapy. |
| Dosing | Intepirdine (35mg) vs placebo |
| Endpoints | quantitative and qualitative measures of gait and balance, safety tolerability, endpoints correlated with risk of falls |
| Patients | n=40 |
| Results | Initiated in 3Q16, incidence of falls will be captured in MINDSET and HEADWAY-DLB expected to read-out in 2H17 |

Source: Company and Laidlaw estimates

Competition for Intepirdine

AXON considers intepirdine's most direct competitor to be idalopirdine (Lu AE58054), the 5-HT₆ receptor antagonist being developed by Lundbeck that recently (9/22/16) failed to hit its primary endpoint (ADAS-cog) in Phase 3. As previously mentioned, Lundbeck still has two other Phase 3 trials that should readout in 1H17. Based on publicly available information, other companies developing 5-HT₆ receptor antagonists include Acorda Therapeutics (after its acquisition of Biotie Therapies), Avineuro and Suven Life Sciences. These other 5-HT₆ receptor antagonists are all in Phase 2 or earlier stages of development for cognitive disorders. While AXON's is valued at ~\$1.3B, many of its competitors are lurking around market capitalizations of ~\$300M. VTV Therapeutics has a market cap of ~\$180M with AD candidate in Phase 3 and a Type 2 diabetes drug in Phase 2. Prior to its first Phase 3 5HT_{6/2a} antagonist failure for AD and while having ~\$2.15B in sales in 2015, LUN.CO had a

~\$7.9B valuation. As mentioned previously Acorda Therapeutics bought Biotie Therapies for ~\$363M with a Phase 3 PD asset and 5HT6/5HT2A antagonist in Phase 2 for PD and Phase 1 for AD.

Alzheimer's Unfortunate History of Failures and Future Milestones

Until scientists are able to better grasp the underlying causes of dementia, the level of risk in clinical trials will always be elevated. Although we don't believe it's necessary to fully understand mechanism in order to relieve both patient symptoms as well as astronomical costs to the healthcare industry, we find it difficult to have great confidence in any late stage trial in the field. Recent studies have found that 244 compounds in 413 clinical trials for AD between 2002 and 2012 resulted in failures. This statistic has unfortunately given AD one of the highest failure rates of any disease area (99.6% vs. 81% for cancer). As a dementia solutions company, AXON is focused on different symptoms such as memory loss, confusion and mood changes. These can result from AD (~66% of the time), frontotemporal dementia and DLB. These are all neurodegenerative diseases in which accelerated cell death is caused by buildup of different proteins depending on the disease. One of the many issues with dementia is its silent nature as symptoms usually only appear a decade or more after the start of the disease.

Additionally, researchers must deal with the heterogeneous nature of the disease, the difficulties of diagnosis, as well as its slow progression. One of the most researched hypothesis has been the beta amyloid plaque accumulation. Unfortunately, on 11/23/16, that hypothesis took a serious blow as Eli Lilly announced that its third Phase 3 compound, EXPEDITION 3, didn't meet its primary endpoint even after changing the patient population and enabling better screening of plaques in the brain. Unfortunately, LLY's share price fell by ~15% on the news and we expect Biogen's (also going after the beta-amyloid plaques) risk of failure in their upcoming Phase 3 read-out to increase.

Figure 11: AD Events to Watch For

| Event | Expected Timing (CY) |
|---|----------------------|
| LUN.CO 2 more phase 3 trial readouts for idalopirdine | 1Q17 |
| Intepirdine for AD MINDSET phase 3 data | 2H17 |
| RVT-103 for AD phase 2 | 2H17 |
| MRK: BACE inhibitor 1rst of 2 phase 3s | 2H17 |
| BIB phase 3 data for Aducanumab | 2020 |

Source: Company Reports and Laidlaw estimates

Nelotanserin

In October 2015, AXON acquired from RSL the global rights to nelotanserin, a 5-HT_{2A} receptor inverse agonist. AXON intends to develop nelotanserin to address visual hallucinations (VH) and sleep disturbances in patients with LBD and RBD in patients with DLB. Nelotanserin has been evaluated in seven clinical studies to date with nearly 800 human subjects exposed to the drug candidate and has been observed to be well tolerated. In in vitro studies, nelotanserin did not antagonize the dopamine D2 receptor. Antagonism of the D2 receptor in LBD/DLB patients can lead to severe side effects including increased parkinsonism, worsening of cognition, heavy sedation, and symptoms resembling neuroleptic malignant syndrome which can be fatal.

