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Avalanche Biotechnologies Inc

CORTELLIS COMPANY DETAILED PIPELINE REPORT

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

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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.

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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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

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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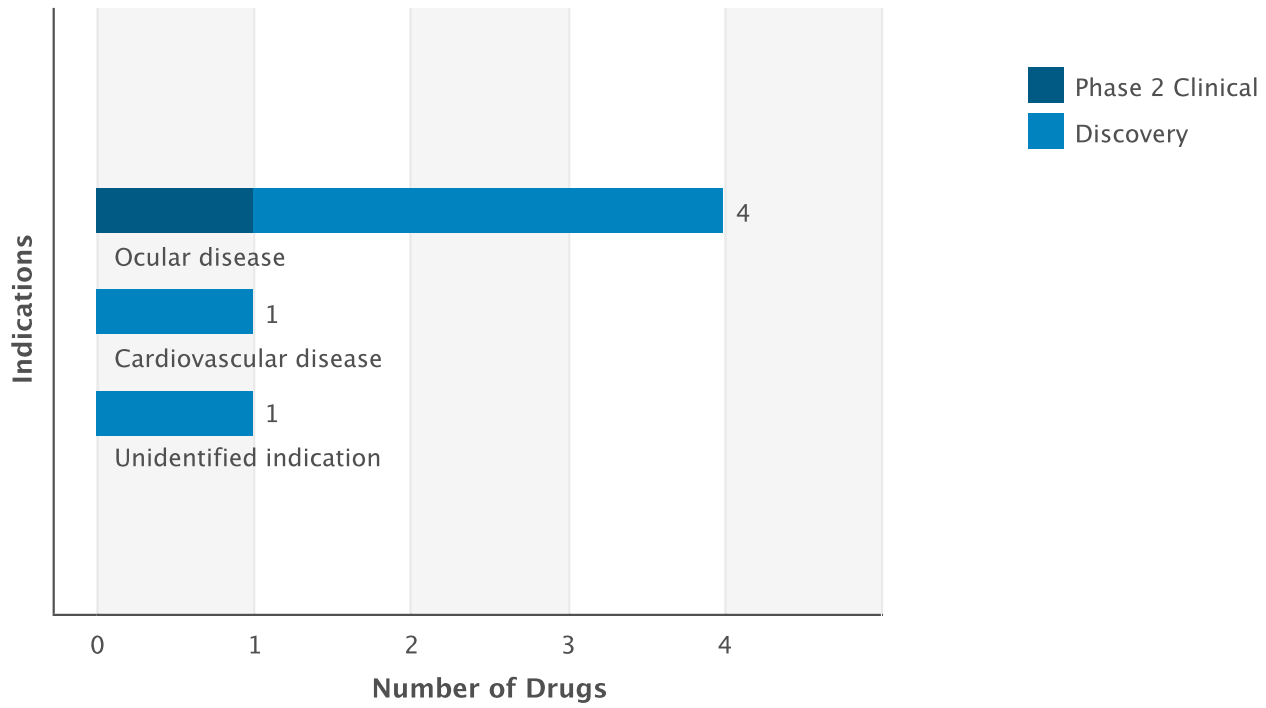
PRODUCT PORTFOLIO SUMMARY

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

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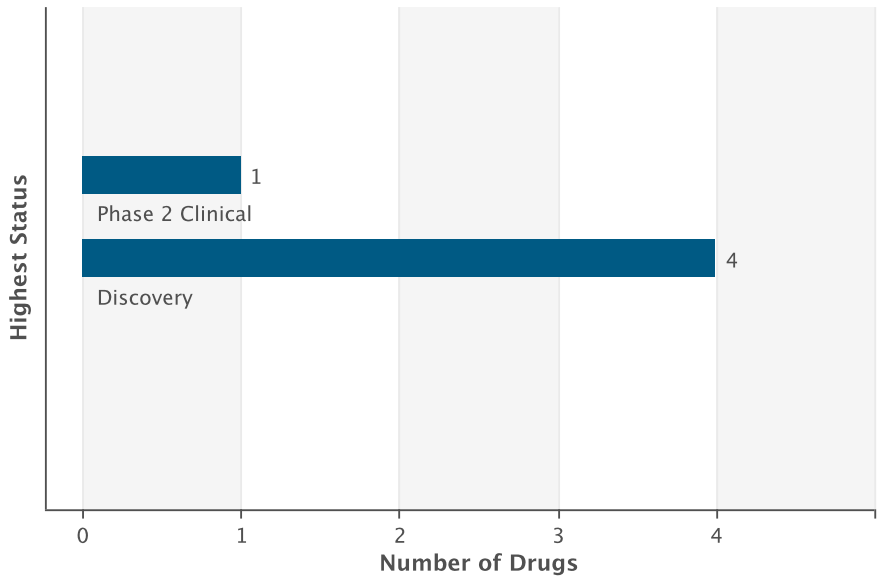
Drugs by Highest Status

