Table 4. Included Studies in Longitudinal Data Analysis

Study	Study type	Diagnostic method of CHM	No. of eyes (patients) in study-level data	No. of eyes (patients) in individual-level data	Age at baseline (years)	Baseline residual RPE area (mm^2)	Mean follow-up duration (years)	Data extraction methods
Aylward et al, 2018[1] Screening Process for Gene Therapy Trials NCT01461213 and NCT02407678	Observational	Clinical†	29 (29)	29 (29)	28.3 ± 9.1	27.91 ± 34.73	1.9	Data were extracted from the text, supplementary Table and Figure 1 (DataThief III software for extracting individual-level data in the figure) in Aylward et al, 2018.
Dimopoulos et al, 2018[2] An Open Label Clinical Trial of Retinal Gene Therapy for Choroideremia NCT02077361	Interventional	Genetics	6 (6)	6 (6)	34.3 ± 5.0	4.54 ± 4.79	2	Data were extracted from the text, Table 1 and Figure 2 (DataThief III software for extracting individual-level data in the figure) in Jolly et al, 2016.
Fischer et al, 2018[3] THOR Study NCT02671539	Interventional	Genetics	6 (6)	6 (6)	54.3 ± 4.1	8.57 ± 10.0	1	Data were extracted from the text and Table 2 in Fischer et al, 2018.
Seitz et al, 2015[4] University Eye Hospital Tübingen	Observational	Clinical†	15 (8)	15 (8)	43.1 ± 13.8	8.87 ± 6.41	4.7	Data were extracted from the text in Seitz et al, 2015. Longitudinal data of individual patients were obtained from the author of the original paper.
Xue et al, 2018[5] Gene Therapy for Blindness Caused by Choroideremia NCT01461213	Interventional	Genetics	12 (12)	12 (12)	47.8 ± 12.4	5.05 ± 3.99	3.5	Data were extracted from the text, supplementary Table 1, supplementary Table 4, and supplementary Figure 5 (DataThief III software for extracting individual-level data in the figure) in Xue et al, 2018

CHM = Choroideremia; RPE = retinal pigment epithelium; THOR = Tübingen Choroideremia Gene Therapy Trial.
* Data reported as mean ± standard deviation
† Genetic confirmation of CHM was performed in some but not all patients.

References:

1. Aylward JW, Xue K, Patricio MI, et al. Retinal degeneration in choroideremia follows an exponential decay function. Ophthalmology 2018;125(7):1122-4.
2. Dimopoulos IS, Hoang SC, Radziwon A, et al. Two-year results after aav2-mediated gene therapy for choroideremia: the alberta experience. Am J Ophthalmol 2018;193:130-42.
3. Fischer MD, Ochakovski GA, Beier B, et al. Changes in retinal sensitivity after gene therapy in choroideremia. Retina 2018.
4. Seitz IP, Zhour A, Kohl S, et al. Multimodal assessment of choroideremia patients defines pre-treatment characteristics. Graefes Arch Clin Exp Ophthalmol 2015;253(12):2143-50.
5. Xue K, Jolly JK, Barnard AR, et al. Beneficial effects on vision in patients undergoing retinal gene therapy for choroideremia. Nat Med 2018;24(10):1507-12.