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Biotechnology
Company Update
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BUY
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AUSPEX PHARMACEUTICALS, INC.

SD-809 Solid in Phase 3: Meaningful Efficacy, Well Tolerated

ASPX (NASDAQ)

Company & Market Data	
Closing Price (as of 12/16/2014):	$25.09
Rating:	BUY
Price Target:	$44.00
Prior Price Target:	*$37.00*
52 Week Range:	$13.25 - $35.78
Shares Outstanding (MM):	28
Market Capitalization (MM):	$692
Cash (MM):	$159.5
Debt (MM):	$15.0
Fiscal Year End:	Dec

Estimates	2013A	2014E	2015E
EPS			
1Q	NM	$(0.81)A	—
2Q	NM	$(0.45)A	—
3Q	NM	$(0.73)A	—
4Q	$(46.00)	$(0.47)	—
Full Year	$(371.00)	$(2.36)	$(1.69)
Revenue (MM)	$0.0	$0.0	$0.0

Ratios			
P/E	NA	NA	NA

Auspex Pharmaceuticals is a late-clinical stage biopharmaceutical company located in San Diego, California, that is focused on the development and commercialization of novel medicines for the treatment of orphan diseases. Auspex utilizes deuterium-based medicinal chemistry to optimize the behavior of molecules. Deuterium, a naturally-occurring relative of hydrogen, can improve a molecule's pharmacokinetics, with minimal change to its intrinsic potency or selectivity. Auspex's most advanced therapeutic candidate, SD-809, is being developed to address unmet medical needs in hyperkinetic movement disorders, such as Huntington's chorea, tardive dyskinesia and Tourette syndrome, as well as other orphan indications. SD-809 is completing a Phase 3 registration clinical trial for the treatment of chorea (abnormal involuntary movements) associated with Huntington's disease, and is also in pivotal work for tardive dyskinesia, an often permanent movement disorder primarily associated with long term antipsychotic use. Auspex also has several additional deuterated molecules under consideration for more advanced development.

Positive Phase 3 Result in ASPX Target Increase. Auspex (ASPX) is focused on the development of novel medicines for orphan diseases using deuterium-based medicinal chemistry. Largely based on the potential of SD-809 in multiple indications, supported by positive Phase 3 results announced yesterday for it in tardive dyskinesia, we maintain our Buy rating and raise our price target for ASPX shares from $37 to $44. SD-809 is based on tetrabenazine, known as Xenazine in the US. By selectively employing deuterium, Auspex created materially different pharmacokinetics for SD-809 compared to tetrabenazine, a profile that enables less frequent dosing, improved tolerability, and reduced drug metabolism variability.

SD-809 Phase 3 Results: Efficacy, Safety/Tolerability Solid. Auspex announced positive top line Phase 3 results for SD-809, its novel VMAT2 inhibitor for neurological movement disorders. In terms of efficacy, the 90-patient 13-week Phase 3 First-HD study in patients with Huntington's chorea showed SD-809 reduced Total Maximal Chorea score (the primary endpoint) by -4.4 points vs -1.9 for placebo, $p<0.0001$. Other motor endpoints, percent change in TMC score (37% impt. vs. 16% for placebo, $p<0.0001$), and change in total motor score (7.4 vs 3.4, $p=0.002$), were also positive, as were three of four pre-specified key secondary endpoints (patient global impression of change, clinical global impression of change, SD-36 physical functioning score). More importantly, SD-809 appeared quite well tolerated overall, with rates of psychiatric and nervous system disorders comparable to placebo. This safety and tolerability profile is quite important in our view, as it will likely be considered a major point of differentiation for SD-809 compared to Xenazine.

Results of the ARC-HD switching study show chorea control is at least maintained (and possibly improved) and patients can potentially be safely and directly switched to SD-809 from Xenazine. Xenazine revenue for Huntington's chorea is approximately $250 million; despite potential exclusivity loss in 2015, Xenazine's distribution via a Specialty Pharmacy should provide barriers that have the potential to largely maintain pricing. With what appears as improved profile compare to the standard of care Xenazine, we estimate revenue in the HD indication for SD-809 to be in excess of $500 million. We now expect filing for Huntington's chorea during 2Q15, with approval in 2016. With positive Phase 3 now in hand, our NPV estimate for this indication moves from $21.58 to $22.89 per ASPX share.

SD-809: Also Rapidly Advancing in Tardive Dyskinesia and Tourette's. Because of its well-tolerated profile, we believe SD-809 could also be considered in other movement disorders such as tardive dyskinesia (TD) and Tourette syndrome. Auspex is advancing SD-809 in pivotal work for TD, a condition largely arising from the long-term use of antipsychotics. Based on earlier clinical results and favorable discussions with FDA, Auspex recently accelerated this indication, and despite potential competition, we expect pricing to evolve favorably, over $20k annually. Initial pivotal data is expected mid-2015, filing in 2016, approval in 2017, with peak revenue potential approaching $1 billion. With the favorable tolerability seen with SD-809 in Huntington's chorea, we now have modestly de-risked the revenue and profitability associated with SD-809 in TD and Tourette's, increasing its NPV more than $5/share.

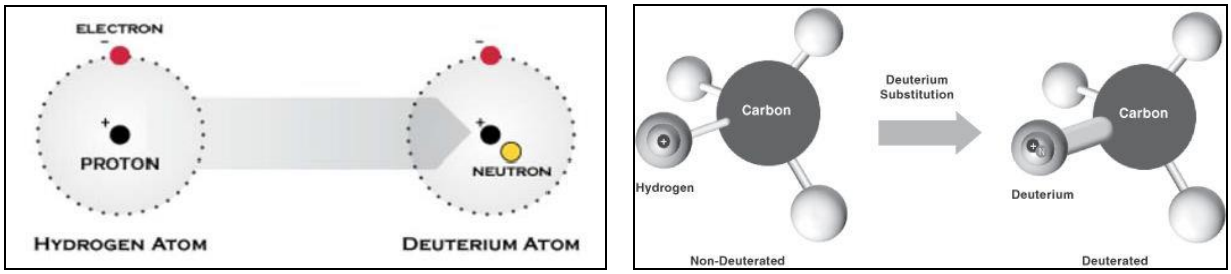
Attractive ASPX Valuation. Using risk-weighted, fully-taxed NPV methodology, we value ASPX shares at $43.74, resulting in our $44 target. With its multiple indications and long patent life, we collectively value SD-809 at $35.51 per ASPX share, with SD-560, other assets, and $5.80 per share in cash providing the remainder.

