Health Technology Briefing
January 2026

GNT0006/ATA-100 for treating limb girdle muscular dystrophy type R9 (FKRP-related)

Company/Developer	Atamyo Therapeutics

☒ **New Active Substance** ☐ **Significant Licence Extension (SLE)**

NIHRIO ID: 34785	**NICE ID:** Not available	**UKPS ID:** Not available

Licensing and Market Availability Plans

Currently in phase 1 clinical development

Summary

GNT0006/ATA-100 is being developed to treat a rare muscle condition called limb-girdle muscular dystrophy type R9 (FKRP-related). This condition is caused by a change in a gene that helps muscles stay strong and work properly. When this gene does not function as it should, the muscles around the hips and shoulders gradually become weaker and smaller. Over time, people may find it harder to move and may eventually need a wheelchair. The condition can also affect the heart and breathing muscles, which may become life-threatening. There are currently no approved medicines that can stop or slow down the disease, and available care focuses on supportive care for managing symptoms and maintaining quality of life.

GNT0006/ATA-100 is a new gene therapy that aims to treat the cause of the disease rather than just the symptoms. It works by delivering a healthy copy of the missing or faulty gene into the muscle cells, helping them produce the protein needed to keep muscles strong. The treatment is given as a single drip into a vein, and doctors monitor patients closely afterwards. By repairing the underlying problem, GNT0006/ATA-100 could help protect muscles from further damage, improve movement, and slow the worsening of weakness. If approved, it would be the first treatment to directly target the cause of limb-girdle muscular dystrophy type R9, offering new treatment to people and families affected by this rare condition.

This briefing reflects the evidence available at the time of writing and a limited literature search. It is not intended to be a definitive statement on the safety, efficacy or effectiveness of the health technology covered and should not be used for commercial purposes or commissioning without additional information. A version of the briefing was sent to the company for a factual accuracy check. The company was unavailable to comment.

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Proposed Indication

For the treatment of FKRP-related limb girdle muscular dystrophy (LGMD) type R9.[1]

Technology

Description

GNT0006 /ATA-100 is an adeno-associated viral vector gene therapy carrying the human fukutin-related protein (FKRP) transgene to skeletal and cardiac muscle cells.[1] Dystroglycan (DG) is a key component of the dystrophin-glycoprotein complex (DGC), which is essential for maintaining muscle membrane stability by providing a physical linkage between the myofiber actin cytoskeleton and the extracellular matrix (ECM).[2-4] The α-subunit of dystroglycan (α-DG) is extensively glycosylated with both N- and O-linked glycans and acts as a cellular receptor for laminin and other ECM proteins, including agrin, perlecan, neurexin, and pikachurin, as well as for certain Old World arenaviruses.[5-11] Mutations in the FKRP gene lead to a spectrum of dystroglycanopathies, including limb-girdle and congenital muscular dystrophies.[12] GNT0006/ATA-100 functions as a gene-replacement therapy, delivering a functional FKRP gene directly to muscle cells via an adeno-associated virus (AAV) vector.[13] This allows systemic AAV-mediated gene transfer addressing muscle diseases.[14]

GNT0006/ATA-100 is currently in clinical development for the treatment of people aged 16 years and older with a confirmed diagnosis of limb-girdle muscular dystrophy type R9 (FKRP-related).[1] In the ongoing phase 1, open-label, dose-escalation study (NCT05224505), participants receive a single intravenous (IV) infusion of ATA-100 at doses of 9.0×10^{12} vg/kg or 2.7×10^{13} vg/kg, followed by long-term safety follow-up for up to five years.[1]

Key Innovation

The management of LGMD is primarily supportive, as no disease-modifying treatments are currently available. The goals of therapy include maintaining mobility and functional independence, managing associated complications, and maximising quality of life.[15] While there are no approved disease-modifying therapies for LGMDR9, various approaches—from small molecules to gene therapy—are being actively investigated.[16-19] FKRP gene therapy is considered one of the most promising therapeutic strategies for treating limb-girdle muscular dystrophy type R9 (LGMDR9).[1,20] This represents a novel therapeutic strategy targeting the underlying genetic cause of FKRP-related muscular dystrophy.[21]

If licensed, GNT0006/ATA-100 would represent the first disease-modifying treatment option for patients with LGMD type R9 (FKRP-related).

Regulatory & Development Status

Limb girdle muscular dystrophy R9 gene therapy does not currently have marketing authorisation in the EU/UK for any indication.

