ACTELION'S MARKETED PRODUCTS

BUSINESS STRATEGY & OPERATIONS

In an effort to optimize market and customer reach, Actelion has expanded and enhanced its commercial infrastructure and capabilities. By the end of 2013 the company employed over 1,000 sales, marketing and medical professionals based in over 30 Actelion operative affiliates and reaching more than 30 additional markets through partner arrangements. This global reach, means that Actelion is fully equipped to optimize returns from current opportunities, as well as launch and commercialize future assets.

Our commercial operations are aligned to:

- Focus on all of Actelion's opportunities and create accountability close to the customer.
- Allow scalability, from both organizational and managerial perspectives to be able to manage growth flexibly.
- Ensure an efficient and effective interaction across functions and with partners.

Business Strategy & Operations has highly experienced people with a proven track record in both specialty and GP markets to compete in an increasingly complex business environment. Together the group is now well placed to not only drive commercial excellence and leverage our unrivalled PAH leadership and orphan drug expertise, but also lead transformational growth initiatives and shape markets and medical utility for the potential which lies ahead.

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) is an orally available endothelin receptor antagonist (ERA) that resulted from a tailored drug discovery process with the target of developing an ERA to address efficacy and safety.

CURRENT INDICATIONS

In the United States, Opsumit is indicated for the treatment of pulmonary arterial hypertension (PAH, WHO Group I) to delay disease progression. Disease progression included: death, initiation of intravenous (IV) or subcutaneous prostanoids, or clinical worsening of PAH (decreased 6-minute walk distance, worsened PAH symptoms and need for additional PAH treatment). Opsumit also reduced hospitalization for PAH.

Effectiveness was established in a long-term study in PAH patients with predominantly WHO Functional Class II-III symptoms treated for an average of 2 years. Patients were treated with Opsumit monotherapy or in combination with phosphodiesterase-5 inhibitors or inhaled prostanoids. Patients had idiopathic and heritable PAH (57%), PAH caused by connective tissue disorders (31%), and PAH caused by congenital heart disease with repaired shunts (8%).

PRODUCT AVAILABILITY & REGULATORY STATUS

The first approval globally of the new drug application for Opsumit (macitentan) was issued by the US Food and Drug Administration (FDA) on 18 October 2013 for the treatment of pulmonary arterial hypertension. Opsumit is also approved for PAH in Australia, Canada, and Switzerland. Regulatory reviews in other countries are ongoing.

For current information please visit our www.actelion.com.

AVAILABLE CLINICAL DATA

ABOUT THE SERAPHIN STUDY

SERAPHIN (Study with an Endothelin Receptor Antagonist in Pulmonary arterial Hypertension to Improve cliNical outcome) was the largest and longest randomized, controlled study in PAH patients to include a clearly defined morbidity/mortality primary endpoint . The pivotal Phase III study was designed to evaluate the efficacy and safety of macitentan - a novel dual endothelin receptor antagonist that resulted from a tailored drug discovery process - through the primary endpoint of time to first morbidity and all-cause mortality event in patients with symptomatic PAH.

Global enrollment was completed in December 2009 with a total of 742 patients. Patients were randomized 1:1:1 to receive two different doses of macitentan (3 mg and 10 mg once daily) or placebo. Patients were allowed to receive PAH background therapy throughout the study, either PDE-5 inhibitors or oral/inhaled prostanoids. This event-driven study was conducted in 151 centers from almost 40 countries in North America, Latin America, Europe, Asia-Pacific and Africa and was completed in the first half of 2012, with 287 patients having an adjudicated event.

ABOUT SERAPHIN STUDY DATA

Patients were randomized to placebo (n=250), macitentan 3 mg (n=250), or macitentan 10 mg (n=242). The primary end point occurred in 46.4%, 38.0%, and 31.4% of the patients in these groups, respectively. The hazard ratio for macitentan 3 mg versus placebo was 0.70 (97.5% CI, 0.52 to 0.96; p=0.0108) and the hazard ratio for macitentan 10 mg versus placebo was 0.55 (97.5% CI, 0.39 to 0.76; p<0.0001). Worsening of pulmonary arterial hypertension was the most frequent primary end point event. The effect of macitentan on this endpoint was observed irrespective of background therapy for pulmonary arterial hypertension.