Auspex Pharmaceuticals – Executive Summary

Auspex – Background. Auspex Pharmaceuticals is a biopharmaceutical company based in San Diego, California, that is focused on the development and commercialization of novel medicines for the treatment of orphan diseases. The company was founded in 2001 and completed its initial public offering in February 2014. Auspex employs a deuterium-based chemistry approach to optimize the behavior of compounds in development in its portfolio. Selectively employing deuterium, a naturally-occurring relative of hydrogen, has the potential to improve a molecule's metabolic properties with minimal-to-no change to its intrinsic pharmacology.

Auspex's lead product candidate, SD-809, a deuterium-modified VMAT2 inhibitor, is in development for various neurological movement disorders. SD-809 is nearing completion of Phase 3 for chorea associated with Huntington's disease, is beginning pivotal studies for its use in tardive dyskinesia, and is being examined in Tourette syndrome. Auspex also has several additional earlier stage deuterium-based product candidates under consideration for advancement in its pipeline. Of particular note due to recent events is SD-560, a deuterated version of pirfenidone. Pirfenidone is Roche's (RHHBY, $36.31, Not rated) molecule that has recently shown important Phase 3 results for idiopathic pulmonary fibrosis (IPF), and was awarded Breakthrough Therapy Status by FDA; the deuterated SD-560 could have advantages in dosing, safety, and possibly efficacy compared to pirfenidone.

Exhibit 1: Hydrogen vs. Deuterium (left), and its Behavior in a Molecule (right)



Source: Auspex Pharmaceuticals Corporate presentation, June 2014; Concert Fact Sheet, May 2013.

Auspex Employs Deuterium Medicinal Chemistry. Deuterium is one of two naturally-occurring stable isotopes of hydrogen. Where hydrogen has one electron and one proton, deuterium also has a neutron in its nucleus, resulting in an atomic mass that is double that of hydrogen (Exhibit 1 – left). Deuterium is not radioactive, and possesses physicochemical properties that are similar to those of hydrogen, but because of its increased mass, carbon-related bonds involving deuterium are generally stronger than similar bonds with hydrogen (Exhibit 1 – right). This bond strengthening with deuterium-based compounds can be enough to result in significant changes in biological reactions compared to related hydrogen-based compounds. Many drugs are metabolized by pathways that involve the breakdown of carbon-hydrogen bonds, and the stronger carbon-deuterium bonds have the potential to alter or deflect the breakdown of the molecule or its metabolites.

Deuterium Can Provide Differentiation and Novel Intellectual Property. Deuterium modification offers an approach to potentially creating materially differentiated new medicines, and importantly, because the behavior of deuterium application is not inherently predictable, novel intellectual property from deuterium-based molecules can be established. Since deuterated assets are often mechanistically similar to a related "parent"

molecule, the application of deuterium to compounds with well understood therapeutic utility can therefore potentially provide an approach with modestly reduced risk with regard to therapeutic development. Once early phase results are established for a deuterated molecule in an indication its parent shares, we believe that program has been materially de-risked. For Auspex's lead asset, SD-809, we believe this to be the case, as it selectively utilizes deuterium to modify the tetrabenazine molecule, improving its profile, resulting in a program that should demonstrate activity and have significant potential in multiple movement disorders, indications and settings.

SD-809: A VMAT2 Inhibitor for Movement Disorders. Auspex's lead product candidate SD-809, is being developed for multiple movement disorders. Its lead indication is for treatment of chorea (abnormal involuntary movements) associated with Huntington's disease (HD). The molecule is in Phase 3 for the indication, with positive results just release. SD-809 is a deuterated form of tetrabenazine, the only therapy approved in the US for Huntington's chorea, known as Xenazine. By selectively employing deuterium to tetrabenazine, SD-809 (dutetrabenazine), the active metabolites of SD-809 have materially different pharmacokinetics than those of Xenazine, providing the Auspex molecule with the potential for less frequent dosing, improved safety/tolerability, reduced variability in drug metabolism, and possibly reduced drug interactions.

SD-809 Has Material Potential in Multiple Indications. Once approved, assuming an improved profile versus Xenazine, we expect SD-809 to be utilized widely for Huntington's chorea. In addition, given SD-809's profile, there is material potential for the Auspex compound to be considered in other movement disorders where Xenazine demonstrated activity, including Tardive dyskinesia (TD) and Tourette syndrome. Along these lines, Auspex has now formally advanced SD-809 into Phase 2/3 clinical studies for tardive dyskinesia, a neurological syndrome that arises from long-term use antipsychotics, and is in early phase work in Tourette's syndrome, a childhood neurodevelopmental disorder involving involuntary movements and sounds. Collectively, these indications provide the SD-809 molecule with material revenue and potential; we estimate peak revenue for SD-809 in Huntington's chorea and other indications in excess of $1 billion annually in the US, and estimate SD-809's risk-weighted, fully-taxed NPV to Auspex at $35.51 per ASPX share. (We currently only consider US revenue for SD-809; if and when international revenue potential manifests, we would consider that upside to our NPV calculations.)

