Auspex Pharmaceuticals, Inc. (ASPX) — Overweight

'809 Data Enhances Conviction on Deuterium Driven Orphan Disease Pipeline

CONCLUSION

Auspex reported positive data for '809 in Huntington's chorea (HC) from its registrational trial (First-HD) and also from ARC-HD Switch. We see no disconcerting observations in the efficacy (Exhibit 1) and importantly safety/tolerability data (Exhibit 2) that are likely to trip up a timely approval in HC and management reiterated plans to file the NDA by mid-'15. The safety data stands out, in our view, with AE levels comparable and in many cases better than placebo, clearly differentiating the candidate from SOC tetrabenazine at 12 weeks.

- **Future implications and share impact.** We believe that, based on this data and possibly a favorable efficacy profile as a result of safety over time (and thus persistence of dosing), there is the potential for none of the restricted access or black box warnings or tolerability issues that hamper Xenazine, in our view. As a result of reduced clinical and perhaps regulatory risk, and a "nod" to mitigated commercial risk, we are lowering our discount rates for HC (20% to 15%) and tardive dyskinesia (TD) (30% to 25%), resulting in our new $72 price target (from $50, Exhibit 3). Despite the stock being up AMC on Tue post the call, we would be buyers in front of several 2015 milestones including additional data from ARC-HD, pre-NDA meeting and 505(b)2 filing, TD and Tourette's data and last but, certainly not least, increased visibility from the pipeline including deuterated-pirfenidone.

- **More data details coming in 1H'15.** Management indicated plans to seek data presentations at the AAN meeting in April and the International Congress of Parkinson's Disease and Movement Disorders in June. We look forward to the additional data which may, with a limited set of physicians to influence use patterns vs. Xenazine, be an excellent venue to gauge interest in adoption and ultimate market potential. However, we expect that most prescribing decisions will be made based on: 1) the label, in terms of tolerability and dosing guidance, 2) in clinic experience, and 3) relative cost, although we don't see the concept of a genericization of the market as viable and believe that a differentiated label may carry the day in terms of orphan protection decisions. Based on the data presented for First-HD, with very few CNS AEs, most notable being somnolence at 11% vs. 4.4% for placebo, we feel there is a strong likelihood for a more lenient black box warning and maybe no REMS requirement.

RISKS TO ACHIEVEMENT OF PRICE TARGET

Failure for SD-809 to perform adequately on safety and/or efficacy in clinical testing.

COMPANY DESCRIPTION

Auspex applies deuterium chemistry to known pharmaceuticals to create novel pharmacological properties and improved safety and/or efficacy characteristics.

PRICE: US$25.09
TARGET: US$72.00
NPV of '809 revs disc @ 15%/25% (HC/TD) thru 2030 & no term value, other inc/exp @ 10% w/ 2x term value

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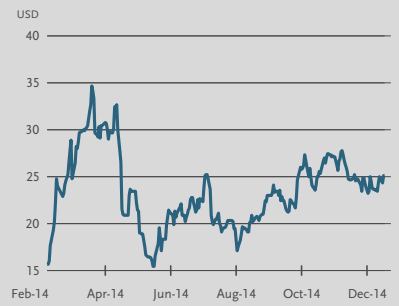
Changes	Previous	Current
Rating	—	Overweight
Price Tgt	US$50.00	US$72.00
FY14E Rev (mil)	—	US$0.0
FY15E Rev (mil)	—	US$0.0
FY14E EPS	—	US$(2.35)
FY15E EPS	—	US$(1.43)

52-Week High / Low	US$35.78 / US$13.25
Shares Out (mil)	27.6
Market Cap. (mil)	US$692.5
Avg Daily Vol (000)	141
Book Value/Share	US$5.70
Net Cash Per Share	US$6.13
Debt to Total Capital	1%
Div (ann)	NA
Fiscal Year End	Dec