Nelotanserin in Visual Hallucinations in Lewy Body Dementia

As mentioned previously, LBD includes two similar conditions, DLB and PDD, which affects approximately 300,000 patients of the 1.4 million LBD patients in the US. Pimavanserin from competitor Acadia Pharmaceuticals is also a 5HT_{2A} inverse agonist that was able to demonstrate a statistically significant difference in the Parkinson's disease-adapted scale for assessment of positive symptoms (SAPS-PD) change from baseline. Pimavanserin was approved on 4/29/16 making it the first drug approved for the treatment of hallucinations and delusions associated with Parkinson's disease psychosis (PDP). Although we do believe pimavanserin's recent approval validates the 5HT_{2a} mechanism of action, we also think this adds competition to the market place for AXON.

In January 2016 AXON initiated Phase 2 clinical study of nelotanserin in DLB and PDD patients suffering from VH. They first expect to receive interim data from this study in 2H16 CY but recently announced that due to strict inclusion criteria (most severe patients), AXON has delayed their interim look until 1Q17.

Figure 12: Visual Hallucinations in LBD Study Design

| Phase 2: Nelotanserin for treatment of Visual Hallucinations in Subjects with Lewy Body Dementia | |
|---|--|
| Aim | Evaluate safety and efficacy of Nelotanserin for the treatment of visual hallucinations in subjects with Lewy body dementia |
| Design | phase 2, randomized, double-blind, crossover assignment |
| Dosing | Nelotanserin (once daily) 40mg then 80mg vs placebo |
| Endpoints | 1) Safety will be evaluated based on incidence of AEs and changes in physical examinations, vital signs, ECGs, and routine clinical lab assessments from baseline to end of each treatment period (28days); 2) Changes in frequency and severity of visual hallucinations from baseline to the end of each treatment period (28 days) with Nelotanserin or placebo (time frame: 28 days) |
| Patients | n=20 |
| Results | interim data (10 out of 20 patients) pushed back and now expected 1Q17 |

Source: Company and Laidlaw estimates

Nelotanserin in REM Behavior Disorder (RBD) in Dementia with Lewy Bodies (DLB)

RBD is a common clinical feature of DLB, and is a condition in which patients physically act out their dreams, impacting their quality of life and endangering themselves and their bed partners. While off-label treatment of RBD with benzodiazepines is common, this class of drugs is associated with concerning

side effects in patients with dementia, including sedation, worsening of cognition and increased risk of falls. AXON believes that there is a need for new therapeutic options that can reduce the frequency of RBD without sedating patients or worsening cognition in patients with dementia.

In March 2016 AXON initiated a Phase 2 study in patients with DLB suffering from RBD. This study will utilize objective measures of efficacy as assessed in a sleep-lab setting. The company designed this study to potentially serve as a pivotal trial in support of an application for regulatory approval, and they expect to receive results in calendar year 2017. There is an overlap between visual hallucinations and dream content during REM behaviors and blocking the 5HT_{2A} receptor reduces visual hallucinations. An objective Phase 2 sleep study (n=173) of adult subjects with primary insomnia compared 10mg and 40mg doses of nelotanserin to placebo and in which patients spent nights 1 and 2, and nights 6 and 7 combined in a sleep laboratory. In the study nelotanserin showed robust benefits on wake time after sleep onset, with the primary endpoint (p<0.0001) as nelotanserin showed statistically significant improvements on objective measures of sleep maintenance and consolidation. Arousals from sleep may trigger disruptive REM behaviors and nelotanserin has shown robust reductions in the number of arousals and awakenings in clinical studies.

Figure 13: Nelotanserin for RBD in DLB Study Design

| Phase 2: Nelotanserin for treatment of REM Sleep Behavior Disorder in Subjects with Dementia with Lewy Bodies | |
|--|--|
| Aim | Evaluate the safety and efficacy of Nelotanserin for the treatment of Rapid Eye Movement (REM) Sleep Behavior Disorder (RBD) in subjects with dementia with Lewy bodies (DLB). |
| Design | Phase 2, randomized, double-blind, placebo-controlled, parallel-arm study |
| Dosing | Nelotanserin once daily (80mg) vs placebo |
| Endpoints | 1)Change in frequency of REM sleep behaviors from baseline to the end of the treatment period (28 days) based on clinical evaluator; 2)Change in the proportion of severe REM sleep behaviors from baseline to the end of the treatment period (28 days) based on a clinical evaluator |
| Patients | n=60 |
| Results | data expected 2H17 |