Exhibit 2: Auspex – Recent Events/Upcoming Catalysts

Date	Event	Comment	Significance
Jun '13	Partnership with Huntington's Study Group for SD-809	Auspex collaborates with a leading Huntington's group	◆◆◆
Jul '13	Phase 3 First-HD Study Initiated for SD-809	Initial pivotal study initiate with Auspex's lead asset	◆◆◆◆
Feb '14	Initial public offering for Auspex (ASPX) raises $87.1mm	Funds to support SD-809, portfolio advancement	◆◆◆◆
Jun '14	SD-809 Interim Data from switching study in Huntington's Chorea	No loss of control at 1/2 dose, established switch protocol	◆◆◆◆◆
Jul '14	Secondary offering of $69.7mm for Auspex (ASPX)	Funds for SD-809 move into tardive dyskinesia, Tourette	◆◆
Jul '14	Phase 2/3 Tardive Dyskinesia Study Initiated for SD-809	TD indication now accelerated based on FDA discussion	◆◆◆
4Q14	SD-809 Initial Top Line Phase 3 Data in Huntington's Chorea	Positive Phase 3 data possible due to Xenazine activity	◆◆◆◆◆
4Q14	SD-809 Final Switching Data from in Huntington's Chorea	Positive data possible since interim ARD-HD data solid	◆◆◆◆
4Q14	Early Phase Data for SD-809 in Tourette Syndrome	Important to inform about safety, efficacy in adolescents	◆◆◆◆
4Q14	Phase 3 Tardive Dyskinesia Study Initiated for SD-809	TD indication accelerated, formal Phase 3 study begun	◆◆◆
2015	SD-809 NDA to be filed for Huntington's Chorea	505(b)2 pathway used, which relies on some Xenazine data	◆◆◆
Mid-2015	Initial Phase 2/3 data for SD-809 in Tardive Dyskinesia	Indication could be material, study could aid off label use	◆◆◆◆◆
Mid-2015	SD-560 (deuterated pirfenidone) results IPF/systemic sclerosis	Phase 1 should inform about feasibility of next steps	◆◆◆
2016	SD-809 FDA approval expected for Huntington's Chorea	Indication to be primary revenue generator for Auspex	◆◆◆◆◆
Mid-2016	Formal Phase 3 data for SD-809 in Tardive Dyskinesia	Confirmatory study to support TD filing, indication	◆◆◆◆◆
2H16	NDA to be filed for Tardive Dyskinesia	TD could also be material to revenue potential for AD-809	◆◆◆◆
2017	SD-809 approval expected for Tardive Dyskinesia	Indication to be primary revenue generator for Auspex	◆◆◆◆◆

(Significance: ◆ least important., ◆◆◆◆◆ most important.)

Source: Ladenburg Thalmann BioPharmaceuticals Research.

Auspex – Upcoming Potential Catalysts for 2014, 2015

- **SD-809 – Initial Phase 3 Data Appears Quite Positive.** The Phase 3 data for SD-809 in the Huntington's chorea provided solid efficacy and safety/tolerability. Based on results of this study, Auspex expects to file SD-809 in 2Q15. We consider these Phase 3 results as the most important event upcoming for the company. Given the activity seen with Xenazine in the setting, a reduction of 5 units vs. 1.5 for placebo ($p<0.0001$) on the in the chorea symptom score of the UHDRS scale, and the comparable pharmacology with SD-809's active metabolites, as well as the interim data from the ARC-HD switching study, we had expected SD-809 to be successful. The efficacy seen with SD-809 in First-HD, a reduction of 4.4 units vs. 1.9 for placebo, in general terms, appears comparable to that seen for Xenazine.

- **SD-809 – Tourette's Also Expected in 2015.** SD-809 has demonstrated excellent interim results in its switching study (ARC-HD), which showed that the drug had biologic activity and solid chorea control. Final results of that study further solidified SD-809's potential and should inform physicians of how readily Xenazine may be switched to Auspex's drug. SD-809 is also being examined in a Phase 1b open label study for treatment of tics associated with Tourette syndrome. Efficacy will be examined (the total tic score using the Yale Global Tic Severity Scale and the global clinical impression), in addition to the safety and tolerability of SD-809 in 12 adolescent patients. The treatment period will last 8 weeks, consisting of 6 weeks of titration to an optimal dose, followed by 2 weeks of maintenance. Using Xenazine's activity as a proxy, we also expect SD-809 to demonstrate activity in this setting.

- **SD-809 – Phase 2/3 Tardive Dyskinesia Top Line in Mid-2015.** The initial Phase 2/3 data for SD-809 in the 90-patient tardive dyskinesia study with moderate-to-severe patients should be released in mid-2015. We expect positive results for SD-809 in this setting, as Xenazine and other VMAT inhibitors have demonstrated activity. The trial's primary endpoint is the change in AIMS score from baseline to week 12, and Auspex management has employed recent canny trial design changes such as centralized video raters, to help clarify the activity of the molecule in the setting. (Also, a full-200-patient phase 3 study is under construction in this indication, with top line data, leading to a potential filing, expected in 2016.)

- **SD-560 - for Idiopathic Pulmonary Fibrosis Initial Clinical Results During 2015.** SD-560, a deuterated pirfenidone, is being examined for its potential to provide improvements in safety and tolerability compared to pirfenidone (Esbriet), the Intermune antifibrotic therapeutic that has recently demonstrated important results in Phase 3 in idiopathic pulmonary fibrosis (it achieved breakthrough designation). Pirfenidone is dosed at 2403 mg per day, given as 3 divided doses 3 times per day, providing the potential for deuterium modification to improve its dosing and therapeutic index. Phase 1 should provide guidance as to a clinical path forward.

Auspex Pharmaceuticals - Valuation

Auspex and ASPX Shares Valuation. Because Auspex's operations have the potential for significant revenue and earnings variability over the coming quarters and years, we value the company and its assets using a fully-taxed, risk-weighted net present value methodology for each of its assets. We note that its most significant clinical program, SD-809, given its multiple advanced indications and potential for clinical and regulatory success, at this point comprises the lion's share of Auspex's asset valuation. Though with its deuteration technology, we believe Auspex has the potential to eventually develop a diverse portfolio of therapeutic assets over time, we currently only assess modest

additional value beyond the SD-809 asset. Collectively, we assess the total value of its assets, including its clinical programs, at $43.74 per ASPX share, which underpins our $44 ASPX price target (Exhibit 3).

Exhibit 3: Auspex – ASPX Shares NPV Summary ($000, except per share amts)

Auspex - Assets	NPValue	NPV/Share
SD-809 (Huntington's Chorea)	$ 628,960	$22.89
SD-809 (Tardive dyskinesia, other)	$ 347,006	$12.63
SD-560 (IPF)	$ 84,476	$3.07
Other Assets, Collaborations	$ 12,453	$0.45
Other Corporate	$ (65,351)	($2.38)
Net Cash	$ 159,482	$5.80
NOLs, Credits, etc.	$ 35,043	$1.28
Auspex Asset Valuation	**$ 1,202,069**	**$43.74**

Source: Ladenburg Thalmann BioPharmaceuticals Research.

