Actelion emphasizes commitment to advance research and care in rare diseases

“Patient Voice — Join us in making the voice of rare diseases heard.” This is the theme of Rare Disease Day 2016 – and a cause for Actelion to emphasize its long-term commitment to support the needs of the patient community.

ALLSCHWIL, SWITZERLAND – 29 February 2016 – Today, on Rare Disease Day 2016, patients, their families and caregivers, patient organizations, medical professionals, politicians and researchers are coming together to raise awareness for what living with a rare disease means for those affected and for their families.

Patients play a crucial role in bringing both research and the standard of care in rare diseases forward, which is recognized in the theme of this year’s Rare Disease Day, ‘Patient Voice’. For Actelion, listening to patients’ voices and working closely with the rare disease community is key to delivering on the company’s mission to discover new innovative drugs for rare diseases and to help more patients with groundbreaking therapies. At the end of 2015, over 65,000 patients were benefiting from Actelion’s rare disease drugs, and the company continues to work with governments and other stakeholders to widen accessibility around the world.

On February 23 in Brussels, our commitment to rare diseases was publicly recognized by the European Organization for Rare Diseases (EURORDIS) as Actelion was honored with the EURORDIS Company Award 2016. Presented to pioneering companies for the development of treatments for rare diseases, the award in particular acknowledges Actelion’s work with patient groups to support the patient and carer community.

Through the company’s collaborative work with patient groups and healthcare professionals, Actelion has become a trusted partner in supporting activities that raise awareness of the challenges facing those living with rare diseases and help bring about positive change. Activities include the development of educational resources such as the innovative PAHuman® eBook to better explain the disease and its impact, and the “Think Again. Think NP-C” campaign to encourage earlier diagnosis of Niemann-Pick type C disease among healthcare professionals unfamiliar with this rare condition.

Gerald Fischer, CEO of the European Pulmonary Hypertension Association (PHA Europe), said: “Actelion has provided invaluable support to our activities and, as a result, our collaboration has led to many great achievements over the years. It’s clear that
Actelion’s commitment extends beyond the discovery and development of new medicines and includes initiatives that increase knowledge and understanding of PAH and other rare diseases, which is crucial to organizations like ours.”

An example of Actelion’s pioneering work in rare diseases is an ongoing clinical study involving patients with Eisenmenger syndrome. Working with ethics committees, patient advocacy, support groups and patients’ families, Actelion has extended the study on Eisenmenger syndrome to Down syndrome patients, which will help to address the high unmet medical need for effective, targeted PAH therapies in this vulnerable and too rarely considered population.

Martine Clozel, MD, Chief Scientific Officer at Actelion, commented: “I am very proud that, together with the PAH community, Actelion has brought about not one, but several paradigm shifts in PAH care. The three established pathways in PAH are now well served, but our discovery efforts continue. We are turning to new pathways in PAH, helped by our strong connections with academia, to further improve the prognosis for patients living with this devastating disease. Actelion also plays a leadership role in Niemann-Pick type C disease, where the lives of many patients have been positively impacted. I am convinced that with our strong and innovative research, Actelion will continue to advance care in many rare diseases.”

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NOTES TO EDITOR:

ABOUT RARE DISEASE DAY 2016

Rare Disease Day takes place on the last day of February each year. Its main objective is to raise awareness amongst the general public and decision-makers about rare diseases and their impact on patients' lives, Since Rare Disease Day was first launched by EURORDIS and its Council of National Alliances in 2008, thousands of events have taken place throughout the world, reaching hundreds of thousands of people. The Rare Disease Day 2016 theme ‘Patient Voice - Join us in making the voice of rare diseases heard’ recognizes the crucial role that patients play in voicing their needs and in instigating change that improves their lives and the lives of their families and caregivers. The theme appeals to a wider audience, those that are not living with or directly affected by a rare disease, to join the community in making known the impact of rare diseases. To learn more, visit [http://www.rarediseaseday.org](http://www.rarediseaseday.org).

ABOUT PAH

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. The symptoms of PAH are non-specific and can range from mild breathlessness and fatigue during normal daily activity to symptoms of right heart failure and severe restrictions on exercise capacity and ultimately reduced life expectancy. PAH is one group within the classification of pulmonary hypertension (PH). This group includes idiopathic PAH, heritable PAH and PAH caused by factors which include connective tissue disease, HIV infection and congenital heart disease. The last decade has seen significant advances in the understanding of the pathophysiology of PAH, which has been paralleled with developments of treatment guidelines and new therapies. Drugs targeting the three pathways that have been established in the pathogenesis of PAH are endothelin receptor antagonists (ERAs),

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prostacyclin receptor agonists, and phosphodiesterase-5 inhibitors. PAH treatments have transformed the prognosis for PAH patients from symptomatic improvements in exercise tolerance 10 years ago to delayed disease progression today. Improved disease awareness and evidence-based guidelines developed from randomized controlled clinical trial data have highlighted the need for early intervention, goal-oriented treatment and combination therapy. Learn more at http://www.pahuman.com/

ABOUT NIEMANN-PICK TYPE C DISEASE (NP-C)
NP-C disease is a treatable rare, neurodegenerative, genetic condition, primarily affecting children and teenagers although the clinical manifestations can become apparent at any age. The symptoms are caused by the storage of some lipids - such as glycosphingolipids and cholesterol - within certain tissues in the body, including the brain. Neurological deterioration is the key feature of the disease and can manifest itself as clumsy body movements, balance problems, slow and slurred speech, difficulty in swallowing, problems with eye movements and seizures. Intellectual decline is also common. In the final stages of the disease the child or young adult is frequently bedridden, has little muscle control and is intellectually impaired. Diagnosis of the disease can be difficult and may take years due to the rarity and heterogeneity of the condition.

Think Again. Think NP-C is a project coordinated by the International Niemann-Pick Disease Alliance with the collaboration and financial support of Actelion Pharmaceuticals Ltd. A multidisciplinary advisory committee of leading clinical experts in the field of NP-C provided advice and input into the content of the campaign to ensure it would resonate with healthcare professionals. http://think-npc.com/

ABOUT EISENMENGER SYNDROME
Eisenmenger syndrome is the most advanced form of pulmonary arterial hypertension (PAH) in conjunction with congenital heart disease (CHD). Down syndrome patients represent an important subset of the Eisenmenger population (between 25% and 50%, depending on cohort studied). To address the high unmet medical need for effective, targeted PAH therapies in this vulnerable population, Actelion has extended its MAESTRO study with macitentan in Eisenmenger syndrome patients to the Down syndrome community. To ensure proper safeguards are established to protect the patients’ rights and safety, we have been working with ethics committees, patient advocacy, support groups and patients’ families. Our outreach was incredibly well received by all parties involved, and their support has enabled us to enroll a number of Down syndrome patients into the study. This is the first time that Down syndrome patients afflicted with Eisenmenger syndrome are included in a randomized clinical trial.

ABOUT THE EURORDIS AWARDS
Actelion has been honored by EURORDIS, the European Organization for Rare Diseases, with the EURORDIS Company Award 2016. EURORDIS is a non-governmental, alliance of patient organizations representing 705 rare disease patient organizations in 63 countries. The purpose of the EURORDIS Awards is to recognize the commitment and achievements of patients’ organizations, volunteers, companies, scientists, media and policy makers who have contributed to reducing the impact of rare diseases on people’s lives. Read more about the 2016 winners on http://www.eurordis.org/eurordis-awards
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