# ACTELION COMPANY PROFILE

## INTRODUCTION

Actelion is a biopharmaceutical company focused on the discovery, development and commercialization of small-molecule drugs as innovative treatments to serve high unmet medical needs. The company has its corporate headquarters in Allschwil/Basel, Switzerland where it was founded in 1997. Its shares have been listed on the SIX Swiss Exchange (ticker symbol ALTN) since 2000. In September 2008, Actelion shares began trading as part of the blue-chip SMI® (Swiss Market Index).

The company has proven its ability to discover new compounds and to rapidly move them from research through development to commercialization. In particular, Actelion scientists were among the first to work in the field of endothelin receptor antagonists, leading to its lead product, Tracleer®.

Actelion has over 30 operative affiliates around the world including the United States, Canada, Brazil, Australia, Japan, Switzerland and a number of EU countries. These subsidiaries provide or will provide distribution, sales and marketing services.

## FINANCIAL OVERVIEW

| In CHF million | Q1 2012 Results | Q1 2011 Results | in % CHF | in % LC |
|---|---|---|---|---|
| Net Revenues | 417.5 | 528.2 | (21) | (18) |
| Operating Expenses | 350.3 | 363.6 | (4) | (2) |
| Operating Income | 67.1 | 164.7 | (59) | (54) |
| Core Earnings | 107.6 | 127.9 | (16) | (9) |
| Net Income / (Loss) | 45.1 | 146.3 | (69) | (64) |
| Diluted EPS in CHF | 0.38 | 1.20 | (68) | (63) |
| No of shares in calculation | 118.9 | 122.1 | | |

The full financial statements can be found on: http://www.actelion.com

**Share information**

IPO: 6 April 2000

Listed: SIX Swiss, Exchange (ATLN), Zürich, Switzerland

Trading Symbols Reuters ATLN.S, Bloomberg ATLN

## COMPANY STRATEGY

Since its founding more than a decade ago, Actelion has become a new kind of biopharmaceutical company: one that blends biotech's innovation, speed and flexibility with big pharma's operating discipline and excellence in execution. With the intrinsic belief that innovation in all domains is the key to growth, Actelion has built a robust pipeline, four approved products, and commercial operations in over 30 countries. Our more than 2,500 employees have a common purpose: to improve patients' lives by creating innovative medicines that make a real difference.

### DRIVING GROWTH

Our strategy is built on four principles:

- **Follow innovation where it leads.** Pursue an R&D-focused growth strategy by driving internal projects, especially those which would accelerate value creation. We seek licensing deals or acquisitions that are synergetic to existing businesses or to provide an infrastructure platform for our pipeline products.

- **Retain the value of innovation.** We develop projects within the company, consider partnerships for strategic or financial benefit; if appropriate to maximize long-term value creation and to strengthen company infrastructure and reach.

- **Excel in Sales & Marketing.** We aim to continue to expand our highly experienced commercial team into new territories as well as new markets when we have suitable products. We intend to build our existing businesses in PAH and lipid storage disorders whilst developing or licensing additional synergetic products.

- **Retain core values**. We constantly strive to strengthen our culture of Innovation, Trust and Teamwork and Open Communication to foster an enthusiastic and stimulating environment for employees. At the same time we maintain our Results Driven orientation.

ACTELION

## EMPLOYEES

| Total Employees Actelion Group | Mar 12 | Mar 11 |
|---|---|---|
| Drug Discovery | 404 | 397 |
| Clinical Development | 676 | 644 |
| Marketing & Sales | 1,040 | 1,028 |
| Support Functions | 442 | 420 |
| **Total** | **2,562** | **2,489** |

as of the end of March 2012

## MARKETED PRODUCTS

### TRACLEER®

Our lead product is Tracleer® (bosentan), a dual endothelin receptor antagonist. Tracleer® was the first oral treatment approved for pulmonary arterial hypertension (PAH), a rare, chronic, life-threatening disorder that severely compromises the functions of the lungs and heart.

Today, Tracleer® has been approved for the treatment of PAH in more than 60 countries, including the United States in November 2001, the European Union in May 2002 and Japan in April 2005. We currently market Tracleer® in all major markets worldwide including the United States, the European Union, Japan, Switzerland, Canada, Australia and China.

