



# ALS Core Clinical Dataset

January 2015

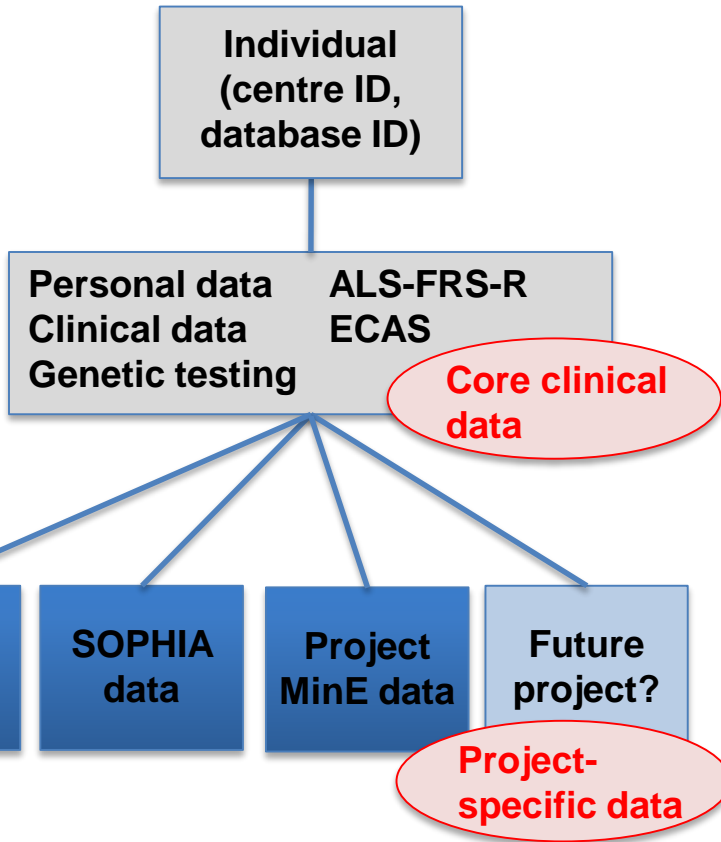


## Background

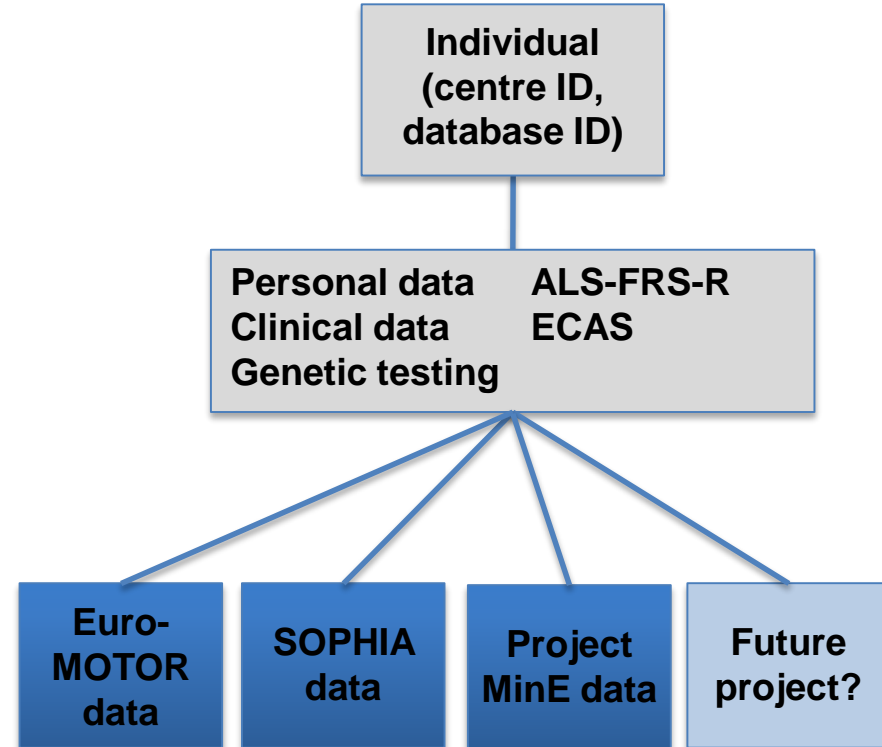
- A standard core clinical dataset has been defined with input from ALS researchers internationally; a minimal dataset that should be obtained for all ALS patients (and controls) that participate in ALS research projects, such as EuroMOTOR ([www.euromotorproject.eu](http://www.euromotorproject.eu)), SOPHIA ([www.sophiaproject.eu](http://www.sophiaproject.eu)), Project MinE ([www.projectmine.com](http://www.projectmine.com)), and many other and future projects
- A template to collect this data has been created in the international ALS database that has been set up for several European projects, using Progeny database software
- Core clinical data is defined as the minimal data to be collected on ALS patients for various studies
- The dataset is fairly short and simple (only a few key items (=“minimal”)), as the goal is compliance in data entry, and not an over-detailed dataset
- The core clinical dataset is clearly defined, to enhance compliance in data entry
- More complex clinical data will be part of separate projects (e.g. what data best stratifies patients, Awaji validation, etc.)