Source: Company reports and Laidlaw estimates

Competition for Nelotanserin

As mentioned previously nelotanserin's most direct competitor is ACAD's pimavanserin. AXON believes the FDA approval of pimavanserin adds further validation to the therapeutic relevance of 5-HT_{2A} as a potential target for the treatment of visual hallucinations. Since being approved on 4/29/16 and available for prescription on 5/31/16, pimavanserin YTD sales as of 3Q16 were \$5.3M and cons projects revenue for 2016 to hit ~\$14M in revenue in U.S sales. A relatively slow launch is modelled in due to low physician awareness in PDP space. While AXON emphasizes the potential read-through to intepirdine of ACAD's ADP Phase 2 pimavanserin data that came out 12/20/16, we believe the lack of clarity between 5HT₆ and 5HT_{2A} receptors raises complicates a potential read-through on the data and the p= 0.0451 value at 6 weeks wasn't very convincing in our opinion. We also remind investors that ACAD took three attempts to get positive data for PDP due to multiple necessary changes in endpoints and trial designs, raising the stakes for the upcoming ADP data.

RVT-103 and RVT-104 Pipeline Expansion

On 8/15/16, AXON announced an exclusive license agreement with Qaam Pharmaceuticals and their plan to develop RVT-103 (glycopyrrolate + donepezil) as a potential treatment for patients with dementia, with a potential for an eventual triple combination with intepirdine. AXON believes this technology has the potential to reduce peripheral side effects of cholinesterase inhibitors and to enhance their efficacy through higher doses. Under the agreement AXON expects to develop and, if successful, commercialize products that combine cholinesterase inhibitors with peripheral muscarinic receptor antagonists including glycopyrrolate, which could mitigate the peripheral side effects of cholinesterase inhibitors. In addition, AXON expects to develop RVT-104 (glycopyrrolate + high-dose rivastigmine). Rivastigmine has shown greater efficacy at higher-than-approved doses with clear dose response (Exelon patch label) and unlike donepezil, which only inhibits the acetylcholinesterase enzyme, rivastigmine also inhibits the butylcholinesterase enzyme, which is involved in the breakdown of acetylcholine. AXON believes this product candidate can limit the peripheral side effects of cholinesterase inhibitors which frequently represent an obstacle for patients to adopt or remain on the therapy. AXON has recently initiated clinical studies of RVT-103 and expects data in 2H17 calendar year. Depending on results, AXON may pursue registration programs in the United States. AXON then intends to evaluate opportunities for higher doses of cholinesterase inhibitors to deliver additional efficacy, potentially in combination with intepirdine (triple combination).

While the financial terms of the transaction weren't disclosed, AXON doesn't expect the program to have material effect on its use of cash in the fiscal year ending March 31, 2017.

Figure 14: RVT-103 and RVT-104 Triple Combinations of Intepirdine



Source: Company Presentations

Competition for RVT-103 and RVT-104

On 11/22/16, Allergan announced the acquisition of Chase Pharmaceuticals (private) to show their commitment to AD for an upfront payment of \$125M and additional potential regulatory and sales milestone payments related to Chase's lead compound, CPC-201, and other backup compounds. CPC-201 consists of a Phase 3 ready patent-protected combination of Aricept and peripherally acting cholinergic blocker, solifenacin. This next generation formulations might offer greater and more tolerable dosing that could lead to significantly improved cognition and function in AD. AGN plans to advance CPC-201 into a single Phase 3 registration study in 2017. Although there isn't any preclinical or clinical data comparing CPC-201 to RVT-103, there is a chance that RVT-103 might penetrate the brain less than CPC-201 due to the lipophilic nature of CPC-201. While it is hard to overstate the size of the market for AD, AXON now finds itself behind AGN in the development of a less toxic

Aricept combination. We believe AXON's advantage here lies in their potential triple combination with intepirdine and we still don't attribute much value to RVT-103/104 since their true potential is still highly correlated with intepirdine's success, in our opinion. We also believe the \$125M upfront for the acquisition, once again, suggests AXON is currently overvalued with a ~\$1.3B market cap.

Agreements

Under the GSK Agreement to get intepirdine, AXON made an upfront payment of \$5M and an additional \$5M payment in June 2016, which they had recorded as a contingent payment liability in the consolidated balance sheet. They are also obligated to pay GSK \$35M, \$25M and \$10M upon the receipt of marketing approval of intepirdine in the United States, the European Union and Japan, respectively, as well as an additional one-time payment of \$85M for the first calendar year in which they achieve global net sales of \$1.2B for intepirdine. Under the GSK Agreement they are also obligated to pay a fixed 12.5% royalty based on net sales of intepirdine, subject to reduction on account of expiration of patent and regulatory exclusivity or upon generic entry. In October 2015, AXON exercised an option to acquire global rights, title, interest and obligations in and to nelotanserine from their parent company RSL.

