

## **Avalanche Biotechnologies Inc**

### **CORTELLIS COMPANY DETAILED PIPELINE REPORT**

A comprehensive coverage of the the company's drug pipeline portfolio including detailed product records.

Publication Date: 05-Aug-2014

#### **THOMSON REUTERS**

3 Times Square  
New York, New York 10036  
United States

Tel: +1 646 223 4000

[thomsonreuters.com](http://thomsonreuters.com)

[Return to Table of Contents](#)

## ABOUT CORTELLIS COMPANY DETAILED PIPELINE REPORT

Thomson Reuters provides the knowledge, tools, and expertise to help support drug discovery and development activities, IP portfolio optimization, identification of licensing and partnering opportunities, delivery of successful regulatory submissions, and the ability to keep current with the rapidly-changing pharmaceutical and chemical markets, supporting informed, early decisions.

This report was created by Thomson Reuters, using information from *Thomson Reuters Cortellis™ for Competitive Intelligence*; a comprehensive, proven intelligence solution that leverages the most accurate, complete, and widely respected drug pipeline information. From drug discovery and development activities to patent reports, the latest deals, and partnering opportunities, *Cortellis* can provide the confidence to make the most informed business decisions, faster. *Cortellis for Competitive Intelligence* provides accurate and validated information on pharmaceutical and biotechnology companies globally, their drug pipelines, deals, patents, and clinical trials, plus breaking industry news and conference coverage. All contained in one simple, highly intuitive research platform.

*Cortellis* Company Detailed Pipeline reports are the second in a series of that track pharmaceutical and biotechnology companies worldwide. All *Cortellis for Competitive Intelligence* content is subject to the most comprehensive editorial review process available, conducted by scientists, pharma professionals, regulatory experts, and generics specialists. Featuring timely drug pipeline information expertly uncovered and integrated from over 400 global meetings each year, you'll always be on top of the latest developments.

Chosen by leading life sciences companies, their executives and investors, *Cortellis for Competitive Intelligence* accelerates your deal-making and gives you timely insights on the development landscape.

**Discover undiscovered opportunities in drug development and licensing faster with *Thomson Reuters Cortellis™ for Competitive Intelligence***

### DISCLAIMER

The information contained in this report is based on sources believed to be correct but Thomson Reuters does not guarantee the accuracy, timeliness, or completeness of this information. Opinions, if any, are those held by the author of any individual report or article at the time of initial publication and do not necessarily reflect the views of Thomson Reuters.

Information in this report on companies is intended for reference use only, and does not constitute a recommendation to buy or sell any particular security or other investment and does not constitute an offer to buy from or sell to any particular investor. Any company or securities mentioned in this report may not be suitable for any particular investor, depending on that investor's financial position and needs.

[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](#)



TABLE OF CONTENTS

Company Overview..... 5

Company Profile..... 6

Product Portfolio Summary..... 6

Product Portfolio Drug Pipeline Detail..... 9

    Phase 2 Clinical..... 10

    Discovery..... 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 | 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 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".

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.

[Return to Table of Contents](#)

