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SQY Therapeutics reports initial dystrophin restoration and reductions in muscle injury biomarkers in the Phase 1/2a AVANCE-1 trial in Duchenne muscular dystrophy

Interim findings also identify exposure-related safety events that have informed dosing adaptations

- Muscle biopsies from the first two patients to complete Phase 2a at the lowest dose (10 mg/kg) show approximately 4–5% dystrophin restoration from near-absent baseline levels.
- Serum creatine kinase (CK) decreased by approximately 60% across the study population (ambulant and non-ambulant patients); reductions of up to 80% in CK and myomesin-3 are reported in non-ambulant patients.
- Exposure-related renal and hematological adverse events, including one case of cerebral venous thrombosis associated with polycythemia, have informed adaptations to dosing.
- Although exploratory motor assessments are descriptive, three years of follow-up are consistent with maintained performance in ambulant patients treated at the highest dose (25 mg/kg).

Data have been presented by Pr. Helge Amthor at OTS in Denver on 14 October 2026 and will be presented by Dr. Luis Garcia at TIDES Europe in Amsterdam on 5 November 2026.

GUYANCOURT, France, 5 November 2026 – SQY Therapeutics, a clinical-stage biotechnology company developing tricyclo-DNA antisense oligonucleotides, today announced provisional pharmacodynamic, pharmacokinetic and safety results from AVANCE-1, its ongoing Phase 1/2a trial of SQY51 in children and adults with Duchenne muscular dystrophy (DMD) amenable to exon 51 skipping. Initial findings include restoration of dystrophin in muscle biopsies and reductions in circulating biomarkers of muscle injury. The interim dataset also identifies exposure-related renal and hematological adverse events, which have informed adaptations to dosing.

Initial evidence of dystrophin restoration

Muscle biopsies from the first two patients to complete Phase 2a at the lowest dose showed dystrophin restoration of 4–5%, compared with near-absent levels at baseline. The presentation includes one patient treated at 10 mg/kg with approximately 5% restoration and uniform distribution across muscle fibers. These findings provide evidence of the biological activity of SQY51 in patients.

Reductions in circulating muscle injury biomarkers

Serum CK decreased by approximately 60% across the study population, with reduction of up to 80% in serum CK and myomesin-3 in non-ambulant patients, two muscle proteins that are released into circulation after muscle injury. These reductions are consistent with decreased muscle damage. Individual longitudinal data show that CK reductions continued into the extension phase and persisted during treatment interruptions, suggesting a prolonged effect of SQY51 in tissues.

Exploratory observations on ambulatory function

Exploratory and descriptive findings include individual longitudinal results of the six-minute walk test, and the 10-metre run test in ambulant participants, showing maintained performances in higher-dose cohorts three years after inclusion. The small, open-label design and the absence of a concurrent control group limit interpretation; follow-up is ongoing.

Pharmacokinetics supporting exposure-based dosing

Maximum serum concentrations (Cmax) of SQY51 increased linearly over the 2–25 mg/kg intra-patient dose escalation. Distribution and elimination half-lives were similar across doses and patients, and consistent with non-human primate pharmacokinetic models. At the same dose per kilogram, Cmax and overall exposure were up to 2.5-fold higher in older, non-ambulant patients than in younger, ambulant patients. These findings informed the assignment of patients to 10, 16 or 25 mg/kg cohorts according to individual exposure parameters rather than body weight alone.

Safety findings and dosing adaptations

Safety and tolerability are the primary objectives of AVANCE-1. Several patients developed exposure-related glomerular proteinuria, fully or partially reversible over time; a renal biopsy in one patient showed minimal change nephropathy. Exposure-related increases in polycythemia was observed in all patients but one, and several patients required therapeutic phlebotomy. One patient developed cerebral venous thrombosis associated with polycythemia, which resolved without sequelae. These findings have informed adaptations to the treatment schedule and cumulative exposure during Phase 2a and the extension. Continued assessment is required to characterize the safety profile and determine an appropriate dosing regimen.

About AVANCE-1

AVANCE-1 (EU CT 2022-500703-49-01) is a single-center, open-label Phase 1/2a study evaluating the safety, pharmacokinetics and pharmacodynamics of SQY51 in pediatric and adult patients with DMD. The study is conducted at AP-HP Raymond Poincaré Hospital in Garches, France, with Professor Helge Amthor as principal investigator. Participants were aged 7–32 years at enrolment. Twelve patients completed the six ascending doses of Phase 1 and entered Phase 2a. At the interim status shown in the presentation, five patients had completed Phase 2a, six remained in that phase and one had withdrawn; three patients had entered the extension phase. The biopsy findings represent a subset of the ongoing trial, and additional data are needed to characterize the magnitude and durability of the effect.

About SQY51 and Duchenne muscular dystrophy

SQY51 is an investigational tricyclo-DNA antisense oligonucleotide designed to induce skipping of exon 51 in *DMD* pre-messenger RNA. In patients with amenable mutations, this approach aims to restore the reading frame and enable production of an internally shortened dystrophin protein. DMD is a progressive genetic disease in which insufficient functional dystrophin leads to muscle degeneration and loss of function.

About SQY Therapeutics

SQY Therapeutics is a biotechnology company based in Guyancourt, France, developing antisense oligonucleotide therapies using tricyclo-DNA chemistry. Its lead clinical compound, SQY51, is being evaluated in the AVANCE-1 trial in Duchenne muscular dystrophy.

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Disclaimer

The clinical data described in this press release were presented at OTS Denver on 14 October 2026 and reflect the results and analyses available at the time of presentation. These findings should be interpreted

in the context of the study design, patient population and study limitations. SQY51 is an investigational product for Duchenne muscular dystrophy and has not been approved by the relevant regulatory authorities for this use. Its safety and efficacy for this indication have not been established. Statements regarding the potential clinical benefits of SQY51, future clinical development, regulatory milestones or potential commercialization reflect the Company's current expectations and are subject to uncertainties inherent in clinical research and regulatory review. Actual outcomes may differ materially from those anticipated.