Impact Story

ALESTA THERAPEUTICS

TCHF III

Founded in
2021

Located in
Netherlands

Disease focus
Rare diseases

CEO
Ilan Ganot

Responsible partner
Michel Briejer

The company in a nutshell

- Alesta Therapeutics (Alesta) is a **biotechnology** company developing **novel small molecules** to **treat** genetically defined tRNA disorders, with an initial focus on **Charcot-Marie-Tooth (CMT) disease**.
- Charcot-Marie-Tooth (CMT) disease is a **rare genetic disease** leading to peripheral neuropathy, starting with disabling muscle weakness, which could ultimately lead to immobility.
- Ilan Ganot, CEO, brings a wealth of leadership experience in the rare diseases field.
- Alesta works together with the Jackson Laboratories – a top tier genetics institute in the United States.
- Alesta is developing drugs targeting the protein GCN2, which is activated in diseases with impaired tRNA loading. Hence, Alesta's GCN2 inhibitors have the **potential to treat a range of diseases**, including cancer.

Healthcare impact potential

Alesta aims to begin its first clinical trials in 2025. In general, for a preclinical stage company, it will take around 10 years for the drug to be available to patients outside of trials. It is worth noting that in the case of rare diseases, there may be faster development pathways available for the company.

The expected healthcare impact of the technology, once it reaches the market, is:

Major impact on quality of life as the GCN2 inhibitor of Alesta aims to halt/reduce disease progression and aggressivity of CMT disease, which has no therapeutic treatment options today.

If successful, Alesta has the potential to change the life of patients with CMT disease by providing an effective drug for a disease where there are no therapeutic treatment options today.

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The problem that Alesta is solving

- Charcot-Marie-Tooth (CMT) disease is a disease of the peripheral nerves that control muscles. It causes progressive loss of function and sensation in the hands, arms, legs, and feet, leading to a range of symptoms[1]:

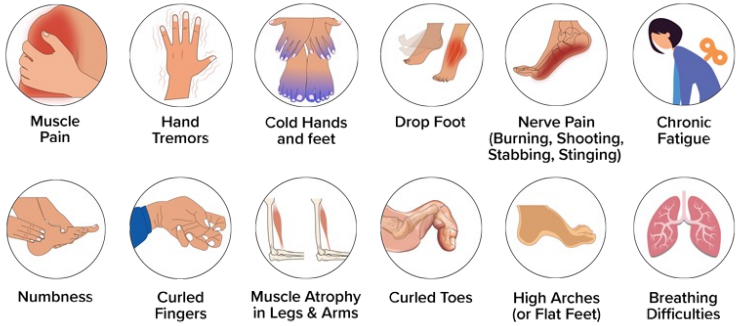

Image source: Charcot-Marie-Tooth Association (CMTA). Used with permission.

- Although CMT disease is a rare disease, it is the the most common inherited neuromuscular disorder with a prevalence of 15 per 100,000 persons.[2] Alesta is focussing on a subgroup of CMT patients, who have genetic mutations in genes involved in transfer RNA (tRNA) loading (estimated incidence of >1 per 100,000).
- tRNAs are molecules that act as temporary carriers of amino acids, the building blocks of proteins. tRNAs are loaded with amino acids and bring those amino acids to so called ribosomes, the cellular factories that build proteins out of those amino acids. In CMT patients with genetic mutations that lead to incorrect tRNA loading with amino acids, GCN2 is activated, and ribosomes are stalled, leading to impaired protein synthesis.
- Therefore, by inhibiting GCN2, Alesta aims to solve the problem of tRNA-associated diseases.

[1] Charcot-Marie-Tooth Association (CMTA); [2] Medscape, Charcot-Marie-Tooth Disease.

Current treatment options

- CMT disease continues to be an incurable condition. No proven medical treatment exists to reverse or slow the natural disease process.[2]
- Current treatment options are treating disease symptoms only and consist of physical therapy, mechanical supports (orthoses and braces) and in severe cases orthopaedic surgery can correct deformity and help maintain mobility and function.[1]
- Hence, **the unmet medical need for patients with CMT is high**.