

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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[Return to Table of Contents](#)



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[Return to Table of Contents](#)



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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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

[Return to Table of Contents](#)



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

[Return to Table of Contents](#)



TABLE OF CONTENTS

Company Overview..... 7

Company Profile..... 7

Company Financials..... 8

Subsidiary Companies..... 12

Product Portfolio Summary..... 12

Product Portfolio Drugs..... 15

[Return to Table of Contents](#)

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 | 6 |
| Number of Inactive Drugs | 0 |
| Number of Patents as Owner | 3 |
| Number of Patents as Third Party | 0 |
| Number of Deals | 7 |
| Key Indications | Wet age related macular degeneration, Color vision defect, Diabetic macular edema, Glaucoma, Retinal venous occlusion, Retinoschisis, Unidentified indication, Diabetic retinopathy, Retinitis pigmentosa, Stargardt disease |
| 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.

[Return to Table of Contents](#)



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) | 33.17 |
| Change (USD) | 1.31 (4.11%) |
| Previous Close Price (USD) | 31.86 |
| Volume | 267,282 |
| 52-Week High (USD) | 62.48 |
| 52-Week Low (USD) | 22.00 |
| Date | 01-May-2015 |
| Exchange | NASDAQ(COMPOSITE) |
| Current Number of Shares Outstanding | 25,221,495 |
| Current Market Capitalization | 947,571,567 |
| EPS (Date Reported) | -2.45764 |

[Return to Table of Contents](#)

