BIG Data, Collaborations and Patient Centricity in Rare Diseases

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May 18, 2017
Patients == Research Participant
Patients == Research Participant
Patients == Research Participant

- Clinical Trials
- EHR
- Nat History
- Bio Banks
- Image Banks
- PRO
- Genetics
- Omics
- Cell Lines
- Mobile Apps
- PRO-ACT™
- PharmaENGINE™
- PRO-ACE™
- NeuroGUID™
- NeuroBANK™
Patients == Research Participant
Welcome to the Pooled Resource Open-Access ALS Clinical Trials Database

PRO-ACT Platform

PRO-ACT provides users with easy access to:

- Over 10,700 fully de-identified clinical patient records
- Placebo and treatment-arm data from 23 Phase II/III clinical trials
- Demographic, lab, medical and family history, and other data elements
- More than 10 million longitudinally collected data points

In Dec. 2015, 6 new ALS clinical trials were merged into the PRO-ACT database.

We thank everyone who has contributed to the creation of PRO-ACT including the companies, the researchers, and most of all the ALS patients, past and present, around the world, to whom the PRO-ACT initiative is dedicated.

In June 22-September 30, 2015, Prize4Life, Sage Bionetwork and The DREAM Challenges launched the ALS Stratification Challenge. The Challenge utilized the PRO-ACT database, as well as data from the Irish National ALS register and The Piemonte and Valle d’Aosta Register for ALS. It asks solvers to identify meaningful sub-groups of ALS patients. Challenge information and solutions are available on the Challenge website on Sage Bionetwork’s Synapse Platform. The Challenge data is available here.
# PRO-ACT Platform: Data

<table>
<thead>
<tr>
<th>Demographics</th>
<th>10,723</th>
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<tbody>
<tr>
<td>Female</td>
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<tr>
<td>Male</td>
<td>6,464</td>
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<table>
<thead>
<tr>
<th>Vital capacity</th>
<th>55,753</th>
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<tbody>
<tr>
<td>FVC</td>
<td>46,223</td>
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<tr>
<td>SVC</td>
<td>9,530</td>
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<table>
<thead>
<tr>
<th>ALSFRS</th>
<th>68,041</th>
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<tbody>
<tr>
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<td>36,469</td>
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<tr>
<td>ALSFRS-R</td>
<td>31,572</td>
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<table>
<thead>
<tr>
<th>Labs</th>
<th>2,485,021</th>
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<tbody>
<tr>
<td>Unique lab tests</td>
<td>268</td>
</tr>
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</table>

<table>
<thead>
<tr>
<th>Top 10 labs:</th>
<th>Records</th>
</tr>
</thead>
<tbody>
<tr>
<td>Lymphocytes</td>
<td>79,178</td>
</tr>
<tr>
<td>Monocytes</td>
<td>79,171</td>
</tr>
<tr>
<td>Eosinophils</td>
<td>79,140</td>
</tr>
<tr>
<td>Basophils</td>
<td>79,091</td>
</tr>
<tr>
<td>ALT(SGPT)</td>
<td>74,705</td>
</tr>
<tr>
<td>AST(SGOT)</td>
<td>71,449</td>
</tr>
<tr>
<td>Red Blood Cells</td>
<td>70,132</td>
</tr>
<tr>
<td>Neutrophils</td>
<td>66,238</td>
</tr>
<tr>
<td>Bilirubin (total)</td>
<td>65,920</td>
</tr>
<tr>
<td>Hemoglobin</td>
<td>65,104</td>
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<table>
<thead>
<tr>
<th>Vital Signs</th>
<th>340,513</th>
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</thead>
<tbody>
<tr>
<td>Blood Pressure</td>
<td>93,753</td>
</tr>
<tr>
<td>Height</td>
<td>9,845</td>
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<tr>
<td>Pulse</td>
<td>93,498</td>
</tr>
<tr>
<td>Respiratory Rate</td>
<td>53,165</td>
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<tr>
<td>Temperature</td>
<td>31,394</td>
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<tr>
<td>Weight</td>
<td>58,858</td>
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</table>
### Adverse events

