

Avalanche Biotechnologies Inc

COMPANY AND PIPELINE OVERVIEW REPORT

A comprehensive coverage of the company and a summary of the drug pipeline portfolio.

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ABOUT COMPANY AND PIPELINE OVERVIEW REPORT

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GLOSSARY

Number of Drugs in Active Development

Number of drugs associated with the company or subsidiary that are currently in active development, i.e. the development status for the drug(s) is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended.

Number of Inactive Drugs

Number of drugs associated with the company or subsidiary that are currently classified as inactive, i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.

Number of Patents as Owner

Number of patents associated with the company where the company is listed as owner; i.e. the relationship type (or way the patent refers to the company) is: Patent Assignee/Owner, Patent owner (not assignee), Licensee for development and marketing, Licensee – marketing only (Distributor), Patent assignee of family member, Inferred assignee.

Number of Patents as Third Party

Number of patents associated with the company where the company is listed as third party; i.e. the relationship type (or way the patent refers to the company) is: Patent assignee (not owner), Ex-Licensee for development and marketing, Ex-Licensee marketing only (Distributor), Customer of technology, Ex-Customer of technology, Patent opponent or infringer, Affiliate organization of inventor, Owner of underlying technology.

Patents summary table

This table represents a summary of the core patent coverage for this company covering Therapeutic EP, US and WO patents since 1990 only.

Number of Deals

A count of deals where the company or one of its subsidiaries is the primary company.

Key Indications

Displays top ten key indications for the company and its subsidiaries based on frequency (indications occurring with high and identical frequency are always included, and this may result in more than ten Key Indications being listed). Includes both indications associated with patents where the company is patent owner and indications associated with drugs in active development. A drug is classified as 'active' if it features on a row (or rows) in the current development status table where the status is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended.

Key Target-based Actions

Displays top ten key target-based actions for the company and its subsidiaries based on frequency (actions occurring with high and identical frequency are always included, and this may result in more than ten Key Target-based Actions being listed). Includes both target-based actions associated with patents where the company patent owner and target-based actions associated with drugs in active development. A drug is classified as 'active' if it features on a row (or rows) in the current development status table where the status is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended. A target-based action is one that is associated with a target.

Key Technologies

Displays top ten key technologies for the company and its subsidiaries based on frequency (technologies occurring with high and identical frequency are always included, and this may result in more than ten Key Technologies being listed). Includes both key technologies associated with patents where the company relationship is patent owner and key technologies associated with drugs in active development. A drug is classified as 'active' if it features on a row (or rows) in the current development status table where the status is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended.

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PLEASE NOTE: the financials section where present in the report includes: Share Information, Stock Performance chart (including the consensus recommendation), and Major Shareholders. Financial Performance presents graphical and tabular data on Worldwide Sales, Operating Income and Net Income over time, together with a Quarterly earnings update. Balance Sheet lists Assets, Liabilities and Stockholders Equity, and Forecasts includes: EPS Forecast and Other Forecasts. The data reported in all sections (except share information and major stock holders) are correct as of the publication date of the report (and not the download date). For share information and major stock holders the data is correct for the date shown with these sections



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Avalanche Biotechnologies Inc

COMPANY OVERVIEW

Company Name	Avalanche Biotechnologies Inc
Parent Company Name	Avalanche Biotechnologies Inc
Website	http://www.avalanchebiotech.com/
Country	US
Number of Drugs in Active Development	5
Number of Inactive Drugs	0
Number of Patents as Owner	2
Number of Patents as Third Party	0
Number of Deals	6
Key Indications	Wet age related macular degeneration, Diabetic macular edema, Glaucoma, Retinal venous occlusion, Retinoschisis, Unidentified indication, Diabetic retinopathy, Age related macular degeneration, Dry age related macular degeneration, Macular edema, Ocular neovascular disorder, Retinitis pigmentosa, Stargardt disease, Usher syndrome
Key Target-based Actions	RS1 gene modulator,VEGF-1 receptor antagonist,Angiostatin gene modulator,CNTF gene modulator,FLT1 gene modulator,GDNF gene modulator,RPE65 gene modulator,TIMP3 gene modulator,VEGF receptor
Key Technologies	Virus recombinant, Biological therapeutic, Parenteral formulation unspecified, Injectable formulation, Ophthalmic formulation, Small molecule therapeutic, Gene transfer system viral, Gene expression regulation, Monoclonal

COMPANY PROFILE

SUMMARY

Avalanche Biotechnologies is developing technologies and products for the sustained delivery of therapeutic proteins in patients with ocular diseases, with a focus on wet AMD.