Price Performance - 1 Year



Source: Bloomberg

YEAR	REVENUE (US$ m)						EARNINGS PER SHARE (US$)					
	Mar	Jun	Sep	Dec	FY	FY	Mar	Jun	Sep	Dec	FY	FY P/E
2013A	—	—	—	—	0.0	NA	—	—	—	—	(371.00)	NM
2014E	0.0A	0.0A	0.0A	0.0	0.0	NA	(0.81)A	(0.45)A	(0.73)A	(0.46)	(2.35)	NM
2015E	0.0	0.0	0.0	0.0	0.0	NA	(0.42)	(0.38)	(0.34)	(0.30)	(1.43)	NM

Impressive data, even while doing a "deeper dive". The baseline total chorea scores for First-HD were an average of 12.7, vs. 14.9 for the Xenazine pivotal studies. This suggests to us that the improvement observed for '809 (4.4 points vs. 1.9 for placebo) could be more meaningful than a simple analysis of the difference of drug and placebo indicates (roughly 2.5 points in First-HD vs. ~4 points in the Xenazine label).

More visibility on broader market potential coming. Recall that ASPX has 2 pivotal studies in TD ongoing (a much larger market opportunity) with the first data expected mid-'15. Phase I data in Tourette's, which we think qualifies as "orphan," is also anticipated mid-'15. Beyond this candidate, we are intrigued with prospects for deuterated-pirfenidone as a pipeline "hidden gem".

Exhibit 1

EFFICACY RESULTS

Pre-Specified Motor Endpoints	SD-809	Placebo	Treatment Effect	Favors	P-Value
Change in TMC Score[1] from Baseline to Maintenance Therapy*	**4.4 Point Decline**	**1.9 Point Decline**	**Improvement of 2.5 Points over Placebo**	**SD-809**	**p <0.0001**
Percent Change in TMC Score from Baseline to Maintenance Therapy	37 Percentage Points Decline	16 Percentage Point Decline	Improvement of 21 Percentage Points over Placebo	SD-809	p <0.0001
Change in Total Motor Score[1] (TMS) from Baseline to Maintenance Therapy	7.4 Point Decline	3.4 Point Decline	Improvement of 4.0 Points over Placebo	SD-809	p = 0.002

[1]TMC and TMS are subscales of the Unified Huntington's Disease Rating Scale (UHDRS)
*Primary efficacy endpoint

Dose at Week 12 was approximately 40 mg of SD-809.

Pre-specified Key Secondary Endpoints[1]	Favors	P-Value
1. Patient Global Impression of Change (PGIC)[2]	SD-809	**p = 0.002**
2. Clinical Global Impression of Change (CGIC)[2]	SD-809	**p = 0.002**
3. SF-36 Physical Functioning Score (a Quality of Life measure) from Baseline to Week 12	SD-809	**p = 0.03**
4. Berg Balance Test	SD-809	**p = 0.14**

[1] Analyzed using a pre-specified hierarchical testing procedure
[2] Success defined as much improved or very much improved

Source: Company reports

Exhibit 2

SAFETY DATA

System Organ Class	SD-809 n = 45	Placebo n = 45
Psychiatric Disorders	8 (17.8%)	8 (17.8%)
Nervous System Disorders	8 (17.8%)	10 (22.2%)
All Other Body Systems		
Cardiac Disorders	0 (0.0%)	3 (6.7%)
Ear & Labyrinth disorders	1 (2.2%)	1 (2.2%)
Eye Disorders	1 (2.2%)	1 (2.2%)
General Disorders	7 (15.6%)	8 (17.8%)
Gastrointestinal Disorders	9 (20%)	9 (20%)
Hepatobiliary Disorders	1 (2.2%)	0 (0.0%)
Infections and Infestations	5 (11.1%)	5 (11.1%)
Injury, Poisoning and Procedural Complications	4 (8.9%)	6 (13.3%)
Investigations	6 (13.3%)	3 (6.7%)
Musculoskeletal and Connective Tissue Disorders	2 (4.4%)	3 (6.7%)
Renal and Urinary Disorders	2 (4.4%)	1 (2.2%)
Reproductive Systems and Breast Disorders	1 (2.2%)	0 (0.0%)
Respiratory, Thoracic and Mediastinal Disorders	1 (2.2%)	3 (6.7%)
Skin and Subcutaneous Tissue Disorders	2 (4.4%)	1 (2.2%)
Vascular Disorders	2 (4.4%)	0 (0.0%)