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	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 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 Institue/ University of Western Australia

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

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

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

CURRENT DEVELOPMENT STATUS

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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 Institue/ University of Western Australia DRUG NAMES

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

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

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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)

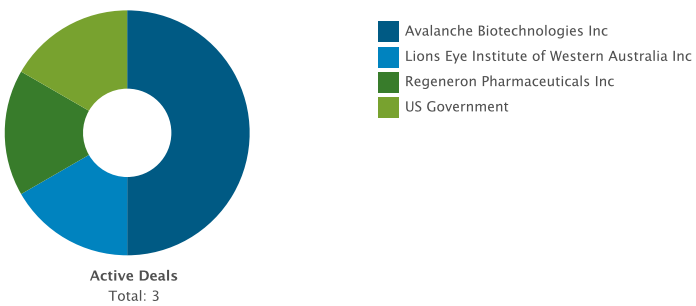
Phase 1 Clinical

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

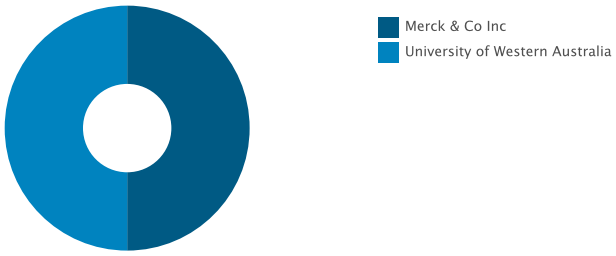
VEGF-1 (Flt-1) receptor antagonist (adenovirus vector, wet AMD), Avalanche Biotechnologies/Lions Eye Institue/ University of Western Australia DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



Active Deals
Total: 3



Inactive Deals
Total: 1

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

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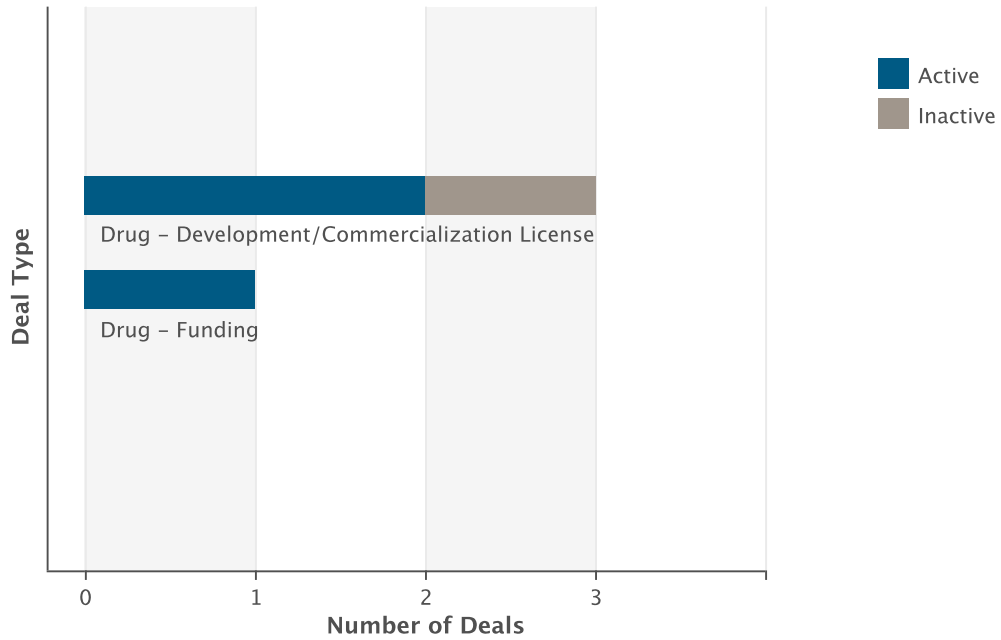
Deals by Type Chart