COMPANY LOCATION

In September 2011, the company opened new office and research facilities in San Francisco, CA.

FINANCIAL

In January 2015, the company filed a registration statement on Form S-1/A with the Securities and Exchange Commission relating to a proposed public offering of 2,000,000 common stock shares. The offering was expected to consist of 1,762,500 shares by Avalanche and 237,500 shares to be offered by existing stockholders. At that time, the underwriters were granted a 30-day option to purchase up to an additional 300,000 shares from the selling shareholders and Avalanche, and agreed for lock-up agreements for a period of 90 days following the follow-on offering; Later that month, the company priced the public offering of 2,399,457 shares of \$141.6 million of common stock, each at a price of \$59.00 per share. At that time, the underwriters were granted a 30-day option to purchase up to an additional 359,918 shares of common stock. The follow-on offering was expected to close on January 13, 2015, which was subjected to customary closing conditions.

By September 2014, the company had completed a \$10 million private placement of common stock to Regeneron Pharmaceuticals Inc.

In July 2014, the company priced its initial public offering of 6 million shares of its common stock, at \$17 each and planned to raise \$102 million. The underwriters were granted a 30-day option to purchase up to 0.9 million additional shares of common stock. The offering was expected to close on August 05, 2014. At that time, the company's common stock began trading on the NASDAQ Global Market under the ticker symbol "AAVL". By September 2014, the company had raised net proceeds of approximately \$106.5 million from the placement of 6.9 million shares of its common stock, which included the full exercise of the underwriters' over-allotment option.



In April 2014, the company raised \$55 million in a series B financing round.

In September 2012, the company completed a series A financing. In November 2013, Avalanche raised additional financing in a follow-on round consisting of both new and existing investors. The financing included both preferred stock and convertible debt.

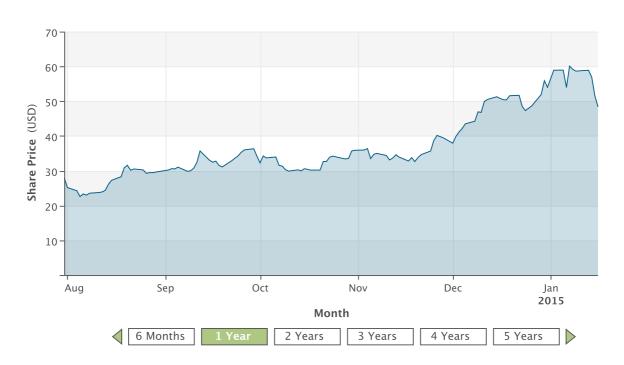
COMPANY FINANCIALS

SHARE INFORMATION

Close Price (USD)	48.37
Change (USD)	-3.19 (-6.19%)
Previous Close Price (USD)	51.56
Volume	696,277
52-Week High (USD)	62.48
52-Week Low (USD)	22.00
Date	16-Jan-2015
Exchange	NASDAQ(COMPOSITE)
Current Number of Shares Outstanding	22,470,299
Current Market Capitalization	1,086,888,363
EPS (Date Reported)	-0.48061



