

ACTELION'S CLINICAL DEVELOPMENT

DEVELOPMENT PROCESS

Actelion's focus of bringing innovative medicines to patients in areas with high unmet medical needs can only be realized with rigorous testing of the compounds in its pipeline and thorough analysis and interpretation of the data.

Our clinical development department aims to fully explore and comprehensively describe the benefits of our compounds for patients. At the same time, we continuously assess and monitor the potential risks of new drug candidates. The group works to efficiently develop and bring innovative pharmaceutical products with an excellent benefit-risk profile to patients worldwide.

Actelion's clinical and pharmacological research comprises multiple functions: clinical science, pharmacology, biostatistics and data management, drug safety, drug regulatory affairs, life cycle management, and clinical operations functions.

Life cycle teams, formed from representatives of preclinical and clinical development functions, technical operations and business strategy and operations, ensure an efficient development of a new medicine. They steer the compounds from the definition of a target profile and entry-into-human studies through to submission of the dossier to health authorities and commercialization until loss of exclusivity of the medicine in the major markets and beyond. They also ensure that all appropriate measures are undertaken to optimize the value creation potential of each product. The collection of innovative compounds in Actelion's pipeline, in combination with each compound's different phase of clinical development and the medicine's stage of commercialization, makes this work highly diverse and demanding, yet it also satisfies our expectations for speed and cost-effectiveness in bringing new therapies to the patient.

Actelion's clinical development functions collectively manage clinical programs to the appropriate scientific, medical and operational standards to generate the information required by health authorities worldwide.

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DEVELOPMENT PIPELINE

	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 (ACT-132577)	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

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CADAZOLID

Cadazolid, a novel antibiotic, is a strong inhibitor of *Clostridium difficile* protein synthesis, leading to potent suppression of toxin and spore formation.

In preclinical studies cadazolid showed potent in vitro activity against *Clostridium difficile* isolates and a low propensity for resistance development. In a human gut model of *Clostridium difficile*-associated diarrhea (CDAD), cadazolid had a very limited impact on the normal gut microflora.

Cadazolid absorption is negligible resulting in high gut lumen concentrations and low systemic exposure, even in severe cases of CDAD where the gut wall can be more damaged and drug absorption potentially increased.

The US FDA has designated cadazolid as both a Qualified Infectious Disease Product (QIDP) and a Fast Track development program for the treatment of CDAD.



CURRENT STATUS

Cadazolid is evaluated in the IMPACT program (International Multi-center Program Assessing Cadazolid Treatment in patients suffering from ***Clostridium difficile*-associated diarrhea**). The program comprises two Phase III studies comparing the efficacy and safety of cadazolid (250 mg administered orally twice daily for 10 days) versus vancomycin (125 mg administered orally four times daily for 10 days).

The IMPACT program is designed to determine whether the clinical response after administration of cadazolid is non-inferior to vancomycin in subjects with CDAD, and whether administration of cadazolid is superior to vancomycin in the sustained clinical response. The program commenced enrollment in the fourth quarter 2013 with a target of approximately 1'260 subjects worldwide, and results are expected in mid-2017.

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AVAILABLE CLINICAL DATA

Cadazolid was studied in a Phase II multi-center, double-blind, randomized, active reference, parallel group, therapeutic exploratory study. The study evaluated the efficacy, safety and tolerability of a 10-day, twice daily oral administration of 3 doses (250 mg, 500 mg or 1,000 mg b.i.d.) of cadazolid in subjects with CDAD. As the current standard of care for CDAD, oral vancomycin (125 mg qid for 10 days) was used as the active reference. The study was completed in December of 2012, after having enrolled 84 subjects with CDAD. The results of the Phase II study indicate that the effect of all doses of cadazolid were numerically similar to, or better than vancomycin on key endpoints including CDAD clinical cure rates as well as sustained cure rates. Clinical cure rate was defined as the resolution of diarrhea and no further need for CDAD therapy at test-of-cure 24 to 72 hours after the last dose of treatment, while sustained cure rate was defined as clinical cure with no recurrence of CDAD up to 4 weeks post-treatment. Recurrence rates were numerically lower for all doses of cadazolid as compared to vancomycin. Cadazolid was safe and well tolerated.

