

## **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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## 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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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

<b>Company Name</b>	Avalanche Biotechnologies Inc
<b>Parent Company Name</b>	Avalanche Biotechnologies Inc
<b>Website</b>	<a href="http://www.avalanchebiotech.com/">http://www.avalanchebiotech.com/</a>
<b>Country</b>	US
<b>Number of Drugs in Active Development</b>	6
<b>Number of Inactive Drugs</b>	0
<b>Number of Patents as Owner</b>	3
<b>Number of Patents as Third Party</b>	0
<b>Number of Deals</b>	7
<b>Key Indications</b>	Wet age related macular degeneration, Color vision defect, Diabetic macular edema, Glaucoma, Retinal venous occlusion, Retinoschisis, Unidentified indication, Diabetic retinopathy, Retinitis pigmentosa, Stargardt disease
<b>Key Target-based Actions</b>	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
<b>Key Technologies</b>	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.

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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)	35.36
Change (USD)	-2.21 (-5.88%)
Previous Close Price (USD)	37.57
Volume	510,565
52-Week High (USD)	62.48
52-Week Low (USD)	22.00
Date	27-Apr-2015
Exchange	NASDAQ(COMPOSITE)
Current Number of Shares Outstanding	25,221,495
Current Market Capitalization	947,571,567
EPS (Date Reported)	-2.45764