Stock Performance



MAJOR SHAREHOLDERS

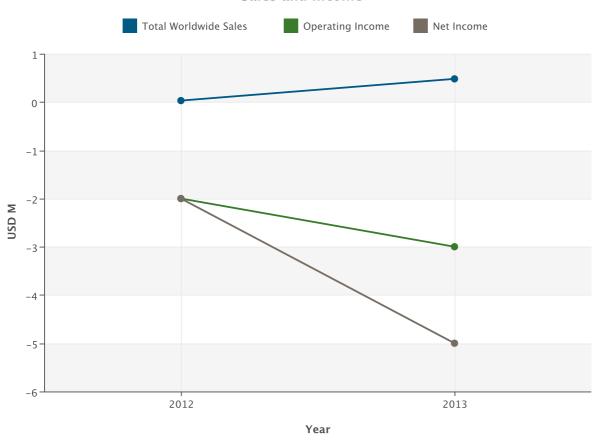
Shareholder	Туре	Country	Shares held	% of shares held	Change in holding	% changes in shares held	Date
Venrock	Venture Capital	United States	2,365,471	9.66	0	0	02-Jan-2015
Zygtech, L.L.C.	Corporati on	United States	2,463,176	10.06	-100,000	-0.04	13-Jan-2015
Regeneron Pharmaceuticals Inc	Corporati on	United States	1,809,098	7.39	0	0	02-Jan-2015
Deerfield Management Company, L.P.	Hedge Fund	United States	1,614,669	6.6	-433,010	-0.21	02-Jan-2015
Chalberg (Thomas W)	Individua I Investor	United States	1,126,401	4.6	-85,000	-0.07	13-Jan-2015
Schwartz (Steven Daniel)	Individua I Investor	United States	897,681	3.67	-75,375	-0.08	13-Jan-2015
Blumenkranz (Mark S)	Individua I Investor	United States	683,582	2.79	-432,500	-0.39	13-Jan-2015
Hull (Hans)	Individua I Investor	United States	81,237	0.33	2,292	0.03	02-Jan-2015
Wachter (Paul D)	Individua I Investor	United States	63,081	0.26	-17,188	-0.21	02-Jan-2015



Gasmi Mehdi	Individua I Investor	United States	36,667	0.15	2,292	0.07	02-Jan-2015
McLaughlin (John P)	Individua I Investor	United States	26,560	0.11	-17,188	-0.39	02-Jan-2015

FINANCIAL PERFORMANCE

Sales and Income



	2013	2012		
Sales (USD M)	0.48	0.03		
Operating Income (USD M)	-2.654	-1.816		
Net Income (USD M)	-5.276	-1.811		



Quarterly earnings update

Period	Sales (USD M)	Operating Income (USD M)	Net Income (USD M)
2014 Q3	0.204	-7.94	-8.256
2014 Q2	0.135	-4.453	-8.324
2014 Q1	0.03	-1.606	-1.663
2013 Q4	0	-1.129	-3.693
2013 Q3	0.03	-0.778	-0.813
2013 Q2	0.15	-0.705	-0.721
2013 Q1	0.3	-0.042	-0.049

BALANCE SHEET

Assets (USD M)

	2012	2013		
Cash & Short Term Investments	0.357	0.564		
Inventories - Total	0	0		
Current Assets - Total	0.382	0.822		
Property, Plant And Equipment - Net	0.004	0.069		
Other Assets - Total	0	0.194		
Total Assets	0.386	1.085		

Liabilities (USD M)

	2012	2013
Accounts Payable	0.522	0.769
Short Term Debt & Current Portion Of Long Term Debt	0	0
Current Liabilities - Total	0.839	1.162
Long Term Debt	0.521	0.091
Deferred Taxes	0	0
Other Liabilities	0.023	0.042
Total Liabilities	1.383	1.303



Stockholders Equity (USD M)

	2012	2013		
Minority Interest	0	0		
Preferred Stock	2.595	7.992		
Common Stock	0	0		
Total Liabilities & Shareholders' Equity	0.386	1.085		

FORECASTS

EPS Forecast

Year ending	Current Forecast	1 month ago	3 months ago
31-Dec-2015	-1.37	-1.37	-1.02
31-Dec-2016	-1.57	-1.57	-1.24
31-Dec-2017	-1.98	-1.98	-1.71

Other Forecasts

Year ending	DPS	CPS	Pretax	Sales
31-Dec-2015			-32.74	3.53
31-Dec-2016			-41	3.03
31-Dec-2017			-56.44	3