MILESTONES

- 2014** Cadazolid receives US FDA QIDP & Fast Track designation for development in CDAD
- 2013** Initiation of Phase III development
- 2012** Completion of Phase II development

KEY SCIENTIFIC LITERATURE

- T. Louie et al. *Antimicrob. Agents Chemother.* 2015; 59(10):6266-73
- D.N. Gerding et al., *J. Antimicrob. Chemother.* 2016; 71(1):213-9.

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MACITENTAN

Macitentan is an orally available endothelin receptor antagonist (ERA) that resulted from a tailored drug discovery process in Actelion's laboratories.

Macitentan (Opsumit®) is currently approved for the treatment of pulmonary arterial hypertension (PAH), a chronic, life-threatening disorder which severely compromises the function of the lungs and heart. The product is commercially available in over 35 markets, including the US (since November 2013), Germany (since January 2014) and Japan (since June 2015). The registration process for other countries is ongoing.



CURRENT STATUS

Macitentan is currently being further evaluated in multiple studies to expanding the clinical utility of this important product in PAH and beyond.

MERIT (Macitentan in the tReatment of Inoperable chronic Thromboembolic pulmonary hypertension) was a Phase II prospective, randomized, placebo-controlled, double-blind, multi-center, parallel-group study to assess

the efficacy, safety and tolerability of 10 mg macitentan in patients with **inoperable chronic thromboembolic pulmonary hypertension (CTEPH)**.

In MERIT, 80 inoperable patients were randomized in a 1:1 ratio into 2 treatment groups (macitentan 10 mg or placebo) over a 24 week treatment period. The study started in August 2014 and was completed in September 2016.

After 16 weeks the treatment effect was a significant 16% reduction in pulmonary vascular resistance (PVR) with macitentan compared with placebo (95% CL: -30%, -1%; p=0.04 intention-to-treat (ITT)). The efficacy observed was consistent across all sub-groups, included patients receiving background PH specific therapy at baseline (61%), including PDE-5 inhibitors (59%). Mean PVR decreased from baseline in both macitentan and placebo groups (geometric mean percent ratios of Week 16/baseline 73.0% and 87.2%, respectively).

The study also showed a significant positive effect of macitentan compared to placebo on exercise capacity. After 24 weeks of treatment, the mean change in 6-minute walk distance (6-MWD) from baseline was an increase of 35 meters (m) in macitentan and 1 m in placebo. The 6-MWD least-squares mean difference at Week 24 was 34.0 meters between macitentan and placebo (95% CL: 2.9, 65.2 m; p=0.03).

Macitentan was well tolerated in this patient population and safety was in general consistent with the known safety profile for macitentan from previous clinical studies. The most frequently reported adverse events that occurred with higher frequency on macitentan vs. placebo were peripheral edema (22.5% vs. 10.0%) and events related to anemia (17.5% vs. 2.5%). Hemoglobin decreases were observed in both macitentan and placebo groups and in only one subject in each group hemoglobin values decreased below 100 g/L during the study.

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TOMORROW (pediatric use of Macitentan to delay disease progression in PAH Worldwide) is a multicenter, controlled, randomized, open-label event-driven study to assess the efficacy, safety and pharmacokinetics of a pediatric formulation of macitentan versus standard of care in **children with PAH**. The study will enroll children between the age of 1 month and 18 years in more than 20 countries and is expected to last up to 6 years.

PORTICO (Portopulmonary Hypertension Treatment with macitentan — a randomized Clinical Trial) is a randomized, double-blind, placebo-controlled, prospective, multicenter, parallel group Phase IV study to assess the safety and efficacy of macitentan in **patients with portopulmonary hypertension (PoPH)**. The primary objective of the study is to evaluate the effect of 10 mg macitentan on pulmonary vascular resistance (PVR) as compared to placebo. Secondary objectives include the evaluation of the effect of macitentan as compared to placebo on exercise capacity and WHO functional class, as well as the evaluation of the safety and tolerability of macitentan in patients with PoPH.

