# Clinical Trial Search Report

*(Fictitious Example Case)*

**Patient Initials:** A.K.

**Age / Sex:** 54 / Male

**City:** Manchester

**Country:** United Kingdom

**Referring Physician:** Dr. S. Donnelly

**Primary Specialty:** Neurology

## 1. Diagnosis Summary

**Primary Diagnosis:** Amyotrophic Lateral Sclerosis (ALS)

**Subtype / Classification:** Limb-onset ALS

**Date of Diagnosis:** March 2024

**Disease Activity / Severity Scale:** ALSFRS-R: 34/48

**Current Symptoms:** Progressive weakness in right hand, mild dysarthria, fasciculations in upper limbs, exertional dyspnoea.

## 2. Medical Background & History

**Relevant Comorbidities:** Hypertension (controlled), mild asthma

**Past Medical / Surgical History:** Appendectomy (2002), knee arthroscopy (2017)

**Allergies:** Penicillin (rash)

**Medications:** Amlodipine 5 mg OD, Riluzole 50 mg BID

**Functional Status Scale:** ECOG 1 / ALSFRS-R 34

**3. Investigations Reviewed**

**Laboratory Tests**

**Key Blood Tests:** Normal FBC, CK mildly elevated at 390 U/L

**Organ Function:** Renal and hepatic function normal

**Disease Biomarkers:** No genetic biomarkers performed prior to referral

**Imaging**

**MRI Brain/Spine (July 2024):** No structural lesion. Mild corticospinal tract hyperintensity.

**Disease Distribution / Progression Notes:** Symptoms progressing slowly over 9 months.

**Pathology / Biopsy** Not applicable (ALS is a clinical diagnosis).

**Genetic / Molecular Testing**

**Whole Exome Panel:** C9orf72 negative; SOD1 pathogenic variant p.Ala5Val identified.

**Additional variants:** None of known significance.

**4. Treatment History**

**Previous Treatments:**- Riluzole 50 mg twice daily (ongoing)

- Physiotherapy and speech therapy support

**Responses:** Slow progression; speech decline in past 3 months.

**Intolerances / Toxicities:** None reported.

**Reason for Stopping or Changing Therapy:** Not applicable; limited approved therapies available.

## 5. Trial Search Methodology

**Registries Searched:**

ClinicalTrials.gov, EU Clinical Trials Register, ISRCTN, ANZCTR, NIHR Portfolio

**Search Filters:**

ALS, Motor Neuron Disease, SOD1 mutation, antisense therapy, gene-targeted trials, Phase I–III, actively recruiting

**Eligibility Mapping Framework:**-

- Symptom onset < 2 years
- ALSFRS-R 30+
- Confirmed SOD1 mutation
- FVC > 50%
- No tracheostomy or ventilatory dependence
- No severe hepatic/renal impairment

## 6. Summary of Trials Identified

**Total Trials Found:** 18

**Trials by Phase:** Phase I (4), Phase II (9), Phase III (5)

**Trials Open to International Patients:** 6

**Trials with Disease-Specific Mechanism:**- 5 antisense oligonucleotide (ASO) therapies targeting SOD1

- 2 gene-silencing viral-vector approaches
- 3 neuroinflammation modulators

## 7. Detailed Eligibility Matching

**Inclusion Criteria Status**

**Key Disease Criteria Met:**-

- Confirmed ALS diagnosis
- Confirmed SOD1 mutation
- Symptom onset < 24 months
- ALSFRS-R above threshold

**Key Lab/Imaging Criteria Met:**-

- Normal organ function
- No contraindicating MRI findings

**Age / Functional Criteria Met:**- Age 54 (within 18–75 range)

- ECOG 1
- FVC 63% → Meets respiratory criterion

**Exclusion Criteria Status**

**Contraindications:** None identified

**Disallowed Medications:** None

**Organ Dysfunction Exclusion:** None

**Overall Eligibility Conclusion:** Strong candidate for SOD1-targeted trials, especially ASO-based therapies.

**8. Trials the Patient IS Eligible For**

| Trial ID / Title | Mechanism of Action | Phase | Location | Why Eligible | Key Considerations |
|---|---|---|---|---|---|
| **NCT89X11234 – SOD1-ASO** | **Antisense oligonucleo** | **II** | **Paris, France** | **Confirmed** | **Lumbar punctures** |

| | | | | | |
|---|---|---|---|---|---|
| **Therapy "NeuroSilence-1"** | **tide silencing SOD1** | | | **SOD1 mutation, FVC > 50%, onset < 24 months** | **every 4–6 weeks** |
| **NCT55X00821 – AAV-SOD1 Gene Therapy "AAV-SOD1-Modulate"** | **Gene delivery viral vector** | **I/II** | **London, UK** | **Meets all genetic + functional criteria** | **Requires 3-day inpatient stay for infusion** |
| **NCT72X99102 – SOD1 ASO + neuroinflammation modulator** | **Combination approach** | **II** | **Barcelona, Spain** | **Accepted for prior riluzole; mutation-targeted** | **Screening lumbar puncture + EMG required** |

**9. Trials the Patient is NOT Eligible For**

- **NCT11X44321:** Excluded → ALSFRS-R must be ≥ 40
- **NCT98X22109:** Excluded → Requires FVC ≥ 70%
- **NCT77X55342:** Excluded → Excludes patients taking riluzole

**10. Operational Feasibility**

**Travel Requirements:**

Trips to London or Paris feasible; Barcelona more complex.

**Visit Frequency:**

Every 4–6 weeks depending on protocol.

**Required Tests & Local Availability:**-

- Blood tests → Local GP/hospital
- Spirometry → Local hospital
- ECG → Local hospital
- MRI and lumbar puncture → Must be done on site

**International Feasibility:**

Feasible for Paris and London; moderate difficulty for Spain.

**Estimated Costs (Travel Only):**-

- £150–£350 per visit (London)
- £220–£420 per visit (Paris)

**11. Recommendations for Treating Physician**

**Trials Best Aligned with Patient Pathology:**

- **NCT89X11234 (Paris) – ASO targeting SOD1**
- **NCT55X00821 (London) – Viral vector gene therapy**

**Rationale:**

Both directly target the confirmed SOD1 mutation and accept international applicants with current functional status.

**Next Steps:**

1. Contact preferred trial sites
2. Confirm slot availability
3. Provide full medical file and genetic confirmation
4. Arrange baseline tests if needed

**12. Notes for Patient and Family (Lay Summary)**

**Explanation of Mechanism:**

SOD1-targeted clinical trials work by turning off or blocking the faulty gene that contributes to nerve cell damage in ALS.

**Risks & Expectations:**

These are experimental treatments. They may help slow disease progression, but **cannot cure ALS** and benefits are not guaranteed.

**What to Expect Next:**- You and your neurologist choose which trial to pursue

- We help with documents, logistics, questions, and communication with trial sites
- Screening assessments will confirm eligibility