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## STOCK PERFORMANCE



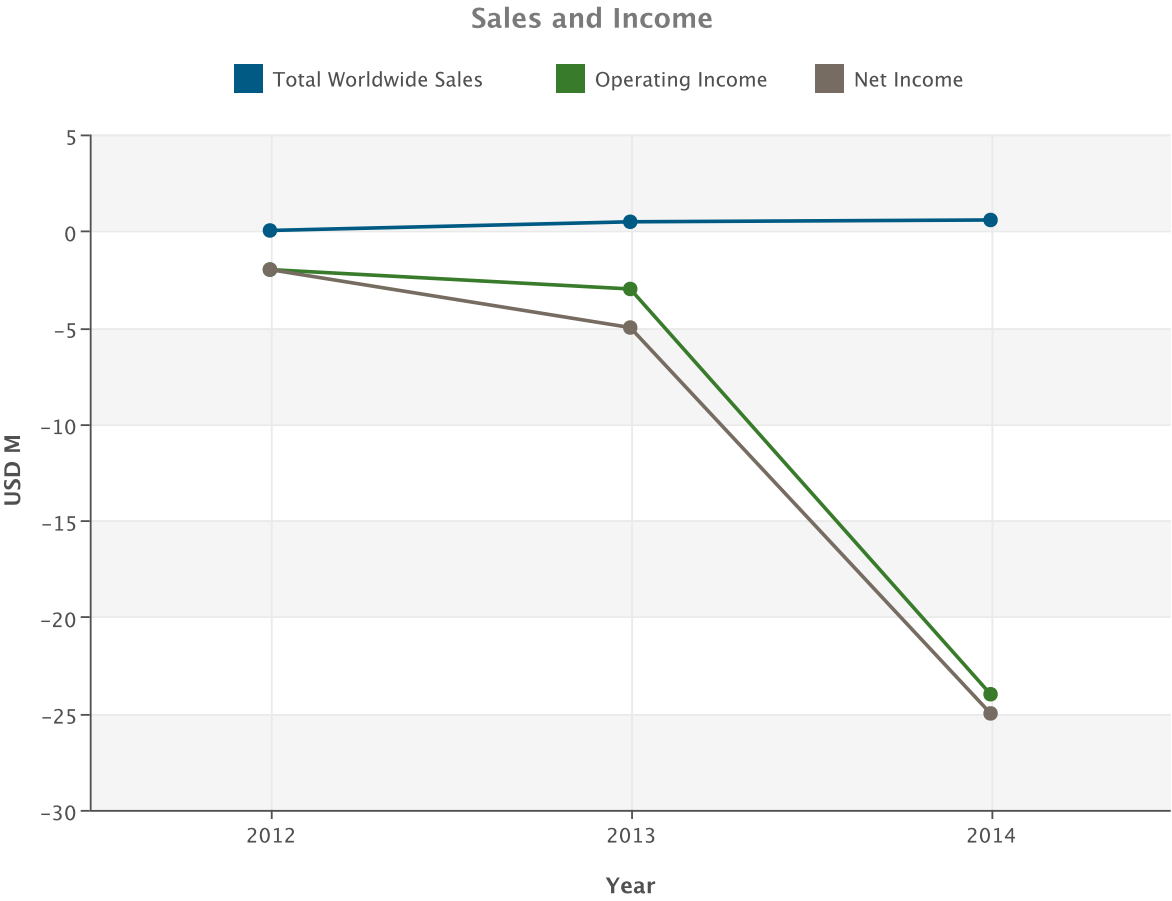
## MAJOR SHAREHOLDERS

Shareholder	Type	Country	Shares held	% of shares held	Change in holding	% changes in shares held	Date
Zygttech, L.L.C.	Corporati on	United States	2,438,176	9.67	-25,000	-0.01	14-Apr-2015
Chalberg (Thomas W)	Individua l Investor	United States	1,149,401	4.56	23,000	0.02	21-Apr-2015
Schwartz (Steven Daniel)	Individua l Investor	United States	865,556	3.43	-32,125	-0.04	23-Apr-2015
Blumenkranz (Mark S)	Individua l Investor	United States	635,582	2.52	-48,000	-0.07	21-Apr-2015
Hull (Hans)	Individua l Investor	United States	89,237	0.35	8,000	0.1	21-Apr-2015
Wachter (Paul D)	Individua l Investor	United States	65,081	0.26	2,000	0.03	21-Apr-2015
Gasmi Mehdi	Individua l Investor	United States	43,667	0.17	7,000	0.19	21-Apr-2015
McLaughlin (John P)	Individua l Investor	United States	28,560	0.11	2,000	0.08	21-Apr-2015
Rubio (Roman G)	Individua l Investor	United States	7,000	0.03	7,000	1	21-Apr-2015

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FINANCIAL PERFORMANCE



	2012	2013	2014		
Sales (USD M)	0.03	0.48	0.572		
Operating Income (USD M)	-1.816	-2.654	-24.402		
Net Income (USD M)	-1.811	-5.276	-25.404		

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## Quarterly earnings update

Period	Sales (USD M)	Operating Income (USD M)	Net Income (USD M)
2014 Q4	0.203	-10.403	-10.391
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	2014		
Cash & Short Term Investments	0.357	0.564	159.404		
Inventories - Total	0	0	0		
Current Assets - Total	0.382	0.822	160.278		
Property, Plant And Equipment - Net	0.004	0.069	1.012		
Other Assets - Total	0	0.194	0.616		
Total Assets	0.386	1.085	161.906		

### Liabilities (USD M)

	2012	2013	2014		
Accounts Payable	0.522	0.769	0.951		
Short Term Debt & Current Portion Of Long Term Debt	0	0	0		
Current Liabilities - Total	0.839	1.162	5.471		
Long Term Debt	0.521	0.091	0		
Deferred Taxes	0	0			
Other Liabilities	0.023	0.042	0		
Total Liabilities	1.383	1.303	12.423		

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## Stockholders Equity (USD M)

	2012	2013	2014		
Minority Interest	0	0	0		
Preferred Stock	2.595	7.992	0		
Common Stock	0	0	0.002		
Total Liabilities & Shareholders' Equity	0.386	1.085	161.906		

## FORECASTS

### EPS Forecast

Year ending	Current Forecast	1 month ago	3 months ago
31-Dec-2015	-1.89	-1.82	-1.37
31-Dec-2016	-2.15	-2.06	-1.57
31-Dec-2017	-2.52	-2.48	-1.98

### Other Forecasts

Year ending	DPS	CPS	Pretax	Sales
31-Dec-2015			-47.49	2.88
31-Dec-2016			-60.01	2.56
31-Dec-2017			-75.13	2.56

## SUBSIDIARY COMPANIES

Names
Avalanche Australia Pty Ltd

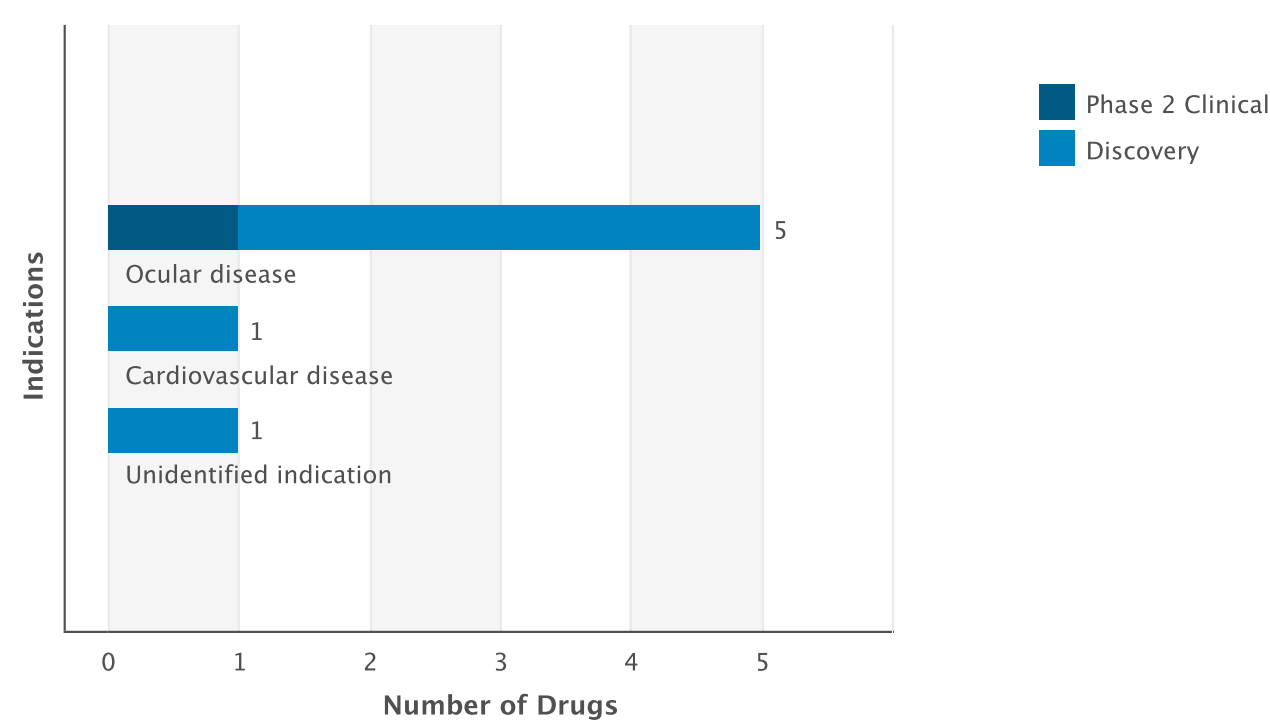
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PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