MILESTONES

2013 › EMA grants market authorization for Opsumit in PAH
2013 › US FDA approval of Opsumit in PAH
2012 › SERAPHIN outcome study meets its primary endpoint
2007 › Initiation of Phase III SERAPHIN study in PAH patients
2005 › Initiation of Phase II dose ranging study
2004 › Entry-into-man
2003 › Selection of macitentan for initiation of preclinical studies

KEY SCIENTIFIC LITERATURE

› Pulido T et al. N Engl J Med 2013;369:809-18.
› Gatfield J et al. PloS ONE 2012;7(10):e47662.
› Iglarz M et al. J Pharmacol Exp Ther. 2008;327(3):736-45.
› Iglarz M et al. Am J Respir Crit Care Med 011;183:A6445.
› Sidharta PN et al. Eur J Clin Pharmacol 2011;67(10):977-84.
› Bruderer S et al. AAPS J 2011 ;14:68-78.
› Bruderer S et al. Xenobiotica 2012; 42:901-10

TRACLEER®

Actelion's lead product, Tracleer® (bosentan) – an endothelin receptor antagonist – was the first oral treatment approved for PAH.

CURRENT INDICATIONS

In the United States, Tracleer is indicated for the treatment of pulmonary arterial hypertension (PAH) (WHO Group 1) to improve exercise ability and to decrease clinical worsening. Studies establishing effectiveness included predominantly patients with NYHA Functional Class II-IV symptoms and etiologies of idiopathic or heritable PAH (60%), PAH associated with connective tissue diseases (21%), and PAH associated with congenital systemic-to-pulmonary shunts (18%). **Considerations for use:** Patients with WHO class II symptoms showed reduction in the rate of clinical deterioration and a trend for improvement in walk distance. Physicians should consider whether these benefits are sufficient to offset the risk of liver injury in WHO class II patients, which may preclude future use as their disease progresses.

In Europe, Tracleer is approved for treatment of PAH Functional Class III to improve exercise capacity and symptoms, as well as PAH Functional Class II, where some improvements have also been shown. In the EU, Tracleer is also indicated to reduce the number of new digital ulcers in patients with systemic sclerosis and ongoing digital ulcer disease.

Regulatory review and approval for the inclusion of Functional Class II in the Tracleer label is ongoing in additional markets.

Additionally, a quadrisect, dispersible 32mg tablet formulation of Tracleer has been approved in the EU for the treatment of PAH in children aged from 2 years.

PRODUCT AVAILABILITY & REGULATORY STATUS

Tracleer is approved for the treatment of PAH in over 60 countries, including the United States since November 2001, the European Union in May 2002, and Japan in April 2005. For current information please visit our corporate website.

ABOUT DIGITAL ULCERS IN SYSTEMIC SCLEROSIS

For information on digital ulcers in systemic sclerosis (DU), diagnosis of DU and treatment of DU please visit www.actelion.com.

AVAILABLE CLINICAL DATA

A comprehensive clinical trial program has been conducted to evaluate the efficacy and safety of Tracleer across a broad range of PAH patient populations.

For a detailed analysis of the study results please refer to the scientific publications - reference information is given in the key scientific literature section.

The results of clinical studies including two pivotal randomized controlled studies, **Study 351** and **BREATHE-1**, demonstrate the efficacy of Tracleer in the treatment of patients with idiopathic PAH (where no specific cause can be identified), or PAH secondary to connective tissue diseases such as scleroderma.

The combination of Tracleer with the initiation of intravenous therapy with epoprostenol in adult patients suffering from PAH is well tolerated, as shown in the randomized controlled **BREATHE-2** study.

The **BREATHE-3** open-label study provided safety and efficacy data in children with PAH treated with Tracleer with or without concomitant prostanoid therapy. It also provided important information on the dose required in the pediatric formulation.

Results of the open-label **BREATHE-4** study in patients whose PAH is related to their infection with the human immuno deficiency virus (HIV) showed improvement in exercise capacity, WHO functional class, and quality of life, as well as cardiopulmonary hemodynamics, compared to baseline after 16 weeks of treatment with Tracleer.

