

ATMP clinical development update 2021 week 6



Logo, company name

Description automatically generatedAmicus Therapeutics gave the first interim update of AT-GTX-502 in CLN3 Batten disease. No treatment related adverse events were seen (n=4). In the low-dose cohort (n=3), a +0,07 mean yearly rate of change in the Unified Batten Disease Rating Scale was seen compared to +2,86 in a historical control group. Click [here](https://ir.amicusrx.com/news-releases/news-release-details/amicus-therapeutics-announces-positive-initial-clinical-data) for more info.

AVROBIO showed biomarker data of AVR-RD-01 in Fabry disease and AVR-RD-02 in Gaucher disease. Furthermore, all with Cystinosis (n=3) who took AVR-RD-04 remain off treatment of rescue therapy. Click [here](https://investors.avrobio.com/news-releases/news-release-details/avrobio-announces-100-kidney-substrate-reduction-12-months-post) for more info.



Orchard Therapeutics showed positive safety and efficacy data of OTL-203 in Mucopolysaccharidose I (n=8). In the same update, no treatment related adverse events and positive biomarker data were seen in Mucopolysaccharidose IIIA patients who received OTL-201 (n=3). Click [here](https://ir.orchard-tx.com/news-releases/news-release-details/orchard-therapeutics-announces-interim-data-otl-203-showing) for more info.



Abeona Therapeutics showed positive safety and efficacy data of ABO-102 in Mucopolysaccharidose IIIA (n=3). In addition, positive biomarker data was seen of ABO-101 in Mucopolysaccharidose IIIB. Click [here](https://investors.abeonatherapeutics.com/press-releases/detail/198/new-positive-phase-12-interim-data-presented-at) for more info.



Lysogene showed positive biomarker data of LYS-SAF302 in Mucopolysaccharidose IIIA and GM2 and GM3 gangliosides. Click [here](https://www.lysogene.com/lysogene-reports-lys-saf302-biomarker-data-presented-at-the-worldsymposium-2021/) for more info.