Valuing Auspex's Proprietary Pipeline. Auspex's most advanced proprietary asset, the deuterated SD-809, is an improved version of the Xenazine (tetrabenazine), a VMAT2 inhibitor approved in the US for chorea related to Huntington's disease, an orphan indication. The deuteration provides SD-809 with what should be an improved profile of equivalent efficacy to Xenazine, with better safety and tolerability, giving it revenue potential exceeding $500 million in that indication alone, even despite the risk of modest generic penetration of the Xenazine franchise. In addition, we believe SD-809's profile provides it with the potential for use in other movement disorders such as tardive dyskinesia and possibly Tourette syndrome, which collectively could more that double the chorea peak revenue. Since these indications have the opportunity to be efficiently marketed to neurology physician specialists, SD-809's $1 billion-plus peak revenue potential creates a risk weighted NPV of $35.51 per ASPX share across all indications.

Additional Auspex assets such as SD-560, a deuterated version of pirfenidone, a molecule that has recently demonstrated considerable promise in idiopathic pulmonary fibrosis, have the potential to show improvements over its related compound. SD-560 and other Auspex pipeline efforts we collectively assess at $3.52 per share, with cash, NOLs and other credits and corporate drag providing another net $4.70 per ASPX share. Other considerations for our NPV calculations include 27.5 million ASPX shares outstanding, and a long-term effective tax rate of 35% for the company.

Deuterium Medicinal Chemistry - In Brief

Deuterium – Fast Facts. Deuterium is one of two naturally-occurring stable isotopes of hydrogen. The far more common hydrogen isotope, protium, has no neutron in its nucleus. Deuterium, however, has a nucleus that contains both one proton and one neutron. Deuterium's symbol is D or ^{2}H. Deuterium possesses physicochemical properties that are similar to those of hydrogen, but its atomic mass is double that of hydrogen due to the presence of the additional neutron. Deuterium has a presence in the ocean water of about 0.0156% per each hydrogen atom, and is generally produced by starting with water, which contains a small amount of heavy water, where deuterium replaces the hydrogen in the water molecules; this heavy water is then separated for the deuterium. According to multiple sources, the average human body contains about 2 grams of deuterium.

Deuteration – General Effects on a Molecule. Chemically, deuterium often behaves comparably to ordinary hydrogen, but there are differences in bond energy and bond length for compounds that are deuterated isotopes; these differences are larger than the isotopic differences in any other element. Bonds involving deuterium (and another more rare hydrogen isotope, tritium) with carbon are somewhat stronger than the corresponding bonds in hydrogen, enough to occasionally create significant changes in biological reactions. This is because many drugs are metabolized by pathways that involve the breaking of carbon-hydrogen bonds. Having stronger deuterium-carbon bonds helps deflect or prevent the breakdown of those bonds. Incorporation of deuterium in place of hydrogen at selective points in a molecule therefore has the potential of retaining the biochemical potency and selectivity of a physiologically active compound, while potentially modifying its metabolic properties and substantially altering its therapeutic profile.

Deuteration in Drug Development: Material Advancements, Fresh IP. Deuterated compounds have been used in the clinic for some time as probes for pharmacokinetic and metabolism studies of the related non-deuterated therapeutics, though only relatively recently has deuterium modification received material attention as an approach to creating enhanced therapeutics. Deuteration can enhance bioavailability and improve the half-life of a compound, and deuterium substitution at specific molecular positions can improve metabolic stability, reduce or eliminate the formation of toxic metabolites, or even increase the formation of desired active metabolites. However, when deuterium is incorporated at a known site of oxidative metabolism, because of the complexity of the process, deuterium's effects on the absorption, distribution, metabolism, and excretion of a molecule is inherently unpredictable. For example, complex enzymatic metabolism may often have other rate-limiting steps, and/or the presence of a stabilized carbon-deuterium bond may cause metabolism to shift to another site or sites on the molecule. Since the behavior of the deuteration of a molecule is largely unpredictable (non-obvious), deuterated compounds are therefore able to generate new intellectual property.

Deuteration May Result In A Truncated Development Scheme. The application of deuterium to a molecule has the potential, depending upon specifics of the program, to have some elements of its development program truncated/reduced. For instance, with SD-809, Auspex is using a 505(b)(2) approval pathway that allows some information to be gleaned from the original parent drug's application, in this case Xenazine (tetrabenazine). Another example is the deuterated program from Avanir (AVNR, $13.17, Not Rated), AVP-786, where FDA has permitted that company to utilize data generated from its original dextromethorphan-based program AVP-923, and apply it to the development package for the deuterated dextromethorphan analog AVP-786. This means SD-809 and AVP-786 can proceed without duplicating the entire clinical program of their respective parent molecules, creating material efficiencies in terms of time and resources. It is uncertain if this type of consideration is possible for all programs, though when it is available, it should clearly be beneficial in terms of development efficiencies.

Auspex Pharmaceuticals - Risks

The following Risks include, but are not limited to:

Regulatory/FDA. As with any company whose main business focuses on the development of pharmaceuticals, Auspex is subject to the strenuous regulatory requirements of the US Food and Drug Administration (FDA) and other international regulatory agencies such as the EMEA to have its new drugs approved. Promotion of its approved drug products is also highly regulated by FDA and related agencies throughout the globe. Also, in general, though the company's specific focus on ethical (prescription) pharmaceuticals places significant risk on its operations due to the scrutiny of FDA and

other governmental regulatory bodies, we believe this specific risk over time should be no greater than that for any other research-based drug development company.

Material Dependence Upon SD-809 Progress. SD-809 for Huntington's chorea and other movement disorders is Auspex's most advanced proprietary clinical candidate in development. It may take material time and resources to finish clinical development, if it is able to complete at all, and there is certainly no guarantee that the company will be successful in doing so. In addition, Auspex may seek collaborators for future development of SD-809, and there is a risk that the company may not be able to enter into a collaboration for the therapy, or is able to enter into one with terms that are beneficial to ASPX shareholders. This development program has garnered major investor interest and we believe represents a material portion of Auspex's valuation; if it does not progress, there is material risk that ASPX shares could trade downward.