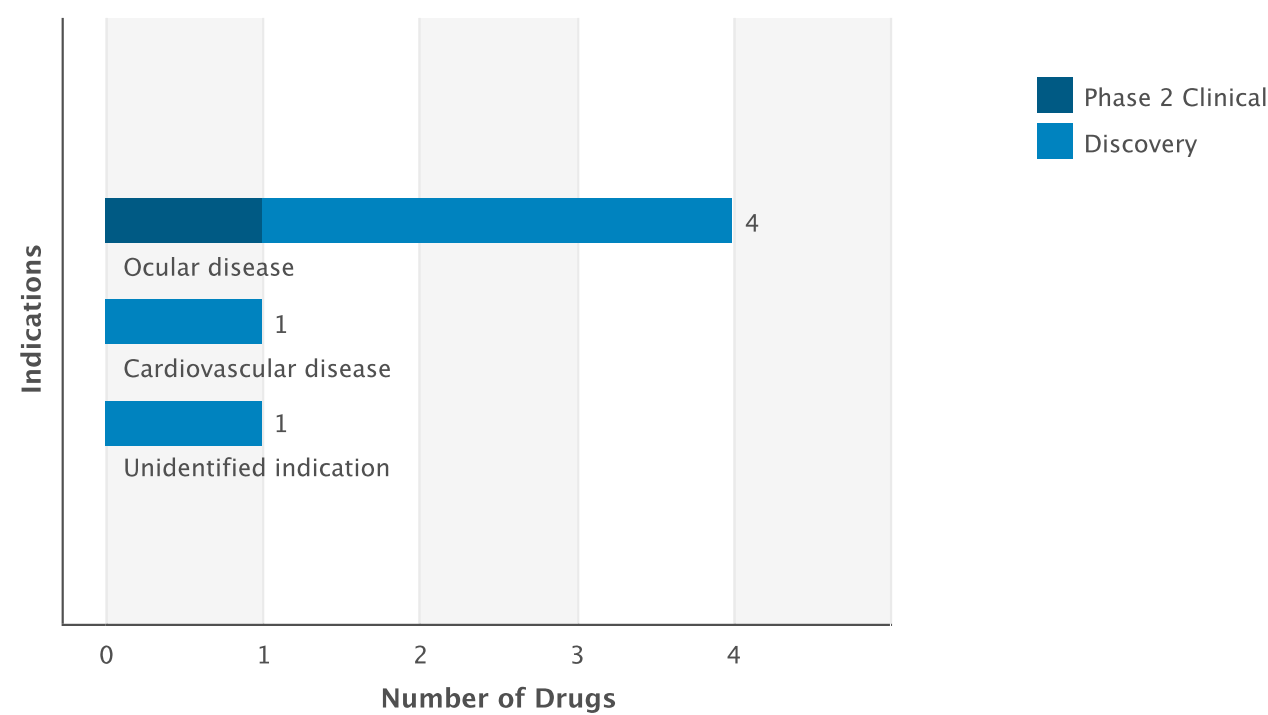


PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



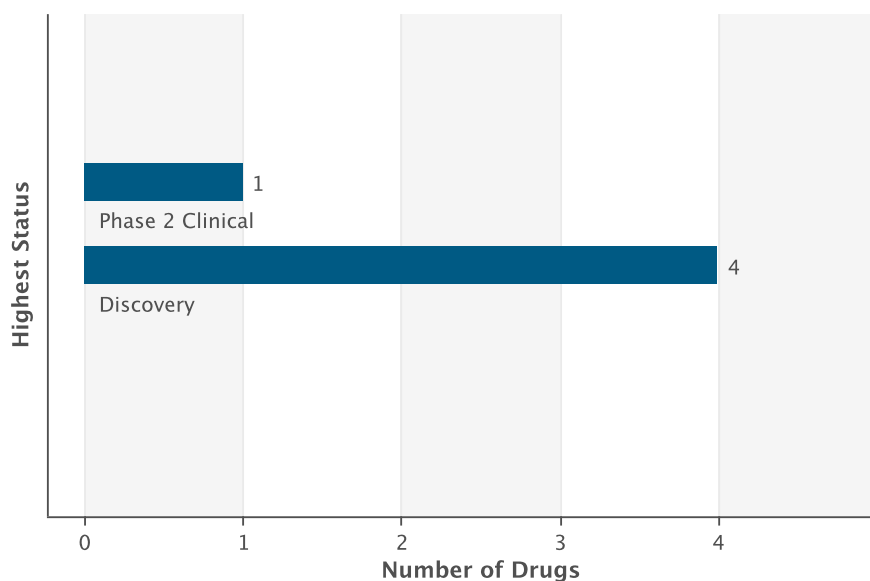
Drugs by Indication Table

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

## DEALS

| Deal Type                                    | Principal |          | Partner |          | Total |
|--|-----------|----------|---------|----------|-------|
|  | Active    | Inactive | Active  | Inactive |       |
| Drug - Development/Commercialization License | 1         | 0        | 1       | 0        | 2     |
| Technology - Delivery/Formulation            | 1         | 0        | 0       | 0        | 1     |
| Patent - Exclusive Rights                    | 0         | 0        | 1       | 0        | 1     |
| Drug - Funding                               | 2         | 0        | 0       | 0        | 2     |

## CLINICAL TRIALS

Trials by Condition Studied

| Condition Studied | Ongoing | All |
|-------------------|---------|-----|
| Ocular disease    | 1       | 1   |

[Return to Table of Contents](#)

## 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.

[Return to Table of Contents](#)





## PRODUCT PORTFOLIO DRUG PIPELINE DETAIL

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

|                             |   |
|-----------------------------|---|
| <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;Ophthalmological 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   |

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

#### SUMMARY

Avalanche Biotechnologies, in collaboration with Lions Eye Institute of the University of Western Australia, is developing a sustained-release, subretinal formulation of AVA-101 (rAAV.sFLT-1), a recombinant adeno-associated virus (AAV)-based therapy delivering Flt-1 (VEGF-1 receptor antagonist), for the potential treatment of wet age-related macular degeneration (AMD) . Avalanche is also investigating the program for diabetic macular edema (DME) and retinal vein occlusion (RVO). In December 2011, a phase I/II trial began ; in May 2012, preliminary data were presented ; in May 2013, further data were presented. In July 2014, top-line phase IIa data was expected mid-2015, and an IND-filing was expected in the second half of 2015. In May 2014, it was stated that upon completion of the phase IIa trial, Regeneron would have a time-limited right of first negotiation for certain rights to AVA-101 . In August 2014, IND enabling studies for DME and RVO were planned to begin in 2014 or 2015.

Merck Sharpe & Dohme was originally developing the drug; however, by November 2011, rights were licensed to Avalanche.