In addition to the indication in PAH, based on compelling clinical data, Tracleer® received approval from the European regulatory authorities in 2007 for the reduction in the number of new digital ulcers in patients suffering from systemic sclerosis and ongoing digital ulcer disease.

### VENTAVIS®

Ventavis® (iloprost) was approved by the FDA in the United States for the treatment of PAH at the end of 2004. Actelion gained the licensing rights to market Ventavis®, the first approved inhaled PAH therapy, in the United States through the acquisition of the US company CoTherix at the end of 2006. Ventavis® has a mode of action that perfectly complements Tracleer® and with its strong performance on the market, represents a substantial contribution to our PAH franchise.

### VELETRI®

Actelion's PAH franchise is further strengthend by Veletri® (Epoprostenol for Injection), an intravenous prostanoid vasodilator. Unlike other epoprostenol formulations approved for PAH, this formulation is stable at room temperature (77 F, 25 C), for up to 24 hours after it has been diluted and administered, making the use of frozen gel packs unnecessary.

Veletri® can be reconstituted with either Sterile Water for Injection, USP, or Sodium Chloride 0.9% Injection, USP, eliminating the need for drug-specific diluents.

Veletri® is approved by the U.S. Food and Drug Administration (FDA) for the treatment of pulmonary arterial hypertension (PAH) (WHO Group 1) to improve exercise capacity. Studies establishing effectiveness included predominantly patients with NYHA Functional Class III-IV symptoms and etiologies of idiopathic or heritable PAH or PAH associated with connective tissue diseases.

### ZAVESCA®

Zavesca® (miglustat) is currently the only approved oral treatment for patients with mild to moderate type 1 Gaucher Disease for whom enzyme replacement therapy is unsuitable. Type 1 Gaucher disease is a rare and debilitating metabolic disorder.

Zavesca® is also approved in the European Union for the treatment of progressive neurological manifestations in adult patients and pediatric patients with Niemann-Pick type C disease (NP-C). Zavesca® is the first treatment to be approved for patients with NP-C, a very rare, invariably progressive and eventually fatal neurodegenerative genetic disorder affecting both children and adults.

More information on our products can be found in Actelion's Marketed Products fact sheet.

## CLINICAL DEVELOPMENT

Delivering on our mission to bring innovative medicines to patients, the Actelion clinical development pipeline is comprised of innovative compounds, including three in late-stage development. The novel compounds address a broad range of diseases, including cardiovascular and immunological disorders as well as central nervous system disorders and infectious disease.

Actelion's late-stage product candidates include macitentan, a novel endothelin receptor antagonist and selexipag a first-in-class, orally active, non-prostanoid IP receptor agonist, both under investigation for pulmonary arterial hypertension.

More information on these and our other development activities can be found in Actelion's Clinical Development fact sheet.

## DRUG DISCOVERY

Actelion's efforts in drug discovery focus on developing platforms of expertise. This focus allows high productivity in the generation of innovative compounds potentially addressing a wide range of high unmet medical needs.

The first focus is the design, synthesis and optimization of small molecular weight drug-like molecules.

Actelion also focuses on the choice of its molecular target families. Initially, the company looked solely at G-protein coupled receptors (GPCRs) and a specific enzyme family known as aspartic proteinases. As the company's capabilities have expanded, so too have the target platforms to include anti-infectives, ion channels and a broad range of soluble enzymes.

More information on our platforms of expertise can be found in Actelion's Drug Discovery fact sheet.

