

Research in MSA

Dysautonomia Center

We are moving forwards

Clinical trials are active and new ones are coming

Knowledge is building

MSA is being recognized sooner

Where are we now?



Population of MSA patients

**Feel better
Progress less fast**



**Drug
Development**



Academic clinicians



Pharma partners



This network of patients, investigators, and partners in the pharmaceutical industry allows us to have a pipeline for treatments to enter the clinic.

We are moving forwards

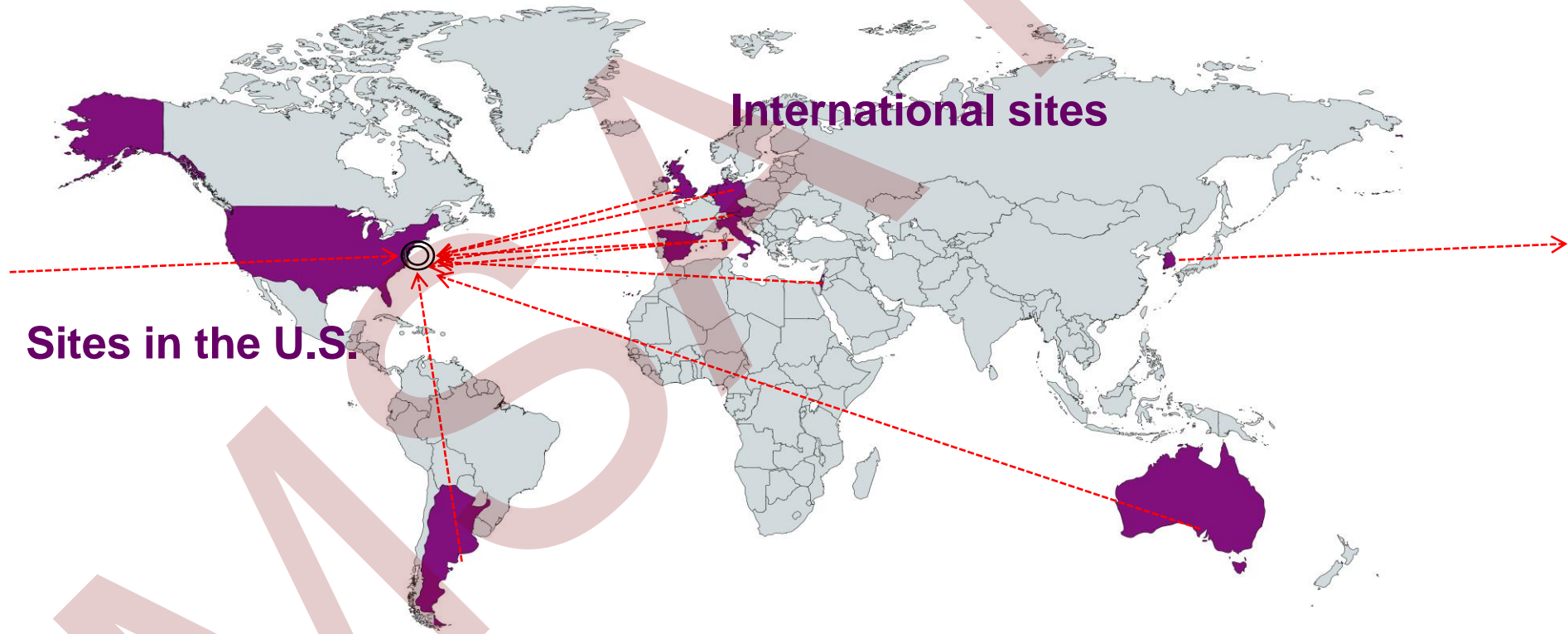
The availability of several potential therapies to prevent or delay MSA is an important advancement in the field

The success of future clinical trials hinges upon having high quality natural history data and sensitive ways to measure change (surveys, biomarkers)

We need to be ready for clinical trials

An established collaborative framework that unites academic research institutions with a specific interest in following patients with MSA

MSA Investigator Team (sites worldwide)



What hurdles do we need to overcome?



- Have many more patients participate (large numbers)
- Improve diagnostic precision in the prodromal phase of MSA
- Find sensitive biomarkers to measure short term changes (scales, PRO, biomarkers)
- Provide an educational program to train fellows in rare disease research