# European database set-up

## ALS CENTER 1



## ALS CENTER 2





## ALS core clinical data - overview

- Date of birth
- Patient/control
- Contact details
- Gender
- Diagnosis
- Date of diagnosis
- Co-morbidity
- El Escorial (plus suspected)
- Date of onset
- Site of onset
- First symptoms
- Respiratory function tests (FVC, SNIP)
- Familial history ALS/MND and FTD/Dementia
- Gastrostomy
- Endpoints: Date of death/tracheostomy/NIV >23 hrs/day/If none, date of checking
- Genetic testing
- ALSFRS-R
- ECAS



## ALS core clinical data – overview (2)

The core clinical data is divided in 4 different categories:

- Personal data
- Clinical data
- Genetic testing
- ALS-FRS-R
- ECAS cognitive & behaviour



## Personal data

- Date of birth (date field)
- Date of death (date field)
- Sex (gender at birth): male, female (dropdown box)
- Patient/control: patient, control (dropdown box)
- Notes (free text)
- ID numbers
- The Personal data can also include identifying personal data if preferred by the research center: First name, Male name prefix, Male name, Female name prefix, Female name, Address, ZIP code, Town, Country, Telephone, Email

*(This way of recording the last name data was deemed most general for Europe, as we're dealing with different countries and there are different standards for use of last names.)*



## Clinical data

- Date of diagnosis (date field)
  - Fill in the date that the diagnosis of ALS was discussed with the patient/relatives
- Diagnosis
  - Choose the diagnosis from the available options: ALS, PMA, PLS, Other namely... (dropdown box)
  - Other diagnoses namely: (free text)
- Co-morbidity

Two co-morbidities can be indicated per individual

  - Choose the co-morbidity from the available options: Cancer, Cardiovascular disease, Parkinson's disease, Psychiatric disorder(s), Auto-immune disorder(s), Other namely..., Absent/unknown (dropdown box)
  - Co-morbidity, other: (free text)



## Clinical data (2)

- El Escorial table:
    - El Escorial categories: Suspected ALS, Possible ALS, Probable ALS lab supported, Probable ALS, Definite ALS (dropdown box)  
*(The dropdown options for El Escorial contain both the old and the revised El Escorial options. This way data of patients assessed with the old El Escorial criteria can be added just like data of patients with the revised El Escorial criteria, in one data field. This simplifies the database set-up and data analysis.)*
    - El Escorial date (date field)
- If a case of suspected ALS (= PMA) progresses to ALS, the diagnostic pathway can be traced





## Clinical data (3)

- Date of onset (date field)

Reported by the patient when:

- Limb symptoms: muscle weakness was noticed
- Speech/swallowing difficulties
- Respiratory symptoms: dyspnea was noticed
- Generalized symptoms

In most cases middle of period:

- E.g. January 2014 -> 15 January 2014
- In 2014 -> 1 July 2014
- Winter (spring, summer, autumn) 2014 -> 1 February 2014 (May, August, November)

Except for:

- Beginning of 2014 -> 1 January 2014
- End of 2014 -> 31 December 2014
- Patient seen on 23 July 2014: “Five months ago...” -> 23 February 2014
- Patient knows exact date

Other definitions are fine, as long as it is applied consistently to one cohort -> Make this explicit and make some sort of a SOP!



## Clinical data (4)

- Site of onset (dropdown box): Bulbar, Spinal, Thoracic/respiratory, Generalized (dropdown box)  
*(E.g. choose Generalised if both swallowing issues and weakness in leg are reported at the same time)*
- If Spinal, then select: Arms, Legs (dropdown box); and select: left, right (dropdown box)
- Other first symptoms:
  - Weight loss (checkbox)  
*(if >10% weight loss in 6 months prior to time of disease onset as defined by date of onset symptoms)*
  - Cognitive or behavioural (checkbox)
  - *(Based on the neurologist's clinical opinion on examination at first visit/time of diagnosis, based on conversation with the patient and caregiver about the disease timeline. In terms of cognitive/behavioural changes, look out for mention of marked personality changes that clearly predate other motor symptom onset and are thought to be part of the MND disease process (rather than being co-morbid). Not exact science, somewhat dependent on the information the patient/caregiver provides!)*