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

#### CURRENT DEVELOPMENT STATUS

[Return to Table of Contents](#)



| Company                                      | Indication                           | Country   | Development Status | Date        |
|--|--------------------------------------|-----------|--------------------|-------------|
| Avalanche Biotechnologies Inc                | Wet age related macular degeneration | Australia | Phase 2 Clinical   | 14-Dec-2011 |
| Lions Eye Institute of Western Australia Inc | Wet age related macular degeneration | Australia | Phase 2 Clinical   | 14-Dec-2011 |
| University of Western Australia              | Wet age related macular degeneration | Australia | Phase 2 Clinical   | 14-Dec-2011 |
| Avalanche Biotechnologies Inc                | Diabetic macular edema               | US        | Discovery          | 10-Sep-2012 |
| Avalanche Biotechnologies Inc                | Retinal venous occlusion             | US        | Discovery          | 08-Jul-2014 |
| Merck Sharp & Dohme Corp                     | Wet age related macular degeneration | US        | Discontinued       | 01-Nov-2011 |

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

| Names  | Type          |
|--|---------------|
| VEGF-1 (Flt-1) receptor antagonist (adenovirus vector, wet AMD), Avalanche Biotechnologies/Lions Eye Institute/ University of Western Australia<br>AVA-101 | Research Code |
| adenovirus-based therapy (wet AMD), Avalanche Biotechnologies/Lions Eye Institute<br>rAAV.sFLT-1   | Research Code |

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

#### Trials by Phase and Condition Studied

| Phase 4 Clinical                     |     | Phase 3 Clinical |     | Phase 2 Clinical |     | Phase 1 Clinical |     | Phase Unspecified |     | Total    |     |
|--------------------------------------|-----|------------------|-----|------------------|-----|------------------|-----|-------------------|-----|----------|-----|
| On-going                             | All | On-going         | All | On-going         | All | On-going         | All | On-going          | All | On-going | All |
| Wet age related macular degeneration |     |                  |     |                  |     |                  |     |                   |     |          |     |
| 0                                    | 0   | 0                | 0   | 0                | 0   | 1                | 1   | 0                 | 0   | 1        | 1   |

#### Total Trials by Phase and Status

| Phase 4 Clinical          |     | Phase 3 Clinical |     | Phase 2 Clinical |     | Phase 1 Clinical |     | Phase Unspecified |     | Total    |     |
|---------------------------|-----|------------------|-----|------------------|-----|------------------|-----|-------------------|-----|----------|-----|
| On-going                  | All | On-going         | All | On-going         | All | On-going         | All | On-going          | All | On-going | All |
| Total by Phase and Status |     |                  |     |                  |     |                  |     |                   |     |          |     |
| 0                         | 0   | 0                | 0   | 0                | 0   | 1                | 1   | 0                 | 0   | 1        | 1   |

#### Phase Definitions

##### Phase 3 Clinical

[Return to Table of Contents](#)

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)

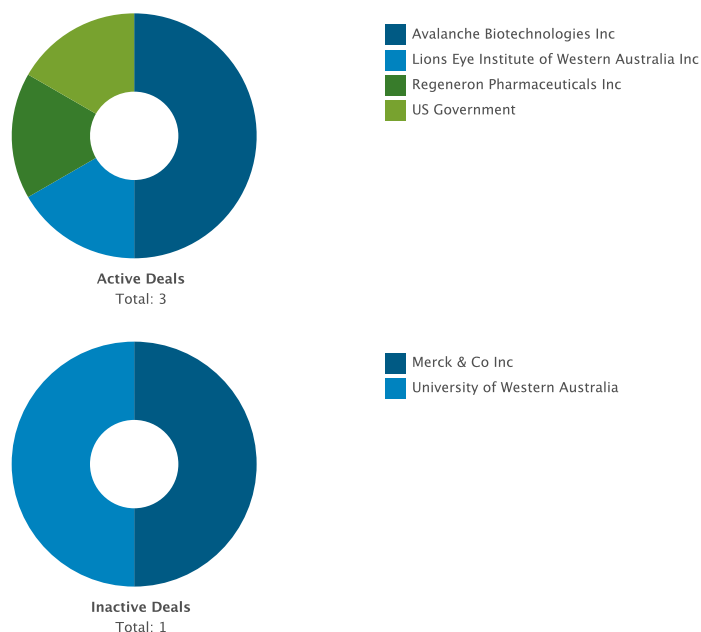
#### Phase 1 Clinical

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

## VEGF-1 (Flt-1) receptor antagonist (adenovirus vector, wet AMD), Avalanche Biotechnologies/Lions Eye Institute/ University of Western Australia DEALS AND PATENTS