SD-809 – Pricing, Competitive Risks. SD-809 is expected to be introduced to patients in the US that suffer from Huntington's chorea. The current approved therapy, Xenazine for the condition is priced well above $50,000 per annum for those patients. Xenazine is due to lose exclusivity during 2015, which could expose the therapy to generic competition. Due to the specialized distribution of that therapy, there is an expectation for limited generic pressures, leading to the maintenance of high pricing for therapeutics for the condition. If competition is more aggressive, pricing for SD-809 could become more challenging. There is the potential for competition for SD-809 from Neurocrine's NBI-98854, a VMAT2 inhibitor in development for tardive dyskinesia. As there is no approved therapy for TD in the US, we believe the TD market is largely untapped and there is room for multiple competitors, overly aggressive (low) pricing for this molecule in tardive dyskinesia (TD) could result in material competitive pressures for SD-809 if it is also successful in TD. We believe Auspex would respond to lower pricing, and that additional utilization could largely offset any pricing decrease as Auspex moved SD-809 into the TD indication, though there is no guarantee of this.

505(b)(2) Pathway. Auspex anticipates utilizing the abbreviated 505(b)(2) registration pathway in pursuit of the approval of SD-809 in Huntington's chorea, tardive dyskinesia, and possibly for Tourette syndrome, which relies to some extent on information from the drug tetrabenazine. Though many molecules have used this pathway previously, there is no guarantee as to the suitability of this type of approach for Auspex with SD-809. If FDA ultimate decides the 505(b)(2) approach is not appropriate for SD-809 in these indications, material additional resources may need to be expended to gain approval, resulting in a significant change in timing for the product's introduction, which would likely significantly negatively pressure ASPX shares.

Deuteration: Approval, Manufacturing Risks. To the best of our knowledge, no deuterated drug has ever been successfully approved or commercialized, and SD-809 is a deuterated version of tetrabenazine, an approved molecule for the treatment of Huntington's chorea. There may be specific risks to gaining licensure for these types of deuterated agents from regulatory authorities, though these do not appear to have not emerged at this point. In addition, the company may also incur unforeseen manufacturing challenges with deuterated compounds, or manufacturing costs that are required for the production of any product candidate that receives marketing approval may turn out to be substantial, though excessive cost have not specifically manifested at this point.

Other Risks. Auspex has incurred significant losses since inception, expects to incur losses for at least the next several years, and may never sustain profitability. Auspex also has a limited operating history and no history of commercializing pharmaceutical products, which may make it difficult to evaluate the prospects for future viability. The terms of

Auspex's term loan require the company to meet certain operating and financial covenants and placing restrictions on their operating and financial flexibility. If it raises additional capital through debt financing, the terms of any new debt could further restrict its ability to operate their business effectively. Auspex's principal stockholders and management own a significant percentage of company shares and will be able to exert significant control over matters subject to stockholder approval. Auspex is an "emerging growth company," and the reduced disclosure requirements applicable to emerging growth companies may make its common stock less attractive to investors.

Auspex - Income Statement ($000)	2012A	2013A	1Q14A	2Q14A	3Q14A	4Q14e	2014e	2015E	2016E	2017E	2018E
Product Revenue											
- US	*0*	*0*	*0*	*0*	*0*	*0*	*0*	*0*	*56,944*	*118,180*	*171,109*
- International	*0*	*0*	*0*	*0*	*0*	*0*	*0*	*0*	*0*	*0*	*0*
SD-809 (Huntington's Chorea)	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 55,944	$ 110,680	$ 140,056
- US	*0*	*0*	*0*	*0*	*0*	*0*	*0*	*0*	*55,944*	*110,680*	*140,056*
- International	*0*	*0*	*0*	*0*	*0*	*0*	*0*	*0*	*0*	*0*	*0*
SD-809 (Tardive dysk., Tourette's, off label)	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ -	$ 1,000	$ 7,500	$ 31,054
- US	*0*	*0*	*0*	*0*	*0*	*0*	*0*	*0*	*1,000*	*7,500*	*31,054*
- International	*0*	*0*	*0*	*0*	*0*	*0*	*0*	*0*	*0*	*0*	*0*
Other collab revenue	*0*	*0*	0	0	0	0	*0*	0	0	$ -	$ -
Total Proprietary Revenue	*0*	*0*	**0**	**0**	**0**	**0**	*0*	**0**	**56,944**	**118,180**	**171,109**
Collaborative Research Revenue	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**
Total Auspex Revenue	**$0**	**$0**	**$0**	**$0**	**$0**	**$0**	**$0**	**$0**	**$56,944**	**$118,180**	**$171,109**
Expenses:											
COGS	$0	$0	*$0*	*$0*	*$0*	*$0*	$0	$0	$4,840	$9,454	$13,689
R & D	11,741	10,003	*3,432*	*7,131*	*10,802*	*9,500*	30,865	30,000	25,000	25,000	27,500
S G & A	1,688	3,189	*2,674*	*2,908*	*3,107*	*3,150*	11,839	15,000	35,000	37,500	50,000
Total Expenses	$13,429	$13,192	$6,106	$10,039	$13,909	$12,650	42,704	$45,000	$64,840	$71,954	$91,189
Operating Income	(13,429)	(13,192)	(6,106)	(10,039)	(13,909)	(12,650)	(42,704)	(45,000)	(7,897)	46,225	79,921
Operating Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Other Income											
Asset sale	0	0	0	0	0	0	0	0	0	0	0
Grant Income	0	0	0	0	0	0	0	0	0	0	0
Interest income (expense) net	-1292	-248	-383	-346	-359	-315	-1403	-1000	-750	-500	-250
Gain from debt restructure	0	0	0	0	0	0	0	0	0	0	0
Other financing expense	*-391*	*-2,189*	(3,634)	0	(4,757)	300	-8,091	0	0	0	1,500
Total Other Income	(1,683)	(2,437)	(4,017)	(346)	(5,116)	(15)	(9,494)	(1,000)	(750)	(500)	1,250
Pretax Income	(15,112)	(15,629)	(10,123)	(10,385)	(19,025)	(12,665)	(50,795)	(46,000)	(8,647)	45,725	81,171
Pretax Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Effective Taxes	-	-	-	-	-	-	-	-	-	2,286	6,088
Tax Rate	*38.0%*	*38.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*5.0%*	*7.5%*
Fully Taxed rate	(5,743)	(5,939)	(3,847)	(3,946)	(7,230)	(4,813)	(19,302)	(17,480)	(3,286)	17,376	30,845
Tax Rate	*38.0%*	*38.0%*	*38.0%*	*38.0%*	*38.0%*	*38.0%*	*38.0%*	*38.0%*	*38.0%*	*38.0%*	*38.0%*
Participation of Preferred Stock	-	-	-	-	-	-	-	-	-	-	-
Net Income - Effective taxed	(15,112)	(15,629)	(10,123)	(10,385)	(19,025)	(12,665)	(52,198)	(46,000)	(8,647)	43,439	75,083
Income - Fully taxed	(9,369)	(9,690)	(6,276)	(6,439)	(11,796)	(7,852)	(32,363)	(28,520)	(5,361)	28,350	50,326
Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Other Comprehensive Loss	$ -	$ -	$ (22)	$ 49	$ (13)	$ -	$ 14	$ -	$ -	$ -	$ -
Comprehensive Gain (Loss)	$ (15,112)	$ (15,629)	$ (10,145)	$ (10,336)	$ (19,038)	$ (12,665)	$ (52,184)	$ (46,000)	$ (8,647)	$ 43,439	$ 75,083
EPS (ex-charges; eff. taxed)	**($114,485)**	**($371)**	**($0.81)**	**($0.45)**	**($0.73)**	**($0.47)**	**($2.36)**	**($1.69)**	**($0.32)**	**$1.54**	**$2.66**
EPS (ex-charges; fully-taxed)	**($70,981)**	**($230)**	**($0.50)**	**($0.28)**	**($0.45)**	**($0.29)**	**($1.46)**	**($1.05)**	**($0.20)**	**$1.01**	**$1.78**
EPS (Comprehensive gain/loss)	**($114,485)**	**($371)**	**($0.81)**	**($0.45)**	**($0.73)**	**($0.47)**	**($2.36)**	**($1.69)**	**($0.32)**	**$1.54**	**$2.66**
Shares O/S (000), Fully Diluted	0.1	42	12,476	22,854	26,032	27,050	22,103	27,150	27,425	28,175	28,275
				-- Expenses (% of sales) --							
Cost of Sales (product sales)	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%	8.5%	8.0%	8.0%
Gross	NM	NM	NM	NM	NM	NM	NM	NM	91.5%	92.0%	92.0%
R & D	NM	NM	NM	NM	NM	NM	NM	NM	43.9%	21.2%	16.1%
S G & A	NM	NM	NM	NM	NM	NM	NM	NM	61.5%	31.7%	29.2%
Total	NM	NM	NM	NM	NM	NM	NM	NM	113.9%	60.9%	53.3%
				-- Year / Year Growth --							
Revenue	NM	NM	NM	NM	NM	NM	NM	NM	NM	107.5%	44.8%
Operating Income	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Pretax Income	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Income	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
EPS (ex-charges)	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
EPS (ex-charges; fully-taxed)	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM

