Grünenthal receives FDA Orphan Drug and Rare Pediatric Disease Designations for Tegacorat for the Treatment of Duchenne Muscular Dystrophy

Wednesday 8 July, 2026

Aachen, 08 July 2026 –
Grünenthal announced today that its investigational compound tegacorat (GRM-01) received Orphan Drug and Rare Pediatric Disease Designations from the US Food and Drug Administration (FDA) for the treatment of Duchenne muscular dystrophy (DMD).

Tegacorat, a non-steroidal Selective Glucocorticoid Receptor Agonist and Modulator (SEGRAM), is an orally available investigational compound being developed to provide an alternative to glucocorticoid-based treatments such as prednisone, the current standard of care in DMD.

Conventional glucocorticoids bind to the glucocorticoid receptor and influence gene expression. Among other mechanisms, this triggers two patterns of activity: transrepression, mainly linked to anti-inflammatory effects, and transactivation, predominantly associated with metabolic and growth-related side effects. In contrast, SEGRAMs are designed to influence receptor activity in a way that emphasises anti-inflammatory pathways. While this has not yet been established in clinical trials, it may allow for more efficacious dosing than current standard treatments and potentially cause fewer side effects.

“With the current standard of care, people affected by DMD, their caregivers and clinicians must constantly balance efficacy and the burden of side effects as they pursue the essential goal of preserving muscle function,” says Uli Brödl, Chief Scientific Officer at Grünenthal. “We aim to address the unmet need for a long-term therapy option with potent anti-inflammatory efficacy while reducing dose- and duration dependent side effects. The Orphan Drug and Rare Pediatric Disease designations are an important milestone in the development of tegacorat.”

Grünenthal is currently preparing a Phase II trial to investigate the efficacy, safety and tolerability of tegacorat in Duchenne muscular dystrophy. The trial is expected to commence later in 2026 at centres in the US and Europe.

About Duchenne muscular dystrophy (DMD)

Duchenne muscular dystrophy is one of the most common recessive genetic disorders, affecting around 1 in every 5,000 boys born.[1], [2] Caused by mutations in the dystrophin gene that encodes for a critical protein that protects muscles from damage, DMD causes progressive muscle weakness throughout the body that eventually impacts mobility, breathing and the heart.[2] DMD is incurable and results in death, usually between 21 and 40 years of age.[3]

Today’s treatment options are neither curative nor preventive of disease progression. Glucocorticoids serve as the standard of care, as they can help slow the progression of muscle deterioration. However, they come with significant side effects, including cushingoid appearance, weight gain and behavioural changes.[4]

About Grünenthal

Grünenthal is a global leader in pain management and related diseases. As a science-based, fully integrated pharmaceutical company, we have a long track record of bringing innovative treatments and state-of-the-art technologies to patients worldwide. Our purpose is to change lives for the better – and innovation is our passion. We focus all our activities and efforts on working towards our vision of a World Free of Pain.

Grünenthal is headquartered in Aachen, Germany, and has affiliates in 28 countries across Europe, Latin America, and the U.S. Our products are available in approx. 100 countries. In 2025, Grünenthal employed around 4,100 people and achieved revenues of €1.8 billion.

Related Sectors:

Business & Finance :: Medical & Pharmaceutical ::

Related Keywords:

Pharmaceuticals :: Healthcare :: Rare Pediatric Disease :: Duchenne Muscular Dystrophy ::

Scan Me:

More information: www.grunenthal.com and follow us on LinkedIn & Instagram

For further information, please contact:

Christopher Jansen, Global Communications at Grünenthal, christopher.jansen@grunenthal.com

[1] Crisafulli S, et al. Global epidemiology of Duchenne muscular dystrophy: an updated systematic review and meta-analysis. Orphanet J Rare Dis. 2020; 15(1):141.

[2] Bez Batti Angulski A, et al. Duchenne muscular dystrophy: disease mechanism and therapeutic strategies. Front Physiol. 2023; 14:1183101.

[3] Landfeldt E, et al. Life expectancy at birth in Duchenne muscular dystrophy: a systematic review and meta-analysis. Eur J Epidemiol. 2020; 35(7):643-653. *[Estimated mortality for those who receive ventilatory support.]*

[4] Fischer R, et al. A Mixed-Method Study Exploring Patient-Experienced and Caregiver-Reported Benefits and Side Effects of Corticosteroid Use in Duchenne Muscular Dystrophy. J Neuromuscul Dis. 2023;10(4):593-613.

Company Contact:

_

news aktuell

E. desk@newsaktuell.de
W. https://www.newsaktuell.de/

View Online

Newsroom: Visit our Newsroom for all the latest stories:
https://www.newsaktuell.pressat.co.uk