In May 2015, RSL entered into an agreement for nelotanserine with Arena Pharmaceuticals, and AXON entered into a Waiver and Option Agreement with RSL. Upon the exercise of their option, they assumed RSL's rights and obligations under the development, marketing and supply agreement with ARNA. AXON will be responsible for future contingent payments under the Arena Development Agreement, including up to \$4M in potential development milestone payments, up to \$37.5M in potential regulatory milestone payments and up to \$60M in potential commercial milestone payments. Under the Arena Development Agreement, AXON is also obligated to purchase finished drug product under a fixed price equal to 15% of net sales of nelotanserine.

Management

Vivek Ramaswamy, CEO. Mr. Ramaswamy is the founder of Roivant Sciences, the parent company of Axovant Sciences, and serves as CEO of Axovant Sciences, Inc. He is also the Chairman of Arbutus Biopharma, a company focused on developing a cure for chronic hepatitis B virus infection. Prior to founding Roivant Sciences, Mr. Ramaswamy was Partner at QVT Financial LP, where he was responsible for biotechnology investments. He also has prior experience as a successful entrepreneur in the technology industry. Mr. Ramaswamy graduated with an AB in Biology from Harvard College and JD from Yale Law School.

Gregory M. Weinhoff, MD, CFO. Dr. Weinhoff has more than twenty years of experience in healthcare finance and operations, including as a venture capital investor, operating executive, and board member with Audit Committee experience. He was a partner at CHL Medical Partners focused on investments into start-up and early-stage companies across therapeutics, diagnostics, medical devices, and healthcare services. Dr. Weinhoff was the founding CEO of Amicus Therapeutics (FOLD) subsequently member of Board Audit Committee. He was also founding President of VaxInnate, Resolvix and served in healthcare at J.H. Whitney & Co. and Morgan Stanley & Co. He has an MD from Harvard Medical School, an MBA from Harvard Business School and an AB in Economics Harvard College.

Lawrence T. Friedhoff, MD, PhD, FACP, Chief Development Officer. Dr. Lawrence T. Friedhoff has a 30-year record of identifying promising drug candidates and managing all phases of their development, always with an eye toward fulfilling drug approval requirements. He has also managed post-FDA-approval activities including marketing-related, safety surveillance, and post-approval studies. Dr. Friedhoff led the development of Aricept (donepezil), the top-selling drug for Alzheimer's disease in history. He also led the new drug approval team for Aciphex (rabeprazole) for the treatment of heartburn. Dr. Friedhoff was senior VP, R&D at Roivant Sciences, Inc. He is the author of the book *New Drugs* and author or co-author of many peer-reviewed publications in major scientific journals. He has a PhD from Columbia University in Chemistry, an MD from NYU and is a Fellow at the American College of Physicians.

Mark Altmeyer, President & Chief Commercial Officer. Mark Altmeyer has three decades of experience leading successful drug commercialization efforts as a pharmaceutical executive, with a particular focus on therapies for CNS disorders. He has served as CEO and President of Otsuka America Pharmaceutical, Inc. from 2009-2014, leading 1,700 employees and growing total revenues from \$2.6B to over \$5B. Mr. Altmeyer has led the launch of Abilify, the top-selling CNS drug in history and the number 1 selling drug in the US in 2013. He held a number of executive leadership roles at Bristol-Myers

