

# ACTELION'S COMPANY PROFILE

Actelion Ltd. is a leading biopharmaceutical company focused on the discovery, development and commercialization of innovative drugs for diseases with significant 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 (ERA), leading to Tracleer® and now the tailored ERA Opsumit®.

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 distribution, sales and marketing services.

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## FINANCIAL OVERVIEW

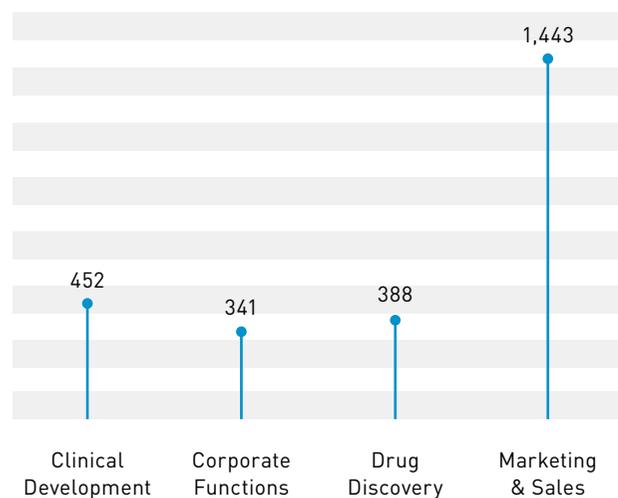
in CHF millions (except for per share data)	FY Results 2016	FY Results 2015	% Variance in CHF	% Variance at CER*
Product sales	2,412	2,042	18	15
Core operating expenses	1,420	1,288	16	14
Core operating income	992	814	22	17
Diluted Core EPS	8.18	6.16	33	27
Diluted US GAAP EPS	6.46	4.91	32	25
No of shares in calculation (million)	107.8	112.5	-	-

**Share information** IPO: 6 April 2000. Listed: SIX Swiss, Exchange (ATLN), Zürich, Switzerland Trading Symbols Reuters ATLN.S, Bloomberg ATLN.  
The full financial statements can be found on [www.actelion.com](http://www.actelion.com).

\*Constant Exchange Rates.

## EMPLOYEES

**Employees Actelion Group** **Total: 2,624**  
(December 2016)



## COMPANY STRATEGY

Since its founding more than fifteen years 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 promising pipeline, seven approved products, and commercial operations in over 30 countries. Actelion's more than 2,600 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:

- **Drive innovation forward.** Pursue top quality science, internally and externally, balanced with medical need and commercial potential.
- **Maximize the value of innovation.** Develop projects ourselves and seek partners or out-license when necessary to maximize value.
- **Leverage our global presence.** Expand innovative commercial capabilities to new customers and regions. Manage alliances, putting the product first.
- **Insist on the highest quality in all we do.** Quality is crucial and needs to be ingrained across all functions.

## MARKETED PRODUCTS

### Our PAH Franchise

Pulmonary arterial hypertension (PAH) is a chronic, life-threatening disorder characterized by abnormally high blood pressure in the arteries between the heart and lungs of an affected individual.

Actelion's PAH franchise encompasses oral, inhaled and intravenous formulations of compounds, for patients at various stages in the course of this disease (PAH Functional Classes II-IV), enabling us to deliver treatments across the entire continuum of care.

### Opsumit®

Opsumit (macitentan), an orally available endothelin receptor antagonist, resulted from a tailored drug discovery process in Actelion's laboratories.



Opsumit is commercially available in over 45 markets, including the US (since November 2013), Germany (since January 2014) and Japan (since June 2015). The registration process for other countries is ongoing.

### Tracleer®

Tracleer (bosentan), an orally available endothelin receptor antagonist, was the first oral treatment approved for PAH.



Tracleer is commercially available in over 60 markets, including the US (since November 2001), the European Union (since May 2002), and Japan (since April 2005).

In addition to the indication in PAH, Tracleer is approved in the EU for the reduction in the number of new digital ulcers in patients suffering from systemic sclerosis and ongoing digital ulcer disease.

### Uptravi®

Uptravi (selexipag), originally discovered and synthesized by Nippon Shinyaku, is the only approved oral, selective IP receptor agonist targeting the prostacyclin pathway in PAH.



Uptravi is commercially available in 9 countries including the US (since January 2016) and Germany (since June 2016). Market authorization has been received in Australia, Canada, the European Union, Japan, New Zealand, South Korea, Switzerland and the US. The registration process for other countries is ongoing.

### Veletri®

Veletri (epoprostenol for injection), an intravenous prostacyclin, is stable at room temperature (77°F/25°C) for up to 24 hours, removing the need for patients to carry ice packs.



Veletri is commercially available in 17 markets, including the US (since 2010), Switzerland and Canada, marketed as Caripul® (since 2012), Japan, marketed as Epoprostenol "ACT", and some European markets (since 2013). The registration process for other countries is ongoing.

### Ventavis®

Ventavis (iloprost), an inhaled formulation of iloprost, is a synthetic compound structurally similar to prostacyclin (PGI<sub>2</sub>).



Actelion has marketed Ventavis in the US since 2007. Bayer Healthcare markets Ventavis elsewhere.