RUBATO is a Phase III prospective, multi-center, double-blind, placebo-controlled parallel group study to assess the efficacy and safety of macitentan in **stable Fontan-palliated adolescents and adults**. The primary objective is to assess the effect of macitentan 10 mg as compared to placebo on exercise capacity through cardiopulmonary exercise testing (peak VO₂). Secondary objectives include the evaluation of the safety and tolerability of macitentan as compared to placebo in stable Fontan-palliated patients. The duration of the study is expected to be approximately 28 months; the start is planned for mid-2017.

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AVAILABLE CLINICAL DATA

Macitentan has been studied in SERAPHIN, a multicenter, double-blind, placebo-controlled, parallel-group, event-driven, Phase III outcome study in 742 patients with symptomatic PAH, who were randomized to three treatment groups (placebo [N = 250], 3 mg [N = 250] or 10 mg [N = 242] of macitentan once daily), to assess the long-term effect on morbidity or mortality.

Treatment with macitentan 10 mg resulted in a 45% risk reduction (hazard ratio [HR] 0.55; 97.5% CI: 0.39 to 0.76; logrank $p < 0.0001$) of the composite morbidity-mortality endpoint up to end of treatment (EOT) when compared to placebo. The treatment effect was established early and was sustained. In patients already on background therapy for PAH treatment with macitentan 10 mg resulted in a 38% risk reduction (HR 0.62; 97.5% CI: 0.43 to 0.8; logrank $p = 0.0094$) of the composite morbidity-mortality endpoint compared to placebo. The risk of PAH related death or hospitalization for PAH up to EOT was reduced by 50% (HR 0.50; 97.5% CI: 0.34 to 0.75; logrank $p < 0.0001$) in patients receiving macitentan 10 mg (50 events) compared to placebo.

Efficacy of macitentan 10 mg on the primary endpoint was consistent across subgroups of age, sex, ethnic origin, geographical region, aetiology, by monotherapy or in combination with another PAH therapy and by WHO FC (I/II and III/IV).

The most commonly reported adverse drug reactions are nasopharyngitis (14.0%), headache (13.6%) and anaemia (13.2%, see section 4.4). The majority of adverse reactions are mild to moderate in intensity.

MILESTONES

- 2013** Opsumit approved by the US FDA and the EU Commission
- 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.
- Iglarz M. et al. J Pharmacol Exp Ther. 2008; 327(3):736-45.
- Iglarz M et al. Am J Respir Crit Care Med 2011; 183:A6445.
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PONESIMOD

Ponesimod is a potent orally active, selective sphingosine-1-phosphate receptor 1 (S1P₁) immunomodulator.

Ponesimod prevents lymphocytes from leaving lymph nodes, thereby reducing circulating blood lymphocyte counts and preventing infiltration of lymphocytes into target tissues. The lymphocyte count reduction is rapid, dose-dependent, sustained upon continued dosing, and quickly reversible upon discontinuation. Initial data suggest that ponesimod does not cause lymphotoxicity by destroying/depleting lymphocytes or interfering with their cellular function. Other blood cells e.g. cells of the innate immune system are largely unaffected. Ponesimod is therefore considered a promising new oral agent for the treatment of a variety of autoimmune disorders.



CURRENT STATUS

OPTIMUM (Oral Ponesimod versus Teriflunomide In relapsing Multiple sclerosis) is a Phase III multi-center, randomized, double-blind, parallel-group, active-controlled superiority study to compare the efficacy and safety of ponesimod to teriflunomide (Aubagio®) in patients with **relapsing multiple sclerosis (RMS)**. The study aims to determine whether ponesimod is more efficacious than teriflunomide in reducing relapses. The study is expected to enroll approximately 1'100 patients, randomized in 2 groups in a 1:1 ratio to receive ponesimod 20 mg/day or teriflunomide 14 mg/day, and is expected to last a little over 3 years.