<table>
<thead>
<tr>
<th>Preferred terms (top 10 by # of records)</th>
<th>Records</th>
</tr>
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<tbody>
<tr>
<td>Constipation</td>
<td>1,720</td>
</tr>
<tr>
<td>Death</td>
<td>1,577</td>
</tr>
<tr>
<td>Diarrhea</td>
<td>1,629</td>
</tr>
<tr>
<td>Dizziness</td>
<td>1,333</td>
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<tr>
<td>Fatigue</td>
<td>1,532</td>
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<tr>
<td>Headache</td>
<td>2,018</td>
</tr>
<tr>
<td>Insomnia</td>
<td>1,338</td>
</tr>
<tr>
<td>Muscular weakness</td>
<td>1,642</td>
</tr>
<tr>
<td>Nausea</td>
<td>2,454</td>
</tr>
</tbody>
</table>

### Concomitant Meds

| Unique Drugs (WHO-Drug) | 6,569 |

### Top 10 drugs

<table>
<thead>
<tr>
<th>Records</th>
</tr>
</thead>
<tbody>
<tr>
<td>VITAMIN E 3,219</td>
</tr>
<tr>
<td>BACLOFEN 2,746</td>
</tr>
<tr>
<td>RILUZOLE 2,525</td>
</tr>
<tr>
<td>VITAMIN C 2,405</td>
</tr>
<tr>
<td>MULTIVITAMIN 1,786</td>
</tr>
<tr>
<td>RILUTEK 1,592</td>
</tr>
<tr>
<td>AMITRIPTYLINE 1,375</td>
</tr>
<tr>
<td>IBUPROFEN 1,157</td>
</tr>
<tr>
<td>CREATINE 1,085</td>
</tr>
<tr>
<td>COENZYME Q10 1,008</td>
</tr>
</tbody>
</table>
Patients == Research Participant
PRO-ACE™

The Pooled Resource Open-Access ALS Clinical Research Platform

PRO-ACE™ facilitates contribution of ALS clinical & clinical research data

- 20 research studies
- 10 clinical datasets
- 8 countries

PRO-ACE™ aggregates data from multiple sources and maps to common data elements

- 15,000 patients records
- 500 common data elements
- 30 million data points shared

PRO-ACE™ accelerates scientific advancement clinical understanding in ALS

- 5,000 downloads
- 5 Dx models
- 1 goal

www.PRO-ACE.org
NeuroBANK™ Platform

Patients == Research Participant
What is NeuroGUID

The Neurological Global Unique Identifier (NeuroGUID) is a universal patient identifier that allows researchers in Neurological and other Central Nervous System diseases to share data specific to a study participant without exposing personally identifiable information (PII). It also enables matching participants across studies and research data repositories. In other words, NeuroGUID:

- Allows aggregation of data from the same patient from multiple studies, regardless of where and when that data was collected
- Prevents a single patient from being created multiple times in the same research database, in case she/he participates in a study at different sites or in multiple studies at the same time
- Enables a researcher to combine patient information with data originating from other sources.
# NeuroBANK™ ALS Core Studies