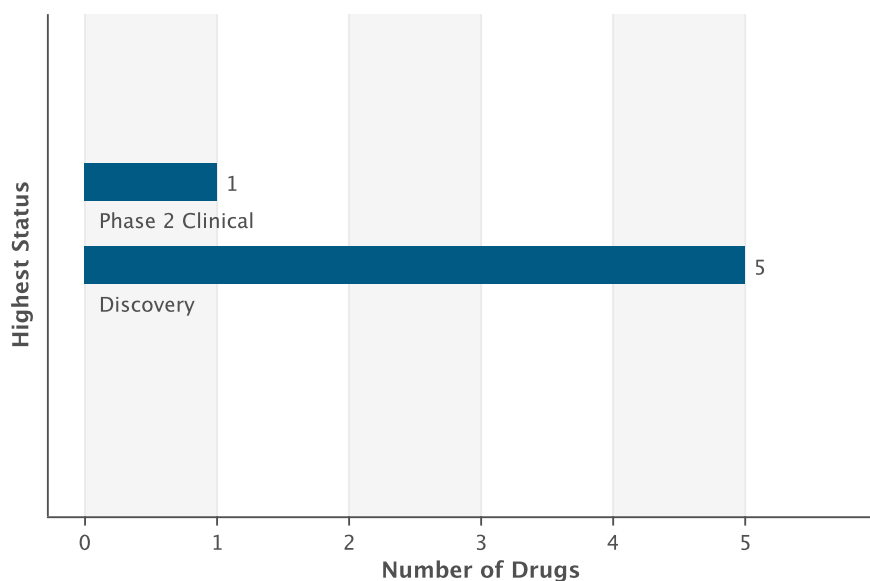
Drugs by Indication Table

Indication	Active	Inactive	Total
Ocular disease	5	0	5
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	5

## DEALS

Deal Type	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Patent - Exclusive Rights	0	0	1	0	1
Drug - Funding	2	0	0	0	2
Drug - Early Research/Development	0	0	1	0	1
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
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## 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 1a, 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	3	0	3
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 Institute/ University of Western Australia

<b>Drug Name</b>	VEGF-1 (Flt-1) receptor antagonist (adenovirus vector, wet AMD), Avalanche Biotechnologies/Lions Eye Institute/ University of Western Australia
<b>Key Synonyms</b>	
<b>Originator Company</b>	University of Western Australia
<b>Active Companies</b>	Avalanche Biotechnologies Inc, Lions Eye Institute of Western Australia Inc, University of Western Australia
<b>Inactive Companies</b>	Merck Sharp & Dohme Corp
<b>Highest Status</b>	Phase 2 Clinical
<b>Active Indications</b>	Diabetic macular edema, Wet age related macular degeneration, Retinal venous occlusion
<b>Target-based Actions</b>	VEGF-1 receptor antagonist
<b>Other Actions</b>	Ocular antineovascularisation agent, Adeno-associated virus based gene therapy, Ophthalmological agent
<b>Technologies</b>	Sustained release formulation, Injectable formulation, Virus recombinant, Ophthalmic formulation, Biological therapeutic, Parenteral formulation unspecified
<b>Last Change Date</b>	06-Mar-2015

### Shh10.GDNF

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

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## AVA-201

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

## AVA-311

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

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

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

**adeno-associated virus (AVV) vector based gene therapy (color vision deficiency), University of Washington/ Avalanche Biotechnologies**

<b>Drug Name</b>	adeno-associated virus (AVV) vector based gene therapy (color vision deficiency), University of Washington/ Avalanche Biotechnologies
<b>Key Synonyms</b>	
<b>Originator Company</b>	University of Washington
<b>Active Companies</b>	Avalanche Biotechnologies Inc, University of Washington
<b>Inactive Companies</b>	
<b>Highest Status</b>	Discovery
<b>Active Indications</b>	Color vision defect
<b>Target-based Actions</b>	
<b>Other Actions</b>	Adeno-associated virus based gene therapy, Unspecified drug target
<b>Technologies</b>	Biological therapeutic
<b>Last Change Date</b>	26-Mar-2015

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