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# **Recent advance in gene therapy for Leber hereditary optic neuropathy: a systematic review**

**(Supplemental Materials)**

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# Supplemental Material

**Supplemental Material S1.** Search strategy

**Supplemental Material S2.** Assessment of Cochrane Risk of Bias tool (ROB)

**Supplemental Material S3.** Assessment of Newcastle-Ottawa Scale (NOS) tool

**Supplemental Material S1**  
**Search strategy**

# Supplemental Material S1

## Search strategy

### Primary search steps:

1. Leber hereditary optic neuropathy
2. Leber hereditary optic atrophy
3. Leber optic atrophy
4. LHON
5. gene therapy
6. recombinant gene
7. gene delivery
8. (#1 or #2 or #3 or #4) and ( #5 or #6)






















### Final syntax in PubMed (an example):

**((((Leber hereditary optic neuropathy) OR (Leber hereditary optic atrophy)) OR (Leber optic atrophy)) OR (LHON)) AND (((gene therapy) OR (recombinant gene)) OR (gene delivery)) Sort by: Publication Date**

**Supplemental Material S2**  
**Assessment of**  
**Cochrane Risk of Bias**  
**tool (ROB)**

Supplemental Material S2

Assessment of Cochrane Risk of Bias tool (ROB)

	Random sequence generation (selection bias)	Allocation concealment (selection bias)	Blinding of participants and personnel (performance bias)	Blinding of outcome assessment (detection bias)	Incomplete outcome data (attrition bias)	Selective reporting (reporting bias)	Other bias
NCT02064569							
NCT02652767 (RESCUE)							
NCT02652780 (REVERSE)							

## **Supplemental Material S3**

# **Assessment of Newcastle-Ottawa Scale (NOS) tool**

## Supplemental Material S3

### Assessment of Newcastle-Ottawa Scale (NOS) tool

Trials	Selection				Comparability	outcomes			Total quality score
	Representativeness of the exposed cohort	Selection of the non-exposed cohort	Ascertainment of exposure	Demonstration that outcome of interest was not present at start of study	Comparability based on the design or analysis	Assessment of outcome	Was follow-up long enough for outcomes to occur	Adequacy of follow up of cohorts	
NCT02161380	*	*	*		*	*		*	6
NCT03153293	*	*	*		*	*		*	6
NCT03406104	*	*	*		**	*	*	*	7
EUDRACT N° 2013-001405-90.	*	*	*		**	*	*	*	7