### DEALS

#### Deals by Parent Company Chart

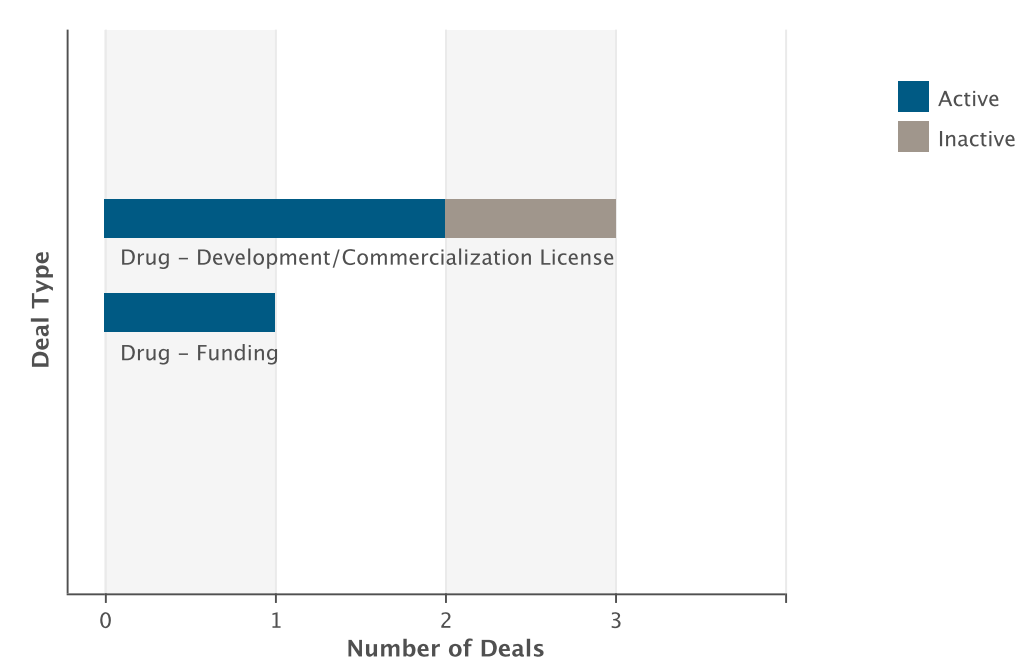


#### Deals by Parent Company Table

| Company Name                                 | Principal |          | Partner |          | Total |
|--|-----------|----------|---------|----------|-------|
|  | Active    | Inactive | Active  | Inactive |       |
| Avalanche Biotechnologies Inc                | 2         | 0        | 1       | 0        | 3     |
| US Government                                | 0         | 0        | 1       | 0        | 1     |
| Merck & Co Inc                               | 0         | 0        | 0       | 1        | 1     |
| University of Western Australia              | 0         | 1        | 0       | 0        | 1     |
| Regeneron Pharmaceuticals Inc                | 0         | 0        | 1       | 0        | 1     |
| Lions Eye Institute of Western Australia Inc | 1         | 0        | 0       | 0        | 1     |

[Return to Table of Contents](#)

Deals by Type Chart



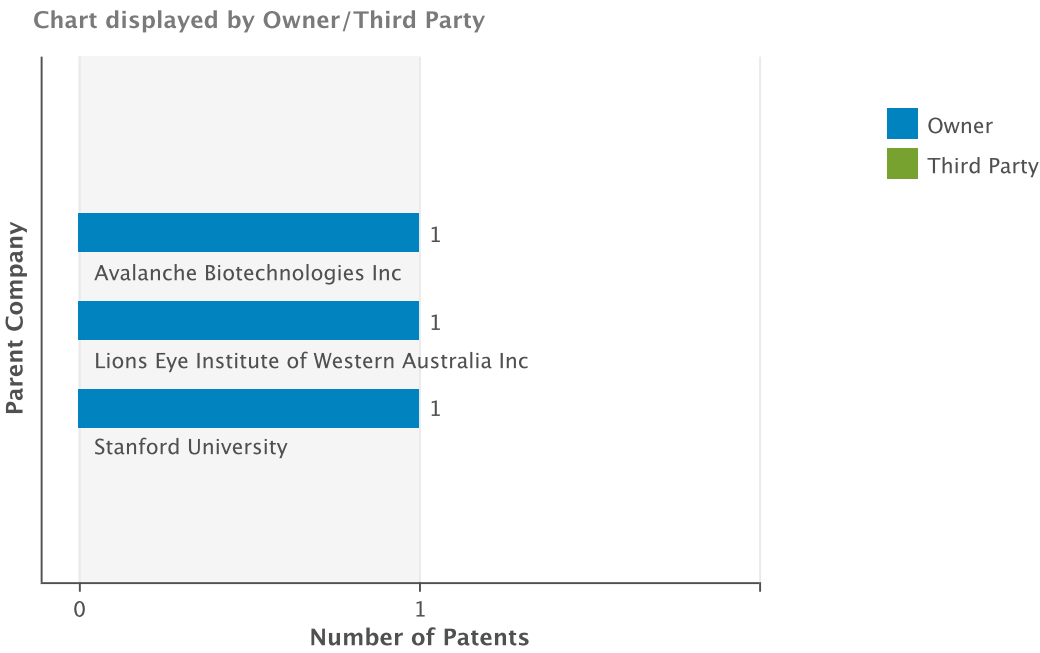
Deals by Type Table

| Deal Type                                    | Active | Inactive | Total |
|--|--------|----------|-------|
| Drug - Development/Commercialization License | 2      | 1        | 3     |
| Drug - Funding                               | 1      | 0        | 1     |