The first ever randomized placebo-controlled study in patients with Eisenmenger's syndrome (PAH associated with a congenital heart defect) **BREATHE-5** showed that Tracleer decreases pulmonary vascular resistance and improves exercise capacity in these patients.

FUTURE-1 (Pediatric FormUlation of bosenTan in pUlmonary arterial hypeRtEnsion) an open-label study, evaluated the safety and pharmacokinetics of a new dispersible tablet formulation of Tracleer. This study provided important pharmacokinetic and dosing information using the new pediatric formulation of Tracleer. In **FUTURE-1**, the observed exposure to Tracleer was similar to that in children who participated in **BREATHE-3**.

FUTURE-2, an open-label safety extension study, is ongoing to assess long-term safety and outcome data.

The **EARLY** (Endothelin Antagonist tRial in miLdlY symptomatic PAH patients) study was a randomized, double-blind, placebo-controlled trial and the only randomized controlled trial to study a dedicated early-stage, or WHO Functional Class II, PAH population. Patients were followed for at least 6 months and results showed a significant reduction in pulmonary vascular resistance and a delay in time to clinical worsening. A trend towards improvement in exercise capacity was observed.

The **RAPIDS** (RAndomized Placebo-controlled Investigation of Digital ulcers in Scleroderma) program, consisting of two Phase III clinical trials (RAPIDS-1 and RAPIDS-2), tested the benefits of Tracleer in ischemic digital ulcers secondary to systemic sclerosis. In both studies, Tracleer reduced the number of new digital ulcers.

TRACLEER (BOSENTAN) STUDY OVERVIEW

Study name	Target patient population	Main result
PAH		
Study 351	Patients with idiopathic PAH and PAH related to connective tissue disease	Tracleer improved exercise capacity, symptoms, cardiopulmonary hemodynamics, and was associated with the reduction in the rate of clinical worsening
BREATHE-1	Patients with idiopathic PAH and PAH related to connective tissue disease	Tracleer improved exercise capacity, symptoms, and was associated with the reduction in the rate of clinical worsening
BREATHE-2 (not in USPI)	Patients with idiopathic PAH and PAH related to connective tissue disease and initiated on IV epoprostenol therapy	Combination of bosentan and IV epoprostenol
BREATHE-4	Patients with PAH related to HIV	Tracleer improved exercise capacity
BREATHE-5	Patients with severe PAH related to congenital heart disease (Eisenmenger's syndrome)	Tracleer improved cardiopulmonary hemodynamics and improved exercise capacity
EARLY	PAH patients in WHO Functional Class II (mildly symptomatic disease)	Tracleer improved cardiac hemodynamics and was associated with a reduction in the rate of clinical worsening. A trend towards an improvement in exercise capacity was observed
Pediatric program		
BREATHE-3	Children with PAH	Defined the pharmacokinetic profile of Tracleer in children
FUTURE-1	Children with PAH	Observed exposure to pediatric formulation of Tracleer was similar to that in children who participated in BREATHE-3
FUTURE-2	Children with PAH	Open-label safety extension study ongoing
SSC - DU		
RAPIDS-1	Patients with digital ulcers secondary to systemic sclerosis	Tracleer was shown to reduce the number of new digital ulcers
RAPIDS-2	Patients with digital ulcers secondary to systemic sclerosis	Tracleer was shown to reduce the number of new digital ulcers

MILESTONES

2009 › Tracleer indication extended in US to include WHO Functional Class II for the treatment of PAH
› Tracleer receives EU approval of pediatric formulation for the treatment of PAH
2008 › Tracleer indication extended in EU to include WHO Functional Class II for the treatment of PAH
2007 › Tracleer indication extended in EU to include reduction of new digital ulcers in systemic sclerosis
2006 › Tracleer indication extended in EU to include Eisenmenger physiology
› Tracleer launched in Brazil, China and South Korea
2005 › Tracleer launched in Japan
2003 › BREATHE-3 combination study presented
› BREATHE-4 PAH-HIV study presented
2002 › Tracleer launched in EU and Switzerland
2001 › Tracleer launched in US and Canada
2000 › Orphan status granted for Tracleer® in PAH in US
1999 › Tracleer PAH clinical development program initiated

KEY SCIENTIFIC LITERATURE

Study 351 Channick RN, Simonneau G, Sitbon O, et al. Lancet 2001;358:1119-23.

