

## **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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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	<a href="http://www.avalanchebiotech.com/">http://www.avalanchebiotech.com/</a>
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

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.

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SUBSIDIARY COMPANIES

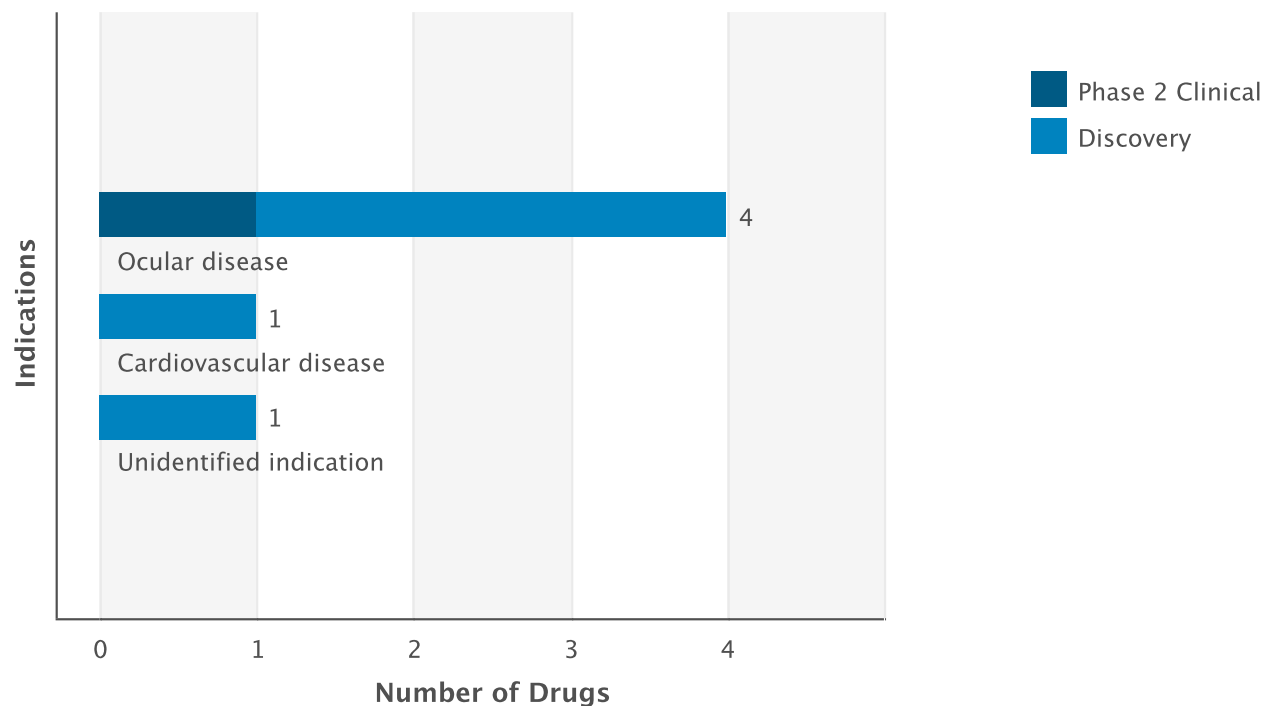
Names
Avalanche Australia Pty Ltd

PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart

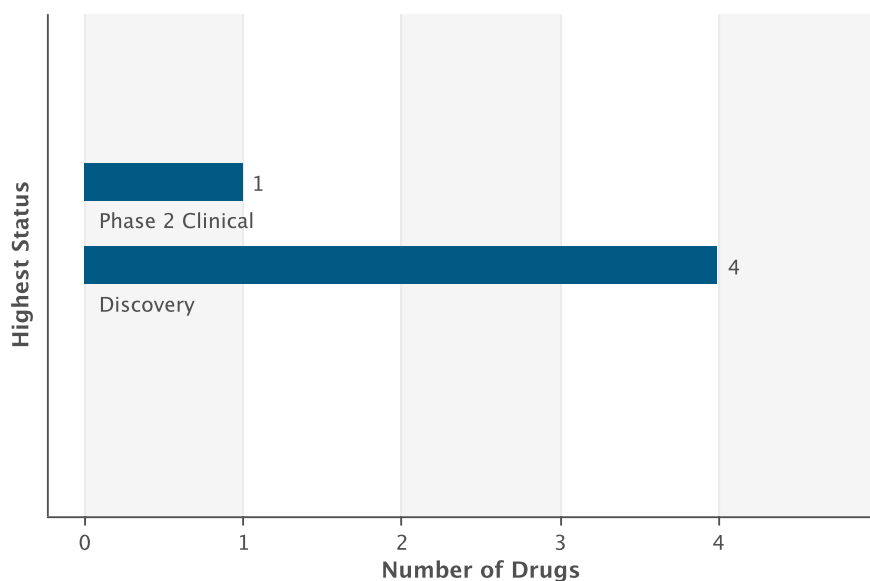


Drugs by Indication Table

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

## 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	Principal		Partner		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	1	1

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## Trials by Phase

Phase	Ongoing	All
Phase 1	1	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	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 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>	Ophthalmological agent, Ocular antineovascularisation agent, Adeno-associated virus based gene therapy
<b>Technologies</b>	Sustained release formulation, Injectable formulation, Virus recombinant, Ophthalmic formulation, Biological therapeutic, Parenteral formulation unspecified
<b>Last Change Date</b>	04-Aug-2014

### 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>	Antiglaucoma agent, Ophthalmological 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, Adeno-associated virus based gene therapy, Ophthalmological agent
<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

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