[Return to Table of Contents](#)

PATENTS

Patents by Parent Company Chart

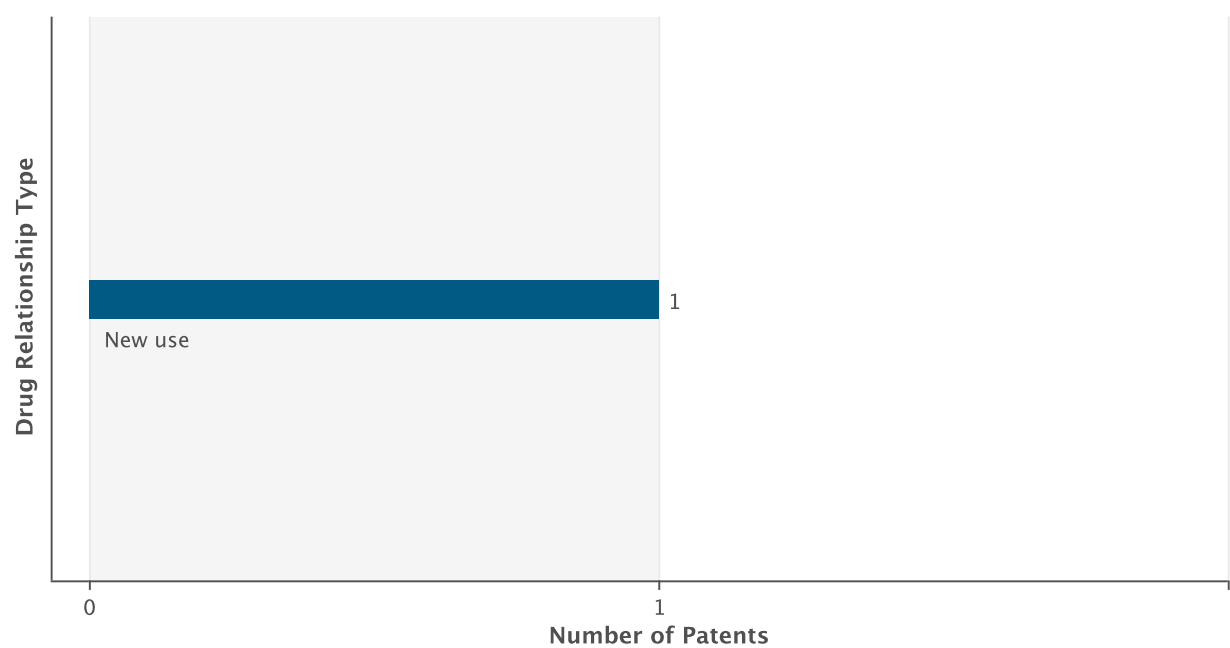


Patents by Parent Company Table

| Company Name                                 | As Owner | As Third Party | Total |
|--|----------|----------------|-------|
| Stanford University                          | 1        | 0              | 1     |
| Lions Eye Institute of Western Australia Inc | 1        | 0              | 1     |
| Avalanche Biotechnologies Inc                | 1        | 0              | 1     |

[Return to Table of Contents](#)

Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

| Drug Relationship | Total |
|-------------------|-------|
| New use           | 1     |

[Return to Table of Contents](#)

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

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

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

### AVA-322/AVA-323 (undisclosed indication), Avalanche DEVELOPMENT PROFILE

#### SUMMARY

Avalanche Biotechnologies is investigating a program including AVA-322 and AVA-323, for the potential treatment of an undisclosed indication. In July 2014, the program was in research, and IND-enabling studies were planned for 2015.

### AVA-322/AVA-323 (undisclosed indication), Avalanche DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

| Company                       | Indication              | Country | Development Status | Date        |
|-------------------------------|-------------------------|---------|--------------------|-------------|
| Avalanche Biotechnologies Inc | Unidentified indication | US      | Discovery          | 08-Jul-2014 |

### AVA-322/AVA-323 (undisclosed indication), Avalanche DRUG NAMES

| Names   | Type |
|---|------|
| AVA-322/AVA-323 (undisclosed indication), Avalanche |      |

[Return to Table of Contents](#)

## AVA-311

### AVA-311 SNAPSHOT

|                      |   |
|----------------------|---|
| 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        | Adeno-associated virus based gene therapy;Ophthalmological agent  |
| Technologies         | Injectable formulation;Virus recombinant;Ophthalmic formulation;Biological therapeutic;Parenteral formulation unspecified |
| Last Change Date     | 08-Jul-2014   |

### AVA-311 DEVELOPMENT PROFILE

#### SUMMARY

Avalanche Biotechnologies in collaboration with Regeneron, is investigating an intravitreal formulation of AVA-311, an adeno-associated virus (AAV)-based gene therapy delivering RS1 gene, using Avalanche's directed evolution technology, for the potential treatment of juvenile X-linked retinoschisis (XLRS). In July 2014, the drug was in preclinical development.