POINT (POnesimod aNd Tecfidera) is a prospective, multicenter, randomized, double-blind, parallel group, add-on, placebo-controlled, superiority study with ponesimod in patients with RMS. The study is designed to compare the efficacy, safety, and tolerability of add-on therapy with ponesimod 20 mg vs. placebo in adult patients with active **RMS** who are treated with dimethyl fumarate (Tecfidera®). Approximately 600 patients receiving dimethyl fumarate twice daily for at least 6 months will be randomized in a 1:1 ratio to ponesimod 20 mg or placebo. Treatment will be given until the last patient enrolled into the study has been treated for 60 weeks, with expected average treatment duration of 2 years, and for a maximum duration of 3 years. Enrollment has commenced and the study is being conducted under a Special Protocol Assessment (SPA) agreement with the FDA.

AVAILABLE CLINICAL DATA

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The decision to move into Phase III development was based on the Phase IIb dose-finding study with ponesimod in patients with relapsing-remitting multiple sclerosis. A total of 464 patients were randomized into this study and the efficacy, safety and tolerability of three ponesimod doses (10, 20, and 40 mg/day) versus placebo, administered once daily for 24 weeks.

The primary endpoint of this study was defined as the cumulative number of new gadolinium-enhancing lesions on T1-weighted magnetic resonance imaging (MRI) scans at weeks 12, 16, 20, and 24 after study drug initiation. A key secondary endpoint of this study was the annualized relapse rate over 24 weeks of treatment. Patients who completed 24 weeks of treatment were offered the opportunity to enter into an extension study. This ongoing trial is investigating the long-term safety, tolerability, and efficacy of 10 and 20 mg/day of ponesimod in patients with relapsing-remitting multiple sclerosis, in a double-blind fashion. The study continues to provide extensive safety and efficacy information for ponesimod in this

indication, with some patients treated for more than 6 years. The safety database from all studies with ponesimod now comprises more than 1,300 patients and healthy volunteers.

MILESTONES

- 2015** Phase III program in multiple sclerosis initiated
- 2011** Phase IIb dose-finding study in multiple sclerosis successfully completed
- 2006** Entry-into-humans
- 2004** Preclinical development initiated

KEY SCIENTIFIC LITERATURE

- Olsson T et al. J Neurol Neurosurg Psychiatr. 2014; 85(11):1198-208.
- Freedman M.S, et al. Multiple Sclerosis Journal, 2012; 18(4 suppl):420(P923).
- Fernández Ó, et al. Multiple Sclerosis Journal, 2012; 18(4 suppl):417 (P919).
- Piali L, et al. J Pharmacol Exp Ther 2011; 337(2):547-56.
- Bolli MH, et al. J Med Chem. 2010; 53(10):4198-211.
- Kappos L et al. N Engl J Med. 2010; 362(5):387-401.

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CENERIMOD

Cenerimod is Actelion's second selective sphingosine-1-phosphate receptor 1 (S1P₁) immunomodulator, it is both potent and orally active.

Cenerimod blocks the egress of lymphocytes from lymphoid organs thereby reducing the availability of circulating effector T and B cells that can invade target organs. This pharmacodynamic effect is sustained with continued daily oral dosing and is reversible upon drug discontinuation.



CURRENT STATUS

Cenerimod is being evaluated in a Phase II prospective, multicenter, multinational, randomized, double-blind, placebo-controlled, dose-response study to investigate the biological activity, safety, and tolerability of cenerimod in adult subjects with **systemic lupus erythematosus (SLE)**. Approximately 64 subjects will be enrolled to receive either 0.5, 1, 2 or 4 mg/day of cenerimod over a treatment period of 12 weeks. The study is being conducted at approximately 20 sites and is expected to last around 20 months.

AVAILABLE CLINICAL DATA

In the Phase I program, cenerimod showed marked and sustained lymphocyte lowering effects, supporting further exploration in patients.

MILESTONES

2015 Initiation of Phase II clinical development in systemic lupus erythematosus

KEY SCIENTIFIC LITERATURE

- Kappos L et al. N Engl J Med. 2010; 362(5):387-401.
- Borchers AT, et al. Autoimmun Rev. 2010; 9(5):A277-87.
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- Rahman A, Isenberg DA. N Engl J Med. 2008; 358:929-39.
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CLAZOSENTAN

Clazosentan is an endothelin receptor antagonist added to Actelion's pipeline through the acquisition of Axovan in 2003.