Source: Company reports

Exhibit 3

CURRENT VALUATION

	Discount rate	NPV (M$)	NPV per share ($)
SD-809 revenues - HC	15%	1,503	48.07
SD-809 revenues - TD	25%	1,071	34.28
Other income & expenses			
Fixed operating costs	10%	(412)	(13.20)
Net cash (YE15)		94	3.02
Total NPV		**2161**	**72.18**

Source: Piper Jaffray

Auspex *($ in thousands, except per share amounts)*

Income Statement	2012A	2013A	Q1 14A	Q2 14A	Q3 14A	Q4 14E	2014E	Q2 15E	Q2 15E	Q3 15E	Q4 15E	2015E	2016E	2017E	2018E
Net sales SD-809 - HC													25,399	105,689	192,410
Net sales SD-809 - TD													44,001	96,082	157,353
Total Revenues	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**69,400**	**201,771**	**349,763**
Costs & Expenses:															
Cost of Goods Sold	0	0	0	0	0	0	0	0	0	0	0	0	6,940	20,177	34,976
R&D	11,741	10,003	3,432	7,131	10,802	9,000	30,365	8,000	7,000	6,000	5,000	26,000	27,760	30,266	31,479
SG&A	1,688	3,189	2,674	2,908	3,107	3,000	11,689	3,000	3,000	3,000	3,000	12,000	24,290	26,230	27,981
Total Operating Expenses	13,429	13,192	6,106	10,039	13,909	12,000	42,054	11,000	10,000	9,000	8,000	38,000	58,990	76,673	94,436
Operating Income	**(13,429)**	**(13,192)**	**(6,106)**	**(10,039)**	**(13,909)**	**(12,000)**	**(42,054)**	**(11,000)**	**(10,000)**	**(9,000)**	**(8,000)**	**(38,000)**	**10,410**	**125,098**	**255,327**
Interest And Other Income, Net	(1,683)	(2,437)	(383)	(346)	(5,116)	(350)	(6,195)	(350)	(350)	(350)	(350)	(1,400)	(1,400)	(1,400)	(1,400)
Change in Fair Value of Preferred Stock warrant liability	0	0	(3,634)	0	0	0	(3,634)	0	0	0	0	0	0	0	0
Total other Income	**(1,683)**	**(2,437)**	**(4,017)**	**(346)**	**(5,116)**	**(350)**	**(9,829)**	**(350)**	**(350)**	**(350)**	**(350)**	**(1,400)**	**(1,400)**	**(1,400)**	**(1,400)**
Pretax Income (Loss)	**(15,112)**	**(15,629)**	**(10,123)**	**(10,385)**	**(19,025)**	**(12,350)**	**(51,883)**	**(11,350)**	**(10,350)**	**(9,350)**	**(8,350)**	**(39,400)**	**9,010**	**123,698**	**253,927**
Income Taxes	0	0	0	0	0	0	0	0	0	0	0	0	3154	43,294	88,874
Tax Rate								35%	35%	35%	35%		35%	35%	35%
Net Income (Loss) as Reported	**(15,112)**	**(15,629)**	**(10,123)**	**(10,385)**	**(19,025)**	**(12,350)**	**(51,883)**	**(11,350)**	**(10,350)**	**(9,350)**	**(8,350)**	**(39,400)**	**5,857**	**80,404**	**165,052**
Other comprehensive loss	-	-	(22)	49			27					-			
Comprehensive Income (Loss)	**(15,112)**	**(15,629)**	**(10,145)**	**(10,336)**	**(19,025)**	**(12,350)**	**(51,856)**	**(11,350)**	**(10,350)**	**(9,350)**	**(8,350)**	**(39,400)**	**5,857**	**80,404**	**165,052**
Diluted Earnings Per Share as Reported	**($114,485)**	**($371)**	**($0.81)**	**($0.45)**	**($0.73)**	**($0.46)**	**($2.35)**	**($0.42)**	**($0.38)**	**($0.34)**	**($0.30)**	**($1.43)**	**$0.19**	**$2.65**	**$5.41**
Diluted Earnings Per Share Adjusted	**($114,485)**	**($371)**	**($0.81)**	**($0.45)**	**($0.73)**	**($0.46)**	**($2.35)**	**($0.42)**	**($0.38)**	**($0.34)**	**($0.30)**	**($1.43)**	**$0.19**	**$2.65**	**$5.41**
Basic Earnings Per Share as Reported	($114,485)	($371)	($0.81)	($0.45)	($0.73)	($0.46)	($2.35)	($0.42)	($0.38)	($0.34)	($0.30)	($1.43)	$0.19	$2.65	$5.41
Diluted Shares Outstanding (000)	**0.132**	**42.1**	**12,476**	**22,854**	**26,032**	**27,085**	**22,112**	**27,247**	**27,410**	**27,574**	**27,738**	**27,492**	**30,157**	**30,337**	**30,518**
Basic Shares Outstanding (000)	0.132	42.1	12,476	22,854	26,032	27,085	22,112	27,247	27,410	27,574	27,738	27,492	30,157	30,337	30,518