Major Risks

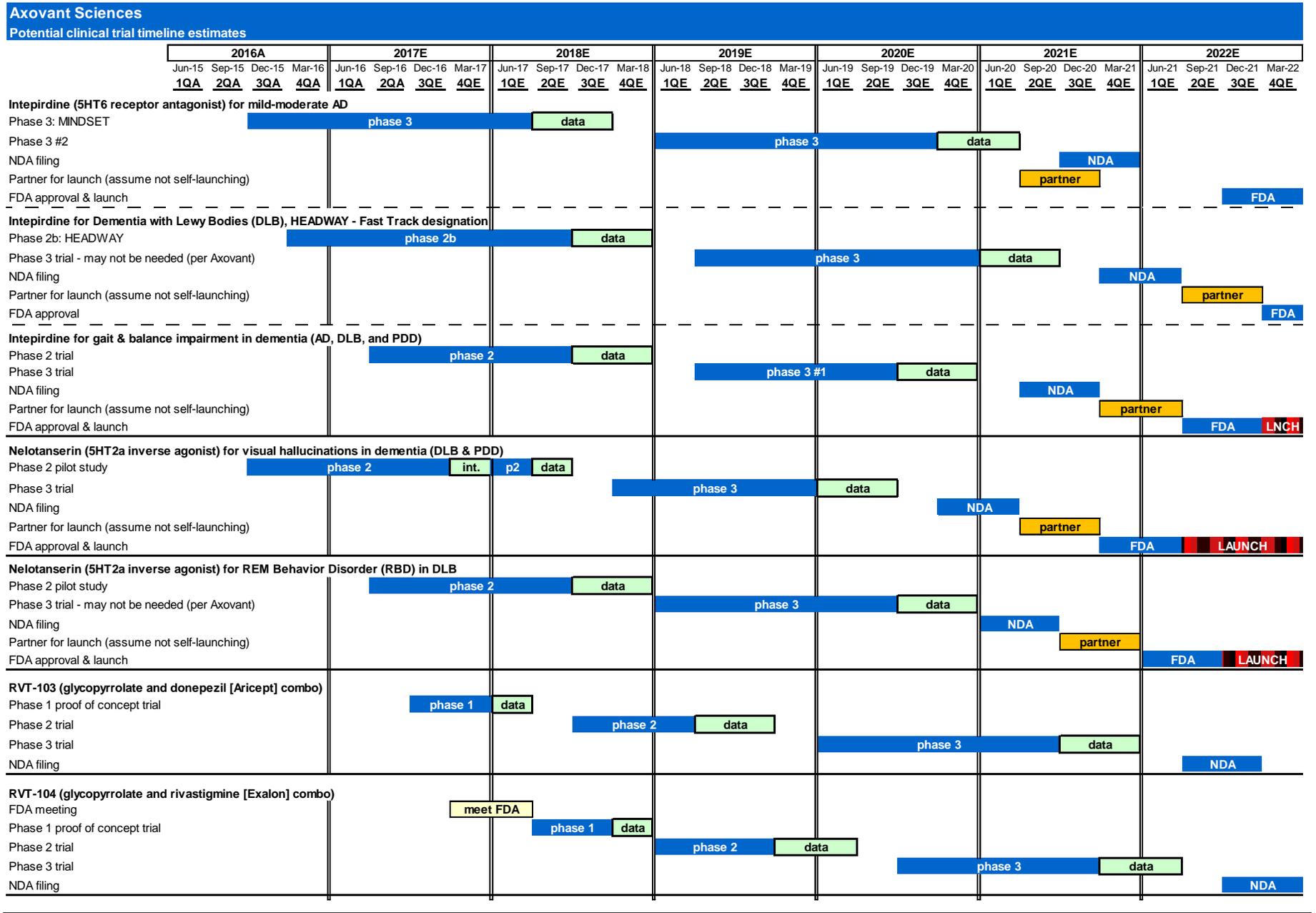
Exogenous events could impact our outlook. We believe pharmaceutical companies have the least control over competitive, political, and regulatory risks. Although we have incorporated competitive assumptions into our forecasts, there may be other risks beyond the scope of our analysis. Changes in the drug reimbursement system, as well as any political or regulatory amendments, may significantly influence the earnings power of these companies.

Actual clinical results and the FDA's conclusions may deviate from expectations. Many of our assumptions are based on a review of incomplete clinical trial data available in the public domain. Often, our conclusions are drawn from early stage data, which may not be reflected by pivotal studies. Furthermore, the FDA's conclusions may not coincide with our own, materially changing our revenue and earnings assumptions.

Compliance issues, product recalls, and other mandates by regulatory authorities could materially change our expectations. Regulatory compliance issues, ranging from accounting irregularities to defective manufacturing practices, could materially change our assumptions and earnings outlook. Unanticipated product recalls and labeling changes could also have adverse consequences on our earnings assumptions.

Legal risks could lead to additional liabilities and revenue loss. In addition to the expenses incurred by patent challenges, product liability and other legal suits could occur and lead to additional liabilities and revenue loss, which could substantially change our financial assumption

