NVGCT Nederlandse Vereniging voor Gen- & Celtherapie

ATMP clinical development update week 1&2 2021

RNA interference

Alnylam PHARMACEUTICALS

Vutrisiran met Primary and all Secondary Endpoints at 9 months, with statistically significant improvements relative to placebo in progression of neuropathy, Quality of Life, and gait speed in patients with hereditary transthyretin-mediated amyloidosis (hATTR) with polyneuropathy.

More info at https://investors.alnylam.com/press-release?id=25366

Gene therapy

SAREPTA THERAPEUTICS

SRP-9001 interim update failed the primary endpoint of improvement in functional NSAA total score compared to placebo at 48 weeks post-treatment. Mean micro-dystrophin expression dropped to 28% (compared to 90% in the phase I study) as measured by western blot.

More info at https://investorrelations.sarepta.com/news-releases/news-release-details/sarepta-therapeutics-announces-top-line-results-part-1-study-102

BIOMARIN

Valoctocogene roxaparvovec interim update announces lower Factor VIII levels compared to their phase I/II trial and still has the same durability issues.

More info at https://investors.biomarin.com/2021-01-10-BioMarin-Announces-Positive-Phase-3-Gene-Therapy-Trial-Results-in-Adults-with-Severe-Hemophilia-A-Study-Met-All-Primary-and-Secondary-Efficacy-Endpoints-in-One-Year-Data-Set