**Table.** **Clinical trials investigating gene therapy for LCA where the drug is still currently in development.\***

|  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- |
|  | **Drug/vector name-target gene (target gene)** | **Sponsor** | **Phase** | **Clinicaltrials.gov identifier** | **Target n** | **Start date**  **End†/completion date**‡ | **Status  (May 2020)** |
| **Leber congenital amaurosis** | | | | | | | |
| LCA1 | SAR439483 | Sanofi | 1/2 | NCT03920007 | 15 | Sep 2019  Feb 2021/Feb 2022 | Active  (not recruiting) |
| LCA2 | AAV2/5-OPTIRPE65 | MeiraGTx UK II Ltd. | 1/2 | NCT02781480 | 15 | Apr 2016  Dec 2018 | Completed |
| rAAVR-CB-hRPE65 | Applied Genetic Technologies Corp. | 1/2 | NCT00749957 | 12 | Jun 2009  Sept 2014/Sept 2017 | Completed1 |
| LCA10 | AGN-151587/EDIT-1010 (CEP290) | Allergan & Editas Medicine Inc. | 1/2 | NCT03872479 | 18 | Sept 2019  Mar 2024 | Recruiting |
| QR-110  (CEP290) | ProQR Therapeutics | 1/2 | NCT03140969 | 12 | Oct 2017 Dec 2019 | Completed |
| ProQR Therapeutics | 1/2 | NCT03913130 | 11 | May 2019  Mar 2021 | Active  (not recruiting) (long term follow-up of above studies) |
| ProQR Therapeutics | 2/3 | NCT03913143 | 30 | Apr 2019  Dec 2020/Dec 2021 | Recruiting |

\*Excluding ongoing studies in indications for which the therapy is licensed, i.e. voretigene naparvovec for LCA2.

†Estimated primary completion date.

‡If different from estimated end/primary completion date.

1. Weleber RG, Pennesi ME, Wilson DJ, et al. Results at 2 years after gene therapy for RPE65-deficient Leber congenital amaurosis and severe early-childhood-onset retinal dystrophy. *Ophthalmology*.2016;123(7):1606-1620