Figure 15: Potential Clinical Trial Timelines



Source: Company reports; Laidlaw & Company estimates

Figure 16: Quarterly Income Statement

| Axovant Sciences | | | | | | | | | | | |
|-----------------------------------|-----------------------|----------------------|----------------------|----------------------|----------------------|-----------------------|----------------------|----------------------|----------------------|----------------------|-----------------------|
| Quarterly income statement | | | | | | | | | | | |
| | 2015A | 2016A | | | | 2016A | 2017E | | | | 2017E |
| | Mar-15 Year | Jun-15 1QA | Sep-15 2QA | Dec-15 3QA | Mar-16 4QA | Mar-16 Year | Jun-16 1QA | Sep-16 2QA | Dec-16 3QE | Mar-17 4QE | Mar-17 Year |
| (\$000's except per share) | | | | | | | | | | | |
| R&D ex non-cash | 11,146 | 3,707 | 6,478 | 19,725 | 17,234 | 46,022 | 20,312 | 27,601 | 22,000 | 23,000 | 92,913 |
| SG&A ex non-cash | 1,604 | 5,100 | 2,939 | 3,773 | 4,927 | 14,754 | 6,034 | 5,985 | 6,000 | 7,000 | 25,019 |
| Total Op Expenses | 12,750 | 8,807 | 9,417 | 23,498 | 22,161 | 60,776 | 26,346 | 33,586 | 28,000 | 30,000 | 117,932 |
| Operating inc/(loss) | (12,750) | (8,807) | (9,417) | (23,498) | (22,161) | (60,776) | (26,346) | (33,586) | (28,000) | (30,000) | (117,932) |
| Income Tax expense | (1) | (74) | (24) | (802) | 918 | 17 | (148) | (729) | (100) | (100) | (1,077) |
| Adj-Net inc/(loss) | (12,751) | (8,881) | (9,441) | (24,300) | (21,243) | (60,759) | (26,494) | (34,315) | (28,100) | (30,100) | (119,009) |
| Total non-cash expenses | (8,296) | (19,177) | (2,620) | (39,056) | (8,428) | (72,386) | (11,561) | (7,937) | (10,000) | (10,000) | (39,498) |
| GAAP net inc/(loss) | (21,047) | (28,058) | (12,061) | (63,356) | (29,671) | (133,145) | (38,055) | (42,252) | (38,100) | (40,100) | (158,507) |
| Adj-EPS ex-non-cash | (\$0.80) | (\$0.11) | (\$0.10) | (\$0.25) | (\$0.21) | (\$0.64) | (\$0.27) | (\$0.35) | (\$0.28) | (\$0.30) | (\$1.20) |
| GAAP EPS as reported | (\$1.32) | (\$0.35) | (\$0.12) | (\$0.64) | (\$0.30) | (\$1.41) | (\$0.38) | (\$0.43) | (\$0.38) | (\$0.40) | (\$1.60) |
| Shares out (000) | 15,987 | 80,308 | 99,150 | 99,150 | 99,253 | 94,465 | 99,150 | 99,160 | 99,260 | 99,360 | 99,233 |
| Fully diluted shares (000) | 19,987 | 85,038 | 104,331 | 104,849 | 107,243 | 100,365 | 106,450 | 106,560 | 106,760 | 106,860 | 106,658 |

Source: Bloomberg LP; Company reports; Laidlaw & Company estimates

Figure 17: Annual Income Statement

| Axovant Sciences | | | | | | | | | |
|------------------------------|------------------|------------------|------------------|------------------|------------------|------------------|------------------|------------------|-----------------------------|
| Annual income statement | | | | | | | | | |
| | Mar-16 | Mar-17 | Mar-18 | Mar-19 | Mar-20 | Mar-21 | Mar-22 | Mar-23 | |
| (\$000's except per share) | 2016A | 2017E | 2018E | 2019E | 2020E | 2021E | 2022E | 2023E | Comments |
| Revenues | | | | | | | | | |
| US Intepirdine sales | | | | | | - | - | \$195,000 | Launch 2Q CY22 |
| EU Intepirdine royalty | | | | | | - | - | 11,025 | Launch 2Q CY22 |
| Total sales | | | | | | \$0 | \$0 | \$206,025 | |
| COGS | | | | | | 0 | 0 | 60,255 | |
| Gross margin | | | | | | 0 | 0 | 145,770 | |
| R&D | 46,022 | 92,913 | 109,000 | 94,000 | 81,000 | 73,000 | 63,750 | 71,000 | |
| SG&A | 14,754 | 25,019 | 35,000 | 35,000 | 46,500 | 63,500 | 97,000 | 117,000 | |
| Operating inc./(loss) | (60,776) | (117,932) | (144,000) | (129,000) | (127,500) | (136,500) | (160,750) | (42,230) | |
| Income tax expense | 17 | (1,077) | 0 | 0 | 0 | 0 | 0 | 0 | Sig. tax loss carryforwards |
| Adj-Net inc./(loss) | (60,759) | (119,009) | (144,000) | (129,000) | (127,500) | (136,500) | (160,750) | (42,230) | |
| Total non-cash exp | (72,386) | (39,498) | | | | | | | |
| GAAP net inc./(loss) | (133,145) | (158,507) | | | | | | | |
| Adj-EPS ex-non-cash | (\$0.64) | (\$1.20) | (\$1.45) | (\$1.20) | (\$1.10) | (\$1.05) | (\$1.15) | (\$0.30) | |
| GAAP EPS as reported | (\$1.41) | (\$1.60) | | | | | | | |
| Shares out (000) | 94,465 | 99,233 | 99,610 | 107,785 | 116,285 | 129,985 | 139,285 | 141,735 | |
| Fully diluted shares (000) | 100,365 | 106,658 | 107,110 | 115,285 | 124,285 | 137,985 | 147,285 | 150,235 | |
| Cash position | \$276,251 | \$218,311 | \$134,161 | \$132,211 | \$120,861 | \$109,661 | \$15,161 | \$34,431 | multiple raises |