## Clinical data (5)

- Forced Vital Capacity table (preferred):
  - Date FVC (date field)
  - FVC (litres) (open text field)
  - FVC (% of predicted) (open text field)
  - Testing not performed or not feasible: Not tested, Not feasible (dropdown box)Follow-up data possible by adding multiple rows
  
- SNIP table (optional):
  - Date SNIP (date field)
  - SNIP (cm H<sub>2</sub>O) (open text field)
  - Testing not performed or not feasible: Not tested, Not feasible (dropdown box)Follow-up data possible by adding multiple rows



## Clinical data (6)

Family history - Number of affected family members with ALS/MND and FTD/Dementia

- First degree (Parents, brothers, sister, sons, daughters):
  - ALS/MND (number field)
  - FTD/Dementia (number field)
- Other family members:
  - ALS/MND (number field)
  - FTD/Dementia (number field)
- Notes family history (free text field)



## Clinical data (7)

### Gastrostomy

- PEG: yes, no, unknown (dropdown box)
- Date of start PEG (date field)
- RIG: yes, no, unknown (dropdown box)
- Date of start RIG (date field)
- PIG: yes, no, unknown (dropdown box)
- Date of start PIG (date field)

### Endpoints

- Date of death (date field)
- If tracheostomy, date of tracheostomy (date field)
- If >23h NIV/day, date of endpoint (date field)
- Date of checking endpoint (if not deceased, no tracheostomy and no >23h NIV/day) (date field)



## Genetic testing

- Which ALS genes were tested: Large range of genes\* (dropdown box per gene: yes, no)
- Was a mutation shown in an ALS gene : Large range of genes\* (dropdown box per gene: yes, no)
- Notes per gene\* (free text field - 'what mutation?')
- Possibility to indicate another tested gene using the open text field at the bottom of the list

\* SOD1, TARDP/TDP43, FUS, C9orf72, VABP, MAPT, NIPA1, hnRNPA1/A2/B1, FIG4, GRN, UNC13A, UBQLN2, OPTN, VCP, PLP1, SPG4, SMN, PON, SETX, ANG, AR\_repeat, ATXN2\_repeat, CHMP2B



## ALS-FRS-R

- Date of ALS-FRS-R (date field)
- ALS-FRS-R 12 subscores (number fields)
- ALS-FRS-R total score (number field)
- ALS-FRS-R total score (automatically calculated)

*(If no ALS-FRS-R subscores are available, then fill in the total score at the end of the table only. The data field for ALS-FRS-R total score (automatically calculated) will fill automatically with either this manually input score or the score based on the subscores that were put in. This is also the data fields wich should be used when extracting the data/running queries.)*

- Rater name (free text field)



## ECAS cognitive

**Use ECAS guidelines for more details on the data collected for ECAS!**

ECAS-specific clinical data

- ECAS\_ID (free text field)
- ECAS: Date of testing (date field)
- Read or write problems (free text field)
  - Specify read or write problems
- Highest Degree Attained: Grade school (grades 1-6), High school diploma, Technical or trade school diploma, University degree, Graduate school (PhD), None (dropdown box)
- NIV: Yes/No (dropdown box)
  - NIV in general (irrespective of the endpoint NIV >23h/day)
- Date of NIV (date field)





## ECAS cognitive (2)

### ECAS additional clinical data

- Regions at testing: Bulbar, Upper limbs, Lower limbs, Respiratory, Combinations of these regions (dropdown box)
- Handedness: Left, Right, Ambidextrous (dropdown box)
- Psychiatric disorder (free text field)
- Antidepressant/riluzole use: yes, no (dropdown box)

### ALS (non-)specific ECAS scores

- All numerical text fields where scores can be entered

**Use ECAS guidelines for more details on the data collected for ECAS!**



## ECAS behaviour

### Carer interview – Behaviour screen & symptoms

- 5 behavioural characteristics are questioned
  - 1-3 questions per characteristic
  - All dropdown boxes: yes, no, don't know
  - If 'Yes' in one of the questions under one characteristic -> Tick the box on the right-hand side
- Fill in number of total 'Yes'-scored questions and ticked boxes

### Carer interview – Psychosis screen

- All dropdown boxes: yes, no, don't know
- If 'Yes' -> Specify psychotic symptom in the open text field on the right
- Fill in number of total 'Yes'-scored questions

### Onset and duration of symptoms

- Questions 1a, 1b, 1c & 2 are data fields with dropdown options Yes/No
- Question 3 is an open text field

**Use ECAS guidelines for more details on the data collected for ECAS!**