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EQUITY RESEARCH
Company Report

April 25, 2018

# Asterias Biotherapeutics (AST - $1.25)

*Healthcare/Biotechnology*

| | |
|---|---|
| Ticker: | **AST** |
| Rating: | **Buy** |
| Price Target: | **$12.00** |

## AST-VAC2 Production is Ready, Phase I in Non-Small Cell Lung Cancer Study to Start Later in 2Q18

AST this morning reported that the first cGMP clinical grade lot of AST-VAC2 was released by the Cancer Research UK. As such, the AST-VAC2 in non-small cell lung cancer (NSCLC) Phase II study could start in 2Q18.

- **Details.** With the release of the clinical lot of AST-VAC2 already on the rearview mirror, AST expects to start the Phase I in NSCLC study later in 2Q18 from three clinical sites. As a reminder, the trial will enroll up to 24 patients grouping into two cohorts. The first cohort (n~12) is comprised of patients with advanced NSCLC and the study group will carry the major histocompatibility gene, HLA-A2. All will receive six weekly injections of AST-VAC2. The objective of the study is for the safety, immune responses to telomerase and OS. These survival results will be compared to patients of a control group who do not possess the HLA-A2 gene. Should the safety be demonstrated by the first cohort, the trial will advance into the second cohort. In this part of the study (n~12), eligible patients are those with early stage NSCLC who have undergone successful tumor resection and without signs of metastasis. Again, patients of the study group will carry the major histocompatibility allele HLA-A2. All will receive six, weekly injections of AST-VAC2. The clinical data readout is the same as the first cohort by comparison with a control group who are not HLA-A2+. Follow-up duration for immune response to telomerase is one year, and survival endpoint is 2 years. AST and Cancer Research UK are also exploring the combination of AST-VAC2 with CPI in NSCLC and other cancer indications going forward. CPI is becoming the SOC in NSCLC. Two CPIs are currently approved in the EU.
- **Implications.** Given that the production of cGMP grade AST-VAC2 has been an issue that held up the commencement of the NSCLC Phase II trial, today's announcement could be an important step to remove this hurdle and advance the clinical program forward. As such, we believe AST could enroll the first patient for the AST-VAC2 in NSCLC Phase I study toward the end of 2Q18. Although the AST-OPC1 in spinal cord injury clinical development has been a major focus by investors, the clinical advancement of AST-VAC2 would afford a second value driver and more diversification for the pipeline offering for investors.
- **Action.** We are reiterating our Buy rating and $12 target price. Our valuation is based on peer comparable, probability adjusted DCF and sum-of-the-parts analyses. We believe AST shares are undervalued given its differentiated SCI treatment modality and potentially positive multiple catalysts in next 18 months.

**Trading Data:**

| | |
|---|---|
| Last Price (4/24/20188) | $1.25 |
| 52–Week High (4/26/20177) | $4.30 |
| 52–Week Low (4/13/2018) | $1.25 |
| Market Cap. (MM) | $71 |
| Shares Out. (MM) | 50.27 |

**Earnings Estimates:** (per share)

| (Dec) | 1Q | 2Q | 3Q | 4Q | FY | P/E |
|---|---|---|---|---|---|---|
| **FY-18E** | -0.16 | -0.10 | -0.09 | -0.10 | -0.44 | N.A. |
| **FY-17A** | -0.13 | -0.18 | -0.14 | -0.12 | -0.56 | N.A. |
| **FY-16A** | -0.27 | -0.12 | -0.24 | -0.20 | -0.83 | N.A. |
| **FY-15A** | -0.09 | -0.10 | -0.09 | -0.13 | -0.42 | N.A. |

*Source: Laidlaw & Company estimates*

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## Anticipated milestones in 2018 and beyond

| Product | Indication | Event | Timing | Importance |
|---|---|---|---|---|
| AST-OPC1 | Subacute spinal cord injury (SCI) cervical complete (AIS-A at C5-C7) | Report 20MM cell 12-month results | **Aug. 18** | *** |
| | | Report 10MM cell 24-month results | **4Q18** | *** |
| | Subacute spinal cord injury (SCI) cervical incomplete (AIS-B at C5-C7) | Report 10MM cell 12-month results | **Aug. 18** | **** |
| | | Report 20MM cell 6-month results | **3Q18** | **** |
| | | Report 20MM cell 12-month results | **4Q18/1Q19** | *** |
| | Subacute spinal cord injury (SCI) cervical | More formal discussion with the FDA for possible Phase II and III trial | **2H18** | *** |
| | | Potentially finalize Phase IIb trial design after FDA discussion | **4Q18/1Q19** | *** |
| | | Potentially start Phase IIb trial | **2019** | *** |
| AST-VAC1 | Acute myeloid leukemia (AML) | Potentially start Phase IIb confirmatory trial | **2018** | *** |
| AST-VAC2 | Non-small cell lung cancer (NSCLC) | Potentially start Phase I/IIa trial | **2Q18** | *** |