BREATHE-1 Rubin LJ, Badesch DB, Barst RJ, et al. N Engl J Med 2002;346:896-903.

BREATHE-2 Humbert M, Barst RJ, Robbins IM, et al. Eur Respir J 2004;24:353-9.

BREATHE-3 Barst RJ, Ivy D, Dingemanse J, et al. Clin Pharmacol Ther 2003;73:372-82.

BREATHE-4 Sitbon O, Gressin V, Speich R, et al. Am J Respir Crit Care Med 2004;170:1212-17.

BREATHE-5 Galiè N, Beghetti M, Gatzoulis MA, et al. Circulation 2006;114:48-54.

EARLY Galiè N, Rubin LJ, Hoeper MM, et al. Lancet 2008;371:2093-100.

RAPIDS-1 Korn JH, Mayes M, Matucci Cerinic M, et al. Arthritis Rheum 2004;50:3985-93.

RAPIDS-2 Seibold JR, Denton CP, Furst DE et al. [abstract]. ACR; San Diego, USA; 2005.

FUTURE-1 Beghetti M, Haworth SG, et al. Br J Clin Pharmacol 2009;68(6):948-55

VELETRI®

Veletri® (Epoprostenol for Injection) is an intravenous prostacyclin. Unlike other epoprostenol formulations approved for PAH, this formulation is stable at room temperature (77 F, 25 C).

Regardless of concentration Veletri is stable at room temperature for up to 48 hours immediately upon reconstitution and dilution, or for up to 24 hours after refrigerated storage (2-8 C) for up to 8 days, making the use of frozen gel packs unnecessary.

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

CURRENT INDICATIONS

In the US, Veletri (Epoprostenol for Injection) is indicated 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.

In the EU (NL and UK), Veletri is indicated for the treatment of pulmonary arterial hypertension (PAH) (idiopathic or heritable PAH and PAH associated with connective tissue diseases) in patients with WHO Functional Class III–IV symptoms to improve exercise capacity. Veletri is also indicated for use in haemodialysis in emergency situations when use of heparin carries a high risk of causing or exacerbating bleeding or when heparin is otherwise contraindicated.

PRODUCT AVAILABILITY & REGULATORY STATUS

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

For current information please visit our corporate website.

MILESTONES

2013 › Epoprostenol "ACT" approved in Japan
2012 › Approved in Canada (trade name Caripul)
2012 › Veletri approved in Switzerland
2010 › Veletri launched in US
2009 › Actelion acquired Veletri from GeneraMedix Inc.
2008 › FDA approved Veletri for treatment of primary pulmonary hypertension in the US

KEY SCIENTIFIC LITERATURE

Veletri (Epoprostenol for Injection) pharmacologic data:

› Nicolas LB, et al. Clin Ther. 2013;35:440-49.

Veletri (Epoprostenol for Injection) product technical data:

› Lambert O et al. Drug Design Dev Ther. 2012;6:235-244

Veletri (Epoprostenol for Injection) clinical data:

› Sitbon O et al. Am J Respir Crit Care Med. 2012;185:A2500.
› Tamura et al. Adv Ther 2013;30:459-71

VENTAVIS®

Ventavis® (iloprost) is an inhaled synthetic analog of prostacyclin (PGI2). Prostacyclin functions as a hormone, binding to receptors on smooth muscle cells, thereby affecting their function. Prostacyclin has multiple physiological effects, including vasodilation, inhibition of platelet aggregation, antiproliferation, anti-inflammation, and enhanced cardiac contractility. Ventavis is an inhaled synthetic prostacyclin which has been shown to:

- Significantly increase (p = 0.0033) patient improvement after 12 weeks of treatment compared to baseline on a composite endpoint of improved exercise capacity 30 minutes after dosing, improvement of at least one NHYA class and no clinical deterioration.
- Significantly improve 6-minute walk distance at week 12 with a 10% or greater increase in individual walk distance (p < 0.01).
- Significantly improve patients' functional class at week 12 (p = 0.03).