Source: Auspex Pharmaceuticals SEC filings and Ladenburg Thalmann BioPharmaceuticals Research estimates.

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Auspex Pharmaceuticals - Balance Sheet & Statement of Cash Flows ($mm)

BALANCE SHEET

ASSETS	**12/31/2012A**	**12/31/2013A**	**12/31/2014E**	**12/31/2015E**	**12/31/2016E**	**12/31/2017E**	**12/31/2018E**
Cash & equivalents	$4.3	$36.7	$144.9	$97.1	$79.9	$118.4	$191.5
Investments	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Account receivable	$0.0	$0.0	$0.0	$0.0	$5.7	$11.8	$17.1
Prepaid & other current assets	$0.2	$0.2	$0.0	$0.0	$0.6	$1.2	$1.7
Total Current Assets	$4.4	$36.9	$144.9	$97.1	$86.1	$131.4	$210.3
Deferred offering costs	$0.0	$1.8	$0.0	$0.0	$0.0	$0.0	$0.0
Property & Equipment, net	$0.0	$0.0	$0.0	$1.0	$2.0	$3.0	$5.0
Other assets	$0.0	$0.1	$0.3	$0.3	$0.3	$0.5	$0.5
Total Assets	**$4.5**	**$38.9**	**$145.2**	**$98.4**	**$88.4**	**$134.9**	**$215.8**
LIABILITIES & S.E.							
Accounts payable	$0.9	$1.4	$2.5	$3.2	$5.7	$11.8	$17.1
Accrued expenses/lliabilities	$0.3	$2.1	$2.3	$2.5	$2.7	$2.9	$3.1
Deferred short-term revenue	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Warrant liability	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Current portion of LTD/Conv notes payable	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Total Current Liabilities	**$1.1**	**$3.5**	**$4.8**	**$5.7**	**$8.4**	**$14.7**	**$20.2**
Note payable	$0.0	$14.4	$14.5	$11.5	$6.5	$1.5	$0.0
Preferred stock warrant/tranche liability	$3.1	$4.0	$0.0	$0.0	$0.0	$0.0	$0.0
Other long term liabilities	$0.0	$0.1	$0.1	$0.1	$0.1	$0.1	$0.1
Total Long-term Liabilities	**$3.1**	**$18.5**	**$14.6**	**$11.6**	**$6.6**	**$1.6**	**$0.1**
Total liabilities	**$4.3**	**$22.0**	**$19.4**	**$17.3**	**$15.0**	**$16.3**	**$20.3**
Convertible preferred	**$49.8**	**$81.8**	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**
Add Paid-in-capital	$0.2	$0.5	$240.3	$245.0	$246.0	$247.0	$247.0
Accumulated deficit	($49.9)	($65.5)	($114.5)	($163.9)	($172.5)	($128.4)	($51.5)
Total Shareholders Equity (Deficit)	**($49.6)**	($64.9)	$125.8	$81.1	$73.5	$118.6	$195.5
Total Liability & SH Equity	**$4.5**	**$38.9**	**$145.2**	**$98.4**	**$88.4**	**$134.9**	**$215.8**