Deals by Type Table

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

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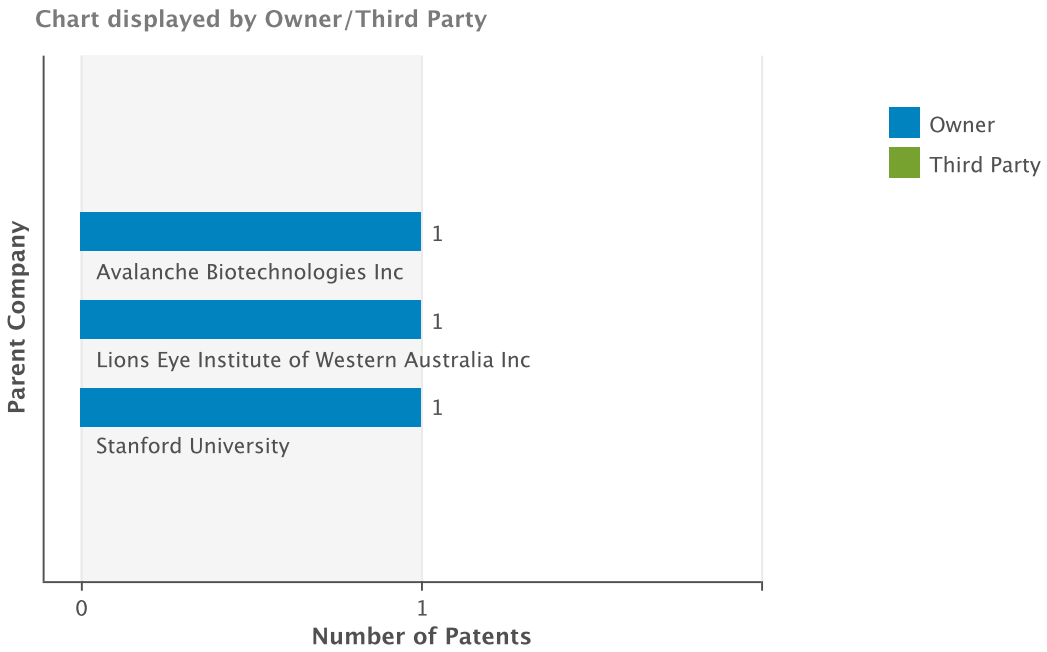
PATENTS

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

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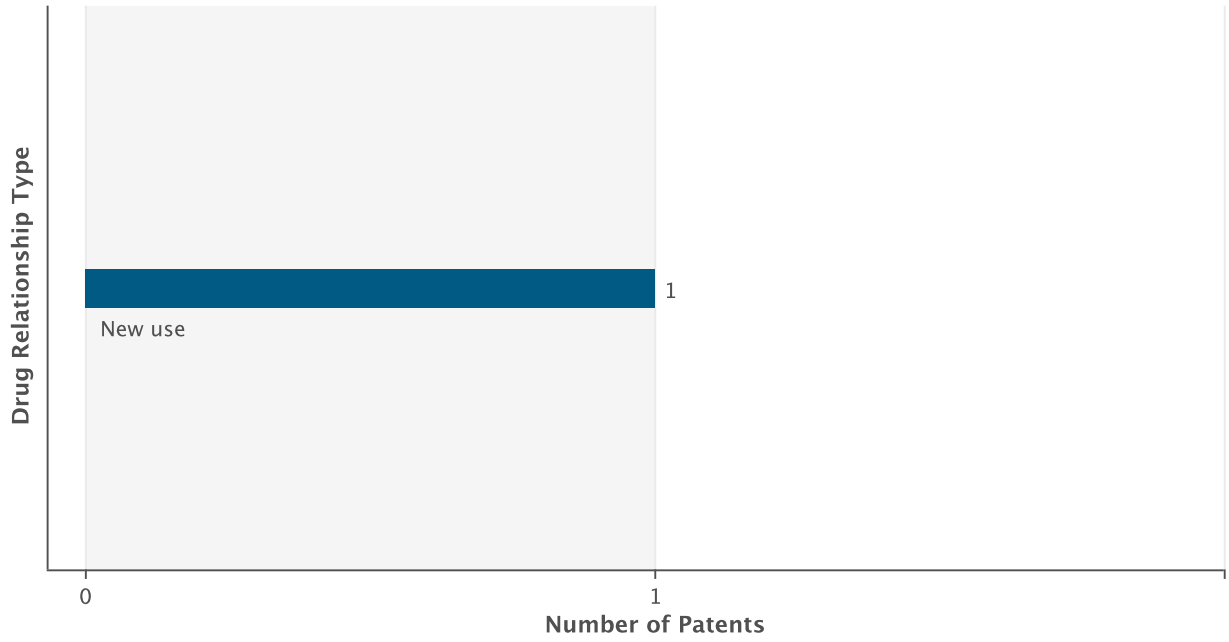
Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
New use	1