### AVA-311 DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

| Company                       | Indication    | Country | Development Status | Date        |
|-------------------------------|---------------|---------|--------------------|-------------|
| Avalanche Biotechnologies Inc | Retinoschisis | US      | Discovery          | 05-May-2014 |
| Regeneron Pharmaceuticals Inc | Retinoschisis | US      | Discovery          | 05-May-2014 |

### AVA-311 DRUG NAMES

| Names   | Type          |
|---------|---------------|
| AVA-311 | Research Code |

[Return to Table of Contents](#)

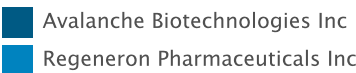
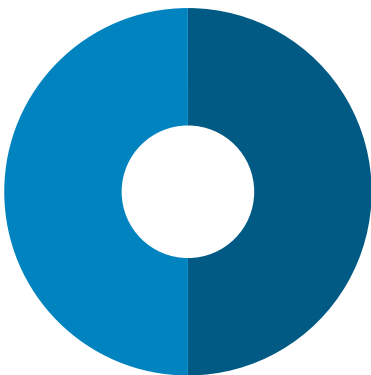




AVA-311 DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



Deals by Parent Company Table

| Company Name                  | Principal |          | Partner |          | Total |
|-------------------------------|-----------|----------|---------|----------|-------|
|                               | Active    | Inactive | Active  | Inactive |       |
| Avalanche Biotechnologies Inc | 1         | 0        | 0       | 0        | 1     |
| Regeneron Pharmaceuticals Inc | 0         | 0        | 1       | 0        | 1     |

[Return to Table of Contents](#)

Deals by Type Chart



Deals by Type Table

| Deal Type                                    | Active | Inactive | Total |
|--|--------|----------|-------|
| Drug - Development/Commercialization License | 1      | 0        | 1     |

[Return to Table of Contents](#)

## Shh10.GDNF

### Shh10.GDNF SNAPSHOT

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

### Shh10.GDNF DEVELOPMENT PROFILE

#### SUMMARY

Avalanche is investigating Shh-10.GDNF, a novel muLLer glia cell-specific adeno-associated viral vector expressing glial cell line-derived neurotrophic factor (GDNF), for the potential treatment of glaucoma. In May 2012, preclinical data were presented. In March 2013, development was ongoing.

### Shh10.GDNF DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

| Company                       | Indication | Country | Development Status | Date        |
|-------------------------------|------------|---------|--------------------|-------------|
| Avalanche Biotechnologies Inc | Glaucoma   | US      | Discovery          | 08-May-2012 |

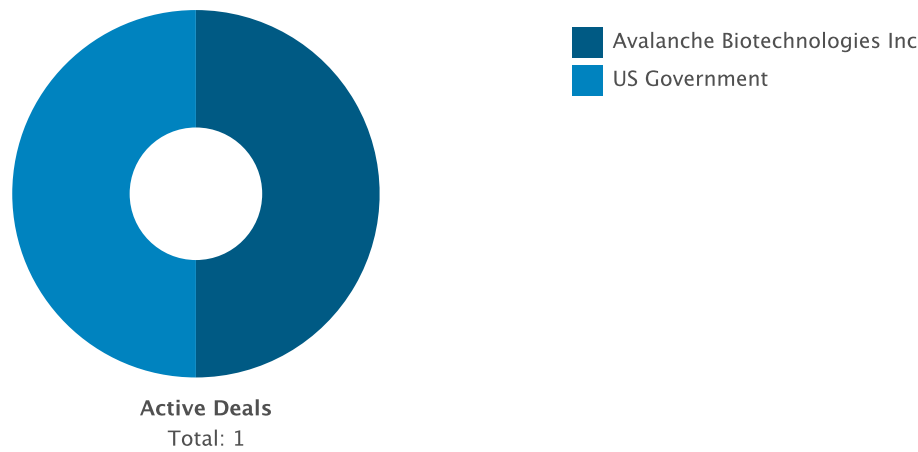
### Shh10.GDNF DRUG NAMES

| Names                                   | Type          |
|---|---------------|
| Shh10.GDNF                              | Research Code |
| AAV-based therapy (glaucoma), Avalanche |               |

[Return to Table of Contents](#)