| # | Projects                                      | CLN | CGN | NTR | BFL | PMT | IMG | DNA | RNA | iPSC | EHR | PRO | MOB |
|---|----------------------------------------------|-----|-----|-----|-----|-----|-----|-----|-----|-----|-----|-----|-----|-----|
| 1 | C9orf72                                      | Y   |     |     |     |     |     | Y   | Y   | Y   |     |     |     |     |
| 2 | Iron Horse                                   | Y   |     |     |     |     |     |     |     |     |     |     |     |     |
| 3 | TRACK ALS                                    | Y   | Y   | Y   | Y   | Y   | Y   |     |     |     |     |     |     |     |
| 4 | VITALITY                                     | Y   |     |     |     |     | Y   |     |     |     |     |     |     |     |
| 5 | Answer ALS                                   | Y   | Y   |     |     | Y   | Y   | Y   | Y   |     |     |     |     |     |
| 6 | GTAC                                         | Y   | Y   | Y   | Y   | Y   |     |     |     |     |     |     |     |     |
| 7 | Postmortem Tissue Bank                       | Y   |     | Y   |     |     |     |     | Y   |     |     |     |     |     |
| 8 | Natural History in ALS                       | Y   | Y   |     |     |     |     | Y   |     | Y   |     |     |     |     |
| 9 | EAT MORE                                     | Y   |     |     |     |     |     |     |     |     |     |     |     |     |
| 10| CABB                                         | Y   | Y   |     |     |     |     |     |     |     |     |     |     |     |
| 11| Microbiome in ALS                            | Y   | Y   |     |     |     |     |     |     |     |     |     |     |     |
| 12| UMN Registry                                 | Y   |     |     |     |     |     |     |     |     |     |     |     |     |
| 13| Lunasin ALS Reversals                        | Y   |     |     |     |     |     |     |     |     |     |     |     |     |
| 14| Olfactory Deficits in Neurological Dx        | Y   |     |     |     |     |     |     |     |     |     |     |     |     |
| 15| Cognitive-behavioral Symptoms (ALS/FTD)      | Y   | Y   |     |     |     |     |     | Y   |     |     |     |     |     |
| 16| Fluid Biomarkers/Deep Phenotyping            | Y   | Y   | Y   |     |     |     |     |     |     |     |     |     |     |
| 17| Providence ALS Patient Registry              | Y   | Y   |     |     |     |     |     |     |     |     |     |     |     |
| 18| C9orf72 Genomic Assessment Protocol          | Y   | Y   |     |     |     |     |     | Y   |     |     |     |     |     |
| 19| Target ALS Fluid Biomarkers                 | Y   |     | Y   | Y   |     |     |     |     |     |     |     |     |     |
| 20| Symptom Monitoring in Real Tim               | Y   | Y   |     |     |     |     |     |     |     |     |     |     |     |
| 21| P4L Mobile ALS App                           | Y   |     |     |     |     |     |     |     |     |     |     |     |     |
| 22| TOTAL STUDIES                                | 21  | 6   | 2   | 14  | 2   | 3   | 10  | 7   | 5   | 2   | 4   | 3   |     |

**Legend:**
- **CLN:** Clinical and phenotypical data
- **CGH:** Cognitive assessment
- **NTR:** Dietary/Nutrition
- **BFL:** Biofluids (CSF, Plasma, Serum)
- **PMT:** Post-mortem tissues
- **IMG:** Imaging
- **DNA:** DNA (WGS)
- **RNA:** RNA
- **iPSC:** Cell lines
- **EHR:** EHR Integration
- **PRO:** Patient-reported outcomes (PRO)
- **MOB:** Integration with mobile apps

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NeuroBANK™ ALS Ecosystem

- **22** ALS-related research projects with total enrollment goal of 6,000 (2680)
- **96** participating sites from **17** countries with **313** NeuroBANK-trained and certified personnel
- **2916** generated Neurological Global Unique Identifiers (NGUIDs)
- **205** volunteers participate in more than one study
- **5** volunteers participate in **4 (!)** and **2 in 5 (!!!)** research projects
- **880** DNAs samples collected for WGS
- **537** PBMC samples collected for cell lines generation
- **99** cases of postmortem tissue collection (>6K tissue samples)
- **34K+** vials of biofluids are in the Virtual BioBank and available for analyses