For patients with PAH (WHO Group 1) with NHYA Class III or IV symptoms.

CURRENT INDICATIONS

Ventavis is indicated for the treatment of pulmonary arterial hypertension (PAH) (WHO Group 1) to improve a composite endpoint consisting of exercise tolerance, symptoms (NYHA Class), and lack of deterioration. Studies establishing effectiveness included predominately patients with NYHA Functional Class III-IV symptoms and etiologies of idiopathic or heritable PAH (65%) or PAH associated with connective tissue diseases (23%).

PRODUCT AVAILABILITY & REGULATORY STATUS

In January 2007, Actelion announced the successful completion of its cash tender offer for shares of CoTherix, Inc., thereby strengthening its PAH franchise by adding Ventavis to its product offerings in the United States. Bayer Schering Pharma currently markets Ventavis as the first inhaled prostacyclin in countries outside the US.

AVAILABLE CLINICAL DATA

In controlled clinical trials, Ventavis improved a composite endpoint consisting of exercise tolerance, symptoms (NYHA Class), and the absence of clinical deterioration.

In December 2006, data from the Phase II/III clinical trial STEP, evaluating the safety and added benefit of using Ventavis (iloprost) inhalation solution therapy in patients with PAH already undergoing treatment with bosentan, were published. The analysis of this study showed that the combination of Ventavis added to bosentan therapy was well tolerated, and was consistent with the safety profile observed in patients receiving only iloprost.

MILESTONES

2009 › Ventavis receives US approval for increased 20 mcg/ml strength formulation

2007 › Actelion acquired CoTherix Inc, adding Ventavis to its product offerings

2004 › FDA approved inhaled iloprost for treatment of PAH in the US

KEY SCIENTIFIC LITERATURE

Ivy et al. J Am Coll Cardiol. 51(2):161-9; 2008.

McLaughlin et al. Am J Respir Crit Care Med. 174(11):1257-63; 2006.

Hoeper et al. Eur Respir J. 26(5):858-63; 2005.

Hossein A. et al. J Am Coll Card. 42 (1): 158-64; 2003.

Olschewski et al. N Engl J Med. 1;347(5):322-9; 2002.

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)gel 0.016%

Valchlor™ (mechlorethamine) gel 0.016% gel is applied topically once-a-day and dries on the skin. The availability of Valchlor allows US physicians to treat mycosis fungoides type CTCL with an US Food and Drug Administration (FDA) approved formulation of topical mechlorethamine. In addition to consistent, controlled manufacturing processes, Valchlor will be provided with labeling that includes data and instructions for correct use.

Mechlorethamine is a chemotherapeutic agent previously approved for intravenous treatment of mycosis fungoides, the most common type of Cutaneous T-Cell Lymphoma (CTCL). Topical mechlorethamine preparations are currently recommended for the treatment of early stage CTCL by the National Comprehensive Cancer Network (NCCN). Valchlor is the first and only FDA-approved topical formulation of mechlorethamine.

In September 2013, Actelion that it has concluded the acquisition of Ceptaris Therapeutics, Inc. adding Valchlor to Actelion's product portfolio

CURRENT INDICATIONS

In the United States, Valchlor (mechlorethamine) gel 0.016% is indicated for the topical treatment of stage IA and IB mycosis fungoides-type cutaneous T-cell lymphoma (MF-CTCL) in patients who have received prior skin-directed therapy.

PRODUCT AVAILABILITY & REGULATORY STATUS

Following US Food and Drug Administration (FDA) approval in August 2013, Actelion made Valchlor available in the US in November 2013.

Actelion is preparing the registration process for other countries.

ABOUT MYCOSIS FUNGOIDES

For information on mycosis fungoides (MF), diagnosis of MF and treatment of MF please visit www.actelion.com

MILESTONES

2013
- Valchlor launched in the US
- Actelion acquires Ceptaris Therapeutics, Inc.
- Valchlor approved in the US

KEY SCIENTIFIC LITERATURE

- Lessin SR, et al. JAMA Dermatol. 2013;149(1):25-32.

