Top-Line Data from the MGT009 Phase 1/2 Clinical Study of Botaretigene Sparoparvovec (AAV-RPGR)

Forward Looking Statements

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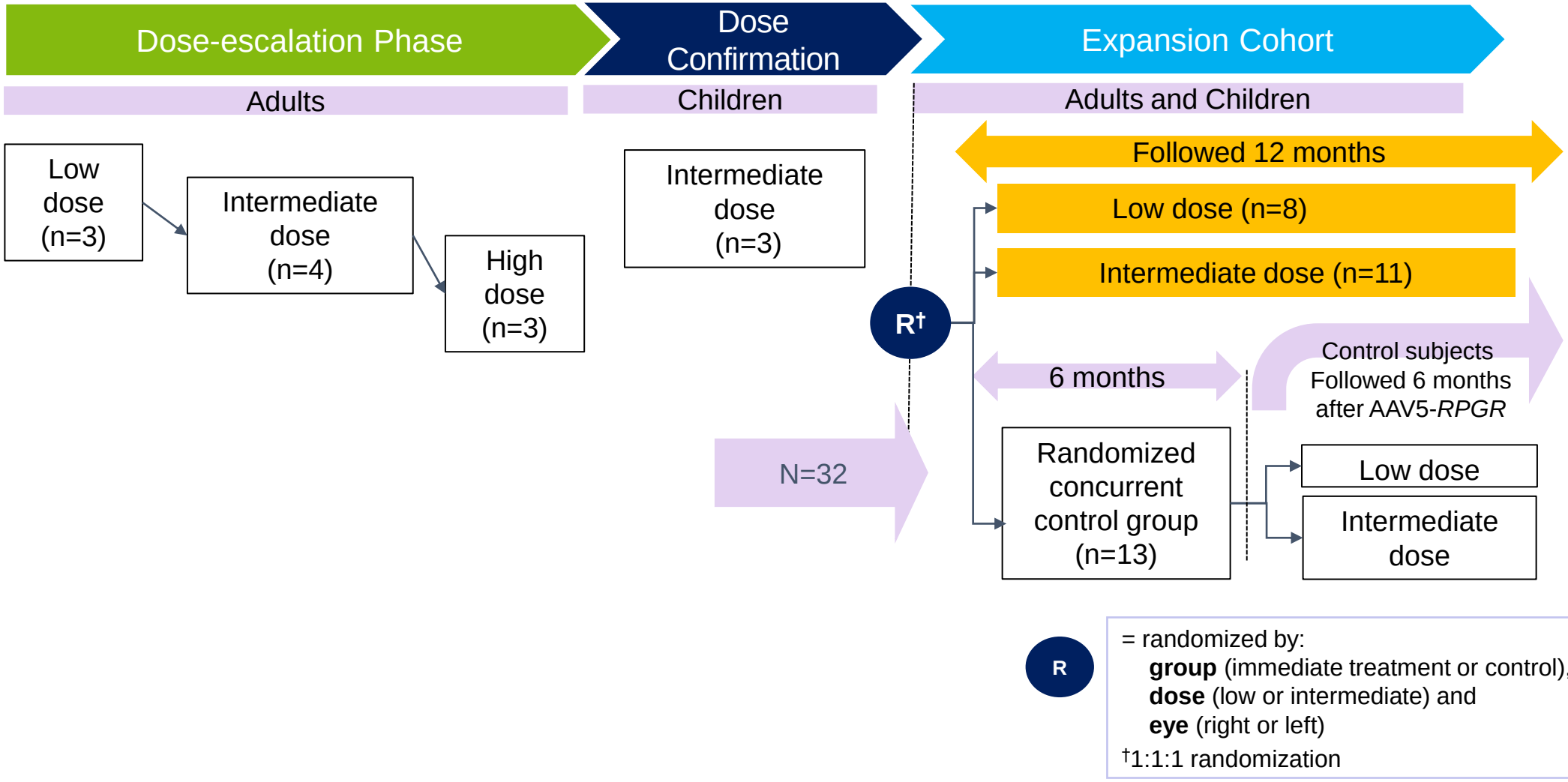
AAV-RPGR: MGT009 Phase 1/2 Study Design

Open-label study of an AAV5-*RPGR* gene therapy (NCT03252847) conducted at 5 sites in the US and UK



Safety Summary

- Botaretigene sparoparvovec is generally safe and well-tolerated.
- Most adverse events (AEs) were related to surgery, were transient and resolved without intervention.
- No dose-limiting events.
- A total of 3 SAEs in the overall study.
- 2 SAEs in the dose-escalation phase of the study (n=10; one retinal tear and one panuveitis in the low dose cohort), which have been previously reported.
- A single additional SAE in the dose expansion phase (n=32). This SAE was increased intraocular pressure and resolved on treatment.
- No SAEs in the pediatric dose confirmation cohort.
- Following the implementation of a modified prophylactic steroid regimen, a reduction in inflammation related AEs was also observed in the expansion phase of the study.

Efficacy Summary

Sensitivity analysis applying the Phase 3 Lumeos eligibility criteria, the following endpoints were significant at 6 months compared to randomized control subjects based on nominal p-values ($p < 0.05$):

Functional Vision:

- Performance in the Visual Mobility Assessment at low levels of illumination (nominal p-values 0.008, 0.005 and 0.008 at lux 16, 4, and 1 respectively).
- Improvement in the extreme lighting domain of the disease related PRO (nominal p-value = 0.020).

Visual Function:

- ETDRS visual acuity (nominal p-value = 0.031).

Retinal Function using Static Perimetry:

- Mean retinal sensitivity in the central 10 degree area of the retina (nominal p value <0.001).

Efficacy Summary continued

Pointwise responder Analysis of Static Perimetry Data:

Responder criteria: at least a 7dB improvement from baseline in 5 or more individual loci, with the same 5 loci showing improvement at 2 timepoints following treatment.

- At 6 months 5/22 (22.7%) of the treated patients met the responder criteria.
- Compared to 0/11 (0%) in the randomized concurrent control arm.
- The responder rate in the treated arm further improved at 12 months to 10/21 (47.6%).

"In the first place, I notice the effect of my mobility in poor lighting when I went to a museum with my friends. And I was able to walk around the museum. I still had my cane, but I didn't actually need it. And three months before the surgery, I could not move through a museum on my own.' 'I was pretty sure that I was going to need to take a few years and learn Braille and then switch to a new career. And this, this treatment is going to allow me to continue being a computer programmer for the foreseeable future, which is just literally the most life changing thing you could ever possibly imagine."

Botaretigene Sparoparvovec Phase 3 Development

- Phase 3 Lumeos study currently enrolling.
- 100% clinical development costs paid by Janssen
- Janssen / MeiraGTx target BLA filing in 2024.
- MeiraGTx receives 20% un-tiered global royalties + performance related milestones.
- MeiraGTx is the commercial manufacturer - leveraging our wholly-owned, end-to-end GMP manufacturing and quality infrastructure to prepare for potential commercial launch by our partner Janssen.