<table>
<thead>
<tr>
<th>Biofluid</th>
<th># Participants</th>
<th># Vials</th>
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<tbody>
<tr>
<td>DNA</td>
<td>880</td>
<td>3483</td>
</tr>
<tr>
<td>CSF</td>
<td>278</td>
<td>3763</td>
</tr>
<tr>
<td>Plasma</td>
<td>772</td>
<td>20222</td>
</tr>
<tr>
<td>Serum</td>
<td>802</td>
<td>7685</td>
</tr>
<tr>
<td>PBMC (iPSC)</td>
<td>569</td>
<td>1707</td>
</tr>
<tr>
<td>Post-mortem tissues</td>
<td>99 cases</td>
<td>~6K tissues</td>
</tr>
</tbody>
</table>
# NeuroBANK™ Nat. Hx Studies

| #  | Projects                                      | CLN | CGN | NTR | BFL | PMT | IMG | DNA | RNA | iPSC | EHR  | PRO  | MOB |
|----|----------------------------------------------|-----|-----|-----|-----|-----|-----|-----|-----|-----|------|------|------|-----|
| 1  | Natural History in C9orf72                   | Y   |     | Y   |     | Y   |     |     | Y   |     | Y    |      |      |     |
| 2  | Natural History in ALS                       | Y   | Y   |     | Y   |     | Y   | Y   |     | Y   | Y    | Y    |      |
| 3  | Providence ALS Patient Registry              | Y   | Y   |     |     |     |     |     |     |     |     |      |      |
| 4  | UMN Registry                                 | Y   |     | Y   |     |     |     |     |     |     |      |      |
| 5  | Natural History in ALD                       | Y   |     |     |     |     |     |     |     | Y   |      |      |
| 6  | Natural History in Canavan Disease           | Y   |     |     |     |     |     |     | Y   |     |      |      |
| 7  | Natural History in DYT3                      | Y   |     | Y   |     |     |     |     |     |     |      |
| 8  | Natural History in LOTS                      | Y   |     |     |     |     |     |     |     |     |      |
| 9  | Natural History in MLD                       | Y   |     | Y   |     |     |     |     |     |     |      |

## CLN, CGN, NTR, BFL, PMT, IMG, DNA, RNA, iPSC, EHR, PRO, MOB

<table>
<thead>
<tr>
<th>CLN</th>
<th>CGH</th>
<th>NTR</th>
<th>BFL</th>
<th>PMT</th>
<th>IMG</th>
<th>DNA</th>
<th>RNA</th>
<th>iPSC</th>
<th>EHR</th>
<th>PRO</th>
<th>MOB</th>
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</thead>
<tbody>
<tr>
<td>Clinical</td>
<td>Cognitive assessment</td>
<td>Dietary/Nutrition</td>
<td>Biofluids</td>
<td>Post-mortem tissues</td>
<td>Imaging</td>
<td>DNA (WGS)</td>
<td>RNA</td>
<td>Cell lines</td>
<td>Integration with mobile apps</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
NeuroBANK Biorepository Label

Study name: GLIANHX
Sample type: C
Timepoint ID: Collection# 3
Volume checkbox: 

Vial ID: 23017519
Barcode: 

Patient ID: NEUASDASDAS
(Use GUID whenever available)

( check if sample is less than volume intended, e.g. if 0.15ml of plasma stored rather than 0.5ml)
Age of Precision Medicine

ANSWER ALS

The Project to End ALS Now

GTAC

Genomic Translation for ALS Care

New York Genome Center
NeuroBANK™ ALS Ecosystem
Patients == Research Participant

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- PRO-ACE™
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Patients == Research Participant
ALS CORE (Community Online Research Engagement)

Working together to fight Amyotrophic Lateral Sclerosis

Who knows the most about motor neuron disease, including amyotrophic lateral sclerosis?

You do.

Yes, there is much to be known about the genetics, biochemistry, and environmental factors that play a role in ALS. Yes, there are many experts and subspecialists. But no one experiences the burden of the disease as you do. No one knows the frustration of the lengthy work-up for diagnosis as you do. No one knows the despair of searching for successful treatments beyond the few treatments available today.

No one else understands the toll this disease can take upon families, relationships and friends as you do.