***** / ***** Major catalyst event that could impact share price very significantly while *** event is more informative*

*Source: Laidlaw & Company and company presentation*


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## Major Risks

**Clinical study failure could have a major impact on AST share value.** Despite promising pre-clinical and clinical results of the company's lead product, AST-OPC1, it remains too early to predict the long-term safety and efficacy outcomes from the upcoming clinical studies. Given that clinical validation has not been fully established, near term, it would be critical for the additional studies of the ongoing Phase I/II trial to demonstrate efficacy and a positive safety profile after a longer follow-up, higher dosage and broader patient population in order to increase the assets and shareholder value. Negative results of ongoing and future clinical studies could have a materially negative impact on the shareholder value; especially since the company has a relatively diverse-limited pipeline profile. In addition, given there has been very limited progress over the last two decades in developing therapeutics for treating SCI, the overall risks in developing an effective treatment in this area could be higher than in other disease areas.

**Yet-to-be-validated pluripotent stem cell platform could remain uncertain.** Although stem cell-based therapies have been tested in many clinical trials in recent years; there is currently no pluripotent stem cell-based therapy approved for the treatment of spinal cord injury or other disease indications. As such, clinical risks for pluripotent stem cell-based therapies are higher than similar products generated from other more proven development platforms.

**Product may not be approved or reach anticipated sales.** Although AST's current pipeline products have exhibited the potential to generate positive clinical outcomes from current and future trials; it remains too early to project whether any of these products would be approved by regulatory agencies. Even if the products were to enter the market, sales could be significantly below projections due to the specific product label under approval, physician consensus for prescribing the drug, changes of treatment paradigms, entrance of competitors, and the possible changes in pricing flexibility that could potentially have a negative impact on payer reimbursement. Other potential commercial risks also include the societal or political pressure that could limit premium pricing capability for many orphan drugs moving forward. Further, a below expectation revenue outlook could also negatively affect AST shareholder value.

**Additional financings could dilute shareholder value.** Although the company currently has ~$25MM total cash, AST would most likely need more financial resources going forward if they want to expand and further develop their pipeline unless the company can continuously explore un-dilutive financial sources. Should the future operational expenses significantly increase, especially in the areas of R&D and SG&A, products not receive FDA approval, or product revenue not reach expectations; the company might need to issue new equity to raise additional cash. Under such a scenario, the share value of existing shareholders could be diluted.


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**Figure 1: Income Statement**

