



Avalanche Biotechnologies

Solving ophthalmic gene therapy

Avalanche is well capitalised to deliver all important proof-of-concept data in 2015 with trial readout in wet AMD. By combining its gene therapy technology platform with vascular endothelial growth factor (VEGF) trap molecules, Avalanche is aiming to break into the \$6bn exudative AMD market. With \$290m in cash and a Regeneron licensing deal for orphan diseases, Avalanche is well placed to deliver on its clinical programmes.

AVA-101 clinical trials progressing

Avalanche has completed enrolment in a Phase IIa trial of its lead product, AVA-101 and is expecting data mid-year. The Phase I trial enrolled eight patients with wet AMD randomised to low dose, high dose and control. All patients in the phase I study received two initial Lucentis doses followed by rescue Lucentis if needed. Control eyes lost five letters of visual acuity in the first year. The low-dose AVA-101 group gained 8.7 letters and the high-dose group gained 6.3 letters. The AVA-101 groups needed an average of 0.33 rescue injections, compared to 3.0 for the control groups. One patient lost vision due to subretinal scarring at baseline.

Phase IIa data coming soon

Phase IIa enrolled 32 patients and top-line data are expected this summer. Interim data presented by the company in mid-2014 suggested the injection continues to be well tolerated. Pending these results, the company plans to submit an IND application to begin a Phase IIb in the US. AVA-201 for the prevention of exudative AMD is in planning stage and AVA-322/323 for colour blindness is preclinical targeting a 2016 IND filing. The next generation platform technology has a wide range of targets.

AVA-101 offers an advantage over current therapy

Competition exists for treatment of exudative AMD. There are two approved anti-VEGF injections, Lucentis and Eylea, each requiring repeated injections. AVA-101's phase I data showed that it may decrease the number of injections needed.

Valuation: EV of \$707m with strong capital position

A market cap of \$997m and \$290m in cash places Avalanche in a good position. There is upside potential this year with expected Phase IIa data and US IND submission. The Regeneron licensing agreement to develop the x-linked retinoschisis product and to identify new targets will provide near-term revenue.

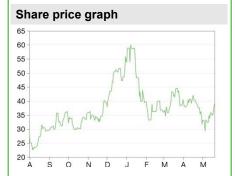
Consensus estimates						
Year end	Revenue (\$m)	PBT (\$m)	EPS (\$)	DPS (\$)	P/E (x)	Yield (%)
12/13	0.5	(5.3)	(1.44)	0.0	N/A	N/A
12/14	0.6	(25.4)	(2.46)	0.0	N/A	N/A
12/15e	3.7	(46.4)	(1.95)	0.0	N/A	N/A
12/16e	4.3	(63.1)	(2.50)	0.0	N/A	N/A

Source: Bloomberg

Pharma & biotech

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Share details	
Code	AAVL
Listing	NASDAQ
Shares in issue	25.53m

Business description

Avalanche is a clinical-stage biotechnology company focused on adeno-associated virus-based gene therapy for age-related macular degeneration. The company's Ocular BioFactory platform is capable of encoding genes to express therapeutic proteins at high yields. The company is in Phase II trials in AMD.

Bull

- Encouraging Phase I completed and Phase IIa data expected soon.
- Scalable platform with wide range of targets.
- Strong capital position with \$290m in cash.

Bear

- Competition in the exudative AMD market.
- Only one compound is currently in clinical stage.
- Current trials conducted in Australia and FDA may require additional data, delaying IND filing.

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