A Collaborative Effort to Fight Amyotrophic Lateral Sclerosis (ALS)

It's a radical idea: patients, families, doctors, and researchers working together as equal partners.

When you join ALS CORE you'll be part of a global effort to fight ALS. You'll work with doctors and researchers to shape the direction of research and care. For perhaps the first time in the history of this disease, your experiences, your concerns, and your ideas will take center stage.
ALS CORE (Community Online Research Engagement)

Working together to fight Amyotrophic Lateral Sclerosis

Home | Join Now | Available Studies | FAQ | Resources | Contact | Login

Join Now to Participate in Research

It Takes Just a Few Minutes to Join . . .

All you have to do to join the fight to end ALS is create a username and password.

You can join the ALS CORE using your computer, tablet, or smartphone—whichever you prefer.

And When You're a Member, You Can . . .

• Join existing research studies
• Be a part of a collaborative effort to find new, better treatments and a cure for ALS

Sign Me Up >>
**ALS CORE (Community Online Research Engagement)**

Working together to fight Amyotrophic Lateral Sclerosis

Welcome george@fanzcall.com

About My Disease

Demographics
Motor Neuron Disease History (ALS History)
Family History
Past Medical History
Medications
Interventions

Microbiome Assessment in People with ALS
Motor Neuron Disease History (ALS History)

Please complete the following:

Where was diagnosis made: *
- Outside Center
- ALS center

Date of diagnosis: *
- May 1978

Date of symptom onset: *
- Mar 1980

Defined as first noticeable weakness in a limb, difficulty with speech/swallow, shortness of breath

Site of symptom onset:
- Bulbar
  - Speech
  - Swallowing
- Axial
  - Neck
  - Trunk
- Limb
- Generalized*

* Onset in more than one region. Unable to distinctly identify the specific sites of onset.

[Buttons]: CANCEL  SAVE
**ALS CORE (Community Online Research Engagement)**

Working together to fight Amyotrophic Lateral Sclerosis

Welcome george@fanzcall.com

**Family History**

<table>
<thead>
<tr>
<th>Relative</th>
<th>Heredity</th>
<th>Gender</th>
<th>GUID of relative</th>
</tr>
</thead>
<tbody>
<tr>
<td>Mother</td>
<td></td>
<td>Female</td>
<td>vieweditdelete</td>
</tr>
</tbody>
</table>

ADD NEW RECORD  CONFIRM

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Past Medical History

Please complete the following:

Have you been diagnosed with a disorder affecting your head, eyes, ears, nose or throat?
(e.g. cataracts, macular degeneration, hearing loss, sinus disease, tonsil removal, wisdom teeth removal)*

- Yes
- No

Have you been diagnosed with a disorder affecting your lungs or ability to breathe?
(e.g. asthma, COPD, pneumonia, lung cancer)*

- Yes
- No

Have you been diagnosed with a disorder affecting your heart or vascular system?
(e.g. heart disease, heart attack, high blood pressure, high cholesterol, peripheral vascular disease)*

- Yes
- No

Have you been diagnosed with a disorder affecting your digestive system or liver?
(e.g. chronic constipation, chronic diarrhea, GERD or acid reflux, stomach or intestinal ulcer, stomach or colon cancer, inflammatory bowel disease, irritable bowel syndrome, diverticulitis, cirrhosis, hepatitis, non-alcoholic fatty liver disease)*

- Yes
- No

Have you been diagnosed with a disorder affecting your urinary or reproductive system?
Interventions

Please complete the following.