SUBSIDIARY COMPANIES

Names

Avalanche Australia Pty Ltd

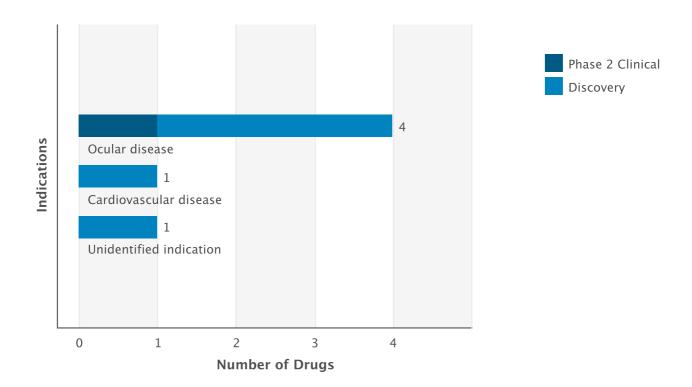


PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



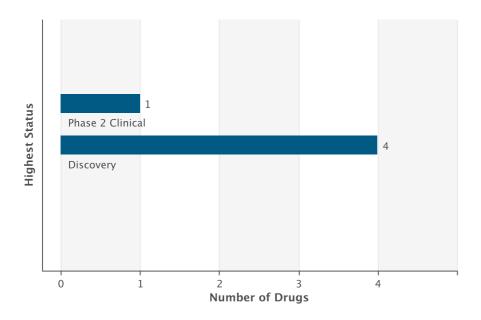
Drugs by Indication Table

Indication	Active	Inactive	Total
Ocular disease	4	0	4
Unidentified indication	1	0	1
Cardiovascular disease	1	0	1

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Drugs by Highest Status

Active Drugs by Highest Status Chart



Drugs by Highest Status Table

Development Status	Number of Drugs
Phase 2 Clinical	1
Discovery	4

DEALS

Deal Type	Prin	cipal	Par	tner	Total
	Active	Inactive	Active	Inactive	
Patent - Exclusive Rights	0	0	1	0	1
Drug - Funding	2	0	0	0	2
Drug - Development/Commercialization License	1	0	1	0	2
Technology - Delivery/Formulation	1	0	0	0	1

CLINICAL TRIALS

Trials by Condition Studied

Condition Studied	Ongoing	All
Ocular disease	0	1



Trials by Phase

Phase	Ongoing	All
Phase 1	0	1

Phase Definitions

Phase 3 Clinical

Includes Phase 3, Phase 3b, Phase 3a, Phase 2/3 (where enrolment count is 300 or over)

Phase 2 Clinical

Includes Phase 2, Phase 2a, Phase 2b, Phase 1/2 (where enrolment count is 100 or over), Phase 2/3 (where enrolment count is under 300 or not specified)

Phase 1 Clinical

Includes Phase 1, Phase 1, Phase 1, Phase 1/2 (where enrolment count is under 100 or not specified), Phase 0

PATENTS *

Indication	As Owner	As Third Party	Total
Cardiovascular disease	2	0	2
Ocular disease	2	0	2
Otorhinolaryngological disease	1	0	1

^{*} This table represents a summary of the core patent coverage for this company covering Therapeutic EP, US and WO patents since 1990 only.

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PRODUCT PORTFOLIO DRUGS

PLEASE NOTE: Highest status refers to highest development of that drug for one of the active companies

VEGF-1 (Flt-1) receptor antagonist (adenovirus vector, wet AMD), Avalanche Biotechnologies/Lions Eye Institue/ University of Western Australia

Drug Name	VEGF-1 (Flt-1) receptor antagonist (adenovirus vector, wet AMD), Avalanche Biotechnologies/Lions Eye Institue/ University of Western Australia
Key Synonyms	
Originator Company	University of Western Australia
Active Companies	Avalanche Biotechnologies Inc, Lions Eye Institute of Western Australia Inc, University of Western Australia
Inactive Companies	Merck Sharp & Dohme Corp
Highest Status	Phase 2 Clinical
Active Indications	Diabetic macular edema, Wet age related macular degeneration, Retinal venous occlusion
Target-based Actions	VEGF-1 receptor antagonist
Other Actions	Ocular antineovascularisation agent, Ophthalmological agent, Adeno- associated virus based gene therapy
Technologies	Sustained release formulation, Injectable formulation, Virus recombinant, Ophthalmic formulation, Biological therapeutic, Parenteral formulation unspecified
Last Change Date	04-Aug-2014

Shh10.GDNF

Drug Name	Shh10.GDNF
Key Synonyms	
Originator Company	Avalanche Biotechnologies Inc
Active Companies	Avalanche Biotechnologies Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Glaucoma
Target-based Actions	
Other Actions	Neuroprotectant, Ophthalmological agent, Antiglaucoma agent
Technologies	Virus recombinant, Biological therapeutic, Parenteral formulation unspecified
Last Change Date	04-Aug-2014