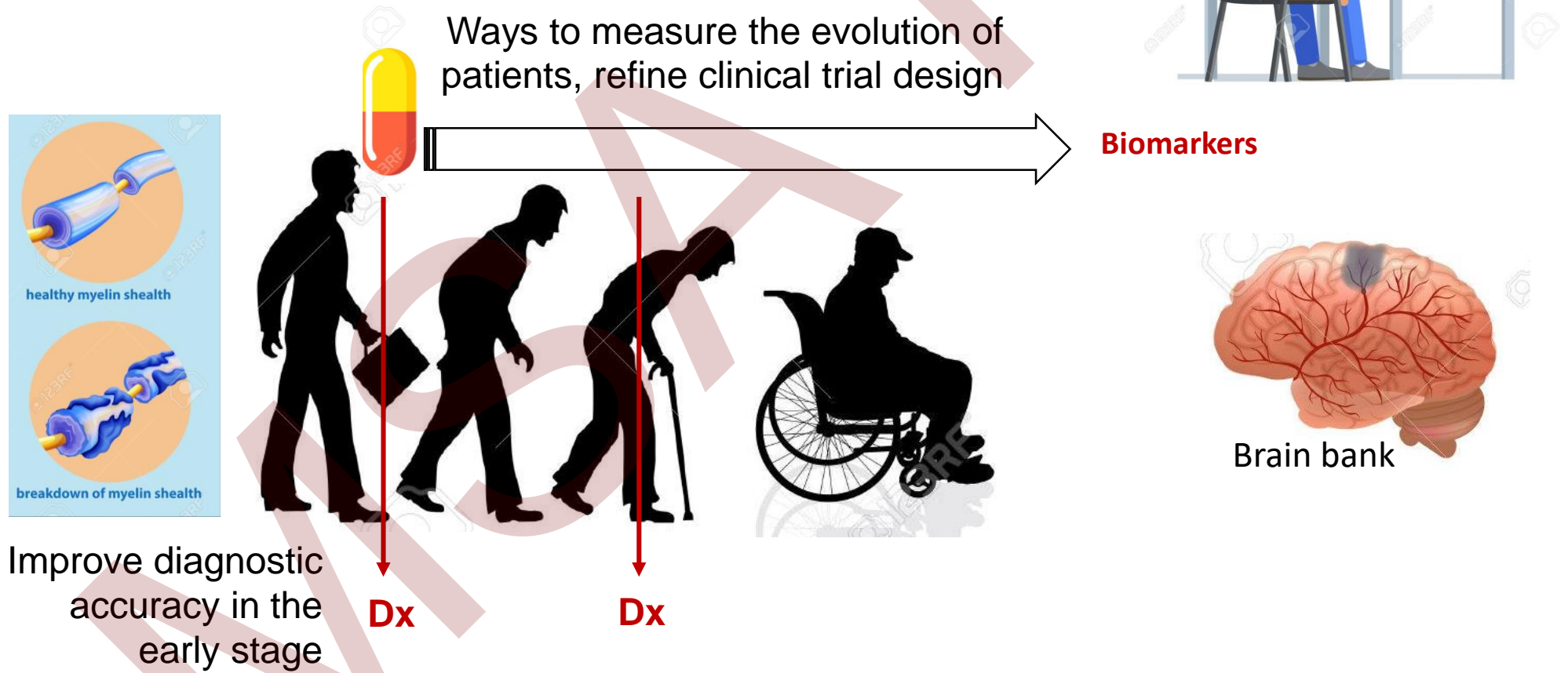
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- 1) How many of you thought that it took too long to be diagnosed with MSA?
- 2) How many of you would like better treatments for MSA?
- 3) How many of you wished more doctors knew about MSA?

What is a natural history study?

Observational study (not a clinical trial)