## DEVELOPMENT PIPELINE – ACTELION'S FOCUS ON HIGH UNMET MEDICAL NEEDS

| Phase | Compound | Indication | Study | Results expected |
|---|---|---|---|---|
| IV | Bosentan | Combination bosentan and sildenafil in PAH | COMPASS-2 | 2013 |
| IV | Bosentan | Pediatric pulmonary arterial hypertension | FUTURE | 2014 |
| III | Macitentan | Pulmonary arterial hypertension | SERAPHIN | Complete |
| III | Selexipag | Pulmonary arterial hypertension | GRIPHON | 2014 |
| III | Macitentan | Digital ulcers associated with systemic sclerosis | - | 2014 |
| II | Cadazolid | *Clostridium difficile* infection | - | 2012 |
| II | Ponesimod | Multiple sclerosis | - | Complete |
| II | Ponesimod | Plaque psoriasis | - | H2 2012 |
| I | Anti-malarial | Malaria | - | - |
| I | Cardiovascular | Cardiovascular disorders | - | - |
| I | CRTH2 receptor antagonist | Asthma / Allergic disorders | - | - |
| I | Metabolic disease | Metabolic disease | - | - |
| I | Immunology | Immunological disorders | - | - |
| I | Macitentan | Glioblastoma | - | - |
| I | Orexin receptor antagonist | Insomnia | - | - |
| I | $S1P_1$ receptor agonist | Immunological disorders | - | - |

## ACTELION'S PARTNERSHIPS

In order to maximize the value of its innovative compounds, Actelion has entered into product-driven partnerships.

### ACTELION/GLAXOSMITHKLINE COLLABORATION

Actelion and GlaxoSmithKline (GSK) entered into an exclusive worldwide (excluding Japan) collaboration in July 2008 to jointly develop and commercialize orexin receptor antagonists. Both companies are working on the discovery and development of new orexin receptor antagonist therapies based on the orexin alliance.

### ACTELION/NIPPON SHINYAKU ALLIANCE

Actelion and Nippon Shinyaku entered into an exclusive worldwide alliance in April 2008 to collaborate on an orally-available long-acting non-prostanoid IP receptor agonist for patients suffering from pulmonary arterial hypertension (PAH). This compound was originally discovered and synthesized by Nippon Shinyaku.

### ACTELION/BAYER SCHERING PHARMA AG ALLIANCE

Actelion holds the exclusive US rights for inhaled iloprost, sold under the brand name Ventavis®, the first approved inhaled treatment for pulmonary arterial hypertension (PAH), licensed from Bayer Schering Pharma (through the acquisition of CoTherix Inc.).

### ACTELION/MERCK & CO., INC. ALLIANCE

Actelion and Merck & Co., Inc. formed an exclusive worldwide alliance in December 2003 to discover, develop and market new classes of orally available renin inhibitors, initially discovered by Actelion, for patients suffering from cardio-renal diseases.

## COMPANY MILESTONES

**2012** › Macitentan meets primary endpoint in pivotal Phase III SERAPHIN outcome study in patients with pulmonary arterial hypertension.

**2011** › Ponesimod meets primary endpoint in Phase II dose-finding study in patients with relapsing-remitting multiple sclerosis.

**2010** › Veletri® is launched in the US further strengthening Actelion's PAH franchise

**2009** › First patient enrolled in Phase III study in PAH with selexipag, a first-in-class orally active IP receptor agonist

› Dose-finding Phase IIb study inititated with selective $S1P_1$ receptor agonist in patients with multiple sclerosis

› Tracleer® receives EU approval of pediatric formulation for the treatment of PAH

› Zavesca® receives EU approval for Niemann-Pick type C disease

**2008** › Tracleer® receives EU approval for treatment of patients with mildly symptomatic PAH

› Actelion and GlaxoSmithKline enter into orexin receptor antagonist collaboration

› Actelion and Nippon Shinyaku enter into a license agreement on novel orally available IP receptor agonist for the treatment of PAH

**2007** › Tracleer® receives EU approval for reduction of number of new digital ulcerations in systemic sclerosis patients

**2006** › Definitive agreement to acquire US-based CoTherix, Inc. adding Ventavis® to Actelion's product offerings in the US

**2003** › Actelion acquires Axovan adding Clazosentan to the pipeline

› First approval of Zavesca® for the treatment of Type 1 Gaucher disease

**2001** › First approval of Tracleer® for the treatment of pulmonary arterial hypertension (PAH)

**2000** › Initial Public Offering (IPO); Actelion shares are listed on the Swiss New Market Stock Exchange with a record valuation of CHF 1.2 billion

**1997** › Foundation of Actelion December 17, 1997

Latest update: April 2012



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