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RETINAL CHANGES AFTER VORETIGENE NEPARVOVEC TREATMENT IN CHILDREN WITH RPE65-RELATED INHERITED RETINAL DYSTROPHY

Oral

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Purpose:

To report quantitative retinal changes assessed by spectral-domain optical coherence tomography (SD-OCT) in children treated with voretigene neparvovec (VN) at a single center in Italy

Methods:

Retrospective review of six consecutive pediatric patients with biallelic RPE65-related dystrophy treated bilaterally with VN. SD-OCT scans were analyzed to extract ETDRS thickness maps of the whole retina and the outer nuclear layer (ONL). Changes in visual function were assessed by best-corrected visual acuity (BCVA) and retinal morphology at Days 30/45 and 180.

Results:

BCVA significantly improved at Day 30/45 and 6 months (both $P < 0.001$). Central foveal retinal thickness and central foveal ONL thickness tended to increase ($6.4 \pm 19.2 \mu\text{m}$; $P = 0.080$ and $3.42 \pm 7.68 \mu\text{m}$; $P = 0.091$, respectively). ONL thickness of the internal ETDRS-ring significantly increased at day 30/45 ($4.7 \pm 8.4 \mu\text{m}$; $P < 0.001$) and day 180 ($5.0 \pm 5.7 \mu\text{m}$; $P = 0.009$). Intra-operative foveal detachment was not associated with a higher function gain in terms of BCVA, but with a mild thinning of foveal ONL after treatment.

Conclusions:

The improvement of BCVA and thickening of the ONL layer suggest that improvement of visual acuity could be related to partial recovery of retinal morphology in the perifoveal ring.