Clazosentan is being developed as an intravenous infusion to treat cerebral vasospasm in patients post aneurysmal subarachnoid hemorrhage (aSAH). The product was granted orphan medicinal product status in Europe in 2003, and in the US in 2006.



CURRENT STATUS

REVERSE (REversal of Vasospasm with clazosentan post-aneurysmal Subarachnoid hemorrhage), is a Phase II prospective, multi-center, open-label, single arm study to evaluate whether clazosentan has an early effect in reversing angiographically-confirmed cerebral vasospasm in patients with aneurysmal subarachnoid hemorrhage treated by endovascular coiling or surgical clipping. The study is expected to enroll approximately 25 subjects, and clazosentan will be administered at the dose of 15 mg/h for a maximum of 10 days.

AVAILABLE CLINICAL DATA

Clazosentan has been investigated for prevention of angiographic vasospasm in patients with aSAH in the Phase IIb CONSCIOUS-1 (Clazosentan to Overcome Neurological ISchemia and Infarct Occurring After

Subarachnoid Hemorrhage) study. Clazosentan (1, 5, and 15 mg/h doses) dose-dependently prevented the occurrence of significant angiographic vasospasm, with a 65% relative risk reduction with the highest dose ($P < 0.0001$); this suggests that endothelin-1 plays an important role in the pathogenesis of vasospasm. Compared with placebo, pulmonary complications, anemia, and hypotension were more common in patients receiving clazosentan.

On the basis of CONSCIOUS-1, two Phase III studies (CONSCIOUS-2 and CONSCIOUS-3) were designed to assess the effect of clazosentan on the incidence of cerebral vasospasm-related morbidity and all-cause mortality within 6 weeks post-aSAH. In CONSCIOUS-2, patients who had their aneurysm secured by clipping received placebo or clazosentan 5 mg/h; however, the treatment effect of clazosentan did not reach statistical significance. In CONSCIOUS-3, patients who had their aneurysm secured by coiling received placebo or clazosentan 5 or 15 mg/h. Recruitment into this study was halted prematurely after the results of CONSCIOUS-2 were known, because the 5 mg/h dose did not achieve its primary end point in clipped patients. However, analysis of the data collected in CONSCIOUS-3 showed that clazosentan 15 mg/h significantly reduced cerebral vasospasm-related morbidity

and all-cause mortality, with a 44% relative risk reduction ($p = 0.0074$). This dose also significantly reduced the incidence of delayed ischemic neurological deficit (DIND) with a 54% relative risk reduction ($p = 0.0038$). In addition, clazosentan reduced the need for rescue therapy for vasospasm. Clazosentan did not improve outcome, possibly due to the imbalance in the use of rescue therapy. The safety profile in CONSCIOUS 2 and 3 was similar to that observed in the CONSCIOUS 1 study.

MILESTONES

- 2015** Initiation of Phase II proof of concept to treat vasospasm in subjects with aSAH
- 2011** Phase III CONSCIOUS-3 study discontinued
- 2010** Phase III CONSCIOUS-2 study concluded - primary endpoint not met
- 2006** Orphan status granted in US
- 2003** Orphan status granted in Europe
- 2003** Axovan acquisition

KEY SCIENTIFIC LITERATURE

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DUAL OREXIN RECEPTOR ANTAGONIST

Actelion's new dual orexin receptor antagonist (DORA), is orally active, effectively crosses the blood-brain barrier and is currently evaluated as a treatment for insomnia.

As part of its drug discovery efforts on G-Protein Coupled Receptors, Actelion has built a library of potent dual oral orexin receptor antagonists. These compounds are active on both OX1 and OX2, the receptors which mediate the actions of orexins. Actelion's work with dual orexin receptor antagonism has demonstrated that blocking the activity of the orexin receptors offers the potential to restore normal physiological sleep.



