

## Avalanche Biotechnologies (AAVL)

Overweight

### Stocks On the Move With SNY Data Update

#### CONCLUSION

AAVL is trading up sharply while AGTC shares are down (with a down tape), coinciding with data presentation for the Sanofi/Applied Genetic Technologies AAV-2 gene therapy data presentation at the Retina Society meeting today. From sources who were at the presentation (slides not available), results were better than we expected with minimal inflammation yet some signs of efficacy. There is likely room for improvement in the vector construct for intra-vitreous delivery, which bodes well for the 2 main players in the wet AMD gene therapy AAV vector field (AAVL- OW and AGTC- NR). SNY will not be advancing this program further due to it's relationship with REGN which precludes ongoing development.

**What we've heard:** 20 patients were treated with the SNY/AGTC product including 8 in an expanded cohort using the highest. The first 4 cohorts of patients prior to the expansion were refractory to VEGF-targeting therapy while the 8 expansion patients were responsive but at an advanced stage. Only 1 high dose patient experienced inflammation (which was responsive to steroid therapy). The high dose patients had nearly 5 letter gain in visual acuity with improvement in retinal thickness on OCT imaging. 4/11 total patients at the high dose (including ones prior to the expansion) were counted as 'responders' although we aren't sure what that means. Geographic atrophy was not seen. We are hoping for conformation although slides may not be accessible until after the AAO conference in October.

**What it means:** This bodes well generally for gene therapy for wet AMD, especially as the programs move toward Intravitreal delivery. It is possible that efficacy could have been improved with higher dosing or (more likely) a novel vector, and safety was manageable in this experience. Both AAVL and AGTC have vector optimization programs to enable an intravitreal gene therapy program for wet AMD and in the meantime AAVL is moving forward with it's sub-retinal approach using the AAV2 vector.

#### COMPANY DESCRIPTION

AAVL is a pioneer in gene therapy, targeting ophthalmic indications.

PRICE: US\$32.43

TARGET: US\$39.00

DCF thru 2024, 11% discount rate, 5% growth rate

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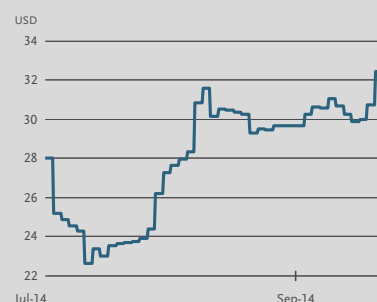
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#### RISKS TO ACHIEVEMENT OF PRICE TARGET

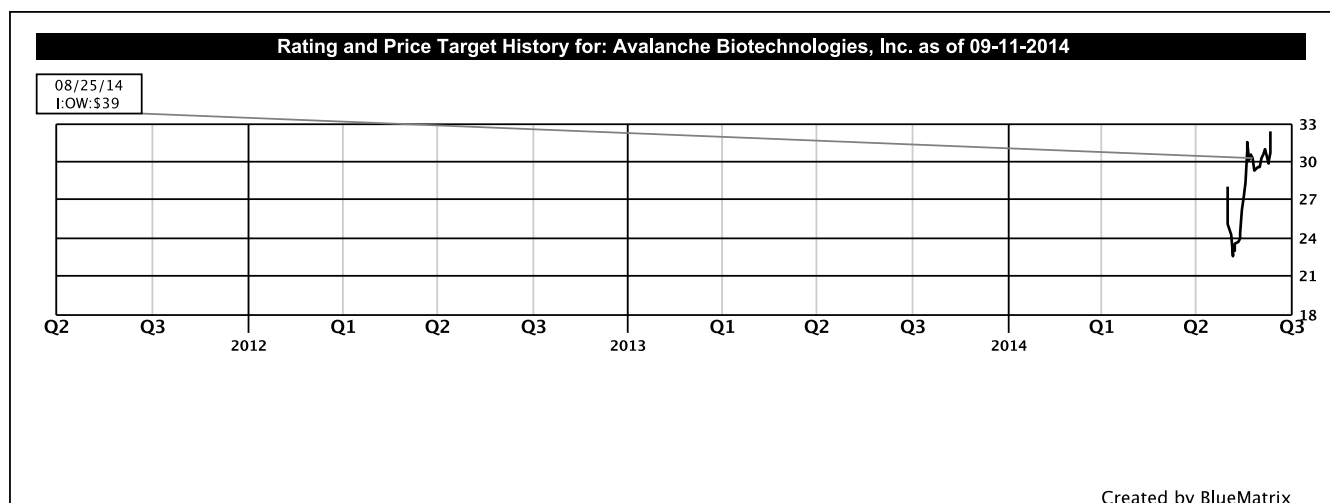
AAVL gene therapy candidates may fail to achieve target development steps.

#### Price Performance - 1 Year



Source: Bloomberg

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### Analyst Certification — Joshua E. Schimmer, MD, Sr Research Analyst — Jerry Yang, Ph.D., Research Analyst

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