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

## Our Specialty Products

Actelion is creating specialty franchises alongside PAH – discovering, developing and/or in-licensing/acquiring products in new therapeutic areas.

### Valchlor®

Valchlor (mechlorethamine) 0.016% gel is applied topically once daily to affected areas of the skin. Valchlor is currently only available in the US and is approved for the topical treatment of Stage IA and IB mycosis fungoides-type cutaneous T-cell lymphoma in patients who have received prior skin-directed therapy.



Valchlor is commercially available in the US (since November 2013) and Israel (since April 2016).

### Zavesca®

Zavesca (miglustat) available as oral capsules, is a glucosylceramide synthase inhibitor indicated as monotherapy for the treatment of adult patients with mild to moderate type I Gaucher disease (GD-1) for whom enzyme replacement therapy is not a therapeutic option.



Zavesca is commercially available for the treatment of GD-1 in 47 countries, including the US and the European Union (since 2003).

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

Zavesca is commercially available for the treatment of NP-C in 46 countries, including the European Union (since 2009) and Japan, marketed as Brazavesca® (since 2012).

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

## CLINICAL DEVELOPMENT

Actelion's promising development pipeline comprises novel compounds addressing 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: a novel antibiotic, cadazolid, under investigation for *Clostridium difficile*-associated diarrhea (CDAD) and a S1P<sub>1</sub> receptor modulator, ponesimod, investigated in multiple sclerosis.

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 the design, synthesis and optimization of small molecular weight molecules, which are active on molecular target families. This focus allows high productivity in the generation of innovative compounds potentially addressing a wide range of high unmet medical needs.

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, adding anti-infective's, 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

	Compound	Indication	Study	Status
Phase III	Cadazolid	<i>Clostridium difficile</i> -associated diarrhea	IMPACT	Ongoing
	Macitentan	Pediatric PAH	TOMORROW	Initiating
	Macitentan	Portopulmonary hypertension (PoPH)	PORTICO	Ongoing
	Macitentan	Fontan-palliated	RUBATO	Initiating
	Ponesimod	Multiple sclerosis	OPTIMUM	Ongoing
	Ponesimod	Multiple sclerosis	POINT	Ongoing
Phase II	Cenerimod	Systemic lupus erythematosus	-	Ongoing
	Clazosentan	Reversal of vasospasm associated with aneurysmal subarachnoid hemorrhage	REVERSE	Ongoing
	Dual Orexin Receptor Antagonist	Insomnia	-	Ongoing
	Endothelin Receptor Antagonist	Specialty cardiovascular disorders	-	Ongoing
	Macitentan	Chronic thromboembolic pulmonary hypertension	MERIT	Complete
Phase Ib	Lucerastat	Fabry disease	-	Complete
Phase I	New Chemical Entity	Cardiovascular disorders	-	Ongoing
	New Chemical Entity	Inflammatory disorders	-	Ongoing
	Selective Orexin 1 Receptor Antagonist	Neurological disorders	-	Ongoing
	T-type Calcium Channel Blocker	Neurological disorders	-	Ongoing

## ACTELION'S PARTNERSHIPS

Actelion has a dedicated team focused on identifying innovation from external sources that complements our business approach. Once identified, Actelion is rapid, proactive and open in creating benefits for both parties. We commit ourselves to the project we share with our partner, to make the product a global success.

### Actelion/ReveraGen

Actelion has obtained an exclusive option to in-license ReveraGen's lead compound vamorolone for the treatment of Duchenne Muscular Dystrophy at two different stages in its development.

### Actelion/Nippon Shinyaku Alliance

Actelion and Nippon Shinyaku entered into an exclusive worldwide alliance in April 2008 to collaborate on selexipag, the first selective oral prostacyclin 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.).

## COMPANY MILESTONES

- 2017** Actelion enters into a definitive transaction agreement with Johnson & Johnson
- 2016** Agreement on in-licensing option for vamorolone from ReveraGen
- Initiation of a Phase II program with Actelion's dual orexin receptor antagonist in insomnia
- Initiation of a Phase III program with macitentan in children with PAH
- Uptravi is launched in the US and in Germany
- Uptravi is approved by the European Medicines Agency for the treatment of PAH
- 2015** Uptravi is approved by the US FDA for the treatment of PAH
- Creation of Vaxxilon - together with the Max Planck Society
- Initiation of Phase III program with ponesimod in patients with relapsing multiple sclerosis
- 2014** Selexipag meets primary endpoint in pivotal Phase III GRIPHON outcome study in patients with PAH
- 2013** Initiation of Phase III program with cadazolid in patients with *Clostridium difficile*-associated diarrhea
- Valchlor is added to Actelion's specialty portfolio in the US
- Opsumit is approved in the US and the EU and launched in the US for PAH
- 2012** Macitentan meets primary endpoint in pivotal Phase III SERAPHIN outcome study in patients with pulmonary arterial hypertension
- 2010** Veletri is launched in the US further strengthening Actelion's PAH franchise
- 2009** 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 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** 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



**Disclaimer** This fact sheet has the sole purpose to provide members of the public with general information about the activities of Actelion Ltd and its associated companies. The forward-looking statements in this fact sheet are based on current expectations and belief of company management, which are subject to numerous risks and uncertainties.

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