

## **Ambit Biosciences Corp**

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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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

## COMPANY OVERVIEW

<b>Company Name</b>	Ambit Biosciences Corp
<b>Parent Company Name</b>	Ambit Biosciences Corp
<b>Website</b>	http://www.ambitbio.com/
<b>Country</b>	US
<b>Number of Drugs in Active Development</b>	3
<b>Number of Inactive Drugs</b>	11
<b>Number of Patents as Owner</b>	41
<b>Number of Patents as Third Party</b>	1
<b>Number of Deals</b>	10
<b>Key Indications</b>	Cancer, Inflammatory disease, Autoimmune disease, Acute myelogenous leukemia, Breast tumor, Leukemia, Insulin dependent diabetes, Multiple sclerosis, Myeloproliferative disorder, Solid tumor
<b>Key Target-based Actions</b>	CSF-1 antagonist, Jak2 tyrosine kinase inhibitor, Flt3 tyrosine kinase inhibitor, Kit tyrosine kinase inhibitor, PDGF receptor antagonist, Aurora protein kinase 2 inhibitor, ErbB4 tyrosine kinase receptor inhibitor, ABL family tyrosine kinase inhibitor, Protein kinase inhibitor, Raf B protein kinase inhibitor, VEGF-2 receptor antagonist
<b>Key Technologies</b>	Small molecule therapeutic, Oral formulation, Oral liquid formulation, Crystalline form, Salt synthesis, Antibody, Drug combination, Drug screening, Fluorescence, Formulation preservation, Intravenous formulation

## COMPANY PROFILE

### SUMMARY

Ambit Biosciences, based in San Diego, CA is a privately held biopharmaceutical company focused on the discovery and development of small molecule kinase inhibitors for cancer and other diseases. The company identifies and manufactures these molecules using its kinase profiling platform technology, KinomeScan.

### COMPANY LOCATION

The company is based in San Diego, CA. It also has a research facility based in Toronto, Canada.

### LICENSING AGREEMENTS

In November 2006, Ambit agreed to screen, discover and develop kinase inhibitors with Cephalon using its KinomeScan technology and Cephalon's kinase libraries. Cephalon would pay Ambit \$18 million upfront and make milestone payments up to \$232.5 million plus royalties. The companies also planned to advance two programs targeting undisclosed kinases.

In December 2005, Ambit and Bristol-Myers Squibb (BMS) expanded their collaboration to discover kinase inhibitors. The new, 5-year agreement provided BMS with access to Ambit's kinase profiling technology, KinomeScan, to accelerate its internal drug discovery and development efforts. Ambit received a license to a BMS preclinical kinase inhibitor program for the treatment of solid tumors. Ambit would also get an upfront payment, equity investment and profiling revenue from BMS. In January 2004, Ambit entered into separate kinase screening collaborations with Bristol-Myers Squibb Co and GlaxoSmithKline plc, whereby Ambit would employ its kinase platform to characterize the specificity of certain compounds from the companies' compound libraries. Ambit also entered into a compound profiling agreement with Pfizer Inc's La Jolla Laboratories. Ambit would employ its Reverse Screening technology to identify and characterize the protein targets of certain Pfizer drug discovery compounds and therefore elucidate their molecular mechanisms of actions.

In August 2004, Roche Holding AG and Ambit entered into a multiyear collaboration. Roche would use Ambit's kinase screening platform to profile and select small-molecule kinase inhibitors. Ambit would receive milestone payments and royalties on drugs developed through the collaboration.

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In March 2003, Ambit signed a technology evaluation agreement with GlaxoSmithKline (GSK) to characterize small molecule kinase inhibitors using Ambit's ProteomeScan platform.

In April 2002, Ambit entered into a research collaboration agreement with AstraZeneca Pharmaceuticals LP (AZ), whereby AZ was to utilize Ambit's ProteomeScan platform to identify and isolate the protein targets of its portfolio of small molecules.

In November 2001, Medarex Inc and Ambit entered into a multiyear strategic collaboration to jointly develop and commercialize therapeutic monoclonal antibodies, utilizing Medarex's UltiMAb human antibody development system and Ambit's ProteomeScan screening technology. Both companies expected to share equally the costs and revenues associated with the preclinical and clinical development and subsequent commercialization of any resulting products. Additionally, Medarex made a \$1 million equity investment in Ambit.

The company was founded to commercialize technology initially developed in Dr Austin's laboratory in the Chemistry Department at Yale University. This technology, called ProteomeScan, has been exclusively licensed by Ambit Biosciences from Yale University and is the subject of pending US and international patent applications.

## FINANCIAL

In June 2013, Ambit was added to the Russell MicroCap Index effective at close of business on June 28, 2013.

In May 2013, Ambit priced its initial public offering of 8,125,000 shares of its common stock at \$8 per share. Underwriters were granted a 30-day option to purchase an additional 1,218,750 shares. The common stock began trading on the NASDAQ Global Market under the symbol "AMBI".