Are you currently using non-invasive ventilation (NIV)?  
- [ ] Yes  
- [x] No

Have you had a feeding tube placed?  
- [ ] Yes  
- [x] No

Have you had a diaphragmatic pacing system (DPS) device placed?  
- [ ] Yes  
- [x] No

Have you had a tracheostomy performed?  
- [ ] Yes  
- [x] No

Are you currently managed on permanent assisted ventilation?  
- [ ] Yes  
- [x] No

CANCEL  SAVE
ALS CORE (Community Online Research Engagement)

Working together to fight Amyotrophic Lateral Sclerosis

Available Studies

ALS Data Repository
Microbiome Assessment in People with ALS (MAP ALS)

Join Now

Coming Together to Find a Cure

Copyright © 2015-2017, ALS Association All rights reserved.
Microbiome Assessment in People with ALS Study Opportunities

The Neurological Clinical Research Institute at Massachusetts General Hospital, under the direction of Dr. Katharine Nicholson, has developed a data repository platform with a patient portal, "ALS Community Online Research Engagement (ALS CORE)."

This website will allow for optimized data collection and expedite the screening process for research studies. Participants will be allowed to consent to research studies online and enroll in studies with remote recruiting and screening. The goal of this data repository is to capture clinical and research data from patients for individual projects. Utilizing the portal will allow participants to contribute their clinical data through questionnaires more feasibly and visualize their own health information in intuitive and helpful ways.

You must be at least 18 years of age and be able to answer questions about your medical and family history. The ALS CORE seeks to actively engage adults with motor neuron diseases (MND), Asymptomatic ALS gene carriers, and people who do not have ALS or positive gene status. If interested, please contact asresearch@partners.org for more information.

If you are interested in reviewing the consent form for this study, please click here.

Do you have questions for study staff about the study?

- Yes
- No
Microbiome Assessment in People with ALS (MAP ALS)

Consent to Participate in a Research Project

Description of Subject Population: Adults with ALS or genetic mutations

About this consent form

Please read this form carefully. It tells you important information about a research study. People who agree to take part in research studies are called "subjects." This term will be used throughout this consent form.

If you have any questions about the research or about this form, please ask us. Taking part in this research study is up to you. If you decide to take part in this research study, you must indicate on this form that you want to take part. It is advised that you print out or save an online copy of this consent form for your records.

Why is this research study being done?

We would like to find out what unique changes can be observed in the stool of people with Amyotrophic Lateral Sclerosis (ALS). These changes are called biomarkers. Biomarkers are:

- Adults
- Adults unable to provide consent due to their medical condition
ALS CORE (Community Online Research Engagement)

Working together to fight Amyotrophic Lateral Sclerosis

About My Disease

Demographics
Family History
Past Medical History
Medications

Click button below to see Additional ALS CORE Study Opportunities

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**ALS CORE** *(Community Online Research Engagement)*

Working together to fight Amyotrophic Lateral Sclerosis

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**Welcome george@fanzcall.com**

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⚠️ Please update expired Microbiome Assessment in People with ALS forms

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Microbiome Assessment in People with ALS Forms

- ALS Functional Rating Scale – Revised (ALSFRS-R)
- Constipation Questionnaire (Wexner)
- Questions about Bowel Function

Data is old. Re-enter

Please verify ALS Data Repository About My Health Forms are up to date

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ALS Functional Rating Scale – Revised (ALSFRS-R)

1. Speech
   - 4 = Normal speech processes
   - 3 = Detectable speech disturbances
   - 2 = Intelligible with repeating
   - 1 = Speech combined with nonvocal communication
   - 0 = Loss of useful speech

2. Salivation
   - 4 = Normal
   - 3 = Slight but definite excess of saliva in mouth, may have nighttime drooling
   - 2 = Moderately excessive saliva, may have minimal drooling
   - 1 = Marked excess of saliva with some drooling
   - 0 = Marked drooling, requires constant tissue or handkerchief

3. Swallowing
   - 4 = Normal eating habits
   - 3 = Early eating problems - occasional choking
   - 2 = Marked swallowing difficulties
Constipation Questionnaire (Wexner)

Please complete the following:

1. Frequency: How often are your bowel movements?*
   - 0 = 1-2 times per 1-2 days
   - 1 = 2 times per week
   - 2 = Once per week
   - 3 = Less than once per week
   - 4 = Less than once per month

