

GRÜNENTHAL GROUP

Press Release



Grünenthal and Akashi Therapeutics Inc. announce joint drug development program for HT-100 in the treatment of patients with Duchenne Muscular Dystrophy (DMD)

- ***HT-100 is an orally available small molecule drug candidate designed to reduce fibrosis and inflammation and to promote healthy muscle fiber regeneration in patients with DMD***
- ***DMD is a rare, yet the most common fatal genetic disorder diagnosed in childhood. Today, no curative treatment exists, and patients typically die in their twenties***

Aachen, Germany, and Cambridge, MA, USA, January 8, 2016 – Grünenthal today announced the joint global drug development program with Akashi Therapeutics, Inc., for HT-100, an orally available small molecule drug candidate designed to reduce fibrosis and inflammation and to promote healthy muscle fiber regeneration in Duchenne Muscular Dystrophy (DMD) patients. The drug has orphan designation in both the U.S. and the EU, and fast track designation in the U.S. Grünenthal will be responsible for commercialization in Europe and Latin America, while Akashi Therapeutics Inc. will retain rights for the U.S. and all other markets. This partnership is a key milestone in Grünenthal's strategy to broaden its portfolio in niche indications by building on its track record of bringing innovative pain therapies to patients.

Grünenthal joins forces with Akashi Therapeutics Inc., a company founded by leading patient organizations, to combine skills and capabilities in a unique way for the benefit of patients with DMD. The alliance could in the future be expanded in the effort to the search for improvements for the treatment of boys and young men affected by DMD. HT-100 is currently in clinical phase 1b/2a at five hospitals across the U.S.

"We are very excited about this unique collaboration with Akashi. At Grünenthal, we are highly committed to innovation and have been focusing on bringing innovative therapies to patients with high medical need. We are very motivated to use all our strength for the development of HT-100 together with our partners from Akashi and the patient groups supporting them", said Dr. Klaus-Dieter Langner, Chief Scientific Officer of Grünenthal.

In 2014, Grünenthal announced its strategy to broaden its focus from pain and inflammation to niche indications with high unmet medical need. On average, the company invests 25% of its revenues back into R&D and is a global leader in the area of prescription opioid analgesics. "As a mid-cap pharmaceutical company, we will be in a position to give the development program of HT-100 high priority in order to hopefully improve the devastating situation of DMD patients", concludes Klaus-Dieter Langner.

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“Grünenthal brings to our collaboration outstanding scientific and intellectual capital, significant financial resources and a powerful commitment to positively impact the treatment of DMD,” said Marc Blaustein, CEO, Akashi Therapeutics Inc., a company that to date has been entirely funded by DMD patient foundations. “Akashi was founded to bring safe and effective treatments to DMD patients, and we look forward to working with our new partners to accelerate achievement of this goal.”

Under the terms of the agreement, Grünenthal will make upfront and milestone payments to Akashi. In addition, the company will assume all post-Phase 2 global development costs through commercialization of an approved product. Akashi will receive royalties on net sales. In total, Grünenthal plans to commit more than \$100 million to the partnership and will receive royalties on U.S. net sales in exchange for funding development of Akashi’s U.S. commercial infrastructure.

About HT-100

HT-100 (delayed-release halofuginone) is an orally available, small molecule drug candidate designed to reduce fibrosis and inflammation and promote healthy muscle fiber regeneration in DMD patients. HT-100 has been granted orphan designation for DMD in both the U.S. and E.U., and fast track designation in the U.S. A phase 1b/2a clinical program is currently underway at five hospitals in the U.S. For more information, please contact Akashi Therapeutics Inc. (www.akashirx.com)

About Duchenne Muscular Dystrophy (DMD)

Duchenne muscular dystrophy (DMD) is an X-linked recessive, inheritable disease that affects approximately 1 in 3,600 boys. DMD results in muscle degeneration and premature death. Symptoms usually become visible in early childhood: progressive proximal muscle weakness of the legs and pelvis associated with loss of muscle mass is observed first, and this weakness spreads to other parts of the body. As the disease progresses, muscle tissue is replaced by fat and fibrotic tissue (fibrosis). Untreated, most patients are wheelchair dependent by age 10. Due to progressive deterioration of muscle, patients lose ambulation, then arm function, and ultimately experience respiratory and/or cardiac failure. While life expectancy varies, patients typically survive until late in the second or the third decade.

About Grünenthal

The Grünenthal Group is an independent, family-owned, international research-based pharmaceutical group headquartered in Aachen, Germany. Grünenthal We are an entrepreneurial specialist delivering true benefits to patients. By sustainably investing in research and development above the industrial average, we are committing to innovation in order to treat unmet medical needs and bring value-adding products to markets. Grünenthal GmbH is a fully integrated research & development company with a long track record of bringing innovative pain treatments and state-of-the-art technologies to patients.

Altogether, the Grünenthal Group is present in 32 countries with affiliates in Europe, Australia, Latin America and the US. Grünenthal products are sold in more than 155 countries and approx. 5,300 employees are working for the Grünenthal Group worldwide. In 2014, Grünenthal achieved revenues of € 1.154 bn. More information: www.grunenthal.com.

About Akashi Therapeutics Inc.

Akashi Therapeutics is a clinical stage biopharmaceutical company whose mission is to develop treatments for Duchenne muscular dystrophy. Akashi was founded by leading patient organizations Charley’s Fund and Nash Avery Foundation in collaboration with biotechnology industry veterans to impact a central problem in rare diseases: rapid therapy development. Akashi is developing a pipeline of therapies with the goal of transforming Duchenne from a 100% fatal, aggressive muscle-wasting disease to a chronic, manageable condition. For more information, please visit www.akashirx.com.

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