In February 2013, Ambit filed a registration (form S-1) for an initial public common stock offering on the NASDAQ market.

In November 2012, Ambit raised \$25 million in the first tranche of a new \$50 million stock financing round.

In June 2011, Ambit raised \$30 million from a series D-2 equity financing round.

In November 2010, Ambit filed a registration statement for an IPO with the SEC. Later that month, the IPO was reported to be expected to raise \$86.3 million.

In November 2007, Ambit raised \$49.3 million from a series D equity financing round. Ambit would use the proceeds to advance its pipeline products.

In May 2005, Ambit closed a series C financing, led by Roche, raising a total of \$31 million. The first \$21 million tranche was secured in August 2004.

The company raised \$10 million in November 2001.

In November 2000, Ambit raised \$18.8 million from a series B financing round.

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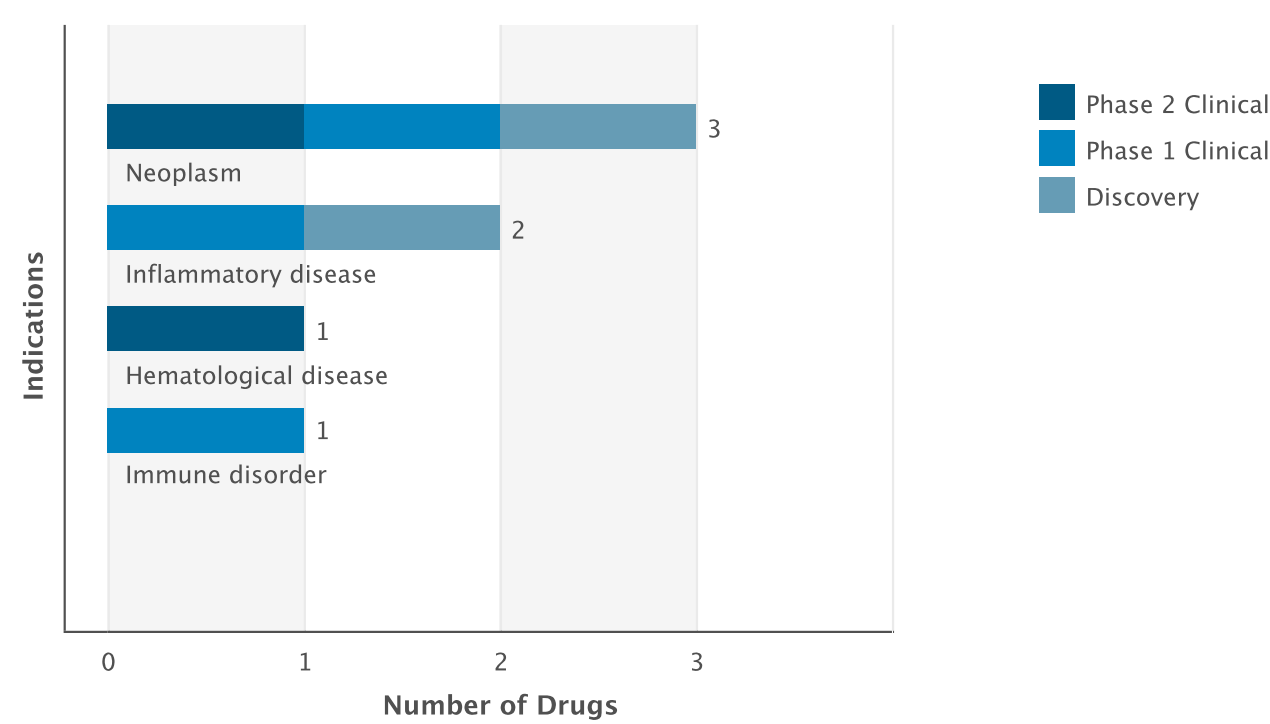


PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



Drugs by Indication Table

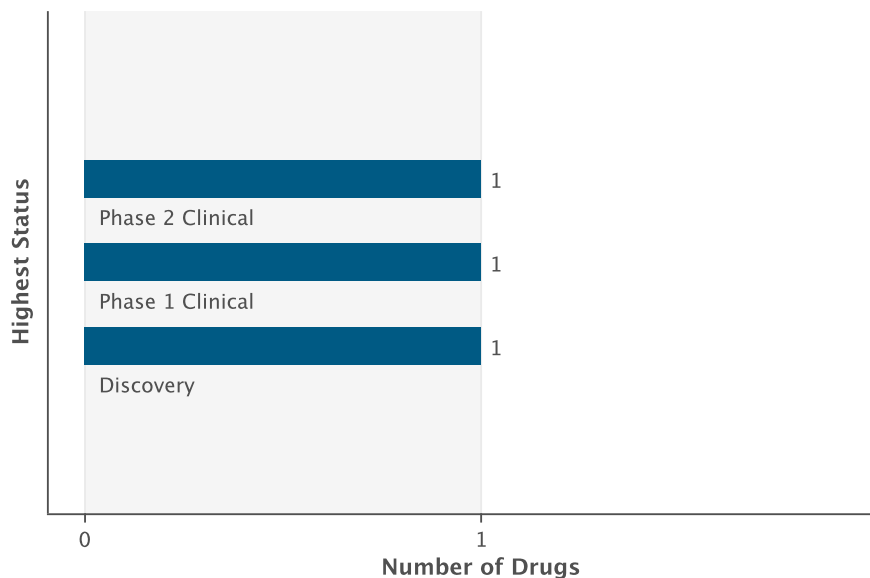
Indication	Active	Inactive	Total
Neoplasm	3	10	13
Inflammatory disease	2	2	4
Immune disorder	1	1	2
Neurological disease	0	2	2
Hematological disease	1	1	2
Musculoskeletal disease	0	1	1
Cardiovascular disease	0	1	1

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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
Phase 1 Clinical	1
Discovery	1
Discontinued	3
No Development Reported	8

## DEALS

Deal Type	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Technology - Other Proprietary	1	0	0	0	1
Drug - Screening/Evaluation	5	0	0	0	5
Drug - Early Research/Development	1	0	1	0	2
Drug - Development/Commercialization License	1	0	1	0	2

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## CLINICAL TRIALS

### Trials by Condition Studied

Condition Studied	Ongoing	All
Neoplasm	5	11
Hematological disease	4	7
Immune disorder	0	2
Inflammatory disease	0	1
Musculoskeletal disease	0	1

### Trials by Phase

Phase	Ongoing	All
Phase 2	2	2
Phase 1	3	10

### 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	8	1	9
Endocrine disease	16	1	17
Gastrointestinal disease	14	1	15
Genitourinary disease	11	0	11
Growth disorder	2	0	2
Hematological disease	24	1	25
Degeneration	3	0	3
Andrology	2	0	2
Immune disorder	24	1	25
Psychiatric disorder	1	0	1

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Musculoskeletal disease	7	0	7
Neoplasm	36	1	37
Ocular disease	5	0	5
Genetic disorder	2	0	2
Metabolic disorder	11	1	12
Neurological disease	14	1	15
Nutritional disorder	1	0	1
Respiratory disease	11	0	11
Infectious disease	4	0	4
Unidentified indication	2	0	2
Inflammatory disease	27	0	27
Otorhinolaryngological disease	2	0	2
Gynecology and obstetrics	10	0	10
Dermatological disease	8	0	8
Surgical procedure	0	1	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

### quizartinib dihydrochloride

<b>Drug Name</b>	quizartinib dihydrochloride
<b>Key Synonyms</b>	quizartinib dihydrochloride, quizartinib
<b>Originator Company</b>	Ambit Biosciences Corp
<b>Active Companies</b>	Ambit Biosciences Corp
<b>Inactive Companies</b>	Astellas Pharma Inc
<b>Highest Status</b>	Phase 2 Clinical
<b>Active Indications</b>	Acute myelogenous leukemia
<b>Target-based Actions</b>	PDGF receptor antagonist, Flt3 tyrosine kinase inhibitor, Kit tyrosine kinase inhibitor
<b>Other Actions</b>	Anticancer protein kinase inhibitor, Apoptosis stimulator
<b>Technologies</b>	Oral formulation, Oral liquid formulation, Small molecule therapeutic
<b>Last Change Date</b>	14-Jun-2013

### AC-410

<b>Drug Name</b>	AC-410
<b>Key Synonyms</b>	
<b>Originator Company</b>	Ambit Biosciences Corp
<b>Active Companies</b>	Ambit Biosciences Corp
<b>Inactive Companies</b>	
<b>Highest Status</b>	Phase 1 Clinical
<b>Active Indications</b>	Cancer, Inflammatory disease, Autoimmune disease
<b>Target-based Actions</b>	Jak2 tyrosine kinase inhibitor
<b>Other Actions</b>	Anti-inflammatory, Anticancer protein kinase inhibitor
<b>Technologies</b>	Oral formulation, Small molecule therapeutic
<b>Last Change Date</b>	26-Feb-2013

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## AC-708

<b>Drug Name</b>	AC-708
<b>Key Synonyms</b>	
<b>Originator Company</b>	Ambit Biosciences Corp
<b>Active Companies</b>	Ambit Biosciences Corp
<b>Inactive Companies</b>	
<b>Highest Status</b>	Discovery
<b>Active Indications</b>	Cancer, Inflammatory disease
<b>Target-based Actions</b>	CSF-1 antagonist
<b>Other Actions</b>	Anti-inflammatory, Anticancer protein kinase inhibitor, Bone modulator
<b>Technologies</b>	Small molecule therapeutic
<b>Last Change Date</b>	24-May-2013

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