## Asterias Biotherapeutics – Income Statement

| ($'000) | 2016 | 2017 | 1Q18E | 2Q18E | 3Q18E | 4Q18E | 2018E | 2019E | 2020E | 2021E | 2022E | 2023E | 2024E | 2025E |
|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|
| **Revenue** | | | | | | | | | | | | | | |
| AST-OPC1 US sales | | | | | | | | | | | 10,466 | 30,891 | 79,246 | 174,950 |
| AST-OPC1 ex-US royalties and COGS | | | | | | | | | | | 0 | 0 | 3,338 | 14,931 |
| AST-OPC1 total revenue | | | | | | | | | | | **10,466** | **30,891** | **82,584** | **189,881** |
| AST-VAC1 in AML revenues | | | | | | | | | | | | | 37,131 | 95,994 |
| AST-VAC2 in NSCLC revenues | | | | | | | | | | | | | | 85,629 |
| **Total product revenues** | | | | | | | | | | | 10,466 | 30,891 | 119,714 | 371,505 |
| Royalties from product sales | 381 | 331 | 124 | 130 | 110 | 130 | 494 | 514 | 534 | 556 | 578 | 601 | 625 | 650 |
| Sale of cell lines | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 |
| Grant income | 6,572 | 3,711 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 |
| **Total revenue** | 6,953 | 4,042 | 124 | 130 | 110 | 130 | 494 | 514 | 534 | 556 | 11,044 | 31,492 | 120,340 | 372,155 |
| COGS of therapeutic products | | | | | | | | | | | (2,093) | (6,178) | (24,489) | (76,744) |
| Cost of sales | (127) | (166) | (56) | (59) | (50) | (59) | (222) | (231) | (240) | (250) | 260 | (5,908) | (24,208) | (76,452) |
| **Total gross profit** | 6,826 | 3,876 | 68 | 72 | 61 | 72 | 272 | 283 | 294 | 306 | 11,304 | 36,799 | 106,792 | 266,333 |
| **Expenses** | | | | | | | | | | | | | | |
| Research and development | (25,468) | (26,640) | (5,598) | (4,926) | (4,532) | (4,554) | (19,610) | (23,336) | (28,236) | (33,036) | (35,349) | (36,763) | (35,660) | (32,094) |
| General and administrative | (15,481) | (10,488) | (1,936) | (1,801) | (1,747) | (1,724) | (7,208) | (7,857) | (8,486) | (9,080) | (9,534) | (10,011) | (10,511) | (11,037) |
| Marketing and sales | | | | | | | | | | | (20,000) | (25,000) | (29,250) | (31,298) |
| **Total operating costs and expenses** | (40,949) | (37,128) | (7,534) | (6,727) | (6,279) | (6,279) | (26,818) | (31,193) | (36,722) | (42,116) | (64,883) | (71,773) | (75,421) | (74,428) |
| **Operating Incomes (losses)** | (34,123) | (33,251) | (7,466) | (6,655) | (6,218) | (6,207) | (26,547) | (30,910) | (36,428) | (41,810) | (53,578) | (34,974) | 31,371 | 191,905 |
| **Other Income/(Expense)** | | | | | | | | | | | | | | |
| Change in fair value on warrant liability | (3,107) | 5,908 | (1,000) | 300 | 340 | 100 | (260) | (1,200) | (1,200) | (1,200) | (1,200) | (1,200) | (1,200) | (1,200) |
| Interest expense, net | (548) | (474) | (110) | (120) | (117) | (125) | (472) | (1,599) | (1,598) | (1,597) | (1,629) | (1,662) | (1,695) | (1,729) |
| Other income (expense), net | (36) | (555) | 30 | 40 | 38 | 42 | 150 | 165 | 182 | 200 | 220 | 242 | 266 | 292 |
| **Total other income (expense), net** | (3,691) | 4,879 | (1,080) | 220 | 261 | 17 | (582) | (2,634) | (2,617) | (2,597) | (2,609) | (2,620) | (2,629) | (2,636) |
| Pretax income | (37,814) | (28,372) | (8,546) | (6,435) | (5,957) | (6,190) | (27,129) | (33,544) | (39,044) | (44,408) | (56,187) | (37,594) | 28,742 | 189,269 |
| Deferred income tax benefit | 2,324 | 0 | | | | | 0 | 0 | 0 | 0 | 0 | 0 | (10,634) | (70,030) |
| **Net Income (Loss)** | **(35,490)** | **(28,372)** | **(8,546)** | **(6,435)** | **(5,957)** | **(6,190)** | **(27,129)** | **(33,544)** | **(39,044)** | **(44,408)** | **(56,187)** | **(37,594)** | **18,107** | **119,239** |
| Basic and diluted net loss per share | ($0.83) | ($0.56) | ($0.16) | ($0.10) | ($0.09) | ($0.10) | ($0.44) | ($0.52) | ($0.58) | ($0.64) | ($0.75) | ($0.50) | $0.24 | $1.59 |
| Weighted average common shares outstanding: basic and undiluted | 42,943 | 50,271 | 53,954 | 63,954 | 64,354 | 64,754 | 61,754 | 64,754 | 67,754 | 69,754 | 74,754 | 74,854 | 74,954 | 75,054 |
| **Margin Analysis (% of Sales/Revenue)** | | | | | | | | | | | | | | |
| Costs of goods | -33% | -50% | -45% | -45% | -45% | -45% | -45% | -45% | -45% | -45% | -45% | -45% | -45% | -45% |
| R&D | -366% | -659% | -4514% | -3789% | -4120% | -3503% | -3970% | -4542% | -5285% | -5945% | -320% | -117% | -30% | -9% |
| SG&A | -223% | -259% | -1562% | -1385% | -1588% | -1326% | -1459% | -1529% | -1588% | -1634% | -86% | -32% | -9% | -3% |
| Operating Income (loss) | -491% | -823% | -6021% | -5119% | -5653% | -4775% | -5374% | -6016% | -6818% | -7524% | -485% | -111% | 26% | 52% |
| Pretax | -544% | -702% | -6892% | -4950% | -5416% | -4762% | -5492% | -6529% | -7307% | -7992% | -509% | -119% | 24% | 51% |
| Tax Rate | | | | | | | | | | | 37% | 37% | 37% | 37% |
| Net Income | -510% | -702% | -6892% | -4950% | -5416% | -4762% | -5492% | -6529% | -7307% | -7992% | -509% | -119% | 15% | 32% |
| **Financial Indicator Growth Analysis (YoY%)** | | | | | | | | | | | | | | |
| Total Revenue | 94% | -42% | -94% | -59% | -93% | 364% | -88% | 4% | 4% | 4% | 1888% | 185% | 282% | 209% |
| Gross Profit | 106% | -43% | -97% | -76% | -96% | 411% | -93% | 4% | 4% | 4% | 3599% | 226% | 190% | 149% |
| Cost of Goods | -53% | 31% | 5% | 225% | -39% | 318% | 34% | 4% | 4% | 4% | -204% | -2372% | 310% | 216% |
| R&D | 47% | 5% | -15% | -29% | -32% | -29% | -26% | 19% | 21% | 17% | 7% | 4% | -3% | -10% |
| SG&A | 96% | -32% | -57% | -2% | -15% | -19% | -31% | 9% | 8% | 7% | 5% | 5% | 5% | 5% |
| Operating Income (Losses) | 62% | -9% | -32% | -24% | -28% | -27% | -28% | 16% | 18% | 15% | 54% | 11% | 5% | -1% |
| Pretax Income | 70% | -25% | 36% | -26% | -13% | -5% | -4% | 24% | 16% | 14% | 27% | -33% | -176% | 559% |
| Net Income | 137% | -20% | 36% | -26% | -13% | -5% | -4% | 24% | 16% | 14% | 27% | -33% | -148% | 559% |
| EPS | 97% | -32% | 22% | -44% | -32% | -22% | -22% | 18% | 11% | 10% | 18% | -33% | -148% | 558% |