GNT0006/ATA-100 has the following regulatory designations/awards;

- Orphan drug designation from the European Medicines Agency (EMA) in May 2022 for the treatment of LGMD type R9 (FKRP-related).[22]
- Orphan drug designation and Investigational New Drug (IND) clearance from the U.S. Food and Drug Administration (FDA) in February 2022 for the treatment of LGMD type R9 (FKRP-related).[23]

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Patient Group

Disease Area and Clinical Need

The LGMDs comprise a group of inherited muscle diseases characterized by progressive proximal weakness and muscle wasting.[24] LGMD type R9 (FKRP-related) is a progressive autosomal-recessive neuromuscular disorder caused by pathogenic variants in the FKRP gene.[25] The disease causes muscle weakness as a result of FKRP deficiency in skeletal and cardiac muscle. [26] The symptoms of LGMDR9 can vary from person to person. They usually start between the ages of 10 and 20 and can develop quickly. Milder forms can start later and develop more slowly.[27] Some people with the disease can have a mild course where they are nearly asymptomatic, while others may have severe symptoms that can be fatal. The most common symptom is muscle weakness of the hip, shoulder, and abdomen that gets worse over time. The rate at which the muscles weaken can vary, but muscle weakening often results in a wheelchair becoming necessary. Other possible features include enlarged calf muscles, shortening and hardening of muscles leading to rigid joints (contractures), prominence (winging) of the shoulder blades, and curvature of the spine (scoliosis). Respiratory complications (seen in approximately 30-50% of individuals) or heart complications (seen in over 50% of individuals) are also associated with these conditions and may lead to death.[26]

The LGMDs individually are rare, with some forms reported in only a few families. In the UK, LGMDs comprised 6.2% of the population of a specialist muscle clinic, giving a prevalence of 2.27/100,000.[28,29] In England, 2024-2025, there were 13,088 finished consultant episodes (FCEs) for Muscular dystrophy (ICD-10 code G71.0), leading to 2,170 admissions, 1549 day cases and 2,336 FCE bed days.[30]

Recommended Treatment Options

There are currently no pharmacological treatment options recommended by the National Institute for Health and Care Excellence (NICE) for LGMD type R9 (FKRP-related).

Clinical Trial Information

Trial	NCT05224505, 2021-004276-33; A Phase 1-2 Multicentre Study (2-stages) to Evaluate the Safety and Efficacy of Intravenous GNT0006/ATA-100, Adeno-associated Viral Vector Carrying the FKRP Gene, in Patients With FKRP-related Limb-girdle Muscular Dystrophy **Phase I –** Active, not recruiting **Locations:** 2 EU countries, UK **Primary completion date:** February 2025
Trial Design	Non-randomised, sequential assignment, single IV administration of ATA-100, open label, 2 sequential cohorts, quadruple (participant, care provider, investigator, outcomes assessor)
Population	N=6; aged 16 years and older; patients with documented LGMDR9 diagnosis based on clinical presentation and genotyping confirming the FKRP gene mutations
Intervention(s)	• Biological: ATA-100 (AAV9 encoding FKRP gene) ○ Single intravenous infusion on day 0
Comparator(s)	No comparator

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Outcome(s)	Primary outcome measure: • Safety and tolerability [Time frame: Baseline through 12 months] ○ Incidence of Adverse Events and significant laboratory changes See trial record for full list of other outcomes.
Results (efficacy)	-
Results (safety)	-

Estimated Cost

The cost of GNT0006/ATA-100 is not yet known.

Relevant Guidance

NICE Guidance

No relevant NICE guidance.

NHS England (Policy/Commissioning) Guidance

- NHS England. *Diagnostic service for rare neuromuscular disorders (All ages).* D04/S(HSS)/a. 2013/14 NHS Standard Contract.[31]

Other Guidance

- Muscular Dystrophy UK. *Standards of Care for Limb-Girdle Muscular Dystrophies (LGMDs).* Updated 2025.[32]
- European Neuromuscular Centre (ENMC). *Limb-girdle muscular dystrophies: nomenclature and reformed classification / workshop recommendations. 2018.*[33]

Additional Information

Atamyo Therapeutics did not enter information about this technology onto the UK PharmaScan database; the primary source of information for UK horizon scanning organisations on new medicines in development.

As a result, the NIHR Innovation Observatory has had to obtain data from other sources. UK PharmaScan is an essential tool to support effective NHS forward planning; allowing more effective decision making and faster uptake of innovative new medicines for patients who could benefit. We urge pharmaceutical companies to use UK PharmaScan so that we can be assured of up-to-date, accurate and comprehensive information on new medicines.

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