Follows the evolution of patients



Ways to measure the evolution of patients, refine clinical trial design

Biomarkers

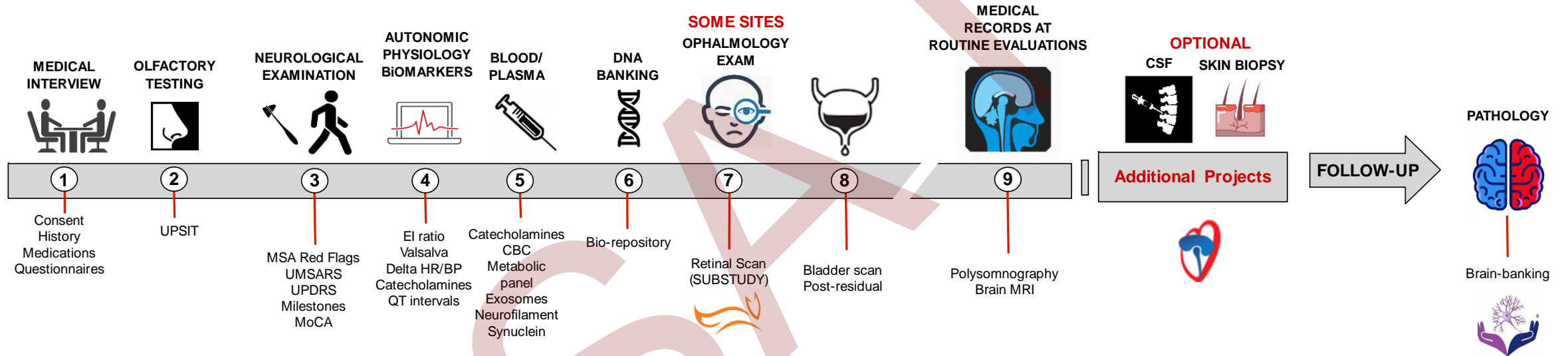
Brain bank

Improve diagnostic accuracy in the early stage

Dx

Dx

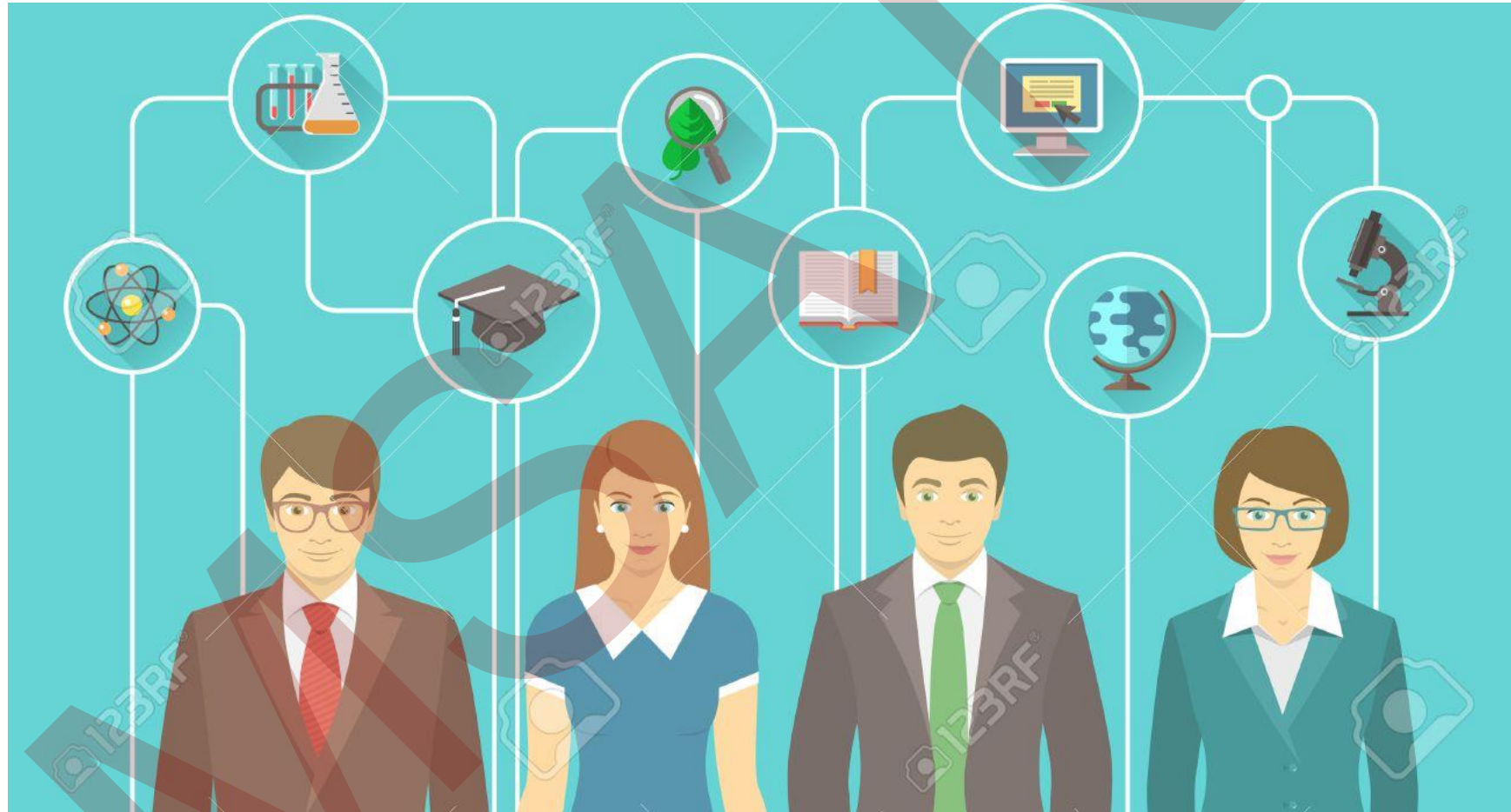
How does a study visit look?



We are moving forwards

- We are helping to be ready for clinical trials
- We are sharing data/samples/images to help other researchers
- We are using this benchmark information to help drug development and drug approval
- We can plan better trials with the best chance of success
- If we can prove a new drug slows the progression, we can get it approved faster

Seize the opportunity to use the collaboration we have built



What can you do now?

Participate



Anyone with MSA can be part of the natural history study

Stay connected



**Share information about
clinical trial opportunities**

Encourage others to enroll

When did you start with

Less arm swing?

RBD?

**Help us train
new doctors**

Falling when turning?

Falling out of bed?



MSA ?

Ataxia?

Screaming at night?

**Share your MSA story with fellows
(Neurologists, Urologists, Sleep doctors,
Autonomic doctors, Movement disorder
doctors, ENT)**

Fainting after meals?

nOH?

Erectile dysfunction?

Laughing/crying?

Peeing 3 times/night?

Urinary urgency?

Hoarseness?

Stay hopeful



Thank you

MSSA NU