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



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# DISCLOSURES:

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***Additional information available upon request.***

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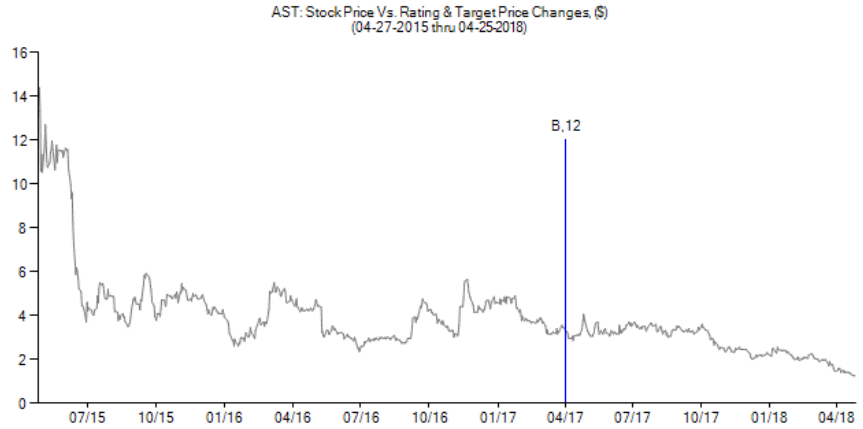


*3 Year Rating Change History*

| Date | Rating | Closing Price ($) |
|---|---|---|
| 04/03/... | Buy (B ) | 3.25 |

*3 Year Price Change History*

| Date | Target Price ($) | Closing Price, ($) |
|---|---|---|
| 04/03/... | 12.00 | 3.25 |

**Source: Laidlaw & Company** **Created by: Blue-Compass.net**

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|---|---|---|---|---|
| | | | Investment Banking | Brokerage |
| **Strong Buy (SB)** | Expected to significantly outperform the sector over 12 months. | 0.00% | 0.00% | 0.00% |
| **Buy (B)** | Expected to outperform the sector average over 12 months. | 65.38% | 26.92% | 3.85% |
| **Hold (H)** | Expected returns to be in line with the sector average over 12 months. | 0.00% | 0.00% | 0.00% |
| **Sell (S)** | Returns expected to significantly underperform the sector average over 12 months. | 0.00% | 0.00% | 0.00% |

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***NOTES:***


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