

Ataxia Global Initiative

Trial Site Registry

(AGI-TSR)

Dataset

Contact Details

Site name
Address
Zip-Code / City
Country
Homepage
PI

- Title
- First name
- Last name
- E-Mail
- Phone

Patient Population

Genetic ataxia subtypes

In the fields underneath please fill in data and information regarding the cohort of ataxia patients you regularly see, e.g. at annual visits. Please be as accurate as possible.

Disease name	Patients presents yes or no	Age groups 0-17 or 18+	Ambulatory (number)	Consented to be contacted for clinical trials (both age groups)
autosomal-dominant ataxias (SCAs)				
repeat SCAs				
SCA1				
SCA2				
SCA3				
SCA6				
SCA7				
SCA17				

SCA27b				
DRPLA				
channelopathies				
KCNA1 (Episodic ataxia type 1)				
CACNA1A (Episodic ataxia type 2)				
SCA19				
SCA29				
neurometabolic				
Alexander disease				
other SCAs				
<i>open entry field</i>				
Autosomal-recessive and x-linked ataxias				
frequent ataxias (frequent, drugs in development and/or strong NHS consortia which will make trial-readiness in close future likely)				
Friedreich's Ataxia (FA)				
Ataxia Telangiectasia (AT)				
Autosomal-recessive spastic ataxia type Charlevoix Saguenay (ARSACS)				
RFC1				
mitochondrial (recurrent, drugs in development drugs in development and/or strong NHS consortia which will make trial-readiness in close future likely)				
SPG7				
COQ8A				
neurometabolic				
Niemann Pick Type C (NPC)				
Tay-Sachs Disease				
Sandhoff Disease				
Gaucher disease type 3				
Cerebrotendinous xanthomatosis (CTX)				
Adrenoleukodystrophy (ALD)				
Spastic ataxia associated with GBA2				

Clinical Trials – Experience

Qualified for GCP (Good Clinical Practice) with documented certifications:

- number of clinicians
- number of study nurses

Number of clinical trials in which the site PI team participated in the last 5 years

- | | | |
|-------------|-------------------------------|-----------------|
| • Phase I | (a) for neurological diseases | (b) for ataxias |
| • Phase II | (a) for neurological diseases | (b) for ataxias |
| • Phase III | (a) for neurological diseases | (b) for ataxias |
| • Phase IV | (a) for neurological diseases | (b) for ataxias |

Clinical Trials – Equipment

1. Certified Laboratory
2. Centrifuge
3. Ice
4. Dry ice
5. Liquid nitrogen
6. Refrigerator lockable/not lockable with the following temperatures:
 - -80°C(-112°F)
 - -20°C (-4°F)
 - 2-8°C(36°F-46,4°F)
7. Lockable rooms or filling cabinets
8. Lockable safes to keep study drugs
9. Temperature recorder for the fridges and safes
10. Possibility for conducting trials in inpatient care setting
11. Dedicated clinical trials unit for children and/or adults
12. Emergency care unit
13. resuscitation team at the study site
14. MRI (at least 1,5 Tesla)
15. Pharmacy that is able to handle GMP grade drugs for trials
16. Possibility *and* previous experience with intrathecal administration of drugs
 - In clinical care setting
 - In trial setting