CURRENT STATUS

In July 2016, Actelion announced that the company is initiating a Phase II program with its new dual orexin receptor antagonist in patients with **insomnia**.

The Phase II program consists of two studies, one in adult and one in elderly patients. It is designed to evaluate the effect of Actelion's DORA versus placebo on sleep maintenance and sleep initiation, as well as next-day residual effect and next-day performance. The adult study will also include an active reference arm with zolpidem, as the most widely used insomnia treatment targeting GABA-A receptors. Both studies will also generate

information on sleep architecture and sleep quality.

The first study is a multi-center, double-blind, randomized, placebo-controlled, active reference, parallel-group, dose-response study to evaluate the efficacy and safety of Actelion's DORA. The study is expected to commence enrollment in Q4 2016 and will recruit approximately 300 adult patients diagnosed with insomnia. The study will comprise 6 treatment arms: placebo; zolpidem; 5, 10, 25, and 50 mg of Actelion's DORA. Treatment duration is 4 weeks. The primary endpoint is wake-time after sleep onset (WASO) at day 1 & 2.

The second study is a multi-center, double-blind, randomized, placebo-controlled, crossover, dose-response study to evaluate the efficacy and safety of Actelion's DORA. The study is also expected to commence enrollment in Q4 2016 and will recruit approximately 50 elderly patients diagnosed with insomnia. The study has a 5-period crossover design with 5 treatment arms: placebo; 5, 10, 25 and 50 mg of Actelion's DORA. Treatment duration in each period is 2 days. The primary endpoint is WASO at day 1 & 2.

Secondary objectives of both studies include evaluation of Actelion's DORA versus placebo on latency to persistent sleep (LPS) as well as subjective latency to sleep onset (sLSO) and subjective WASO (sWASO). Safety and tolerability will also be evaluated.

AVAILABLE CLINICAL DATA

Data from an extensive Phase I program have confirmed the optimal pharmacokinetic and pharmacodynamic profile for a sleep medication, together with excellent safety and tolerability.

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2014 Initiation of Phase I clinical program

KEY SCIENTIFIC LITERATURE

- Brisbare-Roch C. et al. Nat Med. 13(2):150-5; 2007.
- Hoever P et al. Clin Pharmacol Ther Clin Pharmacol Ther. 2012; 91(6); 975-985.
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ENDOTHELIN RECEPTOR ANTAGONIST

As part of its endothelin receptor antagonist (ERA) program, Actelion has discovered an orally active, potent dual ERA, suitable for development in specialty cardiovascular disorders.

Actelion is a leader in the field of endothelin receptor antagonism, with macitentan (Opsumit®), an orally available endothelin receptor antagonist (ERA) that resulted from a tailored drug discovery process in Actelion's laboratories.



CURRENT STATUS

Actelion's latest ERA (ACT-132577) is evaluated in a Phase II prospective, multi-center, double-blind, double-dummy, randomized, placebo- and active-reference, parallel group, dose-finding study in patients with essential hypertension (grade 1 and 2) to establish a dose-effect relationship. The results from this study will form the basis for development decisions in **specialty cardiovascular disorders**. Patients will be randomized in the 6 groups in a 1:1:1:1:1:1 ratio: placebo; dose 1, dose 2, dose 3, dose 4 of Actelion's ERA; and lisinopril 20 mg.

AVAILABLE CLINICAL DATA

Pharmacokinetic, pharmacodynamic, safety and tolerability data from a Phase I program support further exploration of ERA in patients with cardiovascular disorders.

MILESTONES

2015 Initiation of Phase II dose-response study

2014 Initiation of Phase I clinical program

KEY SCIENTIFIC LITERATURE

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PHASE I COMPOUNDS

Actelion has developed platforms of expertise in families of molecular targets which allow high productivity in the generation of innovative compounds potentially addressing a wide range of high unmet medical needs.

Actelion currently has five compounds in Phase I clinical development. These include a selective orexin 1 receptor antagonist (SORA) and a T-type calcium channel blocker, both being developed in the field of neurology, one cardiovascular compound, one anti-inflammatory compound and lucerastat in Phase Ib development for Fabry disease.

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

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