CASH FLOW STATEMENT	**12/31/2012A**	**12/31/2013A**	**12/31/2014E**	**12/31/2015E**	**12/31/2016E**	**12/31/2017E**	**12/31/2018E**
Cash Flow from Operating Activities							
Net income	(15.1)	(15.6)	(52.2)	(46.0)	(8.6)	43.4	75.1
Depreciation	0.0	0.0	0.3	0.3	0.3	0.3	0.3
Chg in preferred stock warrant liability	0.2	1.9	5.0	0.0	0.0	0.0	0.0
Amort of debt discount on convert notes	0.8	0.1	0.1	0.0	0.0	0.0	0.0
Other financing expense	0.2	0.3	0.3	0.5	0.5	0.5	0.3
Noncash compensation expense	0.1	0.3	0.5	0.5	0.5	1.0	1.5
Forgiveness of notes receivable	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Gain on sale of assets	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Noncash interest on converts payable	0.5	0.0	0.0	0.0	0.0	0.0	0.0
Gain on troubled debt restructure	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Accounts receivable	0.0	0.0	0.0	0.0	(5.7)	(6.1)	(5.3)
Prepaid expenses, and other current assets	0.0	(0.1)	0.2	0.0	(0.6)	(0.6)	(0.5)
Accounts payable and accrued expenses	0.6	1.9	1.3	0.9	2.7	6.3	5.5
Other operating activities	(0.0)	(0.1)	-	-	-	-	-
Cash Flow from Operating Activities	**(12.7)**	**(11.3)**	**(44.5)**	**(43.8)**	**(10.9)**	**44.9**	**76.9**
Cash Flow from Investing Activities							
Capital Expenditures, net	(0.0)	(0.0)	(0.3)	(1.0)	(1.3)	(1.3)	(2.3)
Other	0.0	0.0	-	-	-	-	-
Cash Flow from Investing Activities	**(0.0)**	**(0.0)**	**(0.3)**	**(1.0)**	**(1.3)**	**(1.3)**	**(2.3)**
Cash Flow from Financing Activities							
Proceeds from issuance of common stock	0.0	0.0	156.9	0.0	0.0	0.0	0.0
Deferred IPO Costs	0.0	(1.3)	0.0	0.0	0.0	0.0	0.0
Issuance/(Retirement) of Debt, Other	13.8	45.0	(3.9)	(3.0)	(5.0)	(5.0)	(1.5)
Cash Flow from Financing Activities	**13.8**	**43.7**	**153.0**	**(3.0)**	**(5.0)**	**(5.0)**	**(1.5)**
Beginning cash balance	**3.2**	**4.3**	**36.7**	**144.9**	**97.1**	**79.9**	**118.4**
Net increase (decrease) in cash	1.1	32.4	108.2	(47.8)	(17.2)	38.5	73.1
Ending cash balance	**4.3**	**36.7**	**144.9**	**97.1**	**79.9**	**118.4**	**191.5**

Source: Auspex Pharmaceuticals, Inc. SEC filings, Ladenburg Thalmann BioPharmaceuticals research estimates.

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APPENDIX A: IMPORTANT RESEARCH DISCLOSURES

ANALYST CERTIFICATION

I, Robert C. Hazlett, attest that the views expressed in this research report accurately reflect my personal views about the subject security and issuer. Furthermore, no part of my compensation was, is, or will be directly or indirectly related to the specific recommendation or views expressed in this research report, provided, however, that:

The research analyst primarily responsible for the preparation of this research report has or will receive compensation based upon various factors, including the volume of trading at the firm in the subject security, as well as the firm's total revenues, a portion of which is generated by investment banking activities.

Additional information regarding the contents of this publication will be furnished upon request. Please contact Ladenburg Thalmann, Compliance Department, 570 Lexington Avenue, 11th floor, New York, New York 10022 (or call 212-409-2000) for any information regarding current disclosures, and where applicable, relevant price charts, in regard to companies that are the subject of this research report.

COMPANY BACKGROUND

Auspex Pharmaceuticals is a late-clinical stage biopharmaceutical company located in San Diego, California, that is focused on the development and commercialization of novel medicines for the treatment of orphan diseases. Auspex utilizes deuterium-based medicinal chemistry to optimize the behavior of molecules. Deuterium, a naturally-occurring relative of hydrogen, can improve a molecule's pharmacokinetics, with minimal change to its intrinsic potency or selectivity. Auspex's most advanced therapeutic candidate, SD-809, is being developed to address unmet medical needs in hyperkinetic movement disorders, such as Huntington's chorea, tardive dyskinesia and Tourette syndrome, as well as other orphan indications. SD-809 is completing a Phase 3 registration clinical trial for the treatment of chorea (abnormal involuntary movements) associated with Huntington's disease, and is also in pivotal work for tardive dyskinesia, an often permanent movement disorder primarily associated with long term antipsychotic use. Auspex also has several additional deuterated molecules under consideration for more advanced development.

VALUATION METHODOLOGY

Auspex and ASPX Shares Valuation. Because Auspex's operations have the potential for significant revenue and earnings variability over the coming quarters and years, we value the company and its assets using a fully-taxed, risk-weighted net present value methodology for each of its assets. We note that its most significant clinical program, SD-809, given its multiple advanced indications and potential for clinical and regulatory success, at this point comprises the lion's share of Auspex's asset valuation. Though with its deuteration technology, we believe Auspex has the potential to eventually develop a diverse portfolio of therapeutic assets over time, we currently only assess modest additional value beyond the SD-809 asset. Collectively, we assess the total value of its assets, including its clinical programs, at $43.74 per ASPX share, which underpins our $44 ASPX price target (Exhibit 3).

Valuing Auspex's Proprietary Pipeline. Auspex's most advanced proprietary asset, the deuterated SD-809, is an improved version of the Xenazine (tetrabenazine), a VMAT2 inhibitor approved in the US for chorea related to Huntington's disease, an orphan indication. The deuteration provides SD-809 with what should be an improved profile of equivalent efficacy to Xenazine, with better safety and tolerability, giving it revenue potential exceeding $500 million in that indication alone, even despite the risk of modest generic penetration of the Xenazine franchise. In addition, we believe SD-809's profile provides it with the potential for use in other movement disorders such as tardive dyskinesia and possibly Tourette syndrome, which collectively could more that double the chorea peak revenue. Since these indications have the opportunity to be efficiently marketed to neurology physician specialists, SD-809's $1 billion-plus peak revenue potential creates a risk weighted NPV of $35.51 per ASPX share across all indications.