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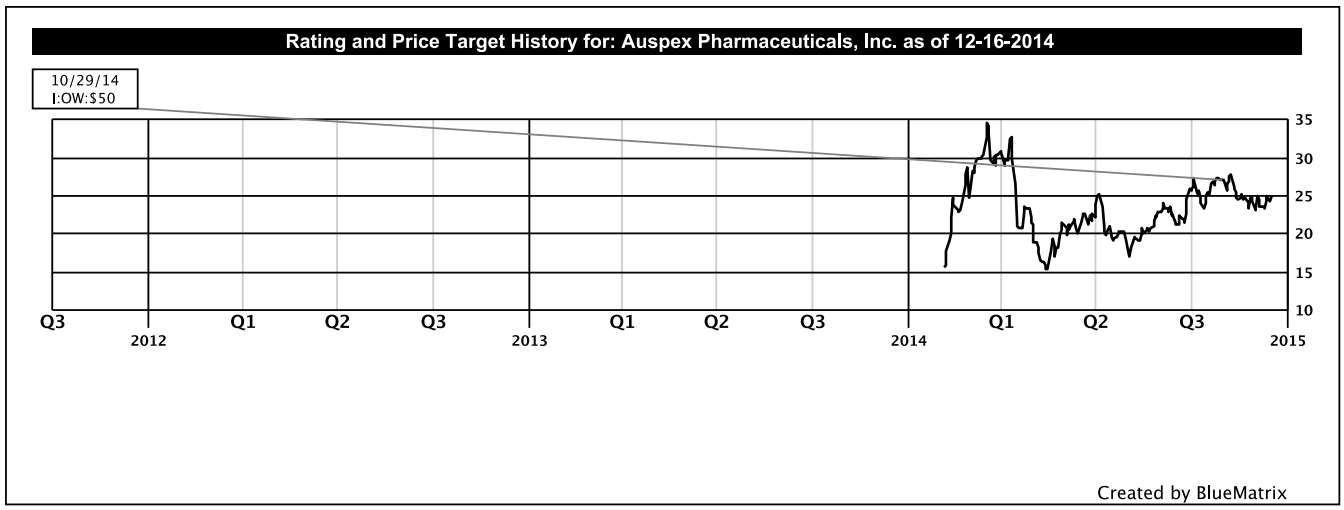

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			IB Serv./Past 12 Mos.	
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HOLD [N]	226	36.22	21	9.29
SELL [UW]	14	2.24	0	0.00

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