DEALS

Deals by Parent Company Chart

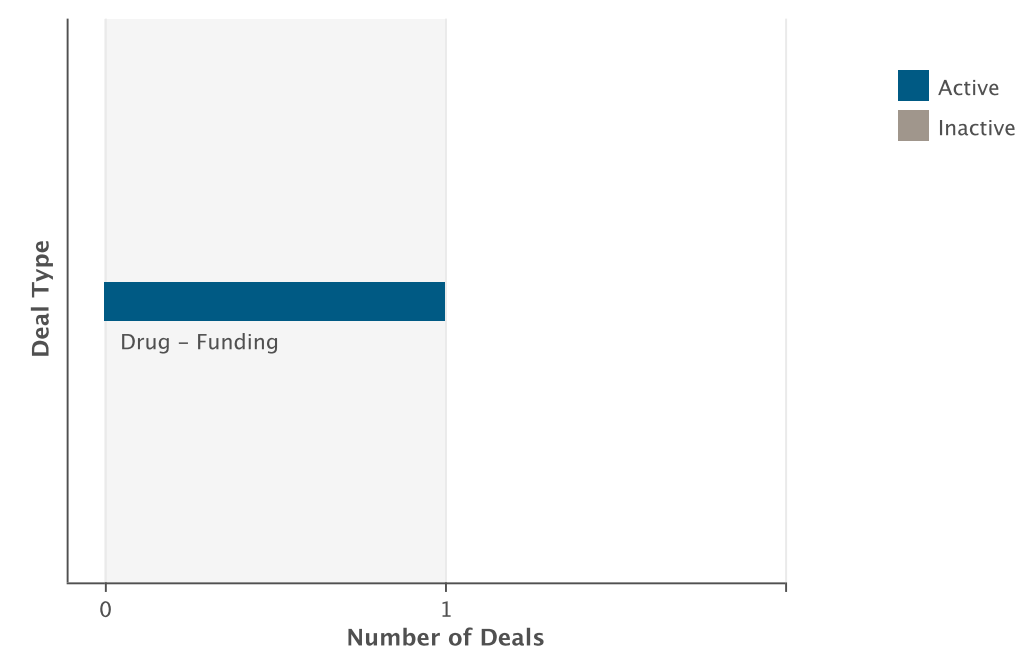


Deals by Parent Company Table

| Company Name                  | Principal |          | Partner |          | Total |
|-------------------------------|-----------|----------|---------|----------|-------|
|                               | Active    | Inactive | Active  | Inactive |       |
| Avalanche Biotechnologies Inc | 1         | 0        | 0       | 0        | 1     |
| US Government                 | 0         | 0        | 1       | 0        | 1     |

[Return to Table of Contents](#)

Deals by Type Chart



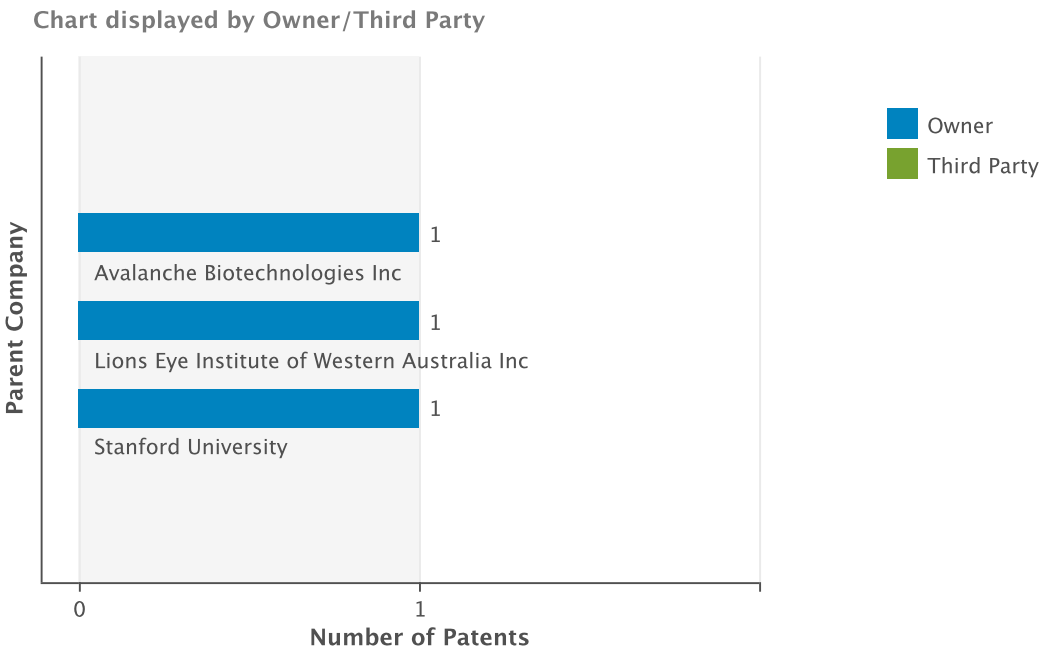
Deals by Type Table

| Deal Type      | Active | Inactive | Total |
|----------------|--------|----------|-------|
| Drug - Funding | 1      | 0        | 1     |

[Return to Table of Contents](#)