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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	

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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	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

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

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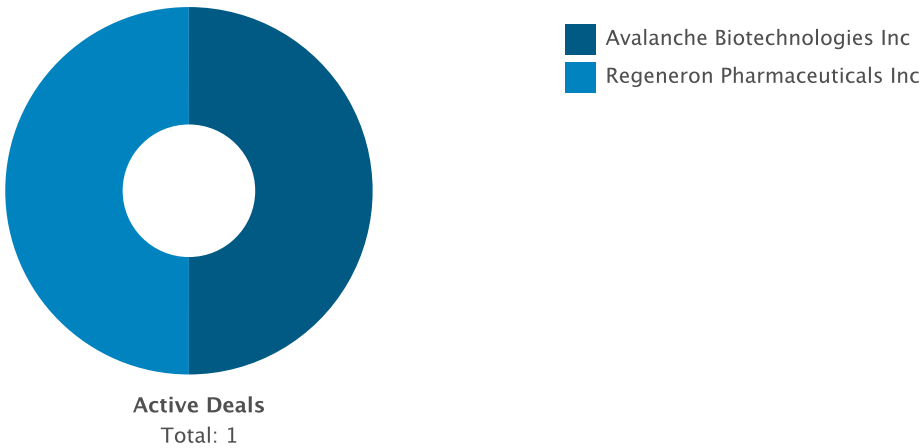
AVA-311 DEALS AND PATENTS

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

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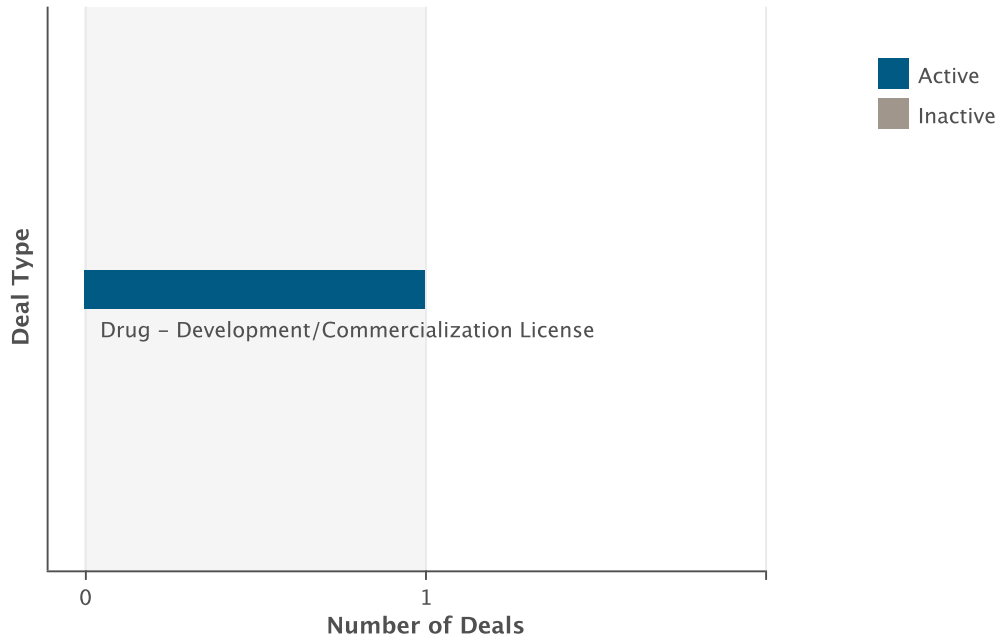
Deals by Type Chart