2. Difficulty: Are your bowel movements ever painful to evacuate?*
   - 0 = Never
   - 1 = Rarely
   - 2 = Sometimes
   - 3 = Usually
   - 4 = Always

3. Completeness: Do you ever feel incomplete evacuation with your bowel movements?*
   - 0 = Never
   - 1 = Rarely
   - 2 = Sometimes
   - 3 = Usually
   - 4 = Always

4. Pain: Do you ever feel abdominal pain with your bowel movements?*
   - 0 = Never
   - 1 = Rarely
   - 2 = Sometimes
   - 3 = Usually
Constipation Questionnaire (Wexner)

Please complete the following:

1. Frequency: How often do you have a bowel movement?
   - 0 = 1-2 times per week
   - 1 = 2 times per week
   - 2 = Once per week
   - 3 = Less than once per week
   - 4 = Less than once a month

2. Difficulty: Are your bowel movements hard?
   - 0 = Never
   - 1 = Rarely
   - 2 = Sometimes
   - 3 = Usually
   - 4 = Always

3. Completeness: Do you feel that your bowel movements are complete?
   - 0 = Never
   - 1 = Rarely
   - 2 = Sometimes
   - 3 = Usually
   - 4 = Always

4. Pain: Do you ever feel pain during or after a bowel movement?
   - 0 = Never
   - 1 = Rarely
   - 2 = Sometimes
   - 3 = Usually

Questions about Bowel Function

1. In the last 3 months, did you have discomfort or pain anywhere in your abdomen? *
   - Yes
   - No

2. Did this discomfort or pain occur more than 2-3 days per month? *
   - Yes
   - No

3. Have you had this discomfort or pain for more than 6 months? *
   - Yes
   - No

4. Would this discomfort or pain get better or stop after having a bowel movement, more than 50% of the time? *
   - Yes
   - No

5. When this discomfort or pain started, did your bowel movements change in frequency? *
   - Yes
   - No

6. When this discomfort or pain started, did your bowel movements change in consistency? *
   - Yes
   - No

7. In the last 3 months, have you had hard or lumpy stools more than 50% of the time? *
   - Yes
   - No
1. I am walking well.
2. I am independent in my ability to move around.
3. I have good balance.
4. My speech is clear.
5. My vision is clear.
6. My hearing is intact / sharp.
7. My nerves are working well.
8. I am free of pain.
9. I am a happy person.
10. I sleep well.
11. I eat on my own.
12. My digestive tract works well.
13. My bladder works well.
15. I am hopeful about my future health.
# New Member Survey

What should researchers **focus on next?**

<table>
<thead>
<tr>
<th></th>
<th>Important</th>
<th>Somewhat important</th>
<th>Not important</th>
<th>Not applicable</th>
</tr>
</thead>
<tbody>
<tr>
<td>My walking speed</td>
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<tr>
<td>My balance</td>
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<td>My speech</td>
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<tr>
<td>My swallowing</td>
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<tr>
<td>My energy level</td>
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<tr>
<td>My comfort / pain</td>
<td></td>
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<td>My adrenal dysfunction</td>
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</tbody>
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**Other**

*Please enter here!*

**Please, provide additional comments:**

---

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How can providers improve their care? What services do you need?

<table>
<thead>
<tr>
<th>Service</th>
<th>Important</th>
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<th>Not important</th>
<th>Not applicable</th>
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<tbody>
<tr>
<td>Coordination of care (specialties working together)</td>
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<td>Managing costs of healthcare</td>
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<tr>
<td>Specialists in ALS: neurologists, endocrinologists, experts in bone marrow transplantation</td>
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<tr>
<td>Additional services: physical therapy, speech and swallow therapy</td>
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<tr>
<td>Distance and transport to healthcare providers</td>
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<tr>
<td>Other</td>
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</tbody>
</table>

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Please, provide additional comments:
Access to what **resources** would help you most?