AVA-201

	11/4 004
Drug Name	AVA-201
Key Synonyms	
Originator Company	Avalanche Biotechnologies Inc
Active Companies	Avalanche Biotechnologies Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Wet age related macular degeneration
Target-based Actions	VEGF-1 receptor antagonist
Other Actions	Adeno-associated virus based gene therapy, Ocular antineovascularisation agent, Ophthalmological agent
Technologies	Injectable formulation, Virus recombinant, Ophthalmic formulation, Biological therapeutic, Parenteral formulation unspecified
Last Change Date	08-Jul-2014

AVA-311

Drug Name AVA-311 Key Synonyms Avalanche Biotechnologies Inc Active Companies Avalanche Biotechnologies Inc, Regeneron Pharmaceuticals Inc Inactive Companies Inactive Companies Highest Status Discovery Active Indications Retinoschisis Target-based Actions RS1 gene modulator Other Actions Ophthalmological agent, Adeno-associated virus based gene therapy Technologies Injectable formulation, Virus recombinant, Ophthalmic formulation, Biological therapeutic, Parenteral formulation unspecified Last Change Date 08-Jul-2014		
Originator Company Avalanche Biotechnologies Inc Active Companies Avalanche Biotechnologies Inc, Regeneron Pharmaceuticals Inc Inactive Companies Highest Status Discovery Active Indications Retinoschisis Target-based Actions RS1 gene modulator Other Actions Ophthalmological agent, Adeno-associated virus based gene therapy Injectable formulation, Virus recombinant, Ophthalmic formulation, Biological therapeutic, Parenteral formulation unspecified	Drug Name	AVA-311
Active Companies Avalanche Biotechnologies Inc, Regeneron Pharmaceuticals Inc Inactive Companies Highest Status Discovery Active Indications Retinoschisis Target-based Actions RS1 gene modulator Other Actions Ophthalmological agent, Adeno-associated virus based gene therapy Injectable formulation, Virus recombinant, Ophthalmic formulation, Biological therapeutic, Parenteral formulation unspecified	Key Synonyms	
Inactive Companies Highest Status Discovery Active Indications Retinoschisis Target-based Actions RS1 gene modulator Other Actions Ophthalmological agent, Adeno-associated virus based gene therapy Injectable formulation, Virus recombinant, Ophthalmic formulation, Biological therapeutic, Parenteral formulation unspecified	Originator Company	Avalanche Biotechnologies Inc
Highest Status Discovery Active Indications Retinoschisis Target-based Actions RS1 gene modulator Other Actions Ophthalmological agent, Adeno-associated virus based gene therapy Injectable formulation, Virus recombinant, Ophthalmic formulation, Biological therapeutic, Parenteral formulation unspecified	Active Companies	Avalanche Biotechnologies Inc, Regeneron Pharmaceuticals Inc
Active Indications Retinoschisis RS1 gene modulator Other Actions Ophthalmological agent, Adeno-associated virus based gene therapy Injectable formulation, Virus recombinant, Ophthalmic formulation, Biological therapeutic, Parenteral formulation unspecified	Inactive Companies	
Target-based Actions RS1 gene modulator Other Actions Ophthalmological agent, Adeno-associated virus based gene therapy Injectable formulation, Virus recombinant, Ophthalmic formulation, Biological therapeutic, Parenteral formulation unspecified	Highest Status	Discovery
Other Actions Ophthalmological agent, Adeno-associated virus based gene therapy Injectable formulation, Virus recombinant, Ophthalmic formulation, Biological therapeutic, Parenteral formulation unspecified	Active Indications	Retinoschisis
Technologies Injectable formulation, Virus recombinant, Ophthalmic formulation, Biological therapeutic, Parenteral formulation unspecified	Target-based Actions	RS1 gene modulator
therapeutic, Parenteral formulation unspecified	Other Actions	Ophthalmological agent, Adeno-associated virus based gene therapy
Last Change Date 08-Jul-2014	Technologies	
	Last Change Date	08-Jul-2014

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AVA-322/AVA-323 (undisclosed indication), Avalanche

Drug Name	AVA-322/AVA-323 (undisclosed indication), Avalanche
Key Synonyms	
Originator Company	Avalanche Biotechnologies Inc
Active Companies	Avalanche Biotechnologies Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Unidentified indication
Target-based Actions	
Other Actions	Unspecified drug target
Technologies	Small molecule therapeutic
Last Change Date	09-Jul-2014



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