ZAVESCA®

Zavesca (miglustat) is a low-molecular-weight inhibitor which competitively and reversibly inhibits glucosylceramide synthase. With its unique physico-chemical properties, miglustat exhibits a large volume of distribution and has the capacity to access deep organs such as bone, brain and lung.

CURRENT INDICATIONS

Zavesca (miglustat) is 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 Niemann-Pick type C disease, a very rare, invariably progressive and eventually fatal neurodegenerative genetic disorder affecting both children and adults.

Zavesca is the first and only oral medication approved for the oral treatment of adult patients with mild to moderate type 1 Gaucher disease, and it may only be used in those patients for whom enzyme replacement therapy is unsuitable.

PRODUCT AVAILABILITY & REGULATORY STATUS

Zavesca is approved for the treatment of Niemann-Pick type C disease in 43 countries, including the European Union since 2009 and Japan since 2012.

Zavesca is approved for the treatment of mild to moderate type 1 Gaucher disease in 43 countries, including the United States and the European Union since 2003.

For full availability listing please visit our corporate website.

ABOUT NIEMANN-PICK TYPE C DISEASE

For information on Niemann-Pick type C disease, diagnosis of NP-C and treatment of NP-C please visit www.actelion.com

ABOUT TYPE 1 GAUCHER DISEASE

For information on Type 1 Gaucher Disease diagnosis of GD1 and treatment of GD1 please visit www.actelion.com

AVAILABLE CLINICAL DATA

In order to gain approval for Zavesca in Niemann-Pick type C disease, a set of clinical data were obtained from one clinical trial OGT918-007 and two multicenter retrospective cohort studies in patients with NP-C. In both the clinical trial and the case series, miglustat was associated with clinically relevant stabilization or improvement in neurological manifestations of the disease. For more information on these studies please visit our corporate website.

Zavesca (miglustat) 100 mg is the only oral drug available for the treatment of type 1 Gaucher disease, and was approved on the basis of three international open-label clinical trials. The rationale for the use of miglustat in type 1 Gaucher disease is to help balance the overall level of glucosylceramide by reducing its production to a level compatible with breakdown by residual glucocerebrosidase activity, a unique mode of action known as "substrate reduction therapy". Results from a pooled analysis of the three open-label clinical trials have shown that Zavesca monotherapy may reduce the incidence of bone crisis and improve bone mineral density in type 1 Gaucher disease patients, including those with a history of splenectomy and/or osteoporosis.

MILESTONES

2012 ›Japanese Health Authority grants approval for miglustat for the treatment of Niemann-Pick type C disease

2009 › Zavesca receives EU approval for Niemann-Pick type C disease

2008 › EU approval for a type II variation for Zavesca and bone disease in type 1 Gaucher disease
› Zavesca approved in Turkey

2007 › Zavesca approved and launched in Australia and Brazil

2005 › Zavesca approved and launched in Canada

2004 › Zavesca launched in the US; approved and launched in Switzerland

2003 › Zavesca approved and launched in the EU; approved in the US

2002 › Zavesca in-licensed; marketing authorization granted by European Commission

KEY SCIENTIFIC LITERATURE

IN NIEMANN-PICK TYPE C DISEASE

› Wraith JE, et al. Mol Genet Metab. 2010; 99:351-357.
› Patterson MC, et al. J Child Neurol. 2010 ;25(3):300-5.
› Pineda M, et al. Mol Genet Metab. 2009; 98:243-9.
› Patterson MC, et al. Lancet Neurol 2007; 6:765-772.

IN TYPE 1 GAUCHER DISEASE

› Pastores G.M. et al. Clinical Therapeutics. 29: 1645-53; 2007.
› Elstein D. et al. Blood. 110: 2296-2301; 2007.
› Giraldo P. et al. Haematologica. 91:125-8; 2006.
› Elstein D. et al. J Inherit Metab Dis 27: 757-66; 2004.

Latest Update: February 2014

Actelion Pharmaceuticals 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 headquartered in Allschwil/Basel, Switzerland and is quoted on the SIX Swiss Exchange (tickersymbol: ATLN). All trademarks are legally protected.

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