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<tr>
<th>Important</th>
<th>Somewhat important</th>
<th>Not important</th>
<th>Not applicable</th>
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<tbody>
<tr>
<td>Access to diagnostic labs (prenatal, postnatal, newborn screening, mutation testing for women)</td>
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<td>Access to new treatments</td>
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<td>Access to new mobility devices</td>
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<tr>
<td>Access to non-medical resources (e.g. fellowship, meetups, financial sponsorship, etc)</td>
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<td>Access to dietary information</td>
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<td>Access to exercise regimen</td>
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<tr>
<td>Other</td>
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</table>

Please enter here!

Please, provide additional comments:
### Patient Perspective - NeuroTracker - Adult

#### My Bladder
- I feel urgency and/or hesitancy: 31
- I have no control over my bladder: 2
- I occasionally have accidents: 40
- My bladder works well: 21

#### My Vision
- I can only see things immediately in front of me: 4
- I can tell light and dark apart: 5
- I see well: 84

#### My Digestion
- I have occasional accidents that completely restrict daily activity: 1
- I have occasional accidents that partially restrict daily activity: 25
- I have occasional diarrhea or constipation: 45
- My digestive tract works well: 27

#### My Language
- I cannot speak: 1
- I often have difficulty speaking: 7
- I sometimes have difficulty speaking: 16
- I speak clearly: 73

#### My Mood
- I am a happy person: 57
- I have extreme mood swings: 20
- My mood interferes with daily activity (e.g. employment): 16

#### My Pain
- I am free of pain: 21
- I have occasional pain: 50
- My pain interferes with daily activity: 21
- My pain is as bad as you can imagine: 1

#### My Hearing
- I cannot hear: 1
- I hear well: 68
- I often have difficulty hearing: 7
- I sometimes have difficulty hearing: 17

#### My Language
- I am in control of my health: 92

#### My Sensation
- I have decreased sensation below the hip: 23
- I have decreased sensation below the knee: 45
- I have decreased sensation in the entire leg or any body part below the head: 3
- I have normal sensation: 24

#### My Movements
- I am occasionally unsteady: 64
- I am wheelchair-bound: 6
- I generally walk well: 22
- I require a wheelchair: 14

#### My Balance
- I can stand with my eyes closed and feet apart: 14
- I can stand with my eyes closed and feet together: 36
- I can stand with my eyes open and feet apart: 20
- I can stand with my eyes open and feet together: 20

#### My Independence
- I have limited restrictions: 15
- I perform activities of daily living on my own: 53
- I require constant assistance: 3
- I require occasional assistance: 30

#### My Spasms
- I am free of muscle spasms: 21
- I have occasional spasms but do not require medicine: 33
- I have spasms that are well controlled on medicine: 21
- My spasms are not well controlled on medicine: 21

#### My Feeding
- I choke occasionally: 11
- I eat without difficulty: 78
- I have modified my food / liquid intake to accommodate and require assistance: 1

#### My Vision
- I cannot see things immediately in front of me: 4
- I can tell light and dark apart: 5
- I see well: 84

#### My Movements
- I am in control of my health: 92
Patients == Research Participant
Patients == Research Participant
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Patients == Research Participant
Bright Future of International Collaboration

• NeuroGUIDization of PALS community

• International NeuroData Authority

• Incentives: Data Contributors vs. Consumers, or WIIFM
  • What’s In It For Me
  • Publications/Co-authorship
  • Data References/Quotations
  • Market (N-euro)

• Legal Matters
  • Data ownership, embargo dates and other matters
  • Important three-letter worlds: DUA, MTA, ICF, IRB/ERB, DCC
  • How to get data from NeuroBANK™, yours and others

• Natural Histories in the Age of Precision

• For future NeuroBANKers
  • How to join the NeuroBANK™ ecosystem
  • You must be kidding – no way it could be free of charge! Well, it could be.
  • How to Start a NeuroBANK™ Project in a Week
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