STOCK PERFORMANCE

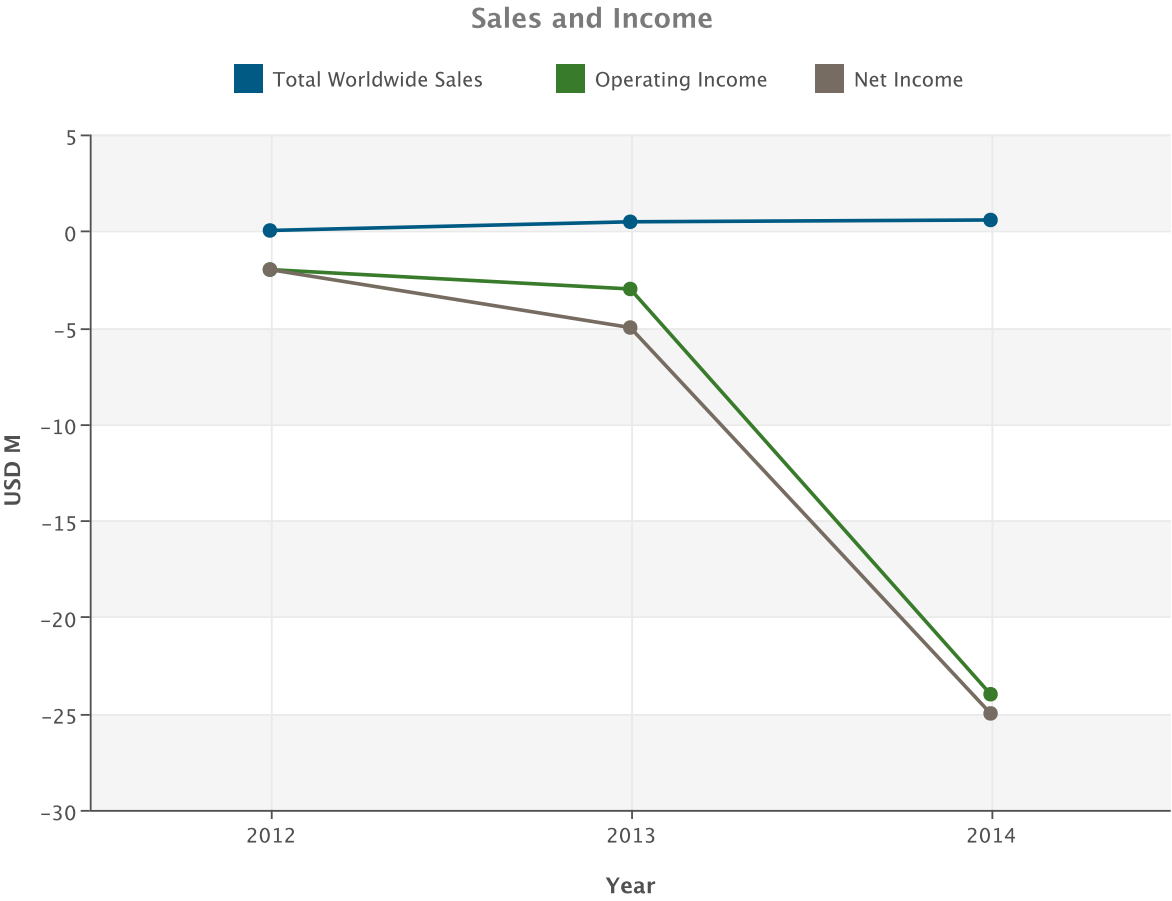


MAJOR SHAREHOLDERS

| Shareholder | Type | Country | Shares held | % of shares held | Change in holding | % changes in shares held | Date |
|--------------------------|----------------------|---------------|-------------|------------------|-------------------|--------------------------|-------------|
| Zygtech, L.L.C. | Corporati on | United States | 2,438,176 | 9.55 | -25,000 | -0.01 | 14-Apr-2015 |
| Chalberg (Thomas W) | Individua l Investor | United States | 1,149,401 | 4.5 | 23,000 | 0.02 | 21-Apr-2015 |
| Schwartz (Steven Daniel) | Individua l Investor | United States | 865,556 | 3.39 | -32,125 | -0.04 | 23-Apr-2015 |
| Blumenkranz (Mark S) | Individua l Investor | United States | 635,582 | 2.49 | -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 | 0.25 | 2,000 | 0.03 | 21-Apr-2015 |
| Gasmi Mehdi | Individua l Investor | United States | 43,667 | 0.17 | 7,000 | 0.19 | 28-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 |

[Return to Table of Contents](#)

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 | | |

[Return to Table of Contents](#)

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 | | |

[Return to Table of Contents](#)



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 |

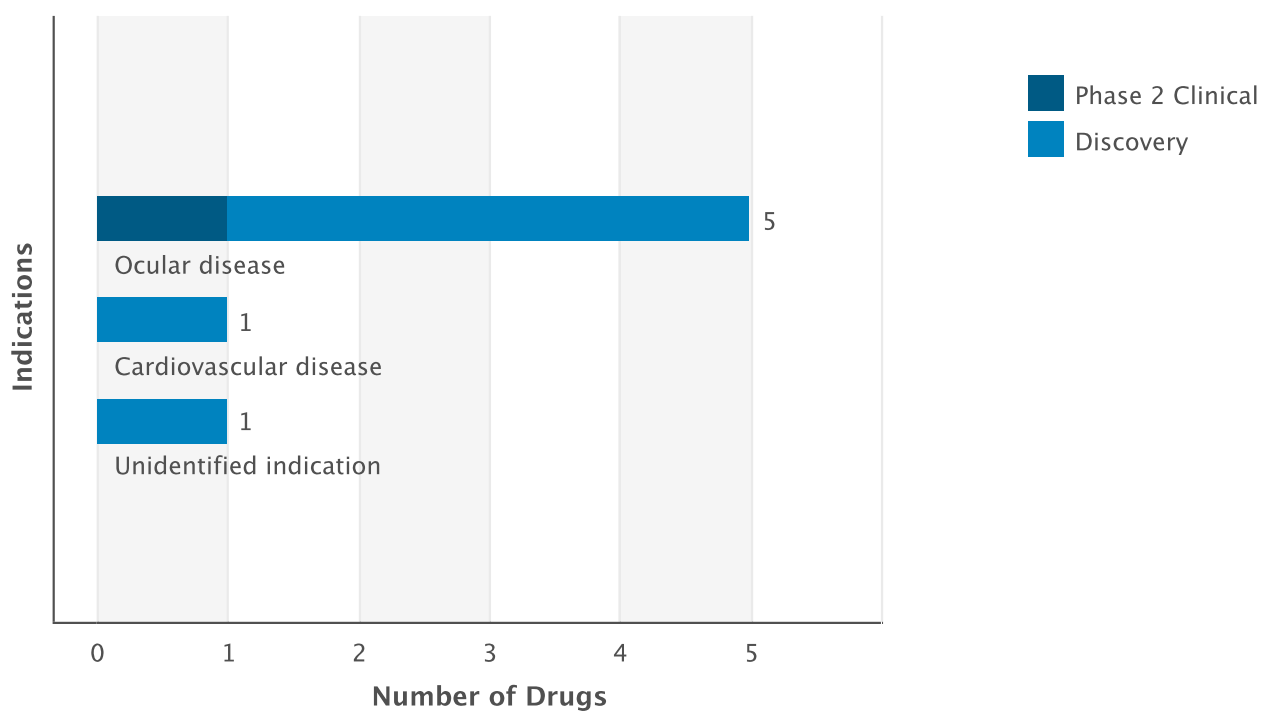
[Return to Table of Contents](#)

PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



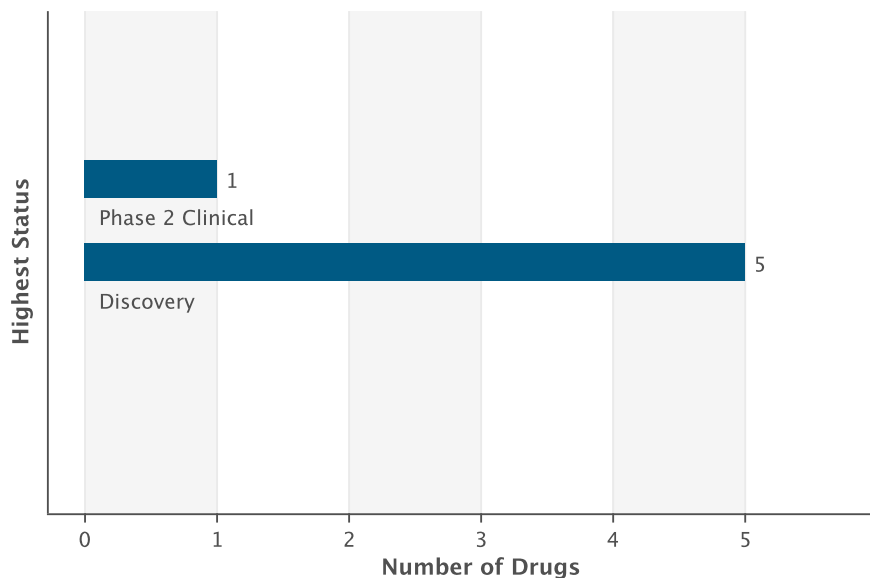
Drugs by Indication Table

| Indication | Active | Inactive | Total |
|-------------------------|--------|----------|-------|
| Ocular disease | 5 | 0 | 5 |
| Unidentified indication | 1 | 0 | 1 |
| Cardiovascular disease | 1 | 0 | 1 |

[Return to Table of Contents](#)

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 |
|-------------------|---------|-----|
|-------------------|---------|-----|

[Return to Table of Contents](#)

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.

[Return to Table of Contents](#)



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

| | |
|-----------------------------|--|
| Drug Name | VEGF-1 (Flt-1) receptor antagonist (adenovirus vector, wet AMD), Avalanche Biotechnologies/Lions Eye Institute/ 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 | 06-Mar-2015 |

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 |

[Return to Table of Contents](#)



AVA-201

| | |
|-----------------------------|---|
| 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 | |
| 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 |
| Technologies | Injectable formulation, Virus recombinant, Ophthalmic formulation, Biological therapeutic, Parenteral formulation unspecified |
| Last Change Date | 08-Jul-2014 |

[Return to Table of Contents](#)



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 |

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

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

[Return to Table of Contents](#)

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[Return to Table of Contents](#)