Deals by Type Table

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

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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	

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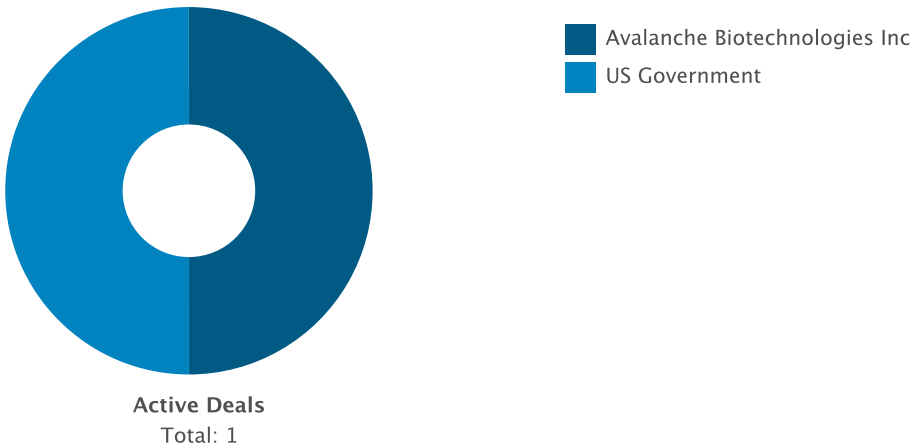
Shh10.GDNF DEALS AND PATENTS

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
US Government	0	0	1	0	1

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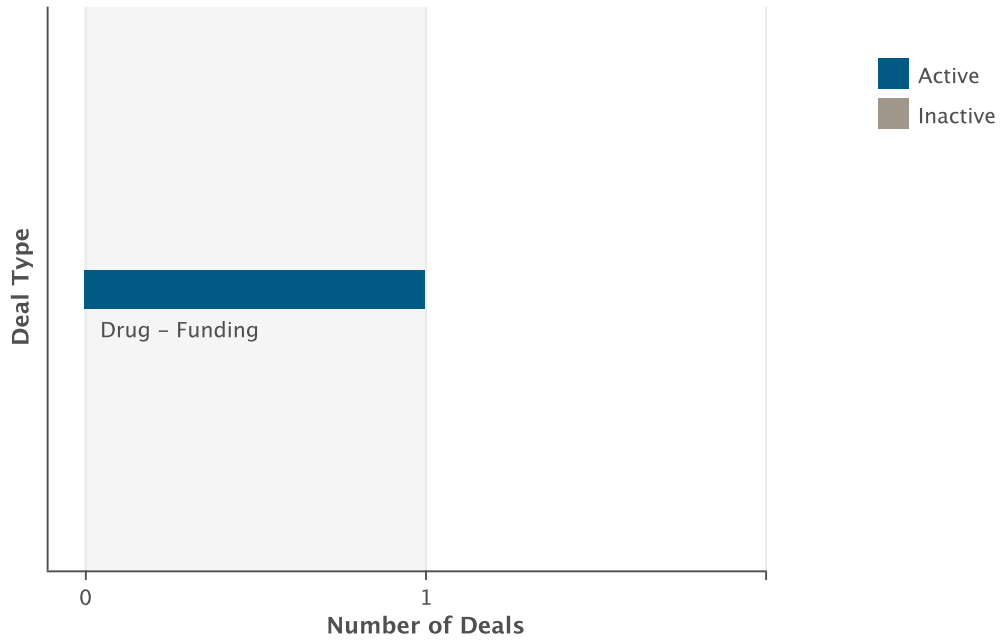
Deals by Type Chart



Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Funding	1	0	1

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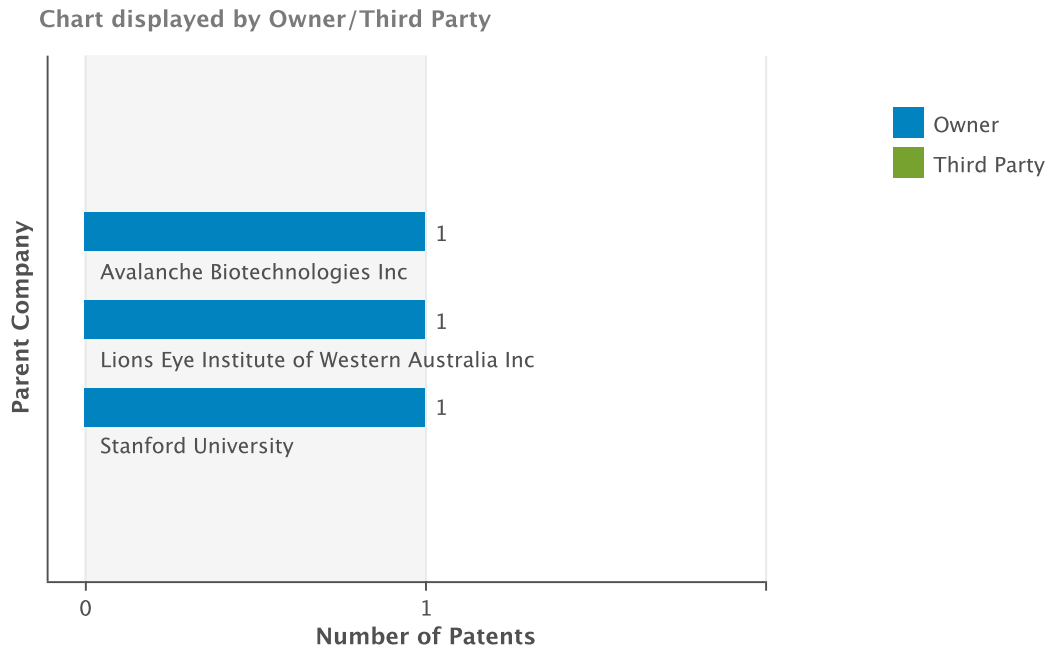
PATENTS

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

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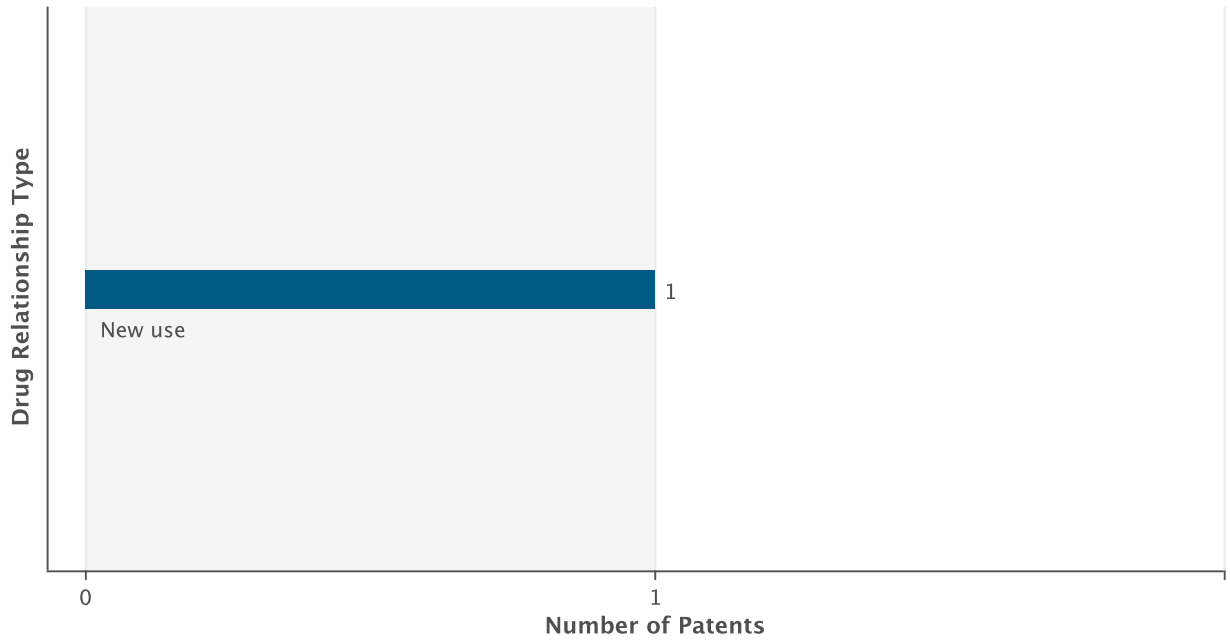
Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
New use	1

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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	Ophthalmological agent;Adeno-associated virus based gene therapy;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

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