PATENTS

Patents by Parent Company Chart

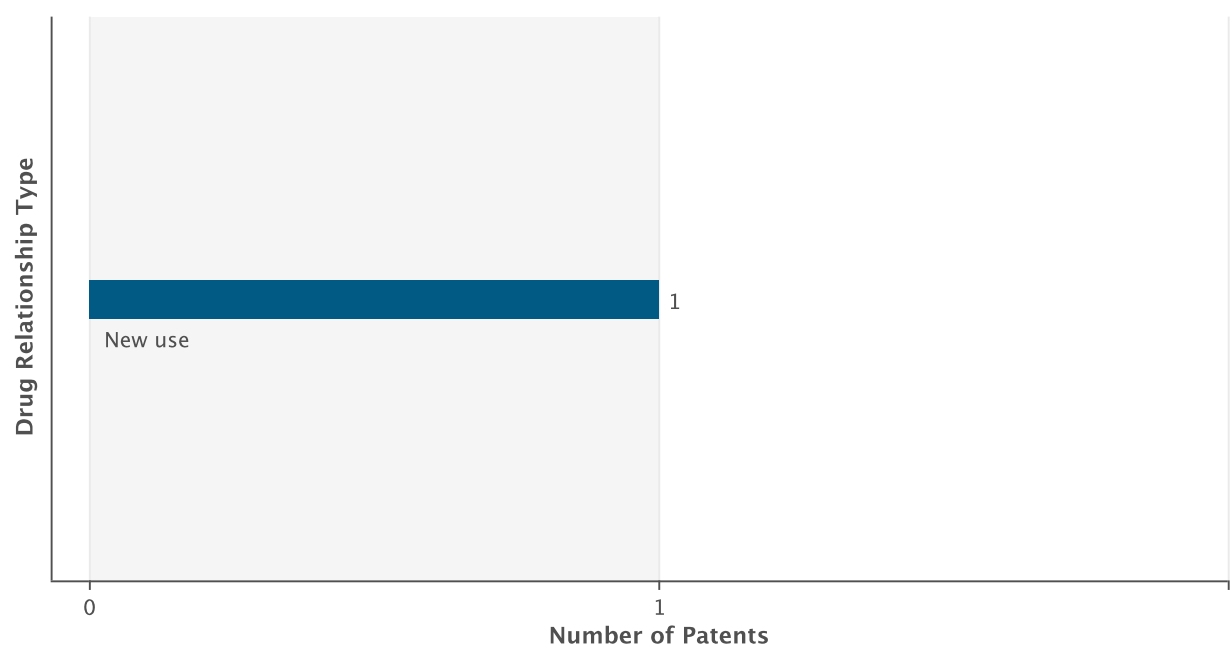


Patents by Parent Company Table

| Company Name                                 | As Owner | As Third Party | Total |
|--|----------|----------------|-------|
| Avalanche Biotechnologies Inc                | 1        | 0              | 1     |
| Stanford University                          | 1        | 0              | 1     |
| Lions Eye Institute of Western Australia Inc | 1        | 0              | 1     |

[Return to Table of Contents](#)

Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

| Drug Relationship | Total |
|-------------------|-------|
| New use           | 1     |

[Return to Table of Contents](#)

## AVA-201

### AVA-201 SNAPSHOT

|                      |   |
|----------------------|---|
| 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;Ophthalmological agent;Ocular antineovascularisation agent                      |
| Technologies         | Injectable formulation;Virus recombinant;Ophthalmic formulation;Biological therapeutic;Parenteral formulation unspecified |
| Last Change Date     | 08-Jul-2014   |

### AVA-201 DEVELOPMENT PROFILE

#### SUMMARY

Avalanche Biotechnologies is investigating an intravitreal formulation of AVA-201, a recombinant adeno-associated virus (AAV)-based gene therapy delivering sFlt-1 (VEGF-1 receptor antagonist) using the company's next-generation AAV vector delivery technology, for the potential prevention of wet age-related macular degeneration (AMD). In July 2014, the drug was in research, and IND-enabling studies were planned for 2015.

### AVA-201 DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

| Company                       | Indication                           | Country | Development Status | Date        |
|-------------------------------|--------------------------------------|---------|--------------------|-------------|
| Avalanche Biotechnologies Inc | Wet age related macular degeneration | US      | Discovery          | 08-Jul-2014 |

### AVA-201 DRUG NAMES

| Names   | Type          |
|---------|---------------|
| AVA-201 | Research Code |

[Return to Table of Contents](#)





This report was created by Thomson Reuters, using information from *Thomson Reuters Cortellis™ for Competitive Intelligence*; a comprehensive, proven intelligence solution that leverages the most accurate, complete, and widely respected drug pipeline information.

For more information about *Cortellis for Competitive Intelligence*, visit:

[http://cortellis.thomsonreuters.com/cortellis\\_for\\_you/?cid=thomsonone](http://cortellis.thomsonreuters.com/cortellis_for_you/?cid=thomsonone).

For subscription information, e-mail [scientific.lifesciences@thomsonreuters.com](mailto:scientific.lifesciences@thomsonreuters.com).

© 2012 Thomson Reuters. All rights reserved.  
Republication or redistribution of Thomson Reuters content, including by framing or similar means, is prohibited without the prior written consent of Thomson Reuters. 'Thomson Reuters' and the Thomson Reuters logo are registered trademarks and trademarks of Thomson Reuters and its affiliated companies.

[Return to Table of Contents](#)