Additional Auspex assets such as SD-560, a deuterated version of pirfenidone, a molecule that has recently demonstrated considerable promise in idiopathic pulmonary fibrosis, have the potential to show improvements over its related compound. SD-560 and other Auspex pipeline efforts we collectively assess at $3.52 per share, with cash, NOLs and other credits and corporate drag providing another net $4.70 per ASPX share. Other considerations for our NPV calculations include 27.5 million ASPX shares outstanding, and a long-term effective tax rate of 35% for the company.

RISKS

Regulatory/FDA. As with any company whose main business focuses on the development of pharmaceuticals, Auspex is subject to the strenuous regulatory requirements of the US Food and Drug Administration (FDA) and other international regulatory agencies such as the EMEA to have its new drugs approved. Promotion of its approved drug products is also highly regulated by FDA and related agencies throughout the globe. **Material Dependence Upon SD-809 Progress.** SD-809 for Huntington's chorea and other movement disorders is Auspex's most advanced proprietary clinical candidate in development. It may take material time and resources to finish clinical development, if it is able to complete at all, and there is certainly no guarantee that the company will be successful in doing so. We believe SD-809 has garnered major investor interest and represents a material portion of Auspex's valuation; if it does not progress, or progresses more slowly than estimated, there is material risk that ASPX shares could trade downward. **Deuteration: Approval, Manufacturing Risks.** To the best of our knowledge,

no deuterated drug has ever been successfully approved or commercialized, and SD-809 is a deuterated version of tetrabenazine, an approved molecule for the treatment of Huntington's chorea. There may be specific risks to gaining licensure for these types of deuterated agents from regulatory authorities, though these do not appear to have not emerged at this point. **Other Risks.** Auspex is an "emerging growth company," and the reduced disclosure requirements applicable to emerging growth companies may make its common stock less attractive to investors.

STOCK RATING DEFINITIONS

Buy: The stock's return is expected to exceed 12.5% over the next twelve months.

Neutral: The stock's return is expected to be plus or minus 12.5% over the next twelve months.

Sell: The stock's return is expected to be negative 12.5% or more over the next twelve months.

Investment Ratings are determined by the ranges described above at the time of initiation of coverage, a change in risk, or a change in target price. At other times, the expected returns may fall outside of these ranges because of price movement and/or volatility. Such interim deviations from specified ranges will be permitted but will become subject to review.

RATINGS DISPERSION AND BANKING RELATIONSHIPS AS OF (December 17, 2014)

Rating	%	IB %
BUY	75.0	57.9
NEUTRAL	25.0	42.1
SELL	0.0	0.0

COMPANIES UNDER ROBERT'S COVERAGE

Acadia Pharmaceuticals Inc. (ACAD)
Cempra Inc. (CEMP)
CTI BioPharma Corp. (CTIC)
OvaScience, Inc. (OVAS)
Paratek Pharmaceuticals, Inc. (PRTK)
Auspex Pharmaceuticals, Inc. (ASPX)
Concert Pharmaceuticals Inc. (CNCE)
Nektar Therapeutics (NKTR)
Prothena Corporation plc (PRTA)
Targacept, Inc. (TRGT)

COMPANY SPECIFIC DISCLOSURES

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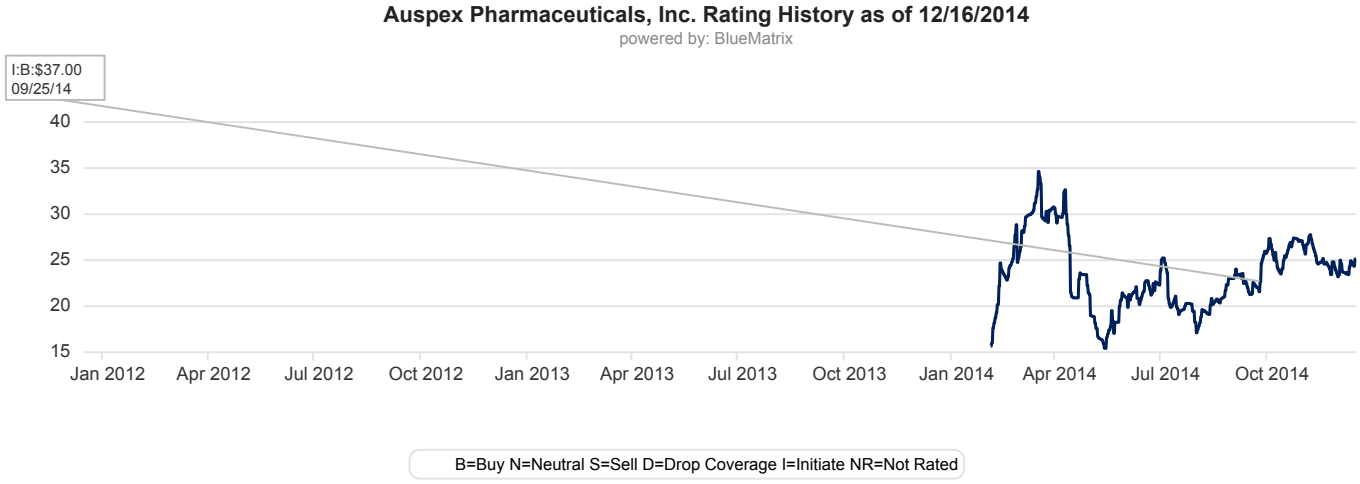
Ladenburg Thalmann & Co. Inc. has managed or co-managed a public offering for Auspex Pharmaceuticals, Inc. within the past 12 months.

Ladenburg Thalmann & Co. Inc received compensation for investment banking services from Auspex Pharmaceuticals, Inc. within the past 12 months.

Ladenburg Thalmann & Co. Inc had an investment banking relationship with the Auspex Pharmaceuticals, Inc. within the last 12 months.

OTHER COMPANIES MENTIONED

INVESTMENT RATING AND PRICE TARGET HISTORY



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