Source: Bloomberg LP; Company reports; Laidlaw & Company estimates

Figure 18: Balance Sheet

| Axovant Sciences | | | | | | | | | | |
|-----------------------------------|----------------|----------------|----------------|----------------|----------------|----------------|----------------|----------------|----------------|---------------|
| Balance sheet | | | | | | | | | | |
| | Mar-16 | Jun-16 | Sep-16 | Mar-17 | Mar-18 | Mar-19 | Mar-20 | Mar-21 | Mar-22 | Mar-23 |
| (\$000's except per share) | <u>2016A</u> | <u>1Q17A</u> | <u>2Q17A</u> | <u>2017E</u> | <u>2018E</u> | <u>2019E</u> | <u>2020E</u> | <u>2021E</u> | <u>2022E</u> | <u>2023E</u> |
| Current Assets | | | | | | | | | | |
| Cash & equiv | 276,251 | 252,657 | 229,664 | \$218,311 | \$134,161 | \$132,211 | \$120,861 | \$109,661 | \$15,161 | \$34,431 |
| Total Current Assets | 282,086 | 256,753 | 234,444 | 224,311 | 145,661 | 146,711 | 140,111 | 132,161 | 41,911 | 77,181 |
| PP&E | 89 | 106 | 103 | 100 | 250 | 500 | 1,250 | 2,000 | 2,500 | 2,501 |
| Deferred tax assets | 323 | 323 | 40 | 300 | 300 | 300 | 300 | 300 | 300 | 301 |
| Total Assets | 282,498 | 257,182 | 234,587 | 224,711 | 146,211 | 147,511 | 141,661 | 134,461 | 44,711 | 79,983 |
| Current Liabilities | | | | | | | | | | |
| Total Liabilities | 15,755 | 16,933 | 28,643 | 32,000 | 42,500 | 42,350 | 47,500 | 51,000 | 52,500 | 61,000 |
| Shareholders' Equity | | | | | | | | | | |
| common shares, par value | 1 | 1 | 1 | 1 | 1 | 1 | 1 | 1 | 1 | 1 |
| Add. Paid-in capital | 420,934 | 432,495 | 440,442 | 465,911 | 439,957 | 468,659 | 494,861 | 556,207 | 516,459 | 461,913 |
| Accumulated deficit | (154,192) | (192,247) | (234,499) | (273,201) | (336,247) | (363,499) | (400,701) | (472,747) | (524,249) | (442,931) |
| Total SE (deficit) | 266,743 | 240,249 | 205,944 | 192,711 | 103,711 | 105,161 | 94,161 | 83,461 | (7,789) | 18,983 |
| Total liabilities & SE | 282,498 | 257,182 | 234,587 | 224,711 | 146,211 | 147,511 | 141,661 | 134,461 | 44,711 | 79,983 |

Source: Bloomberg LP; Company reports; Laidlaw & Company estimate

Figure 19: Cash flow Statement

| Axovant Sciences | | | | | | | | | | |
|---|-----------------|-----------------|-----------------|-----------------|-----------------|-----------------|-----------------|-----------------|-----------------|----------------|
| Statement of cash flows | | | | | | | | | | |
| | Mar-16 | Jun-16 | Sep-16 | Mar-17 | Mar-18 | Mar-19 | Mar-20 | Mar-21 | Mar-22 | Mar-23 |
| | 2016A | 1Q16A | 2Q16A | 2017E | 2018E | 2019E | 2020E | 2021E | 2022E | 2023E |
| (\$000's except per share) | | | | | | | | | | |
| Operating Cash Flow | | | | | | | | | | |
| Net Income/Loss | (\$133,145) | (\$38,055) | (\$80,307) | (\$119,009) | (\$144,000) | (\$129,000) | (\$127,500) | (\$136,500) | (\$160,750) | (\$42,230) |
| Deferred tax assets | (323) | | 283 | | | | | | | |
| Change in op. assets/liabilities | | 7,917 | 18,943 | 16,080 | 5,000 | (3,150) | 400 | 250 | (2,750) | (7,500) |
| Cash from operations | (53,347) | (18,566) | (41,561) | (52,929) | (79,000) | (57,150) | (52,100) | (61,250) | (88,500) | 25,270 |
| Investing Activities | | | | | | | | | | |
| PP&E | (94) | (28) | (36) | (11) | (150) | (250) | (750) | (750) | (1,000) | (1,000) |
| Cash from investing | (5,346) | (28) | (36) | (11) | (150) | (250) | (750) | (750) | (1,000) | (1,000) |
| Financing Activities | | | | | | | | | | |
| Cash proceeds from issuance | 336,893 | | | | | | | | | |
| IPO cost paid | (2,351) | | | | | | | | | |
| cash capital from Roivant | 751 | | | | | | | | | |
| Payment of contingent liability | (627) | (5,000) | (5,000) | (5,000) | (5,000) | (5,000) | (5,000) | (5,000) | (5,000) | (5,000) |
| common stock | | | | | | 60,450 | 46,500 | 55,800 | 0 | 0 |
| exercise of stock options | | | 10 | | | | | | | |
| Due to Roivant | 278 | | | | | | | | | |
| Cash from financing | 334,944 | (5,000) | (4,990) | (5,000) | (5,000) | 55,450 | 41,500 | 50,800 | (5,000) | (5,000) |
| Change in cash | 276,251 | (23,594) | (46,587) | (57,940) | (84,150) | (1,950) | (11,350) | (11,200) | (94,500) | 19,270 |
| Cash, start of period | | 276,251 | 276,251 | 276,251 | 218,311 | 134,161 | 132,211 | 120,861 | 109,661 | 15,161 |
| Cash, end of period | 276,251 | 252,657 | 229,664 | 218,311 | 134,161 | 132,211 | 120,861 | 109,661 | 15,161 | 34,431 |

Source: Bloomberg LP; Company reports; Laidlaw & Company estimates

DISCLOSURES:

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The analyst responsible for the content of this report hereby certifies that the views expressed regarding the company or companies and their securities accurately represent his personal views and that no direct or indirect compensation is to be received by the analyst for any specific recommendation or views contained in this report. Neither the author of this report nor any member of his immediate family or household maintains a position in the securities mentioned in this report.

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Additional information available upon request.

Laidlaw & Co (UK) Ltd. has not provided any investment banking services for the company (IONS) mentioned in this report over the last 12 months.

RATINGS INFORMATION

Rating and Price Target Change History



| 3 Year Rating Change History | | |
|------------------------------|----------|--------------------|
| Date | Rating | Closing Price (\$) |
| 12/20/2016 | Sell (S) | 12.62* |

| 3 Year Price Change History | | |
|-----------------------------|-------------------|---------------------|
| Date | Target Price (\$) | Closing Price, (\$) |
| 12/20/2016 | 7.00 | 12.62* |

* Previous Close 12/19/2016

Source: Laidlaw & Company

Created by: Blue-Compass.net

| Laidlaw & Company Rating System* | | % of Companies Under Coverage With This Rating | % of Companies for which Laidlaw & Company has performed services for in the last 12 months | |
|----------------------------------|---|--|---|-----------|
| | | | Investment Banking | Brokerage |
| Strong Buy (SB) | Expected to significantly outperform the sector over 12 months. | 2.50% | 2.50% | 0.00% |
| Buy (B) | Expected to outperform the sector average over 12 months. | 57.50% | 27.50% | 2.50% |
| Hold (H) | Expected returns to be in line with the sector average over 12 months. | 5.00% | 0.00% | 0.00% |
| Sell (S) | Returns expected to significantly underperform the sector average over 12 months. | 5.00% | 0.00% | 0.00% |

ADDITIONAL COMPANIES MENTIONED

Acadia Pharmaceuticals (ACAD – Not Rated)
 Acorda Therapeutics (ACOR – Not Rated)
 Allergan plc (AGN – Not Rated)
 Arena Pharmaceuticals (ARNA – Not Rated)
 Biogen (BIIB – Not Rated)
 Eisai Co., Ltd (TYO 4523 – Not Rated)
 Eli Lilly (LLY – Not Rated)
 Exelon Corporation (EXC – Not Rated)
 GlaxoSmithKline (GSK – Not Rated)
 Lundbeck (LUN.CO – Not Rated)
 Pfizer (PFE – Not Rated)

Suven Life Sciences Ltd. (NSE: SUVEN – Not Rated)
VTV Therapeutics (VTVT – Not Rated)

ADDITIONAL DISCLOSURES

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